UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 10-K

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended December 31, 2022

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from ____ to _____

Commission File No. 001-36913

Zevra Therapeutics, Inc.
(Exact Name of Registrant as Specified in Its Charter)

Delaware	**20-5894398**
(State or Other Jurisdiction of Incorporation or Organization)	**(I.R.S. Employer Identification No.)**
1180 Celebration Boulevard, Suite 103, Celebration, FL 34747	**(321) 939-3416**
(Address of Principal Executive Offices and Zip Code)	**(Registrant's Telephone Number, Including Area Code)**

Securities registered pursuant to Section 12(b) of the Act:

Title of Each Class	**Trading Symbol**	**Name of Each Exchange on Which Registered**
Common Stock, $0.0001 par value	**ZVRA**	**The Nasdaq Stock Market LLC (Nasdaq Global Select Market)**

Securities registered pursuant to Section 12(g) of the Act: None

Indicate by check mark if the Registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act Yes ☐ No ☒

Indicate by check mark if the Registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act Yes ☐ No ☒

Indicate by check mark whether the Registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the Registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.:

Large accelerated filer ☐ Accelerated filer ☐ Non-accelerated filer ☒ Smaller reporting company ☒ Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☐

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to § 240.10D-1(b). ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

The aggregate market value of the voting and non-voting common equity held by non-affiliates of the registrant as of June 30, 2022, the last business day of the registrant's most recently completed second fiscal quarter, was approximately $152.9 million, based upon the closing sales price for the registrant's common stock, as reported on the Nasdaq Global Select Market on June 30, 2022. The calculation of the aggregate market value of voting and non-voting common equity excludes 210,287 shares of common stock the registrant held by executive officers, directors and stockholders that the registrant concluded were affiliates of the registrant on that date. Exclusion of such shares should not be construed to indicate that any such person possesses the power, direct or indirect, to direct or cause the direction of management or policies of the registrant or that such person is controlled by or under common control with the registrant.

As of March 3, 2023, the registrant had 34,540,414 shares of common stock outstanding.

Documents Incorporated by Reference

Portions of the registrant's definitive proxy statement relating to its 2023 annual meeting of stockholders are incorporated by reference into Part III of this Annual Report on Form 10-K where indicated. Such proxy statement will be filed with the U.S. Securities and Exchange Commission within 120 days after the end of the fiscal year to which this report relates.

ZEVRA THERAPEUTICS, INC.
FORM 10-K

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K, including the section entitled "Management's Discussion and Analysis of Financial Condition and Results of Operations," contains forward-looking statements regarding future events and our future results that are subject to the safe harbors created under the Securities Act of 1933, as amended, or the Securities Act, and the Securities Exchange Act of 1934, as amended, or the Exchange Act. Forward-looking statements relate to future events or our future financial performance. We generally identify forward-looking statements by terminology such as "may," "will," "would," "should," "expects," "plans," "anticipates," "could," "intends," "target," "projects," "contemplates," "believes," "estimates," "predicts," "assume," "intend," "potential," "continue" or other similar words or the negative of these terms. We have based these forward-looking statements largely on our current expectations about future events and financial trends that we believe may affect our business, financial condition and results of operations. The outcome of the events described in these forward-looking statements is subject to risks, uncertainties and other factors described in "Risk Factors" and elsewhere in this report. Accordingly, you should not place undue reliance upon these forward-looking statements. We cannot assure you that the events and circumstances reflected in the forward-looking statements will be achieved or occur, the timing of events and circumstances and actual results could differ materially from those anticipated in the forward-looking statements. Forward-looking statements contained in this report include, but are not limited to, statements about:

- *the progress of, outcome or and timing of any regulatory approval for any of our product candidates and the expected amount or timing of any payment related thereto under any of our collaboration agreements;*
- *the progress of, timing of and expected amount of expenses associated with our research, development and commercialization activities;*
- *our ability to raise additional funds on commercially reasonable terms, or at all, in order to support our continued operations;*
- *the sufficiency of our cash resources to fund our operating expenses and capital investment requirements for any period;*
- *the expected timing of our clinical trials for our product candidates and the availability of data and results of those trials;*
- *our expectations regarding federal, state and foreign regulatory requirements;*
- *the potential therapeutic benefits and effectiveness of our products and product candidates;*
- *the size and characteristics of the markets that may be addressed by our products and product candidates;*
- *our intention to seek to establish, and the potential benefits to us from, any strategic collaborations or partnerships for the development or sale of our products and product candidates;*
- *our expectations as to future financial performance, expense levels and liquidity sources;*
- *the timing of commercializing our products and product candidates, if approved; and*
- *other factors discussed elsewhere in this report.*

The forward-looking statements made in this report relate only to events as of the date on which the statements are made. We have included important factors in the cautionary statements included in this report, particularly in the section entitled "Risk Factors" that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make. Except as required by law, we do not assume any intent to update any forward-looking statements after the date on which the statement is made, whether as a result of new information, future events or circumstances or otherwise.

RISK FACTORS SUMMARY

The risk factors summarized below could materially harm our business, operating results, and/or financial condition, impair our future prospects, and/or cause the price of our common stock to decline. These risks are discussed more fully in the section titled "Risk Factors". Material risks that may affect our business, financial condition, results of operations, and trading price of our common stock include the following:

- *Our research and development activities are focused on discovering and developing proprietary prodrugs, and we are taking an innovative approach to discovering and developing prodrugs, which may never lead to additional marketable prodrug products.*
- *If we are not able to obtain required regulatory approvals for our product candidates, or the approved labels are not sufficiently differentiated from other competing products, we will not be able to commercialize them and our ability to generate revenue or profits or to raise future capital could be limited.*
- *If commercialization of our approved products or our product candidates is not successful, or we experience significant delays in commercialization, our business will be harmed.*
- *Arimoclomol is currently available to Niemann-Pick disease Type C patients in the United States. France, Germany, and other European Union countries through our early access program, or EAP. The EAP is expected to remain in place until arimoclomol becomes commercially available in each of the current EAP markets. If the EAP is terminated prior to commercialization of arimoclomol, it will have a material adverse effect on our business, results of operations, cash flows, financial condition and/or prospects.*
- *Clinical drug development involves a lengthy and expensive process, with an uncertain outcome. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.*
- *We may need substantial additional funding to pursue our business objectives. If we are unable to raise capital when needed, we could be forced to delay, reduce or altogether cease our prodrug development programs or commercialization efforts or cease operations altogether.*
- *We have incurred significant recurring negative net operating losses since our inception. We expect to incur operating losses over the next several years and may never achieve or maintain profitability.*
- *If we are unable to obtain and maintain trade secret protection or patent protection for our technology, our approved products or our product candidates, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize technology and drugs similar or identical to ours, and our ability to successfully commercialize our technology, our approved products and our product candidates, if approved, may be impaired.*
- *If we attempt to rely on Section 505(b)(2) of the Federal Food, Drug and Cosmetic Act and the FDA does not conclude that our product candidates are sufficiently bioequivalent, or have comparable bioavailability, to approved drugs, or if the FDA does not allow us to pursue the 505(b)(2) NDA pathway as anticipated, the approval pathway for our product candidates will likely take significantly longer, cost significantly more and entail significantly greater complications and risks than anticipated, and the FDA may not ultimately approve our product candidates.*
- *The FDA may determine that any NDA we may submit under the 505(b)(2) regulatory pathway for any of our product candidates in the future is not sufficiently complete to permit a substantive review.*
- *We have entered into collaborations with Commave Therapeutics, S.A., or Commave, to develop, manufacture and commercialize AZSTARYS worldwide and KVK-Tech, Inc., or KVK, to manufacture and commercialize APADAZ in the United States. In addition, we may seek collaborations with third parties for the development, manufacturing or commercialization of our other product candidates, or in other territories. If those collaborations are not successful, we may not be able to capitalize on the market potential of our approved products or other product candidates, if approved.*
- *The trading price of the shares of our common stock is likely to be volatile, and purchasers of our common stock could incur substantial losses.*

NOTE REGARDING COMPANY REFERENCE

Unless the context otherwise requires, we use the terms "Zevra," "Company," "we," "us" and "our" in this Annual Report on Form 10-K to refer to Zevra Therapeutics, Inc., formerly known as KemPharm, Inc. prior to February 21, 2023. We have proprietary rights to a number of trademarks used in this Annual Report on Form 10-K that are important to our business, including LAT® and the Zevra logo. All other trademarks, trade names and service marks appearing in this Annual Report on Form 10-K are the property of their respective owners. Solely for convenience, the trademarks and trade names in this Annual Report on Form 10-K are referred to without the ® and ™ symbols, but such references should not be construed as any indicator that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto.

NOTE REGARDING MARKET AND INDUSTRY DATA

This Annual Report on Form 10-K contains statistical data, estimates and forecasts that are based on independent industry publications or other publicly available information, as well as other information based on our internal sources. While we believe the industry and market data included in this Annual Report on Form 10-K is reliable and is based on reasonable assumptions, this data involves many assumptions and limitations, and you are cautioned not to give undue weight to these estimates. We have not independently verified the accuracy or completeness of the data contained in these industry publications and other publicly available information. The industry in which we operate is subject to a high degree of uncertainty and risk due to a variety of factors, including those described in the section titled "Risk Factors" and "Special Note Regarding Forward-Looking Statements" included in this Annual Report on Form 10-K.

PART I

ITEM 1. BUSINESS.

Overview

We are a rare disease company melding science, data and patient need to create transformational therapies for diseases with limited or no treatment options. With unique, data-driven clinical, regulatory, and commercialization strategies, we are overcoming complex drug development challenges to bring much-needed therapies to patients. We have a diverse portfolio of products and product candidates, which includes a combination of both a clinical stage pipeline and commercial stage assets. The Company's pipeline includes arimoclomol, an orally-delivered, first in-class investigational product candidate being developed for Niemann-Pick disease Type C, or NPC, which has been granted orphan drug designation, Fast-Track designation, Breakthrough Therapy designation and rare pediatric disease designation for the treatment of NPC by the U.S. Food and Drug Administration, or FDA, and orphan medicinal product designation for the treatment of NPC by the European Medicines Agency, or EMA .KP1077, is our lead clinical development product candidate, which is being developed as a treatment for idiopathic hypersomnia, or IH, a rare neurological sleep disorder, and narcolepsy. KP1077 is comprised solely of serdexmethylphenidate, or SDX, our proprietary prodrug of d-methylphenidate, or d-MPH. The FDA has granted KP1077 orphan drug designation for the treatment of IH, and the U.S. Drug Enforcement Agency, or DEA, has classified SDX as a Schedule IV controlled substance based on evidence suggesting SDX has a lower potential for abuse when compared to d-MPH, a Schedule II controlled substance.

We have specialized expertise and a track record of success in advancing promising therapies that face complex clinical and regulatory challenges with an approach that balances science and data with patient need. The FDA, has approved AZSTARYS®, formerly referred to as KP415, a once-daily treatment for attention deficit hyperactivity disorder, or ADHD, in patients age six years and older containing our prodrug, SDX, and d-MPH. In September 2019, we entered into a collaboration and license agreement, or the AZSTARYS License Agreement, with Commave Therapeutics SA (formerly known as Boston Pharmaceutical S.A.), or Commave, an affiliate of Gurnet Point Capital, L.P. Under the AZSTARYS License Agreement, we granted to Commave an exclusive, worldwide license, to develop, manufacture, and commercialize AZSTARYS and any of our product candidates containing SDX and used to treat ADHD or any other CNS disease. Commave has tasked Corium, Inc., or Corium, an affiliate of Gurnet Point Capital, L.P., to lead all commercialization activities for AZSTARYS in the U.S. under the AZSTARYS License Agreement. Corium commercially launched AZSTARYS in the U.S. during the third quarter of 2021. In December 2021, Commave sublicensed commercialization rights for AZSTARYS in greater China to Shanghai Ark Biopharmaceutical Ltd. The FDA has also approved APADAZ®, an immediate-release combination product containing benzhydrocodone, our prodrug of hydrocodone, and acetaminophen, for the short-term (no more than 14 days) management of acute pain severe enough to require opioid analgesic and for which alternative treatments are inadequate. In October 2018, we entered into a collaboration and license agreement, or the APADAZ License Agreement, with KVK-Tech, Inc., or KVK, under which we granted to KVK the exclusive license to manufacture and commercialize APADAZ in the U.S.

Our primary mission is to deliver life-changing treatments to people with rare conditions, their families, and caregivers who desperately need better options. This mission guides our efforts to expand our pipeline through both internal development and through our business development activities to collaborate, partner, and potentially acquire additional assets. We intend to target assets that will allow us to leverage the expertise and infrastructure that we have successfully built in order to mitigate risk and enhance our probability of success. In addition, we are considering external opportunities within neurology and neurodegenerative diseases, psychiatric disorders, and other rare diseases, along with adjacent or related therapeutic categories. We are seeking assets that are undergoing Phase 2 clinical trials or Phase 3 clinical trials, subject to our specific evaluation criteria, that we can in-license or acquire. If we are successful, expanding our development pipeline could be accretive to our value proposition by potentially adding new clinical data catalysts and have the potential to create incremental long-term value for stockholders. In addition, we believe that a multi-channel development program with several product candidates addressing various rare disease indications will diversify risk and potentially create an impactful portfolio of commercial-stage products in the future.

For example, in May 2022, we, through our newly formed wholly-owned subsidiary, Zevra Denmark A/S (formerly known as KemPharm Denmark A/S prior to February 21, 2023), or Zevra DK, entered into an Asset Purchase Agreement, or the Arimoclomol Purchase Agreement, with Orphazyme A/S in restructuring, a Danish public limited liability company, or Orphazyme. The transactions agreed to under the Arimoclomol Purchase Agreement closed on May 31, 2022. Under the terms of the Arimoclomol Purchase Agreement, Zevra DK purchased all of the assets and operations of Orphazyme related to arimoclomol and settled all of Orphazyme's actual outstanding liabilities to its creditors with a cash payment of $12.8 million. In addition, Zevra DK agreed to assume an estimated reserve liability of $5.2 million related to revenue generated from Orphazyme's Early Access Program in France, or the Arimoclomol EAP.

Our most advanced product candidate, arimoclomol, is being developed for the treatment of NPC, a lysosomal storage disease, or LSD. NPC is a rare neurodegenerative disease characterized by an inability of the body to transport cholesterol and lipids inside of cells. Symptoms of NPC include a progressive impairment of mobility, cognition, speech, and swallowing, often culminating in premature death. The incidence of NPC is estimated to be one in 100,000 live births. We estimate that approximately 1,500 individuals have been diagnosed, of which approximately 300 are in the United States and approximately 1,200 are in Europe. However, diagnostic challenges may affect the number of potential patients, and we believe that the availability of treatment options could increase awareness of the disease and assist in identifying more cases. Therapies to treat NPC are desperately needed, and for this reason, arimoclomol is currently being made available to NPC patients in the United States, France, Germany, and other European Union countries under various early access programs, or EAPs.

On September 16, 2020, the previous sponsor of the arimoclomol program, Orphazyme, submitted a new drug application, or NDA, seeking approval for arimoclomol to treat NPC. In June 2021, the FDA issued a complete response letter, or CRL, which means the FDA determined that it could not approve the NDA in its present form. Our aim is to prepare and resubmit an NDA that presents meaningful evidence of safety and efficacy of arimoclomol for its intended use. To that end, we are continuing to work diligently to characterize the substantial data generated since the CRL, including the recently completed four-year open-label safety trial which was recently presented at the 19th World*Symposium*™ in February 2023. Results from this analysis, based on up to four years of continuous treatment, suggested that arimoclomol may reduce the long-term progression of NPC. Upon fulfilling the randomized double-blinded portion of the Phase 2/3 clinical trial, both placebo- and arimoclomol-treated patients were given the option to continue into the four-year (48 month) open-label-extension, or OLE, phase of the study with arimoclomol treatment provided in addition to their current standard of care. Progression of NPC disease through the DB and OLE phases was assessed utilizing the five-domain NPC Clinical Severity Scale (5DNPCCSS) and compared with an estimated progression calculated from the combination of untreated patients from the NPC-001 observational trial and placebo patients from the NPC-002 Phase 2/3 trial. We are also investigating correlations between relevant 5DNPCCSS domains and corresponding Scale for the Assessment and Rating of Ataxia, or SARA, test items to potentially provide further supportive evidence for 5DNPCCSS validity as a tool for evaluating NPC progression. The SARA test evaluates impairment related to cerebellar ataxia, which was a secondary endpoint in the Phase 2/3 clinical trial of arimoclomol in NPC (NPC progression based on the 5DNPCCSS was the primary endpoint). Based on a comparative analysis of both measurements, it was determined that individual 5DNPCCSS domains and relevant performance-based SARA test items showed strong associations and alignment between the two instruments for all analysis methods used. These results

provided further support that the evaluated 5DNPCCSS domains are appropriately standardized to allow for reliable and reproducible scoring of disease severity in NPC. We plan to include these data as part of the updated NDA for arimoclomol, which is expected to resubmitted to the FDA as early as the third quarter of 2023.

We also intend to advance our pipeline of prodrug product candidates for the treatment of IH and other CNS/rare diseases, and we reported top-line data from a Phase 1 proof-of-concept study of SDX in the fourth quarter of 2021 and final data for the Phase 1 proof-of-concept study of SDX in the first quarter of 2022. The proof-of-concept study was a dose-escalation study to evaluate the pharmacokinetics, pharmacodynamic stimulant effects, and safety of single oral doses of SDX in subjects with a history of high-dose stimulant use. In the trial, 240 mg and 360 mg doses of SDX were observed to be well-tolerated and produced d-MPH exposure that appeared to increase proportionally with dose. Mean d-MPH plasma concentrations showed a gradual increase after SDX administration, reaching a broad peak from eight to twelve hours post-dose, followed by a shallow decline thereafter. Increased wakefulness, alertness, hypervigilance, and insomnia effects were reported by study participants, which we believe suggests that SDX produced targeted pharmacodynamic effects that have the potential to benefit patients with IH and other sleep disorders. On November 18, 2022, we announced that the FDA has granted the Orphan Drug Designation to SDX for the treatment of IH.

In January 2022, we announced that we have selected KP1077 for the treatment of IH and narcolepsy as our lead clinical development candidate. KP1077 utilizes SDX, our prodrug of d-MPH, as its active pharmaceutical ingredient. During the first quarter of 2022, we initiated a Phase 1 clinical trial comparing the cardiovascular safety of SDX to immediate-release and long-acting formulations of RITALIN®, a commonly prescribed CNS stimulant. In September 2022, we announced topline data from our exploratory Phase 1 clinical trial, which showed the potential for higher dose formulations of SDX to be safe and well tolerated while avoiding the potential for greater cardiovascular safety risk compared to immediate-release and long-acting formulations of Ritalin. Based on the data, we have identified initial dosing strengths for the planned Phase 2 clinical trial of KP1077 which we believe have the potential to be well-tolerated while providing higher overall exposures to d-MPH compared to other methylphenidate products that are often used off-label as a treatment for IH. In addition, on December 21, 2022, we announced the initiation of a Phase 2 clinical trial evaluating KP1077. The Phase 2 clinical trial is a double-blind, placebo-controlled, randomized-withdrawal, dose-optimizing, multi-center study evaluating the efficacy and safety of KP1077 for the treatment of IH. We expect to enroll approximately 48 adult patients with IH in more than 30 centers in the United States. Part 1 of the trial will consist of a five-week open-label titration phase during which patients will be optimized to one of four doses of SDX (80, 160, 240, or 320 mg/day). Part 2 of the trial will entail a two-week randomized, double-blind, withdrawal phase, during which two-thirds of the trial participants will continue to receive their optimized dose while the remaining one-third will receive placebo. Participants will be further assigned into two evenly divided cohorts. The first cohort will receive a single daily dose just before bedtime, and the second cohort will receive half the daily dose shortly after awakening and half the daily dose prior to bedtime. Interim efficacy and safety data for the Phase 2 trial is expected as early as the third quarter of 2023. We anticipate that we will submit the IND for KP1077 for the treatment of narcolepsy as early as the second quarter of 2023, and subsequently begin a trial in narcolepsy soon after interim data from the Phase 2 IH clinical trial is available.

In May 2021, we announced that SDX, our proprietary prodrug of d-MPH and the primary active pharmaceutical ingredient, or API, in AZSTARYS, was classified as a Schedule IV controlled substance by the DEA. AZSTARYS is classified as a Schedule II controlled substance as its formulation includes a 70:30 mixture of SDX (Schedule IV) and d-MPH (Schedule II), respectively.

Our Proprietary Ligand Activated Therapy Platform Technology

We employ our proprietary Ligand Activated Therapy, or LAT® , platform technology to discover and develop prodrugs that are new molecules that may potentially improve one or more of the attributes of approved drugs, such as enhanced bioavailability, extended duration of action, increased safety and reduced susceptibility to abuse. A prodrug is a precursor chemical compound of a drug that is inactive or less than fully active, which is then converted in the body to the active form of the drug through a normal metabolic process. Where possible, we seek, to develop prodrugs that will be eligible for approval under Section 505(b)(2) of the Federal Food, Drug and Cosmetic Act, or the FFDCA, otherwise known as a 505(b)(2) NDA, which allows us to submit an NDA that relies on the FDA's previous findings of safety and effectiveness for one or more approved products, if we demonstrate such reliance is scientifically appropriate.

We believe that our proprietary LAT platform technology offers the following potential benefits:

- ***Improved drug properties.*** We seek to discover and develop prodrugs that are new molecules with potentially improved attributes over FDA-approved drugs, such as enhanced bioavailability, extended duration of action, increased safety and reduced susceptibility to abuse.

- ***Composition-of-matter patent protection.*** Our prodrugs are new molecules and thus may be eligible for patent protection as novel compositions of matter, provided that all other applicable requirements are met. We seek patent protection not only for our product candidates, but also for related compounds with the intention of creating potential heightened barriers to market entry.

- ***Eligibility for 505(b)(2) NDA pathway.*** Our proprietary LAT platform technology allows us to discover and develop prodrugs that may be eligible to use the 505(b)(2) NDA pathway. Under that regulatory pathway, if we are able to provide an adequate bridge between our product candidates and appropriate FDA-approved drugs, we will then be able to reference the FDA's previous findings of safety and effectiveness of the approved drugs in our 505(b)(2) NDA submissions. This approach may allow us to avoid some of the significant time and expense of conducting large clinical trials and potentially eliminate the need for some preclinical activities.

We continue to seek opportunities to expand our pipeline of product candidates through our business development efforts, and by employing our LAT platform technology and development expertise to develop additional product candidates that address significant unmet medical needs in therapeutic indications which have few existing product options. We believe our prodrug product candidates may be eligible for composition-of-matter patent protection and we intend to use the 505(b)(2) NDA pathway when available, which we believe has the potential to reduce drug development time and expense.

Our Product Candidates and Approved Products

We have employed our proprietary LAT platform technology to create a portfolio of approved products that we believe will offer, and product candidates that we believe have the potential to offer, significant improvements over currently available FDA-approved drugs.

A selection of our product candidates and approved products are summarized in the table below:

Selected Zevra Partnered and Other Development Assets

Parent Drug (Effect Profile) - Indication	Product Candidate	Development Status	Next Milestone(s)
Arimoclomol (ER) - NPC	Arimoclomol	NDA Preparation	NDA Submission - as early as Q3 2023
Methylphenidate (ER) - IH	KP1077H*	Clinical - Phase 2	Pivotal Phase 2 Trial ongoing - interim results as early as Q3 2023
Methylphenidate (ER) - Narcolepsy Types I and II	KP1077N*	Clinical - Phase 1/2	IND filing as early as Q2 2023
Methylphenidate (ER) - SUD	KP879	Clinical - Phase 2	Awaiting results of NIH grant application
Methylphenidate (ER) - ADHD	AZSTARYS	FDA Approved/Partnered	Tracking TRx's
Hydrocodone / APAP (IR) - Pain	APADAZ	FDA Approved/Partnered	Tracking Payor Contracts and TRx's

* This product candidate is subject to a right of first negotiation upon completion of a Phase 1 proof-of-concept study in favor of Commave under the terms of the AZSTARYS License Agreement, but is not currently licensed to Commave, thereunder.

These anticipated milestones are based on information currently available to us. Our current plans and expectations are subject to a number of uncertainties, risks and other important factors that could materially impact our plans, including risks which are not solely within our control. See Part I, Item 1A. “Risk Factors” in this Annual Report on Form 10-K.

Arimoclomol

Arimoclomol is an orally-delivered, first-in-class investigational product candidate being developed as a treatment for NPC. NPC is a rare neurodegenerative disease characterized by an inability of the body to transport cholesterol and lipids inside of cells. Symptoms of NPC include a progressive impairment of mobility, cognition, speech, and swallowing, often culminating in premature death. The incidence of NPC is estimated to be one in 100,000 live births. Based on these incidence rates, the number of NPC patients in the United States, France, Germany, and other European Union countries is estimated to be approximately 1,800 individuals. Of these, we estimate that approximately 1,500 individuals have been diagnosed, of which approximately 300 are in the United States and approximately 1,200 are in Europe. However, diagnostic challenges may affect the number of potential patients, and we believe that the availability of treatment options could increase awareness of the disease and assist in identifying more cases. Therapies to treat NPC are desperately needed, and for this reason, arimoclomol is currently being made available to NPC patients in the United States, France, Germany, and other European Union countries under various early access programs, or EAPs.

On September 16, 2020, the previous sponsor of the arimoclomol program, Orphazyme, submitted an NDA seeking approval of arimoclomol for the treatment of NPC. In June 2021, the FDA issued a CRL, which means the FDA determined that it could not approve the NDA in its present form. Our aim is to prepare and resubmit an NDA that presents meaningful evidence of safety and efficacy of arimoclomol for its intended use. To that end, we are continuing to work diligently to characterize the substantial data generated since the CRL, including the recently completed four-year open-label safety trial. We expect to resubmit the arimoclomol NDA to the FDA as early as the third quarter of 2023.

Arimoclomol has received Orphan Drug Designation from the FDA for the treatment of NPC and orphan medicinal product designation from the European Medicines Agency for the treatment of NPC, as well as Fast-Track Designation, Breakthrough Therapy Designation, and Rare Pediatric Disease Designation from the FDA, each for the treatment of NPC.

KP1077

KP1077 is based on SDX and is our lead prodrug product candidate in development for the treatment of IH, an underserved indication. IH is a rare neurological sleep disorder affecting approximately 37,000 patients in the United States. The cardinal feature of IH is excessive daytime sleepiness, or EDS, characterized by daytime lapses into sleep, or an irrepressible need to sleep that persists even with adequate or prolonged nighttime sleep. Additionally, those with IH have extreme difficulty waking, otherwise known as "sleep inertia," suffer from severe and debilitating brain fog, and may fall asleep unintentionally or at inappropriate times, also known as narcolepsy. These symptoms often further lead to reported memory problems, difficulty maintaining focus, and depression. In the fourth quarter of 2022, we initiated a Phase 2 clinical trial in the U.S. investigating KP1077 as a treatment for IH. Interim safety and efficacy data are expected in the third quarter of 2023 and top-line data are expected by year end 2023. We plan to use our learnings from the ongoing Phase 2 trial to design Phase 3 clinical studies of SDX, the sole pharmaceutical agent in KP1077, in IH and narcolepsy with or without cataplexy. We intend to submit an IND for KP1077 for the treatment of narcolepsy as early as the second quarter of 2023, and subsequently begin a trial in narcolepsy soon after interim data from the Phase 2 IH clinical trial is available.

There is currently only one approved product for the treatment of IH, XYWAV®, developed by Jazz Pharmaceuticals. A second product, WAKIX®, developed by Harmony Biosciences and originally approved for the treatment of EDS or cataplexy in adult patients with narcolepsy, is planned to be studied in a Phase 3 trial with IH patients. Prescribers also utilize narcolepsy medications and various stimulant products "off-label" to treat IH symptoms, with methylphenidate, a Schedule IV controlled substance, being one of the most commonly used stimulants for treating IH. While each of these medications can help to address certain IH symptoms, there are also potential shortcomings, including dosing inconvenience, serious adverse events, such as elevated blood pressure and heart rate, and significant drug-to-drug interactions, or DDIs, including with medications used to manage contraception and depression. In addition, patients have indicated that the effectiveness of their current medication was poor.

Narcolepsy is a rare, chronic, debilitating neurologic disorder of sleep-wake state instability that impacts up to 200,000 Americans and is primarily characterized by EDS and cataplexy (sudden loss of muscle tone while a person is awake) along with other manifestations of rapid eye movement, or REM, sleep dysregulation, which intrude into wakefulness. In most patients, narcolepsy is caused by the loss of hypocretin, a neuropeptide in the brain that supports sleep-wake state stability. Typical symptom onset occurs in adolescence or young adulthood, but it can take up to a decade to be properly diagnosed. Although there are several approved medications for narcolepsy, we believe a treatment option based on SDX, our proprietary prodrug of d-MPH, with superior exposure/duration characteristics and low abuse potential may be beneficial.

Based on our preclinical and clinical data, we believe KP1077, if approved by the FDA, may have valuable product features and may provide significant benefits to patients and physicians:

- ***Dosing flexibility.*** Either one or two doses daily, which is designed to address the two primary issues associated with IH: (i) nighttime dose would address sleep inertia, and (ii) morning dose would address daytime brain fog.

- ***No drug-to-drug interactions.*** Our data indicate that there is no drug-to-drug interaction potential, especially with hormonal contraceptives and antidepressants.

- ***Reduced abuse potential as a Schedule IV controlled substance.*** All other methylphenidate-based products have been designated as Schedule II controlled substances, which indicates stricter control over the prescribing and use of such products. KP1077 is based on SDX, which has been designated a Schedule IV controlled substance.

- ***No generic equivalent product.*** KP1077 contains SDX, our proprietary prodrug of d-methylphenidate that was given a new chemical name, serdexmethylphenidate, by the USAN, which means that there may be no generic equivalent product for KP1077 in most states, making drug-equivalent substitution potentially difficult at the pharmacy.

- ***Composition-of-matter patent protection.*** KP1077 is generally protected by a U.S. composition-of-matter patent that will expire, after utilizing all appropriate patent term adjustments but excluding possible term extensions, in 2037.

- ***Orphan drug designation potential.*** Because there are limited treatments available for the extremely small IH patient population, KP1077 may be eligible to receive the orphan drug designation by the FDA, as well as fast-track and breakthrough therapy designation eligibility, which may provide various regulatory benefits for the development program.

KP879

KP879 is based on SDX and is our product candidate in development for the treatment of stimulant use disorder, or SUD, including, for example, abuse or misuse of cocaine, methamphetamine and prescription stimulants. Currently there are no approved drugs in the United States for SUD. We submitted an IND to the FDA for KP879 in December 2020. In January 2021, the FDA completed its review of the KP879 IND, concluding that we may proceed with its planned clinical investigation of the product candidate. We initiated the clinical program in October 2021, reported top-line data from a Phase 1 proof-of-concept study for KP879 in the fourth quarter of 2021 and final data from the Phase 1 proof-of-concept study for KP879 in the first quarter of 2022.

In the second quarter of 2022, we conducted a second Phase 1 trial to evaluate the cardiovascular safety of KP879 compared to immediate-release Ritalin and Ritalin LA in healthy volunteers, and reported top-line data in the third quarter of 2022. In this study, the doses of SDX evaluated were observed to be well-tolerated. d-MPH was observed to have been released as designed and in a manner which we believe to be well-suited for further study in patients with SUD but also in patients with sleep disorders characterized with EDS (see KP1077 above).

In January 2023, we submitted a grant proposal to the National Institute of Drug Abuse (NIDA) for a Phase 1 study in cocaine-dependent volunteers to evaluate the safety of concomitant administration of KP879 and cocaine, in preparation of a Phase 3 trial thereafter. If the grant is approved, a Phase 1 study is planned to be conducted subsequently.

AZSTARYS

Overview

AZSTARYS contains dexmethylphenidate and our prodrug, SDX. On March 2, 2021, the FDA approved the NDA for AZSTARYS in patients age six years and older. In May 2021, we announced that SDX, our proprietary prodrug of d-MPH and the primary API in AZSTARYS, was classified as a Schedule IV controlled substance by the DEA. AZSTARYS is classified as a Schedule II controlled substance as its formulation includes a 70:30 mixture of SDX (Schedule IV) and d-MPH (Schedule II), respectively.

In September 2019, we entered into the AZSTARYS License Agreement with Commave. Under the AZSTARYS License Agreement, we granted to Commave an exclusive, worldwide license, to develop, manufacture, and commercialize AZSTARYS and any of our product candidates containing SDX for the treatment of ADHD. Commave has tasked Corium with leading all commercialization activities for AZSTARYS in the United States under the AZSTARYS License Agreement. Corium commercially launched AZSTARYS in the United States during the third quarter of 2021.

Pursuant to the AZSTARYS License Agreement, Commave agreed to pay up to $63.0 million in milestone payments upon the occurrence of specified regulatory milestones related to the AZSTARYS, including FDA approval and specified conditions with respect to the final approval label. In addition, Corium agreed to make additional payments upon the achievement of specified U.S. sales milestones of up to $420 million in the aggregate. Further, Commave will pay us quarterly, tiered royalty payments based on a percentage of net sales on a product-by-product basis. Corium also agreed to be responsible for and reimburse us for all of development, commercialization and regulatory expenses for any products or product candidates containing SDX, subject to certain limitations as set forth in the AZSTARYS License Agreement, including consultation fees to be paid to us for services provided to Corium in performing such activities.

In April 2021, we entered into the AZSTARYS Amendment. Pursuant to the AZSTARYS Amendment, we and Corium agreed to modify the compensation terms of the AZSTARYS License Agreement. Corium paid us $10.0 million in connection with the entry into the AZSTARYS Amendment as a result of the FDA approval of AZSTARYS in the United States. Corium also paid us $10.0 million following the receipt of the scheduling determination of the compound SDX by the DEA, which occurred on May 7, 2021. In addition, the AZSTARYS Amendment increased the total remaining future regulatory and sales milestone payments related to AZSTARYS up to an aggregate of $590.0 million.

The AZSTARYS License Agreement will continue on a product-by-product basis (i) until expiration of the royalty term for the applicable product candidate in the United States and (ii) perpetually for all other countries.

APADAZ

Overview

APADAZ is approved by the FDA for the short-term (no more than 14 days) management of acute pain severe enough to require an opioid analgesic and for which alternative treatments are inadequate. APADAZ is an IR combination of our prodrug, benzhydrocodone, and acetaminophen, or APAP. APADAZ contains benzhydrocodone, a Schedule II controlled substance. Benzhydrocodone was developed with our proprietary LAT platform technology.

In October 2018, we entered into the APADAZ License Agreement with KVK pursuant to which we have granted an exclusive license to KVK to conduct regulatory activities for the manufacture and commercialization of APADAZ in the United States. We have retained the right to license the international commercial rights to APADAZ to one or more collaborators outside of the U.S. We will be solely responsible for all development activities except for regulatory activities relating to APADAZ in the United States.

Pursuant to the APADAZ License Agreement, KVK agreed to pay us certain payments and cost reimbursements of an estimated total of $3.4 million, which includes a payment of $2.0 million within 10 days of the achievement of a specified milestone related to the initial formulary adoption of APADAZ, or the Initial Adoption Milestone. In addition, KVK has agreed to make additional payments to us upon the achievement of specified sales milestones of up to $53.0 million in the aggregate. Further, we and KVK will share the quarterly net profits of APADAZ by KVK in the United States at specified tiered percentages, ranging from us receiving 30% to 50% of net profits, based on the amount of net sales on a rolling four quarter basis. We are responsible for a portion of commercialization and regulatory expenses for APADAZ until the Initial Adoption Milestone is achieved, after which KVK will be responsible for all expenses incurred in connection with commercialization and maintaining regulatory approval in the United States.

In November 2019, APADAZ and its authorized generic became nationally available. To date, KVK has utilized targeted non-traditional efforts to build product awareness for APADAZ as a responsible alternative to currently available hydrocodone/acetaminophen products and there has been limited adoption of the product by prescribers.

Our Intellectual Property

Our intellectual property strategy includes seeking composition-of-matter patents, among other patents, for our prodrugs and product candidates and conjugates of our prodrugs while also protecting, where appropriate as trade secrets, our proprietary LAT platform technology, the process by which we identify, screen, evaluate and select ligands to be conjugated with parent drugs to create our prodrugs. Our current prodrugs all consist of an approved parent drug and one or more ligands that we have selected using our proprietary LAT platform technology. The parent drug and ligand or ligands together may potentially constitute a new molecule and thus may be eligible for composition-of-matter patent protection, among other patent protections, in the United States and abroad.

As of December 31, 2022, we have been granted and maintain 62 active patents within the United States, and an additional 241 active foreign patents covering our selected prodrugs and product candidates. The terms of the 62 issued U.S. patents extend to various dates ranging, for example, between 2029 and 2040. The term of our overall domestic and foreign patent portfolio related to our selected prodrugs and product candidates, including patent term adjustments but excluding possible patent term extensions, extend to various dates ranging, for example, between 2029 and 2042, if pending patent applications in each of our patent families issue as patents. As of December 31, 2022, we had 19 pending patent applications under active prosecution in the United States, and an additional 117 pending foreign patent applications potentially covering our selected prodrugs and product candidates. Our issued and granted patents provide protection in jurisdictions that include the United States, Australia, Canada, Chile, China, European Countries, Hong Kong, India, Indonesia, Israel, Japan, Kazakhstan, Malaysia, Mexico, New Zealand, Philippines, Romania, Russia, Singapore, South Africa, South Korea, and Vietnam.

We have received composition-of-matter patents and also additionally filed composition-of-matter and method of treatment patent applications related to the AZSTARYS families in the United States and in Argentina, Australia, Brazil, Canada, Chile, China, Egypt, Hong Kong, European Countries, India, Israel, Indonesia, Japan, South Korea, Kazakhstan, Mexico, Malaysia, New Zealand, Philippines, Russia, Singapore, South Africa, Thailand, Ukraine, and Vietnam. We anticipate filing additional patent applications for our prodrugs and product candidates.

Since 2013, the United States Patent and Trademark Office, or the USPTO, has issued 14 composition-of-matter or method of treatment patents covering benzhydrocodone, which will expire, after utilizing all appropriate patent term adjustments but excluding possible patent term extensions, no earlier than 2030. Further, there are granted or recently allowed compositions-of-matter patents covering benzhydrocodone in Australia, Canada, Chile, China, Israel, Mexico, South Africa, and South Korea. Patent applications covering benzhydrocodone were pending as of December 31, 2022, in Thailand and Vietnam.

We have additionally received method of use and method of treatment patents, and have filed related patent applications, related to the arimoclomol families (pursuant to the recent acquisition of Orphazyme) in various jurisdictions, including the United States, European countries, Israel, Japan, South Korea, Canada, China, Brazil, Russia and Turkey, with anticipated patent expiration date of 2029, excluding any potential patent term adjustments or extensions. We anticipate filing additional patent applications related to the arimoclomol families.

We also depend upon the skills, knowledge and experience of our scientific and technical personnel, as well as that of our advisors, consultants and other contractors. To help protect our LAT platform technology, as well as any proprietary know-how and show-how beyond that which is patentable, we rely on trade secret protection and confidentiality agreements to protect our interests. To this end, we generally require our employees, consultants and advisors to enter into confidentiality agreements prohibiting the disclosure of confidential information and, in some cases, requiring disclosure and assignment to us of the ideas, developments, discoveries, inventions and improvements important to our business.

Commercialization

In March 2021, we announced that the FDA approved the NDA for AZSTARYS, a once-daily product for the treatment of ADHD in patients ranging from six years and older. Corium is leading the commercialization of AZSTARYS per the AZSTARYS License Agreement. Corium commercially launched AZSTARYS in the United States during the third quarter of 2021.

In February 2018, we announced that the FDA approved APADAZ for the short-term (no more than 14 days) management of acute pain severe enough to require an opioid analgesic and for which alternative treatments are inadequate. In October 2018, we entered into the APADAZ License Agreement with KVK pursuant to which we have granted an exclusive license to KVK to conduct regulatory activities for, manufacture and commercialize APADAZ in the United States. In November 2019, APADAZ and its authorized generic (AG-APADAZ) became nationally available. To date, KVK has utilized targeted non-traditional efforts to build product awareness for APADAZ as a responsible alternative to currently available hydrocodone/acetaminophen products and there has been limited adoption of the product by prescribers.

With the exception of AZSTARYS and APADAZ, commercialization activities for our product candidates in active development have not yet begun. It is our intention to build a small, nimble commercial team which will be able to fully service the patients and prescribers within the rare disease indications for which we are successful in gaining approval for our product candidates. However, if our product candidates have large potential market opportunities that would require significant marketing resources, we may conclude that the most appropriate approach to their commercialization, if they receive regulatory approval, will involve forming a commercial collaboration or strategic relationship similar to those we have entered into with Corium and KVK, or consummating some type of strategic transaction, with a larger pharmaceutical or other marketing organization. As we get closer to potential approval of our product candidates which are not currently subject to the AZSTARYS License Agreement, we will work to identify and implement the commercialization strategies that we conclude are the most desirable with regard to the specific product candidates.

Competition

Our industry is characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. We will face competition and potential competition from a number of sources, including pharmaceutical and biotechnology companies, specialty pharmaceutical companies, generic drug companies, drug delivery companies and academic and research institutions. Our competitors may develop or market drugs that are more effective, more convenient, more widely used and less costly or have a better safety profile than our products or product candidates and these competitors may also have significantly more resources than us and be more successful than us in manufacturing and marketing their products.

While there are currently no approved products for the treatment of NPC in the U.S., if approved, we expect the most direct competitor with respect to arimoclomol to be ZAVESCA (miglustat), which was originally developed by Actelion Pharmaceuticals and is now owned by Johnson & Johnson. Miglustat is available as a generic product in several countries, including the U.S., where it is currently approved for the treatment of another LSD, Gaucher disease. Miglustat is currently approved for the treatment of NPC in Europe, Canada, Australia, New Zealand, and several countries in Asia and South America where it is marketed as ZAVESCA, and marketed as BRAZAVES in Japan.

If approved, we intend for KP1077 to compete against Jazz Pharmaceuticals' XYWAV, and potentially with other products that are currently in development for the treatment of IH, including Harmony Biosciences' WAKIX. KP1077 could face potential competition from any products for the treatment of IH that are currently in or which may enter into clinical development.

Currently, there are no approved drugs in the United States for the treatment of SUD. If approved, KP879 will face potential competition from any products fo the treatment of SUD that are currently in, or which may enter into, clinical development.

AZSTARYS competes against currently marketed, branded and generic methylphenidate products for the treatment of ADHD. Some of these currently marketed products include Janssen's CONCERTA, Supernus' QUELBREE, Tris Pharma's QUILLIVANT XR and QUILLICHEW ER, Novartis' RITALIN, FOCALIN and FOCALIN XR, UCB's METADATE CD, Noven's DAYTRANA, Neos Therapeutics' CONTEMPLA XR-ODT, Ironshore Pharmaceuticals, Inc.'s JORNAY PM and Adlon Therapeutics' ADHANSIA XR, in addition to multiple other branded and generic methylphenidate products. In addition, AZSTARYS will face potential competition from any other methylphenidate products for the treatment of ADHD that are currently in or which may enter into clinical development.

APADAZ competes against currently marketed, branded and generic hydrocodone/APAP products for short-term (no more than 14 days) management of acute pain severe enough to require an opioid analgesic and for which alternative treatments are inadequate. In addition, APADAZ may face potential competition from any other hydrocodone/APAP products for short-term (no more than 14 days) management of acute pain severe enough to require an opioid analgesic and for which alternative treatments are inadequate that are currently in or which may enter into clinical development.

Manufacturing

Our manufacturing strategy is to rely on contract manufacturers to produce our product candidates for clinical trials and, if approved, drug product for commercial sale. We currently have no manufacturing facilities and limited personnel with manufacturing experience. We have contracted with a third-party manufacturer to supply arimoclomol, and to supply SDX, which is the primary API for KP1077 and KP879, to be used in our non-clinical, clinical and formulation development programs necessary to support an NDA submission. We expect to contract with third-party manufacturers for the manufacture of all API supply needs outside the United States if and when we receive approval from regulatory authorities outside the United States.

Our current and any future third-party manufacturers, their facilities and all lots of drug substance and drug products used in our clinical trials are required to be in compliance with current good manufacturing practices, or cGMPs. The cGMP regulations include requirements relating to organization of personnel, buildings and facilities, equipment, control of components and drug product containers and closures, production and process controls, packaging and labeling controls, holding and distribution, laboratory controls, records and reports, and returned or salvaged products. The manufacturing facilities for our products must meet cGMP requirements and FDA satisfaction before any product is approved and we can manufacture commercial products. Our current and any future third-party manufacturers are also subject to periodic inspections of facilities by the FDA and other authorities, including procedures and operations used in the testing and manufacture of our products to assess our compliance with applicable regulations.

Failure to comply with statutory and regulatory requirements subjects a manufacturer to possible legal or regulatory action, including refusal to approve pending applications, license suspension or revocation, withdrawal of an approval, imposition of a clinical hold or termination of clinical trials, warning letters, untitled letters, cyber letters, modification of promotional materials or labeling, product recalls, product seizures or detentions, refusal to allow imports or exports, total or partial suspension of production or distribution, debarment, injunctions, fines, consent decrees, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreements to resolve allegations of non-compliance with these laws, refusals of government contracts and new orders under existing contracts, exclusion from participation in federal and state healthcare programs, restitution, disgorgement or civil or criminal penalties, including fines and individual imprisonments.

Third-Party Payor Coverage and Reimbursement

Sales of pharmaceutical products depend in significant part on the availability of coverage and adequate reimbursement by third-party payors, such as state and federal governmental authorities, including those that administer the Medicare and Medicaid programs, and private managed care organizations and health insurers. Decisions regarding the extent of coverage and amount of reimbursement to be provided for each of our product and product candidates is and will be made on a plan-by-plan basis. One payor's determination to provide coverage for a product does not assure that other payors will also provide coverage, and adequate reimbursement, for the product. Each third-party payor determines whether or not it will provide coverage for a drug, what amount it will pay providers for the drug, and on what tier of its formulary the drug will be placed. These decisions are influenced by the existence of multiple drug products within a therapeutic class and the net cost to the plan, including the amount of the prescription price, if any, rebated by the drug's manufacturer. Typically, generic versions of drugs are placed in a preferred tier. The position of a drug on the formulary generally determines the co-payment that a patient will need to make to obtain the drug and can strongly influence the adoption of a drug by patients and physicians. Patients who are prescribed treatments for their conditions and providers performing the prescribed services generally rely on third-party payors to reimburse all or part of the associated healthcare costs. Patients are unlikely to use our products unless coverage is provided, and reimbursement is adequate to cover a significant portion of the cost of our products. Additionally, a third-party payor's decision to provide coverage for a drug does not imply that an adequate reimbursement rate will be approved. Also, third-party payors are developing increasingly sophisticated methods of controlling healthcare costs. As a result, coverage, reimbursement and placement determinations are complex and are often the subject of extensive negotiations between the payor and the owner of the drug.

Unless we enter into a strategic collaboration under which our collaborator assumes responsibility for seeking coverage and reimbursement for a given product, we will be responsible for negotiating coverage, reimbursement and placement decisions for our product candidates, if approved. Coverage, reimbursement and placement decisions for a new product are based on many factors including the coverage, reimbursement and placement of already marketed branded drugs for the same or similar indications, the safety and efficacy of the new product, availability of generics for similar indications, the clinical need for the new product and the cost-effectiveness of the product. Increasingly, both purchasers and payors are also conducting comparative clinical and cost effectiveness analyses involving application of metrics, including data on patient outcomes, provided by manufacturers.

Within the Medicare program, as self-administered drugs, our product and product candidates would be reimbursed under the expanded prescription drug benefit known as Medicare Part D. This program is a voluntary Medicare benefit administered by private plans that operate under contracts with the federal government. These plans develop formularies that determine which products are covered and what co-pay will apply to covered drugs. The plans have considerable discretion in establishing formularies and tiered co-pay structures, negotiating rebates with manufacturers and placing prior authorization and other restrictions on the utilization of specific products, subject to review by the Centers for Medicare & Medicaid Services, or CMS, for discriminatory practices. These Part D plans negotiate discounts with drug manufacturers, which are passed on, in whole or in part, to each of the plan's enrollees through reduced premiums. Historically, Part D beneficiaries have been exposed to significant out-of-pocket costs after they surpass an annual coverage limit and until they reach a catastrophic coverage threshold. However, changes made by recent legislation will reduce this patient coverage gap, known as the "donut hole", by transitioning patient responsibility in that coverage range from 100% in 2010 to only 25% currently. To help achieve this reduction, pharmaceutical manufacturers are required to provide quarterly discounts of 70%, which commenced January 1, 2019. In 2020, drug manufacturers became be responsible for a larger share of total drug costs due to an increase to the catastrophic threshold. Such increase will also result in a higher out-of-pocket threshold paid by Part D beneficiaries.

If a drug product is available for reimbursement by Medicare or Medicaid, its manufacturer must comply with various health regulatory requirements and price reporting metrics, which may include, as applicable, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, the Medicaid rebate requirements of the Omnibus Budget Reconciliation Act of 1990, or the OBRA, and the Veterans Health Care Act of 1992, or the VHCA, each as amended. Among other things, the OBRA requires drug manufacturers with certain drugs covered by Medicaid to pay rebates on prescription drugs to state Medicaid programs. States may also negotiate "supplemental" Medicaid rebates on drug products dispensed under Medicaid. Manufacturers participating in Medicaid are also generally required to participate in the Public Health Service 340B Drug Discount Program, which imposes a mandatory discount on purchases by certain customers. Manufacturers of innovator drugs, including 505(b)(2) drugs, that participate in the Medicaid program are also required to offer the drugs on the Federal Supply Schedule purchasing program of the General Services Administration for purchase by the Department of Veterans Affairs, the Department of Defense and other authorized users at a mandatory discount. Additional laws and requirements apply to these contracts. Participation in such federal programs may result in prices for our future products that will likely be lower than the prices we might otherwise obtain.

Third-party payors, including the U.S. government, continue to apply downward pressure on the reimbursement of pharmaceutical products. Also, the trend towards managed health care in the United States and the concurrent growth of organizations such as health maintenance organizations may result in lower reimbursement for pharmaceutical products. We expect that these trends will continue as these payors implement various proposals or regulatory policies, including various provisions of the recent health reform legislation that affect reimbursement of these products. There are currently, and we expect that there will continue to be, a number of federal and state proposals to implement controls on reimbursement and pricing, directly and indirectly.

Government Regulation

The FDA and comparable regulatory agencies in state and local jurisdictions and in foreign countries impose substantial requirements upon the clinical development, manufacture and marketing of pharmaceutical products. These agencies and other federal, state and local entities regulate research and development activities and the testing, manufacture, quality control, safety, effectiveness, labeling, storage, packaging, recordkeeping, tracking, approval, import, export, distribution, advertising and promotion of our products and product candidates. A new drug must be approved by the FDA through the NDA process before it may be legally marketed in the United States. The process of obtaining regulatory approvals and the subsequent compliance with applicable federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources.

The process required by the FDA before product candidates may be marketed in the United States generally involves the following:

- non-clinical laboratory and animal tests that must be conducted in accordance with good laboratory practices, or GLP, requirements and other applicable regulations;
- submission of an IND, which must be received by the FDA and become effective before human clinical trials may begin;
- approval by an independent institutional review board, or IRB, or ethics committee at each clinical site or centrally before each trial may be initiated;
- performance of adequate and well-controlled human clinical trials to establish the safety and efficacy of the proposed product candidate for its intended use, performed in accordance with good clinical practice requirements, or GCPs;
- preparation and submission of a NDA to the FDA;
- satisfactory completion of an FDA advisory committee review, if applicable;
- pre-approval inspection of manufacturing facilities at which the drug is produced to assess their compliance with cGMPs and of selected clinical investigation sites to assess compliance with GCPs; and
- FDA approval of an NDA to permit commercial marketing for particular indications for use.

Clinical Trials

The testing and regulatory approval process requires substantial time, effort and financial resources. Preclinical studies include laboratory evaluation of drug substance chemistry, pharmacology, toxicity and drug product formulation, as well as animal studies to assess potential safety and efficacy. Prior to commencing the first human clinical trial with a product candidate, we must submit the results of the preclinical tests and preclinical literature, together with manufacturing information, analytical data and any available clinical data or literature, among other things, to the FDA as part of an IND. An IND is a request for authorization from the FDA to administer an investigational new drug product to humans. The central focus of an IND submission is on the general investigational plan and the protocol(s) for clinical studies.

The IND becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day time period, raises safety concerns or questions about the conduct of the clinical trial by imposing a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. Submission of an IND may not result in FDA authorization to commence a clinical trial.

Clinical trials involve the administration of the investigational product to human subjects under the supervision of qualified investigators in accordance with GCPs, which include the requirement that all research subjects provide their informed consent for their participation in any clinical study. Clinical trials are conducted under protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A separate submission to the existing IND must be made for each successive clinical trial conducted during product development, as well as amendments to previously submitted clinical trials. Further, an independent IRB for each study site proposing to conduct the clinical trial must review and approve the plan for any clinical trial, its informed consent form and other communications to study subjects before the clinical trial commences at that site. The IRB must continue to oversee the clinical trial while it is being conducted, including any changes to the study plans. Regulatory authorities, an IRB or the sponsor may suspend or discontinue a clinical trial at any time on various grounds, including a finding that the subjects are being exposed to an unacceptable health risk, the clinical trial is not being conducted in accordance with the FDA's or the IRB's requirements, if the drug has been associated with unexpected serious harm to subjects, or based on evolving business objectives or competitive climate. Some studies also include a data safety monitoring board, which receives special access to unblinded data during the clinical trial and may advise us to halt the clinical trial if it determines that there is an unacceptable safety risk for subjects or other grounds, such as no demonstration of efficacy. Depending on its charter, this group may determine whether a trial may move forward at designated check points based on access to certain data from the trial. The FDA or the sponsor may suspend a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients. There are also requirements governing the reporting of ongoing clinical studies and clinical study results to public registries.

In general, for purposes of NDA approval, human clinical trials are typically conducted in three sequential phases that may overlap.

- *Phase 1* - The product candidate is initially introduced into healthy subjects or patients with the target disease or condition. These studies are conducted to test the product candidate for safety, dosage tolerance, structure-activity relationships, mechanism of action, absorption, metabolism, distribution and excretion. If possible, Phase 1 trials may also be used to gain early evidence of product effectiveness.
- *Phase 2* - The product candidate is administered to a limited patient population with a specified disease or condition to evaluate the preliminary efficacy, optimal dosage and dosing schedule and to identify possible adverse side effects and safety risks. Multiple Phase 2 clinical trials may be conducted to obtain information prior to beginning larger and more expensive Phase 3 clinical trials.
- *Phase 3* - The product candidate is administered to an expanded patient population to further evaluate dosage to provide statistically significant evidence of clinical efficacy and to further test for safety in an expanded subject population at multiple clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the product and provide an adequate basis for product labeling.

In the case of a 505(b)(2) NDA, some of the above-described studies and preclinical studies may not be required or may be abbreviated. Pharmacokinetic and other bridging studies may be needed, however, to demonstrate the relevance of the studies that were previously conducted by other sponsors to the drug that is the subject of the NDA.

The FDA may require, or companies may pursue, additional clinical trials after a product is approved. These so-called Phase 4, or post-market, studies may be used to gain additional experience from the treatment of patients in the intended therapeutic indication. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials as a condition of approval of an NDA.

The manufacture of investigational drugs for the conduct of human clinical trials is subject to cGMP requirements. Investigational drugs and active pharmaceutical ingredients imported into the United States are also subject to regulation by the FDA relating to their labeling and distribution. Further, the export of investigational drug products outside of the United States is subject to regulatory requirements of the receiving country as well as U.S. export requirements under the FFDCA. In addition, while the IND is active and before approval, progress reports summarizing the results of the clinical trials and nonclinical studies performed since the last progress report must be submitted at least annually to the FDA, and written IND safety reports must be submitted to the FDA and investigators for serious and unexpected suspected adverse events, findings from other studies suggesting a significant risk to humans exposed to the same or similar drugs, findings from animal or in vitro testing suggesting a significant risk to humans, and any clinically important increased incidence of a serious suspected adverse reaction compared to that listed in the protocol or investigator's brochure.

Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the product candidate as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, must develop methods for testing the identity, strength, quality and purity of the final product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

NDA Submission and Review by the FDA

Assuming successful completion of the required clinical and preclinical testing, among other items, the results of product development, including chemistry, manufacture and controls, non-clinical studies and clinical trials are submitted to the FDA, along with proposed labeling, as part of an NDA requesting approval to market the product. Data can come from company-sponsored clinical studies intended to test the safety and effectiveness of a use of the product, or from a number of alternative sources, including studies initiated by independent investigators. The submission of an NDA requires payment of a substantial application user fee to the FDA. These user fees must be filed at the time of the first submission of the application, even if the application is being submitted on a rolling basis. Fee waivers or reductions are available in some circumstances.

In addition, under the Pediatric Research Equity Act, or PREA, an NDA or supplement to an NDA for a new active ingredient, indication, dosage form, dosage regimen or route of administration must contain data that are adequate to assess the safety and efficacy of the drug for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults or full or partial waivers from the pediatric data requirements.

The FDA may refer drugs which present difficult questions of safety or efficacy to an advisory committee. An advisory committee is a panel that typically includes clinicians and other experts who review, evaluate and make a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Once the FDA receives an application, it has 60 days to review the NDA to determine if it is substantially complete to permit a substantive review, before it accepts the application for filing. Once the submission is accepted for filing, the FDA begins an in-depth review of the NDA. The timeline for the FDA to complete its review of a NDA may differ based on whether the application is a standard review or priority review application. The FDA may give a priority review to applications for drugs that are intended to treat serious conditions and if approved, would provide significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of such serious conditions. Under the goals and policies agreed to by the FDA under the Prescription Drug User Fee Act, or PDUFA, the FDA has set the review goal of ten months from the 60-day filing date to complete its initial review of a standard NDA for a new molecular entity, or NME, and make a decision on the application. For non-NME standard applications, the FDA has set the review goal of ten months from the submission date to complete its initial review and to make a decision on the application. For priority review applications, the FDA has set the review goal of reviewing NME NDAs within six months of the 60-day filing date and non-NME applications within six months of the date of receipt. Such deadlines are referred to as the PDUFA date. The review process and the PDUFA date may also be extended if the FDA requests or the NDA sponsor otherwise provides additional information or clarification regarding the submission.

The FDA reviews applications to determine, among other things, whether a product is safe and effective for its intended use and whether the manufacturing controls are adequate to assure and preserve the product's identity, strength, quality and purity. Before approving an NDA, the FDA will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities, including contract manufacturers and subcontracts, are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA will typically inspect one or more clinical trial sites to assure compliance with GCPs.

Once the FDA's review of the application is complete, the FDA will issue either a CRL or approval letter. A CRL indicates that the review cycle of the application is complete, and the application cannot be approved in its current form. A CRL will describe all of the deficiencies that the FDA has identified in the NDA, except that where the FDA determines that the data supporting the application are inadequate to support approval, the FDA may issue the CRL without first conducting required inspections and/or reviewing proposed labeling. In issuing the CRL, the FDA may recommend actions that the applicant might take to place the NDA in condition for approval, including requests for additional information or clarification. Even with the submission of additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval. If and when those conditions have been met to the FDA's satisfaction, the FDA may issue an approval letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications.

The FDA may delay or refuse approval of an NDA if applicable regulatory criteria are not satisfied, require additional testing or information and/or require post-marketing testing and surveillance to monitor safety or efficacy of a product, or impose other conditions, including distribution restrictions or other risk management mechanisms. For example, the FDA may require a risk evaluation and mitigation strategy, or REMS, as a condition of approval or following approval. A REMS is a safety strategy to manage a known or potential serious risk associated with a medicine and to enable patients to have continued access to such medicines by managing their safe use, and could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries, and other risk minimization tools.

If regulatory approval of a product is granted, such approval may entail limitations on the indicated uses for which such product may be marketed or may include contraindications, warnings or precautions in the product labeling, including a boxed warning. If the FDA requires a boxed warning, the sponsor may also be subject to specified promotional restrictions, such as the prohibition of reminder advertisements. The FDA also may not approve the inclusion of labeling claims necessary for successful marketing. Once approved, the FDA may withdraw the product approval if compliance with pre- and post-marketing regulatory standards is not maintained or if problems occur after the product reaches the marketplace. In addition, the FDA may require Phase 4 post-marketing studies to monitor the effect of approved products and may limit further marketing of the product based on the results of these post-marketing studies.

Post-approval Requirements

Any products manufactured or distributed pursuant to FDA approvals are subject to continuing regulation by the FDA, including manufacturing, periodic reporting, product sampling and distribution, advertising, promotion, drug shortage reporting, compliance with any post-approval requirements imposed as a conditional of approval such as Phase 4 clinical trials, REMS and surveillance, recordkeeping and reporting requirements, including adverse experiences.

After approval, most changes to the approved product, such as adding new indications or other labeling claims are subject to prior FDA review and approval. There also are continuing, annual human prescription drug program fee requirements for approved products. Drug manufacturers and their subcontractors are required to register their establishments with the FDA and certain state agencies and to list their drug products and are subject to periodic announced and unannounced inspections by the FDA and these state agencies for compliance with cGMPs and other requirements, which impose procedural and documentation requirements upon us and our third-party manufacturers. We cannot be certain that we or our present or future suppliers will be able to comply with the cGMP regulations and other FDA regulatory requirements.

Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented, or FDA notification. FDA regulations also require investigation and correction of any deviations from cGMPs and specifications and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance.

Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in withdrawal of marketing approval, mandatory revisions to the approved labeling to add new safety information or other limitations, imposition of post-market studies or clinical trials to assess new safety risks, or imposition of distribution or other restrictions under a REMS program, among other consequences.

The FDA closely regulates the marketing and promotion of drugs. A company can make only those claims relating to safety and efficacy, purity and potency that are approved by the FDA. Physicians, in their independent professional medical judgment, may prescribe legally available products for uses that are not described in the product's labeling and that differ from those tested by us and approved by the FDA. Physicians may believe that such off-label uses are the best treatment for many patients in varied circumstances. The FDA does not regulate the behavior of physicians in their choice of treatments. The FDA does, however, restrict manufacturer's communications on the subject of off-label use of their products. However, companies may share truthful and not misleading information that is otherwise consistent with a product's FDA-approved labelling.

In addition, the distribution of prescription pharmaceutical products, including samples, is subject to the Prescription Drug Marketing Act, or PDMA, which regulates the distribution of drugs and drug samples at the federal level, and sets minimum standards for the registration and regulation of drug distributors by the states. Both the PDMA and state laws limit the distribution of prescription pharmaceutical product samples and impose requirements to ensure accountability in distribution. The Drug Supply Chain Security Act also imposes obligations on manufacturers of pharmaceutical products related to product tracking and tracing.

Failure to comply with any of the FDA's requirements could result in significant adverse enforcement actions. These include a variety of administrative or judicial sanctions, such as refusal to approve pending applications, withdrawal of an approval, imposition of a clinical hold or termination of clinical trials, warning letters, untitled letters, modification of promotional materials or labeling, product recalls, product seizures or detentions, refusal to allow imports or exports, total or partial suspension of production or distribution, debarment, injunctions, fines, consent decrees, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, refusals of government contracts and new orders under existing contracts, exclusion from participation in federal and state healthcare programs, restitution, disgorgement or civil or criminal penalties, including fines and individual imprisonment. Any of these sanctions could result in adverse publicity, among other adverse consequences.

Orphan drug designation and exclusivity

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug intended to treat a rare disease or condition, defined as a disease or condition with a patient population of fewer than 200,000 individuals in the U.S., or a patient population greater than 200,000 individuals in the U.S. and when there is no reasonable expectation that the cost of developing and making available the drug in the U.S. will be recovered from sales in the United States for that drug. Orphan drug designation must be requested before submitting an NDA. After the FDA grants orphan drug designation, the generic identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA.

If a product that has orphan drug designation subsequently receives the first FDA approval for a particular active ingredient for the disease for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications, including a full NDA, to market the same drug for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity or if the FDA finds that the holder of the orphan drug exclusivity has not shown that it can assure the availability of sufficient quantities of the orphan drug to meet the needs of patients with the disease or condition for which the drug was designated. Orphan drug exclusivity does not prevent the FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the NDA application user fee.

A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. In addition, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or, as noted above, if a second applicant demonstrates that its product is clinically superior to the approved product with orphan exclusivity or the manufacturer of the approved product is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

Hatch-Waxman Act

Section 505 of the FFDCA describes three types of marketing applications that may be submitted to the FDA to request marketing authorization for a new drug. A Section 505(b)(1) NDA is an application that contains full reports of investigations of safety and efficacy. A 505(b)(2) NDA is an application that contains full reports of investigations of safety and efficacy but where at least some of the information required for approval comes from investigations that were not conducted by or for the applicant and for which the applicant has not obtained a right of reference or use from the person by or for whom the investigations were conducted. This regulatory pathway enables the applicant to rely, in part, on the FDA's prior findings of safety and efficacy for an existing product, or published literature, in support of its application. The FDA may also require 505(b)(2) applicants to perform additional studies or measurements to support any changes from the reference listed drug. The FDA may then approve the new product candidate for all or some of the labeled indications for which the referenced product has been approved, as well as for any new indication sought by the 505(b)(2) applicant. Section 505(j) establishes an abbreviated approval process for a generic version of approved drug products through the submission of an abbreviated new drug application, or ANDA. An ANDA provides for marketing of a generic drug product that has the same active ingredients, dosage form, strength, route of administration, labeling, performance characteristics and intended use, among other things, to a previously approved product. ANDAs are termed "abbreviated" because they are generally not required to include preclinical and clinical data to establish safety and efficacy. Instead, generic applicants must scientifically demonstrate that their product is bioequivalent to, or performs in the same manner as, the innovator drug through in vitro, in vivo, or other testing. The generic version must deliver the same amount of active ingredients into a subject's bloodstream in the same amount of time as the innovator drug and can often be substituted by pharmacists under prescriptions written for the reference listed drug.

In seeking approval for a drug through an NDA, including a 505(b)(2) NDA, applicants are required to list with the FDA patents whose claims cover the applicant's product. Upon approval of an NDA, each of the patents listed in the application for the drug is then published in the Orange Book. These products may be cited by potential competitors in support of approval of an ANDA or 505(b)(2) NDA.

Any applicant who files an ANDA seeking approval of a generic equivalent version of a drug listed in the Orange Book or a 505(b)(2) NDA referencing a drug listed in the Orange Book must certify to the FDA that (1) no patent information on the drug or method of use that is the subject of the application has been submitted to the FDA; (2) such patent has expired; (3) the date on which such patent expires; or (4) such patent is invalid or will not be infringed upon by the manufacture, use or sale of the drug product for which the application is submitted. This last certification is known as a Paragraph IV certification. Generally, the ANDA or 505(b)(2) NDA cannot be approved until all listed patents have expired, except where the ANDA or 505(b)(2) NDA applicant challenges a listed patent through a Paragraph IV certification. If the applicant does not challenge the listed patents or does not indicate that it is not seeking approval of a patented method of use, the ANDA or 505(b)(2) NDA application will not be approved until all of the listed patents claiming the referenced product have expired, or, if permissible, are carved out.

If the ANDA or 505(b)(2) NDA applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the holder of the NDA for the reference listed drug and the patent owner once the application has been accepted for filing by the FDA. The NDA holder or patent owner may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days of the receipt of a Paragraph IV certification prevents the FDA from approving the application until the earlier of 30 months from the date of the lawsuit, expiration of the patent, settlement of the lawsuit, a decision in the infringement case that is favorable to the applicant or such shorter or longer period as may be ordered by a court. This prohibition is generally referred to as the 30-month stay. In instances where an ANDA or 505(b)(2) NDA applicant files a Paragraph IV certification, the NDA holder or patent owner regularly take action to trigger the 30-month stay, recognizing that the related patent litigation may take many months or years to resolve. Thus, approval of an ANDA or 505(b)(2) NDA could be delayed for a significant period of time depending on the patent certification the applicant makes and the reference drug sponsor's decision to initiate patent litigation. The applicant may also elect to submit a statement certifying that its proposed label does not contain, or carves out, any language regarding the patented method-of-use rather than certify to a listed method-of-use patent.

The FDA provides periods of regulatory exclusivity, which provides the holder of an approved NDA limited protection from new competition in the marketplace for the innovation represented by its approved drug for a period of three or five years following the FDA's approval of the NDA. Five years of exclusivity are available to new chemical entities, or NCEs. An NCE is a drug that contains no active moiety that has been approved by the FDA in any other NDA. An active moiety is the molecule or ion, excluding those appended portions of the molecule that cause the drug to be an ester, salt, including a salt with hydrogen or coordination bonds, or other noncovalent derivatives, such as a complex, chelate, or clathrate, of the molecule, responsible for the therapeutic activity of the drug substance. During the exclusivity period, the FDA may not accept for review or approve an ANDA or a 505(b)(2) NDA submitted by another company that contains the previously approved active moiety. An ANDA or 505(b)(2) application, however, may be submitted one year before NCE exclusivity expires if a Paragraph IV certification is filed. Applicants may also seek to carve out certain drug labeling that is protected by exclusivity.

If a product is not eligible for the NCE exclusivity, it may be eligible for three years of exclusivity. Three-year exclusivity is available to the holder of an NDA, including a 505(b)(2) NDA, for a particular condition of approval, or change to a marketed product, such as a new formulation for a previously approved product, if one or more new clinical trials, other than bioavailability or bioequivalence trials, was essential to the approval of the application and was conducted or sponsored by the applicant. This three-year exclusivity period protects against FDA approval of ANDAs and 505(b)(2) NDAs for the condition of the new drug's approval. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA; however, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and efficacy.

DEA Regulation

Our products and certain of our product candidates are, or if approved, will be regulated as "controlled substances" as defined in the Controlled Substances Act of 1970, or CSA, and the DEA's implementing regulations, which establish registration, security, recordkeeping, reporting, storage, distribution, importation, exportation, inventory, quota and other requirements administered by the DEA. These requirements are directly applicable to us and also applicable to our contract manufacturers and to distributors, prescribers and dispensers of our product candidates. The DEA regulates the handling of controlled substances through a closed chain of distribution. This control extends to the equipment and raw materials used in their manufacture and packaging in order to prevent loss and diversion into illicit channels of commerce.

The DEA regulates controlled substances as Schedule I, II, III, IV or V substances. Schedule I substances by definition have no established medicinal use and may not be marketed or sold in the United States. A pharmaceutical product may be listed as Schedule II, III, IV or V, with Schedule II substances considered to present the highest risk of abuse and Schedule V substances the lowest relative risk of abuse among such substances. Schedule II drugs are those that meet the following criteria:

- the drug has a high potential for abuse;
- the drug has a currently accepted medical use in treatment in the United States or a currently accepted medical use with severe restrictions; and
- abuse of the drug may lead to severe psychological or physical dependence.

APADAZ and AZSTARYS are listed as Schedule II controlled substances under the CSA. For Schedule II controlled substances, the importation of APIs for our product candidates, as well as the manufacture, shipping, storage, sales and use of the products, are subject to a high degree of regulation. In addition to maintaining an importer and/or exporter registration, importers and exporters of controlled substances must obtain a permit for every import of a Schedule I or II substance and a narcotic substance in Schedule III, IV and V, as well as every export of a Schedule I or II substance and a narcotic substance in Schedule III and IV. For all other drugs in Schedule III, IV and V, importers and exporters must submit an import or export declaration. Schedule II drugs are subject to the strictest requirements for registration, security, recordkeeping and reporting. Also, distribution and dispensing of these drugs are highly regulated. For example, all Schedule II drug prescriptions must be signed by a physician, physically presented to a pharmacist and may not be refilled without a new prescription. Electronic prescriptions may also be permissible depending on the state, so long as the prescription complies with the DEA's requirements for electronic prescriptions.

SDX, which is the sole API in KP1077 and KP879, has been listed as a Schedule IV controlled substance under the CSA. Controlled substances classified in Schedule III, IV, and V are also subject to registration, recordkeeping, reporting, and security requirements. For example, Schedule III drug prescriptions must be authorized by a physician and may not be refilled more than six months after the date of the original prescription or more than five times. A prescription for controlled substances classified in Schedules III, IV, and V issued by a physician, may be communicated either orally, in writing or by facsimile to the pharmacies. Controlled substances that are also classified as narcotics, such as hydrocodone, oxycodone and hydromorphone, are also subject to additional DEA requirements, such as manufacturer reporting of the import of narcotic raw material.

Annual registration is required for any facility that manufactures, distributes, dispenses, imports or exports any controlled substance. The registration is specific to the particular location, activity and controlled substance schedule. For example, separate registrations are needed for import and manufacturing, and each registration will specify which schedules of controlled substances are authorized. Similarly, separate registrations are also required for separate facilities. Acquisition and distribution transactions must also be reported for Schedule I and II controlled substances, as well as Schedule III narcotic substances.

The DEA typically inspects a facility to review its security measures prior to issuing a registration and on a periodic basis. Security requirements vary by controlled substance schedule, with the most stringent requirements applying to Schedule I and Schedule II substances. Required security measures include background checks on employees and physical control of inventory through measures such as cages, surveillance cameras and inventory reconciliations. Records must be maintained for the handling of all controlled substances, and periodic reports made to the DEA, for example distribution reports for Schedule I and II controlled substances, Schedule III substances that are narcotics, and other designated substances. Reports must also be made for thefts or losses of any controlled substance, and to obtain authorization to destroy any controlled substance. In addition, special permits and notification requirements apply to imports and exports of narcotic drugs. To enforce these requirements, the DEA conducts periodic inspections of registered establishments that handle controlled substances. Failure to maintain compliance with applicable requirements, particularly as manifested in loss or diversion, can result in administrative, civil or criminal enforcement action that could have a material adverse effect on our business, results of operations and financial condition. The DEA may seek civil penalties, refuse to renew necessary registrations, or initiate administrative proceedings to revoke those registrations. In some circumstances, violations could result in criminal proceedings.

In addition, a DEA quota system controls and limits the availability and production of controlled substances in Schedule I or II. Distributions of any Schedule I or II controlled substance or Schedule III narcotic must also be accompanied by special order forms, with copies provided to the DEA. Because AZSTARYS, APADAZ and our product candidates may be regulated as Schedule II controlled substances, they may be subject to the DEA's production and procurement quota scheme. The DEA establishes annually an aggregate quota for how much of a controlled substance may be produced in total in the United States based on the DEA's estimate of the quantity needed to meet legitimate scientific and medicinal needs. The limited aggregate amount of stimulants that the DEA allows to be produced in the United States each year is allocated among individual companies, which must submit applications annually to the DEA for individual production and procurement quotas. We and our contract manufacturers must receive an annual quota from the DEA in order to produce or procure any Schedule I or Schedule II substances for use in manufacturing of our product candidates. The DEA may adjust aggregate production quotas and individual production and procurement quotas from time to time during the year, although the DEA has substantial discretion in whether or not to make such adjustments. Our, or our contract manufacturers', quota of an active ingredient may not be sufficient to meet commercial demand or complete clinical trials. Any delay, limitation or refusal by the DEA in establishing our, or our contract manufacturers', quota for controlled substances could delay or stop our clinical trials or product launches, which could have a material adverse effect on our business, financial position and results of operations.

Individual states also independently regulate controlled substances. We and our contract manufacturers will be subject to state regulation on distribution of these products, including, for example, state requirements for licensures or registration.

Other Healthcare Regulatory Frameworks

Our business activities, including but not limited to, research, sales, promotion, distribution, medical education and other activities are subject to regulation by numerous regulatory and law enforcement authorities in the United States in addition to the FDA, including potentially the Department of Justice, the U.S. Department of Health and Human Services and its various divisions, including the CMS and the Health Resources and Services Administration, the Department of Veterans Affairs, the Department of Defense and state and local governments. Our business activities must comply with numerous healthcare laws, including those described below. Compliance with government regulations requires the expenditure of substantial time and financial resources.

The federal Anti-Kickback Statute prohibits, among other things, any person or entity, from knowingly and willfully offering, paying, soliciting or receiving any remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward, or in return for, the referral of an individual for, or purchasing, leasing, ordering, or arranging for the purchase, lease or order of, any good, facility, item or service reimbursable under Medicare, Medicaid or other federal healthcare programs. The term remuneration has been interpreted broadly to include anything of value. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution. The exceptions and safe harbors are drawn narrowly and practices that involve remuneration that may be alleged to be intended to induce prescribing, purchasing or recommending may be subject to scrutiny if they do not qualify for an exception or safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the federal Anti-Kickback Statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all of its facts and circumstances. Additionally, a person or entity does not need to have actual knowledge of the federal Anti-Kickback Statute, or the specific intent to violate it to have committed a violation.

The federal civil and criminal false claims laws, including the federal False Claims Act, which can be enforced by private citizens through civil whistleblower or *qui tam* actions, prohibit, among other things, any person or entity from knowingly presenting, or causing to be presented, a false claim for payment to, or approval by, the federal government, including the Medicare and Medicaid programs, or knowingly making, using, or causing to be made or used a false record or statement material to a false or fraudulent claim or to avoid, decrease or conceal an obligation to pay money to the federal government. In addition, a claim for payment of items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act.

The civil monetary penalties statute imposes penalties against any person or entity who, among other things, is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent.

The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, created additional federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of whether the payor is public or private, knowingly and willfully embezzling or stealing from a health care benefit program, willfully obstructing a criminal investigation of a health care offense and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute, or the specific intent to violate it, to have committed a violation.

Additionally, the federal Physician Payments Sunshine Act and its implementing regulations, require certain manufacturers of drugs, devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with specified exceptions) to report annually information related to specified payments or other transfers of value provided to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other healthcare providers (physician assistants, nurse practitioners, clinical nurse specialists, certified nurse anesthetists, anesthesiologist assistants, and certified nurse-midwives) and teaching hospitals, or to entities or individuals at the request of, or designated on behalf of, such providers and teaching hospitals and to report annually specified ownership and investment interests held by physicians and their immediate family members.

Depending on the circumstances, failure to comply with these laws can result in significant penalties, including criminal, civil and/or administrative penalties, damages, fines, disgorgement, debarment from government contracts, individual imprisonment, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, exclusion from government programs, refusal to allow us to enter into supply contracts, including government contracts, reputational harm, diminished profits and future earnings and the curtailment or restructuring of our operations, any of which could adversely affect our business.

Data Privacy and Security Laws

We may be subject to data privacy and security laws, regulations, and standards by foreign, federal, state and local governments that govern the collection, use, access to, confidentiality and security of health-related and other personal information. In the United States, numerous federal and state laws and regulations, including data breach notification laws, health information privacy and security laws and consumer protection laws and regulations govern the collection, use, disclosure, and protection of health-related and other personal information. In addition, certain foreign laws govern the privacy and security of personal data, including health-related data. Privacy and security laws, regulations, and other obligations are constantly evolving, may conflict with each other to complicate compliance efforts, and can result in investigations, proceedings, or actions that lead to significant civil and/or criminal penalties and restrictions on data processing.

Healthcare Reform Measures

The United States and some foreign jurisdictions are considering or have enacted a number of legislative and regulatory proposals designed to change the healthcare system in ways that could affect our ability to sell our products profitably. Among policy makers and payors in the United States and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and/or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives.

For example, in March 2010, the ACA was passed, which has substantially changed health care financing by both governmental and private insurers, and significantly affected the U.S. pharmaceutical industry. The ACA, among other things, subjected manufacturers to new annual fees and taxes for specified branded prescription drugs, increased the minimum Medicaid rebates owed by most manufacturers under the Medicaid Drug Rebate Program, expanded health care fraud and abuse laws, revised the methodology by which rebates owed by manufacturers to the state and federal government for covered outpatient drugs under the Medicaid Drug Rebate Program are calculated, imposed an inflation penalty on new formulations of drugs, extended the Medicaid Drug Rebate program to utilization of prescriptions of individuals enrolled in Medicaid managed care organizations, expanded the 340B program which caps the price at which manufacturers can sell covered outpatient pharmaceuticals to specified hospitals, clinics and community health centers, and provided incentives to programs that increase the federal government's comparative effectiveness research.

Since its enactment there have been executive, judicial and congressional challenges to certain aspects of the ACA. On June 17, 2021, the U.S. Supreme Court dismissed the most recent judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the ACA. Prior to the Supreme Court's decision, President Biden issued an executive order to initiate a special enrollment period from February 15, 2021, through August 15, 2021, for purposes of obtaining health insurance coverage through the ACA marketplace. The executive order also instructed certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the ACA.

Other legislative changes have been proposed and adopted in the United States since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, included aggregate reductions of Medicare payments to providers, which went into effect in April 2013, and, due to subsequent legislative amendments, will remain in effect through 2032 with the exception of a temporary suspension from May 1, 2020, through March 31, 2022, unless additional Congressional action is taken. In addition, in January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several categories of healthcare providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. On March 11, 2021, the American Rescue Plan Act of 2021 was signed into law, which eliminates the statutory Medicaid drug rebate cap, currently set at 100% of a drug's average manufacturer price, beginning January 1, 2024.

Further, there has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the cost of drugs under Medicare, and reform government program reimbursement methodologies for drugs. On August 16, 2022, the Inflation Reduction Act of 2022, or IRA, was signed into law. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023), and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of the Department of Health and Human Services to implement many of these provisions through guidance, as opposed to regulation, for the initial years. For that and other reasons, it is currently unclear how the IRA will be effectuated. At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. It is also possible that additional governmental action is taken in response to the COVID-19 pandemic.

We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare product candidates and services, which could result in reduced demand for our products or additional pricing pressures.

The Foreign Corrupt Practices Act

The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering or authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with accounting provisions requiring the companies to maintain books and records that accurately and fairly reflect all transactions of the companies, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Foreign Regulation

In addition to regulations in the United States, we will be subject to a variety of foreign regulations governing clinical trials and commercial sales and distribution of our products to the extent we choose to develop or sell any products outside of the United States. The approval process varies from country to country and the time may be longer or shorter than that required to obtain FDA approval. The requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary greatly from country to country.

Employees

As of December 31, 2022, we employed 36 employees, 32 of which were full-time employees.

Corporate Information

We were incorporated under the laws of the State of Iowa in October 2006 and were reincorporated under the laws of the State of Delaware in May 2014. We changed our name from KemPharm, Inc. to Zevra Therapeutics, Inc. effective as of February 21, 2023.

ITEM 1A. RISK FACTORS.

You should carefully consider all the risk factors and uncertainties described below, in addition to other information contained in this Annual Report on Form 10-K, including the section of this report titled “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and our financial statements and related notes, before investing in our common stock. If any of the following risks materialize, our business, financial condition and results of operations could be seriously harmed. This Annual Report on Form 10-K also contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those anticipated in the forward-looking statements because of factors that are described below and elsewhere in this Annual Report on Form 10-K.

Risks Related to the Development of Our Product Candidates

If commercialization of our approved products, or any of our product candidates, if approved, are not successful, or we experience significant delays in commercialization, our business will be harmed.

We currently generate minimal commercial revenue from the sale of our approved products and we may never be able to successfully commercialize a product candidate. We cannot guarantee that we, Corium, or any other collaborators will be able to successfully develop, manufacture or commercialize our approved products, or product candidates, if approved, or that we will ever receive any future payments under the AZSTARYS License Agreement or the APADAZ License Agreement.

We have invested substantially all of our internal discovery and development efforts and much of our financial resources in the development of our proprietary LAT ® platform technology, the identification of potential product candidates and the development of our product candidates. Our ability to generate revenue from our approved products and any of our product candidates, if approved, will depend heavily on their successful development and eventual commercialization. The success of our approved products and any of our product candidates will depend on several factors, including:

- successful completion of preclinical studies and requisite clinical trials;
- successful completion and achievement of endpoints in our clinical trials;
- demonstration that the risks involved with our approved products and any of our product candidates are outweighed by the benefits;
- successful development of our manufacturing processes for our approved products and for any of our product candidates, including entering into and maintaining arrangements with third-party manufacturers;
- successful completion of an FDA preapproval inspection of the facilities used to manufacture any of our product candidates for which we may submit an NDA;
- receipt of timely marketing approvals from applicable regulatory authorities, including, if applicable, the determination by the DEA of the controlled substance schedule for a product candidate, taking into account the recommendation of the FDA;
- obtaining differentiating claims in the labels for our product candidates;
- obtaining and maintaining patent, trademark and trade secret protection and regulatory exclusivity for our approved products and any of our product candidates and otherwise protecting our rights in our intellectual property portfolio;
- maintaining compliance with regulatory requirements, including cGMPs;
- launching commercial sales of our approved products, and any of our product candidates, if and when approved, whether alone or in collaboration with Corium or others;
- acceptance of our approved products and any of our product candidates, if approved, by patients, the medical community and third-party payors;
- competing effectively with other therapies;
- obtaining and maintaining healthcare coverage and adequate reimbursement; and
- maintaining a continued acceptable safety and efficacy profile of any of our products following approval.

Whether regulatory approval will be granted is unpredictable and depends upon numerous factors, including the substantial discretion of the regulatory authorities. If, following submission, our NDA for a product candidate is not accepted for substantive review or approval, the FDA or other comparable foreign regulatory authorities may require that we conduct additional studies or clinical trials, provide additional data, take additional manufacturing steps or require other conditions before they will reconsider our application. If the FDA or other comparable foreign regulatory authorities require additional studies, clinical trials or data, we would incur increased costs and delays in the marketing approval process, which may require us to expend more resources than we have available. In addition, the FDA or other comparable foreign regulatory authorities may not consider sufficient any additional required studies, clinical trials, data or information that we perform and complete or generate, or we may decide to abandon the program.

It is possible that none of our product candidates in clinical development or any of our future product candidates will ever obtain regulatory approval, even if we expend substantial time and resources seeking such approval.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays, we could experience an inability to successfully commercialize any of our approved products or product candidates, if approved, or we could experience an inability to successfully commercialize any of our product candidates approved for marketing in the future, if any, which would harm our business.

Our research and development activities are focused on discovering and developing proprietary prodrugs, and we are taking an innovative approach to discovering and developing prodrugs, which may never lead to marketable prodrug products.

A key element of our strategy is to use our proprietary LAT platform technology to build a pipeline of prodrugs and progress product candidates based on these prodrugs through clinical development for the treatment of a variety of diseases and conditions. The scientific discoveries that form the basis for our efforts to discover and develop prodrugs are relatively new. As our scientific efforts are primarily focused on discovering novel prodrugs with new molecular structures, the evidence to support the feasibility of developing product candidates based on these discoveries is both preliminary and limited. Although our research and development efforts to date have resulted in a pipeline of prodrug product candidates, we may not be able to develop those product candidates into prodrugs that are bioequivalent, safe and/or effective or that offer commercially significant improvements over already approved drugs. Even if we are successful in continuing to build our pipeline, the potential product candidates that we identify may not be suitable for clinical development, for reasons including being shown to have harmful side effects, a lack of efficacy, or other characteristics that indicate that they are unlikely to be prodrugs that will receive marketing approval and achieve market acceptance. If our approved products are not successfully commercialized and we do not successfully develop and commercialize any of our product candidates based upon our proprietary LAT platform technology, we will not be able to obtain product revenue in future periods, which likely would result in significant harm to our financial position and adversely affect our stock price.

If we are not able to obtain required regulatory approvals for any of our product candidates, or the approved labels are not sufficiently differentiated from other competing products, we will not be able to commercialize them and our ability to generate revenue or profits or to raise future capital could be limited.

The research, testing, manufacturing, labeling, packaging, storage, approval, sale, marketing, advertising and promotion, pricing, export, import and distribution of drug products are subject to extensive regulation by the FDA and other regulatory authorities in the United States and other countries, which regulations differ from country to country and change over time. We are not permitted to market any of our product candidates in the United States until we receive approval of an NDA from the FDA, or in any foreign countries until we receive the requisite approvals in such countries. In the United States, the FDA generally requires the completion of non-clinical testing and clinical trials of each drug to establish its safety and efficacy and extensive pharmaceutical development to ensure its quality and other factors before an NDA is approved. Regulatory authorities in other jurisdictions impose similar requirements. Of the large number of drugs in development, only a small percentage result in the submission of an NDA to the FDA and even fewer are approved for commercialization.

Even if regulatory approval is obtained, subsequent safety, efficacy, quality or other issues can result in a product approval being suspended or withdrawn, or the approved label for any approved product may not be sufficiently differentiated from other competing products to support market adoption thereof. In March 2021, the FDA approved the NDA for AZSTARYS, a once-daily product for the treatment of ADHD in patients ranging from six years and older. In February 2018, we announced that the FDA approved the NDA for APADAZ for the short-term (no more than 14 days) management of acute pain severe enough to require an opioid analgesic and for which alternative treatments are inadequate. Even with the regulatory approval of AZSTARYS and APADAZ by the FDA, we cannot guarantee that the FDA will approve any of our product candidates for commercial sale or approve any proposed label we may have for any such product candidate. If our development efforts for our product candidates, including our efforts to obtain regulatory approval, are not successful for their planned indications or are delayed, or if adequate demand for our product candidates that are approved for marketing, if any, is not generated, our business will be harmed.

The success of our product candidates will depend on the receipt and maintenance of regulatory approval and the issuance and maintenance of such approval is uncertain and subject to a number of risks, including the following:

- the FDA or comparable foreign regulatory authorities, institutional review boards, or IRBs, or ethics committees may disagree with the design or conduct of our clinical trials;
- the results of our clinical trials may not meet the level of statistical or clinical significance required by the FDA or other regulatory agencies for marketing approval or for us to receive approval for claims that are necessary for commercialization;
- the dosing in a particular clinical trial may not be at an optimal level;
- patients in our clinical trials may suffer adverse effects for reasons that may or may not be related to our product candidates;
- the data collected from clinical trials may not be sufficient to support submissions to regulatory authorities or to obtain regulatory approval in the United States or elsewhere;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies or may later suspend or withdraw such approval;
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval; and
- even if we obtain marketing approval in one or more countries, future safety or other issues could result in the suspension or withdrawal of regulatory approval in such countries.

We have only limited experience in submitting the applications necessary to gain regulatory approvals and have relied, and expect to continue to rely, on consultants and third-party contract research organizations, or CROs, with expertise in this area to assist us in this process. Securing FDA approval requires the submission of extensive non-clinical and clinical data, information about product manufacturing processes and inspection of facilities and supporting information to the FDA for each therapeutic indication to establish a product candidate's safety and efficacy for each indication and manufacturing quality. Additionally, we cannot guarantee that regulators will agree with our assessment of the results of the clinical trials we have conducted or that any future trials will be successful.

Any product candidates we develop may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining regulatory approval or prevent or limit commercial use with respect to one or all intended indications.

The process of obtaining regulatory approvals is expensive, often takes many years, if approval is obtained at all, and can vary substantially based upon, among other things, the type, complexity and novelty of the product candidates involved, the jurisdiction in which regulatory approval is sought and the substantial discretion of the regulatory authorities. Changes in the regulatory approval policy during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for a submitted product application may cause delays in the approval or rejection of an application or may result in future withdrawal of approval. Regulatory approval obtained in one jurisdiction does not necessarily mean that a product candidate will receive regulatory approval in all jurisdictions in which we may seek approval, but the failure to obtain approval in one jurisdiction may negatively impact our ability to seek approval in a different jurisdiction. Failure to obtain regulatory marketing approval of our product candidates in any indication will prevent us from commercializing those product candidates for that indication, and our ability to generate revenue will be impaired.

Disruptions at the FDA and other government agencies caused by funding shortages or global health concerns could hinder their ability to hire, retain or deploy key leadership and other personnel, or otherwise prevent new or modified products from being developed, approved or commercialized in a timely manner or at all, which could negatively impact our business.

The ability of the FDA to review and or approve new products can be affected by a variety of factors, including government budget and funding levels, statutory, regulatory, and policy changes, the FDA's ability to hire and retain key personnel and accept the payment of user fees, and other events that may otherwise affect the FDA's ability to perform routine functions. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable. Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities.

In response to the COVID-19 pandemic, the FDA announced its intention to postpone most inspections of foreign and domestic manufacturing facilities at various points. Even though the FDA has since resumed standard inspection operations of domestic facilities where feasible, the FDA has continued to monitor and implement changes to its inspectional activities to ensure the safety of its employees and those of the firms it regulates as it adapts to the evolving COVID-19 pandemic, and any resurgence of the virus or emergence of new variants may lead to further inspection delays. Regulatory authorities outside the United States may adopt similar restrictions or other policy measures in response to the COVID-19 pandemic. If a prolonged government shutdown occurs, or if global health concerns continue to prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities, it could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

If the FDA does not conclude that our product candidates satisfy the requirements for the 505(b)(2) NDA pathway as anticipated, the approval pathway for our product candidates will likely take significantly longer, cost significantly more and entail significantly greater complications and risks than anticipated, and the FDA may not ultimately approve our product candidates.

A key element of our strategy is to seek FDA approval for most of our product candidates under Section 505(b)(2) of the FFDCA, otherwise known as the 505(b)(2) NDA pathway with any NDA submitted thereunder a 505(b)(2) NDA, where possible. The 505(b)(2) NDA pathway permits the filing of an NDA where at least some of the information required for approval comes from studies not conducted by or for the applicant and for which the applicant has not obtained a right of reference. Section 505(b)(2), if applicable to us under the FFDCA, would allow an NDA we submit to the FDA to rely in part on data in the public domain or the FDA's prior conclusions regarding the safety and effectiveness of approved compounds, which could expedite the development program for our future product candidates by potentially decreasing the amount of nonclinical and/or clinical data that we would need to generate in order to obtain FDA approval. Such reliance is typically predicated on a showing of bioequivalence or comparable bioavailability to an approved drug. AZSTARYS was approved via the 505(b)(2) NDA pathway on March 2, 2021, and APADAZ was approved via the 505(b)(2) NDA pathway on February 23, 2018.

If the FDA does not allow us to pursue the 505(b)(2) NDA pathway as anticipated, or if we cannot demonstrate bioequivalence or comparable bioavailability of our product candidates to approved products, we may need to conduct additional clinical trials, provide additional data and information, and meet additional standards for regulatory approval. Moreover, even if the FDA does allow us to pursue the 505(b)(2) NDA pathway, depending on the product candidate, we may still need to conduct additional clinical trials, including clinical trials to assess product safety or efficacy. If this were to occur, the time and financial resources required to obtain FDA approval for our product candidates, and complications and risks associated with our product candidates, would likely substantially increase.

Moreover, our inability to pursue the 505(b)(2) NDA pathway could result in new competitive products reaching the market more quickly than our product candidates, which could hurt our competitive position and our business prospects. Even if we are allowed to pursue the 505(b)(2) NDA pathway, we cannot assure you that our product candidates will receive the requisite approvals for commercialization on a timely basis, if at all. Other companies may achieve product approval of similar products before we do, which would delay our ability to obtain product approval, expose us to greater competition, and would require that we seek approval via alternative pathways.

In addition, notwithstanding the approval of several products by the FDA under Section 505(b)(2) over the last few years, pharmaceutical companies and others have objected to the FDA's interpretation of Section 505(b)(2). If the FDA's interpretation of Section 505(b)(2) is successfully challenged, the FDA may change its policies and practices with respect to Section 505(b)(2) regulatory approvals, which could delay or even prevent the FDA from approving any NDA that we submit under Section 505(b)(2). In addition, the pharmaceutical industry is highly competitive, and Section 505(b)(2) NDAs are subject to certain requirements designed to protect the patent rights of sponsors of previously approved drugs that are referenced in a Section 505(b)(2) NDA. These requirements may give rise to patent litigation and mandatory delays in approval of our NDAs for up to 30 months or longer depending on the outcome of any litigation. It is not uncommon for a manufacturer of an approved product to file a citizen petition with the FDA seeking to delay approval of, or impose additional approval requirements for, pending competing products. If successful, such petitions can significantly delay, or even prevent, the approval of a new product. Even if the FDA ultimately denies such a petition, the FDA may substantially delay approval while it considers and responds to the petition. In addition, even if we are able to utilize the Section 505(b)(2) regulatory pathway, there is no guarantee this would ultimately lead to streamlined product development or earlier approval.

Clinical drug development involves a lengthy and expensive process, with an uncertain outcome. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.

The risk of failure for our product candidates is high. It is impossible to predict when or if any of our current product candidates will prove effective or safe in humans and will receive regulatory approval. Before obtaining marketing approval from regulatory authorities for the sale of any product candidate, we must complete preclinical development and then conduct clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of preclinical studies and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Interpretation of results from early, usually smaller, studies that suggest positive trends in some subjects, requires caution. Results from later stages of clinical trials enrolling more subjects may fail to show the desired safety and efficacy results or otherwise fail to be consistent with the results of earlier trials of the same product candidates. Later clinical trial results may not replicate earlier clinical trials for a variety of reasons, including differences in trial design, different trial endpoints, or lack of trial endpoints in exploratory studies, subject population, number of subjects, subject selection criteria, trial duration, drug dosage and formulation and lack of statistical power in the earlier studies. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products.

We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval o commercialize our product candidates, including:

- regulators or IRBs may not authorize us or our investigators to commence a clinical trial, conduct a clinical trial at a prospective trial site or amend clinical trial protocols as needed;
- we may experience delays in reaching, or fail to reach, agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites and CROs;
- clinical trials of our product candidates may produce negative or inconclusive results, including failure to demonstrate statistical significance in cases where that is required, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon prodrug development programs;
- we may be unable to obtain sufficient or adequate supply or quality of product candidates or other materials necessary for use in clinical trials, or experience delays in sufficiently developing, characterizing or controlling a manufacturing process suitable for clinical trials;
- we may experience delays in manufacturing, testing, releasing, validating or importing/exporting sufficient stable quantities of our product candidates for use in clinical trials or the inability to do any of the foregoing;
- the number of subjects required for clinical trials of our product candidates may be larger than we anticipate enrollment in these clinical trials may be slower than we anticipate, or participants may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or trial protocols, or meet their contractual obligations to us in a timely manner, or at all;
- regulators or IRBs may require that we or our investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of our product candidates may be greater than we anticipate, including if we are not able to pursue the 505(b)(2) NDA pathway for approval of our product candidates;
- we will need to pay substantial application user fees, which we may not be able to afford;
- we may be required to transfer manufacturing processes to larger-scale facilities operated by a contract manufacturing organization, and we may experience delays or failures by our contract manufacturers to make any necessary changes to such manufacturing process;
- we may abandon our development program or programs based on the changing regulatory or commercial environment;
- regulatory authorities may not agree with our trial design or implementation; and
- our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators or IRBs to suspend or terminate the trials.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our product candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining marketing approval for our product candidates;
- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval but without the claims necessary for us to successfully commercialize our product candidates;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to additional post-marketing testing, surveillance, or other requirements, such as REMS; or
- have the product removed from the market after obtaining marketing approval.

In addition, disruptions caused by the COVID-19 pandemic may increase the likelihood that we encounter such difficulties or delays in initiating, enrolling, conducting or completing our planned and ongoing clinical trials. Any inability to successfully initiate or complete clinical trials could result in additional costs to us or impair our ability to generate revenue from product sales. In addition, if we make manufacturing or formulation changes to our product candidates, we may be required to or we may elect to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays could also shorten any periods during which our products have patent protection and may allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may seriously harm our business.

Clinical trials must be conducted in accordance with the FDA and other applicable regulatory authorities' legal requirements, regulations or guidelines, and are subject to oversight by these governmental agencies and Ethics Committees or IRBs at the medical institutions where the clinical trials are conducted. We could encounter delays if a clinical trial is suspended or terminated by us, by the data safety monitoring board for such trial or by the FDA or any other regulatory authority, or if the IRBs of the institutions in which such trials are being conducted suspend or terminate the participation of their clinical investigators and sites subject to their review. Such authorities may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a product candidate, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

Further, conducting clinical trials in foreign countries, as we may do for our product candidates, presents additional risks that may delay completion of our clinical trials. These risks include the failure of enrolled patients in foreign countries to adhere to clinical protocol as a result of differences in healthcare services or cultural customs, managing additional administrative burdens associated with foreign regulatory schemes, as well as political and economic risks relevant to such foreign countries.

Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the study. The FDA or comparable foreign regulatory authority may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or comparable foreign regulatory authority, as the case may be, and may ultimately lead to the denial of marketing approval of one or more of our product candidates.

Delays in the completion of any clinical trial of our product candidates will increase our costs, slow down our product development and the regulatory approval processes and delay or potentially jeopardize our ability to commence product sales and generate product revenue. Any of these occurrences may harm our business, financial condition and prospects significantly.

If we experience delays or difficulties in the enrollment of subjects in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

We may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible subjects to participate in these trials as may be required by the FDA or similar regulatory authorities outside the United States. We cannot predict how successful we will be at enrolling subjects in future clinical trials. If we are not successful at enrolling subjects in one clinical trial, it may affect when we are able to initiate our next clinical trial, which could result in significant delays in our efforts to pursue regulatory approval of and commercialize our product candidates. In addition, some of our competitors have ongoing clinical trials to treat the same indications as our product candidates, and subjects who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors. Subject enrollment is affected by other factors including:

- the size and nature of the subject population specified in the trial protocol;
- the eligibility criteria for the study in question;
- the perceived risks and benefits of the product candidate under study;
- the fact that the product candidate is a controlled substance;
- severe or unexpected drug-related adverse events experienced by subjects in a clinical trial;
- the availability of drugs approved to treat the diseases or conditions under study;
- the efforts to facilitate timely enrollment in clinical trials;
- the patient referral practices of physicians;
- the severity of the disease or condition under investigation;
- the ability to obtain and maintain subject informed consent;
- the ability to retain subjects in the clinical trial and their return for follow-up;
- the clinical trial design, including required tests, procedures and follow-up;
- the ability to monitor subjects adequately during and after treatment;
- delays in adding new investigators and clinical sites;
- withdrawal of clinical trial sites from clinical trials; and
- the proximity and availability of clinical trial sites for prospective subjects.

Our inability to enroll a sufficient number of subjects for clinical trials would result in significant delays and could require us to abandon one or more clinical trials altogether. Enrollment delays in these clinical trials may result in increased development costs for our product candidates, which could cause our value to decline and limit our ability to obtain additional financing.

Interim, topline, or preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publicly disclose topline or preliminary data from our preclinical studies and clinical trials, which is based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations, and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the preliminary or topline results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data has been received and fully evaluated. Topline data also remains subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, preliminary or topline data should be viewed with caution until the final data are available.

From time to time, we may also disclose interim data from our preclinical studies and clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available or as patients from our clinical trials continue other treatments for their disease. Adverse differences between preliminary or interim data and final data could significantly harm our business prospects.

Further, disclosure of interim data by us or by our competitors could result in volatility in the price of our common stock. Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions, or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate, and our company in general. If the interim, topline, or preliminary data that we report differs from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, any of our potential product candidates may be harmed, which could harm our business, operating results, prospects, or financial condition.

We may not be able to obtain or maintain orphan drug designations which we pursue for any of our product candidates, and we may be unable to maintain the benefits associated with orphan drug designation, including the potential for market exclusivity.

Regulatory authorities in some jurisdictions, including the United States, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, the FDA may designate a drug as an orphan product if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the United States, or a patient population of greater than 200,000 individuals in the United States, but for which there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. We have not received orphan drug designation in for any product candidate. We may seek to obtain orphan drug designation for product but there can be no assurance that the FDA will grant orphan designation for any indication for which we apply, or that we will be able to maintain such designation.

In the United States, orphan designation entitles a party to financial incentives such as opportunities for grant funding toward clinical trial costs, tax advantages and user-fee waivers. In addition, if a product candidate that has orphan designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusivity, which means that the FDA may not approve any other applications, including an NDA, to market the same product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity or where the manufacturer is unable to assure sufficient product quantity.

Even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA or comparable foreign regulatory authority can subsequently approve the same drug for the same condition if such regulatory authority concludes that the later drug is clinically superior because it is shown to be safer, more effective or makes a major contribution to patient care.

Orphan drug exclusivity may also be lost if the FDA later determines that the initial request for designation was materially defective. In addition, orphan drug exclusivity does not prevent the FDA from approving competing drugs for the same or similar indication containing a different active ingredient. In addition, if a subsequent drug is approved for marketing for the same or a similar indication as any of our product candidates that receive marketing approval, we may face increased competition and lose market share regardless of orphan drug exclusivity. Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

Our approved products and certain of our product candidates contain controlled substances, the manufacture, use, sale, importation, exportation, prescribing and distribution of which are subject to regulation by the DEA and other regulatory agencies.

Our approved products and certain of our product candidates are regulated as controlled substances, which are subject to state, federal, and foreign laws and regulations regarding their manufacture, use, sale, importation, exportation, and distribution. Among other things, controlled substances are regulated under the federal Controlled Substances Act of 1970, or CSA, and regulations of the DEA. Before we can commercialize any of our products or product candidates, if approved, the DEA will need to determine the controlled substance schedule, taking into account the recommendation of the FDA. This may be a lengthy process that could delay our marketing of a product candidate and could potentially diminish any regulatory exclusivity periods for which we may be eligible.

The DEA regulates controlled substances as Schedule I, II, III, IV or V substances. Schedule I substances by definition have no established medicinal use and may not be marketed or sold in the United States. A pharmaceutical product may be listed as Schedule II, III, IV or V, with Schedule II substances considered to present the highest risk of abuse and Schedule V substances the lowest relative risk of abuse among such substances. The DEA determined APADAZ and AZSTARYS to be Schedule II controlled substances. In addition, the DEA scheduled SDX as a Schedule IV substance. We expect that most of our product candidates, including KP879 and KP1077, if approved, will also be regulated as "controlled substances" by the DEA, which subjects AZSTARYS, APADAZ and these product candidates to additional restrictions regarding their manufacture, shipment, storage, sale and use, depending on the scheduling of the active ingredients, and may limit the commercial potential of our products and any of our product candidates, if approved.

Various states also independently regulate controlled substances. Though state controlled substances laws often mirror federal law, because the states are separate jurisdictions, they may separately schedule drugs as well. While some states automatically schedule a drug when the DEA does so, in other states there must be rulemaking or a legislative action. State scheduling may delay commercial sale of any controlled substance drug product for which we obtain federal regulatory approval and adverse scheduling could impair the commercial attractiveness of such product. We or our collaborators must also obtain separate state registrations in order to be able to obtain, handle and distribute controlled substances for clinical trials or commercial sale, and failure to meet applicable regulatory requirements could lead to enforcement and sanctions from the states in addition to those from the DEA or otherwise arising under federal law.

For any of our products or product candidates classified as controlled substances, we and our suppliers, manufacturers, contractors, customers and distributors are required to obtain and maintain applicable registrations from state, federal and foreign law enforcement and regulatory agencies and comply with state, federal and foreign laws and regulations regarding the manufacture, use, sale, importation, exportation and distribution of controlled substances. There is a risk that DEA regulations may limit the supply of the compounds used in clinical trials for our product candidates, and, in the future, the ability to produce and distribute our products in the volume needed to meet commercial demand. For example, a DEA quota system controls and limits the availability and production of controlled substances in Schedule I or II. Because our products and most of our product candidates are or may be regulated as Schedule II controlled substances, they may be subject to the DEA's production and procurement quota scheme. The DEA establishes annually an aggregate quota for how much of a controlled substance may be produced in total in the United States based on the DEA's estimate of the quantity needed to meet legitimate scientific and medicinal needs. Manufacturers of Schedule I and II controlled substances are required to apply for quotas on an annual basis. If we or our contract manufacturers or suppliers do not obtain a sufficient quota from the DEA, we may not be able to obtain sufficient quantities of these controlled substances in order to complete our clinical trials or meet commercial demand for any of our products or product candidates classified under Schedule II.

Regulations associated with controlled substances govern manufacturing, labeling, packaging, testing, dispensing, production and procurement quotas, recordkeeping, reporting, handling, shipment and disposal. These regulations increase the personnel needs and the expense associated with development and commercialization of product candidates including controlled substances. The DEA, and some states, conduct periodic inspections of registered establishments that handle controlled substances. Failure to obtain and maintain required registrations or comply with any applicable regulations could delay or preclude us from developing and commercializing our product candidates containing controlled substances and subject us to enforcement action. The DEA may seek civil penalties, refuse to renew necessary registrations or initiate proceedings to revoke those registrations. In some circumstances, violations could lead to criminal proceedings. Because of their restrictive nature, these regulations could limit commercialization of any of our products or product candidates that are classified as controlled substances.

Our products and product candidates may be associated with serious adverse events, undesirable side effects or have other properties that could halt their clinical development, prevent their regulatory approval, limit their commercial potential or result in significant negative consequences.

Adverse events or other undesirable side effects caused by our products or product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approvals by the FDA or other comparable foreign regulatory authorities.

During the conduct of clinical trials, patients report changes in their health, including illnesses, injuries, and discomforts, to their study doctor. Often, it is not possible to determine whether or not the product candidate being studied caused these conditions. It is possible that as we test our product candidates in larger, longer and more extensive clinical trials, or as use of these product candidates becomes more widespread if they receive regulatory approval, illnesses, injuries, discomforts and other adverse events that were observed in previous trials, as well as conditions that did not occur or went undetected in previous trials, will be reported by patients. Many times, side effects are only detectable after investigational products are tested in large-scale clinical trials or, in some cases, after they are made available to patients on a commercial scale following approval.

If any serious adverse events occur, clinical trials or commercial distribution of any product candidates or products we develop could be suspended or terminated, and our business could be seriously harmed. Treatment-related side effects could also affect patient recruitment and the ability of enrolled patients to complete the trial or result in potential liability claims. Regulatory authorities could order us to cease further development of, deny approval of, and/or require us to cease selling our products or product candidates, if approved, for any or all targeted indications. If we are required to delay, suspend or terminate any clinical trial or commercialization efforts, the commercial prospects of our products or product candidates may be harmed, and our ability to generate product revenues from them or other product candidates that we develop may be delayed or eliminated. Additionally, if one or more of our product candidates receives marketing approval and we or others later identify undesirable side effects or adverse events caused by such products, a number of potentially significant negative consequences could result, including but not limited to:

- regulatory authorities may suspend, limit or withdraw approvals of such product, or seek an injunction against its manufacture or distribution;
- regulatory authorities may require additional warnings on the label, including “boxed” warnings, or issue safety alerts, Dear Healthcare Provider letters, press releases or other communications containing warnings or other safety information about the product;
- we may be required to change the way the product is administered or conduct additional clinical trials or post-approval studies;
- we may be required to create a risk evaluation and mitigation strategy, or REMS, which could include a medication guide outlining the risks of such side effects for distribution to patients;
- we may be subject to fines, injunctions or the imposition of criminal penalties;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of our products or any of our product candidates, if approved, and could seriously harm our business.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and management resources, we focus on research programs and product candidates that we identify for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial drugs or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

Social issues around the abuse of opioids and stimulants, including law enforcement concerns over diversion and regulatory efforts to combat abuse, could decrease the potential market for our approved products or any of our other applicable product candidates.

Media stories regarding prescription drug abuse and the diversion of opioids, stimulants and other controlled substances are commonplace. Law enforcement and regulatory agencies may apply policies that seek to limit the availability of opioids and stimulants. Such efforts may inhibit the ability to commercialize our approved products or any of our other applicable product candidates. Aggressive enforcement and unfavorable publicity regarding, for example, the use or misuse of hydrocodone or other opioid drugs and stimulants, the limitations of abuse-deterrent formulations, public inquiries and investigations into prescription drug abuse, litigation or regulatory activity, sales, marketing, distribution or storage of our products could harm our reputation. Such negative publicity could reduce the potential size of the market for our approved products or any of our other applicable product candidates and decrease the revenue we are able to generate from their sale, if approved. Similarly, to the extent prescription drug abuse becomes a less prevalent or less urgent public health issue, regulators and third-party payors may not be willing to pay a premium for formulations with improved attributes of opioids or stimulants.

Additionally, efforts by the FDA and other regulatory bodies to combat abuse of opioids and stimulants may negatively impact the market for our approved products and any of our other applicable product candidates. For example, in April 2014, the FDA approved class-wide labeling changes to the indications for use of all approved ER/LA opioids, so that ER/LA opioids will be indicated only for the management of pain severe enough to require daily, around-the-clock, long-term opioid treatment and for which alternative treatment options are inadequate. These changes have reduced the number of prescriptions for opioids written by physicians and negatively impact the potential market for APADAZ or our other applicable product candidates. The FDA also held a public meeting in October 2014, on the development and regulation of abuse-deterrent formulations of opioid medications. Further, the Centers for Disease Control and Prevention previously issued draft guidelines for the prescribing of opioids for chronic pain, providing recommendations for primary care providers prescribing opioids for chronic pain on when to initiate or continue opioids, opioid selection and discontinuation, and the assessment of the risk and addressing harms of opioid use, among other areas. It is possible that FDA, or other regulatory bodies, will announce new regulatory initiatives at any time that may increase the regulatory burden or decrease the commercial opportunity for our approved products or any of our other applicable product candidates.

If the Arimoclomol EAP is terminated prior to commercialization of arimoclomol, if approved, it will have a material adverse effect on our business, results of operations, cash flows, financial condition and/or prospects.

Arimoclomol is currently available to NPC patients in the United States France, Germany, and other European Union countries through the Arimoclomol EAP. The EAP is expected to remain in place until arimoclomol becomes commercially available in each of the current EAP markets. If the Arimoclomol EAP is terminated prior to commercialization of arimoclomol, it will have a material adverse effect on our business, results of operations, cash flows, financial condition and/or prospects.

.

Risks Related to Our Financial Position and Capital Needs

Any failure to maintain effective internal control over financial reporting could harm us.

Our management is responsible for establishing and maintaining adequate internal control over financial reporting. Internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements in accordance with U.S. generally accepted accounting principles. If our management is unable to conclude that we have effective internal control over financial reporting, or to certify the effectiveness of such controls, or if material weaknesses in our internal control over financial reporting are identified in the future, we could be subject to regulatory scrutiny and a loss of public confidence, which could have a material adverse effect on our business and our stock price. In addition, if we do not maintain adequate financial and management personnel, processes and controls, we may not be able to manage our business effectively or accurately report our financial performance on a timely basis, which could cause a decline in our common stock price and adversely affect our results of operations and financial condition.

We have incurred significant recurring negative net operating cash flows. We expect to incur minimal positive net operating cash flows or negative ne operating cash flows over the next several years and may never achieve or maintain profitability.

We have historically had significant negative net operating cash flows since inception. For the year ended December 31, 2022, net cash used in operations was ($18.7) million. While for the year ended December 31, 2021, we had net cash provided by operations of $10.4 million. We have financed our operations through December 31, 2022 with funds raised in private placements of redeemable convertible preferred stock, the issuance of convertible promissory notes and term debt, our initial public offering and other public and private offerings of our common stock, as well as through revenue received under the AZSTARYS License Agreement, sales of arimoclomol under the Arimoclomol EAP, and the Corium Consulting Agreement.

We have devoted substantially all of our financial resources and efforts to research and development, including preclinical studies and clinical trials. We are in various stages of development of our product candidates, and we have only completed development of, and received regulatory approval for, two products, AZSTARYS and APADAZ. We expect to continue to incur significant expenses and operating losses over the next several years and our net losses may fluctuate significantly from quarter to quarter and year to year as we:

- continue our ongoing preclinical studies, clinical trials and our product development activities for our pipeline of product candidates;
- seek regulatory approvals for product candidates that successfully complete clinical trials;
- continue research and preclinical development and initiate clinical trials of our product candidates;
- seek to discover and develop additional product candidates either internally or in partnership with other pharmaceutical companies;
- adapt our regulatory compliance efforts to incorporate requirements applicable to marketed products;
- maintain, expand and protect our intellectual property portfolio;
- incur additional legal, accounting and other expenses in operating as a public company; and
- add operational systems and personnel, if needed, to support any future commercialization efforts.

To become and remain profitable, we must succeed in developing and eventually commercializing prodrugs that generate significant revenue. This will require us to be successful in a range of challenging activities, including completing preclinical studies and clinical trials and obtaining regulatory approval of our product candidates, and manufacturing, marketing and selling, whether ourselves or through a license with a third party, any of our product candidates for which we may obtain regulatory approval, as well as discovering and developing additional product candidates. We are in various stages of these activities for our product candidates and we cannot guarantee that any strategy we adopt will be successful. We may never succeed in commercialization activities and, even if we do, may never generate revenue that is significant enough to achieve profitability.

In addition, we cannot predict what the long-term effects of the COVID-19 pandemic or a future public health crisis and the resulting economic disruptions may have on our liquidity and results of operations. The extent of a future public health crisis on our liquidity and results of operations will depend on a number of future developments, including the duration, spread and intensity of such crisis, and governmental, regulatory and private sector responses, all of which are uncertain and difficult to predict. A future public health crisis may make it more difficult for us to enroll patients in any future clinical trials or cause delays in the regulatory approval of our product candidates. A portion of our projected revenue is based upon the achievement of milestones in the AZSTARYS License Agreement associated with regulatory matters that may be impacted by the COVID-19 pandemic. As a result, we cannot predict what, if any, impact that the COVID-19 pandemic or a future public health crisis may have on our ability to achieve these milestones. If the pandemic continues to be a severe worldwide crisis, or a future public health crisis prevails, it could have a material adverse effect on our business, results of operations, financial condition, and cash flows.

Because of the numerous risks and uncertainties associated with prodrug development, we are unable to accurately predict the timing or amount of expenses or when, or if, we will be able to achieve profitability. If we are required by regulatory authorities to perform studies in addition to those currently expected, or if there are any delays in the initiation and completion of our clinical trials or the development of any of our product candidates, our expenses could increase.

Even if we achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would depress our value and could impair our ability to raise capital, expand our business, maintain our research and development efforts, obtain product approvals, diversify our product offerings or continue our operations. A decline in our value could also cause you to lose all or part of your investment.

Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may not generate the necessary data or results required to obtain regulatory approval for our product candidates or claims necessary to make such candidates profitable and achieve product sales. In addition, our approved products or any of our product candidates, if approved, may not achieve commercial success. Our commercial revenue will be derived from sales of prodrug products. We cannot guarantee that Corium will be able to successfully commercialize AZSTARYS to any certain level, or any of the product candidates subject to the AZSTARYS License Agreement, even if approved, or that we will ever receive any additional payments under the AZSTARYS License Agreement from the commercial sales of AZSTARYS or any future payments under the AZSTARYS License Agreement, or that KVK will be able to successfully commercialize APADAZ, or that we will receive any future payments under the APADAZ License Agreement from commercial sales of APADAZ. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all. To the extent that we raise additional capital through the sale of equity or debt securities, the terms of those securities or debt may restrict our ability to operate. Any future debt financing and equity financing, if available, may involve agreements that include, covenants limiting and restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, entering into profit-sharing or other arrangements or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. In addition, we may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or altogether cease our research and development programs or future commercialization efforts.

We may need additional funding to pursue our business objectives. If we are unable to raise capital when needed, we could be forced to delay, reduce or altogether cease our prodrug development programs or commercialization efforts or cease operations altogether.

We expect that our only sources of revenues will be through payments arising from our license agreements with Corium, the Arimoclomol EAP, or through the Corium Consulting Agreement or other potential consulting arrangements and any other future arrangements related to one of our other product candidates. We cannot guarantee that we will be able to generate sufficient proceeds from the AZSTARYS License Agreement, the Arimoclomol EAP, Corium Consulting Agreement or other consulting arrangements, or be successful in completing other transactions, that will fully fund our operating expenses. Further, the recent economic uncertainty, may dramatically reduce our ability to secure debt or equity financing necessary to support our operations. If we are delayed in obtaining additional funding or are unable to complete a strategic transaction, we may discontinue our development activities on our product candidates or discontinue our operations. Our future capital requirements will depend on many factors, including:

- the progress and results of our preclinical studies, clinical trials, chemistry, manufacturing and controls, or CMC, and other product development and commercialization activities;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates;
- the ability to obtain differentiating claims in the labels for our product candidates;
- the number and development requirements of other product candidates that we may pursue;
- the costs, timing and outcome of regulatory review of our product candidates;
- the efforts necessary to institute post-approval regulatory compliance requirements;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any of our product candidates for which we receive marketing approval;
- the commercial revenue received from commercial sales of our approved products, or any of our product candidates subject to the terms of the AZSTARYS License Agreement, or sales of our product candidates for which we receive marketing approval in the future, which may be affected by market conditions, including obtaining coverage and adequate reimbursement of our approved products, or any of our product candidates, from third-party payors, including government programs and managed care organizations, and competition within the therapeutic class to which our approved products, or any our product candidates are assigned;
- the success of our partner, Corium, in commercializing AZSTARYS;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims; and
- the extent to which we acquire or in-license other product candidates and technologies.

Risks Related to Our Dependence on Third Parties

We rely on and expect to continue to rely on third parties to conduct our clinical trials for our product candidates, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.

We have engaged and expect to continue to engage CROs for our planned clinical trials of our product candidates. We rely on and expect to continue to rely on CROs, as well as other third parties, such as clinical data management organizations, medical institutions and clinical investigators, to conduct those clinical trials. Agreements with such third parties might terminate for a variety of reasons, including a failure to perform by the third parties. If we need to enter into alternative arrangements, our drug development activities would be delayed.

Our reliance on these third parties for research and development activities reduces our control over these activities but does not relieve us of our responsibilities. For example, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with regulatory standards, commonly referred to as good clinical practices, or GCPs, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. Regulatory authorities enforce these GCPs through periodic inspections of trial sponsors, investigators and trial sites. We also are required to register specified ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within specified timeframes. In addition, we must conduct our clinical trials with product produced under cGMP requirements. Failure to comply with these regulations may require us to repeat preclinical studies and clinical trials, which would delay the regulatory approval process. Failure to comply with the applicable requirements related to clinical investigations by us, our CROs or clinical trial sites can also result in clinical holds and termination of clinical trials, debarment, FDA refusal to approve applications based on the clinical data, warning letters, withdrawal of marketing approval if the product has already been approved, fines and other monetary penalties, delays, adverse publicity and civil and criminal sanctions, among other consequences.

Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates.

In addition, investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and may receive cash or equity compensation in connection with such services. If these relationships and any related compensation result in perceived or actual conflicts of interest, or the FDA concludes that the financial relationship may have affected the interpretation of the study, the integrity of the data generated at the applicable clinical trial site may be questioned and the utility of the clinical trial itself may be jeopardized, which could result in the delay or rejection of any NDA we submit by the FDA. Any such delay or rejection could prevent us from commercializing our product candidates. Further, our arrangements with investigators are also subject to scrutiny under other health care regulatory laws, such as the Anti-Kickback Statute.

We also rely on and expect to continue to rely on other third parties to store and distribute product supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of our products, producing additional losses and depriving us of potential product revenue.

If the third parties with whom we contract do not successfully carry out their contractual duties or obligations or meet expected deadlines or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated, we may need to conduct additional trials, and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenue could be delayed. To the extent we are unable to successfully identify and manage the performance of third-party service providers in the future, our business may be adversely affected.

We contract with third parties for the manufacture of our partnered products and product candidates that utilize benzhydrocodone and SDX as the API used in our clinical trials and with a sole source supplier for the manufacture of bulk quantities of benzhydrocodone and SDX used in the partnered products and product candidates that utilize these moieties as the API and we expect to continue to do so. This reliance on third-party manufacturers increases the risk that we will not have sufficient quantities of benzhydrocodone and SDX, or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.

We do not have any manufacturing facilities. We procure the bulk drug substances for our approved products and product candidates from sole-source, third-party manufacturers and the partnered products and product candidates that utilize these moieties as the API used in our clinical trials from other third parties. We anticipate we will continue to do so for the foreseeable future. We also expect to continue to rely on third parties as we proceed with preclinical and clinical testing of our product candidates, as well as for commercial manufacture of our approved products, or any of our product candidates should such candidates receive marketing approval. This reliance on third parties increases the risk that we will not have sufficient quantities of benzhydrocodone, SDX, other bulk drug substances or our partnered product or product candidates, or such quantities at an acceptable cost or quality, which could delay, prevent or impair our ability to timely conduct our clinical trials or our other development or commercialization efforts.

We may be unable to establish any future agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to maintain our existing third-party relationships or establish any such agreements with other third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- reliance on the third party for FDA and DEA regulatory compliance and quality assurance;
- the possible misappropriation of our proprietary information, including our trade secrets and know-how;
- disruption and costs associated with changing suppliers, including additional regulatory filings;
- the possible breach, termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us;
- a delay or inability to procure or expand sufficient manufacturing capacity;
- manufacturing and product quality issues related to scale-up of manufacturing;
- costs and validation of new equipment and facilities required for scale-up;
- the inability to negotiate manufacturing agreements with third parties under commercially reasonable terms;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us;
- the reliance on a limited number of sources, and in some cases, single sources for product components, such that if we are unable to secure a sufficient supply of these product components, we will be unable to manufacture and sell our product candidates in a timely fashion, in sufficient quantities or under acceptable terms; and
- carrier disruptions or increased costs that are beyond our control.

Any of these events could lead to clinical trial delays, failure to obtain regulatory approval or impact our ability to successfully commercialize our products, if approved. Some of these events could be the basis for FDA action, including injunction, recall, seizure or total or partial suspension of production.

The facilities used by our contract manufacturers to manufacture our approved products, and any of our product candidates are subject to review by the FDA pursuant to inspections that will be conducted after we submit our marketing application to the FDA, and such inspections could result in findings that lead to failure to obtain FDA approval of such marketing applications.

We do not, other than through our contractual arrangements, control the manufacturing process of our approved products, or any of our other product candidates, and we are completely dependent on, our contract manufacturing partners for compliance with cGMP requirements and for manufacture of both active drug substances and finished drug products. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or other regulatory authorities, we will not be able to secure and maintain regulatory approval of our marketing applications for the use of their manufacturing facilities for our products. In addition, other than through our contractual agreements, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA, or a comparable foreign regulatory authority, does not approve these facilities for the manufacturing of our approved products, or any of our other product candidates, or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain marketing approval for or market our approved products, or any of our other product candidates, if approved.

Further, for our approved products and any of our product candidates, if approved, our suppliers will be subject to regulatory requirements, covering manufacturing, testing, quality control and record keeping relating to our approved products or any of our product candidates, if approved, and subject to ongoing inspections by the regulatory agencies. Failure by any of our suppliers to comply with applicable regulations may result in long delays and interruptions to our manufacturing capacity while we seek to secure another supplier that meets all regulatory requirements, as well as market disruption related to any necessary recalls or other corrective actions.

Third-party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including warning letters, clinical holds or termination of clinical trials, fines, injunctions, restitution, disgorgement, civil penalties, delays, suspension or withdrawal of approvals or other permits, FDA refusal to approve pending applications, product detentions, FDA or DEA consent decrees placing significant restrictions on or suspending manufacturing and distribution operations, debarment, refusal to allow import or export, product detentions, adverse publicity, dear-health-care-provider letters or other warnings, license revocation, seizures or recalls of product candidates, operating restrictions, refusal of government contracts or future orders under existing contracts and civil and criminal liability, including False Claims Act liability, exclusion from participation in federal health care programs, and corporate integrity agreements among other consequences, any of which could significantly and adversely affect supplies of our prodrugs.

Our product candidates and any prodrugs that we may develop may compete with other product candidates and drugs for access to manufacturing facilities and we may be unable to obtain access to these facilities on favorable terms.

There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us. Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval. We do not currently have arrangements in place for redundant supply or a second source for our approved products or product candidates bulk drug substance. If our current contract manufacturer for our approved products or product candidates bulk drug substance cannot perform as agreed, we may be required to replace such manufacturer and we may incur added costs and delays in identifying and qualifying any such replacement. Any performance failure or significant delay could negatively affect our business, results of operations and financial condition.

We have entered into collaborations with Commave, to develop, manufacture and commercialize AZSTARYS worldwide, and KVK, for the commercialization of APADAZ in the United States. In addition, we may seek collaborations with third parties for the development or commercialization of APADAZ or our other product candidates, or in other territories. If those collaborations are not successful, we may not be able to capitalize on the market potential of AZSTARYS, APADAZ, or any of our other product candidates, if approved.

We entered into the AZSTARYS License Agreement with Commave pursuant to which we granted an exclusive, worldwide license to Commave to develop, manufacture and commercialize AZSTARYS. Corium was tasked by Commave to lead all commercialization activities for AZSTARYS under the AZSTARYS License Agreement. We cannot guarantee that the AZSTARYS License Agreement will be successful or that we will receive any future payments under the AZSTARYS License Agreement. For instance, Commave has the option to terminate the AZSTARYS License Agreement, in its entirety or on a product-by-product and country-by-country basis, at their convenience either (i) prior to the first regulatory approval of a product upon sixty days prior written notice or (ii) subsequent to the first regulatory approval of a product upon one hundred twenty days prior written notice. Further, even if Commave does not terminate the AZSTARYS License Agreement, we cannot guarantee that we will receive any additional milestone or royalty payments under the AZSTARYS License Agreement. In addition, under the AZSTARYS License Agreement, we have limited control over the amount and timing of resources that Corium will dedicate to the development, manufacturing or commercialization of AZSTARYS, and we may not always agree with Corium's efforts. Our ability to generate revenue under the AZSTARYS License Agreement will depend, in part, on Corium's ability to successfully perform the functions assigned to it under the AZSTARYS License Agreement.

In addition, we have entered into the APADAZ License Agreement with KVK pursuant to which we granted an exclusive license to KVK to commercialize APADAZ in the United States. We cannot guarantee that our collaboration with KVK will be successful or that we will ever receive any payments under the APADAZ License Agreement. For instance, if the Initial Adoption Milestone under the APADAZ License Agreement is not achieved, KVK may terminate the APADAZ License Agreement without making any payments to us. Further, even if the Initial Adoption Milestone under the APADAZ License Agreement is achieved, we cannot guarantee that we will receive any additional milestone or royalty payments under the APADAZ License Agreement. Further, under the APADAZ License Agreement, we have limited control over the amount and timing of resources that KVK will dedicate to the commercialization of APADAZ, and we may not always agree with KVK's commercialization efforts. Our ability to generate revenue under the APADAZ License Agreement will depend on KVK's ability to successfully perform the functions assigned to it under the APADAZ License Agreement. The commercialization strategy under the APADAZ License Agreement is novel and untested, and, even if successful we expect that the pricing for any sales of APADAZ will be at or near the prices of currently available generic equivalent drugs. As a result, even if KVK does successfully perform its functions under the APADAZ License Agreement, we cannot guarantee that there will be sufficient market demand for APADAZ for us to receive any revenue under the APADAZ License Agreement.

We may also seek additional third-party collaborators for the commercialization of APADAZ or for the development or commercialization of any of our other product candidates, which are not subject to the AZSTARYS License Agreement, or those that are subject to the AZSTARYS License Agreement, but the option is not exercised by Commave. In such cases, our likely collaborators would include large and mid-size pharmaceutical companies, regional, national and international pharmaceutical companies and biotechnology companies. If we do enter into any such collaboration arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our approved products or any of our other product candidates. Our ability to generate revenue from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements.

Our collaborations with Commave and KVK, or combined, the Collaborators, pose the following risks to us:

- the Collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- the Collaborators may not perform their obligations as expected;
- the Collaborators may not pursue commercialization of AZSTARYS, any of our product candidates covered under the AZSTARYS License Agreement, if approved, or APADAZ, or may elect not to continue or renew commercialization programs based on post-approval clinical trial results, changes in a Collaborator's strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;
- the Collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with AZSTARYS, any of our other products covered under the AZSTARYS License Agreement, or APADAZ, as applicable, if the Collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- AZSTARYS, any of our other products covered under the AZSTARYS License Agreement, if approved, and APADAZ may be viewed by the Collaborators as competitive with their own product candidates or products, which may cause the Collaborators to cease to devote resources to the commercialization of AZSTARYS, any of our other products covered under the AZSTARYS License Agreement, if approved, or APADAZ;
- the Collaborators may not commit sufficient resources to the development, marketing and distribution of AZSTARYS, any of our other products covered under the AZSTARYS License Agreement, and APADAZ, as applicable;
- disagreements with the Collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development or commercialization, might cause delays or termination of the development or commercialization of AZSTARYS, or any of our other products covered under the AZSTARYS License Agreement, or APADAZ, as applicable, might lead to additional responsibilities for us with respect to AZSTARYS, any of our other products covered under the AZSTARYS License Agreement, or APADAZ, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- the Collaborators may not properly maintain or defend our or their intellectual property rights or may use our or their proprietary information in such a way as to invite litigation that could jeopardize or invalidate such intellectual property or proprietary information or expose us to potential litigation;
- the Collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- the license agreements may be terminated by the Collaborators under specified circumstances and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of AZSTARYS, any of our other products covered under the AZSTARYS License Agreement, or APADAZ.

If we enter into any future collaborations we will face similar risks with any future collaborators as well.

The AZSTARYS License Agreement, the APADAZ License Agreement, and any other licensing or collaboration agreements we may enter into may not lead to commercialization of AZSTARYS, commercialization of APADAZ, or development of KP1077, KP879 or any of our other product candidates in the most efficient manner or at all. If Corium or KVK or a future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our drug development or commercialization program could be delayed, diminished or terminated.

If we are not able to establish collaborations for our product candidates, we may have to alter our development and commercialization plans.

Our prodrug development programs and the potential commercialization of our product candidates, if approved, will require substantial additional capital. For our product candidates, which are not subject to the terms of the AZSTARYS License Agreement or the APADAZ License Agreement, we may need to collaborate with pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally.

The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate.

Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of product candidates, reduce or delay one or more of our development programs, delay potential commercialization of our product candidates or reduce the scope of any sales or marketing activities of our product candidates, or increase our expenditures and undertake development or commercialization activities at our own expense of our product candidate. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring our product candidates to market and generate product revenue.

Provisions in our agreements with Aquestive and Commave may inhibit our ability to enter into future collaborations with third parties.

We are party to a termination agreement with Aquestive Therapeutics, or Aquestive, that may limit the value of any sale, license or commercialization of AZSTARYS, KP879 or KP1077. Under this termination agreement, Aquestive has the right to receive a royalty amount equal to 10% of any value generated by AZSTARYS, KP879 or KP1077, and any product candidates which contain SDX, including royalty payments on any license of AZSTARYS, KP879 or KP1077, the sale of AZSTARYS, KP879 or KP1077 to a third party or the commercialization of AZSTARYS, KP879 or KP1077. As part of the AZSTARYS License Agreement, we paid Aquestive a royalty equal to 10% of the regulatory milestone and royalty payments we received in 2021 from AZSTARYS.

We also granted to Commave a right of first refusal to acquire, license or commercialize any additional product candidate which contains SDX and is intended to treat ADHD or any other CNS disorder with such right of first refusal expiring upon the acceptance of a new drug application for such product candidate. We also granted Commave a right of first negotiation and a right of first refusal, subject to specified exceptions, for any assignment of our rights under the AZSTARYS License Agreement. We cannot predict if these obligations will limit the value we may receive from any future sale or license of any additional product candidate.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain trade secret protection or patent protection for our technology, our approved products or our product candidates, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize technology and drugs similar or identical to ours, and our ability to successfully commercialize our technology, our approved products, or our product candidates, if approved, may be impaired.

Our success depends in large part on our ability to obtain and maintain trade secret protection of our proprietary LAT platform technology as well as patent protection in the United States and other countries with respect to our approved products, and any of our product candidates. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our product technology and product candidates. As part of the AZSTARYS License Agreement, Commave obtained from us an exclusive, worldwide license to certain patents that cover AZSTARYS. In addition, as part of the APADAZ License Agreement, KVK obtained from us an exclusive license to certain patents that cover APADAZ.

The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. We may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the rights to patents, licensed to third parties by us.

Further, we may also not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the rights to patents, licensed from third parties to us. Therefore, any such patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business. If such licensors or licensees fail to maintain such patents, or lose rights to those patents, the rights we have in- or out-licensed may be reduced or eliminated.

The patent position of biotechnology and pharmaceutical companies, generally, is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States or visa-versa. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and utility, or equivalent, patent applications in the United States and other jurisdictions are typically, for example, not published until 18 months after the filing date of such patent applications, or in some cases not at all. Therefore, we cannot know with certainty whether we were the first to make and/or use the inventions claimed in our owned or licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions. As a result, the issuance, scope, priority, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued that protect our product candidates, in whole or in part, or which effectively prevent others from commercializing competitive technologies and drugs. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

Our patent position is subject to numerous additional risks, including the following:

- we may fail to seek patent protection for inventions that are important to our success;
- our pending patent applications may not result in issued patents;
- we cannot be certain that we are the first to invent the inventions covered by pending patent applications or that we are the first to file such applications and, if we are not, we may be subject to priority disputes or lose rights;
- we may be required to disclaim part or all of the term of certain patents or all of the term of certain patent applications;
- we may not be able to acquire patent term extensions or supplemental certificates of certain patents, domestic or foreign, due to regulatory delays, among others, which may affect the term of enforceability of such patents over time;
- we may file patent applications but have claims restricted or we may not be able to supply sufficient data to support our claims and, as a result, may not obtain the original claims desired or we may receive restricted claims; alternatively, it is possible that we may not receive any patent protection from an application;
- even if our owned and licensed patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, and may not be of sufficient scope or strength to provide us with any commercial advantage;
- our competitors may be able to design around our owned or licensed patents by developing similar or alternative technologies or drugs without infringing on our intellectual property rights;
- we could inadvertently abandon a patent or patent application, resulting in the loss of protection of intellectual property rights in a particular country, and we, our collaborators or our patent counsel may take action resulting in a patent or patent application becoming abandoned which may not be able to be reinstated or if reinstated, may suffer patent term adjustments or loss;
- the claims of our issued patents or patent applications when issued may not cover our product candidates;
- no assurance can be given that our patents would be declared by a court or tribunal, domestic or foreign, to be valid or enforceable or that a competitor's technology or product would be found by a court or tribunal, domestic or foreign, to infringe our patents and our patents or patent applications may be challenged by third parties in patent litigation, domestic or foreign, or in proceedings before the United States Patent and Trademark Office, or the USPTO, or its foreign counterparts, and may ultimately be declared invalid or unenforceable or narrowed in scope;
- there may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim and there may be prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim;
- third parties may develop products that have the same or similar effect as our products without infringing our patents;
- third parties may intentionally circumvent our patents by means of alternate designs or processes or file applications or be granted patents that would block or hurt our efforts;
- there may be dominating or intervening patents relevant to our product candidates of which we are not aware;
- obtaining regulatory approval for pharmaceutical products is a lengthy and complex process, and as a result, any patents covering our product candidates may expire before or shortly after such product candidates are approved and commercialized;
- the patent and patent enforcement laws of some foreign jurisdictions do not protect intellectual property rights to the same extent as laws in the United States, and many companies have encountered significant difficulties in protecting and defending such rights in foreign jurisdictions; and
- we may not develop additional proprietary technologies that are patentable.

Any of these factors could hurt our ability to gain full patent protection for our products. Registered trademarks and trademark applications in the United States and other countries are subject to similar risks as described above for patents and patent applications, in addition to the risks described below.

Further, a third party may misappropriate or reverse engineer our proprietary LAT platform technology, which could limit our ability to stop others from using or commercializing similar or identical technology and resultant product candidates, product technology or prodrugs, or limit the duration of the trade secret protection of our proprietary LAT platform technology.

Moreover, we may be subject to a third-party pre-issuance submission of prior art to the USPTO, or become involved in opposition, litigation, nullity, derivation, reexamination, inter partes review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or drugs and compete directly with us, without payment to us or result in our inability to manufacture or commercialize drugs without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to seek patent protection or to license, develop or commercialize current or future product candidates.

In addition, the issuance of a patent is not conclusive as to its inventorship, ownership, scope, priority, validity or enforceability, and our owned and licensed patents may be challenged in the courts, patent offices and tribunals in the United States and abroad. Such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and drugs, or limit the duration of the patent protection of our product technology, product candidates and prodrugs.

Changes in patent law in the United States and other jurisdictions could alter or diminish the value of patents in general, thereby impairing our ability to protect our products and technologies.

The standards that the USPTO and patent offices in other countries use to grant patents are not always applied predictably or uniformly and can change. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries could increase the uncertainties and costs, and may diminish our ability to protect our inventions, obtain, maintain, and enforce our intellectual property rights and, more generally, could affect the value of our intellectual property or narrow the scope of our owned and licensed patents.

In addition, the patent positions of companies in the development and commercialization of pharmaceuticals are particularly uncertain. The United States Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. Depending on future actions by the United States Congress, the United States courts, the USPTO and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. For example, in the 2013 case *Assoc. for Molecular Pathology v. Myriad Genetics, Inc.*, the United States Supreme Court held that certain claims to DNA molecules are not patentable. While we do not believe that any of the patents owned or licensed by us will be found invalid based on this decision, future decisions by the courts, the United States Congress or the USPTO may impact the value of our patents.

For another example, on September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted in the United States, redefine prior art and may also affect patent litigation. The USPTO recently developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first-to-file provisions, only became effective on March 16, 2013.

The Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. For instance, the Leahy-Smith Act established the *inter partes* review and post grant review procedures that has lowered the burden of proof for invalidity challenges to issued patents and limited the ability to amend patent claims in response to such challenges. In addition, patent reform legislation may pass in the future that could lead to additional uncertainties and increased costs surrounding the prosecution, enforcement and defense of our owned and licensed patents and/or patent applications.

We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time consuming and unsuccessful.

Competitors or other third parties may infringe our patents, trademarks, copyrights or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming and divert the time and attention of our management and scientific personnel. Our pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents, in addition to counterclaims asserting that our patents are invalid or unenforceable, or both. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, non-enablement or insufficient written description. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO or made a misleading statement during prosecution. The outcome following legal assertions of invalidity and unenforceability is unpredictable. In any patent infringement proceeding, there is a risk that a court will decide that a patent of ours is invalid or unenforceable, in whole or in part, and that we do not have the right to stop the other party from using the invention at issue. There is also a risk that, even if the validity of such patents is upheld, the court will construe the patent's claims narrowly or decide that we do not have the right to stop the other party from using the invention at issue on the grounds that our patent claims do not cover the invention, or decide that the other party's use of our patented technology falls under the safe harbor to patent infringement under 35 U.S.C. §271(e)(1). An adverse outcome in a litigation or proceeding involving our patents could limit our ability to assert our patents against those parties or other competitors and may curtail or preclude our ability to exclude third parties from making and selling similar or competitive products and technologies. Any of these occurrences could adversely affect our competitive business position, business prospects and financial condition. Similarly, if we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could significantly harm our business.

Our commercial success depends upon our ability, and the ability of any collaborators, to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing the proprietary rights of third parties. There is considerable intellectual property litigation in the biotechnology and pharmaceutical industries. In particular, we are focused on developing product candidates based on widely used therapeutic agents or drugs, many of which may be protected by proprietary rights of third parties.

Although we seek to develop proprietary prodrug formulations that do not infringe the intellectual property rights of others, we may become party to, or threatened with, future adversarial proceedings or litigation, domestic or foreign, regarding intellectual property rights with respect to our prodrugs or other aspects of our technology, including, for example, interference or derivation proceedings before the USPTO. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future.

If we are found to infringe a third party's intellectual property rights, we could be required to obtain a license from such third party to continue developing and marketing our technology and drugs. However, we may not be able to obtain any required license on commercially reasonable terms, or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. We could be forced, including by court order, to cease commercializing the infringing technology or product. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some or all of our business operations.

Competing products may also be sold in other countries in which our patent coverage might not exist or be as strong. If we lose a foreign patent lawsuit alleging our infringement of a competitor's patent, we could be prevented from marketing our products in one or more foreign countries. As a result, our ability to grow our business and compete in the market may be harmed.

Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities.

In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could hurt the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could compromise our ability to compete in the marketplace.

We may need to license intellectual property from third parties, and such licenses may not be available or may not be available on commercially reasonable terms.

A third party may hold intellectual property rights, including patent rights, which are important or necessary to the development of our product candidates. It may be necessary for us to use the patented or proprietary technology of third parties to commercialize our product candidates, in which case we would be required to obtain a license from these third parties. Such a license may not be available on commercially reasonable terms, or at all, and we could be forced to accept unfavorable contractual terms. If we are unable to obtain such licenses on commercially reasonable terms, our business could be harmed.

If we or our third-party licensors fail to comply with our obligations in our intellectual property licenses and funding arrangements with third parties, we could lose rights that are important to our business.

We are currently party to license agreements for technologies that we anticipate using in our product development activities. In the future, we may become party to licenses that are important for product development and commercialization. If we or our third-party licensors fail to comply with the obligations under current or future license and funding agreements, our counterparties may have the right to terminate these agreements, we may be forced to terminate these agreement or we may no longer effectively rely on any licenses to us under these agreements, in which event we might not be able to develop, manufacture or market any product or utilize any technology that is covered by these agreements or may face other penalties under the agreements. Such an occurrence could materially and adversely affect the value of a product candidate being developed under any such agreement or could restrict our drug discovery activities. Termination of these agreements or reduction or elimination of our rights under these agreements may result in our having to negotiate new or reinstated agreements with less favorable terms or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology.

The scope of our intellectual property may be reduced or may need to be reduced due to third-party intellectual property claims.

Our competitors may have filed, and may in the future file, patent applications covering technology similar to ours. Any such patent application may have priority over our patent applications, which could further require us to obtain rights to issued patents covering such technologies. If another party has filed a U.S. patent application on inventions similar to ours that claims priority to an application filed prior to March 16, 2013, we may have to participate in an interference proceeding declared by the USPTO to determine priority of invention in the United States. The costs of these proceedings could be substantial, and it is possible that such efforts would be unsuccessful if, unbeknownst to us, the other party had independently arrived at the same or similar invention prior to our own invention, resulting in a loss of our U.S. patent position with respect to such inventions. In addition, changes enacted on March 16, 2013, to the U.S. patent laws under the Leahy-Smith Act resulted in the United States changing from a “first to invent” country to a “first to file” country. As a result, we may lose the ability to obtain a patent if another party files with the USPTO first and could become involved in proceedings before the USPTO to resolve disputes related to inventorship. We may also become involved in similar proceedings in other jurisdictions.

Furthermore, recent changes in U.S. patent law under the Leahy-Smith Act allow for post-issuance challenges to U.S. patents, including ex parte re-examinations, *inter partes* reviews and post-grant reviews. There is significant uncertainty as to how the new laws will be applied. If our U.S. patents are challenged using such procedures, we may not prevail, possibly resulting in altered or diminished claim scope or loss of patent rights altogether. Similarly, some countries, notably Europe, also have post-grant opposition proceedings or nullity proceedings that can result in changes in scope or cancellation of patent claims.

We may be subject to claims by third parties asserting that we or our employees have misappropriated their intellectual property or claiming ownership of what we regard as our own intellectual property.

Many of our employees were previously employed at other biotechnology or pharmaceutical companies. Although we try to ensure that our employees do not use the proprietary information, show-how or know-how of others in their work for us, we may be subject to claims that these employees or we have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of any such employee's former employer. For example, in March 2012, we settled litigation regarding similar matters with Shire plc. We may also in the future be subject to claims that we have caused an employee to breach the terms of his or her non-competition or non-solicitation agreement. Litigation may be necessary to defend against these potential claims.

In addition, while it is our policy to require our employees and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own. Our and their assignment agreements may not be self-executing or may be breached, and we may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property.

If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. A court could prohibit us from using technologies or features that are essential to our products, if such technologies or features are found to incorporate or be derived from the trade secrets or other proprietary information of the former employers. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and could be a distraction to management. In addition, any litigation or threat thereof may adversely affect our ability to hire employees or contract with independent service providers. Moreover, a loss of key personnel or their work product could hamper or prevent our ability to commercialize our products.

Any trademarks we may obtain may be infringed or successfully challenged, resulting in harm to our business.

We expect to rely on trademarks as one means to distinguish our product candidates that are approved for marketing from the products of our competitors. We have registered trademarks for LAT andZevra. In addition, we have solicited and applied for trademarks for the Zevra logo and several potential trade names and logos for future product candidates. Third parties may oppose or attempt to cancel our trademark applications or trademarks, or otherwise challenge our use of the trademarks. If our trademarks are successfully challenged, we could be forced to rebrand our products, which could result in loss of brand recognition and could require us to devote resources to advertising and marketing new brands. Our competitors may infringe our trademarks and we may not have adequate resources to enforce our trademarks.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patent and trademark protection for our product candidates, we also rely on trade secrets, including unpatented show-how, know-how, technology and other proprietary information, to maintain our competitive position. We seek to protect our trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets. Further, we may need to share our trade secrets and confidential know-how with future business partners, collaborators, contractors and others located in countries at heightened risk of theft of trade secrets, including through direct intrusion by private parties or actors in other countries, and those affiliated with or controlled by state actors.

Monitoring unauthorized uses and disclosures of our intellectual property, including our trade secrets, is difficult, and we do not know whether the steps we have taken to protect our intellectual property will be effective. In addition, we may not be able to obtain adequate remedies for any such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets.

Moreover, our competitors may independently develop or reverse engineer knowledge, methods, show-how and know-how equivalent to our trade secrets. Competitors could purchase our products and replicate some or all of the competitive advantages we derive from our development efforts for technologies on which we do not have patent protection. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate such trade secrets, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

We may not be able to protect our intellectual property rights throughout the world.

Patents are of national or regional effect, and filing, prosecuting and defending patents on all of our products and technologies throughout the world would be prohibitively expensive. As such, our intellectual property rights in some countries outside the United States can be less extensive than those in the United States and we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products or technology and may export otherwise infringing products or technology to territories where we have patent protection, but enforcement rights are not as strong as those in the United States. These products may compete with our products and technologies, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing. Further, the legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection, particularly those relating to pharmaceuticals or biologics, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in other jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any such lawsuits that we initiate and the damages and other remedies awarded, if any, may not be commercially meaningful. Similarly, if our trade secrets are disclosed in another jurisdiction, competitors worldwide could have access to our proprietary information and we may be without satisfactory recourse. Such disclosure could have a material adverse effect on our business. Moreover, our ability to protect and enforce our intellectual property rights may be adversely affected by unforeseen changes in intellectual property laws of other countries. In addition, certain developing countries, including China and India, have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In those countries, we and our licensors may have limited remedies if patents are infringed or if we or our licensors are compelled to grant a license to a third party, which could materially diminish the value of those patents. In addition, many countries limit the enforceability of patents against government agencies or government contractors. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

In 2012, the European Patent Package, or EU Patent Package, regulations were passed with the goal of providing a single pan-European Unitary Patent and a new European Unified Patent Court, or UPC, for litigation involving European patents. Implementation of the EU Patent Package will likely occur in the first half of 2023. Under the UPC, all European patents, including those issued prior to ratification of the European Patent Package, will by default automatically fall under the jurisdiction of the UPC. The UPC will provide our competitors with a new forum to centrally revoke our European patents, and allow for the possibility of a competitor to obtain pan-European injunctions. It will be several years before we will understand the scope of patent rights that will be recognized and the strength of patent remedies that will be provided by the UPC. Under the EU Patent Package as currently proposed, we will have the right to opt our patents out of the UPC over the first seven years of the court's existence, but doing so may preclude us from realizing the benefits of the new unified court.

Our decision to seek approval of our product candidates under the 505(b)(2) NDA pathway, if available, may increase the risk that patent infringement suits are filed against us, which would delay the FDA's approval of such product candidates.

Regarding any NDA that we may submit under the 505(b)(2) NDA pathway, if there are patents that claim the approved drug contained in our product candidates and referenced in our 505(b)(2) NDA, we must certify to the FDA and notify the patent holder that any patents listed for the approved drug in the FDA's Orange Book publication are invalid, unenforceable or will not be infringed by the manufacture, use or sale of our prodrug. If a patent infringement lawsuit is filed against us within 45 days of its receipt of notice of our certification, the FDA is automatically prevented from approving our 505(b)(2) NDA until the earliest of 30 months, expiration of the patent, settlement of the lawsuit or a court decision in the infringement case that is favorable to us, or such shorter or longer period as may be ordered by a court. Such actions are routinely filed by patent owners. Accordingly, we may invest considerable time and expense in the development of our product candidates only to be subject to significant delay and patent litigation before our product candidates may be commercialized. We may not be successful in defending any patent infringement claim. Even if we are found not to infringe, or a plaintiff's patent claims are found invalid or unenforceable, defending any such infringement claim would be expensive and time-consuming, and would delay launch of our product candidates and distract management from their normal responsibilities.

Risks Related to the Commercialization of Our Partnered Products and Product Candidates

If we are unable to establish sales, marketing and distribution capabilities for our product candidates, if approved, we may not be successful in commercializing any approved product candidate in the United States.

We have only a limited sales and marketing infrastructure and have no experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any product candidate for which we may obtain marketing approval in the United States, we will need to enter into collaborations with one or more parties or establish our own sales and marketing organization. While we entered into the AZSTARYS License Agreement to establish a collaboration for the commercialization of AZSTARYS and any of our product candidates which are subject to such agreement, and we entered into the APADAZ License Agreement to establish a collaboration for the commercialization of APADAZ, we may not choose to enter into a collaboration for any future approved product. Should we decide to establish our own sales, marketing and distribution capabilities, we would encounter a number of risks. For example, recruiting and training a sales force is expensive and time consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our product candidates on our own include:

- our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- our inability to access government and commercial health plan formularies or secure preferred coverage and adequate reimbursement levels;
- the inability of sales personnel to obtain access to physicians or achieve adequate numbers of physicians to prescribe any future prodrug products;
- the lack of complementary drugs to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines;
- liability for personnel, including sales personnel, failing to comply with applicable legal requirements; and
- costs associated with maintaining compliance with the FDA's marketing and promotional requirements, including ongoing training and monitoring, as well as unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we decide not to or are unable to establish our own sales, marketing and distribution capabilities and, instead, enter into arrangements with third parties to perform these services, our product revenue and our profitability, if any, are likely to be lower than if we were to sell, market and distribute any product candidates that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell, market and distribute our product candidates in the future, or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, including Corium and KVK, and any of them may fail to devote the necessary resources and attention to sell and market our approved products, or any of our product candidates, if approved, effectively. Further, we may be liable for conduct of third parties, including Corium and KVK, acting on our behalf, including failure to comply with legal requirements applicable to sales and marketing of our product or product candidates, if approved. If we do not establish sales, marketing and distribution capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our approved products, or any of our product candidates, if approved.

Our approved products, or any of our product candidates that may receive marketing approval, may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.

Our approved products, or any of our product candidates that may receive marketing approval, may fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. If our approved products, or any of our product candidates, if approved for commercial sale, do not achieve an adequate level of market acceptance, they may not generate significant product revenue and we may not become profitable. For instance, under the APADAZ License Agreement, we are entitled to milestone and royalty payments only if APADAZ sales in the United States are above specified levels. If APADAZ does not achieve an adequate level of market acceptance, it is unlikely that sales will satisfy these thresholds and we may not be entitled to any payments under the APADAZ License Agreement. Additionally, the commercialization strategy under the APADAZ License Agreement is novel and untested, and, even if successful we expect that the pricing for any sales of APADAZ will be at or near the prices of currently available generic equivalent drugs. Accordingly, we expect that APADAZ will need to achieve broad market acceptance in order for this strategy to be successful. The degree of market acceptance of our approved products, or any of our product candidates if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and potential advantages compared to alternative treatments, including less expensive generic treatments;
- the ability to obtain differentiating claims in the labels for most of our product candidates;
- our ability to offer our prodrug products for sale at competitive prices;
- the clinical indications for which our product candidates are approved;
- the convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the cost of treatment in relation to alternative treatments;
- the steps that prescribers and dispensers must take, since our approved products are, and we expect that most of our product candidates are likely going to be considered controlled substances, as well as the perceived risks based upon their controlled substance status;
- the ability to manufacture our product in sufficient quantities and yields;
- the strength of marketing and distribution support;
- the availability of third-party coverage and adequate reimbursement or willingness of patients to pay out of pocket in the absence of third-party coverage;
- the prevalence and severity of any side effects;
- any potential unfavorable publicity;
- any restrictions on the use, sale or distribution of our approved products or any of our product candidates, including through REMS; and
- any restrictions on the use of our prodrug products together with other medications.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.

Our industry is characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. We will face competition and potential competition from a number of sources, including pharmaceutical and biotechnology companies, specialty pharmaceutical companies, generic drug companies, drug delivery companies and academic and research institutions. Our competitors may develop or market drugs that are more effective, more convenient, more widely used and less costly or have a better safety profile than our products or product candidates and these competitors may also have significantly more resources than us and be more successful than us in manufacturing and marketing their products.

Currently, there are no approved drugs in the United States for the treatment of NPC. We consider our most direct competitor with respect to arimoclomol to be Zavesca (miglustat), which was originally developed by Actelion Pharmaceuticals and is now owned by Johnson & Johnson. Miglustat is currently approved for the treatment of NPC in Europe, Canada, Australia, New Zealand, and several countries in Asia and South America as Zavesca and in Japan as Brazaves.

If approved, we intend for KP1077 to compete against Jazz Pharmaceuticals' XYWAV, and potentially with other products that are currently in development for the treatment of IH, including Harmony Biosciences' WAKIX. KP1077 could face potential competition from any products for the treatment of IH that are currently in or which may enter into clinical development.

Currently, there are no approved drugs in the United States for the treatment of SUD. If approved, KP879 will face potential competition from any products fo the treatment of SUD that are currently in, or which may enter into, clinical development.

AZSTARYS currently competes against currently marketed, branded and generic methylphenidate products for the treatment of ADHD. Some of these currently marketed products include Janssen's CONCERTA, Supernus' QUELBREE, Tris Pharma's QUILLIVANT XR and QUILLICHEW ER, Novartis' RITALIN, FOCALIN and FOCALIN XR, UCB's METADATE CD, Noven's DAYTRANA, Neos Therapeutics' CONTEMPLA XR-ODT, Ironshore Pharmaceuticals, Inc.'s JORNAY PM and Adlon Therapeutics' ADHANSIA XR, in addition to multiple other branded and generic methylphenidate products. In addition, AZSTARYS will face potential competition from any other methylphenidate products for the treatment of ADHD that are currently in or which may enter into clinical development.

APADAZ competes against currently marketed, branded and generic hydrocodone/APAP products for short-term (no more than 14 days) management of acute pain severe enough to require an opioid analgesic and for which alternative treatments are inadequate. In addition, APADAZ may face potential competition from any other hydrocodone/APAP products for short-term (no more than 14 days) management of acute pain severe enough to require an opioid analgesic and for which alternative treatments are inadequate that are currently in or which may enter into clinical development.

Many of our potential competitors have substantially greater financial, technical and human resources than we do, as well as more experience in the development of product candidates, obtaining FDA and other regulatory approvals of products and the commercialization of those products. Consequently, our competitors may develop products for indications we are pursuing or may pursue in the future, and such competitors' products may be more effective, better tolerated and less costly than our product candidates. Our competitors may also be more successful in manufacturing and marketing their products than we are. We will also face competition in recruiting and retaining qualified personnel and establishing clinical trial sites and patient enrollment in clinical trials.

Our competitors also may obtain FDA or other regulatory approval for their product candidates more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. If the competitor's product were similar to our product candidates, we may be required to seek approval via alternative pathways, such as the ANDA, which is used for the development of generic drug products. We may also be blocked from product marketing by periods of patent protection or regulatory exclusivity.

In addition, our ability to compete may be affected in many cases by insurers or other third-party payors seeking to encourage the use of generic drugs or giving drugs with improved attributes sufficient weight in a comparative clinical cost effectiveness analysis. For some of the indications that we are pursuing, drugs used off-label serve as cheaper alternatives to our product candidates. Their lower prices could result in significant pricing pressure, even if our product candidates are otherwise viewed as a preferable therapy. Additional drugs may become available on a generic basis over the coming years.

Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

Consequently, our competitors may develop products for the treatment of ADHD, pain or for other indications we may pursue in the future, and such competitors' products may be more effective, better tolerated and less costly than our product candidates. Our competitors may also be more successful in manufacturing and marketing their products than we are. We will also face competition in recruiting and retaining qualified personnel and establishing clinical trial sites and subject enrollment in clinical trials.

We may not be able to obtain either five-year FDA regulatory exclusivity as a new chemical entity or three-year FDA regulatory exclusivity.

The FDA provides periods of regulatory exclusivity following their approval of an NDA, which provide the holder of an approved NDA limited protection from new competition in the marketplace for the innovation represented by its approved drug. Five-year exclusivity precludes approval of 505(b)(2) applications or ANDAs by delaying the submission or approval of the application, while three-year exclusivity precludes the approval of the application. We intend to seek new chemical entity, or NCE, status for any of our prodrug product candidates as appropriate. Five years of exclusivity are available to NCEs following the approval of an NDA by the FDA. An NCE is a drug that contains no active moiety that has been approved by the FDA in any other NDA. If a product is not eligible for the NCE exclusivity, it may be eligible for three years of exclusivity. Three-year exclusivity is available to the holder of an NDA, including a 505(b)(2) NDA, for a particular condition of approval, or change to a marketed product, such as a new formulation for a previously approved product, if one or more new clinical trials, other than bioavailability or bioequivalence trials, were essential to the approval of the application and were conducted or sponsored by the applicant.

There is a risk that the FDA may disagree with any claim that we may make that any of our prodrug product candidates are NCEs and therefore entitled to five-year exclusivity. The FDA may also take the view that the studies that we are conducting are not clinical trials, other than bioavailability and bioequivalence studies, that are essential to approval and therefore do not support three-year exclusivity. Further, to the extent that the basis for exclusivity is not clear, the FDA may determine to defer a decision until it receives an application which necessitates a decision.

If we do obtain either five or three years of exclusivity, such exclusivity will not block all potential competitors from the market. Competitors may be able to obtain approval for similar products with different forms of competitive differentiating mechanisms or may be able to obtain approval for similar products without a competitive differentiating mechanism.

Even if we or our collaborators are able to commercialize our approved products, or any of our product candidates, if approved, they may be subject to unfavorable pricing regulations, third-party coverage and reimbursement policies.

The successful commercialization of our approved products, and any of our product candidates for which marketing approval is obtained will depend, in part, on the extent to which coverage and adequate reimbursement for AZSTARYS, APADAZ, or any of our product candidates for which marketing approval is obtained, will be available from government payor programs at the federal and state levels, including Medicare and Medicaid, private health insurers and managed care plans and other third-party payors. Government authorities and other third-party payors decide which medical products they will pay for and establish reimbursement levels, including co-payments. A trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medical products. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for drugs and products. Coverage and reimbursement may not be available for any product that we commercialize and, even if these are available, the level of reimbursement may not be satisfactory. Inadequate reimbursement levels may adversely affect the demand for, or the price of, our approved products, or any of our product candidates for which we obtain marketing approval. Obtaining and maintaining adequate reimbursement for our prodrug products may be difficult. We may be required to conduct expensive pharmacoeconomic studies to justify coverage and reimbursement or the level of reimbursement relative to other therapies. Moreover, the trend has been for government and commercial health plans and their pharmacy benefit managers to commoditize drug products through therapeutic equivalence determinations, making formulary decisions based on cost. If coverage and adequate reimbursement are not available or reimbursement is available only at limited levels, we may not be able to successfully commercialize our approved products, or commercialize any of our product candidates for which marketing approval is obtained.

There may be significant delays in obtaining coverage and reimbursement for newly approved prodrug products, and coverage may be more limited than the indications for which the product is approved by the FDA or similar regulatory authorities outside the United States. Moreover, eligibility for coverage and reimbursement does not imply that a product will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution expenses. Interim reimbursement levels for new prodrug products, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the product and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for prodrug products may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Private third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. Except for certain government health care programs, such as the Department of Defense's TRICARE Uniform Formulary, no uniform policy requirement for coverage and reimbursement for drug products exists among third-party payors in the United States. Even state Medicaid programs have their own preferred drug lists that may disadvantage non-preferred brand drugs. Therefore, coverage and reimbursement can differ significantly from payor to payor. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained at all. Our inability to promptly obtain coverage and adequate reimbursement rates from both government-funded and private payors for any approved prodrug products that we develop could significantly harm our operating results, our ability to raise capital needed to commercialize prodrugs and our overall financial condition.

The regulations that govern marketing approvals, pricing, coverage and reimbursement for new drugs vary widely from country to country. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a product before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenue able to be generated from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

There can be no assurance that our approved products, or any of our product candidates, if they are approved for sale in the United States or in other countries, will be considered medically reasonable and necessary for a specific indication, that they will be considered cost-effective by third-party payors, that coverage or an adequate level of reimbursement will be available, or that third-party payors' reimbursement policies will not adversely affect the ability to sell our approved products, or our ability to sell any of any of our product candidates profitably if they are approved for sale.

We may be subject to enforcement action if we engage in improper marketing or promotion of our products.

The FDA closely regulates promotional materials and other promotional activities. Even if the FDA initially approves product labeling that includes a description of our improved attribute claims, the FDA may object to our marketing claims and product advertising campaigns. Failure to comply with the FDA's promotional, marketing and advertising laws and regulations could lead to the issuance of warning letters, cyber letters, or untitled letters, adverse publicity, the requirement for dear-health-care-provider letters or other corrective information, fines and other monetary penalties, civil or criminal prosecution, including False Claims Act liability, restrictions on our operations and other operating requirements through consent decrees or corporate integrity agreements, debarment, exclusion from participation in federal health care programs and refusal of government contracts or future orders under existing contracts, among other consequences. Any of these consequences would harm the commercial success of our products.

Further, our promotional materials, statements and training methods must comply with the FDA's prohibition of the promotion of unapproved, or off-label, use. Any regulatory approval that the FDA grants is limited to those specific diseases and indications for which a product is deemed to be safe and effective by FDA. For example, the FDA-approved label for AZSTARYS is limited to the acute treatment of ADHD in patients 6 years of age and older, and the FDA-approved label for APADAZ is limited to the short-term (no more than 14 days) management of acute pain severe enough to require an opioid analgesic and for which alternative treatments are inadequate. Physicians may use our products off-label, as the FDA does not restrict or regulate a physician's independent choice of treatment within the practice of medicine. However, if the FDA determines that our promotional materials, statements or training constitutes promotion of an off-label use, it could request that we modify our promotional materials, statements or training methods or subject us to regulatory or enforcement actions, such as the issuance of an untitled letter, a warning letter, injunction, seizure, civil fine, disgorgement of money, operating restrictions or criminal penalties. We may also be subject to actions by other governmental entities or private parties, such as the False Claims Act, civil whistleblower or "qui tam" actions. It is also possible that other federal, state or foreign enforcement authorities might take action if they consider our promotional or training materials to constitute promotion of an off-label use, which could result in significant fines or penalties under other statutory authorities, such as laws prohibiting false claims for reimbursement. In that event, our reputation could be damaged and adoption of the products could be impaired. In addition, the off-label use of our products may increase the risk of product liability claims. Product liability claims are expensive to defend and could divert our management's attention, result in substantial damage awards against us and harm our reputation.

Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of our approved products, or any of our product candidates that we may develop.

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials and will face an even greater risk as our approved products, and any of our product candidates that may be approved in the future, are commercialized. This includes the risk that our products may be misused. For example, our approved products do, and we anticipate that any of our product candidates we may choose to develop in the future, if approved, may carry, a boxed warning regarding lethality if our oral tablets or capsules are prepared for injection and hepatotoxicity, as is commonly done by abusers of opioids. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities on behalf of ourselves. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for our approved products, and any of our product candidates that we may develop;
- injury to our reputation and significant negative media attention;
- termination of clinical trial sites or entire trial programs;
- withdrawal of clinical trial participants;
- initiation of investigations by regulators;
- significant costs to defend the related litigation;
- a diversion of management's time and our resources;
- substantial monetary awards paid to trial participants or patients;
- product recalls, withdrawals or labeling revisions and marketing or promotional restrictions;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to successfully commercialize any of our approved products, or any of our product candidates that might be approved in the future.

We currently hold $10.0 million in product liability insurance coverage in the aggregate, with a per incident limit of $10.0 million, which may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage as we expand our clinical trials or upon commencement of commercialization of any product approved in the future. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

A variety of risks associated with international operations could materially adversely affect our business.

We expect to engage in significant cross-border activities, and we will be subject to risks related to international operations, including:

- different regulatory requirements for maintaining approval of drugs in foreign countries;
- differing payor reimbursement regimes, governmental payors or patient self-pay systems and price controls;
- reduced protection for contractual and intellectual property rights in some countries;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, labor shortages, supply chain shortages, or other economic or political uncertainties or instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in North America;
- tighter restrictions on privacy and the collection and use of patient and clinical trial participant data; and
- business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters including earthquakes, typhoons, floods and fires.

Risks Related to Regulatory Approval of Our Product Candidates and Other Legal Compliance Matters

Failure to obtain marketing approval in international jurisdictions would prevent our approved products, and any of our other product candidates from being marketed abroad.

In order to market and sell our products in the European Union and any other jurisdictions, we must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. However, failure to obtain approval in one jurisdiction may impact our ability to obtain approval elsewhere. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market.

A variety of risks associated with marketing our approved products and any of our product candidates, if approved, internationally, could affect our business.

We may seek regulatory approval for our approved products and any of our product candidates, if approved, outside of the United States and, accordingly, we expect that we will be subject to additional risks related to operating in foreign countries if we obtain the necessary approvals, including:

- differing regulatory requirements in foreign countries;
- the potential for so-called parallel importing, which is what happens when a local seller, faced with high or higher local prices, opts to import goods from a foreign market with low or lower prices rather than buying them locally;
- unexpected changes in tariffs, trade barriers, price and exchange controls and other regulatory requirements;
- economic weakness, including inflation, labor shortages, supply chain shortages, or other economic or political uncertainties or instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- difficulties staffing and managing foreign operations;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- potential liability under the Foreign Corrupt Practices Act of 1977 or comparable foreign regulations;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geo-political actions, including war and terrorism, such as the current situation with Ukraine and Russia.

These and other risks associated with our international operations may compromise our ability to achieve or maintain profitability.

Our approved products are, and any of our product candidates for which we obtain marketing approval will remain subject to significant post-marketing regulatory requirements and oversight.

Our approved products are, and any of our product candidates for which we obtain marketing approval could be, subject to significant post-marketing regulatory requirements and oversight, including the submission of reports to regulatory authorities and surveillance to monitor the safety and efficacy of the product, significant limitations related to use restrictions for specified age groups, warnings, precautions or contraindications, and burdensome post-approval study or risk management requirements.

For example, we are required to conduct pediatric studies related to AZSTARYS to evaluate its safety and effectiveness for the claimed indication in pediatric patients. Under the AZSTARYS License Agreement, Corium will be responsible for these regulatory activities going forward, and we cannot guarantee they will be complied with. In addition, we are required to conduct pediatric studies related to APADAZ to evaluate its safety and effectiveness for the claimed indication in pediatric patients. Under the APADAZ License Agreement, KVK will be responsible for these regulatory activities going forward, and we cannot guarantee they will be complied with.

In addition, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export and recordkeeping for any approved drugs are subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, payment of substantial annual product and establishment fees, labeling requirements, promotional, marketing and advertising requirements, requirements related to further development, packaging, storage and distribution requirements, cGMP requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. If there are any modifications to the drug, including changes in indications, labeling, manufacturing processes or facilities, or new safety issues arise, a new or supplemental NDA, a post-implementation notification or other reporting may be required or requested depending on the change, which may require additional data or additional preclinical studies and clinical trials.

Our approved products are, and if marketing approval of any of our product candidates is granted, such product candidates may be, subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, including the requirement to implement a REMS, which could involve requirements for, among other things, a medication guide, special training for prescribers and dispensers, and patient registries. For example, in September 2018, the FDA approved the Opioid Analgesic REMS for ER/LA and IR opioids as one strategy among multiple national and state efforts to reduce the risk of abuse, misuse, addiction, overdose, and deaths due to prescription opioid analgesics. APADAZ is subject to this REMS, and we anticipate that any of our other opioid product candidates that we may choose to develop in the future, if approved by the FDA, are likely to also be subject to a REMS requirement. In addition, the FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product. APADAZ is subject to a post-marketing requirement for four deferred pediatric assessments that must be completed pursuant to the FDA's February 2018 approval letter.

Products may be approved with a label that limits their approved uses, including more limited subject populations, than we request, and regulatory authorities may require that contraindications, warnings or precautions be included in the product labeling, including a boxed warning, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate, which could limit sales of the product. For instance, the labels for both AZSTARYS and APADAZ contain black box warnings regarding the risks of abuse and dependence, and the label for APADAZ also includes warnings regarding the risks of hepatoxicity and respiratory depression, among others.

Violations of the FFDCA relating to the promotion of prescription drugs may lead to a number of actions and penalties, including warning letters, cyber letters, or untitled letters, adverse publicity, the requirement for dear-health-care-provider letters or other corrective information, fines and other monetary penalties, civil or criminal prosecution, including False Claims Act liability, restrictions on our operations and other operating requirements through consent decrees or corporate integrity agreements, debarment, exclusion from participation in federal health care programs and refusal of government contracts or future orders under existing contracts, among other consequences.

In addition, later discovery of previously unknown adverse events or other problems with our prodrug products, including those related to manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may have negative consequences, including:

- adverse inspectional findings;
- restrictions on such prodrug products, distribution, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a drug;
- additional warnings or other restrictions on the product's indicated use, label, or marketing;
- issuance of safety alerts, dear-healthcare-provider letters, press releases or other communications containing warnings regarding the product;
- requirement to establish or modify a REMS;
- requirement to conduct post-marketing studies or surveillance;
- restrictions on drug distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters;
- recall or withdrawal of the prodrug products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit and other delays;
- clinical holds, or the suspension or termination of ongoing clinical trials;
- fines, restitution or disgorgement of profits or revenue;
- suspension or withdrawal of marketing approvals or other permits or voluntary suspension of marketing;
- refusal to permit the import or export of our prodrug products;
- reputational harm;
- refusal of government contracts or future orders under existing contracts, exclusion from participation in federal health care programs, and corporate integrity agreements;
- product seizure or detention; or
- injunctions or the imposition of civil or criminal penalties, including False Claims Act liability.

Non-compliance with European Union requirements regarding safety monitoring or pharmacovigilance, and with requi rements related to the development of drugs for the pediatric population, can also result in significant financial penalties. Similarly, failure to comply with the European Union's requirements regarding the protection of personal information can also lead to significant penalties and sanctions.

The occurrence of any event or penalty described above may inhibit our ability to commercialize our products and generate revenue, and could require us to expend significant time and resources in response and could generate negative publicity. In addition, the FDA's and other regulatory authorities' policies may change, and additional government regulations may be enacted that could impair our business. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may be subject to enforcement action, and we may not achieve or sustain profitability.

Our employees, independent contractors, principal investigators, CROs, consultants, commercial collaborators, contract manufacturers, service providers and other vendors may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements.

We are exposed to the risk of misconduct by employees and independent contractors, such as principal investigators, CROs, consultants, commercial collaborators, contract manufacturers, service providers and other vendors. Such misconduct could include failures to comply with FDA regulations, to provide accurate information to the FDA, to comply with manufacturing standards that we have established or that are established by regulation, to comply with federal and state contracting and healthcare fraud and abuse laws, to report drug pricing, financial information or data accurately or to disclose unauthorized activities to us. In particular, sales, marketing and other business arrangements in the healthcare industry are subject to extensive laws intended to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws may restrict or prohibit a wide range of business activities, including, but not limited to, research, manufacturing, distribution, pricing, discounting, marketing, advertising and promotion, sales commissions, customer incentive programs and other business arrangements. Employee and independent contractor misconduct could also involve the improper use of individually identifiable information, including, without limitation, information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. In addition, federal procurement laws impose substantial penalties for misconduct in connection with government contracts and require certain contractors to maintain a code of business ethics and conduct and self-disclose credible evidence of False Claims Act violations. It is not always possible to identify and deter employee and independent contractor misconduct, and any precautions we take to detect and prevent improper activities may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws. If any such actions are instituted against us, those actions could have a significant impact on our business, including the imposition of warning letters, untitled letters, cyber letters, seizure or recall of products, injunctions, withdrawal of product approval or other permits, clinical holds and termination of clinical trials, FDA refusal to approve pending applications, product detentions, FDA or DEA consent decrees, restriction or suspension of manufacturing and distribution, debarment, refusal to allow product import or export, adverse publicity, refusal of government contracts or future orders under existing contracts, dear-health-care-provider letters or other warnings or corrective information, recalls, delays, significant civil, criminal and administrative penalties including False Claims Act liability, damages, monetary fines, disgorgement, restitution, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, corporate integrity agreements, contractual damages, reputational harm, diminished profits and future earnings and curtailment or restructuring of our operations, among other consequences, any of which could adversely affect our ability to operate.

Our current and future relationships with healthcare professionals, principal investigators, consultants, customers and third-party payors in the United States and elsewhere may be subject, directly or indirectly, to applicable anti-kickback, fraud and abuse, false claims, physician payment transparency, and other healthcare laws and regulations, which could expose us to penalties.

Healthcare providers and third-party payors in the United States and elsewhere play a primary role in the recommendation and prescription of our products, as well as any product candidates for which we obtain marketing approval. Our and our commercial partners' current and future arrangements with healthcare professionals, principal investigators, consultants, customers and third-party payors may expose us to broadly applicable fraud and abuse and other healthcare laws, including, without limitation, the Anti-Kickback Statute and the False Claims Act, that may constrain the business or financial arrangements and relationships through which we and our commercial partners sell, market and distribute any product candidates for which we obtain marketing approval. In addition, we may be subject to transparency laws with respect to drug pricing and transfers of value made to physicians and other healthcare professionals. The applicable federal, state and foreign healthcare laws that may affect our ability to operate include the following:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or paying remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, lease, order or arranging for the purchase, lease or order of, any good, facility, item or service, for which payment may be made, in whole or in part, under federal and state healthcare programs such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation;

- federal civil and criminal false claims laws, including the False Claims Act, which impose criminal and civil penalties, including through civil whistleblower or *qui tam* actions, against individuals or entities for, among other things, knowingly presenting, or causing to be presented, to the federal government, including the Medicare and Medicaid programs, claims for payment that are false or fraudulent or making or using a false record or statement material to a false or fraudulent claim or to avoid, decrease or conceal an obligation to pay money to the federal government, including erroneous pricing information on which mandatory rebates, discounts and reimbursement amounts are based, or in the case of the False Claims Act, for violations of the federal Anti-Kickback Statute in connection with a claim for payment or for conduct constituting reckless disregard for the truth;

- the civil monetary penalties statute, which imposes penalties against any person or entity who, among other things, is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent;

- the Health Insurance Portability and Accountability Act, or HIPAA, which created additional federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of whether the payor is public or private, knowingly and willfully embezzling or stealing from a health care benefit program, willfully obstructing a criminal investigation of a health care offense and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation;

- the federal Physician Payments Sunshine Act and its implementing regulations, impose new annual reporting requirements for manufacturers of drugs, devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with certain exceptions, to annually report certain payments and transfers of value provided to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other healthcare professionals (physician assistants, nurse practitioners, clinical nurse specialists, certified nurse anesthetists, anesthesiologist assistants and certified nurse-midwives) and teaching hospitals, or to entities or individuals at the request of, or designated on behalf of, such providers and teaching hospitals, and to report annually certain ownership and investment interests held by physicians and their immediate family members; and

- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; certain state laws which require drug manufacturers to comply with the industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and may require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; certain state laws that require manufacturers to report information on the pricing of certain drug products; and certain state and local laws require the registration or pharmaceutical sales representatives.

These laws may affect our and our commercial partners' sales, marketing, and other promotional activities by imposing administrative and compliance burdens on us and our commercial partners.

Efforts to ensure that our current and future business arrangements with third parties will comply with applicable healthcare laws and regulations may involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws, or that our compliance systems are inadequate to detect and report such conduct or to report accurate pricing information to the government. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, including, without limitation, damages, fines, imprisonment, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, corporate integrity agreements or similar agreements to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations, which could significantly harm our business. If any of the physicians or other healthcare providers or entities with whom we currently, or expect to, do business, including future collaborators, is found not to be in compliance with applicable laws, they and we may be subject to significant penalties and potential exclusion from participation in healthcare programs as a result of their non-compliance.

Recently enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of our product candidates and increase the cost to commercialize our approved products, and any of our product candidates that may be approved in the future and affect the prices thereof.

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect the ability to profitably sell our approved products and our ability to profitably sell any of our product candidates for which we obtain marketing approval.

Among policy makers and payors in the United States and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and/or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. For example, in March 2010, the ACA was signed into law and was intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for the healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms.

Among the provisions of the ACA of importance to our potential product candidates are the following:

- an annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program to 23.1% and 13.0% of the average manufacturer price for branded drugs and generic drugs, respectively;
- expansion of healthcare fraud and abuse laws, including the False Claims Act and the federal Anti-Kickback Statute, new government investigative powers and enhanced penalties for non-compliance;
- establishment of a new and distinct methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 70% point-of-sale discounts off negotiated prices (generally as negotiated between the Medicare Part D plan and the pharmacy) of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D;
- extension of manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations and extension of the inflation percentage applicable to existing branded drugs to new formulations for purposes of computing the inflation penalty component of Medicaid rebates;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals and by adding new mandatory eligibility categories for certain individuals with income at or below 133% of the Federal Poverty Level, thereby potentially increasing manufacturers' Medicaid rebate liability;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- the new requirements under the federal Open Payments program and its implementing regulations;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

Since its enactment, there have been executive, judicial and congressional challenges to certain aspects of the ACA. On June 17, 2021, the U.S. Supreme Court dismissed the most recent judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the ACA. Thus, the ACA will remain in effect in its current form. Further, prior to the U.S. Supreme Court ruling, President Biden issued an executive order that initiated a special enrollment period from February 15, 2021 through August 15, 2021 for purposes of obtaining health insurance coverage through the ACA marketplace. The executive order also instructed certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the ACA.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, included aggregate reductions to Medicare payments to providers, which went into effect in April 2013, and, due to subsequent legislative amendments, will stay in effect through 2032, with the exception of a temporary suspension from May 1, 2020 through March 31, 2022 due to the COVID-19 pandemic, unless additional Congressional action is taken. In January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, further reduced Medicare payments to several providers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. In addition, on March 11, 2021, the American Rescue Plan Act of 2021 was signed into law, which eliminates the statutory Medicaid drug rebate cap, currently set at 100% of a drug's average manufacturer price, beginning January 1, 2024.

Further, there has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the cost of drugs under Medicare, and reform government program reimbursement methodologies for drugs. On August 16, 2022, the Inflation Reduction Act of 2022, or IRA, was signed into law. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023), and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of the Department of Health and Human Services to implement many of these provisions through guidance, as opposed to regulation, for the initial years. For that and other reasons, it is currently unclear how the IRA will be effectuated. At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. These new laws may result in additional reductions in Medicare and other healthcare funding, which could negatively impact customers for our product candidates, if approved, and, accordingly, our financial operations. It is also possible that additional governmental action is taken in response to the COVID-19 pandemic.

We expect that the healthcare reform measures that have been adopted and may be adopted in the future, may, among other things, result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our prodrug product candidates.

Legislative and regulatory proposals and enacted statutes have been made to expand post-approval requirements and restrict sales and promotional activities for drugs. For instance, the Drug Supply Chain Security Act imposes obligations on manufacturers of pharmaceutical products, among others, related to product tracking and tracing. Among the requirements of this new legislation, manufacturers are required to provide specified information regarding the drug products they produce to individuals and entities to which product ownership is transferred, label drug products with a product identifier and keep specified records regarding the drug products. The transfer of information to subsequent product owners by manufacturers will eventually be required to be done electronically. Manufacturers are also required to verify that purchasers of products are appropriately licensed. Further, under this legislation, manufacturers have drug product investigation, quarantine, disposition and FDA and trading-partner notification responsibilities related to counterfeit, diverted, stolen and intentionally adulterated products, as well as products that are the subject of fraudulent transactions or which are otherwise unfit for distribution such that they would be reasonably likely to result in serious health consequences or death.

We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

Governments outside the United States tend to impose strict price controls, which may affect our revenue, if any.

In some countries, particularly the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain coverage and reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. If reimbursement of our prodrug products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be harmed, possibly materially.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Our failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

If our security measures are compromised now, or in the future, or the security, confidentiality, integrity or availability of, our information technology, software, services, communications or data is compromised, limited or fails, this could result in a material adverse impact, including without limitation, a material interruption to our operations, harm to our reputation, significant fines, penalties and liability, breach or a triggering of data protection laws, privacy policies and data protection obligations, loss of customers or sales, or material disruption of our clinical trials or other business activity.

In the ordinary course of our business, we may collect, process and store proprietary, confidential and sensitive information, including personal information (including key-coded data and health information), intellectual property, trade secrets and proprietary business information owned or controlled by ourselves or other parties.

Despite the implementation of security measures, our information technology systems and data, and those of our CROs and other third parties on which we rely, are vulnerable to system failure, interruption, compromise, attack or damage from several sources, such as data corruption; breakdown; malicious human acts; malware (such as ransomware); malicious code (such as computer viruses or worms); fraudulent activity; employee misconduct, theft or error; denial-of-service attacks; public health epidemics (such as the COVID-19 pandemic); cyber-attacks by sophisticated nation-state and nation-state supported actors; natural disasters; terrorism; war (such as the current situation with Ukraine and Russia); and telecommunication and electrical failures. Our recovery systems (and those of third parties upon whom we rely) are similarly vulnerable. Any of these events could lead to the unauthorized access, disclosure and use of proprietary, confidential, or otherwise non-public information (such as personal information). The techniques used by criminal actors to attack computer systems are sophisticated, change frequently and may originate from less regulated and remote areas of the world. As a result, we may not be able to address these techniques proactively or implement adequate preventative measures. Attacks upon information technology systems are also increasing in their levels of persistence and intensity, and are being conducted by sophisticated and organized groups and individuals with a wide range of motives and expertise. As a result of the COVID-19 pandemic, and the continued hybrid working environment, we may also face increased cybersecurity risks due to our reliance on internet technology and the number of our employees who are working remotely, which may create additional opportunities for cybercriminals to exploit vulnerabilities. We may also experience security breaches that may remain undetected for an extended period. We may be required to expend significant resources, fundamentally change our business activities and practices, or modify our operations, including our clinical trial activities, or information technology in an effort to protect against security breaches and to mitigate, detect and remediate actual and potential vulnerabilities. Due to the nature of some of these threats, we may be unable to anticipate threats, and there is a risk that a threat may remain undetected for a period of time. The costs of maintaining or upgrading our cyber-security systems at the level commercially reasonable to keep up with our expanding operations and prevent against potential attacks are increasing, and despite our best efforts, our network security and data recovery measures and those of our vendors may still not be adequate to protect against such security breaches and disruptions, which could cause harm to our business, financial condition and results of operations.

We and certain of our service providers are from time to time subject to cyberattacks and security incidents. While we do not believe that we have experienced any significant system failure, accident or security breach to date, if we, our service providers, partners, or other relevant third parties experience any such incident that results in any data loss, deletion or destruction, unauthorized access to, loss of, acquisition or disclosure of, exposure or disclosure of sensitive information, or compromise related to the security, confidentiality, integrity or availability of our (or their) information technology, software, services, communications or data, it may result in a material adverse impact, including without limitation, fines, damages, litigation, enforcement actions, loss of trade secrets, a material disruption of our drug development programs, or other harm to our business. For example, the loss of clinical trial data from completed or ongoing or planned clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach was to result in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development of our product candidates could be delayed. Additionally, applicable data protection laws, privacy policies and data protection obligations (such as contractual obligations) may require us to notify relevant stakeholders of security breaches, including affected individuals, customers and regulators. Such disclosures are costly, and the disclosure or the failure to comply with such requirements, could lead to material adverse impacts, including without limitation, negative publicity, a loss of confidence in our products or operations or breach of contract claims. There can be no assurance that the limitations of liability in our contracts would be enforceable or adequate or would otherwise protect us from liabilities or damages if we fail to comply with applicable data protection laws, privacy policies or data protection obligations related to information security or security breaches.

Further, failures or significant downtime of our information technology or telecommunication systems or those used by our third-party service providers could cause significant interruptions in our operations and adversely impact the confidentiality, integrity and availability of sensitive or confidential information, including preventing us from conducting clinical trials, tests or research and development activities and preventing us from managing the administrative aspects of our business.

We cannot be sure that our insurance coverage will be adequate or otherwise protect us from or adequately mitigate liabilities or damages with respect to claims, costs, expenses, litigation, fines, penalties, business loss, data loss, regulatory actions or materially adverse impacts arising out of our privacy and cybersecurity practices, processing or security breaches that we may experience, or that such coverage will continue to be available on acceptable terms at all. The successful assertion of one or more large claims against us that exceeds our available insurance coverage, or results in changes to our insurance policies (including premium increases or the imposition of a large deductible or co-insurance requirements), could have an adverse effect on our business. In addition, we cannot be sure that our existing coverage and coverage for errors and omissions will continue to be available on acceptable terms or that our insurers will not deny coverage as to any future claim.

Failure or perceived failure to comply with existing or future laws, regulations, contracts, self-regulatory schemes, policies, standards and other obligations related to data privacy or security could lead to government enforcement actions (which could include civil or criminal fines or penalties), a disruption of our clinical trials or commercialization of our products, private litigation, other liabilities, and/or adverse publicity. Compliance or the failure to comply with such obligations could increase the costs of our products, could limit their use or adoption, and could otherwise negatively affect our operating results and business.

Regulation of data (including personal data) is evolving, as federal, state, and foreign governments continue to adopt new, or modify existing, laws and regulations addressing data privacy and security, and the collection, processing, storage, transfer, and use of data. We and our collaborators may be subject to current, new, or modified federal, state, and foreign data protection laws and regulations. These new or proposed laws and regulations are subject to differing interpretations and may be inconsistent among jurisdictions, and guidance on implementation and compliance practices are often updated or otherwise revised, which adds to the complexity of processing personal data. Moreover, we are subject to the terms of our privacy and security policies, representations, certifications, standards, publications, contracts and other obligations to third parties related to data privacy, security and processing. These and other requirements could require us or our collaborators to incur additional costs to achieve compliance, limit our competitiveness, necessitate the acceptance of more onerous obligations in our contracts, restrict our ability to use, store, transfer, and process data, impact our or our collaborators' ability to process or use data in order to support the provision of our products, affect our or our partners' ability to offer our products or operate in certain locations, cause regulators to reject, limit, or disrupt our clinical trial activities, result in increased expenses, reduce overall demand for our products and services and make it more difficult to meet expectations of or commitments to customers or collaborators.

In the United States, numerous federal and state laws and regulations govern the collection, use, disclosure, and protection of health-related and other personal information. These laws and regulations could apply to our operations, the operations of our collaborators or other relevant stakeholders upon whom we depend. In addition, we may obtain health information from third parties (including research institutions from which we may obtain clinical trial data) that are subject to privacy and security requirements under HIPAA. We do not believe that we are currently acting as a covered entity or business associate under HIPAA and thus are not directly subject to its requirements or penalties. However, any person may be prosecuted under HIPAA's criminal provisions either directly or under aiding-and-abetting or conspiracy principles. Consequently, depending on the facts and circumstances, we could be subject to civil and criminal penalties, including if we knowingly obtain, use, or disclose individually identifiable health information maintained by a HIPAA-covered entity in a manner that is not authorized or permitted by HIPAA.

Certain states have also adopted comparable privacy and security laws and regulations, some of which may be more stringent than HIPAA. For example, the California Consumer Privacy Act of 2018, or CCPA, became effective on January 1, 2020. The CCPA gives California residents individual privacy rights, including the right to access and delete their personal information, opt out of certain personal information sharing and receive detailed information about how their personal information is used. It also provides for civil penalties for violations, as well as a private right of action and statutory damages for data breaches that has increased the likelihood of, and risks associated with, class action data breach litigation. Although there are limited exemptions for clinical trial data, the CCPA's implementation standards and enforcement practices are likely to remain uncertain for the foreseeable future. The CCPA may increase our compliance costs and potential liability. Further, the California Privacy Rights Act, or CPRA, went into effect on January 1, 2023, and significantly amends the CCPA. The CPRA, among other things, gives California residents the ability to limit use of certain sensitive information, establish restrictions on the retention of personal information, expand the types of data breaches subject to the CCPA's private right of action and establish a new California Privacy Protection Agency to implement and enforce the new law. Similar laws have passed in Virginia, Colorado, Connecticut, and Utah, and have been proposed in other states and at the federal level, reflecting a trend toward more stringent privacy legislation in the United States. As we expand our operations, these and similar laws may increase our compliance costs and potential liability.

Foreign data protection laws, such as the GDPR and member state data protection laws, may also apply to health-related and other personal data that we process, including personal data relating to clinical trial participants. European data protection laws impose strict obligations on the ability to process health-related and other personal data of data subjects in Europe, including standards relating to the privacy and security of personal data. For example, the GDPR went into effect in May 2018 and imposes strict requirements for processing the personal data of individuals within the EEA. Companies that must comply with the GDPR face increased compliance obligations and risk, including more robust regulatory enforcement of data protection requirements and potential fines for noncompliance of up to €20 million or 4% of the annual global revenues of the noncompliant company, whichever is greater. Among other requirements, the GDPR regulates transfers of personal data subject to the GDPR to third countries that have not been found to provide adequate protection to such personal data, including the United States, and the efficacy and longevity of current transfer mechanisms between the European Union and the United States remains uncertain. A decision by the Court of Justice of the European Union limited how organizations could lawfully transfer personal data from the European Union/EEA to the United States by invalidating the EU-U.S. Privacy Shield Framework, and raised questions about whether the European Commission's Standard Contractual Clauses, or SCCs, one of the primary alternatives to the Privacy Shield, can lawfully be used for personal information transfers from Europe to the United States or most other countries. Similarly, the Swiss Federal Data Protection and Information Commissioner recently opined that the Swiss-U.S. Privacy Shield is inadequate for transfers of personal data from Switzerland to the U.S. In March 2022, the US and EU announced a new regulatory regime intended to replace the invalidated regulations; however, this new EU-US Data Privacy Framework has not been implemented beyond an executive order signed by President Biden on October 7, 2022 on Enhancing Safeguards for United States Signals Intelligence Activities. European court and regulatory decisions subsequent to the CJEU decision of July 16, 2020 have taken a restrictive approach to international data transfers. As supervisory authorities issue further guidance on personal data export mechanisms, including circumstances where the SCCs cannot be used, and/or start taking enforcement action, we could suffer additional costs, complaints and/or regulatory investigations or fines, and/or if we are otherwise unable to transfer personal data between and among countries and regions in which we operate, it could affect the manner in which we provide our services, the geographical location or segregation of our relevant systems and operations, and could adversely affect our financial results. Additionally, other countries have passed or are considering passing laws requiring local data residency and/or restricting the international transfer of data.

Further, from January 1, 2021, companies have had to comply with the GDPR and also the UK data protection.regime, which imposes separate but similar obligations to those under the GDPR in UK national law. The UK GDPR mirrors the fines under the GDPR, i.e., fines up to the greater of €20 million (£17.5 million) or 4% of global turnover. As we continue to expand into other foreign countries and jurisdictions, we may be subject to additional laws and regulations that may affect how we conduct business.

Failure, or perceived failure, to comply with federal, state and foreign data protection laws and regulations, privacy policies, contracts and other data protection obligations could result in government investigations and enforcement actions (which could include civil or criminal penalties, fines, or sanctions), private litigation, a diversion of management's attention, adverse publicity and other negative effects on our operating results and business. There can be no assurance that the limitations of liability in our contracts would be enforceable or adequate or would otherwise protect us from liabilities or damages if we fail to comply with applicable data protection laws, privacy policies or data protection obligations related to information security or security breaches. Moreover, clinical trial participants or subjects about whom we or our collaborators obtain information, as well as the providers who share this information with us, may contractually limit our ability to use and disclose the information. Claims that we have violated individuals' privacy rights or failed to comply with data protection laws, contracts, privacy notices or other obligations even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business.

Any of these matters could materially adversely affect our business, financial condition, or operational results.

Risks Related to Employee Matters and Managing Our Growth

Our future success depends on our ability to retain key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on the management, research and development, clinical, financial and business development expertise of Richard W. Pascoe, our chief executive officer, R. LaDuane Clifton, CPA, our chief financial officer, Sven Guenther, Ph.D., our chief scientific officer, Christal Mickle, M.A., our chief product development officer, Joshua Schafer, our chief commercial officer and executive vice president, business development, as well as the other members of our scientific and clinical teams. Although we have employment agreements with each of our executive officers, these agreements do not obligate them to continue working for our company and they may terminate their employment with us at any time.

Recruiting and retaining qualified scientific and clinical personnel and, if we progress the development of our product candidate pipeline toward scaling up for commercialization, manufacturing and sales and marketing personnel, will also be critical to our success. The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize our prodrug product candidates. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

Risks Related to Ownership of Our Common Stock and Our Status as a Public Company

The trading price of the shares of our common stock is likely to be volatile, and purchasers of our common stock could incur substantial losses.

Our stock price has been, and is likely to continue to be, volatile. Since shares of our common stock were sold in our initial public offering in April 2015 at a price of $176.00 per share (adjusted to give effect to the 1-for-16 reverse stock split), our stock price has ranged from a low of $1.94 to a high of $418.40 through March 1, 2023. In addition, the stock market in general and the market for pharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, investors may not be able to sell their common stock at or above the price paid for the shares. The market price for our common stock may be influenced by many factors, including:

- actual or anticipated variations in our operating results;
- changes in financial estimates by us or by any securities analysts who might cover our stock;
- conditions or trends in our industry, including without limitation changes in the structure of healthcare payment systems;
- stock market price and volume fluctuations of comparable companies and, in particular, those that operate in the pharmaceutical industry;
- announcements by us or our competitors of significant acquisitions, strategic partnerships or divestitures;
- announcements of investigations or regulatory scrutiny of our operations or lawsuits filed against us;
- adverse regulatory announcements or determinations regarding our product candidates;
- capital commitments;
- investors' general perception of us and our business;
- global macroeconomic conditions, including inflation, labor shortages, supply chain shortages, or other economic, political or legal uncertainties or adverse developments;
- political unrest, terrorism and wars, such as the current situation with Ukraine and Russia, which could delay or disrupt our business, and if such political unrest escalates or spills over to or otherwise impacts additional regions it could heighten many of the other risk factors included in this Item 1A;
- other events or factors, including those resulting from system failures and disruptions, earthquakes, hurricanes, other natural disasters, pandemics, or responses to these events;
- recruitment or departure of key personnel; and
- sales of our common stock, including sales by our directors and officers or specific stockholders.

Many of the factors described above are not within our control. For instance, in May 2016, we announced that the Anesthetic and Analgesic Drug Products Advisory Committee and the Drug Safety and Risk Management Advisory Committee of the FDA voted 16 to 4 for the approval of APADAZ but voted 18 to 2 against inclusion of abuse-deterrent labeling for APADAZ. The announcement was followed by a substantial decrease in the trading price of our common stock on Nasdaq. Additionally, when we announced in June 2016 that the FDA had issued a CRL for the APADAZ NDA, the trading price of our common stock on Nasdaq was subject to another substantial decrease. We cannot guarantee that future announcements will not have similar effects on the trading price of our common stock.

In addition, in the past, stockholders have initiated class action lawsuits against pharmaceutical and biotechnology companies following periods of volatility in the market prices of these companies' stock. For instance, in December 2016, we received notice of a class action suit filed against us in the Iowa District Court in Johnson County by a stockholder alleging that we, certain of our senior executives and directors who signed the registration statement in connection with our initial public offering, and each of the investment banks that acted as underwriters for the offering negligently issued untrue statements of material facts and omitted to state material facts required to be stated in the registration statement and incorporated offering materials that we filed with the U.S. Securities and Exchange Commission, or the SEC, in support of the offering. In June 2018, the case was dismissed without prejudice to members of the putative class. Future litigation could cause us to incur substantial costs and divert management's attention and resources from our business. Further, biotechnology and pharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance.

A portion of our outstanding warrants are entitled to certain anti-dilution protections which, if triggered, may cause dilution to your investment.

The warrant held by OTA LLC, or the OTA Warrant, includes an exercise price protection provision, pursuant to which the exercise price of the OTA Warrant will be adjusted downward on a broad-based weighted-average basis if we issue or sell any shares of common stock, convertible securities, warrants or options at a sale or exercise price per share less than the greater of (i) $38.34 per share, which represents the OTA Warrant's exercise price, or (ii) the closing sale price of our common stock on the last trading date immediately prior to such issuance or, in the case of a firm commitment underwritten offering, on the date of execution of the underwriting agreement between us and the underwriters for such offering. Additionally, if we effect an "at the market offering", as defined in Rule 415 of the Securities Act, of our common stock, the exercise price of the OTA Warrant will be adjusted downward pursuant to this anti-dilution adjustment only if such sales are made at a price less than $38.34 per share, provided that this anti-dilution adjustment will not apply to certain specified sales.

Future sales and issuances of equity and debt could result in additional dilution to our stockholders.

We may need additional capital in the future to fund our planned future operations, including to complete potential clinical trials for our product candidates. To raise capital, we may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time.

For instance, in January 2021, we issued and sold 6,765,463 shares of our common stock, pre-funded warrants to purchase 926,844 shares of our common stock and warrants to purchase 7,692,307 shares of our common stock in the Public Offering (as defined herein). Also in January 2021, the underwriter exercised its over-allotment option, in part, for warrants to purchase 754,035 shares of our common stock. Further, in February 2021, the underwriter again exercised its over-allotment option, in part, to purchase 374,035 shares of our common stock.

Also in January 2021, we entered into the January 2021 Inducement Letters with certain holders of the Existing Warrants, pursuant to which such holders exercised for cash their Existing Warrants to purchase 6,620,358 shares of our common stock in exchange for the January 2021 Inducement Warrants on substantially the same terms as the Existing Warrants, except as set forth in the following sentence, to purchase up to 7,944,430 shares of our common stock, which was equal to 120% of the number of shares of our common stock issued upon exercise of the Existing Warrants. The purchase price of the January 2021 Inducement Warrants was $0.125 per share underlying each January 2021 Inducement Warrant, and the January 2021 Inducement Warrants have an exercise price of $6.36 per share.

In addition, in June 2021, we entered into the June 2021 Inducement Letters with certain holders of the January 2021 Inducement Warrants, pursuant to which such holders exercised for cash their January 2021 Inducement Warrants to purchase 6,117,509 shares of our common stock in exchange for the June 2021 Inducement Warrants on substantially the same terms as the January 2021 Inducement Warrants, except as set forth in the following sentence, to purchase up to 1,529,379 shares of our common stock, which was equal to 25% of the number of shares of our common stock issued upon exercise of the January 2021 Inducement Warrants. The purchase price of the June 2021 Inducement Warrants was $0.125 per share underlying each June 2021 Inducement Warrant, and the June 2021 Inducement Warrants have an exercise price of $16.50 per share.

If the holders of any of these warrants elect to exercise such warrants and receive shares of common stock, your ownership interest will be diluted and the market price of our common stock may be materially and adversely effected.

In June 2021, at the annual meeting of stockholders, the stockholders approved the Amended and Restated 2014 Equity Incentive Plan, which, among other things, added 4,900,000 shares to the equity plan pool, and the employee stock purchase plan, or the 2021 ESPP, which allows for employees to purchase up to 1.5 million shares of stock through the plan. In addition, in July 2021, the Company entered into the Equity Distribution Agreement with JMP Securities LLC, or JMP, and RBC Capital Markets, LLC, or RBCCM, under which the Company may offer and sell, from time to time at its sole discretion, shares of its common stock having an aggregate offering price of up to $75,000,000 through JMP and RBCCM as its sales agents.

Pursuant to our equity incentive plan, we may grant equity awards and issue additional shares of our common stock to our employees, directors and consultants, and the number of shares of our common stock reserved for future issuance under this plan will be subject to automatic annual increases in accordance with the terms of the plans. In January 2023, our Board approved the 2023 Employment Inducement Award Plan, or the 2023 Plan, under which we may grant equity awards to certain new employees. The maximum number of shares of common stock to be issued under the 2023 Plan is 1,500,000. To the extent that new options are granted and exercised, or we issue additional shares of common stock in the future, either through the 2021 ESPP, Equity Distribution Agreement, 2023 Plan, or through other means, our stockholders may experience additional dilution, which could cause our stock price to fall.

The accounting method for the OTA Warrant and certain other warrants issued could have a material effect on our reported financial results.

The OTA Warrant and certain other warrants we issued contain embedded derivatives, which require mark-to-market accounting treatment and could result in a gain or loss on a quarterly basis with regards to the mark-to-market value of that feature. Such accounting treatment could have a material impact on, and could potentially result in significant volatility in, our results of operations.

Sales of a substantial number of shares of our common stock in the public market could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. If our stockholders sell, or the market perceives that our stockholders intend to sell, substantial amounts of our common stock in the public market, the market price of our common stock could decline significantly.

We cannot guarantee that any share repurchase program will be fully consummated or will enhance long-term stockholder value, and share repurchases could increase the volatility of our stock prices and could diminish our cash reserves.

On December 20, 2021, we initiated a share repurchase program, or the Share Repurchase Program, pursuant to which we may repurchase up to $50 million of shares of our common stock through December 31, 2023. Capital allocation to the Share Repurchase Program will be based on a variety of factors, including our business results, the receipt of royalties and sales milestones under the AZSTARYS License Agreement, and potentially other sources of non-dilutive capital that may become available to the Company. The exact number of shares to be repurchased by us is not guaranteed and the program may be suspended, modified, or discontinued at any time without prior notice. As of December 31, 2022, we had repurchased 909,953 shares of our common stock for approximately $7.5 million under the Share Repurchase Program. Our share repurchases could affect our share trading prices, increase their volatility, reduce our cash reserves and may be suspended or terminated at any time, which may result in a decrease in the trading prices of our stock.

Anti-takeover provisions in our certificate of incorporation and bylaws, as well as provisions of Delaware law and the terms of some or our contracts, might discourage, delay or prevent a change in control of our company or changes in our board of directors or management and, therefore, depress the price of our common stock.

Our certificate of incorporation and bylaws and Delaware law contain provisions that may discourage, delay or prevent a merger, acquisition or other change in control that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares of our common stock or transactions that our stockholders might otherwise deem to be in their best interests. These provisions may also prevent or frustrate attempts by our stockholders to replace or remove members of our board of directors or our management. Therefore, these provisions could adversely affect the price of our stock. Our corporate governance documents include provisions:

- establishing a classified board of directors with staggered three-year terms so that not all members of our board of directors are elected at one time;
- providing that directors may be removed by stockholders only for cause;
- preventing the ability of our stockholders to call and bring business before special meetings and to take action by written consent in lieu of a meeting;
- requiring advance notice of stockholder proposals for business to be conducted at meetings of our stockholders and for nominations of candidates for election to our board of directors;
- permitting the board of directors to issue up to 10,000,000 shares of preferred stock with any rights, preferences and privileges they may designate;
- limiting the liability of, and providing indemnification to, our directors and officers;
- providing that vacancies may be filled by remaining directors;
- preventing cumulative voting; and
- providing for a supermajority requirement to amend our bylaws.

As a Delaware corporation, we are also subject to provisions of Delaware law, including Section 203 of the General Corporation Law of the State of Delaware, which prohibits a Delaware corporation from engaging in a broad range of business combinations with any "interested" stockholder for a period of three years following the date on which the stockholder became an "interested" stockholder.

In addition, the provisions of our termination agreement with Aquestive may delay or prevent a change in control of our company. For example, if we enter into a merger, an asset sale or any other change of control transaction, then Aquestive will be entitled to a royalty equal to 10% of the price being paid to us and our stockholders in such transaction which is attributable to the value of AZSTARYS, KP879 or KP1077.

Any provision of our certificate of incorporation, bylaws or Delaware law or any term of our contracts that has the effect of discouraging, delaying or preventing a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock and could also affect the price that some investors are willing to pay for our common stock.

If we engage in acquisitions to grow our business, we will incur a variety of costs and may potentially face numerous risks that could adversely affect our business and operations and cause our stock price to decline.

If appropriate opportunities become available, we may seek to acquire businesses or assets to enhance our business. For example, in May 2022, we acquired all of the assets and operations of Orphazyme related to arimoclomol. In connection with any acquisitions, we could issue additional equity securities, which would dilute our stockholders, incur substantial debt to fund the acquisitions or assume significant liabilities.

Acquisitions involve many and diverse risks and uncertainties, including problems integrating the purchased operations or assets as well as unanticipated costs, liabilities, and economic, political, legal and regulatory challenges due to our inexperience operating in new regions or countries, and we may fail to successfully integrate acquired companies, such as Orphazyme, or retain key personnel from the acquired company. To date, we have limited experience with acquisitions and the integration of acquired operations and personnel. Acquisitions may divert our attention from our core business. Acquisitions may require us to record goodwill and non-amortizable intangible assets that will be subject to testing on a regular basis and potential period impairment charges, incur amortization expenses related to certain intangible assets, and incur write offs and restructuring and other related expenses, any of which could harm our operating results and financial condition.

New business strategies, especially those involving acquisitions, are inherently risky and may not be successful. Failure to successfully identify, complete, manage and integrate acquisitions could materially and adversely affect our business, financial condition and results of operations and could cause our stock price to decline.

We could be negatively affected as a result of a proxy contest and the actions of activist stockholders.

On January 26, 2023, our board of directors received notice from a stockholder of his intention to nominate three nominees to stand for election to our board of directors at our 2023 annual meeting of stockholder and to submit a proposal at the annual meeting. A proxy contest with respect to election of our directors, or other activist stockholder activities, could adversely affect our business because: (1) responding to a proxy contest and other actions by activist stockholders can be costly and time-consuming, disruptive to our operations and divert the attention of management and our employees; (2) perceived uncertainties as to our future direction caused by activist activities may result in the loss of potential business opportunities, and may make it more difficult to attract and retain qualified personnel and business partners; and (3) if individuals are elected to our board of directors with a specific agenda, it may adversely affect our ability to effectively and timely implement our strategic plans.

Our certificate of incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or other employees.

Our certificate of incorporation provides that the Court of Chancery of the State of Delaware is the sole and exclusive forum for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a breach of fiduciary duty owed by any of our directors, officers or other employees to us or our stockholders, (iii) any action asserting a claim against us arising pursuant to any provisions of the Delaware General Corporation Law, or DGCL, our certificate of incorporation or our bylaws, or (iv) any action asserting a claim against us that is governed by the internal affairs doctrine.

In addition, on and effective July 15, 2020, we amended and restated our bylaws, or the Bylaws, pursuant to which: (i) unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if and only if the Court of Chancery of the State of Delaware lacks subject matter jurisdiction, any state court located within the State of Delaware or, if and only if all such state courts lack subject matter jurisdiction, the federal district court for the District of Delaware) shall be the sole and exclusive forum for the following types of actions or proceedings under Delaware statutory or common law: (A) any derivative action or proceeding brought on behalf of us; (B) any action or proceeding asserting a claim of breach of a fiduciary duty owed by any of our current or former directors, officers or other employees, to us or our stockholders; (C) any action or proceeding asserting a claim against us or any of our current or former directors, officers or other employees, arising out of or pursuant to any provision of the DGCL our certificate of incorporation or our Bylaws (as each may be amended from time to time); (D) any action or proceeding to interpret, apply, enforce or determine the validity of our certificate of incorporation or our Bylaws (including any right, obligation, or remedy thereunder); (E) any action or proceeding as to which the DGCL confers jurisdiction to the Court of Chancery of the State of Delaware; and (F) any action or proceeding asserting a claim against us or any of our directors, officers or other employees, governed by the internal affairs doctrine, in all cases to the fullest extent permitted by law and subject to the court's having personal jurisdiction over the indispensable parties named as defendants, *provided that* this provision shall not apply to suits brought to enforce a duty or liability created by the Securities Act or the Exchange Act, or any other claim for which the federal courts have exclusive jurisdiction; (ii) unless we consent in writing to the selection of an alternative forum, to the fullest extent permitted by law, the federal district courts of the United States of America shall be the exclusive forum for the resolution of any complaint asserting a cause of action arising under the Securities Act; and (iii) any person or entity holding, owning or otherwise acquiring any interest in any security of us shall be deemed to have notice of and consented to the provisions of the Bylaws.

These choice of forum provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees, which may discourage such lawsuits against us and our directors, officers and other employees. If a court were to find the choice of forum provision contained in our certificate of incorporation or our Bylaws to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions.

We might not be able to utilize a significant portion of our net operating loss carryforwards, which could adversely affect our profitability.

As of December 31, 2022, we had federal net operating loss carryforwards of approximately $236.0 million, due to prior period losses, $138.1 million of which, if not utilized, will begin to expire in 2027. These net operating loss carryforwards could expire unused and be unavailable to offset future income tax liabilities, which could adversely affect our profitability. These federal net operating loss carryforwards are fully reserved under a valuation allowance in the consolidated balance sheet as of December 31, 2022. On December 22, 2017, the U.S. government enacted H.R. 1, "An Act to provide for reconciliation pursuant to titles II and V of the concurrent resolution on the budget for fiscal year 2018" (informally titled the Tax Cuts and Jobs Act). Under the Tax Cuts and Jobs Act, U.S. federal net operating losses incurred in 2018 and in future years may be carried forward indefinitely, but the deductibility of such federal net operating losses is limited. It is uncertain if and to what extent various states will conform to the Tax Cuts and Jobs Act. To the extent that we continue to generate taxable losses in the United States, unused losses will carry forward to offset future taxable income (subject to any applicable limitations), if any. In addition, under Section 382 and Section 383 of the Code, if a corporation undergoes an "ownership change," which is generally defined as a greater than 50% change, by value, in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change income may be limited. We performed a Section 382 ownership change analysis in 2017 and determined that we experienced an ownership change in 2010, which resulted in a portion of our net operating loss carryforwards being subject to an annual limitation under Section 382 through 2012. No other ownership changes or limitations on our historical net operating loss carryforwards were noted through the year ended December 31, 2017. In addition, we may have already experienced an ownership change, or may experience ownership changes in the future, as a result of shifts in our stock ownership, including as a result of the conversion of our outstanding convertible debt or as a result of changes in our stock ownership. If we determine that an ownership change has occurred and our ability to use our historical net operating loss carryforwards is materially limited, it would harm our future operating results by increasing our future tax obligations. As of December 31, 2022, we maintain a full valuation allowance over our deferred tax assets for financial reporting purposes.

Changes in tax laws or regulations that are applied adversely to us may have a material adverse effect on our business, cash flow, financial condition or results of operations.

New income, sales, use or other tax laws, statutes, rules, regulations or ordinances could be enacted at any time, which could affect the tax treatment of our domestic earnings, if any. Any new taxes could adversely affect our business operations, and our business and financial performance. Further, existing tax laws, statutes, rules, regulations or ordinances could be interpreted, changed, modified or applied adversely to us. For example, legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act, significantly revised the Internal Revenue Code of 1986, as amended. Future guidance from the Internal Revenue Service and other tax authorities with respect to the Tax Cuts and Jobs Act may affect us, and certain aspects of the Tax Cuts and Jobs Act could be repealed or modified in future legislation. In addition, the recent Inflation Reduction Act enacted in the United States introduced, among other changes, a 15% corporate minimum tax on certain United States corporations and a 1% excise tax on certain stock redemptions by United States corporations. Furthermore, it is uncertain if and to what extent various states will conform to the Tax Cuts and Jobs Act or any newly enacted federal tax legislation. Changes in corporate tax rates, the realization of net deferred tax assets relating to our operations, the taxation of foreign earnings, and the deductibility of expenses under the Tax Cuts and Jobs Act or future reform legislation could have a material impact on the value of our deferred tax assets, could result in significant one-time charges, and could increase our future U.S. tax expense.

If we fail to maintain proper and effective internal control, our ability to produce accurate financial statements on a timely basis could be impaired.

We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, and the rules and regulations of the stock market on which our common stock is listed. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal control over financial reporting. For our fiscal year ended December 31, 2022, we performed system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting in our Annual Report on Form 10-K, as required by Section 404 of the Sarbanes-Oxley Act. We will be required to perform this evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting on an annual basis. This requires that we incur substantial additional professional fees and internal costs and that we expend significant management efforts on an annual basis. We have and will be required to test our internal control within a specified period, and, as a result, we may experience difficulty in meeting these reporting requirements.

We may discover weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act, or if we are unable to maintain proper and effective internal control, we may not be able to produce timely and accurate financial statements. If that were to happen, the market price of our stock could decline and we could be subject to sanctions or investigations by the stock exchange on which our common stock is listed, the SEC or other regulatory authorities.

General Risk Factors

An active trading market for our common stock may not be sustained and you may not be able to resell your shares of our common stock for a profit, if at all.

An active trading market for our shares may not be sustained. If an active market for our common stock is not sustained, it may be difficult for you to sell our shares at an attractive price, or at all.

If equity research analysts do not publish research or reports, or publish unfavorable research or reports, about us, our business or our market, our stock price and trading volume could decline.

The trading market for our common stock is influenced by the research and reports that securities or industry analysts publish about us or our business, our market and our competitors. We do not have any control over these analysts. If one or more of the analysts who cover us downgrade our shares or change their opinion of our shares, our share price would likely decline. If one or more of these analysts cease coverage of our company or fail to regularly publish reports on us, we could lose visibility in the financial markets, which could cause our share price or trading volume to decline.

Because we do not anticipate paying any cash dividends on our common stock in the foreseeable future, capital appreciation, if any, will be your sole source of gains and you may never receive a return on your investment.

You should not rely on an investment in our common stock to provide dividend income. We have not declared or paid cash dividends on our common stock to date. We currently intend to retain our future earnings, if any, to fund the development and growth of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future. Investors seeking cash dividends should not purchase our common stock.

We incur increased costs and demands upon management as a result of being a public company.

As a public company listed in the United States, we incur significant additional legal, accounting and other costs. These additional costs could negatively affect our financial results. In addition, changing laws, regulations and standards relating to corporate governance and public disclosure, including regulations implemented by the SEC, may increase legal and financial compliance costs and make some activities more time consuming. These laws, regulations and standards are subject to varying interpretations and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. We intend to invest resources to comply with evolving laws, regulations and standards, and this investment may result in increased general and administrative expenses and a diversion of management's time and attention from revenue-generating activities to compliance activities. If, notwithstanding our efforts to comply with new laws, regulations and standards, we fail to comply, regulatory authorities may initiate legal proceedings against us.

Failure to comply with these rules might also make it more difficult for us to obtain some types of insurance, including director and officer liability insurance, and we might be forced to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, on committees of our board of directors or as members of senior management.

Global economic uncertainty and other global economic or political and regulatory developments could have a material adverse effect on our business, cash flows, financial condition and/or prospects. Growth in the global pharmaceutical market has become increasingly tied to (i) global economic growth as an economic downturn may, for example as a result of the COVID-19 pandemic's paralyzing effects on economic activities, reduce the amount of funding for the pharmaceutical sector as a whole or certain diseases targeted by us and (ii) political conditions, tension and uncertainty which could, for instance, impact the regulations applicable to us. Uncertain political and geopolitical conditions currently exist in various parts of the world, such as the ongoing conflict between Russia and Ukraine. As a result, the United States and other governments have announced certain sanctions against Russia. The conflict in Ukraine and any retaliatory measures taken by the United States and other governments could threaten global security and result in further regional conflict and otherwise have a lasting impact on regional and global economies, any or all of which could adversely affect our business. In addition, the full effects of the United Kingdom's exit from the European Union in January 2020 are impossible to predict but may result in significant market volatility and dislocation, and adversely affect the United Kingdom, European and global economy.

Future legal or regulatory changes in jurisdictions where we currently operate, or in such jurisdictions in which we may choose to operate in the future, could materially and adversely affect our business, results of operations, cash flows, financial condition and/or prospects, including by imposing regulatory and operational restrictions and compliance obligations on our business, reducing our revenue or increasing our expenses.

The above circumstances, individually or in the aggregate, could have a material adverse effect on our business, cash flows, financial condition and/or prospects.

The increasing focus on environmental sustainability and social initiatives could increase our costs, harm our reputation and adversely impact our financia results.

There has been increasing public focus by investors, patients, environmental activists, the media and governmental and nongovernmental organizations on a variety of environmental, social and other sustainability matters. We may experience pressure to make commitments relating to sustainability matters that affect us, including the design and implementation of specific risk mitigation strategic initiatives relating to sustainability. If we are not effective in addressing environmental, social and other sustainability matters affecting our business, or setting and meeting relevant sustainability goals, our reputation and financial results may suffer. In addition, even if we are effective at addressing such concerns, we may experience increased costs as a result of executing upon our sustainability goals that may not be offset by any benefit to our reputation, which could have an adverse impact on our business and financial condition.

In addition, this emphasis on environmental, social and other sustainability matters has resulted and may result in the adoption of new laws and regulations, including new reporting requirements. If we fail to comply with new laws, regulations or reporting requirements, our reputation and business could be adversely impacted.

ITEM 1B. UNRESOLVED STAFF COMMENTS

Not applicable.

ITEM 2. PROPERTIES

As of December 31, 2022, we have leased approximately 17,000 square feet of headquarters office space in Celebration, Florida, comprised of two contiguous office suites, under a non-cancelable lease agreement that expires in August 2025 and February 2026, respectively. One such office suite, which is approximately 6,300 square feet and has an expiration date of February 2026, is subleased to a third party. We have the right to extend the term of the lease for two successive five-year terms upon expiration. In addition, we occupy leased laboratory space in Coralville, Iowa and Blacksburg, Virginia and leased office space in Chapel Hill, North Carolina and Denmark. We believe that our facilities are adequate for our current needs.

ITEM 3. LEGAL PROCEEDINGS

From time to time, we may be involved in routine legal proceedings, as well as demands, claims and threatened litigation, which arise in the normal course of our business. We believe there is no litigation pending that would reasonably be expected to, individually or in the aggregate, have a material adverse effect on our results of operations or financial condition.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

INFORMATION ABOUT OUR EXECUTIVE OFFICERS AND DIRECTORS

The following table sets forth information regarding our executive officers and directors as of the date of this Annual Report on Form 10-K.

Name	Age	Position
Executive Officers		
Richard W. Pascoe	59	Chief Executive Officer and Director
Travis C. Mickle, Ph.D.	50	President and Director(1)
Sven Guenther, Ph.D.	51	Chief Scientific Officer
R. LaDuane Clifton, CPA	50	Chief Financial Officer, Secretary and Treasurer
Joshua Schafer	52	Chief Commercial Officer and Executive Vice President of Business Development
Christal M.M. Mickle, M.A.	44	Chief Product Development Officer
Non-Employee Directors		
Tamara A. Favorito	64	Director
Matthew R. Plooster	41	Director
Joseph B. Saluri	56	Director
David S. Tierney, M.D.	59	Director
Christopher Posner	53	Director

(1) On January 6, 2023, Travis C. Mickle, Ph.D. resigned as a Class I director and as president of the Company, in each case effective as of the date of the Company's annual meeting of stockholders in 2023.

Executive Officers

Richard W. Pascoe

Richard W. Pascoe has served as a director of our Company since January 2014 and our Company's Chief Executive Officer since January 2023. He served as our Company's executive chairman from November 2021 to January 2023. From January 2019 to November 2021, Mr. Pascoe served as the president and chief executive officer and on the board of directors of Histogen Inc., a biologics company. From March 2013 to January 2019, Mr. Pascoe was the chief executive officer and director of Apricus Biosciences. From August 2008 to March 2013, Mr. Pascoe was the president and chief executive officer and a director of Pernix Sleep, Inc. (formerly known as Somaxon Pharmaceuticals, Inc.), a specialty pharmaceutical company. Prior to Pernix, from 2005 to 2008, Mr. Pascoe worked for ARIAD Pharmaceuticals, Inc., a specialty pharmaceutical company, where he was most recently senior vice president and chief operating officer. Mr. Pascoe also serves as a director of Seelos Therapeutics, Inc, a specialty pharmaceutical company. Mr. Pascoe received his B.S. degree from the United States Military Academy at West Point. Our Board believes that Mr. Pascoe's experience as a chief executive officer at multiple public biotechnology and pharmaceutical companies provides him with the qualifications and skills to serve as a director of our Company. Mr. Pascoe has deep experience in drug development and public company management and leadership, which provides invaluable insight to the Board on long-term strategic planning and execution.

Travis C. Mickle, Ph.D.

Travis C. Mickle, Ph.D. is a co-founder of our Company, the Company's President and has served as a member of our Board since our inception in 2006. He previously served as chairman of our Board from November 2014 to November 2021. Dr. Mickle has served as our president since 2006, as our chief scientific officer from 2006 to October 2010, and as our chief executive officer from October 2010 to January 2023. Prior to founding our Company, Dr. Mickle spent five years with New River Pharmaceuticals, a specialty pharmaceutical company, where he was a senior research scientist from 2001 to 2002, the director of chemistry from 2002 to 2003 and the director of drug discovery and CMC from 2003 to 2005. Dr. Mickle received his Ph.D. degree from the University of Iowa and his B.A. degree from Simpson College. Our Board believes that Dr. Mickle's leadership of our company since its inception, knowledge of our company as founder and experience with pharmaceutical companies provides him with the qualifications and skills to serve as a director of our company. On January 6, 2023, Dr. Mickle resigned as a Class I director and as President effective as of the date of the Annual Meeting and thereafter, Dr. Mickle intends to continue to support the Company as a consultant.

Sven Guenther, Ph.D.

Sven Guenther, Ph.D. has served as our Chief Scientific Officer since January 2023. Dr. Guenther was one of the first members of Zevra, joined our Company as our group leader of research in 2007, and served as our Executive Vice President, Research and Development, from May 2014 to January 2023. In this role, he was a key contributor to the strategy and execution of all of Zevra's early discovery work, as well as, the development and approval of three NDAs. As Chief Scientific Officer, he continues to lead Zevra's research team and play a central role in the advancement of the Company's pipeline. Dr. Guenther previously served as a Research Scientist for New River Pharmaceuticals, where he was part of the development team for Vyvanse®. He earned his Ph.D. from the University of Iowa and is listed as an inventor on numerous patents, as well as an author of several research papers.

R. LaDuane Clifton, CPA

R. LaDuane Clifton, CPA, has served as our Chief Financial Officer since June 2015 and as our secretary and treasurer since February 2016. Previously, Mr. Clifton served as our vice president of finance and corporate controller from April 2015 to June 2015. Prior to joining our company, Mr. Clifton served in a variety of positions with The LGL Group, Inc., a publicly held producer of industrial and commercial products and services, from August 2009 to February 2015, including chief financial officer, secretary and treasurer from December 2012 to February 2015, chief accounting officer and secretary from March 2010 to December 2012, and corporate controller from August 2009 to March 2010. From August 2008 to August 2009, Mr. Clifton served as the chief financial officer of a21, Inc., a publicly held holding company with businesses in stock photography and the online retail and manufacturer of framed art, and as its corporate controller from March 2007 to August 2008. Mr. Clifton served in a variety of finance and medical cost analysis roles with Aetna, Inc., a publicly held provider of healthcare benefits, from August 1991 to August 2004. Mr. Clifton was an auditor with KPMG, LLP from August 2004 to March 2007. Mr. Clifton received his B.B.A. and M.B.A. degrees from the University of North Florida and is a certified public accountant in the state of Florida.

Joshua Schafer

Joshua Schafer has served as our Chief Commercial Officer and Executive Vice President since January 2023. Mr. Schafer brings to Zevra over 25 years of pharmaceutical commercial, new product development and merger and acquisition ("M&A") experience. Mr. Schafer previously served as Senior Vice President and General Manager of the Autoimmune and Rare Disease business at Mallinckrodt Pharmaceuticals from December 2020 to November 2022. Prior to that at Mallinckrodt Pharmaceuticals, he served as Chief Strategy and Business Officer from September 2019 to December 2020 and Senior Vice President of Business Development and General Manager from January 2018 to September 2019. Prior to Mallinckrodt, Mr. Schafer served as Vice President and Oncology Therapeutic Area Head, Global Marketing and Strategy at Astellas Pharmaceuticals, where he was responsible for building the company's global oncology franchise, and also held senior roles at Takeda Pharmaceuticals, Accenture (formerly Anderson Consulting), G. D. Searle & Co. (later acquired by Pfizer) and Cognia Corporation. During his professional career, he has successfully led over $16 billion in aggregate M&A transactions. Mr. Schafer currently serves as a board member of Pharnext SA and Shuttle Pharmaceuticals. He received his B.A. in Biology and German at the University of Notre Dame, and both an M.S. in Biotechnology and an M.B.A. from Northwestern University.

Christal M.M. Mickle, M.A.

Christal M.M. Mickle, M.A. has served as our Chief Product Development Officer since January 2023. Ms. Mickle, who co-founded and has held a variety of positions at Zevra, most recently served as Senior Vice President, Operations and Product Development from June 2022 to January 2023. In this role, she managed the development of each of Zevra's products through strategic collaborations across the various drug development disciplines including clinical, regulatory, nonclinical, and manufacturing, enabling efficient use of funds and the ability to meet timelines and milestones. From January 2018 through June 2022, Ms. Mickle served as Zevra's Vice President, Product Development and Operations. Before founding Zevra in 2006, Ms. Mickle started her career as a Research Associate for New River Pharmaceuticals, preparing compounds in ADHD, pain, and thyroid dysfunctions for further study. Throughout her more than 20 years in the pharmaceutical industry, Ms. Mickle has been involved in early discovery as a medicinal chemist, starting and helping build a pharmaceutical company, and interacting with the FDA. In addition, her efforts managing a team of talented scientists has led to the approval of three NDAs. Ms. Mickle received her M.A. degree in Medicinal Chemistry from the University of Virginia and her B.A. and B.S. degrees in Chemistry and Biochemistry, respectively, from Virginia Polytechnic Institute and State University. She is also listed as an inventor on several patents.

Directors

Tamara A. Favorito (formerly Tamara A. Seymour)

Tamara A. Favorito has served as a director of our Company since August 2021. Ms. Favorito has more than 30 years of life sciences industry experience including 20 years as a chief financial officer. She currently serves as a board member and audit committee chair of Artelo Biosciences, Inc. and Kintara Therapeutics, Inc., both publicly-traded clinical-development stage companies. Ms. Favorito served on the board of directors of Beacon Discovery, Inc. from 2018 until their acquisition in March 2021. Ms. Favorito was interim CFO of Immunic, Inc., a publicly-traded clinical-stage drug development company in 2019. She served as CFO of Signal Genetics, Inc. (now Viridian Therapeutics, Inc.), a publicly-traded molecular diagnostics company, from 2014 to 2017, HemaQuest Pharmaceuticals, Inc., a venture-backed clinical-stage drug development company, from 2010 to 2014 and Favrille, Inc. (now MMR Global, Inc.), a publicly-traded clinical-stage drug development company, from 2001 to 2009. Earlier in her career, she spent eight years in public accounting with Deloitte & Touche LLP and PricewaterhouseCoopers LLP. Ms. Favorito is a Certified Public Accountant (inactive). She received an MBA, emphasis in Finance, from Georgia State University, and a BBA, emphasis in Accounting, from Valdosta State University. Our Board believes that Ms. Favorito's deep experience in corporate management, finance and life science as chief financial officer of multiple public companies provides her with the qualifications and skills to serve as a director of our Company. Her extensive experience includes leading multiple private and public financings and M&A transactions as well as leading the finance, investor relations, human resources, administration and managed care functions.

Matthew R. Plooster

Matthew R. Plooster has served as a director of our Company since March 2011 and the Chairman of our Board since January 2023. Mr. Plooster co-founded Bridgepoint Investment Banking, a division of Bridgepoint Holdings, LLC and Bridgepoint Holdings NE, LLC, where he has served as Managing Director since March 2012. Previously, Mr. Plooster worked as an investment banker at DeWaay Investment Banking from October 2010 to March 2012, Morgan Stanley from August 2009 to November 2009 and Deutsche Bank from 2004 to July 2009. Mr. Plooster received his Certificate in Business Excellence from Columbia Business School and his B.A. degree from the University of Chicago. Our Board believes that Mr. Plooster’s experience as an investor in and transaction experience with healthcare companies provides him with the qualifications and skills to serve as a director of our Company. Mr. Plooster has deep experience and expertise in capital raising, capital allocation, strategy and mergers and acquisitions.

Joseph B. Saluri

Joseph B. Saluri has served as a director of our Company since January 2014. Since August 2018, Mr. Saluri has served as the chief executive officer and chairman of the board of BlueAllele, LLC, a start-up biotechnology company located in Oakdale, Minnesota. Mr. Saluri previously served as general counsel and executive vice president for Calyxt, Inc. from June 2017 to March 2018. Prior to his employment with Calyxt, Inc., Mr. Saluri served as general counsel for Stine Seed Company and its affiliates from July 1999 to March 2017. Mr. Saluri practiced as an attorney and solicitor at law with Nicholas Critelli Associates, PC, in Des Moines, Iowa and London, England from June 1993 to June 1999. Mr. Saluri served as a director of Newlink Genetics Corporation, a public biopharmaceutical company from May 2010 to July 2017. Mr. Saluri received his J.D. degree from Drake University Law School and his B.S.B.A. degree from Drake University Law School and his B.S.B.A. degree from Drake University. Our Board believes that Mr. Saluri’s extensive legal background and experience in corporate management, governance, finance and investor relations provides him with the qualifications and skills to serve as a director of our Company.

David S. Tierney, M.D.

David S. Tierney, M.D. has served as a director of our Company since March 2015. Since December 2020, Dr. Tierney has served as president and chief executive officer of Aramis Biosciences, a clinical-stage immuno-ophthalmology biopharmaceutical company. From February 2020 until December 2020, Dr. Tierney served as chief executive officer of Pharma Two B, Ltd., a privately held company developing innovative therapeutics based on previously approved drugs for Parkinson disease. From September 2019 until January 2020, Dr. Tierney served as president and chief executive officer of BiopharmX, a dermatology drug development company. From March 2014 until March 2018, Dr. Tierney was president and chief executive officer of Icon Bioscience, Inc., a privately held ophthalmology company, which was merged into Psivida, Inc to form Eyepoint Pharmaceuticals. From January 2013 until March 2014, Dr. Tierney was a venture partner at Signet Healthcare Partners, a New York City based life science private equity fund. Dr. Tierney served as president and chief operating officer and as a member of the board of directors of Oceana Therapeutics, Inc., a private specialty pharmaceutical company, from its organization in 2008 through its sale to Salix Pharmaceuticals, Ltd. in December 2011. Dr. Tierney served as the president and chief executive officer and as a member of the board of directors of Valera Pharmaceuticals, Inc., a specialty pharmaceutical company, between August 2000 and April 2007, when Valera completed a merger with lndevus Pharmaceuticals, Inc. From January 2000 to August 2000, Dr. Tierney served as president of Biovail Technologies, a division of Biovail Corporation, a Canadian drug delivery company. From March 1997 to January 2000, Dr. Tierney was senior vice president of drug development at Roberts Pharmaceutical Corporation and from December 1989 to March 1997, Dr. Tierney was employed by Elan Corporation, a pharmaceutical company, in a variety of management positions. Dr. Tierney is also currently a director of Catalyst Pharmaceuticals, Inc. and previously served as a director of BioPharmX from 2019 to 2020. Dr. Tierney received his medical degree from the Royal College of Surgeons in Dublin, Ireland and was subsequently trained in internal medicine. Our Board believes that Dr. Tierney's extensive experience as chief executive officer at multiple pharmaceutical companies provide him with the qualifications and skills to serve as a director of our Company. Combined with his background in drug development and his medical and scientific expertise, Dr. Tierney’s experience brings impactful perspectives to the Board.

Christopher A. Posner

Christopher A. Posner has served as a director of our Company since November 2022. Mr. Posner has served as the President and Chief Executive Officer of Cara Therapeutics, Inc. since November 2021 and has served as a member of its board since August 2018. He has broad experience in commercial and marketing operations and product management at both large and specialty pharmaceutical companies, where he has focused on products for autoimmune, inflammatory and pain conditions, including XELJANZ® and ENBREL®. From July 2017 to October 2021, he served as the Chief Executive Officer of LEO Pharma, Inc. US, a subsidiary of LEO Pharma A/S, a global healthcare company specializing in dermatology and critical care, including such conditions as psoriasis and atopic dermatitis. Prior to joining LEO, he was the head of worldwide commercial operations at R-Pharma-US, LLC, a specialty pharmaceutical company focused on oncology and chronic immune disorders, from 2014 until 2017. Previously, Mr. Posner held a variety of senior management positions in commercial and marketing operations at Bristol-Myers Squibb Company, Pfizer Inc., Wyeth Pharmaceuticals, Inc. and Endo International plc. Mr. Posner holds an M.B.A. from Fuqua School of Business, Duke University and a B.A. in Economics from Villanova University. Our Board believes that Mr. Posner’s extensive experience in global pharmaceutical management, sales and products provides him with the qualifications and skills to serve as a director of our Company. His deep experience in commercial and marketing operations and product management provides a critical perspective to the Board as the Company executes on its diverse product portfolio.

PART II

ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS, AND ISSUER PURCHASES OF EQUITY SECURITIES

Common Stock Listing

On March 1, 2023, our common stock began trading on The Nasdaq Global Select Market under the ticker symbol "ZVRA". Prior to that, on October 19, 2021, our common stock began trading on The Nasdaq Global Select Market under the ticker symbol "KMPH". Prior to that date, our common stock was listed on the Nasdaq Capital Market under the ticker symbol "KMPH".

Holders of our Common Stock

As of December 31, 2022, there were approximately 98 holders of record of our common stock. The actual number of stockholders is greater than this number of record holders, and includes stockholders who are beneficial owners, but whose shares are held in street name by brokers and other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

Dividend Policy

We have never declared or paid any cash dividends on our common stock. We anticipate that we will retain all of our future earnings, if any, for use in the operation and expansion of our business and do not anticipate paying cash dividends in the foreseeable future.

Securities Authorized for Issuance under Equity Compensation Plans

The information regarding securities authorized for issuance under equity compensation plans is included in Part III of this report.

Recent Sales of Unregistered Securities

None.

Purchases of Equity Securities By the Issuer and Affiliated Purchasers

None.

ITEM 6. [Reserved]

ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with our financial statements and related notes thereto included elsewhere in this Annual Report on Form 10-K. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report on Form 10-K, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this Annual Report on Form 10-K, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a rare disease company melding science, data and patient need to create transformational therapies for diseases with limited or no treatment options. With unique, data-driven clinical, regulatory, and commercialization strategies, we are overcoming complex drug development challenges to bring much-needed therapies to patients. We have a diverse portfolio of products and product candidates, which includes a combination of both a clinical stage pipeline and commercial stage assets. The Company's pipeline includes arimoclomol, an orally-delivered, first in-class investigational product candidate being developed for Niemann-Pick disease Type C, or NPC, which has been granted orphan drug designation, Fast-Track designation, Breakthrough Therapy designation and rare pediatric disease designation for the treatment of NPC by the U.S. Food and Drug Administration, or FDA, and orphan medicinal product designation for the treatment of NPC by the European Medicines Agency, or EMA. KP1077 is our lead clinical development product candidate which is being developed as a treatment for idiopathic hypersomnia, or IH, a rare neurological sleep disorder, and narcolepsy. KP1077 is comprised solely of serdexmethylphenidate, or SDX, our proprietary prodrug of d-methylphenidate, or d-MPH. The FDA has granted KP1077 orphan drug designation for the treatment of IH, and the U.S. Drug Enforcement Agency, or DEA, has classified SDX as a Schedule IV controlled substance based on evidence suggesting SDX has a lower potential for abuse when compared to d-MPH, a Schedule II controlled substance.

We have specialized expertise and a track record of success in advancing promising therapies that face complex clinical and regulatory challenges with an approach that balances science and data with patient need. The FDA has approved AZSTARYS®, formerly referred to as KP415, a once-daily treatment for attention deficit hyperactivity disorder, or ADHD, in patients age six years and older containing our prodrug, SDX, and d-MPH. In September 2019, we entered into a collaboration and license agreement, or the AZSTARYS License Agreement, with Commave Therapeutics S.A. (formerly known as Boston Pharmaceutical S.A.), or Commave, an affiliate of Gurnet Point Capital, L.P. Under the AZSTARYS License Agreement, we granted to Commave an exclusive, worldwide license, to develop, manufacture, and commercialize AZSTARYS and any of our product candidates containing SDX and used to treat ADHD or any other CNS disease. Commave has tasked Corium, Inc., or Corium, an affiliate of Gurnet Point Capital, L.P., to lead all commercialization activities for AZSTARYS in the U.S. under the AZSTARYS License Agreement. Corium commercially launched AZSTARYS in the U.S. during the third quarter of 2021. In December 2021, Commave sublicensed commercialization rights for AZSTARYS in greater China to Shanghai Ark Biopharmaceutical Ltd. The FDA has also approved APADAZ®, an immediate-release combination product containing benzhydrocodone, our prodrug of hydrocodone, and acetaminophen, for the short-term (no more than 14 days) management of acute pain severe enough to require opioid analgesic and for which alternative treatments are inadequate. In October 2018, we entered into a collaboration and license agreement , or the APADAZ License Agreement, with KVK-Tech, Inc., or KVK, under which we granted to KVK the exclusive license to manufacture and commercialize APADAZ in the U.S.

Our primary mission is to deliver life-changing treatments to people with rare conditions, their families, and caregivers who desperately need better options. This mission guides our efforts to expand our pipeline through both internal development and through our business development activities to collaborate, partner, and potentially acquire additional assets. We intend to target assets that will allow us to leverage the expertise and infrastructure that we have successfully built in order to mitigate risk and enhance our probability of success. In addition, we are considering external opportunities within neurology and neurodegenerative diseases, psychiatric disorders, and other rare diseases, along with adjacent or related therapeutic categories. We are seeking assets that are undergoing Phase 2 clinical trials or Phase 3 clinical trials, subject to our specific evaluation criteria, that we can in-license or acquire. If we are successful, expanding our development pipeline could be accretive to our value proposition by potentially adding new clinical data catalysts and have the potential to create incremental long-term value for stockholders. In addition, we believe that a multi-channel development program with several product candidates addressing various CNS/rare disease indications will diversify risk and potentially create an impactful portfolio of commercial-stage products in the future.

For example, in May 2022, we, through our newly formed wholly-owned subsidiary, Zevra Denmark A/S (formerly known as KemPharm Denmark A/S prior to February 21, 2023, or Zevra DK, entered into an Asset Purchase Agreement, or the Arimoclomol Purchase Agreement, with Orphazyme A/S in restructuring, a Danish public limited liability company, or Orphazyme. The transactions agreed to under the Arimoclomol Purchase Agreement closed on May 31, 2022. Under the terms of the Arimoclomol Purchase Agreement, Zevra DK purchased all of the assets and operations of Orphazyme related to arimoclomol and settled all of Orphazyme's actual outstanding liabilities to its creditors with a cash payment of $12.8 million. In addition, Zevra DK agreed to assume an estimated reserve liability of $5.2 million related to revenue generated from Orphazyme's Early Access Program in France, or the Arimoclomol EAP.

Our most advanced product candidate, arimoclomol, is being developed for the treatment of NPC, a lysosomal storage disease, or LSD. NPC is a rare neurodegenerative disease characterized by an inability of the body to transport cholesterol and lipids inside of cells. Symptoms of NPC include a progressive impairment of mobility, cognition, speech, and swallowing, often culminating in premature death. The incidence of NPC is estimated to be one in 100,000 live births. We estimate that approximately 1,500 individuals have been diagnosed, of which approximately 300 are in the United States and approximately 1,200 are in Europe. However, diagnostic challenges may affect the number of potential patients, and we believe that the availability of treatment options could increase awareness of the disease and assist in identifying more cases. Therapies to treat NPC are desperately needed, and for this reason, arimoclomol is currently being made available to NPC patients in the United States, France, Germany, and other European Union countries under various early access programs, or EAPs.

On September 16, 2020, the previous sponsor of the arimoclomol program, Orphazyme, submitted a new drug application, or NDA, seeking approval for arimoclomol to treat NPC. In June 2021, the FDA issued a complete response letter, or CRL, which means the FDA determined that it could not approve the NDA in its present form. Our aim is to prepare and resubmit an NDA that presents meaningful evidence of safety and efficacy of arimoclomol for its intended use. To that end, we are continuing to work diligently to characterize the substantial data generated since the CRL, including the recently completed four-year open-label safety trial which was recently presented at the 19th World*Symposium*™ in February 2023. Results from this analysis, based on up to four years of continuous treatment, suggest that arimoclomol may reduce the long-term progression of NPC. Upon fulfilling the randomized double-blinded portion of the phase 2/3 clinical trial, both placebo- and arimoclomol-treated patients were given the option to continue into the four-year (48 month) open-label-extension, or OLE, phase of the study with arimoclomol treatment provided in addition to their current standard of care. Progression of NPC disease through the DB and OLE phases was assessed utilizing the five-domain NPC Clinical Severity Scale (5DNPCCSS) and compared with an estimated progression calculated from the combination of untreated patients from the NPC-001 observational trial and placebo patients from the NPC-002 Phase 2/3 trial. We are also investigating correlations between relevant 5DNPCCSS domains and corresponding Scale for the Assessment and Rating of Ataxia, or SARA, test items to potentially provide further supportive evidence for 5DNPCCSS validity as a tool for evaluating NPC progression. The SARA test evaluates impairment related to

cerebellar ataxia, which was a secondary endpoint in the Phase 2/3 clinical trial of arimoclomol in NPC (NPC progression based on the 5DNPCCSS was the primary endpoint). Based on a comparative analysis of both measurements, it was determined that individual 5DNPCCSS domains and relevant performance-based SARA test items showed strong associations and alignment between the two instruments for all analysis methods used. These results provide further support that the evaluated 5DNPCCSS domains are appropriately standardized to allow for reliable and reproducible scoring of disease severity in NPC. We plan to include these data as part of the updated NDA for arimoclomol, which is expected to be resubmitted to the FDA as early as the third quarter of 2023.

We also intend to advance our pipeline of prodrug product candidates for the treatment of IH and other CNS/rare diseases, and we reported top-line data from a Phase 1 proof-of-concept study of SDX in the fourth quarter of 2021 and final data for the Phase 1 proof-of-concept study of SDX in the first quarter of 2022. The proof-of-concept study was a dose-escalation study to evaluate the pharmacokinetics, pharmacodynamic stimulant effects, and safety of single oral doses of SDX in subjects with a history of high-dose stimulant use. In the trial, 240 mg and 360 mg doses of SDX were observed to be well-tolerated and produced d-MPH exposure that appeared to increase proportionally with dose. Mean d-MPH plasma concentrations showed a gradual increase after SDX administration, reaching a broad peak from eight to twelve hours post-dose, followed by a shallow decline thereafter. Increased wakefulness, alertness, hypervigilance, and insomnia effects were reported by study participants, which we believe suggests that SDX produced targeted pharmacodynamic effects that have the potential to benefit patients with IH and other sleep disorders. On November 18, 2022, we announced that the FDA has granted the Orphan Drug Designation to SDX for the treatment of IH.

In January 2022, we announced that we have selected KP1077 for the treatment of IH and narcolepsy as our lead clinical development candidate. KP1077 utilizes SDX, our prodrug of d-MPH, as its active pharmaceutical ingredient. During the first quarter of 2022, we initiated a Phase 1 clinical trial comparing the cardiovascular safety of SDX to immediate-release and long-acting formulations of RITALIN®, a commonly prescribed CNS stimulant. In September 2022, we announced topline data from our exploratory Phase 1 clinical trial, which showed the potential for higher dose formulations of SDX to be safe and well tolerated while avoiding the potential for greater cardiovascular safety risk compared to immediate-release and long-acting formulations of Ritalin. Based on the data, we have identified initial dosing strengths for the planned Phase 2 clinical trial of KP1077 which we believe have the potential to be well-tolerated while providing higher overall exposures to d-MPH compared to other methylphenidate products that are often used off-label as a treatment for IH. In addition, on December 21, 2022, we announced the initiation of a Phase 2 clinical trial evaluating KP1077. The Phase 2 clinical trial is a double-blind, placebo-controlled, randomized-withdrawal, dose-optimizing, multi-center study evaluating the efficacy and safety of KP1077 for the treatment of IH. We expect to enroll approximately 48 adult patients with IH in more than 30 centers in the United States. Part 1 of the trial will consist of a five-week open-label titration phase during which patients will be optimized to one of four doses of SDX (80, 160, 240, or 320 mg/day). Part 2 of the trial will entail a two-week randomized, double-blind, withdrawal phase, during which two-thirds of the trial participants will continue to receive their optimized dose while the remaining one-third will receive placebo. Participants will be further assigned into two evenly divided cohorts. The first cohort will receive a single daily dose just before bedtime, and the second cohort will receive half the daily dose shortly after awakening and half the daily dose prior to bedtime. Interim efficacy and safety data for the Phase 2 trial is expected as early as the third quarter of 2023. We anticipate that we will submit the IND for KP1077 for the treatment of narcolepsy as early as the second quarter of 2023, and subsequently begin a trial in narcolepsy soon after interim data from the Phase 2 IH clinical trial is available.

In May 2021, we announced that SDX, our proprietary prodrug of d-MPH and the primary active pharmaceutical ingredient, or API, in AZSTARYS, was classified as a Schedule IV controlled substance by the DEA. AZSTARYS is classified as a Schedule II controlled substance as its formulation includes a 70:30 mixture of SDX (Schedule IV) and d-MPH (Schedule II), respectively.

In March 2021, we announced that the FDA approved the NDA for AZSTARYS, a once-daily product for the treatment of ADHD in patients ranging from six years and older. Commave has licensed the commercial rights for AZSTARYS as provided by the AZSTARYS License Agreement and has tasked Corium with leading all commercialization activities for AZSTARYS in the United States. Corium commercially launched AZSTARYS in the United States during the third quarter of 2021. In December 2021, Commave sublicensed commercialization rights for AZSTARYS in greater China to Shanghai Ark Biopharmaceutical Ltd.

We employ our proprietary Ligand Activated Therapy, or LAT ®, platform technology to discover and develop prodrugs that are new molecules that may potentially improve one or more of the attributes of approved drugs, such as enhanced bioavailability, extended duration of action, increased safety and reduced susceptibility to abuse. A prodrug is a precursor chemical compound of a drug that is inactive or less than fully active, which is then converted in the body to the active form of the drug through a normal metabolic process. Where possible, we seek, to develop prodrugs that will be eligible for approval under Section 505(b)(2) of the FFDCA, otherwise known as a 505(b)(2) NDA, which allows us to submit an NDA that relies on the FDA's previous findings of safety and effectiveness for one or more approved products, if we demonstrate such reliance is scientifically appropriate.

We continue to seek opportunities to expand our pipeline of product candidates through our business development efforts, and by employing our LAT platform technology and development expertise to develop additional product candidates that address significant unmet medical needs in therapeutic indications which have few existing product options. We believe our prodrug product candidates may be eligible for composition-of-matter patent protection and we intend to use the 505(b)(2) NDA pathway when available, which we believe has the potential to reduce drug development time and expense.

We have historically had minimal positive net cash flows from operations. Our cash flows (used in) provided by operations for the years ended December 31, 2022, and 2021, were $(18.7) million and $10.4 million, respectively. However, the positive net cash flows from operations for the year ended December 31, 2021, were primarily due to an adjustment to reconcile net loss to net cash used in operations of approximately $16.1 million related to a loss on extinguishment of debt during the period.

We expect to continue to incur significant expenses and minimal positive net cash flows from operations or negative net cash flows from operations for the foreseeable future, and those expenses and losses may fluctuate significantly from quarter-to-quarter and year-to-year. We anticipate that our expenses will fluctuate substantially as we:

- continue our ongoing preclinical studies, clinical trials and our product development activities for our pipeline of product candidates;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- continue research and preclinical development and initiate clinical trials of our product candidates;
- seek to discover and develop additional product candidates either internally or in partnership with other pharmaceutical companies;
- adapt our regulatory compliance efforts to incorporate requirements applicable to marketed products;
- maintain, expand and protect our intellectual property portfolio; and
- incur additional legal, accounting and other expenses in operating as a public company.

Recent Financial Developments

In January and February 2021, we undertook a series of transactions to regain our Nasdaq Capital Market listing and improve our balance sheet. These included a reverse stock split, an underwritten public offering, or the Public Offering, a restructuring of our debt and ultimately a payoff of all remaining debt and a transaction in which we induced the holders of some of the warrants we issued in the public offering to exercise those warrants for cash.

Reverse Stock Split and Nasdaq Relisting

Effective May 21, 2020, our common stock had been delisted from the Nasdaq Capital Market and we began listing on the OTC Markets Venture Market under the symbol "KMPH". In anticipation of the Public Offering, we determined we would need to regain our Nasdaq listing.

In order to relist our common stock on the Nasdaq Capital Market, our common stock was required to satisfy the initial listing standards of the Nasdaq Capital Market., which included among others, that we have stockholders' equity of at least $5.0 million, a market value of unrestricted publicly held shares of at least $15.0 million, at least 1.0 million unrestricted publicly held shares, at least 300 unrestricted round lot stockholders, at least three market makers and a bid price of at least $4.00 per share, or, collectively, the Nasdaq Listing Requirements. Accordingly, on December 23, 2020, we effectuated a 1-for-16 reverse stock split of our common stock in order to satisfy the bid price component of the Nasdaq Listing Requirements.

On January 7, 2021, our common stock was approved for listing on the Nasdaq Capital Market. Our common stock began trading on the Nasdaq Capital Market on January 8, 2021, under the ticker symbol "KMPH".

On October 19, 2021, our common stock was approved for listing on the Nasdaq Global Select Market. Our common stock began trading on the Nasdaq Global Select Market on that date, under the ticker symbol "KMPH". On March 1, 2023, our common stock began trading on The Nasdaq Global Select Market under the ticker symbol "ZVRA".

Debt Restructuring

In anticipation of the Public Offering, and to meet the Nasdaq Listing Requirements, we agreed in December 2020 to restructure our outstanding senior secured convertible notes issued in December 2019 and January 2020, or the Senior Secured Notes, in the aggregate principal amount of $60.8 million and the senior secured convertible promissory note held by Deerfield Private Design Fund III, L.P., or Deerfield, in the principal amount of $7.5 million, or the Deerfield Note, and, collectively the Senior Secured Notes, the Facility Notes. The total outstanding principal and accrued interest under the Facility Notes was $69.4 million as of December 31, 2020.

Under the terms of the agreement we entered into with our lenders in connection with this debt restructuring, which we refer to as the December 2020 Exchange Agreement, on January 12, 2021, in connection with the closing of our Public Offering, we:

- exchanged $31.5 million of the outstanding principal and accrued interest on the Facility Notes for (i) 31,476.98412 shares of our Series B-2 convertible preferred stock, and (ii) warrants exercisable for 3,632,019 shares of our common stock, or the Exchange Warrants; and
- made a payment of $30.3 million, or the Debt Payment, in partial repayment of the remaining outstanding principal and accrued interest on the Facility Notes.

Each share of Series B-2 convertible preferred stock issued pursuant to the December 2020 Exchange Agreement has a stated value of $1,000 and is convertible into shares of our common stock at any time, subject to specified limits. The shares of Series B-2 convertible preferred stock issued under the December 2020 Exchange Agreement are convertible into an aggregate of 4,842,690 shares of our common stock.

The Exchange Warrants have substantially the same terms and conditions as the warrants sold by us in our January 2021 underwritten offering.

Following the completion of these transactions, the aggregate balance of principal and accrued interest remaining outstanding under the Facility Notes was approximately $7.6 million. With respect to this remaining outstanding balance under the Facility Notes, the December 2020 Exchange Agreement amended the terms of that debt to provide that:

- the maturity date was changed to March 31, 2023, and the debt is prepayable upon specified conditions; and
- interest would accrue at the rate of 6.75% per annum, payable quarterly, would be added to principal until June 30, 2021, and then be payable in cash thereafter.

In connection with the closing of the transactions contemplated under the December 2020 Exchange Agreement, we filed an amended and restated certificate of designation with the Secretary of State of Delaware in order to modify the terms of our authorized, but unissued, shares of Series B-2 convertible preferred stock to reflect those contemplated under the December 2020 Exchange Agreement.

Public Offering

On January 8, 2021, we issued and sold 6,765,463 shares of our common stock, pre-funded warrants to purchase 926,844 shares of our common stock and warrants to purchase 7,692,307 shares of our common stock in the Public Offering. In addition, we granted the underwriter for the Public Offering an option to purchase, for a period of 45 days, up to an additional 1,153,846 shares of our common stock and/or warrants to purchase up to an additional 1,153,846 shares of our common stock. On January 8, 2021, the underwriter exercised its over-allotment option, in part, for warrants to purchase 754,035 shares of our common stock. On January 12, 2021, we closed the Public Offering. Further, on February 1, 2021, the underwriter again exercised its over-allotment option, in part, to purchase 374,035 shares of our common stock. On February 3, 2021, we closed the underwriter's partial exercise of its over-allotment option.

The aggregate gross proceeds to us from the Public Offering, including from the exercises by the underwriter of its over-allotment option, totaled $52.4 million, before deducting underwriting discounts and commissions and offering expenses payable by us.

On January 25, 2022, we filed an amendment to the registration statement on Form S-1 (File No. 333-250945) on Form S-3 covering the issuance of the shares of our common stock issuable upon the exercise of the warrants issued in the Public Offering and remaining unexercised as of the date of the amendment, which was declared effective on February 1, 2022.

December 2020 Exchange Agreement Amendment

On January 12, 2021, in connection with the transactions contemplated by the December 2020 Exchange Agreement, we entered into an Amendment to Senior Secured Convertible Notes and Amendment to Warrant, or the January 2021 Amendment, with Deerfield and Deerfield Special Situations Fund, LP, collectively the Deerfield Holders. The January 2021 Amendment modified certain specified terms of (i) the Facility Notes and (ii) the warrant held, issued on June 2, 2014, to, among other things, exclude the transactions contemplated by the December 2020 Exchange Agreement and issuance of securities pursuant to the Underwriting Agreement from the anti-dilution provisions of the Facility Notes and the warrant. Deerfield assigned the warrant, or the OTA Warrant, to OTA LLC, or OTA, in December 2022.

January 2021 Warrant Exercise Inducement Letters and Issuance of Warrants

On January 26, 2021, we entered into warrant exercise inducement offer letters, or the January 2021 Inducement Letters, with certain holders of warrants issued in the Public Offering, or the Existing Warrants, and collectively, the Exercising Holders, pursuant to which such holders exercised for cash their Existing Warrants to purchase 6,620,358 shares of our common stock in exchange for new warrants, or the January 2021 Inducement Warrants, on substantially the same terms as the Existing Warrants, except as set forth in the following sentence, to purchase up to 7,944,430 shares of our common stock, which was equal to 120% of the number of shares of our common stock issued upon exercise of the Existing Warrants, or the January 2021 Inducement Transaction. The purchase price of the January 2021 Inducement Warrants was $0.125 per share underlying each January 2021 Inducement Warrant, and the January 2021 Inducement Warrants have an exercise price of $6.36 per share. We received aggregate gross proceeds of $44.0 million from the exercise of the Existing Warrants by the Exercising Holders and the sale of the January 2021 Inducement Warrants.

We also filed a registration statement on Form S-3 covering the resale of the shares of our common stock issued or issuable upon the exercise of the January 2021 Inducement Warrants which was declared effective on February 16, 2021.

Payoff of Facility Agreement Notes and Termination of Facility Agreement

On February 8, 2021, we entered into a payoff letter with the Facility Note holders, pursuant to which we agreed to pay off and thereby terminate the Facility Agreement.

Pursuant to the payoff letter, we paid a total of $8.0 million to the Facility Note holders, representing the principal balance, accrued interest outstanding and a prepayment fee in repayment of our outstanding obligations under the Facility Agreement.

Pursuant to the payoff letter, all outstanding indebtedness and obligations owed by us to the Facility Note holders under the Facility Agreement have been paid in full. The Facility Agreement and the notes thereunder

June 2021 Warrant Exercise Inducement Letters and Issuance of Warrants

On June 18, 2021, we entered into warrant exercise inducement offer letters, or the June 2021 Inducement Letters, with certain holders of the January 2021 Inducement Warrants, pursuant to which such holders exercised for cash their January 2021 Inducement Warrants to purchase 6,117,509 shares of our common stock in exchange for new warrants, or the June 2021 Inducement Warrants, on substantially the same terms as the January 2021 Inducement Warrants, except as set forth in the following sentence, to purchase up to 1,529,379 shares of our common stock, which was equal to 25% of the number of shares of our common stock issued upon exercise of the January 2021 Inducement Warrants, or the June 2021 Inducement Transaction. The purchase price of the June 2021 Inducement Warrants was $0.125 per share underlying each June 2021 Inducement Warrant, and the June 2021 Inducement Warrants have an exercise price of $16.50 per share and expire on December 31, 2026. We received aggregate gross proceeds of $39.1 million from the exercise of the January 2021 Inducement Warrants and the sale of the June 2021 Inducement Warrants.

We also filed a registration statement on Form S-3 covering the resale of the shares of our common stock issued or issuable upon the exercise of the June 2021 Inducement Warrants which was declared effective on July 7, 2021.

Equity Distribution Agreement

On July 2, 2021, we entered into an Equity Distribution Agreement with JMP and RBCCM, under which we may offer and sell, from time to time at our sole discretion, shares of our common stock having an aggregate offering price of up to $75.0 million through JMP and RBCCM as our sales agents. The issuance and sale, if any, of our common stock under the Equity Distribution Agreement will be made pursuant to a registration statement on Form S-3.

We filed a registration statement on Form S-3 covering the sale of the shares of our common stock up to $350.0 million, $75.0 million of which was allocated to the sales of the shares of common stock issuable under the Equity Distribution Agreement. The Form S-3 was declared effective on July 12, 2021. As of December 31, 2022, no shares have been issued or sold under the Equity Distribution Agreement.

Share Repurchase Program

On December 20, 2021, the Company initiated a share repurchase program, or the Share Repurchase Program, pursuant to which the Company may repurchase up to $50 million of shares of its common stock through December 31, 2023. Capital allocation to the Share Repurchase Program will be based on a variety of factors, including our business results, the receipt of royalties and sales milestones under the AZSTARYS License Agreement, and potentially other sources of non-dilutive capital that may become available to the Company. Repurchases will be made in compliance with Rule 10b-18 of the Securities Exchange Act of 1934, as amended, subject to a variety of factors, including the market price of the Company's common stock, general market and economic conditions and applicable legal requirements. The exact number of shares to be repurchased by the Company is not guaranteed and the program may be suspended, modified, or discontinued at any time without prior notice. As of December 31, 2022, the Company has repurchased 909,953 shares of its common stock for approximately $7.5 million under the Share Repurchase Program.

Third-Party Agreements

AZSTARYS License Agreement

See Part I, Item 1. "Business—AZSTARYS" of this Annual Report on Form 10-K for information regarding the AZSTARYS License Agreement.

APADAZ License Agreement

See Part I, Item 1. “Business—APADAZ” of this Annual Report on Form 10-K for information regarding the APADAZ License Agreement.

Other Third-Party Agreements

Under our March 2012 termination agreement with Aquestive, Aquestive has the right to receive a royalty amount equal to 10% of any value generated by AZSTARYS and any product candidates containing SDX. In connection with the AZSTARYS License Agreement, we paid Aquestive a royalty equal to 10% of the regulatory milestone and royalty payments we received in 2021 from AZSTARYS.

In July 2020, we entered into the Corium Consulting Agreementunder which Corium and Commave, respectively, engaged us to guide the product development and regulatory activities for certain current and potential future products in their portfolio, as well as continue supporting preparation for the potential commercial launch of AZSTARYS. Under the Corium Consulting Agreement, we are entitled to receive payments from Corium of up to $15.6 million, $13.6 million of which will be paid in quarterly installments through March 31, 2022. The remaining $2.0 million was conditioned upon the approval by the FDA of the NDA for Corium's product candidate, ADLARITY. This $2.0 million was earned in the first quarter of 2022. Corium also agreed to be responsible for and reimburse us for all development, commercialization and regulatory expenses incurred as part of the performance of the consulting services.

Components of our Results of Operations

Revenue

Our commercial revenue is, and will be, primarily derived from sales of our approved products or any of our product candidates for which we obtain regulatory approval, and sales of arimoclomol under the Arimoclomol EAP. We expect that our other sources of revenues will be through payments arising from our license agreements with Corium and KVK, and through any other future arrangements related to one of our product candidates. To date, we have generated revenue from the AZSTARYS License Agreement, sale of arimoclomol under the Arimoclomol EAP, reimbursement of out-of-pocket third-party costs, and the performance of consulting services. We cannot guarantee that either Corium will be able to successfully commercialize AZSTARYS or our product candidates covered under the AZSTARYS License Agreement, or KVK will be able to successfully commercialize APADAZ, or that we will ever receive any payments under the APADAZ License Agreement from commercial sales of APADAZ or any future payments under the APADAZ License Agreement. We also do not know when, if ever, any other product candidate will be commercially available.

Cost of Revenue

The components of our cost of revenue are royalties and expenses directly attributable to revenue. To date, we have generated revenue from the AZSTARYS License Agreement, sales of arimoclomol under the Arimoclomol EAP, reimbursement of out-of-pocket third-party costs and the performance of consulting services. In connection with the AZSTARYS License Agreement, we paid Aquestive a royalty equal to 10% of the upfront license payment and all regulatory milestone and royalty payments. In addition, we capitalized incremental costs directly attributable to the AZSTARYS License Agreement, these costs are amortized to royalties and contract costs as revenue is recognized.

Operating Expenses

We classify our operating expenses into three categories: research and development expenses, general and administrative expenses and severance expense. Salaries and personnel-related costs, including benefits, bonuses and stock-based compensation expense, comprise a significant component of each of these expense categories. We allocate expenses associated with our facilities, information technology costs and depreciation and amortization between research and development expenses and general and administrative expenses based on employee headcount and the nature of work performed by each employee.

Research and Development Expense

Research and development expense consists of expenses incurred while performing research and development activities to discover and develop potential product candidates. This includes conducting preclinical studies and clinical trials, manufacturing development efforts and activities related to regulatory filings for product candidates. We recognize research and development expenses as they are incurred. Our research and development expense primarily consists of:

- salaries and personnel-related costs, including benefits and any stock-based compensation, for our scientific personnel performing research and development activities;
- costs related to executing preclinical studies and clinical trials;
- fees paid to consultants and other third parties who support our product candidate development;
- other costs in seeking regulatory approval of our products; and
- allocated facility-related costs and overhead.

We typically use our employee, consultant and infrastructure resources across our development programs. We track outsourced development costs by product candidate or development program, but we do not allocate personnel costs, other internal costs or external consultant costs to specific product candidates or development programs.

The following table summarizes our research and development costs for the years ended December 31, 2022 and 2021 (in thousands):

	Year Ended December 31,	
	2022	**2021**
Outsourced development costs directly identified to programs:		
Arimoclomol	$ 2,940	$ -
KP1077	5,325	86
KP879	34	1,647
AZSTARYS	111	68
Total outsourced development costs directly identified to programs	8,410	1,801
Research and development costs not directly identified to programs:		
Personnel costs including cash compensation, benefits and stock-based compensation	7,709	5,801
Facilities costs	575	525
Other costs	2,920	2,034
Total research and development costs not directly allocated to programs	11,204	8,360
Total research and development expenses	$ 19,614	$ 10,161

We anticipate that our research and development expense will fluctuate for the foreseeable future as we continue our efforts to advance the development of our product candidates, subject to the availability of additional funding. In accordance with the AZSTARYS License Agreement, Corium has also agreed to be responsible and reimburse us for all of development, commercialization and regulatory expenses for the Licensed Product Candidates, subject to certain limitations as set forth in the AZSTARYS License Agreement.

The successful commercialization of AZSTARYS, APADAZ, and any of our product candidates that may be approved and the development of our product candidates is highly uncertain. At this time, we cannot reasonably estimate the nature, timing or costs required to commercialize AZSTARYS, APADAZ, or any of our product candidates, if approved, and complete the remaining development of any product candidates. This is due to the numerous risks and uncertainties associated with the commercialization and development of our products and product candidates.

General and Administrative Expense

General and administrative expense consists primarily of salaries and personnel-related costs, including employee benefits and any stock-based compensation, for employees performing functions other than research and development. This includes personnel in executive, finance, human resources and administrative support functions. Other general and administrative expenses include facility-related costs not otherwise allocated to research and development expense, professional fees for auditing, tax and legal services, expenses associated with obtaining and maintaining patents, consulting costs and costs of our information systems.

We expect that our general and administrative expense will fluctuate as we continue to operate as a public reporting company and continue to develop our product candidates. We believe that these fluctuations will likely include costs related to the hiring of additional personnel and fees for outside consultants, lawyers and accountants. We also expect to continue to incur costs to comply with corporate governance, internal control, investor relations, disclosure and similar requirements applicable to public reporting companies.

Other (Expense) Income

Other (expense) income consists primarily of non-cash costs associated with fair value adjustments to our derivative and warrant liability, amortization of debt issuance costs and debt discount to interest expense and a net loss on extinguishment of debt arising primarily from the debt payoff in the first quarter of 2021. Other (expense) income also includes interest expense incurred on our outstanding borrowings as well as interest and other income consisting primarily of interest earned on investments. These items are unrelated to our core business and thus are recognized as other (expense) income in our consolidated statements of operations.

Income Tax (Expense) Benefit

Income tax (expense) benefit consists of refundable state income tax credits and adjustments to those credits. To date, we have not been required to pay U.S. federal or state income taxes or Denmark taxes because we have not generated taxable income. We have received state income tax credits related to our qualified research activities in Iowa and Denmark. These refundable state income tax credits and adjustments to those credits are recognized as income tax expense (benefit) in our consolidated statements of operations, and to the extent the refundable state income tax credits are not collected as of period end, they are recognized as accounts receivable in our consolidated balance sheets.

Results of Operations

Comparison of the Years Ended December 31, 2022 and 2021 (in thousands):

	Year Ended December 31,		Period-to
	2022	2021	Period Change
Revenue, net	$ 10,458	$ 28,650	$ (18,192)
Operating expenses:			
Cost of revenue	343	2,059	(1,716)
Research and development	19,614	10,161	9,453
General and administrative	15,343	8,701	6,642
Acquired in-process research and development	17,663	-	17,663
Total operating expenses	52,963	20,921	32,042
(Loss) income from operations	(42,505)	7,729	(50,234)
Other (expense) income:			
Loss on extinguishment of debt	-	(16,096)	16,096
Interest expense related to amortization of debt issuance costs and discount	-	(150)	150
Interest expense	(335)	(226)	(109)
Fair value adjustment related to derivative and warrant liability	328	(26)	354
Fair value adjustment related to investments	(577)	(13)	(564)
Interest and other income, net	760	261	499
Total other income (expense)	176	(16,250)	16,426
Loss before income taxes	(42,329)	(8,521)	(33,808)
Income tax benefit (expense)	786	(34)	820
Net loss	$ (41,543)	$ (8,555)	$ (32,988)

Net Loss

Net loss for the year ended December 31, 2022, was $41.5 million compared to net loss of $8.6 million for the year ended December 31, 2021. The change was primarily attributable to a change in (loss) income from operations of $50.2 million, partially offset by a $16.1 million decrease in loss on extinguishment of debt, an increase in net interest income and other income of $0.3 million and an increase in income tax benefit of $0.8 million. Acquired in-process research and development expense of $17.7 million for the year ended December 31, 2022, resulted from the acquisition of an intangible asset related to arimoclomol. The portion of the purchase that was allocated to in-process research and development assets acquired from Orphazyme was immediately expensed in accordance with ASC Subtopic 730-10-25, *Accounting for Research and Development Costs.*

Revenue

Revenue for the year ended December 31, 2022, was $10.5 million, a decrease of $18.2 million compared to revenue of $28.7 million for the year ended December 31, 2021. The decrease was primarily attributable to a decrease in revenue from the AZSTARYS License Agreement of approximately $20.1 million. a decrease in revenue from the Corium Consulting Agreement of approximately $0.9 million and a decrease in other consulting and reimbursement revenue of $2.6 million, partially offset by an increase in sales under the Arimoclomol EAP of approximately $5.5 million.

Cost of Revenue

Cost of revenue for the year ended December 31, 2022, was $0.3 million, a decrease of $1.7 million compared to cost of revenue of $2.0 million for the year ended December 31, 2021. The decrease was primarily attributable to a decrease in royalty payments related to revenue from the AZSTARYS License Agreement of approximately $2.0 million, partially offset by an increase in costs of goods sold under the Arimoclomol EAP.

Research and Development

Research and development expenses increased by $9.5 million, from $10.1 million for the year ended December 31, 2021, to $19.6 million for the year ended December 31, 2022. This increase was primarily attributable to an increase in third-party research and development costs of $6.9 million, an increase in other research and development costs of $0.6 million and an increase in personnel-related costs of $1.9 million.

General and Administrative

General and administrative expenses increased by $6.6 million, from $8.7 million for the year ended December 31, 2021, to $15.3 million for the year ended December 31, 2022. This increase was primarily attributable to an increase in professional fees of $2.2 million and an increase in personnel-related costs of $3.3 million and an increase in other expenses of $1.0 million.

Acquired in-process research and development

Acquired in-process research and development increased by $17.7 million for the year ended December 31, 2022, due to the acquisition of an intangible asset related to the arimoclomol acquisition on May 15, 2022. In accordance with Accounting Standards Codification (ASC) Subtopic 730-10-25, *Accounting for Research and Development Costs*, the up-front payments to acquire a new drug compound, as well as future milestone payments when paid or payable, are immediately expensed as acquired IPR&D in transactions other than a business combination provided that the drug has not achieved regulatory approval for marketing and, absent obtaining such approval, has no alternative future use. Therefore, the portion of the purchase price that was allocated to the IPR&D assets acquired was immediately expensed.

Other Income (Expense)

Other income (expense) increased by $16.4 million, from $16.3 million expense for the year ended December 31, 2021, to $0.2 million of income for the year ended December 31, 2022. This period-to-period decrease in expense was primarily attributable to a loss on extinguishment of debt of $16.1 million during the first quarter of 2021 and an increase in net interest and other income of $0.3 million.

Liquidity and Capital Resources

Sources of Liquidity

Through December 31, 2022, we have funded our research and development and operating activities primarily through the issuance of debt, private placements of redeemable convertible preferred stock and the sale of common stock in our initial public offering, at-the-market offering, underwritten public offerings, through our purchase agreements with Lincoln Park Capital LLC, or Lincoln Park, and from revenue received under the Arimoclomol EAP, AZSTARYS License Agreement, the Corium Consulting Agreement and other consulting arrangements. As of December 31, 2022, we had cash, cash equivalents and investments of $102.9 million.

To date, we have generated revenue from the Arimoclomol EAP, AZSTARYS License Agreement, reimbursement of out-of-pocket third-party costs, and the performance of consulting services.

In July 2020, we entered into the Corium Consulting Agreement under which Corium and Commave, respectively, engaged us to guide the product development and regulatory activities for certain current and potential future products in their portfolio, as well as continue supporting preparation for the potential commercial launch of AZSTARYS. Under the Corium Consulting Agreement, we are entitled to receive payments from Corium of up to $15.6 million, $13.6 million of which was paid in quarterly installments through March 31, 2022. The remaining $2.0 million was conditioned upon the approval by the FDA of the NDA for Corium's product candidate, ADLARITY. This $2.0 million was earned in the first quarter of 2022. Corium also agreed to be responsible for and reimburse us for all development, commercialization and regulatory expenses incurred as part of the performance of the consulting services.

In February 2020, we entered into the 2020 ELOC Agreement, with Lincoln Park, which provided that, upon the terms and subject to the conditions and limitations set forth therein, we may sell to Lincoln Park up to $4.0 million of shares of our common stock, from time to time over the 12-month term of the 2020 ELOC Agreement, and upon execution of the 2020 ELOC Agreement we issued an additional 19,289 shares of our common stock to Lincoln Park as commitment shares in accordance with the closing conditions contained within the 2020 ELOC Agreement. In May 2020, we reached the maximum allowable shares to be issued under the registration statement on Form S-3 covering the sale from time to time of up to $80.0 million of our common stock, preferred stock, and debt and/or warrants, which was declared effective by the SEC on April 10, 2020, of 579,260 shares and therefore we cannot issue additional shares under the 2020 ELOC Agreement. Through May 2020, we sold 559,971 shares of common stock (exclusive of the 19,289 commitment shares previously issued to Lincoln Park) under the 2020 ELOC Agreement for approximately $2.3 million in gross proceeds.

We have had recurring negative net operating cash flows and we anticipate that we may continue to incur minimal positive net cash flows from operations or negative net cash flows from operations for at least the next several years. We expect that our sources of revenue will be through payments arising from our license agreements with Corium and KVK, or through our Corium Consulting Agreement, and other potential consulting arrangements and any other future arrangements related to one of our product candidates.

In January 2021, we completed the Public Offering. The aggregate gross proceeds to us from the Public Offering, including from the exercises by the underwriter of its over-allotment option, totaled $52.4 million, before deducting underwriting discounts and commissions and offering expenses payable by us.

In January 2021, we entered into the January 2021 Inducement Letters. We received aggregate gross proceeds of $44.0 million from the exercise of the Existing Warrants by the Exercising Holders and the sale of the January 2021 Inducement Warrants.

In April 2020, we received proceeds of $0.8 million from a loan, or the PPP Loan, under the Paycheck Protection Program, or the PPP, of the Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act, which we used to retain current teams, maintain payroll and make lease and utility payments. In May 2021, we received notice from the U.S. Small Business Administration that our PPP Loan was forgiven in full, including all principal and interest.

In June 2021, we entered into the June 2021 Inducement Letters. We received aggregate gross proceeds of $39.1 million for the exercise of the select January 2021 Inducement Warrants by the select warrant holders and the sale of the June 2021 Inducement Warrants.

In July 2021, we entered into the Equity Distribution Agreement with JMP and RBCCM under which we may offer and sell, from time to time at our sole discretion, shares of our common stock, having an aggregate offering price of up to $75.0 million through JMP and RBCCM as its sales agents. The issuance and sale, if any, of our common stock the Equity Distribution Agreement will be made pursuant to a registration statement on Form S-3.

We filed a registration statement on Form S-3 covering the sale of the shares of our common stock up to $350.0 million, $75.0 million of which was allocated to the sales of the shares of common stock issuable under the Equity Distribution Agreement. The Form S-3 was declared effective on July 12, 2021. As of December 31, 2022, no shares have been issued or sold under the Equity Distribution Agreement.

Share Repurchase Program

On December 20, 2021, we initiated the Share Repurchase Program pursuant to which we may repurchase up to $50 million of shares of its common stock through December 31, 2023. Capital allocation to the Share Repurchase Program will be based on a variety of factors, including our business results, the receipt of royalties and sales milestones under the AZSTARYS License Agreement, and potentially other sources of non-dilutive capital that may become available to us. Repurchases will be made in compliance with Rule 10b-18 of the Securities Exchange Act of 1934, as amended, subject to a variety of factors, including the market price of our common stock, general market and economic conditions and applicable legal requirements. The exact number of shares to be repurchased by us is not guaranteed and the program may be suspended, modified, or discontinued at any time without prior notice. As of December 31, 2022, we have repurchased 909,953 shares of its common stock for approximately $7.5 million under the Share Repurchase Program.

Line of Credit

On May 31, 2022, we and Ameris Bank, as lender, entered into a $20.0 million revolving loan agreement, or the Line of Credit. Proceeds of the revolving facility provided by the Line of Credit are to be used for general corporate purposes. Loans under the Line of Credit bear interest at the Secured Overnight Financing Rate, or the SOFR, plus 1.60%, with a SOFR floor of 0.00%.

The revolving facility under the Line of Credit is secured by a perfected security interest in deposit accounts. The revolving facility under the Line of Credit is subject to customary affirmative and negative covenants.

The latest maturity date of the loans under the Line of Credit is May 31, 2025. The Line of Credit contains customary events of default that could lead to an acceleration of the loans, including cross-default, bankruptcy and payment defaults. As of December 31, 2022, we have drawn $12.8 million from the Line of Credit to finance the transactions under the Arimoclomol Purchase Agreement, and this amount is supported by a $12.8 million certificate of deposit which is shown as long-term investments in the consolidated balance sheets. The remaining $7.2 million under the Line of Credit is in a separate interest-bearing certificate of deposit and is also recorded as long-term investments in the consolidated balance sheet as of December 31, 2022. These certificates of deposit are pledged as collateral against the Line of Credit and cannot be redeemed so long as the $20.0 million remains available under the Line of Credit. The total value of the certificates of deposit held with Ameris Bank must meet or exceed the amount available to borrow under the Line of Credit so long as the Line of Credit remains active.

Convertible Debt

Deerfield Facility Agreement

In June 2014, we entered into the Deerfield Facility Agreement as a $60.0 million multi-tranche credit facility with Deerfield. At the time we entered into the Deerfield Facility Agreement, we borrowed the first tranche, which consisted of a $15.0 million term note and the $10.0 million convertible note, or the Deerfield Convertible Note. The Deerfield Convertible Note originally bore interest at 9.75% per annum but was subsequently reduced to 6.75%.

Pursuant to the Deerfield Facility Agreement, we also issued to Deerfield a warrant to purchase 14,423,076 shares of our Series D Preferred. Upon closing of our initial public offering, this warrant converted into a warrant exercisable for 1,923,077 shares of our common stock (effected for the 16-for-1 reverse stock split in December 2020, this became a warrant exercisable for 120,192 shares of our common stock). Deerfield assigned the warrant to OTA LLC in December 2022.

2021 Notes

In February 2016, we issued our 5.50% Senior Convertible Notes due 2021, or the 2021 Notes, in aggregate principal amount of $86.3 million. The 2021 Notes were originally issued to Cowen and Company and RBCCM LLC as representatives of the several initial purchasers, who subsequently resold the 2021 Notes to qualified institutional buyers in reliance on the exemption from registration provided by Rule 144A under the Securities Act.

In multiple exchanges occurring in October 2018, December 2019 and January 2020, all outstanding 2021 Notes were exchanged by the holders thereof for either shares of our common stock or the December 2019 Notes and January 2020 Note issued under the terms of the Deerfield Facility Agreement.

2021 Note Exchange Effected in January 2020

In January 2020, we entered into a January 2020 Exchange Agreement, or the January 2020 Exchange Agreement, with M.Kingdon Offshore Master Fund, LP, or Kingdon. Under the January 2020 Exchange Agreement, we issued the January 2020 Note as a senior secured convertible note in the aggregate principal amount of approximately $3.0 million in exchange for the cancellation of an aggregate of approximately $3.0 million of principal amount and accrued interest of the 2021 Note then owned by Kingdon. The terms of the January 2020 Note include (a) a conversion price of $93.60 per share and (b) a floor price of $9.328 per share, and (ii) amended Deerfield Facility Agreement. The Deerfield Facility Agreement was amended to (x) provide for Kingdon to join the Deerfield Facility Agreement as a Lender (as defined in the Deerfield Facility Agreement) and (y) provide that the 2020 Note and shall constitute a "Senior Secured Convertible Note" (as defined in the Deerfield Facility Agreement) for purposes of the Deerfield Facility Agreement and other Transaction Documents (as defined in the Deerfield Facility Agreement).

Debt Restructuring

In December 2020, we entered into the December 2020 Exchange Agreement, which was amended on December 24, 2020. Pursuant to the December 2020 Exchange Agreement, (a) we made the Debt Payment as a cash pre-payment of a portion of principal amount of the Senior Secured Notes and the Deerfield Note to the Deerfield Lenders, DSC and Kingdon, or, collectively, the Holders, in an aggregate amount equal to approximately $30.3 million; and (b) we issued 31,476.98412 shares of our Series B-2 Preferred Stock and warrants exercisable for 3,632,019 shares of our common stock, or the Exchange Warrants, in exchange for the cancellation of a portion of the principal amount of the Senior Secured Notes and Deerfield Note owned by the Holders, with such transaction referred to as the Exchange. Immediately following the completion of the Exchange and Debt Payment, the aggregate balance of principal and accrued interest remaining outstanding under the Facility Notes was approximately $7.6 million.

The December 2020 Exchange Agreement amended the Senior Secured Notes to provide that the failure of our common stock to remain listed on an eligible securities market will not constitute a "Major Transaction" unless such failure occurs after March 31, 2023.

The Exercise Warrants are subject to substantially the same terms and conditions as the Existing Warrants, with an exercise price equal to the exercise price per share of the Existing Warrants and will provide that the Holders will be limited from exercising such Exchange Warrants if, as a result of such exercise, such holders (together with certain affiliates and "group" members of such holders) would beneficially own more than 4.985% of the total number of shares of our common stock then issued and outstanding.

In connection with the December 2020 Exchange Agreement, we filed an Amended and Restated Certificate of Designation of Preferences, Rights and Limitations of Series B-2 Convertible Preferred Stock, or the Amended and Restated Series B-2 Certificate of Designation, with the Secretary of State of the State of Delaware, setting forth the preferences, rights and limitations of the Series B-2 Preferred Stock.

The shares of Series B-2 Preferred Stock were convertible into an aggregate of 4,842,690 shares of our common stock. Each share of Series B-2 Preferred Stock had an aggregate stated value of $1,000 and was convertible into shares of our common stock.

In March 2021, all shares of Series B-2 Preferred Stock converted into common stock.

In June 2021, we filed with the Secretary of State of the State of Delaware a Certificate of Elimination of Series B-2 Convertible Preferred Stock, eliminating from our Certificate of Incorporation the 31,480 shares designated as Series B-2 Convertible Preferred Stock.

Payoff of Facility Agreement Notes and Termination of Facility Agreement

On February 8, 2021, we entered into a payoff letter with the Facility Agreement Note Holders, pursuant to which we agreed to pay off and thereby terminate the Facility Agreement.

Pursuant to the payoff letter, we paid a total of $8.0 million to the Facility Agreement Note Holders, representing the principal balance, accrued interest outstanding and a prepayment fee in repayment of our outstanding obligations under the Facility Agreement.

Pursuant to the payoff letter, all outstanding indebtedness and obligations owed by us to the Facility Agreement Note Holders under the Facility Agreement have been paid in full. The Facility Agreement and the notes thereunder, as well as the security interests in the assets of us securing the Facility Agreement and note obligations, have been terminated. The Facility Agreement Note Holders will retain the warrants previously issued to them.

Cash Flows

The following table summarizes our cash flows for the years ended December 31, 2022 and 2021 (in thousands):

	Year Ended December 31,		Period-to
	2022	2021	Period Change
Net cash (used in) provided by operating activities	$ (18,717)	$ 10,439	$ (29,156)
Net cash used in investing activities	(36,719)	(15,524)	(21,195)
Net cash provided by financing activities	8,352	113,109	(104,757)
Effect of exchange rate changes on cash and cash equivalents	204	-	204
Net (decrease) increase in cash and cash equivalents	$ (46,880)	$ 108,024	$ (154,904)

Operating Activities

For the year ended December 31, 2022, net cash used in operating activities of $18.7 million consisted of a net loss of $41.5 million and $0.5 million in changes in working capital, partially offset by $23.3 million in adjustments for non-cash items. Net loss was primarily attributable to our spending on research and development programs and operating costs, partially offset by revenue received under the AZSTARYS License Agreement, Arimoclomol EAP and the Corium Consulting Agreement. The changes in working capital consisted of $0.7 million related to a change in prepaid expenses and other assets, $6.8 million related to a change in accounts and other receivables, $0.4 million related to a change in operating lease liabilities and $0.4 million related to a change in other liabilities, partially offset by $3.1 million related to a change in accounts payable and accrued expenses, $0.1 million related to a change in inventories, $0.3 million related to a change in operating lease right-of-use assets, $0.4 million related to other long-term assets, and $3.8 million related to a change in discount and rebate liabilities. The adjustments for non-cash items primarily consisted of stock-based compensation expense of $4.3 million, consulting fees paid in stock of $0.2 million, a change in the fair value adjustment related to investments of $0.6 million, $17.7 million related to acquired in-process research and development which was expensed as part of the transactions under the Arimoclomol Purchase Agreement and $0.9 million related to depreciation, amortization and other items, partially offset by a change in the fair value adjustment related to derivative and warrant liabilities of $0.3 million.

For the year ended December 31, 2021, net cash provided by operating activities of $10.4 million consisted of $19.4 million in adjustments for non-cash items, partially offset by a net loss of $8.6 million and $0.4 million in changes in working capital. The adjustments for non-cash items primarily consisted of a loss on extinguishment of debt of $16.1 million, stock-based compensation expense of $2.4 million, amortization of debt issuance costs and debt discount of $0.2 million, a change in the fair value adjustment related to derivative and warrant liabilities of $0.1 million and $0.6 million related to depreciation, amortization and other items. The changes in working capital consisted of $1.1 million related to a change in accounts payable and accrued expenses, $0.3 million related to a change in operating lease liabilities and $0.8 million related to a change in prepaid expenses and other assets, partially offset by $1.1 million related to a change in accounts and other receivables, $0.1 million related to a change in operating lease right-of-use assets and $0.7 million related to a change in other liabilities. Net loss was primarily attributable to a loss on extinguishment of debt and our spending on research and development programs and operating costs, partially offset by revenue received under the AZSTARYS License Agreement and the Corium Consulting Agreement.

Investing Activities

For the year ended December 31, 2022, net cash used in investing activities was $36.7 million, which was attributable to net acquisition costs of the transactions under the Arimoclomol Purchase Agreement of $14.1 million and purchases of investments of $23.8 million, partially offset by maturities of investments of $1.3 million.

For the year ended December 31, 2021, net cash used in investing activities was $15.5 million, which was attributable to purchases of property and equipment of $0.1 million and purchases of long-term investments of $15.4 million.

Financing Activities

For the year ended December 31, 2022, net cash provided by financing activities was $8.3 million, which was primarily attributable to proceeds from the issuance of debt of $12.8 million, proceeds from insurance financing arrangements of $1.3 million and proceeds from sales of common stock under the Employee Stock Purchase Plan, or the ESPP, of $0.3 million, partially offset by payments to repurchase shares as part of the Share Repurchase Program of $4.7 million, and payments of principal on insurance financing arrangements of $1.3 million.

For the year ended December 31, 2021, net cash provided by financing activities was $113.1 million, which was primarily attributable to net proceeds from sales of our common stock in the Public Offering of $49.3 million, net proceeds from the January 2021 Inducement Transaction of $41.4 million, net proceeds from the June 2021 Inducement Transaction of $36.8 million and net proceeds from the exercise of common stock warrants of $30.8 million, partially offset by payment for repurchase of shares of $2.8 million, payment of offering costs of $1.3 million, repayment of principal on finance lease liabilities of $0.2 million, payment of debt issuance costs of $2.9 million, and repayment of principal on convertible notes of $37.9 million.

Future Funding Requirements

Based on our current operating forecast, we believe that our existing cash, cash equivalents and long-term investments will be sufficient to fund our operations into 2026. This estimate does not include our projected revenue, a portion of which is based on royalties from commercial sales and upon the achievement of milestones in the AZSTARYS License Agreement and the APADAZ License Agreement. Certain of the milestones are associated with regulatory matters that are outside our control.

Potential near-term sources of additional funding include:

- any revenues generated under either the AZSTARYS License Agreement or the APADAZ License Agreement;
- any consulting services revenue or short-term milestone payments generated under the AZSTARYS License Agreement;
- any product sales under the Arimoclomol EAP; and
- any consulting services revenue generated under other potential consulting arrangements.

We cannot guarantee that we will be able to generate sufficient proceeds from any of these potential sources to fund our operating expenses.

To date, we have generated revenue from the AZSTARYS License Agreement, reimbursements of out-of-pocket third-party costs, the performance of consulting services and product sales under the Arimoclomol EAP. We expect that, for the foreseeable future, our only sources of revenues will be through payments arising from the AZSTARYS License Agreement, the APADAZ License Agreement, through potential consulting arrangements and any other future arrangements related to one of our product candidates and product sales under the Arimoclomol EAP. While we have entered into the APADAZ License Agreement to commercialize APADAZ in the United States, and entered into the AZSTARYS License Agreement to develop, manufacture and commercialize AZSTARYS, we cannot guarantee that this, or any strategy we adopt in the future, will be successful. For instance, we received milestone payments under the AZSTARYS License Agreement, but we cannot guarantee that we will earn any additional milestone or royalty payments under this agreement in the future. We also cannot guarantee that we will continue to generate revenue under the Arimoclomol EAP. We also expect to continue to incur additional costs associated with operating as a public company.

The COVID-19 pandemic has caused major disruptions to businesses and markets worldwide. We cannot predict what the long-term effects of this pandemic or future public health crises and the resulting economic disruptions may have on our liquidity and results of operations. The extent of the effect of the COVID-19 pandemic on our liquidity and results of operations will depend on a number of future developments, including the duration, spread and intensity of the pandemic, and governmental, regulatory and private sector responses, all of which are uncertain and difficult to predict. A future public health crisis may make it more difficult for us to enroll patients in any future clinical trials or cause delays in the regulatory approval of our product candidates. A portion of our projected revenue is based upon the achievement of milestones in the AZSTARYS License Agreement associated with regulatory matters that may be impacted by the COVID-19 pandemic. As a result, we cannot predict what, if any, impact that the COVID-19 pandemic or a future public health crisis may have on our ability to achieve these milestones. The economic uncertainty surrounding the COVID-19 pandemic and as a result of rising inflation and interest rates may also dramatically reduce our ability to secure debt or equity financing necessary to support our operations.

We have based our estimates of our cash needs and cash runway on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect and we cannot guarantee that we will be able to generate sufficient proceeds from the AZSTARYS License Agreement, the APADAZ License Agreement, product reimbursements under the Arimoclomol EAP and potential consulting arrangements or other funding transactions to fund our operating expenses. To meet any additional cash requirements, we may seek to sell additional equity or convertible securities that may result in dilution to our stockholders, issue additional debt or seek other third-party funding, including potential strategic transactions, such as licensing or collaboration arrangements. Because of the numerous risks and uncertainties associated with the development and commercialization of product candidates and products, we are unable to estimate the amounts of increased capital outlays and operating expenditures necessary to complete the commercialization and development of our partnered product or product candidates, should they obtain regulatory approval.

Critical Accounting Estimates

This management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with accounting principles generally accepted in the United States. The preparation of our financial statements requires us to make estimates that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of our financial statements, as well as the reported revenues and expenses during the reported periods. We evaluate these estimates on an ongoing basis. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are more fully described in Note B to our audited financial statements appearing elsewhere in this Annual Report on Form 10-K, we believe that the following accounting policies are critical to the process of making significant estimates in the preparation of our financial statements and understanding and evaluating our reported financial results.

In-process research and development

We accounted for the arimoclomol acquisition as an asset acquisition as the majority of the value of the assets acquired related to the arimoclomol acquired in-process research and development, or the IPR&D asset. The intangible asset associated with IPR&D relates to arimoclomol. The estimated fair value of $17.7 million was determined using the excess earnings valuation method, a variation of the income valuation approach. The excess earnings valuation method estimates the value of an intangible asset equal to the present value of the incremental after-tax cash flows attributable to that intangible asset over its remaining economic life. Some of the more significant assumptions utilized in our asset valuations included projected revenues, probability of commercial success, and the discount rate. The fair value using the excess earnings valuation method was determined using an estimated weighted average cost of capital of 42%, which reflects the risks inherent in future cash flow projections and represents a rate of return that a market participant would expect for this asset. This fair value measurement was based on significant inputs not observable in the market and thus represent Level 3 fair value measurement.

Accrued Expenses

We enter into contractual agreements with third-party vendors who provide research and development, manufacturing, and other services in the ordinary course of business. Some of these contracts are subject to milestone-based invoicing and services are completed over an extended period of time. We record liabilities under these contractual commitments when an obligation has been incurred. This accrual process involves reviewing open contracts and purchase orders, communicating with our applicable personnel to identify services that have been performed and estimating the level of service performed and the associated cost when we have not yet been invoiced or otherwise notified of actual cost. The majority of our service providers invoice us monthly in arrears for services performed. We make estimates of our accrued expenses as of each balance sheet date based on the facts and circumstances known to us. We periodically confirm the accuracy of our estimates with the service providers and make adjustments if necessary. Examples of estimated accrued expenses include:

- fees paid to CROs in connection with preclinical and toxicology studies and clinical trials;
- fees paid to investigative sites in connection with clinical trials;
- fees paid to contract manufacturers in connection with the production of our raw materials, drug substance and product candidates; and
- professional fees.

We base our expenses related to clinical trials on our estimates of the services received and efforts expended pursuant to contracts with multiple research institutions and CROs that conduct and manage clinical trials on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. Payments under some of these contracts depend on factors such as the successful enrollment of patients and the completion of clinical trial milestones. In accruing service fees, we estimate the time period over which services will be performed and the level of effort expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we will adjust the accrual accordingly. If we do not identify costs that we have begun to incur or if we underestimate or overestimate the level of these costs, our actual expenses could differ from our estimates.

Stock-Based Compensation

We record the fair value of stock options issued as of the grant date as compensation expense. We recognize compensation expense over the requisite service period, which is equal to the vesting period. Stock-based compensation expense has been reported in our statements of operations as follows (in thousands):

	Year Ended December 31,	
	2022	2021
Research and development	$ 1,443	$ 914
General and administrative	2,851	1,522
Total stock-based compensation	$ 4,294	$ 2,436

Determination of the Fair Value of Stock-Based Compensation Grants

We calculate the fair value of stock-based compensation arrangements using the Black-Scholes option-pricing model. The Black-Scholes option-pricing model requires the use of subjective assumptions, including the expected volatility of our common stock, the assumed dividend yield, the expected term of our stock options, the risk-free interest rate for a period that approximates the expected term of our stock options and the fair value of the underlying common stock on the date of grant. In applying these assumptions, we considered the following factors:

- historically we have not had sufficient experience to estimate the volatility of our common stock. As such, we calculated the expected volatility based on reported data for selected similar publicly traded companies for which the historical information is available, or peer volatility, and blended it with our historical volatility, or leverage-adjusted peer volatility. For the purpose of identifying peer companies, we consider characteristics such as industry, length of trading history, similar vesting terms and in-the-money option status. We utilized this leverage-adjusted peer volatility for grants prior to the initial public offering, as well as grants within the two-year period immediately following the initial public offering. For grants after the second anniversary of the initial public offering we utilized our historical volatility to determine the expected volatility;

- the assumed dividend yield is based on our expectation of not paying dividends for the foreseeable future;

- we determine the average expected life of "plain vanilla" stock options based on the simplified method in accordance with SEC Staff Accounting Bulletin Nos. 107 and 110, as our common stock to date has been publicly traded for a limited amount of time. We expect to use the simplified method until we have sufficient historical exercise data to provide a reasonable basis upon which to estimate expected term. For options that are not considered "plain vanilla," such as those with exercise prices in excess of the fair market value of the underlying stock, we use an expected life equal to the contractual term of the option;

- we determine the risk-free interest rate by reference to implied yields available from U.S. Treasury securities with a remaining term equal to the expected life assumed at the date of grant; and

- we estimate forfeitures based on our historical analysis of actual stock option forfeitures.

We account for stock-based compensation arrangements with directors and consultants that contain only service conditions for vesting using a fair value approach. The grant date fair value of these options is measured using the Black-Scholes option pricing model reflecting the same assumptions as applied to employee options in each of the reported periods, other than the expected life, which is assumed to be the remaining contractual life of the option.

The following summarizes the assumptions used for estimating the fair value of stock options granted to employees for the periods indicated:

	Year Ended December 31,	
	2022	**2021**
Risk-free interest rate	1.70% - 3.80%	0.92% - 1.55%
Expected term (in years)	5.50 - 7.00	5.50 - 10.00
Expected volatility	91.28% - 98.91%	96.91% - 102.57%
Expected dividend yield	0	0

Fair Value of Financial Instruments

We have a common stock warrant issued to OTA, put options embedded within those OTA warrants, and common stock warrants issued to KVK that meet the definition of derivative financial instruments and are accounted for as derivatives. The fair value of the common stock warrant issued to OTA, and put options embedded within the OTA Warrants are based on Monte Carlo simulations, while the common stock warrant issued to KVK is valued using a probability-weighted Black-Scholes option pricing model. These derivatives are fair valued at each reporting period.

The derivative liability for the OTA common stock warrant was $1,000 and $288,000 at December 31, 2022, and 2021, respectively. The derivative liability for the put options embedded within the OTA common stock warrant was $0 and $18,000 at December 31, 2022, and 2021, respectively. The derivative liability for the KVK common stock warrant was $0 and $24,000 at December 31, 2022 and 2021, respectively. A 10% increase in the enterprise value would result in a negligible increase in the estimated fair value of the OTA common stock warrant, the put options embedded within the OTA common stock warrant and the KVK common stock warrant at December 31, 2022. Upon exercise of the warrants, we will adjust the associated derivative liability to fair value with any changes recorded in other (expense) income. At such time, such derivative liability will also be reclassified to additional paid-in capital, and no further revaluations will be necessary.

Utilization of Net Operating Loss Carryforwards and Research and Development Credits

As of December 31, 2022, we had federal net operating loss, or NOL, carryforwards of approximately $236.0 million, due to prior period losses, $138.1 million of which, if not utilized, will begin to expire in 2027 and $97.9 million with no expiration. We also had research and development credit carryforwards of $3.8 million with expiration dates ranging from 2027 to 2037 and $3.1 million with no expiration. As of December 31, 2022, we maintain a full valuation allowance over our deferred tax assets for financial reporting purposes.

In accordance with Section 382 of the Code, a change in equity ownership of greater than 50% within a three-year period results in an annual limitation on a company's ability to utilize its NOL carryforwards created during the tax periods prior to the change in ownership. We performed a Section 382 ownership change analysis in 2017 and determined that we experienced an ownership change in 2010, which resulted in a portion of our net operating loss carryforwards being subject to an annual limitation under Section 382 through 2012. No other ownership changes or limitations on our historical net operating loss carryforwards were noted through the year ended December 31, 2017. In addition, we may have already experienced an ownership change, or may experience an ownership changes in the future, as a result of shifts in our stock ownership. If we determine that an ownership change has occurred and our ability to use our historical net operating loss carryforwards is materially limited, it would harm our future operating results by increasing our future tax obligations.

Recent Accounting Pronouncements

In August 2020, the FASB issued ASU 2020-06, *Debt—Debt with Conversion and Other Options (Subtopic 470-20) and Derivatives and Hedging—Contracts in Entity's Own Equity (Subtopic 815-40); Accounting for Convertible Instruments and Contracts in an Entity's Own Equity*, or ASU 2020-06, which addresses issues identified as a result of the complexities associated with applying U.S. GAAP for certain financial instruments with characteristics of liabilities and equity. This update addresses, among other things, the number of accounting models for convertible debt instruments and convertible preferred stock, targeted improvements to the disclosures for convertible instruments and earnings-per-share, or EPS, guidance and amendments to the guidance for the derivatives scope exception for contracts in an entity's own equity, as well as the related EPS guidance. This update applies to all entities that issue convertible instruments and/or contracts in an entity's own equity. This guidance is effective for financial statements issued for fiscal years beginning after December 15, 2021, and interim periods within those fiscal years. FASB specified that an entity should adopt the guidance as of the beginning of its annual fiscal year. The adoption of ASU 2020-06 did not have a material impact on our financial statements and disclosures.

In May 2021, the FASB issued ASU 2021-04, *Earnings Per Share (Topic 260), Debt— Modifications and Extinguishments (Subtopic 470-50), Compensation —Stock Compensation (Topic 718), and Derivatives and Hedging— Contracts in Entity's Own Equity (Subtopic 815-40); Issuer's Accounting for Certain Modifications or Exchanges of Freestanding Equity-Classified Written Call Options, a consensus of the FASB Emerging Issues Task Force*, or ASU 2021-04, which aims to clarify and reduce diversity in issuer's accounting for modifications or exchanges of freestanding equity-classified written call options that remain equity classified after modification or exchange. This update applies to all entities that issue freestanding written call options that are classified in equity. This guidance is effective for financial statements issued for fiscal years beginning after December 15, 2021, and interim periods within those fiscal years. FASB specified that an entity should adopt the guidance as of the beginning of its annual fiscal year. The adoption of ASU 2021-04 did not have a material impact on our consolidated financial statements and disclosures.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Not applicable.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

The financial statements required by this Item 8 are set forth beginning in Item 15 of this report and are incorporated herein by reference.

ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE

The information required by Item 304 of Regulation S-K has been previously reported.

ITEM 9A. CONTROLS AND PROCEDURES

Limitations on effectiveness of controls and procedures

In designing and evaluating our disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives. In addition, the design of disclosure controls and procedures must reflect the fact that there are resource constraints and that management is required to apply judgment in evaluating the benefits of possible controls and procedures relative to their costs.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our chief executive officer and our chief financial officer, evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2022. Based on the evaluation of our disclosure controls and procedures as of December 31, 2022, our chief executive officer and our chief financial officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Management's Report on Internal Control over Financial Reporting

Management is responsible for establishing and maintaining adequate internal control over our financial reporting, as such term is defined in Rules 13a-15(f) and 15d-15(e) under the Exchange Act. Under the supervision and with the participation of our management, including our chief executive officer and chief financial officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting. Management has used the framework set forth in the report entitled "*Internal Control – Integrated Framework (2013)*" published by the Committee of Sponsoring Organizations of the Treadway Commission to evaluate the effectiveness of our internal control over financial reporting. Our evaluation of internal control over financial reporting did not include internal controls of Zevra Denmark A/S (formerly known as KemPharm Denmark A/S prior to February 21, 2023), our wholly-owned subsidiary formed in May 2022, through which we acquired all of the assets and operations of Orphazyme related to arimoclomol during the second quarter of fiscal year 2022. This fiscal year 2022 acquisition represented 9.5% of our consolidated total assets and 51.5% of our consolidated total revenue as of and for the fiscal year ended December 31, 2022. We have included the financial results of the acquired operations in our consolidated financial statements included elsewhere in this Annual Report on Form 10-K from the date of acquisition, and we are in the process of incorporating Zevra Denmark A/S into our internal control over financial reporting. Based on its evaluation, management has concluded that our internal control over financial reporting was effective as of December 31, 2022, the end of our most recent fiscal year.

Our independent registered public accounting firm has not performed an evaluation of our internal control over financial reporting during any period in accordance with the provisions of the Sarbanes-Oxley Act. For as long as we remain a non-accelerated filer, we intend to take advantage of the exemption permitting us not to comply with the requirement that our independent registered public accounting firm provide an attestation on the effectiveness of our internal control over financial reporting.

Changes in Internal Control over Financial Reporting

There was no change in our internal control over financial reporting identified in connection with the evaluation required by Rules 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during our fiscal quarter ended December 31, 2022, that materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. OTHER INFORMATION

None.

ITEM 9C. DISCLOSURES REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTIONS

Not applicable.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

The information required by this Item 10 will be set forth in the headings “Proposal 1 – Election of Directors,” “Executive Officers” and “Information Regarding the Board of Directors and Corporate Governance” in our definitive proxy statement for our 2023 annual meeting of stockholders, or the proxy statement, and, is incorporated herein by reference.

We have adopted a Code of Business Conduct and Ethics, or the Code of Conduct, applicable to all of our employees, executive officers and directors, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. The Code of Conduct is available on our website at www.zevra.com. The nominating and corporate governance committee of our board of directors is responsible for overseeing the Code of Conduct and must approve any waivers of the Code of Conduct for employees, executive officers and directors. We intend to post any amendments to the Code of Conduct or any waivers of its requirements for any executive officer or director on our website.

ITEM 11. EXECUTIVE COMPENSATION

The information required by this Item 11 will be set forth under the headings “Executive Compensation", "Director Compensation" and “Information Regarding the Board of Directors and Corporate Governance” in our proxy statement and is incorporated herein by reference.

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT

The information required by this Item 12 will be set forth under the headings “Security Ownership of Certain Beneficial Owners and Management” and “Securities Authorized for Issuance under the Equity Compensation Plans” in the proxy statement and is incorporated herein by reference.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE

The items required by this Item 13 will be set forth under the headings “Information Regarding the Board of Directors and Corporate Governance” and “Transactions with Related Persons” in the proxy statement and is incorporated herein by reference.

ITEM 14. PRINCIPAL ACCOUNTING FEES AND SERVICES

The information required by this Item 14 will be set forth under the proposal with the heading “Proposal 2 - Ratification of Appointment of Independent Registered Public Accounting Firm” in the proxy statement and is incorporated herein by reference.

PART IV

ITEM 15. EXHIBITS AND FINANCIAL STATEMENT SCHEDULES

(a) The following documents are filed as part of this report:

(1) *Index list to Financial Statements:*

(2) *Financial Statement Schedules*

All other schedules are omitted because they are not required or the required information is included in the financial statements or notes thereto.

(3) *Exhibits*

The exhibits listed in the accompanying Exhibit Index are filed or incorporated by reference as part of this report.

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

Stockholders and the Board of Directors of KemPharm, Inc.

Opinion on the Financial Statements

We have audited the accompanying balance sheets of KemPharm, Inc. (the Company) as of December 31, 2021 and 2020, the related statements of operations, changes in stockholders′ equity (deficit) and cash flows for the years then ended, and the related notes to the financial statements. In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2021 and 2020, and the results of its operations and its cash flows for the years then ended, in conformity with accounting principles generally accepted in the United States of America.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical Audit Matters

Critical audit matters are matters arising from the current period audit of the financial statements that were communicated or required to be communicated to the audit committee and that: (1) relate to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective or complex judgments. We determined that there are no critical audit matters.

/s/ RSM US LLP

We have served as the Company's auditor since 2017.

Orlando, Florida
March 30, 2022

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of Zevra Therapeutics, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheet of Zevra Therapeutics, Inc. (the Company) as of December 31, 2022, the related consolidated statements of operations, comprehensive income (loss), changes in stockholders' equity (deficit) and cash flows for the year then ended, and the related notes (collectively referred to as the “consolidated financial statements”). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2022, and the results of its operations and its cash flows for the year then ended in conformity with U.S. generally accepted accounting principles.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company’s financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audit we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audit included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audit also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audit provides a reasonable basis for our opinion.

Critical Audit Matter

The critical audit matter communicated below is a matter arising from the current period audit of the financial statements that was communicated or required to be communicated to the audit committee and that: (1) relates to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective or complex judgments. The communication of the critical audit matter does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which they relate.

Asset Purchase

Description of the Matter

As described in Note A to the consolidated financial statements, on May 31, 2022, the Company completed a transaction to acquire the net assets of Arimoclomol from the third party seller (Orphazyme) in a transaction accounted for as an asset purchase ("the Transaction"). The net assets acquired were recorded at fair value and included acquired in-process research and development ("IPR&D") associated with Arimoclomol of $17.7 million and was recorded as an operating expense in the Company's consolidated statement of operations.

Auditing the Company's accounting for the Transaction was complex because of the judgment involved in evaluating whether the transaction met the criteria of a business combination or an asset acquisition among other accounting considerations. The subjective considerations included whether substantially all of the fair value of the gross assets acquired is concentrated in a single identifiable asset or group of similar identifiable assets which was supported by a fair value analyses of the acquired units of account, the most significant of which is the IPR&D asset.

How We Addressed the Matter in Our Audit

To test the Company's accounting for the Transaction, we performed audit procedures that included, among others, evaluating the Company's application of the relevant accounting guidance as well as procedures over the Company's fair value estimates of the units of account which included the value assigned to the IPR&D asset. The procedures over the fair value estimates included the involvement of our valuation specialists to assist in evaluating the methodology used to estimate the fair value of the IPR&D asset and to test certain significant assumptions, including the discount rate. Our valuation specialists independently calculated the fair values of the acquired units of account to compare to the Company's recorded amounts. We also tested the significant assumptions used to develop the prospective financial information, including the completeness and accuracy of the underlying data supporting the significant assumptions and estimates. For example, we compared the significant assumptions to current industry, market and economic trends, historical results of the Company's business and other guideline companies within the same industry and to other relevant factors. We also performed sensitivity analyses of the significant assumptions to evaluate the change in the fair value of the IPR&D asset resulting from changes in the assumptions. We also assessed the appropriateness of the related disclosures in the consolidated financial statements.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2022.

Orlando, Florida

March 7, 2023

ZEVRA THERAPEUTICS, INC.
BALANCE SHEETS
(in thousands, except share and par value amounts)

	December 31,	
	2022	**2021**
Assets		
Current assets:		
Cash and cash equivalents	$ 65,466	$ 112,346
Securities at fair value	16,900	-
Short-term investments - other	481	-
Accounts and other receivables	8,299	1,528
Prepaid expenses and other current assets	1,877	1,182
Total current assets	93,023	115,056
Inventories	671	-
Property and equipment, net	794	884
Operating lease right-of-use assets	988	1,141
Securities at fair value	-	14,932
Long-term investments - other	20,000	490
Other long-term assets	53	438
Total assets	$ 115,529	$ 132,941
Liabilities and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable and accrued expenses	$ 6,169	$ 3,038
Current portion of operating lease liabilities	480	356
Current portion of discount and rebate liabilities	4,655	-
Other current liabilities	422	836
Total current liabilities	11,726	4,230
Line of credit payable	12,800	-
Derivative and warrant liability	1	330
Operating lease liabilities, less current portion	843	1,232
Discount and rebate liabilities, less current portion	4,327	-
Other long-term liabilities	25	31
Total liabilities	29,722	5,823
Commitments and contingencies (Note H)		
Stockholders' equity:		
Preferred stock:		
Undesignated preferred stock, $0.0001 par value, 10,000,000 shares authorized, no shares issued or outstanding as of December 31, 2022 or December 31, 2021	-	-
Common stock, $0.0001 par value, 250,000,000 shares authorized, 35,450,257 shares issued and 34,540,304 shares outstanding as of December 31, 2022; 35,325,801 shares issued and 35,005,640 shares outstanding as of December 31, 2021	3	4
Additional paid-in capital	401,799	396,957
Treasury stock, at cost	(7,536)	(2,814)
Accumulated deficit	(308,572)	(267,029)
Accumulated other comprehensive income	113	-
Total stockholders' equity	85,807	127,118
Total liabilities and stockholders' equity	$ 115,529	$ 132,941

See accompanying notes to consolidated financial statements

ZEVRA THERAPEUTICS, INC.
STATEMENTS OF OPERATIONS
(in thousands, except share and per share amounts)

	Year Ended December 31,	
	2022	**2021**
Revenue, net	$ 10,458	$ 28,650
Operating expenses:		
Cost of revenue	343	2,059
Research and development	19,614	10,161
General and administrative	15,343	8,701
Acquired in-process research and development	17,663	-
Total operating expenses	52,963	20,921
(Loss) income from operations	(42,505)	7,729
Other (expense) income:		
Loss on extinguishment of debt	-	(16,096)
Interest expense related to amortization of debt issuance costs and discount	-	(150)
Interest expense	(335)	(226)
Fair value adjustment related to derivative and warrant liability	328	(26)
Fair value adjustment related to investments	(577)	(13)
Interest and other income, net	760	261
Total other income (expense)	176	(16,250)
Loss before income taxes	(42,329)	(8,521)
Income tax benefit (expense)	786	(34)
Net loss	(41,543)	(8,555)
Deemed dividend	-	(54,342)
Net loss attributable to common stockholders	$ (41,543)	$ (62,897)
Basic and diluted net loss per share of common stock:		
Net loss attributable to common stockholders	$ (1.20)	$ (2.11)
Weighted average number of shares of common stock outstanding:		
Basic and diluted	34,488,800	29,766,347

See accompanying notes to consolidated financial statements

ZEVRA THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF COMPREHENSIVE INCOME (LOSS)
(in thousands, except share and per share amounts)

ZEVRA THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF COMPREHENSIVE INCOME (LOSS)
(in thousands, except share and per share amounts)

	Year Ended December 31,	
	2022	**2021**
Net loss attributable to common stockholders	$ (41,543)	$ (62,897)
Other comprehensive income:		
Foreign currency translation adjustment	113	-
Other comprehensive income	113	-
Comprehensive loss	$ (41,430)	$ (62,897)

ZEVRA THERAPEUTICS, INC.
STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY (DEFICIT)
(in thousands)

	Series B-2 Convertible Preferred Stock	Common Stock	Additional Paid-in Capital	Treasury Stock	Accumulated Deficit	Other Comprehensive Income	Total Stockholders' (Deficit) Equity
Balance as of January 1, 2021	$ -	$ -	$ 192,062	$ -	$ (258,474)		$ (66,412)
Net loss	-	-	-	-	(8,555)		(8,555)
Stock-based compensation expense	-	-	2,436	-	-		2,436
Issuance of common stock in connection with Public Offering, net of discounts and commissions	-	1	49,284	-	-		49,285
Issuance of common stock in connection with the exercise of warrants in the January 2021 Inducement Transaction, net of discounts and commissions	-	1	40,390	-	-		40,391
Issuance of common stock in connection with the exercise of common stock warrants	-	-	30,819	-	-		30,819
Fair value of warrants issued in connection with the Exchange Agreement	-	-	15,990	-	-		15,990
Fair value of Series B-2 Preferred Stock issued in accordance with the Exchange Agreement	29,056	-	-	-	-		29,056
Issuance of common stock as a result of Series B-2 Preferred Stock conversion	(29,056)	1	29,055	-	-		-
Fair value of warrants issued in connection with the January 2021 Inducement Transaction	-	-	38,437	-	-		38,437
Deemed dividend related to the January 2021 Inducement Transaction	-	-	(37,444)	-	-		(37,444)
Issuance of common stock in connection with the exercise of warrants in the June 2021 Inducement Transaction, net of discounts and commissions	-	1	36,559	-	-		36,560
Fair value of warrants issued in connection with the June 2021 Inducement Transaction	-	-	17,089	-	-		17,089
Deemed dividend related to the June 2021 Inducement Transaction	-	-	(16,898)	-	-		(16,898)
Shares repurchased as part of the Share Repurchase Program	-	-	-	(2,814)	-		(2,814)
Offering expenses charged to equity	-	-	(1,124)	-	-		(1,124)
Issuance of common stock in exchange for consulting services	-	-	302	-	-		302
Balance as of December 31,2021	$ -	$ 4	$ 396,957	$ (2,814)	$ (267,029)	$ -	$ 127,118
Net loss	-	-	-	-	(41,543)	-	(41,543)
Stock-based compensation expense	-	-	4,294	-	-	-	4,294
Shares repurchased as part of the Share Repurchase Program	-	(1)	-	(4,722)	-	-	(4,723)
Issuance of common stock as part of the Employee Stock Purchase Plan		-	324	-	-	-	324
Issuance of common stock in exchange for consulting services		-	224	-	-	-	224
Other comprehensive income	-	-	-	-	-	113	113
Balance as of December 31, 2022	$ -	$ 3	$ 401,799	$ (7,536)	$ (308,572)	$ 113	$ 85,807

See accompanying notes to consolidated financial statements

ZEVRA THERAPEUTICS, INC.
STATEMENTS OF CASH FLOWS
(in thousands)

	Year Ended December 31,	
	2022	**2021**
Cash flows from operating activities:		
Net loss	$ (41,543)	$ (8,555)
Adjustments to reconcile net loss to net cash used in operating activities:		
Loss on extinguishment of debt	-	16,096
Stock-based compensation expense	4,294	2,436
Non-cash interest expense	-	8
Amortization of debt issuance costs and debt discount	-	150
Depreciation and amortization expense	944	257
Fair value adjustment related to derivative and warrant liability	(328)	26
Fair value adjustment related to investments	577	-
Loss on sublease and disposal of property and equipment	9	76
Consulting fees paid in common stock	224	302
Acquired in-process research and development	17,663	-
Gain on foreign currency exchange rates	(91)	-
Change in assets and liabilities:		
Accounts and other receivables	(6,772)	1,051
Prepaid expenses and other assets	(657)	(825)
Inventories	147	-
Operating lease right-of-use assets	277	133
Other long-term assets	386	-
Accounts payable and accrued expenses	3,131	(1,113)
Discount and rebate liabilities	3,782	-
Operating lease liabilities	(389)	(326)
Other liabilities	(371)	723
Net cash (used in) provided by operating activities	(18,717)	10,439
Cash flows from investing activities:		
Acquisitions, net	(14,090)	-
Purchases of property and equipment	(93)	(102)
Purchases of long-term investments, net	(22,536)	(15,422)
Net cash used in investing activities	(36,719)	(15,524)
Cash flows from financing activities:		
Proceeds from Public Offering, net of discounts and commissions	-	49,285
Proceeds from January 2021 Inducement Transaction, net of discounts and commissions	-	41,384
Proceeds from June 2021 Inducement Transaction, net of discounts and commissions	-	36,751
Proceeds from issuance of debt	12,800	-
Proceeds from insurance financing arrangements	1,273	-
Proceeds from Employee Stock Purchase Plan	324	-
Payments of principal on insurance financing arrangements	(1,306)	-
Payment to repurchase shares as part of the Share Repurchase Program	(4,723)	(2,814)
Payment of offering costs	-	(1,338)
Repayment of principal on finance lease liabilities	(16)	(173)
Payment of debt issuance costs	-	(2,881)
Repayment of principal on convertible notes	-	(37,924)
Net proceeds from exercise of common stock warrants	-	30,819
Net cash provided by financing activities	8,352	113,109
Effect of exchange rate changes on cash and cash equivalents	204	-
Net (decrease) increase in cash and cash equivalents	(46,880)	108,024
Cash and cash equivalents, beginning of period	112,346	4,322
Cash and cash equivalents, end of period	$ 65,466	$ 112,346
Supplemental cash flow information:		
Cash paid for interest	$ 321	$ 218
Right-of-use assets obtained in exchange for lease liabilities	123	-
Facility Notes principal converted to Series B-2 Preferred Stock	-	31,477
Amounts due for deferred offering costs included in accounts payable and accrued expenses	-	48
Fair value of warrants issued to underwriters in connection with Public Offering	-	3,485

See accompanying notes to consolidated financial statements

ZEVRA THERAPEUTICS, INC.
NOTES TO FINANCIAL STATEMENTS

A. Description of Business, Basis of Presentation, and Significant Transactions

Organization

Zevra Therapeutics, Inc. (the "Company") is a biotechnology company focused on the discovery, development, and commercialization of novel treatments for rare central nervous system ("CNS") and neurodegenerative diseases, lysosomal storage disorders, and related treatment areas. The Company has a diverse product portfolio, combining a clinical-stage pipeline with new drug application ("NDA") stage and commercial assets. The Company's pipeline includes arimoclomol, an orally-delivered, first-in-class investigational product candidate for Niemann-Pick disease type C ("NPC"), and KP1077, which the Company is developing as a treatment for idiopathic hypersomnia ("IH"), a rare neurological sleep disorder, and narcolepsy. In addition, the U.S. Food and Drug Administration ("FDA") has approved AZSTARYS®, formerly referred to as KP415, a once-daily treatment for attention deficit hyperactivity disorder ("ADHD") in patients six years and older containing the Company's prodrug, serdexmethylphenidate ("SDX"), which is being commercialized by Corium, Inc. ("Corium"), an affiliate of Gurnet Point Capital, L.P. ("GPC"), in the U.S. The FDA has also approved APADAZ®, an immediate-release combination product containing benzhydrocodone, the Company's prodrug of hydrocodone, and acetaminophen, which is being commercialized by KVK-Tech, Inc. ("KVK") in the U.S. The Company changed its name from KemPharm, Inc. to Zevra Therapeutics, Inc. effective as of February 21, 2023.

Basis of Presentation

The Company prepared the consolidated financial statements in accordance with accounting principles generally accepted in the United States of America ("US GAAP") and the rules and regulations of the Securities and Exchange Commission ("SEC") and, in the Company's opinion, reflect all adjustments, including normal recurring items that are necessary. All significant intercompany accounts and transactions have been eliminated in consolidation.

Arimoclomol Acquisition

On May 15, 2022, the Company and Zevra Denmark A/S (formerly known as KemPharm Denmark A/S prior to February 21, 2023) ("Zevra DK"), a newly formed Danish company and wholly-owned subsidiary of the Company entered into an asset purchase agreement (the "Arimoclomol Purchase Agreement") with Orphazyme A/S in restructuring, a Danish public limited liability company ("Orphazyme"). The Arimoclomol Purchase Agreement closed on May 31, 2022. Under the terms of the Arimoclomol Purchase Agreement, Zevra DK purchased all of the assets and operations of Orphazyme related to arimoclomol and settled all of Orphazyme's actual outstanding liabilities to its creditors with a cash payment of $12.8 million. In addition, Zevra DK agreed to assume an estimated reserve liability of $5.2 million related to revenue generated from Orphazyme's Early Access Program in France.

The Company accounted for the arimoclomol acquisition as an asset acquisition as the majority of the value of the assets acquired related to the arimoclomol acquired in-process research and development ("IPR&D") asset. The intangible asset associated with IPR&D relates to arimoclomol. The estimated fair value of $17.7 million was determined using the excess earnings valuation method, a variation of the income valuation approach. The excess earnings valuation method estimates the value of an intangible asset equal to the present value of the incremental after-tax cash flows attributable to that intangible asset over its remaining economic life. Some of the more significant assumptions utilized in our asset valuations included projected revenues, probability of commercial success, and the discount rate. The fair value using the excess earnings valuation method was determined using an estimated weighted average cost of capital of 42%, which reflects the risks inherent in future cash flow projections and represents a rate of return that a market participant would expect for this asset. This fair value measurement was based on significant inputs not observable in the market and thus represent Level 3 fair value measurement.

In accordance with Accounting Standards Codification (ASC) Subtopic 730-10-25, *Accounting for Research and Development Costs*, the up-front payments to acquire a new drug compound, as well as future milestone payments when paid or payable, are immediately expensed as acquired IPR&D in transactions other than a business combination provided that the drug has not achieved regulatory approval for marketing and, absent obtaining such approval, has no alternative future use. Therefore, the portion of the purchase price that was allocated to the IPR&D assets acquired was immediately expensed. Other assets acquired and liabilities assumed, were recorded at fair value. The company also recorded a $0.8 million income tax benefit for the year-ended December 31, 2022, related to research and development credits that are expected to be realized from the local jurisdiction in Denmark.

The following represents the consideration paid and purchase price allocation for the acquisition of arimoclomol (in thousands):

Cash	$	12,800
Assumed reserve liability		5,200
Total consideration	$	18,000
Total consideration		18,000
Direct transaction costs associated with the acquisition (1)		1,290
Total purchase price to be allocated	**$**	**19,290**
Property and equipment, inventory and assembled workforce acquired		1,627
IPR&D (2)		17,663
Total allocated purchase price	**$**	**19,290**

(1) As a result of the asset acquisition accounting, the transaction costs associated with the acquisition should be included in the costs of the assets acquired and allocated amongst qualifying assets using the relative fair value basis. The transaction costs primarily included financial advisor fees and legal expenses.
(2) The primary asset acquired, the IPR&D asset, was expensed and the allocated transaction related costs were included with and expensed with this asset.

Underwriting Agreement

On January 8, 2021, the Company entered into an underwriting agreement (the "Underwriting Agreement") with Roth Capital Partners, LLC (the "Underwriter" or "Roth"), to issue and sell 6,765,463 shares of common stock of the Company, pre-funded warrants to purchase 926,844 shares of common stock and warrants to purchase 7,692,307 shares of common stock in an underwritten public offering (the "Public Offering") pursuant to a registration statement on Form S-1 (File No. 333-250945) and a related prospectus, in each case filed with the SEC. The offering price to the public was $6.50 per share of common stock and accompanying warrant, representing a public offering price of $6.4999 per share of common stock and $0.0001 per related warrant. In addition, the Company granted the Underwriter an option to purchase, for a period of 45 days, up to an additional 1,153,846 shares of the Company's common stock and/or warrants to purchase up to an additional 1,153,846 shares of the Company's common stock and/or warrants to purchase up to an additional 1,153,846 of the Company's common stock.

On January 8, 2021, the Underwriter exercised its over-allotment option, in part, for warrants to purchase 754,035 shares of the Company's common stock. Further on February 1, 2021, the Underwriter again exercised its over-allotment option to purchase 374,035 shares of common stock.

On January 12, 2021, the Company closed the Public Offering. The aggregate gross proceeds to the Company from the Public Offering, including over-allotment, totaled approximately $52.4 million, before deducting underwriting discounts and commissions and offering expenses payable by the Company.

On January 25, 2022, the Company filed an amendment to the registration statement on Form S-1 (File No. 333-250945) on Form S-3 covering the issuance of the shares of our common stock issuable upon the exercise of the warrants issued in the Public Offering and remaining unexercised as of the date of the amendment, which was declared effective on February 1, 2022.

Listing on the Nasdaq Stock Market

On January 7, 2021, the Company's common stock was approved for listing on the Nasdaq Capital Market. The Company's common stock began trading on the Nasdaq Capital Market on January 8, 2021, under the ticker symbol "KMPH".

On October 19, 2021, the Company announced that its shares of common stock were approved for listing to the Nasdaq Global Select Market. Trading on the Nasdaq Global Select Market commenced effective with the open of business on October 19, 2021, under the Company's ticker symbol, "KMPH". The Company was previously listed on the Nasdaq Capital Market, following its uplisting to the exchange in January 2021.

On March 1, 2023, following its name change, the Company's common stock began trading on the Nasdaq Global Select Market under the ticker symbol "ZVRA".

Entry into 2021 ATM Agreement

On July 2, 2021, the Company entered into an equity distribution agreement (the "2021 ATM Agreement") with JMP Securities LLC ("JMP") and RBC Capital Markets, LLC ("RBCCM") under which the Company may offer and sell, from time to time at its sole discretion, shares of its common stock having an aggregate offering price of up to $75.0 million through JMP and RBCCM as its sales agents. The issuance and sale, if any, of common stock by the Company under the 2021 ATM Agreement will be made pursuant to a registration statement on Form S-3. JMP and RBCCM may sell the common stock by any method permitted by law deemed to be an "at the market offering" as defined in Rule 415 of the Securities Act of 1933, as amended. JMP and RBCCM will use commercially reasonable efforts to sell the common stock from time to time, based upon instructions from the Company (including any price, time or size limits or other customary parameters or conditions the Company may impose). The Company will pay JMP and RBCCM a commission equal to 3.0% in the aggregate of the gross sales proceeds of any common stock sold through JMP and RBCCM under the 2021 ATM Agreement. The Company filed a registration statement on Form S-3 covering the sale of the shares of its common stock up to $350.0 million, $75.0 million of which was allocated to the sales of the shares of common stock issuable under the 2021 ATM Agreement, which was declared effective on July 12, 2021. As of December 31, 2022 and 2021, no shares have been issued or sold under the 2021 ATM Agreement.

Share Repurchase Program

On December 20, 2021, the Company initiated a share repurchase program (the "Share Repurchase Program") pursuant to which the Company may repurchase up to $50 million of shares of its common stock through December 31, 2023. Capital allocation to the Share Repurchase Program will be based on a variety of factors, including our business results, the receipt of royalties and sales milestones under the AZSTARYS License Agreement (refer to Note B), and potentially other sources of non-dilutive capital that may become available to the Company. Repurchases will be made in compliance with Rule 10b-18 of the Securities Exchange Act of 1934, as amended, subject to a variety of factors, including the market price of the Company's common stock, general market and economic conditions and applicable legal requirements. The exact number of shares to be repurchased by the Company is not guaranteed and the program may be suspended, modified, or discontinued at any time without prior notice. As of December 31, 2022, the Company has repurchased 909,953 shares of its common stock for approximately $7.5 million under the Share Repurchase Program.

Reclassifications

Certain reclassifications were made to the 2021 consolidated financial statements to conform to the classifications used in 2022. These reclassifications had no impact on the consolidated net loss, changes in stockholder's equity, or cash flows previously reported.

B. Summary of Significant Accounting Policies

Use of Estimates

The preparation of these consolidated financial statements in conformity with U.S. GAAP requires the Company to make estimates and assumptions that affect the amounts reported in the consolidated financial statements and accompanying notes. Actual results could differ from those estimates.

On an ongoing basis, the Company evaluates its estimates and assumptions, including those related to revenue recognition, the useful lives of property and equipment, the recoverability of long-lived assets, the incremental borrowing rate for leases, and assumptions used for purposes of determining stock-based compensation, income taxes, the fair value of long-term investments, the fair value of the derivative and warrant liability and discount and rebate liabilities, among others. The Company bases its estimates on historical experience and on various other assumptions that it believes to be reasonable, the results of which form the basis for making judgments about the carrying value of assets and liabilities.

Concentration of Credit Risk

Financial instruments that potentially expose the Company to concentrations of credit risk consist principally of cash on deposit and investments with multiple financial institutions, the balances of which frequently exceed insured limits, and accounts receivable, which are concentrated amongst a limited number of customers.

Cash and Cash Equivalents

The Company considers any highly liquid investments with an original maturity of three months or less to be cash equivalents.

Investments

The Company maintains investment securities that are classified as available-for-sale securities for which the Company has elected the fair value option under ASC 825, *Financial Instruments.* As such, these securities are carried at fair value with unrealized gains and losses included in fair value adjustment related to investments on the consolidated statements of operations. The securities primarily consist of U.S. Treasury securities and U.S. government-sponsored agency securities and are included in Securities at fair value in the consolidated balance sheets. As of December 31, 2022, and 2021, the Company held securities with an aggregate fair value of $16.9 million and $14.9 million, respectively, that contained aggregate unrealized losses of approximately $577,000 and $13,000, respectively. Applying fair value accounting to these debt securities more accurately represents the Company's investment strategy due to the fact that excess cash is currently being invested for the purpose of funding future operation **s.** In addition, the Company holds certificates of deposit totaling $20.5 million and $0.5 million as of December 31, 2022, and 2021, respectively, and are included in long-term investments - other in the consolidated balance sheets. Interest income is recognized as earned using an effective yield method giving effect to the amortization of premium and accretion of discount and is based on the economic life of the securities Interest income is included in Interest and other income, net in the consolidated statements of operations.

Property and Equipment

The Company records property and equipment at cost less accumulated depreciation and amortization. Costs of renewals and improvements that extend the useful lives of the assets are capitalized. Maintenance and repairs are expensed as incurred. Depreciation is determined on a straight-line basis over the estimated useful lives of the assets, which generally range from three to ten years. Leasehold improvements are amortized over the shorter of the useful life of the asset or the term of the related lease. Upon retirement or disposition of assets, the costs and related accumulated depreciation and amortization are removed from the accounts with the resulting gains or losses, if any, reflected in the consolidated statements of operations.

Debt Issuance Costs

Debt issuance costs incurred in connection with financing arrangements are recorded as a reduction of the related debt on the balance sheet and amortized over the life of the respective financing arrangement using the effective interest method. In the first quarter of 2021, all outstanding debt was extinguished through a series of debt payments and a conversion of debt principal and interest to Series B-2 Preferred Stock (Note G). As a result of the debt extinguishment, the associated discount and debt issuance costs were written off and recorded as a loss on extinguishment.

Impairment of Long-Lived Assets

Long-lived assets to be held and used are reviewed for impairment whenever events or changes in circumstances indicate that the carrying amounts of the assets may not be recoverable. When such events occur, the Company compares the carrying amounts of the assets to their undiscounted expected future cash flows. If the undiscounted cash flows are insufficient to recover the carrying values, an impairment loss is recorded for the difference between the carrying values and fair values of the asset. No such impairment occurred for the years ended December 31, 2022 , or 2021 .

Revenue Recognition

The Company recognizes revenue in accordance with ASC 606, *Revenue from Contracts with Customers* (“ASC 606”) and, as a result, follows the five-step model when recognizing revenue: 1) identifying a contract; 2) identifying the performance obligations; 3) determining the transaction price; 4) allocating the price to the performance obligations; and 5) recognizing revenue when the performance obligations have been fulfilled.

Arimoclomol Early Access Program

Net revenue includes revenue from the sale of arimoclomol for the treatment of NPC under the remunerated early access compassionate use program in France (“French nATU”). An early access compassionate use program is a program giving specific patients access to a drug, which is not yet approved for commercial sale. Only drugs targeting serious or rare indications and for which there is currently no appropriate treatment are considered for early access compassionate use programs. Further, to be considered for the early access compassionate use program, the drug must have proven efficacy and safety and must either be undergoing price negotiations or seeking marketing approval. In accordance with ASC 606, the Company recognizes revenue when fulfilling its performance obligation under the Arimoclomol Early Access Program ("Arimoclomol EAP") by transferring control of promised goods or services to its customer, in an amount that reflects the consideration that the Company expects to receive in exchange for those goods or services. In determining when the customer obtains control of the product, the Company considers certain indicators, including whether the Company has a present right to payment from the customer, whether title and/or significant risks and rewards of ownership have transferred to the customer and whether customer acceptance has been received. Revenue is recognized net of sales deductions, including discounts, rebates, applicable distributor fees, and revenue-based taxes. Under the French nATU, the manufacturer can set its own price for the drug products until a price agreement with the authorities is in place. Any excess in the price charged by the manufacturer compared to the price agreed with the health authorities once the drug product is approved in France must be repaid. The repayment is considered in the clawback liability (rebate). An estimate of net revenue and clawback liability are recognized using the ‘expected value’ method. Accounting for net revenue and clawback liability requires determination of the most appropriate method for the expected final transaction price, which depends on the terms and conditions in the contracts with the French Health Authorities and is subject to price negotiations with the French Health Authorities, following marketing approval. This estimate also requires assumptions with respect to inputs into the method, including current pricing of comparable marketed products within the rare disease area in France. Management has considered the expected final sales price as well as the price of similar drug products. The Company is operating within a rare disease therapeutic area where there is unmet treatment need and hence a limited number of comparable commercialized drugs products. The limited available relevant market information for directly comparable commercialized drugs within rare disease increases the uncertainty in management's estimate. For the year ended December 31, 2022, the Company recognized revenue related to the Arimoclomol EAP in France of $4.8 million, which is net of a clawback liability of $3.8 million. As part of the Arimoclomol Purchase Agreement the Company assumed an estimated reserve liability of $5.2 million related to revenue generated from the Arimoclomol EAP in France. The total estimate reserve liability as of December 31, 2022, including the additional clawback liability for the year ended December 31, 2022, was $9.0 million. As of December 31, 2022, this estimated reserve liability is recorded as discount and rebate liabilities in the consolidated balance sheet and is separated into current and long-term based upon the timing of the expected payment to the French regulators.

Licensing Agreements

The Company enters into licensing agreements with licensees that fall under the scope of ASC 606.

The terms of the Company's licensing agreements typically include one or more of the following: (i) upfront fees; (ii) milestone payments related to the achievement of development, regulatory, or commercial goals; and (iii) royalties on net sales of licensed products. Each of these payments may result in licensing revenues.

As part of the accounting for these agreements, the Company must develop estimates and assumptions that require judgment to determine the underlying stand-alone selling price for each performance obligation which determines how the transaction price is allocated among the performance obligations. Generally, the estimation of the stand-alone selling price may include such estimates as, independent evidence of market price, forecasted revenues or costs, development timelines, discount rates, and probability of regulatory success. The Company evaluates each performance obligation to determine if they can be satisfied at a point in time or over time, and it measures the services delivered to the licensee which are periodically reviewed based on the progress of the related program. The effect of any change made to an estimated input component and, therefore revenue or expense recognized, would be recorded as a change in estimate. In addition, variable consideration (e.g., milestone payments) must be evaluated to determine if it is constrained and, therefore, excluded from the transaction price.

Up-front Fees: If a license to the Company's intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, the Company recognizes revenues from the transaction price allocated to the license when the license is transferred to the licensee and the licensee is able to use and benefit from the license. For licenses that are bundled with other promises, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time.

Milestone Payments: At the inception of each arrangement that includes milestone payments (variable consideration), the Company evaluates whether the milestones are considered probable of being reached and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the Company's or the licensee's control, such as non-operational developmental and regulatory approvals, are generally not considered probable of being achieved until those approvals are received. At the end of each reporting period, the Company re-evaluates the probability of achievement of milestones that are within its or the licensee's control, such as operational developmental milestones and any related constraint, and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect collaboration revenues and earnings in the period of adjustment. Revisions to the Company's estimate of the transaction price may also result in negative licensing revenues and earnings in the period of adjustment.

AZSTARYS License Agreement

In September 2019, the Company entered into a Collaboration and License Agreement (the "AZSTARYS License Agreement") with Commave Therapeutics SA ("Commave"), an affiliate of GPC. Under the AZSTARYS License Agreement, the Company granted to Commave an exclusive, worldwide license to develop, manufacture and commercialize the Company's product candidates containing SDX and d-methylphenidate ("d-MPH"), including AZSTARYS, or any other product candidate developed by the Company containing SDX and developed to treat ADHD or any other CNS disorder. Corium was tasked by Commave, to lead all commercialization activities for AZSTARYS under the AZSTARYS License Agreement. Pursuant to the AZSTARYS License Agreement, Corium agreed to pay milestone payments upon the occurrence of specified regulatory milestones related to AZSTARYS, additional fixed payments upon the achievement of specified U.S. sales milestones, and quarterly, tiered royalty payments based on a range of percentages of net sales. Commave is obligated to make such royalty payments on a product-by-product basis until expiration of the royalty term for the applicable product.

In April 2021, the Company entered into Amendment No. 1 to the AZSTARYS License Agreement (the "AZSTARYS Amendment"). Pursuant to the AZSTARYS Amendment, the Company and Commave agreed to modify the compensation terms of the AZSTARYS License Agreement. Pursuant to the AZSTARYS Amendment, Commave paid the Company $10.0 million in connection with the entry into the AZSTARYS Amendment as a result of the regulatory approval of AZSTARYS in the United States, which occurred on March 2, 2021. Commave also paid the Company $10.0 million following the receipt of the scheduling determination of the compound SDX by the U.S. Drug Enforcement Agency ("DEA"), which occurred on May 7, 2021. In addition, the AZSTARYS Amendment increased the total remaining future regulatory and sales milestone payments related to AZSTARYS up to an aggregate of $590.0 million in payments upon the occurrence of specified regulatory milestones related to AZSTARYS and upon the achievement of specified U.S. net sales milestones.

Commave also agreed to be responsible for and reimburse the Company for all of the development, commercialization and regulatory expenses incurred on the licensed products, subject to certain limitations as set forth in the AZSTARYS License Agreement. As part of this agreement, the Company is obligated to perform consulting services on behalf of Commave related to the licensed products. For these consulting services, Commave has agreed to pay the Company a set rate per hour on any consulting services performed on behalf of Commave for the benefit of the licensed products.

In accordance with the terms of the Company's March 20, 2012 Termination Agreement with Aquestive Therapeutics, Aquestive Therapeutics has the right to receive an amount equal to 10% of any royalty or milestone payments made to the Company related to AZSTARYS, KP879 or KP1077 under the AZSTARYS License Agreement.

The AZSTARYS License Agreement is within the scope of ASC 606, as the transaction represents a contract with a customer where the participants function in a customer / vendor relationship and are not exposed equally to the risks and rewards of the activities contemplated under the AZSTARYS License Agreement. Using the concepts of ASC 606, the Company identified the grant of the exclusive, worldwide license and the performance of consulting services, which includes the reimbursement of out-of-pocket third-party research and development costs, as its only two performance obligations at inception. The Company further determined that the transaction price, at inception, under the agreement was $10.0 million upfront payment plus the fair value of the Development Costs (as defined in the AZSTARYS License Agreement) which was allocated among the performance obligations based on their respective related stand-alone selling price.

The consideration allocated to the grant of the exclusive, worldwide license was $10.0 million, which reflects the standalone selling price. The Company utilized the adjusted market assessment approach to determine this standalone selling price which included analyzing prospective offers received from various entities throughout our licensing negotiation process as well as the consideration paid to other competitors in the market for a similar type of transaction. The Company determined that the intellectual property licensed under the AZSTARYS License Agreement represented functional intellectual property and it has significant standalone functionality and therefore should be recognized at a point in time as opposed to over time. The revenue related to the grant of the exclusive, worldwide license was recognized at a point in time at the inception of the AZSTARYS License Agreement.

The Company is entitled to additional payments from Commave conditioned upon the achievement of specified regulatory milestones related to AZSTARYS and the achievement of certain U.S. sales milestones. Further, Commave will pay the Company quarterly, tiered royalty payments based on a range of percentages of Net Sales (as defined in the AZSTARYS License Agreement). The Company concluded that these regulatory milestones, sales milestones and royalty payments each contain a significant uncertainty associated with a future event. As such, these milestone and royalty payments are constrained at contract inception and are not included in the transaction price as the Company could not conclude that it is probable a significant reversal in the amount of cumulative revenue recognized will not occur surrounding these milestone payments. At the end of each reporting period, the Company updates its assessment of whether the milestone and royalty payments are constrained by considering both the likelihood and magnitude of the potential revenue reversal. For the years ended December 31, 2022, and 2021, the Company recognized revenue under the AZSTARYS License Agreement of $0.9 million and $20.9 million, respectively. There was no deferred revenue related to this agreement as of December 31, 2022, and 2021.

Consulting Arrangements

The Company enters into consulting arrangements with third parties that fall under the scope of ASC 606. These arrangements may require the Company to deliver various rights, services, including research and development services, regulatory services and/or commercialization support services. The underlying terms of these arrangements generally provide for consideration to the Company in the form of consulting fees and reimbursements of out-of-pocket third-party research and development, regulatory and commercial costs.

Corium Consulting Agreement

In July 2020, the Company entered into a consultation services arrangement (the "Corium Consulting Agreement") with Corium, Inc. ("Corium") under which Corium engaged the Company to guide the product development and regulatory activities for certain current and potential future products in Corium's portfolio, as well as continue supporting preparation for the potential commercial launch of AZSTARYS (together, "Corium Consulting Services"). Corium is a portfolio company of GPC and was tasked by Commave to lead all commercialization activities for AZSTARYS under the AZSTARYS License Agreement, as discussed above.

Under the Corium Consulting Agreement, the Company is entitled to receive payments from Corium of up to $15.6 million, $13.6 million of which was paid in quarterly installments through March 31, 2022. The remaining $2.0 million was conditioned upon the approval by the FDA of the NDA for Corium's product candidate, ADLARITY. This $2.0 million was earned in the first quarter of 2022. Corium also agreed to be responsible for and reimburse the Company for all development, commercialization and regulatory expenses incurred as part of the performance of the Corium Consulting Services. The Corium Consulting Agreement is within the scope of ASC 606, as the transaction represents a contract with a customer where the participants function in a customer / vendor relationship and are not exposed equally to the risks and rewards of the activities contemplated under the Corium Consulting Agreement. The Company identified the performance of consulting services, which includes the reimbursement to the Company of third-party pass-through costs, as its only performance obligation at inception. The Company further determined that the transaction price, at inception, under the agreement was $13.6 million which is the fair value of the consulting services, including the reimbursement of third-party pass-through costs. The Company concluded that the regulatory milestone contains a significant uncertainty associated with a future event. As such, this milestone is constrained at contract inception and is not included in the transaction price as the Company could not conclude that it is probable a significant reversal in the amount of cumulative revenue recognized will not occur surrounding these milestone payments.

The Company determined that the performance of consulting services, including reimbursement of third-party pass-through costs, is a performance obligation that is satisfied over time as the services are performed and the reimbursable costs are paid. As such, the revenue related to the performance obligation will be recognized as the consulting services are performed and the services associated with the reimbursable third-party pass-through costs are incurred and paid by the Company, in accordance with the practical expedient allowed under ASC 606 regarding an entity's right to consideration from a customer in an amount that corresponds directly to the value to the customer of the entity's performance completed to date. As of December 31, 2022, the Company has recognized approximately all of the consulting services and third-party pass-through costs under the Corium Consulting Agreement.

For the years ended December 31, 2022, and 2021, the Company recognized revenue under the Corium Consulting Agreement of $4.7 million and $7.7 million, respectively. As of December 31, 2022, the Company had no deferred revenue related to this agreement. As of December 31, 2021, the Company had deferred revenue related to this agreement of $0.4 million.

Other Consulting Arrangements

For the years ended December 31, 2022, and 2021, the Company recognized revenue under other consulting arrangements of $0.1 million and $0.1 million, respectively. There was no deferred revenue from other consulting arrangements as of December 31, 2022, or 2021.

Accounts and Other Receivables

Accounts and other receivables consist of receivables under the AZSTARYS License Agreement and Arimoclomol EAP, as well as receivables related to consulting arrangements, income tax receivables and other receivables due to the Company. Receivables under the AZSTARYS License Agreement are recorded for amounts due to the Company related to reimbursable third-party costs and royalties on product sales. Receivables under the Arimoclomol EAP are recorded for product sales under the French nATU. These receivables, as well as the receivables related to consulting arrangements, are evaluated to determine if any reserve or allowance should be established at each reporting date.

Inventory

As discussed in Note A, a portion of the purchase price was allocated to inventory acquired representing the value of inventory associated with forecasted EAP revenues and as of December 31, 2022, the Company had inventory of $0.7 million remaining. Inventories are stated at net realizable value using the first-in, first-out method of valuation.

The Company may scale-up and make commercial quantities of its product candidates prior to the date it anticipates that such product will receive final regulatory approval. The scale-up and commercial production of pre-launch inventory involves the risk that such products may not be approved for marketing on a timely basis, or ever. This risk notwithstanding, the Company may scale-up and build pre-launch inventory of product that have not received final regulatory approval when the Company believes such action is appropriate in relation to the commercial value of the product launch opportunity. Inventory manufactured prior to regulatory approval is recorded as research and development expense until regulatory approval for the product is obtained. As of December 31, 2022, and 2021, the Company did not have pre-launch inventory that qualified for capitalization.

Research and Development

Major components of research and development costs include cash compensation, stock-based compensation, depreciation and amortization expense on research and development property and equipment, costs of preclinical studies, clinical trials and related clinical manufacturing, costs of drug development, costs of materials and supplies, facilities cost, overhead costs, regulatory and compliance costs, and fees paid to consultants and other entities that conduct certain research and development activities on the Company's behalf. Costs incurred in research and development are expensed as incurred.

The Company records nonrefundable advance payments it makes for future research and development activities as prepaid expenses. Prepaid expenses are recognized as expense in the statements of operations as the Company receives the related goods or services.

The Company enters into contractual agreements with third-party vendors who provide research and development, manufacturing, and other services in the ordinary course of business. Some of these contracts are subject to milestone-based invoicing and services are completed over an extended period of time. The Company records liabilities under these contractual commitments when an obligation has been incurred. This accrual process involves reviewing open contracts and purchase orders, communicating with the applicable personnel to identify services that have been performed and estimating the level of service performed and the associated cost when the Company has not yet been invoiced or otherwise notified of actual cost. The majority of the service providers invoice the Company monthly in arrears for services performed. The Company makes estimates of the accrued expenses as of each balance sheet date based on the facts and circumstances known. The Company periodically confirms the accuracy of the estimates with the service providers and make adjustments, if necessary.

Patent Costs

Patent costs, including related legal costs, are expensed as incurred and recorded within general and administrative expenses on the statements of operations.

Income Taxes

The Company recognizes deferred tax assets and liabilities for the expected future tax consequences of temporary differences between the financial reporting and tax basis of assets and liabilities, as well as for operating loss and tax credit carryforwards. Deferred tax assets and liabilities are measured using the tax rates that are expected to apply to taxable income for the years in which those tax assets and liabilities are expected to be realized or settled. Valuation allowances are recorded to reduce deferred tax assets to the amount the Company believes is more likely than not to be realized.

Uncertain tax positions are recognized only when the Company believes it is more likely than not that the tax position will be upheld on examination by the taxing authorities based on the merits of the position. The Company recognizes interest and penalties, if any, related to unrecognized income tax uncertainties in income tax expense. The Company did not have any accrued interest or penalties associated with uncertain tax positions as of December 31, 2022 and 2021.

The Company files income tax returns in the United States for federal and various state jurisdictions. With few exceptions, the Company is no longer subject to U.S. federal and state and local income tax examinations for years prior to 2015, although carryforward attributes that were generated prior to 2017 may still be adjusted upon examination by the Internal Revenue Service if used in a future period. No income tax returns are currently under examination by taxing authorities.

On December 22, 2017, the U.S. government enacted H.R. 1, “An Act to provide for reconciliation pursuant to titles II and V of the concurrent resolution on the budget for fiscal year 2018” ("Tax Act"). Effective January 1, 2018, the Tax Act provides for a new global intangible low-taxed income ("GILTI") provision. Under the GILTI provision, certain foreign subsidiary earnings in excess of an allowable return on the foreign subsidiary’s tangible assets are included in U.S. taxable income. The Company has not recorded any deferred taxes for future GILTI inclusions as any future inclusions are expected to be treated as a period expense and offset by net operating loss carryforwards in the U.S.

Stock-Based Compensation

The Company measures and recognizes compensation expense for all stock-based payment awards made to employees, officers and directors based on the estimated fair values of the awards as of the grant date. The Company records the value of the portion of the award that is ultimately expected to vest as expense over the requisite service period. The Company also accounts for equity instruments issued to non-employees using a fair value approach under Accounting Standards Codification ("ASC") subtopic 505-50. The Company values equity instruments and stock options granted using the Black-Scholes option pricing model.

Basic and Diluted Net Loss per Share of Common Stock

The Company uses the two-class method to compute net loss per common share because the Company has issued securities, other than common stock, that contractually entitle the holders to participate in dividends and earnings of the Company. The two-class method requires earnings for the period to be allocated between common stock and participating securities based upon their respective rights to receive distributed and undistributed earnings. Holders of each series of the Company’s convertible preferred stock and select warrants are entitled to participate in distributions, when and if declared by the board of directors, that are made to common stockholders and, as a result, are considered participating securities.

Segment and Geographic Information

Operating segments are defined as components of an enterprise (business activity from which it earns revenue and incurs expenses) for which discrete financial information is available and regularly reviewed by the chief operating decision maker ("CODM") in deciding how to allocate resources and in assessing performance. The Company’s CODM is its Chief Executive Officer. The Company views its operations and manages its business as a single operating and reporting segment. The Company holds assets in both the United States and Europe as of December 31, 2022 and 2021.

Foreign currency

Assets and liabilities are translated into the reporting currency using the exchange rates in effect on the consolidated balance sheet dates. Equity accounts are translated at historical rates, except for the change in retained earnings during the year, which is the result of the income statement translation process. Revenue and expense accounts are translated using the weighted average exchange rate during the period. The cumulative translation adjustments associated with the net assets of foreign subsidiaries are recorded in accumulated other comprehensive income/loss in the accompanying consolidated statements of stockholders' equity.

Application of New or Revised Accounting Standards—Adopted

From time to time, the Financial Accounting Standards Board (the "FASB") or other standard-setting bodies issue accounting standards that are adopted by the Company as of the specified effective date.

In August 2020, the FASB issued Accounting Standards Update ("ASU") No. 2020-06, *Debt—Debt with Conversion and Other Options (Subtopic 470-20) and Derivatives and Hedging—Contracts in Entity's Own Equity (Subtopic 815-40); Accounting for Convertible Instruments and Contracts in an Entity's Own Equity* ("ASU 2020-06"), which addresses issues identified as a result of the complexities associated with applying U.S. GAAP for certain financial instruments with characteristics of liabilities and equity. This update addresses, among other things, the number of accounting models for convertible debt instruments and convertible preferred stock, targeted improvements to the disclosures for convertible instruments and earnings-per-share ("EPS") guidance and amendments to the guidance for the derivatives scope exception for contracts in an entity's own equity, as well as the related EPS guidance. This update applies to all entities that issue convertible instruments and/or contracts in an entity's own equity. This guidance is effective for financial statements issued for fiscal years beginning after December 15, 2021, and interim periods within those fiscal years. FASB specified that an entity should adopt the guidance as of the beginning of its annual fiscal year. The adoption of ASU 2020-06 did not have a material impact on the Company's consolidated financial statements and disclosures.

In May 2021, the FASB issued ASU No. 2021-04, *Earnings Per Share (Topic 260), Debt—Modifications and Extinguishments (Subtopic 470-50), Compensation—Stock Compensation (Topic 718), and Derivatives and Hedging— Contracts in Entity's Own Equity (Subtopic 815-40); Issuer's Accounting for Certain Modifications or Exchanges of Freestanding Equity-Classified Written Call Options, a consensus of the FASB Emerging Issues Task Force* ("ASU 2021-04"), which aims to clarify and reduce diversity in issuer's accounting for modifications or exchanges of freestanding equity-classified written call options that remain equity classified after modification or exchange. This update applies to all entities that issue freestanding written call options that are classified in equity. This guidance is effective for financial statements issued for fiscal years beginning after December 15, 2021, and interim periods within those fiscal years. FASB specified that an entity should adopt the guidance as of the beginning of its annual fiscal year. The adoption of ASU 2021-04 did not have a material impact on the Company's consolidated financial statements and disclosures.

C. Accounts and Other Receivables

Accounts and other receivables consist of the following (in thousands):

	December 31,	
	2022	**2021**
Accounts receivable	$ 7,185	$ 1,421
Other receivables	1,114	107
Total accounts and other receivables	$ 8,299	$ 1,528

D. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consist of the following (in thousands):

	December 31,	
	2022	**2021**
Prepaid insurance	$ 577	$ 637
Prepaid offering costs	-	293
Other prepaid expenses and current assets	1,300	252
Total prepaid expenses and other current assets	$ 1,877	$ 1,182

E. Property and Equipment

Property and equipment consists of the following (in thousands):

	December 31,	
	2022	2021
Laboratory equipment	$ 710	$ 645
Furniture and office equipment	104	71
Computers and hardware	462	338
Leasehold improvements	710	724
Finance lease right-of-use assets	251	1,031
Total property and equipment	2,237	2,809
Less: accumulated depreciation and amortization	(1,443)	(1,925)
Property and equipment, net	$ 794	$ 884

The estimated useful lives of property and equipment are as follows:

Asset Category	Useful Life (in years)
Laboratory equipment	10
Furniture and office equipment	5 - 10
Computers and hardware	3 - 7
Leasehold improvements	9

Depreciation and amortization expense, including amounts pertaining to assets held under finance leases, was approximately $298,000 and $257,000 for the years ended December 31, 2022 and 2021, respectively.

F. Accounts Payable and Accrued Expenses

Accounts payable and accrued expenses consist of the following (in thousands):

	December 31,	
	2022	2021
Accrued banking fees	-	700
Accrued payroll	1,673	1,054
Accrued professional fees	256	259
Accounts payable	3,611	516
Other accrued expenses	629	509
Total accounts payable and accrued expenses	$ 6,169	$ 3,038

G. Debt Obligations

Deerfield Facility Agreement

In June 2014, the Company entered into a $60 million multi-tranche credit facility (the "Deerfield Facility Agreement") with Deerfield Private Design Fund III, LP ("Deerfield"). At the time the Company entered into the Deerfield Facility Agreement, the Company borrowed the first tranche, which consisted of a term loan of $15 million (the "Term Note") and a senior secured loan of $10 million (the "Deerfield Convertible Note"). The Deerfield Convertible Note originally bore interest at 9.75% per annum but was subsequently reduced to 6.75%.

Pursuant to the Deerfield Facility Agreement, the Company issued to Deerfield a warrant to purchase 14,423,076 shares of Series D Preferred, which is exercisable until June 2, 2024, and Deerfield assigned this warrant to OTA LLC in December of 2022 (the "OTA Warrant"). Upon completion of the Company's initial public offering, the OTA Warrant automatically converted into a warrant to purchase 120,192 shares of the Company's common stock. This warrant qualifies as a participating security under ASC Topic 260, *Earnings per Share*, and is treated as such in the net loss per share calculation (Note O). If a Major Transaction occurs (as defined in the Deerfield Facility Agreement), OTA may require the Company to redeem the OTA Warrant for a cash amount equal to the Black-Scholes value of the portion of the OTA Warrant to be redeemed (the "Warrant Put Option").

The Company recorded the fair value of the shares of Series D Preferred to debt issuance costs on the date of issuance. The Company also recorded the fair value of the OTA Warrant and the embedded Warrant Put Option to debt discount on the date of issuance. The debt issuance costs and debt discount were amortized over the term of the related debt and the expense was recorded as interest expense related to amortization of debt issuance costs and discount in the consolidated statements of operations. As discussed below, in the first quarter of 2021, the debt was extinguished, through a series of debt payments and a conversion of debt principal and interest to Series B-2 Preferred Stock. As a result of the debt extinguishment, the associated discount and debt issuance costs were written off and recorded as a loss on extinguishment in the consolidated statements of operations.

5.50% Senior Convertible Notes

In February 2016, the Company issued $86.3 million aggregate principal amount of its 5.50% Senior Convertible Notes due 2021 (the "2021 Notes") to Cowen and RBC Capital Markets, LLC, as representatives of several initial purchasers, who subsequently resold the 2021 Notes to qualified institutional buyers, including Kingdon Offshore Master Fund, LP ("Kingdon") in reliance on the exemption from registration provided by Rule 144A under the Securities Act of 1933, as amended.

As described in more detail below, in multiple exchanges, all outstanding 2021 Notes were exchanged by the holders thereof for either shares of our common stock or senior secured convertible promissory notes issued under the terms of the Deerfield Facility Agreement.

2021 Note Exchange Effected in December 2019

In December 2019, the Company entered into the December 2019 Exchange Agreement and Amendment to Facility Agreement, Senior Secured Convertible Notes and Warrants (the "December 2019 Exchange Agreement") with Deerfield and Deerfield Special Situations Fund LP (collectively, "the Deerfield Lenders") and Delaware Street Capital Master Fund, L.P. ("DSC" and, collectively with the Deerfield Lenders, the "December 2019 Holders"). Under the December 2019 Exchange Agreement, the Company issued senior secured convertible promissory notes under the Deerfield Facility Agreement in the aggregate principal amount of approximately $71.4 million (the "December 2019 Notes"), in exchange for the cancellation of an aggregate of approximately $71.4 million principal amount and accrued interest of the Company's 2021 Notes. The December 2019 Notes bore interest at 6.75% per annum.

2021 Note Exchange Effected in January 2020

In January 2020, the Company entered into the January 2020 Exchange Agreement (the "January 2020 Exchange Agreement") with Kingdon. Under the January 2020 Exchange Agreement, the Company issued a senior secured convertible note in the aggregate principal amount of approximately $3.0 million (the "January 2020 Note") in exchange for the cancellation of an aggregate of $3.0 million principal amount and accrued interest of the 2021 Note then owned by Kingdon.

December 2020 Exchange Agreement and Amendment to Facility Agreement, Notes and Investors' Rights Agreement

In December 2020, the Company entered into a December 2020 Exchange Agreement and Amendment to Facility Agreement, Notes and Investors' Rights Agreement (the "December 2020 Exchange Agreement"). with the Deerfield Lenders, DSC and Kingdon (collectively, the "Facility Agreement Note Holders"). Under the December 2020 Exchange Agreement, the Company and the Facility Agreement Note Holders agreed that the Company would make a cash pre-payment of a portion of principal amount of the Deerfield Convertible Note, the December 2019 Notes and the January 2020 Note to the Facility Agreement Note Holders (the "Debt Payment") equal to approximately $30.3 million, plus accrued interest and that the Company would issue shares of its Series B-2 Preferred Stock and warrants exercisable for shares of its common stock (the "Exchange Warrants"), in exchange for the cancellation of a portion of the principal amount of the Deerfield Facility Agreement (such transaction, the "December 2020 Exchange").

In anticipation of the Public Offering, and to meet the Nasdaq Listing Requirements, the Company agreed in December 2020 to restructure the December 2019 Notes and the January 2020 Note in the aggregate principal amount of $60.8 million and the Deerfield Note in the principal amount of $7.5 million (collectively the "the Facility Notes").

Under the terms of the December 2020 Exchange Agreement, the Company, on January 12, 2021, in connection with the closing of the Public Offering:

- Exchanged $31.5 million of the outstanding principal and accrued interest on the Facility Notes for (i) 31,476.98412 shares of Series B-2 Preferred Stock, and (ii) Exchange Warrants exercisable for 3,632,019 shares of the Company's common stock, and
- Made the Debt Payment, in partial repayment of the remaining outstanding principal and accrued interest of the Facility Notes.

Following the completion of these transactions, the aggregate balance of principal and accrued interest remaining outstanding under the Facility Notes was approximately $7.6 million. With respect to this remaining outstanding balance under the Facility Notes, the December 2020 Exchange Agreement amended the terms of that debt to provide that:

- The maturity date was amended to March 31, 2023, and the debt was prepayable upon specified conditions, and
- Interest would accrue at the rate of 6.75% per annum, payable quarterly, would be added to principal until June 30, 2021, and then be payable in cash thereafter.

The changes to the Facility Notes, under the December 2020 Exchange Agreement, were accounted for as a debt extinguishment as the cash flows immediately after the December 2020 Exchange Agreement were substantially different from the cash flows immediately prior to the December 2020 Exchange Agreement and while the Company was experiencing financial difficulties it was determined that the lender did not grant a concession. As such, a loss on extinguishment of debt is recorded in the consolidated statement of operations for the year ended December 31, 2021, and additional debt issuance costs related the new notes were capitalized and amortized using the effective interest method through the Payoff of Facility Agreement Notes (discussed below).

Payoff of Facility Agreement Notes and Termination of Facility Agreement

On February 8, 2021, the Company entered into a payoff letter with the Facility Agreement Note Holders, pursuant to which the Company agreed to pay off and thereby terminate the Facility Agreement.

Pursuant to the payoff letter, the Company paid a total of $8.0 million to the Facility Agreement Note Holders, representing the principal balance, accrued interest outstanding and a prepayment fee in repayment of the Company's outstanding obligations under the Facility Agreement.

Pursuant to the payoff letter, all outstanding indebtedness and obligations of the Company owing to the Facility Agreement Note Holders under the Facility Agreement have been paid in full. The Facility Agreement and the notes thereunder, as well as the security interests in the assets of the Company securing the Facility Agreement and note obligations, have been terminated. The Facility Agreement Note Holders will retain the warrants previously issued to them by the Company.

The Company determined the payoff letter met the liability derecognition threshold under ASC 405-20, *Liabilities - Extinguishment of Liabilities*, as the Company repaid the debt (and has been relieved of the related obligation) without entering into new debt with the Facility Agreement Note Holders and there is no other continuing debt with the Facility Agreement Note Holders. The payoff letter resulted in a loss on extinguishment of debt which is shown within other (expense) income in the statements of operations for the year ended December 31, 2021.

PPP Loan

On April 23, 2020, the Company received proceeds of $0.8 million from a loan under the Paycheck Protection Program ("PPP Loan") of the Coronavirus Aid, Relief, and Economic Security Act ("CARES Act"), a portion of which may be forgiven, which the Company used to retain current employees, maintain payroll and make lease and utility payments. In May 2021, the Company received notice from the U.S. Small Business Administration that the principal and interest due under its PPP Loan had been forgiven in full. This gain on extinguishment is shown, net of the loss on extinguishment of debt discussed above, within other (expense) income in the statements of operations for the year ended December 31, 2021.

Line of Credit

On May 31, 2022, the Company and Ameris Bank, as lender, entered into a $20.0 million revolving loan agreement (the "Line of Credit"). Proceeds of the revolving facility provided by the Line of Credit are to be used for general corporate purposes. Loans under the Line of Credit bear interest at the Secured Overnight Financing Rate ("SOFR") plus 1.60%, with a SOFR floor of 0.00%.

The revolving facility under the Line of Credit is secured by a perfected security interest in deposit accounts. The revolving facility under the Line of Credit is subject to customary affirmative and negative covenants.

The latest maturity date of the loans under the Line of Credit is May 31, 2025. The Line of Credit contains customary events of default that could lead to an acceleration of the loans, including cross-default, bankruptcy and payment defaults. As of December 31, 2022, the Company has drawn $12.8 million from the Line of Credit to finance the transactions under the Arimoclomol Purchase Agreement, and this amount is supported by a $12.8 million certificate of deposit which is shown as long-term investments - other in the consolidated balance sheets. The remaining $7.2 million under the Line of Credit is in a separate interest-bearing certificate of deposit and is also recorded as long-term investments - other in the consolidated balance sheet as of December 31, 2022. These certificates of deposit are pledged as collateral against the Line of Credit and cannot be redeemed so long as the $20.0 million remains available under the Line of Credit. The total value of the certificates of deposit held with Ameris Banks must meet or exceed the amount available to borrow under the Line of Credit so long as the Line of Credit remains active.

On January 26, 2023, the Company and Wells Fargo, as lender, entered into a margin account agreement under which the Company initially borrowed $12.8 million. The margin account bears interest at the Prime rate minus 225 basis-points.

On January 31, 2023, the Company repaid the $12.8 million outstanding under the Line of Credit in full, but the line of credit remains available for future use. As such, the certificates of deposit continue to be classified as long-term investments - other.

H. Commitments and Contingencies

Legal Matters

From time to time, the Company is involved in various legal proceedings arising in the normal course of business. For some matters, a liability is not probable, or the amount cannot be reasonably estimated and, therefore, an accrual has not been made. However, for such matters when it is probable that the Company has incurred a liability and can reasonably estimate the amount, the Company accrues and discloses such estimates. As of December 31, 2022, and 2021, no accruals have been made related to commitments and contingencies.

I. Preferred Stock and Warrants

Authorized, Issued, and Outstanding Preferred Stock

As of December 31, 2022, and 2021, the Company had 10,000,000 shares of authorized, unallocated and unissued preferred stock.

As of December 31, 2022, and 2021, no shares of preferred stock were designated, issued or outstanding.

Series B-2 Preferred Stock

Pursuant to the December 2020 Exchange Agreement, on January 12, 2021, the Company issued to the Facility Agreement Note Holders an aggregate of 31,476.98412 shares of its Series B-2 Preferred Stock and warrants exercisable for an aggregate of 3,632,019 shares of the Company's common stock (the "Exchange Warrants"). The Series B-2 Preferred Stock was convertible into an aggregate of 4,842,690 shares of the Company's common stock upon issuance. at a conversion price equal to $6.4999.

Amended and Restated Certificate of Designation of Preferences, Rights and Limitations of the Series B-2 Convertible Preferred Stock

On January 11, 2021, as a condition to the closing of the transactions contemplated by the December 2020 Exchange Agreement, the Company filed an Amended and Restated Certificate of Designation of Preferences, Rights and Limitations of Series B-2 Convertible Preferred Stock (the "Series B-2 Certificate of Designation") with the Secretary of State of the State of Delaware, setting forth the preferences, rights and limitations of the Series B-2 Preferred Stock.

Immediately following the closing of the Public Offering, pursuant to the terms of the December 2020 Exchange Agreement, the Company:

- exchanged approximately $31.5 million of the outstanding principal and accrued interest on the Facility Notes for (i) the Series B-2 Preferred Stock and (ii) the Exchange Warrants; and
- made a payment of approximately $30.3 million in partial repayment of the remaining outstanding principal and accrued interest on the Facility Notes.

In March 2021, all shares of Series B-2 Preferred Stock converted into 4,842,699 shares of common stock.

J. Common Stock and Warrants

Authorized, Issued, and Outstanding Common Shares

As of December 31, 2022, and 2021, the Company had authorized shares of common stock of 250,000,000 shares. Of the authorized shares, 35,450,257 and 35,325,801 shares of common stock were issued as of December 31, 2022, and 2021, respectively, and 34,540,304 and 35,005,640 shares of common stock were outstanding as of December 31, 2022, and 2021, respectively.

As of December 31, 2022, and 2021, the Company had reserved authorized shares of common stock for future issuance as follows:

	December 31,	
	2022	2021
Outstanding awards under equity incentive plans	2,456,407	1,273,879
Outstanding common stock warrants	4,252,600	4,252,600
Possible future issuances under equity incentive plans	4,421,508	4,209,935
Possible future issuances under employee stock purchase plan	1,417,365	1,500,000
Total common shares reserved for future issuance	12,547,880	11,236,414

Common Stock Activity

The following table summarizes common stock activity for the years ended December 31, 2022 and 2021:

	Shares of Common Stock
Balance as of January 1, 2021	4,537,321
Common stock issued as a result of the Public Offering	7,139,498
Common stock issued as a result of Series B-2 Preferred Stock conversions	4,842,699
Common stock issued as a result of warrant exercises	18,772,510
Common stock issued as compensation to third-parties	35,129
Adjustment for cash paid in lieu of DTC fractional shares	(1,356)
Common stock repurchased as a result of the Stock Repurchase Program	(320,161)
Balance as of December 31, 2021	35,005,640
Common stock issued as compensation to third-parties	41,821
Common stock repurchased as a result of the Stock Repurchase Program	(589,792)
Common stock issued as a result of the Employee Stock Purchase Plan	82,635
Balance as of December 31, 2022	34,540,304

Warrants

On June 2, 2014, pursuant to the terms of the Deerfield Facility Agreement, the Company issued the OTA Warrant to purchase 14,423,076 shares of Series D Preferred (Note G). The Company recorded the fair value of the OTA Warrant as a debt discount and a warrant liability. The OTA Warrant, if unexercised, expires on the earlier of June 2, 2024, or upon a liquidation event. Upon completion of the Company's initial public offering (the "IPO"), the OTA Warrant automatically converted into a warrant to purchase 1,923,077 shares of the Company's common stock. at an exercise price of $5.85 per share. After giving effect to the Reverse Stock Split effected in December 2020, the exercise price of the OTA Warrant became $93.60 and the shares of the Company's common stock issuable upon exercise of the OTA Warrant became 120,192 shares of common stock. As a result of the January 2021 Inducement Transaction (discussed below) the anti-dilution provisions within the OTA Warrant were triggered and the exercise price was reduced from $93.60 per share to $46.25 per share. In addition, as a result of the June 2021 Inducement Transaction (discussed below) the anti-dilution provision within the OTA Warrant were triggered and the exercise price was reduced from $46.25 per share to $38.34 per share. The Company amortized the debt discount over the term of the Deerfield Convertible Note and the expense was recorded as interest expense related to amortization of debt issuance costs and discount in the consolidated statements of operations. The Deerfield Convertible Note was extinguished in February 2021 and the remaining debt discount was written off and recognized as a loss on extinguishment of debt.

The Company determined that the OTA Warrant should be recorded as a liability and stated at fair value at each reporting period upon inception. As stated above, upon completion of the IPO, the OTA Warrant automatically converted into warrants to purchase the Company's common stock. The OTA Warrant remains classified as a liability and is recorded at fair value at each reporting period since it can be settled in cash. Changes to the fair value of the warrant liability are recorded through the consolidated statements of operations as a fair value adjustment (Note L).

In connection with the APADAZ License Agreement, in October 2018, the Company issued to KVK a warrant to purchase up to 31,250 shares of common stock of the Company at an exercise price of $2.30 per share (the "KVK Warrant"). The KVK Warrant is initially not exercisable for any shares of common stock. Upon the achievement of each of four specified milestones under the KVK Warrant, the KVK Warrant will become exercisable for an additional 7812.5 shares, up to an aggregate of 31,250 shares of the Company's common stock. The exercise price and the number and type of shares underlying the KVK Warrant are subject to adjustment in the event of specified events, including a reclassification of the Company's common stock, a subdivision or combination of the Company's common stock, or in the event of specified dividend payments. The KVK Warrant is exercisable until October 24, 2023. Upon exercise, the aggregate exercise price may be paid, at KVK's election, in cash or on a net issuance basis, based upon the fair market value of the Company's common stock at the time of exercise. After giving effect to the Reverse Stock Split effected in December 2020, the exercise price of the KVK Warrant became $36.80 and the shares of common stock issuable upon exercise of the KVK Warrant became 31,250 shares of common stock.

The Company determined that, since KVK qualifies as a customer under ASC 606, the KVK Warrant should be recorded as a contract asset and recognized as contra-revenue as the Company recognizes revenue from the APADAZ License Agreement. In addition, the Company determined that the KVK Warrant qualifies as a derivative under ASC 815 and should be recorded as a liability and stated at fair value each reporting period. The Company calculates the fair value of the KVK Warrant using a probability-weighted Black-Scholes option pricing model. Changes in fair value resulting from changes in the inputs to the Black Scholes model are accounted for as changes in the fair value of the derivative under ASC 815 and are recorded as fair value adjustment related to derivative and warrant liability in the consolidated statements of operations. Changes in the number of shares that are expected to be issued are treated as changes in variable consideration under ASC 606 and are recorded as a change in contract asset on the consolidated balance sheets.

Pre-Funded Warrants

On January 12, 2021, pursuant to the terms of the Underwriting Agreement, the Company issued pre-funded warrants to purchase 926,844 shares of the Company's common stock to specified investors in the Public Offering. Each pre-funded warrant had an initial exercise price per share equal to $0.0001. The pre-funded warrants were immediately exercisable. In January 2021, all pre-funded warrants were exercised for 926,841 shares of common stock and gross proceeds of approximately $72.

Warrants to Purchase Common Stock

On January 12, 2021, pursuant to the terms of the Underwriting Agreement and December 2020 Exchange Agreement, the Company issued warrants to purchase 12,078,361 shares of the Company's common stock (collectively, the "Offering Warrants") in the Public Offering and in connection with the transactions contemplated under the December 2020 Exchange Agreement. The Offering Warrants were immediately exercisable and expire on the fifth anniversary of their issuance date, at an exercise price per share of common stock equal to $6.50 per share. A holder (together with its affiliates) may not exercise any portion of the Offering Warrant to the extent that the holder and its affiliates would own more than 4.99% of the Company's outstanding common stock immediately after exercise. Except as otherwise provided in the Offering Warrants or by virtue of such holder's ownership of shares of the Company's common stock, the holders of the Offering Warrants do not have the rights or privileges of holders of common stock with respect to the shares of common stock underlying the Offering Warrants, including any voting rights, until they exercise their Offering Warrants. The Offering Warrants provide that holders have the right to participate in distributions or dividends paid on the Company's common stock. In the event of a fundamental transaction (as described in the Offering Warrants), the holders of the Offering Warrants will be entitled to receive upon exercise of the Warrants the kind and amount of securities, cash or other property that the holders would have received had they exercised the Offering Warrants immediately prior to such fundamental transaction. In addition, in the event of a fundamental transaction which is approved by the Company's board of directors, the holders of the Offering Warrants have the right to require the Company or a successor entity to redeem the Offering Warrants for cash in the amount of the Black Scholes value of the unexercised portion of the Offering Warrants on the date of the consummation of the fundamental transaction. In the event of a fundamental transaction which is not approved by the Company's board of directors, the holders of the Offering Warrants have the right to require the Company or a successor entity to redeem the Offering Warrants in the amount of the Black Scholes value of the unexercised portion of the Offering Warrants on the date of the consummation of the fundamental transaction payable in the form of consideration paid to the holders of common stock in such fundamental transaction. The Offering Warrants meet the equity classification requirements and thus are recorded in additional paid-in capital on the accompanying consolidated balance sheets. As of December 31, 2022, 3,461,858 Offering Warrants have been exercised for 3,030,881 shares of common stock and gross proceeds of approximately $16.9 million. These amounts are exclusive of the Offering Warrants exercised as part of the January 2021 Inducement Transaction discussed below.

Underwriter Warrant

On January 12, 2021, pursuant to the terms of the Underwriting Agreement, the Company issued to the Underwriter a warrant to purchase 806,932 shares of the Company's common stock (the "Underwriter Warrant"). The Underwriter Warrant is subject to substantially the same terms and conditions as the Warrants. If the Underwriter exercises any additional portion of its over-allotment option, then the Company shall issue the Underwriter an additional Underwriter Warrant exercisable for a number of shares of common stock equal to 5.0% of the number of shares of common stock issued in such over-allotment exercise (including the shares of common stock issuable upon the exercise of any Warrants issued in connection therewith). In connection with the closing of the Underwriter's partial exercise of its over-allotment option, on February 3, 2021, the Underwriter was issued an additional warrant to purchase 18,702 shares of common stock. As of December 31, 2022, 400,000 Underwriter Warrants have been exercised for 400,000 shares of common stock and gross proceeds of approximately $3.3 million.

January 2021 Warrant Exercise Inducement Letters and Issuance of Warrants

On January 26, 2021, the Company entered into warrant exercise inducement offer letters ("January 2021 Inducement Transaction") with certain holders of warrants issued in the Public Offering discussed above (the "Existing Warrants") (collectively, the "Exercising Holders") pursuant to which such holders agreed to exercise for cash their Existing Warrants to purchase 6,620,358 shares of the Company's common stock in exchange for the Company's agreement to issue new warrants to purchase up to 7,944,430 shares of the Company's common stock (the "January 2021 Inducement Warrants") on substantially the same terms as the Existing Warrants. The Company received aggregate gross proceeds of approximately $44.0 million from the exercise of the Existing Warrants by the Exercising Holders and the sale of the January 2021 Inducement Warrants. The Company engaged Roth as its exclusive placement agent in connection with these transactions and paid Roth a fee equal to 6% of gross proceeds from the exercise of the Existing Warrants by the Exercising Holders and the sale of the January 2021 Inducement Warrants.

The January 2021 Inducement Warrants were immediately exercisable and expire on the fifth anniversary of their issuance date. The January 2021 Inducement Warrants are exercisable, at the option of each holder, in whole or in part, by delivering to the Company a duly executed exercise notice, provided that payment in full for the number of shares of the Company's common stock purchased upon such exercise is delivered to the Company in accordance with the terms of the January 2021 Inducement Warrants. In lieu of making the cash payment otherwise contemplated to be made to the Company upon such exercise in payment of the aggregate exercise price, the holder may elect instead to receive upon such exercise (either in whole or in part) the net number of shares of common stock determined according to a formula set forth in the warrants. A holder (together with its affiliates) may not exercise any portion of the January 2021 Inducement Warrant to the extent that the holder and its affiliates would own more than 4.99% (or, upon election by a holder prior to the issuance of its January 2021 Inducement Warrants, 9.99%) of the Company's outstanding common stock immediately after exercise. Except as otherwise provided in the January 2021 Inducement Warrants or by virtue of such holder's ownership of shares of the Company's common stock, the holders of the January 2021 Inducement Warrants do not have the rights or privileges of holders of common stock with respect to the shares of common stock underlying the January 2021 Inducement Warrants, including any voting rights, until they exercise their January 2021 Inducement Warrants. The January 2021 Inducement Warrants provide that holders have the right to participate in distributions or dividends paid on the Company's common stock. In the event of a fundamental transaction (as described in the January 2021 Inducement Warrants), the holders of the January 2021 Inducement Warrants will be entitled to receive upon exercise of the January 2021 Inducement Warrants the kind and amount of securities, cash or other property that the holders would have received had they exercised the January 2021 Inducement Warrants immediately prior to such fundamental transaction. In addition, in the event of a fundamental transaction which is approved by the Company's board of directors, the holders of the January 2021 Inducement Warrants have the right to require the Company or a successor entity to redeem the January 2021 Inducement Warrants for cash in the amount of the Black Scholes value of the unexercised portion of the January 2021 Inducement Warrants on the date of the consummation of the fundamental transaction. In the event of a fundamental transaction which is not approved by the Company's board of directors, the holders of the January 2021 Inducement Warrants have the right to require the Company or a successor entity to redeem the January 2021 Inducement Warrants in the amount of the Black Scholes value of the unexercised portion of the January 2021 Inducement Warrants on the date of the consummation of the fundamental transaction payable in the form of consideration paid to the holders of common stock in such fundamental transaction. As of December 31, 2022, 1,676,921 January 2021 Inducement Warrants have been exercised for 1,676,921 shares of common stock and gross proceeds of approximately $10.7 million. These amounts are exclusive of the January 2021 Inducement Warrants exercised as part of the June 2021 Inducement Transaction discussed below.

In accordance with the January 2021 Inducement Transaction we recognized a deemed dividend of $37.4 million for the year ended December 31, 2021, which is the difference between the grant date fair value of the January 2021 Inducement Warrants and the purchase price of the January 2021 Inducement Warrants. This deemed dividend is added to net loss to arrive at net loss attributable to common stockholders on the consolidated statements of operations.

June 2021 Warrant Exercise Inducement Letters and Issuance of Warrants

On June 18, 2021, the Company entered into warrant exercise inducement offer letters ("June 2021 Inducement Transaction") with certain holders of warrants issued in the January 2021 Inducement Transaction discussed above (the "January 2021 Inducement Warrants") (collectively, the "June 2021 Exercising Holders") pursuant to which such holders agreed to exercise for cash their January 2021 Inducement Warrants to purchase 6,117,509 shares of the Company's common stock in exchange for the Company's agreement to issue new warrants to purchase up to 1,529,379 shares of the Company's common stock (the "June 2021 Inducement Warrants") on substantially the same terms as the January 2021 Inducement Warrants,. The Company received aggregate gross proceeds of approximately $39.1 million from the exercise of the January 2021 Inducement Warrants by the June 2021 Exercising Holders and the sale of the June 2021 Inducement Warrants. The Company engaged Roth as its exclusive placement agent in connection with these transactions and paid Roth a fee equal to 6% of gross proceeds from the exercise of the January 2021 Inducement Warrants by the June 2021 Exercising Holders and the sale of the June 2021 Inducement Warrants.

The June 2021 Inducement Warrants were immediately exercisable and expire on December 31, 2026. The June 2021 Inducement Warrants are exercisable, at the option of each holder, in whole or in part, by delivering to the Company a duly executed exercise notice, provided that payment in full for the number of shares of the Company's common stock purchased upon such exercise is delivered to the Company in accordance with the terms of the June 2021 Inducement Warrants. In lieu of making the cash payment otherwise contemplated to be made to the Company upon such exercise in payment of the aggregate exercise price, the holder may elect instead to receive upon such exercise (either in whole or in part) the net number of shares of common stock determined according to a formula set forth in the warrants. A holder (together with its affiliates) may not exercise any portion of the June 2021 Inducement Warrant to the extent that the holder and its affiliates would own more than 4.99% (or, upon election by a holder prior to the issuance of its June 2021 Inducement Warrants, 9.99%) of the Company's outstanding common stock immediately after exercise. Except as otherwise provided in the June 2021 Inducement Warrants or by virtue of such holder's ownership of shares of the Company's common stock, the holders of the June 2021 Inducement Warrants do not have the rights or privileges of holders of common stock with respect to the shares of common stock underlying the June 2021 Inducement Warrants, including any voting rights, until they exercise their June 2021 Inducement Warrants. The June 2021 Inducement Warrants provide that holders have the right to participate in distributions or dividends paid on the Company's common stock. In the event of a fundamental transaction (as described in the June 2021 Inducement Warrants), the holders of the June 2021 Inducement Warrants will be entitled to receive upon exercise of the June 2021 Inducement Warrants the kind and amount of securities, cash or other property that the holders would have received had they exercised the June 2021 Inducement Warrants immediately prior to such fundamental transaction. In addition, in the event of a fundamental transaction which is approved by the Company's board of directors, the holders of the June 2021 Inducement Warrants have the right to require the Company or a successor entity to redeem the June 2021 Inducement Warrants for cash in the amount of the Black Scholes value of the unexercised portion of the June 2021 Inducement Warrants on the date of the consummation of the fundamental transaction. In the event of a fundamental transaction which is not approved by the Company's board of directors, the holders of the June 2021 Inducement Warrants have the right to require the Company or a successor entity to redeem the June 2021 Inducement Warrants in the amount of the Black Scholes value of the unexercised portion of the June 2021 Inducement Warrants on the date of the consummation of the fundamental transaction payable in the form of consideration paid to the holders of common stock in such fundamental transaction. As of December 31, 2022, and 2021, no June 2021 Inducement Warrants have been exercised.

In accordance with the June 2021 Inducement Transaction, we recognized a deemed dividend of $16.9 million for the year ended December 31, 2021, which is the difference between the grant date fair value of the June 2021 Inducement Warrants and the purchase price of the June 2021 Inducement Warrants. This deemed dividend is added to net loss to arrive at net loss attributable to common stockholders on the consolidated statements of operations.

K. Stock-Based Compensation

The Company maintains a stock-based compensation plan (the “Incentive Stock Plan”) that governs stock awards made to employees and directors prior to completion of the IPO.

In November 2014, the Board of Directors of the Company ("the Board"), and in April 2015, the Company’s stockholders, approved the Company’s 2014 Equity Incentive Plan (the “2014 Plan”), which became effective in April 2015. The 2014 Plan provides for the grant of stock options, other forms of equity compensation, and performance cash awards. In June 2021, the Company's stockholders approved an Amended and Restated 2014 Equity Incentive Plan (the "A&R 2014 Plan"), following its adoption by the Board in April 2021, which among other things added 4,900,000 shares to the maximum number of shares of common stock to be issued under the plan and extended the annual automatic increases (discussed further below) until January 1, 2031 and eliminated individual grant limits that applied under the 2014 Plan to awards that were intended to comply with the exemption for "performance-based compensation" under Code Section 162(m). The maximum number of shares of common stock that may be issued under the A&R 2014 Plan is 6,889,885 as of December 31, 2022. The number of shares of common stock reserved for issuance under the A&R 2014 Plan will automatically increase on January 1 of each year, beginning on January 1, 2016, and ending on and including January 1, 2031, by 4% of the total number of shares of the Company’s capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by the Board. Pursuant to the terms of the A&R 2014 Plan, on January 1, 2023, the common stock reserved for issuance under the A&R 2014 Plan automatically increased by 1,381,612 shares.

During the years ended December 31, 2022, and 2021, no stock options were exercised.

In June 2021, the Company's stockholders approved an Employee Stock Purchase Plan (the "ESPP"), following its adoption by the Board in April 2021. The maximum number of shares of common stock that may be issued under the ESPP is 1,500,000. The first offering period under the ESPP began on October 1, 2021, and the first purchase date occurred on May 31, 2022. As of December 31, 2022, 82,635 shares have been issued under the ESPP.

In January 2023, our Board approved the 2023 Employment Inducement Award Plan (“2023 Plan”). The maximum number of shares of common stock that may be issued under the 2023 Plan is 1,500,000.

Stock-based compensation expense recorded under the Incentive Stock Plan, 2014 Plan, A&R 2014 Plan, and ESPP is included in the following line items in the accompanying consolidated statements of operations (in thousands):

	Year ended December 31,	
	2022	2021
Research and development	$ 1,443	$ 914
General and administrative	2,851	1,522
Total stock-based compensation expense	$ 4,294	$ 2,436

Stock Option Awards

The Company estimates the fair value of stock options using the Black-Scholes option-pricing model, which requires the use of subjective assumptions, including the expected term of the option, the expected stock price volatility, expected dividend yield and the risk-free interest rate for the expected term of the option. The expected term represents the period of time the stock options are expected to be outstanding. Due to the lack of sufficient historical exercise data to provide a reasonable basis upon which to otherwise estimate the expected term of the stock options, the Company uses the simplified method to estimate the expected term for its "plain vanilla" stock options. Under the simplified method, the expected term of an option is presumed to be the mid-point between the vesting date and the end of the contractual term. Some options, for example those that have exercise prices in excess of the fair value of the underlying stock, are not considered "plain vanilla" stock options. For these options, the Company uses an expected term equal to the contractual term of the option. Expected volatility is based on the Company's historical volatility over the estimated expected term of the stock options. The Company assumes no dividend yield because dividends are not expected to be paid in the near future, which is consistent with the Company's history of not paying dividends.

The Company recognizes compensation expense related to stock-based payment transactions upon satisfaction of the requisite service or vesting requirements. Forfeitures are estimated at the time of grant and revised based on actual forfeitures, if necessary, in subsequent periods if actual forfeitures differ from those estimates.

Using the Black-Scholes option-pricing model, the weighted-average fair value of awards granted during the years ended December 31, 22 and 2021, fair value was $5.01 and $7.53 per share, respectively. The assumptions used to estimate fair value are as follows:

	Year Ended December 31,	
	2022	**2021**
Risk-free interest rate	1.70% - 3.80%	0.92% - 1.55%
Expected term (in years)	5.50 - 7.00	5.50 - 10.00
Expected volatility	91.28% - 98.91%	96.91% - 102.57%
Expected dividend yield	0	0

The activity under the Incentive Stock Plan, 2014 Plan and A&R 2014 Plan for the year ended December 31, 2022, is summarized as follows:

	Number of Options	**Weighted Average Exercise Price**	**Weighted Avg Remaining Contractual Term**	**Aggregate Intrinsic Value**
Outstanding balance at January 1, 2022	1,273,879	$ 27.58	8.73	$ 254,207
Granted	1,434,245	$ 6.32		
Exercised or released	-	$ -		
Canceled or forfeited	245,593	$ 9.23		$ 74,033
Expired	6,124	$ 93.60		
Outstanding balance at December 31, 2022	2,456,407	$ 16.84	8.51	$ 11,819
Exercisable at December 31, 2022	615,001	$ 44.52	6.99	$ 2,722
Vested and expected to vest at December 31, 2022	2,375,700	$ 17.15	8.49	$ 11,628

Information regarding currently outstanding and exercisable options as of December 31, 2022, is as follows:

	Options Outstanding		**Options Exercisable**	
Exercise Price	**Number of Shares**	**Weighted Avg Remaining Contractual Term**	**Number of Shares**	**Weighted Avg Remaining Contractual Term**
$2.848 to $10.00	2,175,436	8.90	351,151	8.19
$10.01 to $30.00	69,375	8.17	69,375	8.17
$30.01 to $50.00	68,514	6.11	51,393	6.11
$50.01 to $70.00	41,866	4.08	41,866	4.08
$70.01 to $327.20	101,216	3.70	101,216	3.70
	2,456,407	8.51	**615,001**	6.99

The total fair value of stock options vested during the years ended December 31, 2022 and 2021, was $3.7 million and $2.0 million, respectively.

Unvested stock options as of December 31, 2022 and 2021, were as follows:

	Number of Unvested Options December 31,	
Exercise Price	**2022**	**2021**
$2.848 to $10.00	1,824,285	861,139
$10.01 to $30.00	-	60,000
$30.01 to $50.00	17,121	36,190
$50.01 to $70.00	-	-
$70.01 to $327.20	-	9,335
Total number of unvested stock options	**1,841,406**	**966,664**

As of December 31, 2022, there was $8.2 million of total unrecognized compensation cost related to unvested share-based compensation arrangements granted under the 2014 Plan and A&R 2014 Plan. That compensation cost is expected to be recognized over a weighted-average period of 2.82 years. There was $0.4 million and $0.4 million

of

stock-based compensation expense related to performance-based awards recognized during the years ended December 31, 2022, and 2021, respectively.

L. Fair Value of Financial Instruments

The accounting standard for fair value measurements provides a framework for measuring fair value and requires disclosures regarding fair value measurements. Fair value is defined as the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date, based on the Company's principal or, in absence of a principal, most advantageous market for the specific asset or liability.

The Company uses a three-tier fair value hierarchy to classify and disclose all assets and liabilities measured at fair value on a recurring basis, as well as assets and liabilities measured at fair value on a non-recurring basis, in periods subsequent to their initial measurement. The hierarchy requires the Company to use observable inputs when available, and to minimize the use of unobservable inputs, when determining fair value. The three tiers are defined as follows:

- Level 1—Observable inputs that reflect quoted market prices (unadjusted) for identical assets or liabilities in active markets;
- Level 2—Observable inputs other than quoted prices in active markets that are observable either directly or indirectly in the marketplace for identical or similar assets and liabilities; and
- Level 3—Unobservable inputs that are supported by little or no market data, which require the Company to develop its own assumptions.

The carrying amounts of certain financial instruments, including cash and cash equivalents and accounts payable and accrued expenses, approximate thei respective fair values due to the short-term nature of such instruments.

Assets and Liabilities Measured at Fair Value on a Recurring Basis

The Company evaluates its financial assets and liabilities subject to fair value measurements on a recurring basis to determine the appropriate level in which to classify them for each reporting period. This determination requires significant judgments to be made. The following table summarizes the conclusions reached regarding fair value measurements as of December 31, 2022 and 2021 (in thousands):

	Balance at December 31, 2022	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
OTA Warrant liability	$ 1	$ -	$ -	$ 1
Total liabilities	$ 1	$ -	$ -	$ 1
Securities:				
U.S. government-sponsored agency securities	$ 7,189	$ -	$ 7,189	$ -
U.S. Treasury securities	9,711	9,711	-	-
Total assets	$ 16,900	$ 9,711	$ 7,189	$ -

	Balance at December 31, 2021	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
OTA Warrant liability	$ 288	$ -	$ -	$ 288
Embedded Warrant Put Option	18	-	-	18
KVK Warrant liability	24	-	24	-
Total liabilities	$ 330	$ -	$ 24	$ 306

The Company's OTA Warrant liability, embedded Warrant Put Option and securities are measured at fair value on a recurring basis. As of December 31, 2022, and 2021, the OTA Warrant liability and embedded Warrant Put Option are reported on the accompanying consolidated balance sheets in derivative and warrant liability. As of December 31, 2022, and 2021, the securities are reported on the accompanying consolidated balance sheets in short-term and long-term investments. The Company used a Monte Carlo simulation to value the OTA Warrant liability and embedded Warrant Put Option for all periods presented herein. Significant unobservable inputs used in measuring the fair value of these financial instruments included the Company's estimated enterprise value, an estimate of the timing of a liquidity or fundamental change event and a present value discount rate. Changes in the fair value of the OTA Warrant liability and embedded Warrant Put Option are reflected in the accompanying consolidated statements of operations for the years ended December 31, 2022, and 2021, as a fair value adjustment related to derivative and warrant liability.

The derivative liability for the OTA Warrant was $1,000 and $288,000 at December 31, 2022, and 2021, respectively. The derivative liability for the embedded Warrant Put Option was $0 and $18,000 at December 31, 2022, and 2021, respectively. A 10% increase in the enterprise value would result in a negligible increase in the estimated fair value of the OTA Warrant liability and the estimated fair value of the embedded Warrant Put Option liability. In addition, the Company assumed a weighted-average probability of a liquidity event occurring of approximately 25% with an estimated probability-weighted value of approximately $30.0 million and a weighted-average probability of a fundamental change event occurring of approximately 28% with an estimated probability-weighted value of approximately $379 million, respectively, with estimated timing in each scenario of the fourth quarter of 2023.

The Company's KVK Warrant liability is measured at fair value on a recurring basis. As of December 31, 2022, and 2021, the KVK Warrant liability is reported on the accompanying consolidated balance sheets in derivative and warrant liability. The Company estimates the fair value of the KVK Warrant using a probability-weighted Black-Scholes option-pricing model, which requires the use of subjective assumptions, including the expected term of the warrant, the expected stock price volatility, expected dividend yield and the risk-free interest rate for the expected term of the warrant. The expected term represents the period of time the warrant is expected to be outstanding. For the KVK Warrant, the Company used an expected term equal to the contractual term of the warrant. Expected volatility is based on the Company's historical volatility since the IPO. The Company assumes no dividend yield because dividends are not expected to be paid in the near future, which is consistent with the Company's history of not paying dividends. Changes in the fair value of the KVK Warrant liability are reflected in the consolidated statements of operations for the years ended December 31, 2022, and 2021, as a fair value adjustment related to derivative and warrant liability.

A reconciliation of the beginning and ending balances for the derivative and warrant liability measured at fair value on a recurring basis using significan unobservable inputs (Level 3) is as follows (in thousands):

	2022	2021
Balance as of beginning of period	$ 306	$ 255
Adjustment to fair value	(305)	51
Balance as of end of period	$ 1	$ 306

M. Income Taxes

The Company's financial statements include a total state tax expense of $31,000 and state tax benefit of $34,000 related to research and development credits on a loss before income taxes of approximately $42.3 million and $8.5 million for the years ended December 31, 2022 and 2021, respectively. The Company met the requirements to receive a tax credit of $0.8 million for losses in Denmark resulting from research and development costs, which is included in income tax benefit for the year ended December 31, 2022. A reconciliation of the difference between the (expense)/benefit for income taxes and income taxes at the statutory U.S. federal income tax rate is as follows (in thousands, except amounts pertaining to rate which are shown as a percentage):

	Year ended December 31,	
	2022	**2021**
Federal statutory rate	21.00%	21.00%
Effect of:		
Change in valuation allowance	(22.06)	(28.39)
Return to provision and deferred true-up	(3.41)	(0.26)
Federal research and development credit	1.16	-
State research and development credit	0.07	-
Change in rate	0.51	3.00
State tax benefit (net of federal)	3.03	5.14
Warrant liability	0.16	(0.06)
Stock-based compensation	(0.69)	(2.68)
PPP Loan forgiveness	-	1.94
Global intangible low-taxed income credit	(1.22)	-
Foreign research and development excess benefit	3.16	-
Other	0.15	(0.08)
Federal income tax provision effective rate	1.86%	(0.39)%

The components of deferred tax assets and liabilities are as follows (in thousands):

	December 31,	
	2022	**2021**
Deferred tax assets relating to:		
Net operating loss carryforwards	$ 66,373	$ 60,977
Research and development tax carryforward	6,900	6,411
Stock-based compensation	4,185	4,807
174 expenses	3,730	-
Right-of-use liability	359	424
Property and equipment	22	14
Other deferred tax assets	598	288
Total gross deferred tax assets	82,167	72,921
Deferred tax liabilities relating to:		
Right-of-use asset	(336)	(401)
Other deferred tax liabilities	-	(9)
Total gross deferred tax liabilities	(336)	(410)
Deferred tax assets less liabilities	81,831	72,511
Valuation allowance	(81,831)	(72,511)
Net deferred tax asset (liability)	$ -	$ -

In assessing the realizability of deferred tax assets, management considers whether it is more likely than not that some portion or all of the deferred tax assets will not be realized. The ultimate realization of deferred tax assets is dependent upon the generation of future taxable income during the periods in which those temporary differences become deductible. Management considers the scheduled reversal of deferred tax liabilities, projected future taxable income and tax planning strategies in making this assessment. Based upon the level of historical taxable income (losses) and projections for future taxable income (losses) over the periods in which the deferred tax assets are deductible, management believes it is more likely than not that the Company will not realize the benefits of these deductible differences in the future.

The Company recorded certain retainable research and development tax credits as income tax benefit in the consolidated statement of operations for the year ended December 31, 2022. These refundable tax credits are a result of increased qualified research and development spending in certain jurisdictions which allow for a refundable credit even when the Company has no current period income tax expense.

In accordance with the Tax Cuts and Jobs Act of 2017, the Company evaluated its plans for reinvestment or repatriation of current and future earnings of foreign operations and determined to indefinitely reinvest current and future earnings of foreign operations in the foreign operation. The Company has not repatriated funds to the U.S. to satisfy domestic liquidity needs, nor does the Company anticipate the need to do so. If in the foreseeable future, the Company can no longer demonstrate that these earnings are indefinitely reinvested, a deferred tax liability will be recognized.

The Company has federal operating loss carryforwards totaling $236.0 million, $138.1 million of which, if not utilized, will begin to expire in 2027 and $97.9 million of which have no expiration date.

The Company also has certain state net operating loss carryforwards totaling $225.2 million, which, if not utilized, will begin to expire in 2027. Due to potential ownership changes that may have occurred or would occur in the future, Internal Revenue Code Section 382 may place additional limitations on the Company’s ability to utilize the net operating loss carryforward.

ASC 740-10, *Accounting for Uncertainty in Income Taxes*, uses the term “more likely than not” to evaluate whether or not a tax position will be sustained upon examination. The Company has not identified any tax positions that do not meet the more likely than not threshold.

On March 27, 2020, the CARES Act was enacted into law in response to the COVID-19 pandemic. The Company has evaluated the various income and payroll tax provisions and expects little or no impact to income tax expense.

N. Net Loss Per Share

For all periods presented herein, the Company did not use the two-class method to compute net loss attributable to common stockholders per share of common stock, even though it had issued securities, other than common stock, that contractually entitled the holders to participate in dividends and earnings, because these holders are not obligated to participate in a loss. The two-class method requires earnings for the period to be allocated between common stock and participating securities based upon their respective rights to receive distributed and undistributed earnings.

Under the two-class method, for periods with net income attributable to common stockholders, basic net income attributable to common stockholders per share of common stock is computed by dividing the undistributed net income attributable to common stockholders by the weighted average number of shares of common stock outstanding during the period. Undistributed net income attributable to common stockholders is computed by subtracting from net income the portion of current period earnings that participating securities would have been entitled to receive pursuant to their dividend rights had all of the period's earnings been distributed and subtracting the actual or deemed dividends declared. No such adjustment to earnings is made during periods with a net loss as the holders of the participating securities have no obligation to fund losses. Diluted net income attributable to common stockholders per share of common stock is computed under the two-class method by using the weighted average number of shares of common stock outstanding plus the potential dilutive effects of stock options and warrants. In addition to analyzing under the two class method, the Company analyzes the potential dilutive effect of stock options and warrants, under the treasury-stock method when calculating diluted income (loss) attributable to common stockholders per share of common stock, in which it is assumed that the stock options and warrants convert into common stock at the beginning of the period or date of issuance, if the stock option or warrant was issued during the period. The Company reports the more dilutive of the approaches (two-class or treasury-stock/if-converted) as its diluted net income (loss) attributable to common stockholders per share of common stock during the period.

As noted above, for all periods presented herein, the Company did not utilize the two-class approach as the Company was in a net loss position and the holders of the participating securities have no obligation to fund losses. The Company did analyze diluted net loss attributable to common stockholders per share of common stock under the treasury-stock/if-converted method and noted that all outstanding stock options and warrants were anti-dilutive for the periods presented. For all period presented, basic net loss attributable to common stockholders per share of common stock was the same as diluted net loss attributable to common stockholders per share of common stock.

The following securities, presented on a common stock equivalent basis, have been excluded from the calculation of weighted average number of shares of common stock outstanding because their effect is anti-dilutive:

	December 31,	
	2022	**2021**
Awards under equity incentive plans	2,456,407	1,273,879
Common stock warrants	4,252,600	4,221,350
Total securities excluded from the calculation of weighted average number of shares of common stock outstanding	6,709,007	5,495,229

A reconciliation from net loss to basic net loss attributable to common stockholders per share of common stock and diluted net loss attributable to common stockholders per share of common stock for the years ended December 31, 2022, and 2021, is as follows (in thousands):

	December 31,	
	2022	**2021**
Basic and diluted net loss per share of common stock:		
Net loss	$ (41,543)	$ (8,555)
Less: Dividends declared or accumulated	-	(54,342)
Net loss attributable to common stockholders	$ **(41,543)**	$ **(62,897)**
Less: Net loss attributable to participating securities	$ -	$ -
Undistributed net loss attributable to shares of common stockholders, basic and diluted	$ **(41,543)**	$ **(62,897)**
Weighted average number of shares of common stock outstanding, basic and diluted	34,489	29,766
Basic and diluted net loss attributable to common stockholders per share of common stock	$ (1.20)	$ (2.11)

O. Leases

The Company has operating and finance leases for office space, laboratory facilities and various laboratory equipment, furniture and office equipment and leasehold improvements. The Company determines if an arrangement is a lease at contract inception. Lease assets and lease liabilities are recognized based on the present value of lease payments over the lease term at the commencement date. The Company does not separate lease and non-lease components. Leases with a term of 12 months or less at commencement are not recorded on the consolidated balance sheets. Lease expense for these arrangements is recognized on a straight-line bases over the lease term. The Company's leases have remaining lease terms of less than 1 year to approximately 4 years, some of which include options to extend the leases for up to 5 years, and some which include options to terminate the leases within 1 year.

Effective June 1, 2021, the Company agreed to sublease office space in Florida, comprised of one of the two contiguous suites, under a non-cancelable operating lease, which expires in February 2026.

The components of lease expense were as follows (in thousands):

	Year Ended December 31,	
Lease Cost	**2022**	**2021**
Finance lease cost:		
Amortization of right-of-use assets	$ 129	$ 129
Interest on lease liabilities	2	9
Total finance lease cost	131	138
Operating lease cost	418	365
Short-term lease cost	210	199
Variable lease cost	39	47
Less: sublease income	(157)	(91)
Total lease costs	$ 641	$ 658

Supplemental cash flow information related to leases was as follows (in thousands):

	Year Ended December 31,	
	2022	**2021**
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows from finance leases	$ 2	$ 9
Financing cash flows from finance leases	16	173
Operating cash flows from operating leases	522	460
Operating cash flows from short-term leases	210	199
Operating cash flows from variable lease costs	39	47
Right-of-use assets obtained in exchange for lease liabilities:		
Finance leases	$ -	$ -
Operating leases	123	-

Supplemental balance sheet information related to leases was as follows (in thousands, except weighted average remaining lease term and weighted average discount rate):

	December 31,	
	2022	2021
Finance Leases		
Property and equipment, at cost	$ 1,031	$ 1,031
less: accumulated depreciation and amortization	(780)	(651)
Property and equipment, net	$ 251	$ 380
Other current liabilities	$ 6	$ 15
Other long-term liabilities	-	6
Total finance lease liabilities	$ 6	$ 21
Operating Leases		
Operating lease right-of-use assets	$ 988	$ 1,141
Total operating lease right-of-use assets	$ 988	$ 1,141
Current portion of operating lease liabilities	$ 480	$ 356
Operating lease liabilities, less current portion	843	1,232
Total operating lease liabilities	$ 1,323	$ 1,588
Weighted Average Remaining Lease Term		
Finance leases	1 year	1 year
Operating leases	3 years	4 years
Weighted Average Discount Rate		
Finance leases	14.3%	12.0%
Operating leases	7.3%	7.5%

Maturities on lease liabilities were as follows (in thousands):

Year Ending December 31,	Finance Leases	Operating Leases
2023	$ 7	$ 563
2024	-	488
2025	-	389
2026	-	30
2027	-	-
Thereafter	-	-
Total lease payments	7	1,470
Less: future interest expense	(1)	(147)
Lease liabilities	$ 6	$ 1,323

P. Employee Benefit Plan

The Company has a 401(k) retirement plan (the "401(k) Plan") that covers substantially all employees. The Company may provide a discretionary match with a maximum amount of 4% of the participant's compensation, which vests immediately. The Company made matching contributions under the 401(k) Plan of $197,000 and $164,000 for the years ended December 31, 2022, and 2021, respectively.

The Company has a discretionary profit sharing plan (the "Profit Sharing Plan") that covers all employees. Employees become eligible participants in the Profit Sharing Plan once they have provided three years of service to the Company. The Company made no contributions to the Profit Sharing Plan in 2022, or 2021.

Q. Significant Events

On January 6, 2023, the Board appointed Richard W. Pascoe to serve as the Company's Chief Executive Officer, effective immediately. Concurrently with his appointment as Chief Executive Officer, Mr. Pascoe stepped down as the Company's Executive Chairman. Mr. Pascoe will continue to serve as a member of the Board. Mr. Pascoe has been designated as the Company's principal executive officer, succeeding Travis C. Mickle, Ph.D., the Company's President and former Chief Executive Officer, in such role. On January 6, 2023, Dr. Mickle resigned his role (i) as Chief Executive Officer, effective immediately, and (ii) as President and as a member of the Board, in each case, effective as of the date of the Company's 2023 Annual Meeting of Stockholders. Additionally, the Board has appointed Matthew R. Plooster, a member of the Board, as the Chairman of the Board.

In connection with Mr. Pascoe's appointment as the Company's Chief Executive Officer, the Company and Mr. Pascoe entered into an amendment to the employment agreement, dated November 5, 2021, by and between the Company and Mr. Pascoe (the "Amendment"). Pursuant to the Amendment, Mr. Pascoe became entitled to receive an option under the A&R 2014 Plan to purchase 700,000 shares of the Company's common stock at an exercise price equal to the closing price of the Company's common stock on January 9, 2023. The option will vest in four equal annual installments, with the first such installment occurring on January 6, 2024 (subject to Mr. Pascoe's continued service to the Company through the applicable vesting date).

In connection with the management transition, the Company entered into (i) a transition agreement with Dr. Mickle (the "Transition Agreement") and (ii) a consulting agreement with Dr. Mickle (the "Consulting Agreement"). Pursuant to the terms of the Transition Agreement, subject to his timely delivering a release of claims in the Company's favor, Dr. Mickle will receive severance payments and benefits consisting of (i) continued payment of his base salary for 18 months following the date on which Dr. Mickle's employment with the Company ends (the "Separation Date"), (ii) up to 18 months of continued medical, dental and vision coverage pursuant to COBRA and (iii) a one-time, lump sum bonus payment equal to a pro rata amount of his annual performance-based target bonus for the year in which the Separation Date occurs. In addition, immediately prior to the Separation Date, all outstanding options to purchase the Company's common stock held by Dr. Mickle will be vested in full, and such accelerated vested options may be exercised through the later of (i) the 18-month anniversary of the date of the Transition Agreement and (ii) the date of the termination of the Consulting Agreement. Pursuant to the terms of the Consulting Agreement, Dr. Mickle has agreed to provide consulting services until the first anniversary of the Company's 2023 Annual Meeting of Stockholders. In exchange for such services, Dr. Mickle will receive consulting fees of $40,000 per month. In addition, Dr. Mickle was granted, under the A&R 2014 Plan, 547,945 performance-based restricted stock units, which will vest in full upon the timely achievement of a clinical and development milestone, subject to forfeiture upon certain disqualifying events.

R. Subsequent Events

The Company evaluated events and transactions occurring subsequent to December 31, 2022, through March 7, 2023, the date the accompanying financial statements were issued. During this period, there were no subsequent events, other than the debt activity discussed in Note G, the adoption of the 2023 Plan discussed in Note K, and the significant events disclosed in Note Q, that required recognition in the accompanying consolidated financial statements nor were there any additional nonrecognized subsequent events that required disclosure.

EXHIBITS

Exhibit No.	Description
3.1	Amended and Restated Certificate of Incorporation of Zevra Therapeutics, Inc.
3.1.1	Certificate of Amendment of Amended and Restated Certificate of Incorporation of the Company, effective as of December 23, 2020 (incorporated herein by reference to Registrant's Current Report on Form 8-K as filed with the SEC on December 23, 2020).
3.1.2	Certificate of Elimination of Series A Convertible Preferred Stock of Zevra Therapeutics, Inc. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on June 23, 2021).
3.1.3	Certificate of Elimination of Series B-1 Convertible Preferred Stock of Zevra Therapeutics, Inc. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on June 23, 2021).
3.1.4	Certificate of Elimination of Series B-2 Convertible Preferred Stock of Zevra Therapeutics, Inc. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on June 23, 2021).
3.1.5	Certificate of Amendment of Amended and Restated Certificate of Incorporation of Zevra Therapeutics, Inc. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on February 24, 2023).
3.2	Amended and Restated Bylaws, as currently in effect, of Zevra Therapeutics, Inc. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on February 24, 2023).
4.1	Specimen stock certificate evidencing shares of Common Stock (incorporated herein by reference to the Registrant's Annual Report on Form 10-K as filed with the SEC on March 12, 2021).
4.2	Form of Common Stock Purchase Warrant and schedule of holders (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on January 13, 2021).
4.3	Form of Inducement Warrant (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on January 26, 2021).
4.4	Form of Inducement Warrant (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on June 23, 2021).
4.5*	Description of the Registrant's Securities Registered Pursuant to Section 12 of the Securities Exchange Act of 1934.
10.1+	Material Supply Agreement, by and between the Registrant and Johnson Matthey, Inc., dated as of November 2, 2009 (incorporated by reference Registrant's Amendment No. 1 to Registration Statement on Form S-1/A (File No. 333-202660) as filed with the SEC on April 3, 2015).
10.2	Payoff letter, dated as of February 8, 2021, by and among the Company and the lenders named therein (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on February 9, 2021).
10.3	Second Amendment to Senior Secured Convertible Note and Warrant, by and between Registrant and Deerfield Private Design Fund III, L.P., dated January 6, 2016 (incorporated by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on January 11, 2016).
10.3.1	Fourth Amendment to Senior Secured Convertible Note and Warrant, effective as of October 3, 2016, by and between the Registrant and Deerfield Private Design Fund III, L.P. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on October 3, 2016).
10.3.2	Amendment to Convertible Note and Warrant Agreement, dated November 20, 2018, between the Company and Deerfield Private Design Fund III, L.P. (as incorporated herein by reference to the Company's Current Report on Form 8-K filed with the SEC on November 20, 2018).
10.3.3	Seventh Amendment to Senior Secured Convertible Note and Sixth Amendment to Warrant, dated February 28, 2019, between the Company and Deerfield Private Design Fund III, L.P. (incorporated herein by reference to the Registrant's Annual Report on Form 10-K as filed with the SEC on March 1, 2019).
10.3.4	Amendment to Senior Secured Convertible Notes and Amendment to Warrant, dated as of February 17, 2020, by and among Registrant and the noteholders party thereto (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on February 18, 2020)
10.3.5	Amendment to Senior Secured Convertible Notes and Amendment to Warrant, dated as of January 12, 2021, by and among the Company, Deerfield Private Design Fund III, L.P. and Deerfield Special Situations Fund, L.P.(incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on January 13, 2021)
10.4	Warrant to Purchase Shares of Series D Preferred Stock issued to Deerfield Private Design Fund III, L.P., dated as of June 2, 2014 (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 (File No. 333-202660) as filed with the SEC on March 11, 2015).
10.4.1	Form of Stock Purchase Warrant to purchase shares of Series D Convertible Preferred Stock issued in bridge financing, along with a schedule of warrant holders (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 (File No. 333-202660) as filed with the SEC on March 11, 2015).

EXHIBITS, CONTINUED

Exhibit No.	Description
10.5	Amended and Restated Investors' Rights Agreement, dated as of February 19, 2015, by and among the Registrant and certain of its stockholders (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 (File No. 333-202660) as filed with the SEC on March 11, 2015).
10.6+	Agreement to Terminate CLA, by and between MonoSol Rx, LLC and the Registrant, dated as of March 20, 2012 (incorporated herein by reference to the Registrant's Amendment No. 1 to Registration Statement on Form S-1/A (File No. 333-202660) as filed with the SEC on April 3, 2015).
10.7#	Incentive Stock Plan, as amended to date (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 (File No. 333-202660) as filed with the SEC on March 11, 2015).
10.7.1#	Form of Incentive Stock Option Agreement under Incentive Stock Plan (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 (File No. 333-202660) as filed with the SEC on March 11, 2015).
10.7.2#	Form of Nonqualified Stock Option Agreement under Incentive Stock Plan (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 (File No. 333-202660) as filed with the SEC on March 11, 2015).
10.7.3#	Amended and Restated 2014 Equity Incentive Plan (incorporated herein by reference to the Registrant's Quarterly Report on Form 10-Q as filed with the SEC on August 13, 2021).
10.7.4#	Form of Stock Option Grant Notice and Stock Option Agreement under 2014 Equity Incentive Plan (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 File No. 333-202660) as filed with the SEC on March 11, 2015).
10.7.5#	Form of Restricted Stock Unit Grant Notice and Restricted Stock Unit Agreement under 2014 Equity Incentive Plan (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 File No. 333-202660) as filed with the SEC on March 11, 2015).
10.7.6#	Form of Restricted Stock Award Grant Notice and Restricted Stock Award Agreement under 2014 Equity Incentive Plan (incorporated herein by reference to the Registrant's Quarterly Report on Form 10-Q as filed with the SEC on May 14, 2019)
10.7.7#	2021 Employee Stock Purchase Plan (incorporated herein by reference to the Registrant's Quarterly Report on Form 10-Q as filed with the SEC on August 13, 2021).
10.7.8*#	2023 Employment Inducement Award Plan and forms of award agreements thereunder.
10.8*#	Eighth Amended and Restated Non-Employee Director Compensation Policy effective February 15, 2023.
10.9#	Form of Indemnification Agreement with the Registrant's directors and executive officers (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 (File No. 333-202660) as filed with the SEC on March 11, 2015).
10.10#	Amended and Restated Employment Agreement by and between the Registrant and R. LaDuane Clifton, dated as of June 25, 2015 (incorporated herein by reference to the Registrant's Quarterly Report on Form 10-Q as filed with the SEC on August 14, 2015).
10.10.1#	Amendment to Amended and Restated Employment Agreement by and between the Registrant and R. LaDuane Clifton, dated as of October 13, 2015 (incorporated herein by reference to the Registrant's Quarterly Report on Form 10-Q as filed with the SEC on November 13, 2015).
10.11#	Employment Agreement by and between the Registrant and Christal M.M. Mickle, dated as of May 30, 2014 (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 (File No. 333-202660) as filed with the SEC on March 11, 2015).
10.11.1#	Amendment to Employment Agreement by and between the Registrant and Christal M.M. Mickle, dated as of October 13, 2015 (incorporated herein by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q filed with the SEC on November 13, 2015).
10.12#	Employment Agreement by and between the Registrant and Travis C. Mickle, Ph.D., dated as of May 30, 2014 (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 (File No. 333-202660) as filed with the SEC on March 11, 2015).
10.12.1#	Amendment to Employment Agreement by and between the Registrant and Travis C. Mickle, Ph.D., dated as of October 13, 2015 (incorporated herein by reference to the Registrant's Quarterly Report on Form 10-Q filed with the SEC on November 13, 2015).
10.12.2#	Transition Agreement by and between the Registrant and Travis C. Mickle, Ph.D., dated as of January 4, 2023, as amended (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on January 9, 2023).
10.12.3#+	Consulting Agreement by and between the Registrant and Travis C. Mickle, Ph.D., dated as of January 6, 2023 (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on January 9, 2023).
10.13#	Amended and Restated Employment Agreement by and between the Registrant and Sven Guenther, dated as of April 13, 2016 (incorporated herein by reference to the Registrant's Quarterly Report on Form 10-Q as filed with the SEC on May 13, 2016).
10.14#	Executive Employment Agreement by and between the Registrant and Richard W. Pascoe, dated as of November 5, 2021 (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on November 10, 2021).
10.14.1#	Amendment to Executive Employment Agreement by and between the Registrant and Richard W. Pascoe, dated as of January 6, 2023 (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on January 9, 2023).

EXHIBITS, CONTINUED

Exhibit No.	Description
10.15	Lease Agreement, by and between the Registrant and BRE/COH FL LLC, dated as of November 3, 2014 (incorporated herein by reference to the Registrant's Annual Report on Form 10-K as filed with the SEC on March 10, 2017).
10.15.1	First Amendment to the Lease Agreement, by and between the Registrant and BRE/COH FL LLC, dated as of April 21, 2015 (incorporated herein by reference to the Registrant's Annual Report on Form 10-K as filed with the SEC on March 10, 2017).
10.15.2	Second Amendment to the Lease Agreement, by and between the Registrant and BRE/COH FL LLC, dated as of December 22, 2015 (incorporated herein by reference to the Registrant's Annual Report on Form 10-K as filed with the SEC on March 10, 2017).
10.15.3	Third Amendment to the Lease Agreement, by and between the Registrant and BRE/COH FL LLC, dated as of July 15, 2016 (incorporated herein by reference to the Registrant's Annual Report on Form 10-K as filed with the SEC on March 10, 2017).
10.16+	Collaboration and License Agreement by and between the Company and KVK Tech, Inc. dated as of October 25, 2018 (incorporated herein by reference to the Registrant's Annual Report on Form 10-K as filed with the SEC on March 1, 2019).
10.16.1+	Warrant to Purchase Shares of Common Stock issued to KVK Tech, Inc. dated October 25, 2018 (incorporated herein by reference to the Registrant's Annual Report on Form 10-K as filed with the SEC on March 1, 2019).
10.17	Purchase Agreement, dated February 17, 2020, by and between the Registrant and Lincoln Park Capital Fund, LLC. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on February 18, 2020).
10.18+	Collaboration and License Agreement, dated as of September 3, 2019, by and between the Registrant and Boston Pharmaceuticals Holdings SA (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on September 4, 2019).
10.19+	Amendment No. 1 to Collaboration and License Agreement, effective as of April 8, 2021, by and between the Company and Commave Therapeutics SA (formerly known as Boston Pharmaceuticals Holdings SA) (incorporated herein by reference to the Registrant's Quarterly Report on Form 10-Q as filed with the SEC on August 13, 2021).
10.20	Form of Inducement Letter (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on January 26, 2021).
10.21	Form of Inducement Letter (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on June 23, 2021).
10.22	Equity Distribution Agreement, dated July 2, 2021, by and among the Company, JMP Securities LLC and RBC Capital Markets, LLC (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on July 2, 2021).
10.23†	Asset Purchase Agreement by and among the Registrant, Zevra Denmark A/S and Orphazyme A/S, in restructuring, dated May 15, 2022 (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on May 16, 2022).
10.24	Revolving Loan Agreement dated May 31, 2022 by and among the Registrant and Ameris Bank, as lender (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on June 1, 2022).
16.1	Letter from RSM US LLP dated June 17, 2022 (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on June 17, 2022).
23.1*	Consent of RSM US LLP, Independent Registered Public Accounting Firm.
23.2*	Consent of Ernst & Young LLP, Independent Registered Public Accounting Firm.
24.1*	Power of Attorney (included on signature page).
31.1*	Certification of the Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as amended.
31.2*	Certification of the Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as amended.
32.1**	Certification of the Principal Executive Officer pursuant to Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, and 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2**	Certification of the Principal Financial Officer pursuant to Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, and 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.

EXHIBITS, CONTINUED

Exhibit No.	Description
101.INS*	Inline XBRL Instance Document.
101.SCH*	Inline XBRL Taxonomy Extension Schema Document.
101.CAL*	Inline XBRL Taxonomy Extension Calculation Linkbase Document.
101.DEF*	Inline XBRL Taxonomy Extension Definition Linkbase Document.
101.LAB*	Inline XBRL Taxonomy Extension Label Linkbase Document.
101.PRE*	Inline XBRL Taxonomy Extension Presentation Linkbase Document.
104	Cover Page Interactive Data File (embedded within the Inline XBRL and contained in Exhibit 101)

*	Filed herewith
**	Furnished herewith
#	Indicates management contract or compensatory plan.
+	Certain portions of the exhibit, identified by the mark, "[*]", have been omitted because such portions contained information that is both (i) not material and (ii) the type that the Registrant treats as private or confidential.
†	Schedules have been omitted pursuant to Item 601(a)(5) of Regulation S-K. A copy of any omitted schedule will be furnished to the Securities and Exchange Commission upon request; provided, however, that the parties may request confidential treatment pursuant to Rule 24b-2 of the Exchange Act for any document so furnished.

ITEM 16. FORM 10-K SUMMARY

None.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

Zevra Therapeutics, Inc.

Dated: March 7, 2023

By: /s/ Richard W. Pascoe
Richard W. Pascoe
Chief Executive Officer
(Principal Executive Officer)

Dated: March 7, 2023

By: /s/ R. LaDuane Clifton
R. LaDuane Clifton, CPA
Chief Financial Officer, Secretary and Treasurer
(Principal Financial Officer)

POWER OF ATTORNEY

KNOW ALL BY THESE PRESENTS, that each person whose signature appears below hereby constitute and appoint Richard W. Pascoe and R. LaDuane Clifton, and each of them (with full power to each of them to act alone), as his true and lawful attorneys-in-fact and agents, with full power of substitution and resubstitution in each of them for him and in his name, place and stead, and in any and all capacities, to sign any and all amendments to this report, and to file the same, with all exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing requisite or necessary to be done in and about the premises, as fully to all intents and purposes as he might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact and agents or any of them, or their or his substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated.

Signature	Title	Date
/s/ Richard W. Pascoe Richard W. Pascoe	Chief Executive Officer and Director (Principal Executive Officer)	March 7, 2023
/s/ R. LaDuane Clifton R. LaDuane Clifton, CPA	Chief Financial Officer, Secretary and Treasurer (Principal Financial Officer)	March 7, 2023
/s/ Timothy J. Sangiovanni Timothy J. Sangiovanni, CPA	Senior Vice President, Corporate Controller (Principal Accounting Officer)	March 7, 2023
/s/Travis C. Mickle, Ph.D. Travis C. Mickle, Ph.D.	President and Director	March 7, 2023
/s/ Matthew R. Plooster Matthew R. Plooster	Chairman of the Board of Directors	March 7, 2023
/s/ Joseph B. Saluri Joseph B. Saluri	Director	March 7, 2023
/s/ Tamara A. Favorito Tamara A. Favorito	Director	March 7, 2023
/s/ David S. Tierney David S. Tierney	Director	March 7, 2023
/s/ Christopher Posner Christopher Posner	Director	March 7, 2023

Exhibit 4.5

DESCRIPTION OF THE REGISTRANT'S SECURITIES REGISTERED PURSUANT TO SECTION 12 OF THE SECURITIES EXCHANGE ACT OF 1934

Zevra Therapeutics, Inc. ("we," "our," or "us") has one class of securities registered under Section 12 of the Securities Exchange Act of 1934, as amended, our common stock, par value $0.0001 per share, or common stock. The following description of our capital stock is a summary and does not purport to be complete. It is qualified in its entirety by, and should be read in conjunction with, our amended and restated certificate of incorporation, amended and restated bylaws and applicable Delaware law.

General

Under our amended and restated certificate of incorporation we are authorized to issue up to 250,000,000 shares of common stock and 10,000,000 shares of preferred stock, $0.0001 par value per share, all of which are currently undesignated. Our board of directors may establish the rights and preferences of the undesignated preferred stock from time to time.

Common Stock

Voting Rights

Each holder of our common stock is entitled to one vote for each share on all matters submitted to a vote of the stockholders, including the election of directors. Under our amended and restated certificate of incorporation and amended and restated bylaws, our stockholders do not have cumulative voting rights. The affirmative vote of the majority (plurality, in the case of the election of directors) of shares present and entitled to vote generally on a subject matter shall be the act of the stockholders.

Dividends

Subject to preferences that may be applicable to any then-outstanding preferred stock, holders of our common stock are entitled to receive ratably those dividends, if any, as may be declared from time to time by the board of directors out of legally available funds.

Liquidation

In the event of our liquidation, dissolution or winding up, holders of our common stock will be entitled to share ratably in the net assets legally available for distribution to stockholders after the payment of all of our debts and other liabilities and the satisfaction of any liquidation preference granted to the holders of any then-outstanding shares of preferred stock.

Rights and Preferences

Holders of our common stock have no preemptive, conversion or subscription rights and there are no redemption or sinking fund provisions applicable to our common stock. The rights, preferences and privileges of the holders of our common stock are subject to, and may be adversely affected by, the rights of the holders of shares of any series of our preferred stock that we may designate in the future.

Preferred Stock

Pursuant to our amended and restated certificate of incorporation, our board of directors has the authority, without further action by the stockholders (unless such stockholder action is required by applicable law or stock exchange listing rules), to designate and issue up to 10,000,000 shares of preferred stock in one or more series, to establish from time to time the number of shares to be included in each such series, to fix the designations, powers, preferences, privileges and relative participating, optional or special rights and the qualifications, limitations or restrictions thereof, including dividend rights, conversion rights, voting rights, terms of redemption and liquidation preferences, any or all of which may be greater than the rights of our common stock, and to increase or decrease the number of shares of any such series, but not below the number of shares of such series then outstanding. Our board of directors, without stockholder approval, can issue preferred stock with voting, conversion or other rights that could adversely affect the voting power and other rights of the holders of our common stock. Preferred stock could be issued quickly with terms designed to delay or prevent a change in control of our company or make removal of management more difficult. Additionally, the issuance of preferred stock may have the effect of decreasing the market price of our common stock and may adversely affect the voting power of holders of our common stock and reduce the likelihood that our common stockholders will receive dividend payments and payments upon liquidation.

Our board of directors will fix the designations, voting powers, preferences and rights, as well as the qualifications, limitations or restrictions, of the preferred stock of each series that we offer in the certificate of designation relating to that series.

The Delaware General Corporation Law, the law governing corporations in the state of our incorporation, provides that the holders of preferred stock will have the right to vote separately as a class (or, in some cases, as a series) on an amendment to our certificate of incorporation if the amendment would change the par value or, unless the certificate of incorporation provided otherwise, the number of authorized shares of the class or change the powers, preferences or special rights of the class or series so as to adversely affect the class or series, as the case may be. This right is in addition to any voting rights that may be provided for in the applicable certificate of designation.

Outstanding Warrants

Our warrants include a net exercise provision and contain provisions for the adjustment of the exercise price and the number of shares issuable upon the exercise of each warrant in the event of certain stock dividends, stock splits, reorganizations, reclassifications and consolidations. We have also granted registration rights to OTA LLC, or OTA, as more fully described below under "—Registration Rights."

In June 2014, in connection with our entering into a facility agreement, we issued to Deerfield Private Design Fund III, L.P., or Deerfield, a warrant, or the Deerfield Warrant, to purchase 14,423,076 shares of Series D redeemable convertible preferred stock at an exercise price of $0.78 per share, which is exercisable until June 2, 2024. Upon completion of our initial public offering, the Deerfield Warrant automatically converted into a warrant to purchase 120,192 shares of our common stock at an exercise price of $93.60 per share. According to the terms of the Deerfield Warrant, in no event may Deerfield exercise this warrant if such exercise would result in Deerfield beneficially owning more than 4.985% of the then issued and outstanding shares of our common stock. This exercise limitation may not be waived and any purported exercise that is inconsistent with this exercise limitation is null and void. This exercise limitation will not apply to any exercise made immediately prior to a change of control transaction. If Deerfield is only able to exercise the Deerfield Warrant for a limited number of shares due to this exercise limitation, the Deerfield Warrant could subsequently become exercisable to purchase the remainder of the shares as a result of a variety of events. This could occur, for example, if we issue more shares or Deerfield sells some of its existing shares. The Deerfield Warrant includes a net exercise provision and contains provisions for the adjustment of the exercise price and the number of shares issuable upon the exercise of the warrant in the event of certain stock dividends, stock splits, recapitalizations, reclassifications and consolidations. Under the Deerfield Warrant, Deerfield also has the right to demand upon the occurrence of specified events, including a merger, asset sale or other change of control transaction, that we redeem the Deerfield Warrant for a cash amount equal to the Black-Scholes value of the portion of the Deerfield Warrant to be redeemed. If Deerfield chooses not to redeem the Deerfield Warrant upon the occurrence of such an event, we may not enter into any such transaction unless our successor entity assumes in writing all our obligations under both the Deerfield Warrant and the Deerfield facility and provides Deerfield with certain registration rights.

The Deerfield Warrant includes certain exercise price protection provisions pursuant to which the exercise price of the Deerfield Warrant will be adjusted downward on a broad-based weighted average basis if we issue or sell any shares of common stock, convertible securities, warrants or options, at a sale or exercise price per share less than the greater of the Deerfield Warrant's exercise price or the closing sale price of our common stock on our principal market or exchange on the last trading date immediately prior to such issuance or, in the case of a firm commitment underwritten offering, on the date of execution of the underwriting agreement between us and the underwriters for such offering. The sale price for purposes of this adjustment is measured after giving effect to any underwriting discounts and commissions. This exercise price adjustment does not apply to certain specified sales and in any offering deemed by the Securities and Exchange Commission, or the SEC, to constitute an "at the market offering" as defined in Rule 415 of the Securities Act of 1933, or the Securities Act, of our common stock, the exercise price of the Deerfield Warrant will be adjusted downward pursuant to this anti-dilution adjustment only if such sales are made at a price less than $93.60 per share. In 2021, we entered into a two separate warrant inducement transactions which triggered these exercise price protection provision and the exercise price of the Deerfield Warrant was reduced from $93.60 per share to $46.25 per share and finally to $38.34 per share. In December 2022, Deerfield assigned the Deerfield Warrant to OTA, herein referred to as the OTA Warrant.

In October 2018, in connection with the APADAZ License Agreement, we issued to KVK-Tech, or KVK, a warrant to purchase up to 500,000 shares of our common stock of at an exercise price of $2.30 per share, which reflected the closing price of our common stock on the Nasdaq Stock Market on the execution date of the APADAZ License Agreement, or the KVK Warrant. The KVK Warrant is initially not exercisable for any shares of common stock. Upon the achievement of each of four specified milestones under the KVK Warrant, the KVK Warrant will become exercisable for an additional 125,000 shares, up to an aggregate of 500,000 shares of our common stock. The exercise price and the number and type of shares underlying the KVK Warrant are subject to adjustment in the event of specified events, including a reclassification of our common stock, a subdivision or combination of our common stock, or in the event of specified dividend payments. The KVK Warrant is exercisable until October 24, 2023. Upon exercise, the aggregate exercise price may be paid, at KVK's election, in cash or on a net issuance basis, based upon the fair market value of our common stock at the time of exercise. After giving effect to the reverse stock split, effected in December 2020, the exercise price of the KVK Warrant became $36.80 and the shares of common stock issuable upon exercise of the KVK Warrant became 31,250 shares of common stock.

In January 2021, pursuant to the terms of the underwriting agreement and the December 2020 exchange agreement, we issued warrants to purchase 12,078,361 shares of our common stock, or collectively, the Offering Warrants, in a public offering and in connection with the transactions contemplated under the December 2020 exchange agreement. The Offering Warrants were immediately exercisable and expire on the fifth anniversary of their issuance date, at an exercise price per share of common stock equal to $6.50 per share. The Offering Warrants are exercisable, at the option of each holder, in whole or in part, by delivering to us a duly executed exercise notice, provided that payment in full for the number of shares of our common stock purchased upon such exercise is delivered to us in accordance with the terms of the Offering Warrants. In lieu of making the cash payment otherwise contemplated to be made to us upon such exercise in payment of the aggregate exercise price, the holder may elect instead to receive upon such exercise (either in whole or in part) the net number of shares of common stock determined according to a formula set forth in the Offering Warrants. A holder (together with its affiliates) may not exercise any portion of the Offering Warrant to the extent that the holder and its affiliates and any other person or entities with which such holder would constitute a Section 13(d) "group" would own more than 4.99% of our outstanding common stock immediately after exercise. Except as otherwise provided in the Offering Warrants or by virtue of such holder's ownership of shares of our common stock, the holders of the Offering Warrants do not have the rights or privileges of holders of common stock with respect to the shares of common stock underlying the Offering Warrants, including any voting rights, until they exercise their Offering Warrants. The Offering Warrants provide that holders have the right to participate in distributions or dividends paid on our common stock. In the event of a fundamental transaction, as described in the Offering Warrants and generally including any reorganization, recapitalization or reclassification of our common stock, the sale, transfer or other disposition of all or substantially all of our properties or assets, our consolidation or merger with or into another person, the acquisition of more than 50% of our outstanding common stock, or any person or group becoming the beneficial owner of 50% of the voting power represented by our outstanding common stock, the holders of the Offering Warrants will be entitled to receive upon exercise of the warrants the kind and amount of securities, cash or other property that the holders would have received had they exercised the Offering Warrants immediately prior to such fundamental transaction. In addition, in the event of a fundamental transaction which is approved by our board of directors, the holders of the warrants have the right to require us or a successor entity to redeem the Offering Warrants for cash in the amount of the Black Scholes value of the unexercised portion of the Offering Warrants on the date of the consummation of the fundamental transaction. In the event of a fundamental transaction which is not approved by our board of directors, the holders of the Offering Warrants have the right to require us or a successor entity to redeem the Offering Warrants in the amount of the Black Scholes value of the unexercised portion of the Offering Warrants on the date of the consummation of the fundamental transaction payable in the form of consideration paid to the holders of common stock in such fundamental transaction. The Offering Warrants meet the equity classification requirements and thus are recorded in additional paid-in capital on the balance sheets.

In January 2021, pursuant to the terms of an underwriting agreement, we issued to the underwriter a warrant to purchase 806,932 shares of our common stock, or the Underwriter Warrant. The Underwriter Warrant is subject to substantially the same terms and conditions as the Offering Warrants, provided that the exercise price for the Underwriter Warrant is $8.125 per share. In connection with the closing of the underwriter's partial exercise of its over-allotment option, in February 2021, we issued to the underwriter additional warrants to purchase 18,702 shares of common stock under the same terms as the Underwriter Warrant, collectively these are known as the Underwriter Warrants.

In January 2021, we entered into warrant exercise inducement offer letters, or the January 2021 Inducement Transaction, with certain holders of the Offering Warrants pursuant to which such holders agreed to exercise for cash their existing warrants to purchase 6,620,358 shares of our common stock in exchange for our agreement to issue new warrants, or the January 2021 Inducement Warrants, on substantially the same terms as the existing warrants, except as set forth in the following sentence, to purchase up to 7,944,430 shares of our common stock, which is equal to 120% of the number of shares of our common stock issued upon exercise of the existing warrants. The purchase price of the January 2021 Inducement Warrants was $0.125 per share underlying each January 2021 Inducement Warrant, and the January 2021 Inducement Warrants have an exercise price of $6.36 per share.

In June 2021, we entered into warrant exercise inducement offer letters, or the June 2021 Inducement Transaction, with certain holders of the January 2021 Inducement Warrants pursuant to which such holders agreed to exercise for cash their January 2021 Inducement Warrants to purchase 6,117,509 shares of our common stock in exchange for our agreement to issue new warrants, the June 2021 Inducement Warrants, on substantially the same terms as the January 2021 Inducement Warrants, except as set forth in the following sentence, to purchase up to 1,529,379 shares of our common stock, which is equal to 25% of the number of shares of our common stock issued upon exercise of the January 2021 Inducement Warrants. The purchase price of the June 2021 Inducement Warrants was $0.125 per share underlying each June 2021 Inducement Warrant, and the June 2021 Inducement Warrants have an exercise price of $16.50 per share.

Registration Rights

Investors' Rights Agreement

We and the holders of shares of our common stock issued upon the conversion or reclassification of our redeemable convertible preferred stock have entered into an investors' rights agreement. The registration rights provisions of this agreement expired as to all holders of our capital stock, other than Deerfield, on the second anniversary of our initial public offering. The registration rights provisions of our investors' rights agreement currently provide Deerfield with the registration rights described in more detail below.

Demand Registration Rights

OTA has the right to demand that we file a Form S-1 registration statement, as long as the anticipated aggregate offering price, net of underwriting discounts and commissions, would exceed $15.0 million. These registration rights are subject to specified conditions and limitations, including the right of the underwriters, if any, to limit the number of shares included in any such registration under specified circumstances. Upon such a request, we are required to effect the registration as soon as reasonably possible.

Piggyback Registration Rights

If we propose to register any of our securities under the Securities Act either for our own account or for the account of other stockholders, OTA will be entitled to include its shares of our common stock in the registration statement. These piggyback registration rights are subject to specified conditions and limitations, including the right of the underwriters to limit the number of shares included in any such registration under specified circumstances.

Registration on Form S-3

OTA is entitled, upon its written request, to have such shares registered by us on a Form S-3 registration statement at our expense, subject to other specified conditions and limitations.

Expenses of Registration

We will pay all expenses relating to any demand, piggyback or Form S-3 registration, other than underwriting discounts and commissions, subject to specified conditions and limitations.

Termination of Registration Rights

The registration rights granted under the investors' rights agreement terminated as to all the holders of our capital stock, other than OTA, on the two-year anniversary of our initial public offering. These registration rights will terminate as to OTA upon the earliest to occur of (i) written consent of OTA, (ii) such time that the OTA Warrant has been exercised in full and Rule 144 or another similar exemption under the Securities Act is available for the sale of all shares of our capital stock held by OTA without limitation during a three-month period without registration or (iii) six-months following the expiration of the OTA Warrant.

Anti-Takeover Provisions

Section 203 of the Delaware General Corporation Law

We are subject to Section 203 of the Delaware General Corporation Law, which prohibits a Delaware corporation from engaging in any business combination with any interested stockholder for a period of three years after the date that such stockholder became an interested stockholder, with the following exceptions:

- before such date, the board of directors of the corporation approved either the business combination or the transaction that resulted in the stockholder becoming an interested stockholder;

- upon completion of the transaction that resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction began, excluding for purposes of determining the voting stock outstanding, but not the outstanding voting stock owned by the interested stockholder, those shares owned (i) by persons who are directors and also officers and (ii) employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or

- on or after such date, the business combination is approved by the board of directors and authorized at an annual or special meeting of the stockholders, and not by written consent, by the affirmative vote of at least 66 2/3% of the outstanding voting stock that is not owned by the interested stockholder.

In general, Section 203 defines a "business combination" to include the following:

- any merger or consolidation involving the corporation and the interested stockholder;

- any sale, transfer, pledge or other disposition of 10% or more of the assets of the corporation involving the interested stockholder;

- subject to certain exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder;

- any transaction involving the corporation that has the effect of increasing the proportionate share of the stock or any class or series of the corporation beneficially owned by the interested stockholder; or

- the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits by or through the corporation.

In general, Section 203 defines an "interested stockholder" as an entity or person who, together with the person's affiliates and associates, beneficially owns, or within three years prior to the time of determination of interested stockholder status did own, 15% or more of the outstanding voting stock of the corporation.

Certificate of Incorporation and Bylaws

Our amended and restated certificate of incorporation provides for our board of directors to be divided into three classes with staggered three-year terms. Only one class of directors will be elected at each annual meeting of our stockholders, with the other classes continuing for the remainder of their respective three-year terms. The affirmative vote of the majority (plurality, in the case of the election of directors) of shares present and entitled to vote generally on a subject matter shall be the act of the stockholders. Our amended and restated certificate of incorporation and our amended and restated bylaws also provide that directors may be removed by the stockholders only for cause upon the vote of 66 2/3% or more of our outstanding common stock. Furthermore, the authorized number of directors may be changed only by resolution of the board of directors, and vacancies and newly created directorships on the board of directors may, except as otherwise required by law or determined by the board of directors, only be filled by a majority vote of the directors then serving on the board of directors, even though less than a quorum.

Our amended and restated certificate of incorporation and amended and restated bylaws also provide that all stockholder actions must be effected at a duly called meeting of stockholders and eliminates the right of stockholders to act by written consent without a meeting. Our amended and restated bylaws also provide that only our chairman of the board, chief executive officer or the board of directors pursuant to a resolution adopted by a majority of the total number of authorized directors may call a special meeting of stockholders.

Our amended and restated bylaws also provide that stockholders seeking to present proposals before a meeting of stockholders or to nominate candidates for election as directors at a meeting of stockholders must provide timely advance notice in writing, and specify requirements as to the form and content of a stockholder's notice.

Our amended and restated certificate of incorporation and amended and restated bylaws provide that the stockholders cannot amend many of the provisions described above except by a vote of 66 2/3% or more of our outstanding common stock.

The combination of these provisions make it more difficult for our existing stockholders to replace our board of directors as well as for another party to obtain control of us by replacing our board of directors. Since our board of directors has the power to retain and discharge our officers, these provisions also make it more difficult for existing stockholders or another party to effect a change in management. In addition, the authorization of undesignated preferred stock makes it possible for our board of directors to issue preferred stock with voting or other rights or preferences that could impede the success of any attempt to change our control.

These provisions are intended to enhance the likelihood of continued stability in the composition of our board of directors and its policies and to discourage coercive takeover practices and inadequate takeover bids. These provisions are also designed to reduce our vulnerability to hostile takeovers and to discourage certain tactics that may be used in proxy fights. However, such provisions could have the effect of discouraging others from making tender offers for our shares and may have the effect of delaying changes in our control or management. As a consequence, these provisions may also inhibit fluctuations in the market price of our stock that could result from actual or rumored takeover attempts. We believe that the benefits of these provisions, including increased protection of our potential ability to negotiate with the proponent of an unfriendly or unsolicited proposal to acquire or restructure our company, outweigh the disadvantages of discouraging takeover proposals, because negotiation of takeover proposals could result in an improvement of their terms.

Choice of Forum

Our amended and restated bylaws provide that (1) unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if and only if the Court of Chancery of the State of Delaware lacks subject matter jurisdiction, any state court located within the State of Delaware or, if and only if all such state courts lack subject matter jurisdiction, the federal district court for the District of Delaware) shall be the sole and exclusive forum for the following types of actions or proceedings under Delaware statutory or common law: (A) any derivative action or proceeding brought on behalf of us; (B) any action or proceeding asserting a claim of breach of a fiduciary duty owed by any current or former director, officer or other employee of us, to us or our stockholders; (C) any action or proceeding asserting a claim against us any current or former director, officer or other employee of us, arising out of or pursuant to any provision of the Delaware General Corporation Law, or the DGCL, our amended and restated certificate of incorporation or our amended and restated bylaws (as each may be amended from time to time); (D) any action or proceeding to interpret, apply, enforce or determine the validity of our amended and restated certificate of incorporation or our amended and restated bylaws (including any right, obligation, or remedy thereunder); (E) any action or proceeding as to which the DGCL confers jurisdiction to the Court of Chancery of the State of Delaware; and (F) any action or proceeding asserting a claim against us or any director, officer or other employee of us, governed by the internal affairs doctrine, in all cases to the fullest extent permitted by law and subject to the court's having personal jurisdiction over the indispensable parties named as defendants, provided that this provision shall not apply to suits brought to enforce a duty or liability created by the Securities Act or the Exchange Act or any other claim for which the federal courts have exclusive jurisdiction; (2) unless we consent in writing to the selection of an alternative forum, to the fullest extent permitted by law, the federal district courts of the United States of America shall be the exclusive forum for the resolution of any complaint asserting a cause of action arising under the Securities Act; and (3) any person or entity holding, owning or otherwise acquiring any interest in any of our securities shall be deemed to have notice of and consented to the provisions of our amended and restated bylaws.

Listing on the Nasdaq Stock Market

Our common stock is listed on the Nasdaq Global Select Market under the symbol "ZVRA."

Attachment #1

KemPharm, Inc.
Eighth Amended and Restated
Non-Employee Director Compensation Policy
Effective: February 15, 2023

Each member of the board of director (the "***Board***") of KemPharm, Inc. (the "***Company***") who is not also an employee of the Company or any subsidiary of the Company shall be entitled to the following compensation for service on the Board and its committees:

Cash Compensation

Cash compensation shall be paid in the following annual amounts. Payments shall be made in quarterly installments in arrears on the last day of each calendar quarter in which service occurred and shall be *prorated* as appropriated for a director who does not serve for the full quarter. For the avoidance of doubt, the cash compensation set forth below shall apply for the entire quarter in which this policy is adopted by the Board.

1. Annual Board Service Retainer:
    a. All non-employee directors: $40,000
    b. Chairman of the Board, if not an employee (in addition to the retainer for all non-employee directors): $75,000
    c. Lead independent director, if any (in addition to the retainer for all non-employee directors): $15,000

2. Annual Committee Member Service Retainer:
    a. Member of the Audit Committee: $9,000
    b. Member of the Compensation Committee: $7,000
    c. Member of the Nominating and Corporate Governance Committee: $5,000

3. Annual Committee Chair Service Retainer:
    a. Chair of the Audit Committee: $22,500
    b. Chair of the Compensation Committee: $15,000
    c. Chair of the Nominating and Corporate Governance Committee: $12,500

Equity Compensation

The equity compensation set forth below will be granted under the Company's 2014 Equity Incentive Plan, or, as the case may be, any successor equity incentive plan approved by the stockholders of the Company (the "***Plan***"). All stock options granted under this policy will be nonqualified stock options using the Company's standard form of Nonqualified Stock Option Agreement under the Plan, with an exercise price per share equal to the last reported sale price of the Company's common stock on the NASDAQ Global Select Market on the date of grant or, if such grant date is not a trading date, on the last trading date prior to the grant date, and with a term of ten (10) years from the date of grant (subject to earlier termination in connection with a termination of service as provided in the Plan).

Annual Grant: On the date of each annual stockholders meeting of the Company, each director who continues to serve as a non-employee member of the Board following such stockholders meeting will be automatically, and without further action by the Board or the Compensation Committee of the Board, be granted a stock option for 39,200 shares of common stock. The Chairman of the Board, if not an employee, shall receive a stock option for a total of 49,200 shares of common stock, which is based on the foregoing grant of 39,200 shares as a director plus an additional 10,000 shares. The stock options will vest and become exercisable in full on the earlier of (1) the first anniversary of the grant date, (2) the day before the first annual stockholders meeting occurring after the grant date or (3) immediately prior to a "Change in Control" as defined in the Plan, subject in each case to the director's continued service on such vesting date.

ZEVRA THERAPEUTICS, INC.
2023 EMPLOYMENT INDUCEMENT AWARD PLAN
ADOPTED BY THE BOARD OF DIRECTORS: JANUARY 6, 2023

1. General.

(a) Eligible Award Recipients. Eligible Individuals are eligible to receive Awards.

(b) Available Awards. The Plan provides for the grant of the following Awards: (i) Nonstatutory Stock Options, (ii) Stock Appreciation Rights (iii) Restricted Stock Awards, (iv) Restricted Stock Unit Awards, (v) Performance Stock Awards, (vi) Performance Cash Awards, and (vii) Other Stock Awards.

(c) Purpose. This Plan, through the granting of Awards, is intended to help the Company secure and retain the services of eligible award recipients, provide incentives for such persons to exert maximum efforts for the success of the Company and any Affiliate, and provide a means by which the eligible recipients may benefit from increases in value of the Common Stock.

2. Administration.

(a) Administration by Board. The Board will administer the Plan. The Board may delegate administration of the Plan to a Committee or Committees, as provided in Section 2(c).

(b) Powers of Board. The Board will have the power, subject to, and within the limitations of, the express provisions of the Plan:

(i) To determine: (A) which Eligible Individuals will be granted Awards; (B) when and how each Award will be granted; (C) what type of Award will be granted; (D) the provisions of each Award (which need not be identical), including when a person will be permitted to exercise or otherwise receive cash or Common Stock under the Award; (E) the number of shares of Common Stock subject to, or the cash value of, an Award; and (F) the Fair Market Value applicable to a Stock Award.

(ii) To construe and interpret the Plan and Awards granted under it, and to establish, amend and revoke rules and regulations for administration of the Plan and Awards. The Board, in the exercise of these powers, may correct any defect, omission or inconsistency in the Plan or in any Award Agreement or in the written terms of a Performance Cash Award, in a manner and to the extent it will deem necessary or expedient to make the Plan or Award fully effective.

(iii) To settle all controversies regarding the Plan and Awards granted under it.

(iv) To accelerate, in whole or in part, the time at which an Award may be exercised or vest (or at which cash or shares of Common Stock may be issued).

(v) To suspend or terminate the Plan at any time. Except as otherwise provided in the Plan or an Award Agreement, suspension or termination of the Plan will not materially impair a Participant's rights under his or her then-outstanding Award without his or her written consent except as provided in subsection (viii) below.

(vi) To amend the Plan in any respect the Board deems necessary or advisable, including, without limitation, by adopting amendments relating to certain nonqualified deferred

compensation under Section 409A of the Code and/or to bring the Plan or Awards granted under the Plan compliant with or exempt from the requirements for nonqualified deferred compensation under Section 409A of the Code, subject to the limitations, if any, of applicable law.

(vii) To adopt procedures from time to time that are intended to ensure that an individual is an Eligible Individual prior to the granting of any Awards to such individual (including without limitation a requirement that each such individual certify to the Company prior to the receipt of an Award that he or she is not currently employed by the Company or a Subsidiary and, if previously so employed, has had a bona fide period of interruption of employment, and that the grant of Awards is an inducement material to the Eligible Individual's agreement to enter into employment with the Company or a Subsidiary).

(viii) To approve forms of Award Agreements for use under the Plan and to amend the terms of any one or more Awards, including, but not limited to, amendments to provide terms more favorable to the Participant than previously provided in the Award Agreement, subject to any specified limits in the Plan that are not subject to Board discretion; provided however, that a Participant's rights under any Award will not be impaired by any such amendment unless (A) the Company requests the consent of the affected Participant, and (B) such Participant consents in writing. Notwithstanding the foregoing, (1) a Participant's rights will not be deemed to have been impaired by any such amendment if the Board, in its sole discretion, determines that the amendment, taken as a whole, does not materially impair the Participant's rights, and (2) subject to the limitations of applicable law, if any, the Board may amend the terms of any one or more Awards without the affected Participant's consent (A) to clarify the manner of exemption from, or to bring the Award into compliance with, Section 409A of the Code; or (B) to comply with other applicable laws or listing requirements.

(ix) Generally, to exercise such powers and to perform such acts as the Board deems necessary or expedient to promote the best interests of the Company and that are not in conflict with the provisions of the Plan or Awards.

(x) To adopt such procedures and sub-plans as are necessary or appropriate to permit participation in the Plan by Eligible Individuals who are foreign nationals or employed outside the United States (provided that Board approval will not be necessary for immaterial modifications to the Plan or any Award Agreement that are required for compliance with the laws of the relevant foreign jurisdiction).

(xi) To effect, with the consent of any adversely affected Participant, (A) the reduction of the exercise, purchase or strike price of any outstanding Stock Award; (B) the cancellation of any outstanding Stock Award and the grant in substitution therefor of a new (1) Option or SAR, (2) Restricted Stock Award, (3) Restricted Stock Unit Award, (4) Other Stock Award, (5) cash award and/or (6) award of other valuable consideration determined by the Board, in its sole discretion, with any such substituted award

(x) covering the same or a different number of shares of Common Stock as the cancelled Stock Award and (y) granted under the Plan or another equity or compensatory plan of the Company; or (C) any other action that is treated as a repricing under generally accepted accounting principles.

(c) Delegation to Committee. (i) General. The Board may delegate some or all of the administration of the Plan to a Committee or Committees. If administration of the Plan is delegated to a Committee, the Committee will have, in connection with the administration of the Plan, the powers theretofor

possessed by the Board that have been delegated to the Committee, including the power to delegate to a subcommittee of the Committee any of the administrative powers the Committee is authorized to exercise (and references in this Plan to the Board will thereafter be to the Committee or subcommittee, as applicable). Any delegation of administrative powers will be reflected in resolutions, not inconsistent with the provisions of the Plan, adopted from time to time by the Board or Committee (as applicable). The Board may retain the authority to concurrently administer the Plan with the Committee and may, at any time, revest in the Board some or all of the powers previously delegated.

(ii) Rule 16b-3 Compliance. To the extent an Award is intended to qualify for the exemption from Section 16(b) of the Exchange Act that is available under Rule 16b-3 of the Exchange Act, the Award will be granted by the Board or a Committee that consists solely of two or more Non-Employee Directors, as determined under Rule 16b-3(b)(3) of the Exchange Act and thereafter any action establishing or modifying the terms of the Award will be approved by the Board or a Committee, meeting such requirements to the extent necessary for such exemption to remain available.

(d) Effect of Board's Decision. All determinations, interpretations and constructions made by the Board or the Committee in good faith will not be subject to review by any person and will be final, binding and conclusive on all persons.

3. Shares Subject to the Plan.

(a) Share Reserve. Subject to Section 9(a) relating to Capitalization Adjustments, the aggregate number of shares of Common Stock that may be issued pursuant to Stock Awards will not exceed 1,500,000 (the "Share Reserve"). For clarity, the Share Reserve is a limitation on the number of shares of Common Stock that may be issued under the Plan. Accordingly, this Section 3(a) does not limit the granting of Stock Awards except as provided in Section 7(a).

(b) Reversion of Shares to the Share Reserve. If a Stock Award or any portion thereof (i) expires or otherwise terminates without all of the shares covered by such Stock Award having been issued or (ii) is settled in cash (i.e., the Participant receives cash rather than stock), such expiration, termination or settlement will not reduce (or otherwise offset) the number of shares of Common Stock that may be available for issuance under the Plan. If any shares of Common Stock issued pursuant to a Stock Award are forfeited back to or repurchased by the Company because of the failure to meet a contingency or condition required to vest such shares in the Participant, then the shares that are forfeited or repurchased will revert to and again become available for issuance under the Plan. Any shares reacquired by the Company in satisfaction of tax withholding obligations on a Stock Award or as consideration for the exercise or purchase price of a Stock Award will again become available for issuance under the Plan.

(c) Source of Shares. The stock issuable under the Plan will be shares of authorized but unissued or reacquired Common Stock, including shares repurchased by the Company on the open market or otherwise. 4. Eligibility for Specific Stock Awards. Stock Awards may be granted to Eligible Individuals.

4. Eligibility for Specific Stock Awards.

Stock Awards may be granted to Eligible Individuals.

5. Provisions Relating to Options and Stock Appreciation Rights.

Each Option or SAR will be in such form and will contain such terms and conditions as the Board deems appropriate. All Options will be Nonstatutory Stock Options, and, if certificates are issued, a separate certificate or certificates will be issued for shares of Common Stock purchased on exercise of each Option. The provisions of separate Options or SARs need not be identical; provided, however, that each Award Agreement will conform to (through incorporation of provisions hereof by reference in the applicable Award Agreement or otherwise) the substance of each of the following provisions:

(a) Term. No Option or SAR will be exercisable after the expiration of ten years from the date of its grant or such shorter period specified in the Award Agreement.

(b) Exercise Price. The exercise or strike price of each Option or SAR will be not less than 100% of the Fair Market Value of the Common Stock subject to the Option or SAR on the date the Award is granted. Each SAR will be denominated in shares of Common Stock equivalents.

(c) Purchase Price for Options. The purchase price of Common Stock acquired pursuant to the exercise of an Option may be paid, to the extent permitted by applicable law and as determined by the Board in its sole discretion, by any combination of the methods of payment set forth below. The Board will have the authority to grant Options that do not permit all of the following methods of payment (or otherwise restrict the ability to use certain methods) and to grant Options that require the consent of the Company to use a particular method of payment. The permitted methods of payment are as follows:

(i) by cash, check, bank draft or money order payable to the Company;

(ii) pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board that, prior to the issuance of the stock subject to the Option, results in either the receipt of cash (or check) by the Company or the receipt of irrevocable instructions to pay the aggregate exercise price to the Company from the sales proceeds;

(iii) by delivery to the Company (either by actual delivery or attestation) of shares of Common Stock;

(iv) by a "net exercise" arrangement pursuant to which the Company will reduce the number of shares of Common Stock issuable upon exercise of an Option by the largest whole number of shares with a Fair Market Value that does not exceed the aggregate exercise price; provided, however, that the Company will accept a cash or other payment from the Participant to the extent of any remaining balance of the aggregate exercise price not satisfied by such reduction in the number of whole shares to be issued. Shares of Common Stock will no longer be subject to an Option and will not be exercisable thereafter to the extent that (A) shares issuable upon exercise are reduced to pay the exercise price pursuant to the "net exercise," (B) shares are delivered to the Participant as a result of such exercise, and (C) shares are withheld to satisfy tax withholding obligations; or

(v) in any other form of legal consideration that may be acceptable to the Board and specified in the applicable Award Agreement.

(d) Exercise and Payment of a SAR. To exercise any outstanding SAR, the Participant must provide written notice of exercise to the Company in compliance with the provisions of the Stock Appreciation Right Agreement evidencing such SAR. The appreciation distribution payable on the exercise of a SAR will be not greater than an amount equal to the excess of (A) the aggregate Fair Market Value (on

the date of the exercise of the SAR) of a number of shares of Common Stock equal to the number of Common Stock equivalents in which the Participant is vested under such SAR, and with respect to which the Participant is exercising the SAR on such date, over (B) the aggregate strike price of the number of Common Stock equivalents with respect to which the Participant is exercising the SAR on such date. The appreciation distribution may be paid in Common Stock, in cash, in any combination of the two or in any other form of consideration, as determined by the Board and contained in the Award Agreement evidencing such SAR.

(e) Transferability of Options and SARs. The Board may, in its sole discretion, impose such limitations on the transferability of Options and SARs as the Board will determine. In the absence of such a determination by the Board to the contrary, the following restrictions on the transferability of Options and SARs will apply:

(i) Restrictions on Transfer. An Option or SAR will not be transferable except by will or by the laws of descent and distribution (or pursuant to subsections (ii) and (iii) below), and will be exercisable during the lifetime of the Participant only by the Participant. The Board may permit transfer of the Option or SAR in a manner that is not prohibited by applicable tax and securities laws. Except as explicitly provided herein, neither an Option nor a SAR may be transferred for consideration.

(ii) Domestic Relations Orders. Subject to the approval of the Board or a duly authorized Officer, an Option or SAR may be transferred pursuant to the terms of a domestic relations order, official marital settlement agreement.

(iii) Beneficiary Designation. Subject to the approval of the Board or a duly authorized Officer, a Participant may, by delivering written notice to the Company, in a form approved by the Company (or the designated broker), designate a third party who, on the death of the Participant, will thereafter be entitled to exercise the Option or SAR and receive the Common Stock or other consideration resulting from such exercise. In the absence of such a designation, the executor or administrator of the Participant's estate will be entitled to exercise the Option or SAR and receive the Common Stock or other consideration resulting from such exercise. However, the Company may prohibit designation of a beneficiary at any time, including due to any conclusion by the Company that such designation would be inconsistent with the provisions of applicable laws.

(f) Vesting Generally. The total number of shares of Common Stock subject to an Option or SAR may vest and become exercisable in periodic installments that may or may not be equal. The Option or SAR may be subject to such other terms and conditions on the time or times when it may or may not be exercised (which may be based on the satisfaction of Performance Goals or other criteria) as the Board may deem appropriate. The vesting provisions of individual Options or SARs may vary. The provisions of this Section 5(f) are subject to any Option or SAR provisions governing the minimum number of shares of Common Stock as to which an Option or SAR may be exercised.

(g) Termination of Continuous Service. Except as otherwise provided in the applicable Award Agreement or other agreement between the Participant and the Company, if a Participant's Continuous Service terminates (other than for Cause and other than upon the Participant's death or Disability), the Participant may exercise his or her Option or SAR (to the extent that the Participant was entitled to exercise such Award as of the date of termination of Continuous Service) within the period of time ending on the earlier of (i) the date three months following the termination of the Participant's Continuous Service (or such longer or shorter period specified in the applicable Award Agreement), and (ii) the expiration of the term of the Option or SAR as set forth in the Award Agreement. If, after termination of Continuous Service,

the Participant does not exercise his or her Option or SAR (as applicable) within the applicable time frame, the Option or SAR will terminate.

(h) Extension of Termination Date. If the exercise of an Option or SAR following the termination of the Participant's Continuous Service (other than for Cause and other than upon the Participant's death or Disability) would be prohibited at any time solely because the issuance of shares of Common Stock would violate the registration requirements under the Securities Act, then the Option or SAR will terminate on the earlier of (i) the expiration of a total period of time (that need not be consecutive) equal to the applicable post termination exercise period after the termination of the Participant's Continuous Service during which the exercise of the Option or SAR would not be in violation of such registration requirements, and (ii) the expiration of the term of the Option or SAR as set forth in the applicable Award Agreement. In addition, unless otherwise provided in a Participant's Award Agreement, if the sale of any Common Stock received on exercise of an Option or SAR following the termination of the Participant's Continuous Service (other than for Cause) would violate the Company's insider trading policy, then the Option or SAR will terminate on the earlier of (i) the expiration of a period of months (that need not be consecutive) equal to the applicable post-termination exercise period after the termination of the Participant's Continuous Service during which the sale of the Common Stock received upon exercise of the Option or SAR would not be in violation of the Company's insider trading policy, or (ii) the expiration of the term of the Option or SAR as set forth in the applicable Award Agreement.

(i) Disability of Participant. Except as otherwise provided in the applicable Award Agreement or other agreement between the Participant and the Company, if a Participant's Continuous Service terminates as a result of the Participant's Disability, the Participant may exercise his or her Option or SAR (to the extent that the Participant was entitled to exercise such Option or SAR as of the date of termination of Continuous Service), but only within such period of time ending on the earlier of (i) the date 12 months following such termination of Continuous Service (or such longer or shorter period specified in the Award Agreement), and (ii) the expiration of the term of the Option or SAR as set forth in the Award Agreement. If, after termination of Continuous Service, the Participant does not exercise his or her Option or SAR within the applicable time frame, the Option or SAR (as applicable) will terminate.

(j) Death of Participant. Except as otherwise provided in the applicable Award Agreement or other agreement between the Participant and the Company, if (i) a Participant's Continuous Service terminates as a result of the Participant's death, or (ii) the Participant dies within the period (if any) specified in the Award Agreement for exercisability after the termination of the Participant's Continuous Service for a reason other than death, then the Option or SAR may be exercised (to the extent the Participant was entitled to exercise such Option or SAR as of the date of death) by the Participant's estate, by a person who acquired the right to exercise the Option or SAR by bequest or inheritance or by a person designated to exercise the Option or SAR upon the Participant's death, but only within the period ending on the earlier of (i) the date 18 months following the date of death (or such longer or shorter period specified in the Award Agreement), and (ii) the expiration of the term of such Option or SAR as set forth in the Award Agreement. If, after the Participant's death, the Option or SAR is not exercised within the applicable time frame, the Option or SAR (as applicable) will terminate.

(k) Termination for Cause. Except as explicitly provided otherwise in a Participant's Award Agreement or other individual written agreement between the Company or any Affiliate and the Participant, if a Participant's Continuous Service is terminated for Cause, the Option or SAR will terminate immediately upon such Participant's termination of Continuous Service, and the Participant will be prohibited from exercising his or her Option or SAR from and after the date of such termination of Continuous Service.

(l) Non-Exempt Employees. If an Option or SAR is granted to an Employee who is a nonexempt employee for purposes of the Fair Labor Standards Act of 1938, as amended, the Option or SAR

will not be first exercisable for any shares of Common Stock until at least six months following the date of grant of the Option or SAR (although the Award may vest prior to such date). Consistent with the provisions of the Worker Economic Opportunity Act, (i) if such non-exempt Employee dies or suffers a Disability, (ii) upon a Corporate Transaction in which such Option or SAR is not assumed, continued, or substituted, (iii) upon a Change in Control, or (iv) upon the Participant's retirement (as such term may be defined in the Participant's Award Agreement in another agreement between the Participant and the Company, or, if no such definition, in accordance with the Company's then current employment policies and guidelines), the vested portion of any Options and SARs may be exercised earlier than six months following the date of grant. The foregoing provision is intended to operate so that any income derived by a non-exempt employee in connection with the exercise or vesting of an Option or SAR will be exempt from his or her regular rate of pay. To the extent permitted and/or required for compliance with the Worker Economic Opportunity Act to ensure that any income derived by a non-exempt employee in connection with the exercise, vesting or issuance of any shares under any other Stock Award will be exempt from the employee's regular rate of pay, the provisions of this Section 5(l) will apply to all Stock Awards and are hereby incorporated by reference into such Stock Award Agreements.

6. ***Provisions of Stock Awards other than Options and SARs.***

(a) Restricted Stock Awards. Each Restricted Stock Award Agreement will be in such form and will contain such terms and conditions as the Board will deem appropriate. To the extent consistent with the Company's bylaws, at the Board's election, shares of Common Stock may be (x) held in book entry form subject to the Company's instructions until any restrictions relating to the Restricted Stock Award lapse; or (y) evidenced by a certificate, which certificate will be held in such form and manner as determined by the Board. The terms and conditions of Restricted Stock Award Agreements may change from time to time, and the terms and conditions of separate Restricted Stock Award Agreements need not be identical. Each Restricted Stock Award Agreement will conform to (through incorporation of the provisions hereof by reference in the agreement or otherwise) the substance of each of the following provisions:

(i) Consideration. A Restricted Stock Award may be awarded in consideration for (A) cash, check, bank draft or money order payable to the Company, (B) past services to the Company or an Affiliate, or (C) any other form of legal consideration (including future services) that may be acceptable to the Board, in its sole discretion, and permissible under applicable law.

(ii) Vesting. Shares of Common Stock awarded under the Restricted Stock Award Agreement may be subject to forfeiture to the Company in accordance with a vesting schedule to be determined by the Board.

(iii) Termination of Participant's Continuous Service. If a Participant's Continuous Service terminates, the Company may receive through a forfeiture condition or a repurchase right any or all of the shares of Common Stock held by the Participant as of the date of termination of Continuous Service under the terms of the Restricted Stock Award Agreement.

(iv) Transferability. Rights to acquire shares of Common Stock under the Restricted Stock Award Agreement will be transferable by the Participant only upon such terms and conditions as are set forth in the Restricted Stock Award Agreement, as the Board will determine in its sole discretion, so long as Common Stock awarded under the Restricted Stock Award Agreement remains subject to the terms of the Restricted Stock Award Agreement.

(v) *Dividends.* A Restricted Stock Award Agreement may provide that any dividends paid on Restricted Stock will be subject to the same vesting and forfeiture restrictions as apply to the shares subject to the Restricted Stock Award to which they relate.

(b) Restricted Stock Unit Awards. Each Restricted Stock Unit Award Agreement will be in such form and will contain such terms and conditions as the Board will deem appropriate. The terms and conditions of Restricted Stock Unit Award Agreements may change from time to time, and the terms and conditions of separate Restricted Stock Unit Award Agreements need not be identical. Each Restricted Stock Unit Award Agreement will conform to (through incorporation of the provisions hereof by reference in the Agreement or otherwise) the substance of each of the following provisions:

(i) *Consideration.* At the time of grant of a Restricted Stock Unit Award, the Board will determine the consideration, if any, to be paid by the Participant upon delivery of each share of Common Stock subject to the Restricted Stock Unit Award. The consideration to be paid (if any) by the Participant for each share of Common Stock subject to a Restricted Stock Unit Award may be paid in any form of legal consideration that may be acceptable to the Board, in its sole discretion, and permissible under applicable law.

(ii) *Vesting.* At the time of the grant of a Restricted Stock Unit Award, the Board may impose such restrictions on or conditions to the vesting of the Restricted Stock Unit Award as it, in its sole discretion, deems appropriate.

(iii) *Payment.* A Restricted Stock Unit Award may be settled by the delivery of shares of Common Stock, their cash equivalent, any combination thereof or in any other form of consideration, as determined by the Board and contained in the Restricted Stock Unit Award Agreement.

(iv) *Additional Restrictions.* At the time of the grant of a Restricted Stock Unit Award, the Board, as it deems appropriate, may impose such restrictions or conditions that delay the delivery of the shares of Common Stock (or their cash equivalent) subject to a Restricted Stock Unit Award to a time after the vesting of such Restricted Stock Unit Award.

(v) *Dividend Equivalents.* Dividend equivalents may be credited in respect of shares of Common Stock covered by a Restricted Stock Unit Award, as determined by the Board and contained in the Restricted Stock Unit Award Agreement. At the sole discretion of the Board, such dividend equivalents may be converted into additional shares of Common Stock covered by the Restricted Stock Unit Award in such manner as determined by the Board. Any additional shares covered by the Restricted Stock Unit Award credited by reason of such dividend equivalents will be subject to all of the same terms and conditions of the underlying Restricted Stock Unit Award Agreement to which they relate.

(vi) *Termination of Participant's Continuous Service*. Except as otherwise provided in the applicable Restricted Stock Unit Award Agreement, such portion of the Restricted Stock Unit Award that has not vested will be forfeited upon the Participant's termination of Continuous Service. (c) Performance Awards.

(i) *Performance Stock Awards.* A Performance Stock Award is a Stock Award that is payable (including that may be granted, may vest or may be exercised) contingent upon the attainment during a Performance Period of certain Performance Goals. A Performance Stock

Award may, but need not, require the Participant's completion of a specified period of Continuous Service. The length of any Performance Period, the Performance Goals to be achieved during the Performance Period, and the measure of whether and to what degree such Performance Goals have been attained will be conclusively determined by the Committee or the Board, in its sole discretion. In addition, to the extent permitted by applicable law and the applicable Award Agreement, the Board may determine that cash may be used in payment of Performance Stock Awards.

(ii) *Performance Cash Awards.* A Performance Cash Award is a cash award that is payable contingent upon the attainment during a Performance Period of certain Performance Goals. A Performance Cash Award may also require the completion of a specified period of Continuous Service. At the time of grant of a Performance Cash Award, the length of any Performance Period, the Performance Goals to be achieved during the Performance Period, and the measure of whether and to what degree such Performance Goals have been attained will be conclusively determined by the Committee or the Board, in its sole discretion. The Board may specify the form of payment of Performance Cash Awards, which may be cash or other property, or may provide for a Participant to have the option for his or her Performance Cash Award, or such portion thereof as the Board may specify, to be paid in whole or in part in cash or other property.

(iii) Board Discretion. The Board retains the discretion to reduce or eliminate the compensation or economic benefit due upon attainment of Performance Goals and to define the manner of calculating the Performance Criteria it selects to use for a Performance Period.

(d) Other Stock Awards. Other forms of Stock Awards valued in whole or in part by reference to, or otherwise based on, Common Stock, including the appreciation in value thereof (e.g., options or stock rights with an exercise price or strike price less than 100% of the Fair Market Value of the Common Stock at the time of grant) may be granted either alone or in addition to Stock Awards provided for under Section 5 and the preceding provisions of this Section 6. Subject to the provisions of the Plan, the Board will have sole and complete authority to determine the persons to whom and the time or times at which such Other Stock Awards will be granted, the number of shares of Common Stock (or the cash equivalent thereof) to be granted pursuant to such Other Stock Awards and all other terms and conditions of such Other Stock Awards.

7. ***Covenants of the Company.***

(a) Availability of Shares. The Company will keep available at all times the number of shares of Common Stock reasonably required to satisfy then-outstanding Awards.

(b) Securities Law Compliance. The Company will seek to obtain from each regulatory commission or agency having jurisdiction over the Plan such authority as may be required to grant Stock Awards and to issue and sell shares of Common Stock upon exercise of the Stock Awards; provided, however, that this undertaking will not require the Company to register under the Securities Act the Plan, any Stock Award or any Common Stock issued or issuable pursuant to any such Stock Award. If, after reasonable efforts and at a reasonable cost, the Company is unable to obtain from any such regulatory commission or agency the authority that counsel for the Company deems necessary for the lawful issuance and sale of Common Stock under the Plan, the Company will be relieved from any liability for failure to issue and sell Common Stock upon exercise of such Stock Awards unless and until such authority is obtained. A Participant will not be eligible for the grant of an Award or the subsequent issuance of cash or Common Stock pursuant to the Award if such grant or issuance would be in violation of any applicable securities law.

(c) No Obligation to Notify or Minimize Taxes. The Company will have no duty or obligation to any Participant to advise such holder as to the time or manner of exercising such Stock Award. Furthermore, the Company will have no duty or obligation to warn or otherwise advise such holder of a pending termination or expiration of an Award or a possible period in which the Award may not be exercised. The Company has no duty or obligation to minimize the tax consequences of an Award to the holder of such Award.

8. ***Miscellaneous.***

(a) Use of Proceeds from Sales of Common Stock. Proceeds from the sale of shares of Common Stock pursuant to Awards will constitute general funds of the Company.

(b) Corporate Action Constituting Grant of Awards. Corporate action constituting a grant by the Company of an Award to any Participant will be deemed completed as of the date of such corporate action, unless otherwise determined by the Board, regardless of when the instrument, certificate, or letter evidencing the Award is communicated to, or actually received or accepted by, the Participant. In the event that the corporate records (e.g., Board consents, resolutions or minutes) documenting the corporate action constituting the grant contain terms (e.g., exercise price, vesting schedule or number of shares) that are inconsistent with those in the Award Agreement or related grant documents as a result of a clerical error in the papering of the Award Agreement or related grant documents, the corporate records will control and the Participant will have no legally binding right to the incorrect term in the Award Agreement or related grant documents.

(c) Stockholder Rights. No Participant will be deemed to be the holder of, or to have any of the rights of a holder with respect to, any shares of Common Stock subject to an Award unless and until (i) such Participant has satisfied all requirements for exercise of, or the issuance of shares under, the Award pursuant to its terms, and (ii) the issuance of the Common Stock subject to such Award has been entered into the books and records of the Company.

(d) No Employment or Other Service Rights. Nothing in the Plan, any Award Agreement or any other instrument executed thereunder or in connection with any Award granted pursuant thereto will confer upon any Participant any right to continue to serve the Company or an Affiliate in the capacity in effect at the time the Award was granted or will affect the right of the Company or an Affiliate to terminate (i) the employment of an Employee with or without notice and with or without cause, (ii) the service of a Consultant pursuant to the terms of such Consultant's agreement with the Company or an Affiliate, or (iii) the service of a Director pursuant to the bylaws of the Company or an Affiliate, and any applicable provisions of the corporate law of the state in which the Company or the Affiliate is incorporated, as the case may be.

(e) Change in Time Commitment. In the event a Participant's regular level of time commitment in the performance of his or her services for the Company and any Affiliates is reduced (for example, and without limitation, if the Participant is an Employee of the Company and the Employee has a change in status from a full-time Employee to a part-time Employee or takes an extended leave of absence) after the date of grant of any Award to the Participant, the Board has the right in its sole discretion to (x) make a corresponding reduction in the number of shares or cash amount subject to any portion of such Award that is scheduled to vest or become payable after the date of such change in time commitment, and (y) in lieu of or in combination with such a reduction, extend the vesting or payment schedule applicable to such Award. In the event of any such reduction or extension, the Participant will have no right with respect to any portion of the Award that is so reduced or extended.

(f) Investment Assurances. The Company may require a Participant, as a condition of exercising or acquiring Common Stock under any Award, (i) to give written assurances satisfactory to the Company as to the Participant's knowledge and experience in financial and business matters and/or to employ a purchaser representative reasonably satisfactory to the Company who is knowledgeable and experienced in financial and business matters and that he or she is capable of evaluating, alone or together with the purchaser representative, the merits and risks of exercising the Award; and (ii) to give written assurances satisfactory to the Company stating that the Participant is acquiring Common Stock subject to the Award for the Participant's own account and not with any present intention of selling or otherwise distributing the Common Stock. The foregoing requirements, and any assurances given pursuant to such requirements, will be inoperative if (A) the issuance of the shares upon the exercise or acquisition of Common Stock under the Award has been registered under a then currently effective registration statement under the Securities Act, or (B) as to any particular requirement, a determination is made by counsel for the Company that such requirement need not be met in the circumstances under the then applicable securities laws. The Company may, upon advice of counsel to the Company, place legends on stock certificates issued under the Plan as such counsel deems necessary or appropriate in order to comply with applicable securities laws, including, but not limited to, legends restricting the transfer of the Common Stock.

(g) Withholding Obligations. Unless prohibited by the terms of an Award Agreement, the Company may, in its sole discretion, satisfy any federal, state or local tax withholding obligation relating to an Award by any of the following means or by a combination of such means: (i) causing the Participant to tender a cash payment; (ii) withholding shares of Common Stock from the shares of Common Stock issued or otherwise issuable to the Participant in connection with the Award; provided, however, that no shares of Common Stock are withheld with a value exceeding the minimum amount of tax required to be withheld by law (or such lesser amount as may be necessary to avoid classification of the Stock Award as a liability for financial accounting purposes); (iii) withholding cash from an Award settled in cash; (iv) withholding payment from any amounts otherwise payable to the Participant; or (v) by such other method as may be set forth in the Award Agreement.

(h) Electronic Delivery. Any reference herein to a "written" agreement or document will include any agreement or document delivered electronically, filed publicly at www.sec.gov (or any successor website thereto) or posted on the Company's intranet (or other shared electronic medium controlled by the Company to which the Participant has access).

(i) Deferrals. To the extent permitted by applicable law, the Board, in its sole discretion, may determine that the delivery of Common Stock or the payment of cash, upon the exercise, vesting or settlement of all or a portion of any Award may be deferred and may establish programs and procedures for deferral elections to be made by Participants. Deferrals by Participants will be made in accordance with Section 409A of the Code. Consistent with Section 409A of the Code, the Board may provide for distributions while a Participant is still an employee or otherwise providing services to the Company. The Board is authorized to make deferrals of Awards and determine when, and in what annual percentages, Participants may receive payments, including lump sum payments, following the Participant's termination of Continuous Service, and implement such other terms and conditions consistent with the provisions of the Plan and in accordance with applicable law.

(j) Compliance with Section 409A. Unless otherwise expressly provided for in an Award Agreement, the Plan and Award Agreements will be interpreted to the greatest extent possible in a manner that makes the Plan and the Awards granted hereunder exempt from Section 409A of the Code, and, to the extent not so exempt, in compliance with Section 409A of the Code. If the Board determines that any Award granted hereunder is not exempt from and is therefore subject to Section 409A of the Code, the Award Agreement evidencing such Award will incorporate the terms and conditions necessary to avoid the consequences specified in Section 409A(a)(1) of the Code, and to the extent an Award Agreement is silent

on terms necessary for compliance, such terms are hereby incorporated by reference into the Award Agreement. Notwithstanding anything to the contrary in this Plan (and unless the Award Agreement specifically provides otherwise), if the shares of Common Stock are publicly traded, and if a Participant holding an Award that constitutes "deferred compensation" under Section 409A of the Code is a "specified employee" for purposes of Section 409A of the Code, no distribution or payment of any amount that is due because of a "separation from service" (as defined in Section 409A of the Code without regard to alternative definitions thereunder) will be issued or paid before the date that is six months following the date of such Participant's "separation from service" or, if earlier, the date of the Participant's death, unless such distribution or payment can be made in a manner that complies with Section 409A of the Code, and any amounts so deferred will be paid in a lump sum on the day after such six month period elapses, with the balance paid thereafter on the original schedule.

(k) Clawback/Recovery. All Awards granted under the Plan will be subject to recoupment in accordance with any clawback policy that the Company is required to adopt pursuant to the listing standards of any national securities exchange or association on which the Company's securities are listed or as is otherwise required by the Dodd-Frank Wall Street Reform and Consumer Protection Act or other applicable law. In addition, the Board may impose such other clawback, recovery or recoupment provisions in an Award Agreement as the Board determines necessary or appropriate, including but not limited to a reacquisition right in respect of previously acquired shares of Common Stock or other cash or property upon the occurrence of an event constituting Cause. No recovery of compensation under such a clawback policy will be an event giving rise to a right to resign for "good reason" or "constructive termination" (or similar term) under any agreement with the Company.

(l) Action Required Upon Grant of Award. Promptly following the grant of an Award, the Company shall, in accordance with NASDAQ Rule 5635(c), (i) issue a press release disclosing the material terms of the Award, including the recipient(s) of the Award and the number of Shares involved and (ii) provide written notice to NASDAQ of the grant.

(m) Stockholder Approval Not Required. It is expressly intended that approval of the Company's stockholders not be required as a condition of the effectiveness of the Plan, and the Plan's provisions shall be interpreted in a manner consistent with such intent for all purposes. Specifically, NASDAQ Rule 5635(c) generally requires stockholder approval for equity compensation plans adopted by companies whose securities are listed on the NASDAQ Stock Market that provide for the delivery of equity securities to any employees, directors or other service providers of such companies as compensation for services. NASDAQ Rule 5635(c)(4) provides an exemption in certain circumstances for employment inducement awards. Notwithstanding anything to the contrary herein, in accordance with NASDAQ Rule 5635(c)(4), Awards may only be granted as material inducements to Eligible Individuals being hired or rehired as Employees, as applicable, and must be approved by (i) the Board, acting through a majority of the Company's Independent Directors or (ii) the independent Compensation Committee of the Board. Accordingly, pursuant to NASDAQ Rule 5635(c)(4), the issuance of Awards and the Shares issuable upon exercise or vesting of such Awards pursuant to the Plan is not subject to the approval of the Company's stockholders.

9. Adjustments upon Changes in Common Stock; Other Corporate Events.

(a) Capitalization Adjustments. In the event of a Capitalization Adjustment, the Board will appropriately and proportionately adjust: (i) the class(es) and maximum number of securities subject to the Plan pursuant to Section 3(a) and (ii) the class(es) and number of securities and price per share of stock subject to outstanding Stock Awards. The Board will make such adjustments, and its determination will be final, binding and conclusive.

(b) Dissolution or Liquidation. Except as otherwise provided in the Stock Award Agreement, in the event of a dissolution or liquidation of the Company, all outstanding Stock Awards (other than Stock Awards consisting of vested and outstanding shares of Common Stock not subject to a forfeiture condition or the Company's right of repurchase) will terminate immediately prior to the completion of such dissolution or liquidation, and the shares of Common Stock subject to the Company's repurchase rights or subject to a forfeiture condition may be repurchased or reacquired by the Company notwithstanding the fact that the holder of such Stock Award is providing Continuous Service; provided, however, that the Board may, in its sole discretion, cause some or all Stock Awards to become fully vested, exercisable and/or no longer subject to repurchase or forfeiture (to the extent such Stock Awards have not previously expired or terminated) before the dissolution or liquidation is completed but contingent on its completion.

(c) Corporate Transaction. The following provisions will apply to Stock Awards in the event of a Corporate Transaction unless otherwise provided in the instrument evidencing the Stock Award or any other written agreement between the Company or any Affiliate and the Participant or unless otherwise expressly provided by the Board at the time of grant of a Stock Award. In the event of a Corporate Transaction, then, notwithstanding any other provision of the Plan, the Board will take one or more of the following actions with respect to Stock Awards, contingent upon the closing or completion of the Corporate Transaction:

(i) arrange for the surviving corporation or acquiring corporation (or the surviving or acquiring corporation's parent company) to assume or continue the Stock Award or to substitute a similar stock award for the Stock Award (including, but not limited to, an award to acquire the same consideration paid to the stockholders of the Company pursuant to the Corporate Transaction);

(ii) arrange for the assignment of any reacquisition or repurchase rights held by the Company in respect of Common Stock issued pursuant to the Stock Award to the surviving corporation or acquiring corporation (or the surviving or acquiring corporation's parent company);

(iii) accelerate the vesting, in whole or in part, of the Stock Award (and, if applicable, the time at which the Stock Award may be exercised) to a date prior to the effective time of such Corporate Transaction as the Board will determine (or, if the Board will not determine such a date, to the date that is five days prior to the effective date of the Corporate Transaction), with such Stock Award terminating if not exercised (if applicable) at or prior to the effective time of the Corporate Transaction;

(iv) arrange for the lapse, in whole or in part, of any reacquisition or repurchase rights held by the Company with respect to the Stock Award;

(v) cancel or arrange for the cancellation of the Stock Award, to the extent not vested or not exercised prior to the effective time of the Corporate Transaction, in exchange for such cash consideration, if any, as the Board, in its sole discretion, may consider appropriate; and

(vi) make a payment, in such form as may be determined by the Board equal to the excess, if any, of (A) the value of the property the Participant would have received upon the exercise of the Stock Award immediately prior to the effective time of the Corporate Transaction, over (B) any exercise price payable by such holder in connection with such exercise.

The Board need not take the same action or actions with respect to all Stock Awards or portions thereof or with respect to all Participants. The Board may take different actions with respect to the vested and unvested portions of a Stock Award.

(d) Change in Control. A Stock Award may be subject to additional acceleration of vesting and exercisability upon or after a Change in Control as may be provided in the Stock Award Agreement for such Stock Award or as may be provided in any other written agreement between the Company or any Affiliate and the Participant, but in the absence of such provision, no such acceleration will occur.

10. ***Plan Term; Earlier Termination or Suspension of the Plan.***

The Board may suspend or terminate the Plan at any time. No Awards may be granted under the Plan while the Plan is suspended or after it is terminated.

11. ***Effective Date of the Plan.***

The Plan will become effective on the Effective Date.

12. ***Choice of Law.***

The law of the State of Delaware will govern all questions concerning the construction, validity and interpretation of the Plan, without regard to that state's conflict of laws rules.

13. ***Definitions.***

As used in the Plan, the following definitions will apply to the capitalized terms indicated below:

(a) ***"Affiliate"*** means, at the time of determination, any "parent" or "subsidiary" of the Company as such terms are defined in Rule 405 of the Securities Act. The Board will have the authority to determine the time or times at which "parent" or "subsidiary" status is determined within the foregoing definition.

(b) ***"Award"*** means a Stock Award or a Performance Cash Award.

(c) ***"Award Agreement"*** means a written or electronic agreement between the Company and a Participant evidencing the terms and conditions of an Award Each Award Agreement will be subject to the terms and conditions of the Plan.

(d) ***"Board"*** means the Board of Directors of the Company.

(e) ***"Capital Stock"*** means each and every class of common stock of the Company, regardless of the number of votes per share.

(f) ***"Capitalization Adjustment"*** means any change that is made in, or other events that occur with respect to, the Common Stock subject to the Plan or subject to any Stock Award after the Effective Date without the receipt of consideration by the Company through merger, consolidation, reorganization, recapitalization, reincorporation, stock dividend, dividend in property other than cash, large nonrecurring cash dividend, stock split, reverse stock split, liquidating dividend, combination of shares, exchange of shares, change in corporate structure or any similar equity restructuring transaction, as that term is used in Statement of Financial Accounting Standards Board Accounting Standards Codification Topic 718 (or any successor thereto). Notwithstanding the foregoing, the conversion of any convertible securities of the Company will not be treated as a Capitalization Adjustment.

(g) ***"Cause"*** will have the meaning ascribed to such term in any written agreement between the Participant and the Company defining such term and, in the absence of such agreement, such term shall

mean, with respect to a Participant, the occurrence of any of the following events that has a material negative impact on the business or reputation of the Company: (i) such Participant's attempted commission of, or participation in, a fraud or act of dishonesty against the Company; (ii) such Participant's intentional, material violation of any contract or agreement between the Participant and the Company or of any statutory duty owed to the Company; (iii) such Participant's unauthorized use or disclosure of the Company's confidential information or trade secrets; or (iv) such Participant's gross misconduct. The determination that a termination of the Participant's Continuous Service is either for Cause or without Cause shall be made by the Company, in its sole discretion. Any determination by the Company that the Continuous Service of a Participant was terminated with or without Cause for the purposes of outstanding Awards held by such Participant shall have no effect upon any determination of the rights or obligations of the Company or such Participant for any other purpose.

(h) "Change in Control" means the occurrence, in a single transaction or in a series of related transactions, of any one or more of the following events:

(i) any Exchange Act Person becomes the Owner, directly or indirectly, of securities of the Company representing more than 50% of the combined voting power of the Company's then outstanding securities other than by virtue of a merger, consolidation or similar transaction. Notwithstanding the foregoing, a Change in Control will not be deemed to occur (A) on account of the acquisition of securities of the Company directly from the Company, (B) on account of the acquisition of securities of the Company by an investor, any affiliate thereof or any other Exchange Act Person that acquires the Company's securities in a transaction or series of related transactions the primary purpose of which is to obtain financing for the Company through the issuance of equity securities, or (C) solely because the level of Ownership held by any Exchange Act Person (the ***"Subject Person"***) exceeds the designated percentage threshold of the outstanding voting securities as a result of a repurchase or other acquisition of voting securities by the Company reducing the number of shares outstanding, provided that if a Change in Control would occur (but for the operation of this sentence) as a result of the acquisition of voting securities by the Company, and after such share acquisition, the Subject Person becomes the Owner of any additional voting securities that, assuming the repurchase or other acquisition had not occurred, increases the percentage of the then outstanding voting securities Owned by the Subject Person over the designated percentage threshold, then a Change in Control will be deemed to occur;

(ii) there is consummated a merger, consolidation or similar transaction involving (directly or indirectly) the Company and, immediately after the consummation of such merger, consolidation or similar transaction, the stockholders of the Company immediately prior thereto do not Own, directly or indirectly, either (A) outstanding voting securities representing more than 50% of the combined outstanding voting power of the surviving Entity in such merger, consolidation or similar transaction or (B) more than 50% of the combined outstanding voting power of the parent of the surviving Entity in such merger, consolidation or similar transaction, in each case in substantially the same proportions as their Ownership of the outstanding voting securities of the Company immediately prior to such transaction; provided, however, that a merger, consolidation or similar transaction will not constitute a Change in Control under this prong of the definition if the outstanding voting securities representing more than 50% of the combined voting power of the surviving Entity or its parent are owned by the IPO Entities;

(iii) there is consummated a sale, lease, exclusive license or other disposition of all or substantially all of the consolidated assets of the Company and its Subsidiaries, other than a sale, lease, license or other disposition of all or substantially all of the consolidated assets of the Company and its Subsidiaries to an Entity, more than 50% of the combined voting power of the voting securities of which are Owned by stockholders of the Company in substantially the same

proportions as their Ownership of the outstanding voting securities of the Company immediately prior to such sale, lease, license or other disposition; provided, however, that a sale, lease, exclusive license or other disposition of all or substantially all of the consolidated assets of the Company and its Subsidiaries will not constitute a Change in Control under this prong of the definition if the outstanding voting securities representing more than 50% of the combined voting power of the acquiring Entity or its parent are owned by the IPO Entities; or

(iv) individuals who, on the date the Plan is adopted by the Board, are members of the Board (the ***"Incumbent Board"***) cease for any reason to constitute at least a majority of the members of the Board; provided, however, that if the appointment or election (or nomination for election) of any new Board member was approved or recommended by a majority vote of the members of the Incumbent Board then still in office, such new member will, for purposes of this Plan, be considered as a member of the Incumbent Board.

Notwithstanding the foregoing definition or any other provision of this Plan, the term Change in Control will not include a sale of assets, merger or other transaction effected exclusively for the purpose of changing the domicile of the Company and the definition of Change in Control (or any analogous term) in an individual written agreement between the Company or any Affiliate and the Participant will supersede the foregoing definition with respect to Awards subject to such agreement; provided, however, that if no definition of Change in Control or any analogous term is set forth in such an individual written agreement, the foregoing definition will apply.

(i) ***"Code"*** means the Internal Revenue Code of 1986, as amended, including any applicable regulations and guidance thereunder.

(j) ***"Committee"*** means a committee of one or more Directors to whom authority has been delegated by the Board in accordance with Section 2(c).

(k) ***"Common Stock"*** means, the common stock of the Company.

(l) ***"Company"*** means Zevra Therapeutics, Inc., a Delaware corporation.

(m) ***"Consultant"*** means any person, including an advisor, who is (i) engaged by the Company or an Affiliate to render consulting or advisory services and is compensated for such services, or (ii) serving as a member of the board of directors of an Affiliate and is compensated for such services. However, service solely as a Director, or payment of a fee for such service, will not cause a Director to be considered a "Consultant" for purposes of the Plan. Notwithstanding the foregoing, a person is treated as a Consultant under this Plan only if a Form S-8 Registration Statement under the Securities Act is available to register either the offer or the sale of the Company's securities to such person.

(n) ***"Continuous Service"*** means that the Participant's service with the Company or an Affiliate, whether as an Employee, Director or Consultant, is not interrupted or terminated. A change in the capacity in which the Participant renders service to the Company or an Affiliate as an Employee, Consultant or Director or a change in the entity for which the Participant renders such service, provided that there is no interruption or termination of the Participant's service with the Company or an Affiliate, will not terminate a Participant's Continuous Service; provided, however, that if the Entity for which a Participant is rendering services ceases to qualify as an Affiliate, as determined by the Board, in its sole discretion, such Participant's Continuous Service will be considered to have terminated on the date such Entity ceases to qualify as an Affiliate. To the extent permitted by law, the Board or the chief executive officer of the Company, in that party's sole discretion, may determine whether Continuous Service will be considered interrupted in the case of (i) any leave of absence approved by the Board or chief executive

officer, including sick leave, military leave or any other personal leave, or (ii) transfers between the Company, an Affiliate, or their successors. Notwithstanding the foregoing, a leave of absence will be treated as Continuous Service for purposes of vesting in an Award only to such extent as may be provided in the Company's leave of absence policy, in the written terms of any leave of absence agreement or policy applicable to the Participant, or as otherwise required by law.

(o) ***"Corporate Transaction"*** means the consummation, in a single transaction or in a series of related transactions, of any one or more of the following events:

(i) a sale or other disposition of all or substantially all, as determined by the Board, in its sole discretion, of the consolidated assets of the Company and its Subsidiaries;

(ii) a sale or other disposition of at least 50% of the outstanding securities of the Company;

(iii) a merger, consolidation or similar transaction following which the Company is not the surviving corporation; or

(iv) a merger, consolidation or similar transaction following which the Company is the surviving corporation but the shares of Common Stock outstanding immediately preceding the merger, consolidation or similar transaction are converted or exchanged by virtue of the merger, consolidation or similar transaction into other property, whether in the form of securities, cash or otherwise.

(p) ***"Director"*** means a member of the Board.

(q) ***"Disability"*** means, with respect to a Participant, the inability of such Participant to engage in any substantial gainful activity by reason of any medically determinable physical or mental impairment that can be expected to result in death or that has lasted or can be expected to last for a continuous period of not less than 12 months, as provided in Sections 22(e)(3) and 409A(a)(2)(c)(i) of the Code, and will be determined by the Board on the basis of such medical evidence as the Board deems warranted under the circumstances.

(r) ***"Effective Date"*** means the effective date of this Plan document which is the date the Board approved the Plan.

(s) ***"Eligible Individual"*** means any individual who was not previously an Employee or Director hired as a new Employee or rehired as an Employee following a bona fide period of interruption of employment if such person is granted an Award as a material inducement to his or her entering into employment with the Company or a Subsidiary (within the meaning of the NASDAQ Rule 5635(c)(4)).

(t) ***"Employee"*** means any person employed by the Company or an Affiliate. However, service solely as a Director, or payment of a fee for such services, wil not cause a Director to be considered an "Employee" for purposes of the Plan.

(u) ***"Entity"*** means a corporation, partnership, limited liability company or other entity.

(v) ***"Exchange Act"*** means the Securities Exchange Act of 1934, as amended, and the rules and regulations promulgated thereunder.

(w) ***"Exchange Act Person"*** means any natural person, Entity or "group" (within the meaning of Section 13(d) or 14(d) of the Exchange Act), except that "Exchange Act Person" will not include (i) the Company or any Subsidiary of the Company, (ii) any employee benefit plan of the Company or any Subsidiary of the Company or any trustee or other fiduciary holding securities under an employee benefit plan of the Company or any Subsidiary of the Company, (iii) an underwriter temporarily holding securities pursuant to a registered public offering of such securities, (iv) an Entity Owned, directly or indirectly, by the stockholders of the Company in substantially the same proportions as their Ownership of stock of the Company; or (v) any natural person, Entity or "group" (within the meaning of Section 13(d) or 14(d) of the Exchange Act) that, as of the Effective Date, is the Owner, directly or indirectly, of securities of the Company representing more than 50% of the combined voting power of the Company's then outstanding securities.

(x) ***"Fair Market Value"*** means, as of any date, the value of the Common Stock determined as follows:

(i) If the Common Stock is listed on any established stock exchange or traded on any established market, the Fair Market Value of a share of Common Stock will be, unless otherwise determined by the Board, **the closing sales price** for such stock as quoted on such exchange or market (or the exchange or market with the greatest volume of trading in the Common Stock) **on the date of determination**, as reported in a source the Board deems reliable.

(ii) Unless otherwise provided by the Board, if there is no closing sales price for the Common Stock on the date of determination, then the Fair Market Value will be the closing selling price on the last preceding date for which such quotation exists.

(iii) In the absence of such markets for the Common Stock, the Fair Market Value will be determined by the Board in good faith and in a manner that complies with Sections 409A and 422 of the Code.

(y) ***"Incentive Stock Option"*** means an option intended to qualify as an "incentive stock option" within the meaning of Section 422 of the Code.

(z) ***"Independent Director"*** means a Director who qualifies as "independent" within the meaning of NASDAQ Rule 5635(c)(4), or any successor rule, as such rule may be amended from time to time.

(aa) ***"NASDAQ Rule 5635(c)(4)"*** means NASDAQ Rule 5635(c)(4), or any successor rule, and all guidance and other interpretative authority thereunder, as such rule, guidance and other authority may be amended from time to time.

(bb) ***"Non-Employee Director"*** means a Director who either (i) is not a current employee or officer of the Company or an Affiliate, does not receive compensation, either directly or indirectly, from the Company or an Affiliate for services rendered as a consultant or in any capacity other than as a Director (except for an amount as to which disclosure would not be required under Item 404(a) of Regulation S-K promulgated pursuant to the Securities Act ("Regulation S-K")), does not possess an interest in any other transaction for which disclosure would be required under Item 404(a) of Regulation S-K, and is not engaged in a business relationship for which disclosure would be required pursuant to Item 404(b) of Regulation SK; or (ii) is otherwise considered a "non-employee director" for purposes of Rule 16b-3.

(cc) ***"Nonstatutory Stock Option"*** means any Option granted pursuant to Section 5 of the Plan that does not qualify as an Incentive Stock Option.

(dd) ***"Officer"*** means a person who is an officer of the Company within the meaning of Section 16 of the Exchange Act.

(ee) ***"Option"*** means a Nonstatutory Stock Option to purchase shares of Common Stock granted pursuant to the Plan.

(ff) ***"Option Agreement"*** means a written agreement between the Company and an Optionholder evidencing the terms and conditions of an Option grant. Each Option Agreement will be subject to the terms and conditions of the Plan.

(gg) ***"Optionholder"*** means a person to whom an Option is granted pursuant to the Plan or, if applicable, such other person who holds an outstanding Option.

(hh) ***"Other Stock Award"*** means an award based in whole or in part by reference to the Common Stock which is granted pursuant to the terms and conditions of Section 6(d).

(ii) ***"Other Stock Award Agreement"*** means a written agreement between the Company and a holder of an Other Stock Award evidencing the terms and conditions of an Other Stock Award grant. Each Other Stock Award Agreement will be subject to the terms and conditions of the Plan.

(jj) ***"Own," "Owned," "Owner," "Ownership"*** means a person or Entity will be deemed to "Own," to have "Owned," to be the "Owner" of, or to have acquired "Ownership" of securities if such person or Entity, directly or indirectly, through any contract, arrangement, understanding, relationship or otherwise, has or shares voting power, which includes the power to vote or to direct the voting, with respect to such securities.

(kk) ***"Participant"*** means an Eligible Individual to whom an Award is granted pursuant to the Plan or, if applicable, such other person who holds an outstanding Stock Award.

(ll) ***"Performance Cash Award"*** means an award of cash granted pursuant to the terms and conditions of Section 6(c)(ii).

(mm) ***"Performance Criteria"*** means the one or more criteria that the Board will select for purposes of establishing the Performance Goals for a Performance Period. The Performance Criteria that will be used to establish such Performance Goals may be based on any one of, or combination of, the following as determined by the Board: (i) earnings (including earnings per share and net earnings); (ii) earnings before interest, taxes and depreciation; (iii) earnings before interest, taxes, depreciation and amortization; (iv) earnings before interest, taxes, depreciation, amortization and legal settlements; (v) earnings before interest, taxes, depreciation, amortization, legal settlements and other income (expense); (vi) earnings before interest, taxes, depreciation, amortization, legal settlements, other income (expense) and stock-based compensation; (vii) earnings before interest, taxes, depreciation, amortization, legal settlements, other income (expense), stock-based compensation and changes in deferred revenue; (viii) total stockholder return; (ix) return on equity or average stockholder's equity; (x) return on assets, investment, or capital employed; (xi) stock price; (xii) margin (including gross margin); (xiii) income (before or after taxes); (xiv) operating income; (xv) operating income after taxes; (xvi) pre-tax profit; (xvii) operating cash flow; (xviii) sales or revenue targets; (xix) increases in revenue or product revenue; (xx) expenses and cost reduction goals; (xxi) improvement in or attainment of working capital levels; (xxii) economic value added (or an equivalent metric); (xxiii) market share; (xxiv) cash flow; (xxv) cash flow per share; (xxvi) share price performance; (xxvii) debt reduction; (xxviii) implementation or completion of projects or processes; (xxix) customer satisfaction; (xxx) stockholders' equity; (xxxi) capital expenditures; (xxxii) debt levels; (xxxiii) operating profit or net operating profit; (xxxiv) workforce diversity; (xxxv) growth of net income

or operating income; (xxxvi) billings; (xxxvii) bookings; (xxxviii) the number of customers, including but not limited to customers users; (xxxix) employee retention; (xl) pre-clinical development related compound goals; (xli) financing; (xlii) regulatory milestones, including approval of a compound; (xliii) stockholder liquidity; (xliv) corporate governance and compliance; (xlv) product commercialization; (xlvi) intellectual property; (xlvii) personnel matters; (xlviii) progress of internal research or clinical programs; (xlix) progress of partnered programs; (l) implementation or completion of projects and processes; (li) partner satisfaction; (lii) budget management; (liii) clinical achievements; (liv) completing phases of a clinical study (including the treatment phase); (lv) announcing or presenting preliminary or final data from clinical studies; in each case, whether on particular timelines or generally; (lvi) timely completion of clinical trials; (lvii) submission of INDs and NDAs and other regulatory achievements; (lviii) partner or collaborator achievements; (lix) internal controls, including those related to the Sarbanes-Oxley Act of 2002; (lx) research progress, including the development of programs; (lxi) investor relations, analysts and communication; (lxii) manufacturing achievements (including obtaining particular yields from manufacturing runs and other measurable objectives related to process development activities); (lxiii) strategic partnerships or transactions (including in-licensing and out-licensing of intellectual property; (lxiv) establishing relationships with commercial entities with respect to the marketing, distribution and sale of the Company's products (including with group purchasing organizations, distributors and other vendors); (lxv) supply chain achievements (including establishing relationships with manufacturers or suppliers of active pharmaceutical ingredients and other component materials and manufacturers of the Company's products); (lxvi) co-development, co-marketing, profit sharing, joint venture or other similar arrangements; and (lxvii) and other measures of performance selected by the Board.

(nn) ***"Performance Goals"*** means, for a Performance Period, the one or more goals established by the Board for the Performance Period based upon the Performance Criteria. Performance Goals may be based on a Company-wide basis, with respect to one or more business units, divisions, Affiliates, or business segments, and in either absolute terms or relative to the performance of one or more comparable companies or the performance of one or more relevant indices. Unless specified otherwise by the Board (i) in the Award Agreement at the time the Award is granted or (ii) in such other document setting forth the Performance Goals at the time the Performance Goals are established, the Board will appropriately make adjustments in the method of calculating the attainment of Performance Goals for a Performance Period as follows: (1) to exclude restructuring and/or other nonrecurring charges; (2) to exclude exchange rate effects; (3) to exclude the effects of changes to generally accepted accounting principles; (4) to exclude the effects of any statutory adjustments to corporate tax rates; (5) to exclude the effects of any "extraordinary items" as determined under generally accepted accounting principles; (6) to exclude the dilutive effects of acquisitions or joint ventures; (7) to assume that any business divested by the Company achieved performance objectives at targeted levels during the balance of a Performance Period following such divestiture; (8) to exclude the effect of any change in the outstanding shares of common stock of the Company by reason of any stock dividend or split, stock repurchase, reorganization, recapitalization, merger, consolidation, spin-off, combination or exchange of shares or other similar corporate change, or any distributions to common stockholders other than regular cash dividends; (9) to exclude the effects of stock based compensation and the award of bonuses under the Company's bonus plans; (10) to exclude costs incurred in connection with potential acquisitions or divestitures that are required to expensed under generally accepted accounting principles; (11) to exclude the goodwill and intangible asset impairment charges that are required to be recorded under generally accepted accounting principles, (12) to exclude the effects of the timing of acceptance for review and/or approval of submissions to the Food and Drug Administration or any other regulatory body and (13) to exclude the effect of any other unusual, nonrecurring gain or loss or other extraordinary item. In addition, the Board retains the discretion to reduce or eliminate the compensation or economic benefit due upon attainment of Performance Goals and to define the manner of calculating the Performance Criteria it selects to use for such Performance Period. Partial achievement of the specified criteria may result in the payment or vesting corresponding to the degree of achievement as specified in the Stock Award Agreement or the written terms of a Performance Cash Award.

(oo) ***"Performance Period"*** means the period of time selected by the Board over which the attainment of one or more Performance Goals will be measured for the purpose of determining a Participant's right to and the payment of a Stock Award or a Performance Cash Award. Performance Periods may be of varying and overlapping duration, at the sole discretion of the Board.

(pp) ***"Performance Stock Award"*** means a Stock Award granted under the terms and conditions of Section 6(c)(i).

(qq) ***"Plan"*** means this 2023 Employment Inducement Award Plan, as it may be amended and/or restated from time to time.

(rr) ***"Restricted Stock Award"*** means an award of shares of Common Stock which is granted pursuant to the terms and conditions of Section 6(a).

(ss) ***"Restricted Stock Award Agreement"*** means a written agreement between the Company and a holder of a Restricted Stock Award evidencing the terms and conditions of a Restricted Stock Award grant. Each Restricted Stock Award Agreement will be subject to the terms and conditions of the Plan.

(tt) ***"Restricted Stock Unit Award"*** means a right to receive shares of Common Stock which is granted pursuant to the terms and conditions of Section 6(b).

(uu) ***"Restricted Stock Unit Award Agreement"*** means a written agreement between the Company and a holder of a Restricted Stock Unit Award evidencing the terms and conditions of a Restricted Stock Unit Award grant. Each Restricted Stock Unit Award Agreement will be subject to the terms and conditions of the Plan.

(vv) ***"Rule 16b-3"*** means Rule 16b-3 promulgated under the Exchange Act or any successor to Rule 16b-3, as in effect from time to time.

(ww) ***"Securities Act"*** means the Securities Act of 1933, as amended.

(xx) ***"Stock Appreciation Right"*** or "SAR" means a right to receive the appreciation on Common Stock that is granted pursuant to the terms and conditions of Section 5.

(yy) ***"Stock Appreciation Right Agreement"*** means a written agreement between the Company and a holder of a Stock Appreciation Right evidencing the terms and conditions of a Stock Appreciation Right grant. Each Stock Appreciation Right Agreement will be subject to the terms and conditions of the Plan.

(zz) ***"Stock Award"*** means any right to receive Common Stock granted under the Plan, including a Nonstatutory Stock Option, a Restricted Stock Award, a Restricted Stock Unit Award, a Stock Appreciation Right, a Performance Stock Award or any Other Stock Award.

(aaa) ***"Stock Award Agreement"*** means a written agreement between the Company and a Participant evidencing the terms and conditions of a Stock Award grant. Each Stock Award Agreement will be subject to the terms and conditions of the Plan.

(bbb) ***"Subsidiary"*** means, with respect to the Company, (i) any corporation of which more than 50% of the outstanding capital stock having ordinary voting power to elect a majority of the board of directors of such corporation (irrespective of whether, at the time, stock of any other class or classes of such corporation will have or might have voting power by reason of the happening of any contingency) is at the time, directly or indirectly, Owned by the Company, and (ii) any partnership, limited liability company or other entity in which the Company has a direct or indirect interest (whether in the form of voting or participation in profits or capital contribution) of more than 50%.

ZEVRA THERAPEUTICS, INC.
STOCK OPTION GRANT NOTICE
(2023 EMPLOYMENT INDUCEMENT AWARD PLAN)

.Zevra Therapeutics, Inc. (the "***Company***"), pursuant to its 2023 Employment Inducement Award Plan (the "***Plan***"), hereby grants to Optionholder an option to purchase the number of shares of the Company's Common Stock set forth below. This option is subject to all of the terms and conditions as set forth in this notice, in the Option Agreement, the Plan and the Notice of Exercise, all of which are attached hereto and incorporated herein in their entirety. Capitalized terms not explicitly defined herein but defined in the Plan or the Option Agreement will have the same definitions as in the Plan or the Option Agreement. If there is any conflict between the terms in this notice and the Plan, the terms of the Plan will control.

Optionholder:[1]
Date of Grant:
Vesting Commencement Date:
Number of Shares Subject to Option:
Exercise Price (Per Share):
Total Exercise Price:
Expiration Date:

Type of Grant: Nonstatutory Stock Option

Exercise Schedule: ☐ Same as Vesting Schedule ☐ Early Exercise Permitted

Vesting Schedule: [As determined by the Board of Directors]

Payment: By one or a combination of the following items (described in the Option Agreement):

☐ By cash, check, bank draft or money order payable to the Company
☐ Pursuant to a Regulation T Program if the shares are publicly traded
☐ By delivery of already-owned shares if the shares are publicly traded
☐ Subject to the Company's consent at the time of exercise, by a "net exercise" arrangement

Additional Terms/Acknowledgements: Optionholder acknowledges receipt of, and understands and agrees to, this Stock Option Grant Notice, the Option Agreement and the Plan. Optionholder acknowledges and agrees that this Stock Option Grant Notice and the Option Agreement may not be modified, amended or revised except as provided in the Plan. Optionholder further acknowledges that as of the Date of Grant, this Stock Option Grant Notice, the Option Agreement, and the Plan set forth the entire understanding between Optionholder and the Company regarding this option award and supersede all prior oral and written agreements, promises and/or representations on that subject with the exception of (i) options previously granted and delivered to Optionholder, (ii) any compensation recovery policy that is adopted by the Company or is otherwise required by applicable law and (iii) any written employment or severance arrangement that would provide for vesting acceleration of this option upon the terms and conditions set forth therein. By accepting this option, Optionholder consents to receive such documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

[*Signature Page Follows*]

ZEVRA THERAPEUTICS, INC.	**OPTIONHOLDER:**
By:	
Signature	Signature
Title:	Date:
Date:	

ATTACHMENTS: Option Agreement, 2023 Employment Inducement Award Plan and Notice of Exercise

ATTACHMENT I
ZEVRA THERAPEUTICS, INC.
2023 Employment Inducement Award Plan
OPTION AGREEMENT
(STOCK OPTION)

Pursuant to your Stock Option Grant Notice ("***Grant Notice***") and this Option Agreement, Zevra Therapeutics, Inc. (the "***Company***") has granted you an option under its 2023 Employment Inducement Award Plan (the "***Plan***") to purchase the number of shares of the Company's Common Stock indicated in your Grant Notice at the exercise price indicated in your Grant Notice. The option is granted to you effective as of the date of grant set forth in the Grant Notice (the "***Date of Grant***"). If there is any conflict between the terms in this Option Agreement and the Plan, the terms of the Plan will control. Capitalized terms not explicitly defined in this Option Agreement or in the Grant Notice but defined in the Plan will have the same definitions as in the Plan.

The details of your option, in addition to those set forth in the Grant Notice and the Plan, are as follows:

1. VESTING. Subject to the provisions contained herein, your option will vest as provided in your Grant Notice. Vesting will cease upon the termination of your Continuous Service.

2. NUMBER OF SHARES AND EXERCISE PRICE. The number of shares of Common Stock subject to your option and your exercise price per share in your Grant Notice will be adjusted for Capitalization Adjustments.

3. EXERCISE RESTRICTION FOR NON-EXEMPT EMPLOYEES. If you are an Employee eligible for overtime compensation under the Fair Labor Standards Act of 1938, as amended (that is, a "***Non-Exempt Employee***"), and except as otherwise provided in the Plan, you may not exercise your option until you have completed at least six (6) months of Continuous Service measured from the Date of Grant, even if you have already been an employee for more than six (6) months. Consistent with the provisions of the Worker Economic Opportunity Act, you may exercise your option as to any vested portion prior to such six (6) month anniversary in the case of (i) your death or disability, (ii) a Corporate Transaction in which your option is not assumed, continued or substituted, (iii) a Change in Control or (iv) your termination of Continuous Service on your "retirement" (as defined in the Company's benefit plans).

4. EXERCISE PRIOR TO VESTING ("EARLY EXERCISE"). You may not exercise your option prior to vesting.

5. METHOD OF PAYMENT. You must pay the full amount of the exercise price for the shares you wish to exercise. You may pay the exercise price in cash or by check, bank draft or money order payable to the Company or in any other manner ***permitted by your Grant Notice,*** which may include one or more of the following:

(a) Provided that at the time of exercise the Common Stock is publicly traded, pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board that, prior to the issuance of Common Stock, results in either the receipt of cash (or check) by the Company or the receipt of irrevocable instructions to pay the aggregate exercise price to the Company from the sales proceeds. This manner of payment is also known as a "broker-assisted exercise", "same day sale", or "sell to cover".

(b) Provided that at the time of exercise the Common Stock is publicly traded, by delivery to the Company (either by actual delivery or attestation) of already-owned shares of Common Stock that are owned free and clear of any liens, claims, encumbrances or security interests, and that are valued at Fair Market Value on the date of exercise. "Delivery" for these purposes, in the sole discretion of the Company at the time you exercise your option, will include delivery to the Company of your attestation of ownership of such shares of Common Stock

in a form approved by the Company. You may not exercise your option by delivery to the Company of Common Stock if doing so would violate the provisions of any law, regulation or agreement restricting the redemption of the Company's stock.

(c) Subject to the consent of the Company at the time of exercise, by a "net exercise" arrangement pursuant to which the Company will reduce the number of shares of Common Stock issued upon exercise of your option by the largest whole number of shares with a Fair Market Value that does not exceed the aggregate exercise price. You must pay any remaining balance of the aggregate exercise price not satisfied by the "net exercise" in cash or other permitted form of payment. Shares of Common Stock will no longer be outstanding under your option and will not be exercisable thereafter if those shares (i) are used to pay the exercise price pursuant to the "net exercise," (ii) are delivered to you as a result of such exercise, and (iii) are withheld to satisfy your tax withholding obligations.

6. WHOLE SHARES. You may exercise your option only for whole shares of Common Stock.

7. SECURITIES LAW COMPLIANCE. In no event may you exercise your option unless the shares of Common Stock issuable upon exercise are then registered under the Securities Act or, if not registered, the Company has determined that your exercise and the issuance of the shares would be exempt from the registration requirements of the Securities Act. The exercise of your option also must comply with all other applicable laws and regulations governing your option, and you may not exercise your option if the Company determines that such exercise would not be in material compliance with such laws and regulations (including any restrictions on exercise required for compliance with Treas. Reg. 1.401(k)-1(d)(3), if applicable).

8. TERM. You may not exercise your option before the Date of Grant or after the expiration of the option's term. The term of your option expires, subject to the provisions of Section 5(h) of the Plan, upon the earliest of the following:

(a) immediately upon the termination of your Continuous Service for Cause;

(b) three (3) months after the termination of your Continuous Service for any reason other than Cause, your Disability or your death (except as otherwise provided in Section 8(d) below); *provided, however,* that if during any part of such three (3) month period your option is not exercisable solely because of the condition set forth in the section above relating to "Securities Law Compliance," your option will not expire until the earlier of the Expiration Date or until it has been exercisable for an aggregate period of three (3) months after the termination of your Continuous Service; *provided further,* if during any part of such three (3) month period, the sale of any Common Stock received upon exercise of your option would violate the Company's insider trading policy, then your option will not expire until the earlier of the Expiration Date or until it has been exercisable for an aggregate period of three (3) months after the termination of your Continuous Service during which the sale of the Common Stock received upon exercise of your option would not be in violation of the Company's insider trading policy. Notwithstanding the foregoing, if (i) you are a Non-Exempt Employee, (ii) your Continuous Service terminates within six (6) months after the Date of Grant, and (iii) you have vested in a portion of your option at the time of your termination of Continuous Service, your option will not expire until the earlier of (x) the later of (A) the date that is seven (7) months after the Date of Grant, and (B) the date that is three (3) months after the termination of your Continuous Service, and (y) the Expiration Date;

(c) twelve (12) months after the termination of your Continuous Service due to your Disability (except as otherwise provided in Section 8(d)) below;

(d) eighteen (18) months after your death if you die either during your Continuous Service or within three (3) months after your Continuous Service terminates for any reason other than Cause;

(e) the Expiration Date indicated in your Grant Notice; or

(f) the day before the tenth (10th) anniversary of the Date of Grant.

9. Exercise

(a) You may exercise the vested portion of your option (and the unvested portion of your option if your Grant Notice so permits) during its term by (i) delivering a Notice of Exercise (in a form designated by the Company) or completing such other documents and/or procedures designated by the Company for exercise and (ii) paying the

exercise price and any applicable withholding taxes to the Company's Secretary, stock plan administrator, or such other person as the Company may designate, together with such additional documents as the Company may then require.

(b) By exercising your option you agree that, as a condition to any exercise of your option, the Company may require you to enter into an arrangement providing for the payment by you to the Company of any tax withholding obligation of the Company arising by reason of (i) the exercise of your option, (ii) the lapse of any substantial risk of forfeiture to which the shares of Common Stock are subject at the time of exercise, or (iii) the disposition of shares of Common Stock acquired upon such exercise.

(c) By accepting your option you agree that you will not sell, dispose of, transfer, make any short sale of, grant any option for the purchase of, or enter into any hedging or similar transaction with the same economic effect as a sale, any shares of Common Stock or other securities of the Company held by you, for a period of one hundred eighty (180) days following the effective date of a registration statement of the Company filed under the Securities Act or such longer period as the underwriters or the Company will request to facilitate compliance with FINRA Rule 2711 or NYSE Member Rule 472 or any successor or similar rules or regulation (the "***Lock-Up Period***"); *provided, however*, that nothing contained in this section will prevent the exercise of a repurchase option, if any, in favor of the Company during the Lock-Up Period. You further agree to execute and deliver such other agreements as may be reasonably requested by the Company or the underwriters that are consistent with the foregoing or that are necessary to give further effect thereto. In order to enforce the foregoing covenant, the Company may impose stop-transfer instructions with respect to your shares of Common Stock until the end of such period. You also agree that any transferee of any shares of Common Stock (or other securities) of the Company held by you will be bound by this Section 9(c). The underwriters of the Company's stock are intended third party beneficiaries of this Section 9(c) and will have the right, power and authority to enforce the provisions hereof as though they were a party hereto.

10. TRANSFERABILITY. Except as otherwise provided in this Section 10, your option is not transferable, except by will or by the laws of descent and distribution, and is exercisable during your life only by you.

(a) Certain Trusts. Upon receiving written permission from the Board or its duly authorized designee, you may transfer your option to a trust if you are considered to be the sole beneficial owner (determined under Section 671 of the Code and applicable state law) while the option is held in the trust. You and the trustee must enter into transfer and other agreements required by the Company.

(b) Domestic Relations Orders. Upon receiving written permission from the Board or its duly authorized designee, and provided that you and the designated transferee enter into transfer and other agreements required by the Company, you may transfer your option pursuant to the terms of a domestic relations order or official marital settlement agreement that contains the information required by the Company to effectuate the transfer. You are encouraged to discuss the proposed terms of any division of this option with the Company prior to finalizing the domestic relations order or marital settlement agreement to help ensure the required information is contained within the domestic relations order or marital settlement agreement.

(c) Beneficiary Designation. Upon receiving written permission from the Board or its duly authorized designee, you may, by delivering written notice to the Company, in a form approved by the Company and any broker designated by the Company to handle option exercises, designate a third party who, on your death, will thereafter be entitled to exercise this option and receive the Common Stock or other consideration resulting from such exercise. In the absence of such a designation, your executor or administrator of your estate will be entitled to exercise this option and receive, on behalf of your estate, the Common Stock or other consideration resulting from such exercise.

11. OPTION NOT A SERVICE CONTRACT. Your option is not an employment or service contract, and nothing in your option will be deemed to create in any way whatsoever any obligation on your part to continue in the employ of the Company or an Affiliate, or of the Company or an Affiliate to continue your employment. In addition, nothing in your option will obligate the Company or an Affiliate, their respective stockholders, boards of directors, officers or employees to continue any relationship that you might have as a Director or Consultant for the Company or an Affiliate.

12. Withholding Obligations.

(a) At the time you exercise your option, in whole or in part, and at any time thereafter as requested by the Company, you hereby authorize withholding from payroll and any other amounts payable to you, and otherwise agree to make adequate provision for (including by means of a "same day sale" pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board to the extent permitted by the Company), any sums required to satisfy the federal, state, local and foreign tax withholding obligations of the Company or an Affiliate, if any, which arise in connection with the exercise of your option.

(b) Upon your request and subject to approval by the Company, and compliance with any applicable legal conditions or restrictions, the Company may withhold from fully vested shares of Common Stock otherwise issuable to you upon the exercise of your option a number of whole shares of Common Stock having a Fair Market Value, determined by the Company as of the date of exercise, not in excess of the minimum amount of tax required to be withheld by law (or such lower amount as may be necessary to avoid classification of your option as a liability for financial accounting purposes). If the date of determination of any tax withholding obligation is deferred to a date later than the date of exercise of your option, share withholding pursuant to the preceding sentence shall not be permitted unless you make a proper and timely election under Section 83(b) of the Code, covering the aggregate number of shares of Common Stock acquired upon such exercise with respect to which such determination is otherwise deferred, to accelerate the determination of such tax withholding obligation to the date of exercise of your option. Notwithstanding the filing of such election, shares of Common Stock shall be withheld solely from fully vested shares of Common Stock determined as of the date of exercise of your option that are otherwise issuable to you upon such exercise. Any adverse consequences to you arising in connection with such share withholding procedure shall be your sole responsibility.

(c) You may not exercise your option unless the tax withholding obligations of the Company and/or any Affiliate are satisfied. Accordingly, you may not be able to exercise your option when desired even though your option is vested, and the Company will have no obligation to issue a certificate for such shares of Common Stock or release such shares of Common Stock from any escrow provided for herein, if applicable, unless such obligations are satisfied.

13. TAX CONSEQUENCES. You hereby agree that the Company does not have a duty to design or administer the Plan or its other compensation programs in a manner that minimizes your tax liabilities. You will not make any claim against the Company, or any of its Officers, Directors, Employees or Affiliates related to tax liabilities arising from your option or your other compensation. In particular, you acknowledge that this option is exempt from Section 409A of the Code only if the exercise price per share specified in the Grant Notice is at least equal to the "fair market value" per share of the Common Stock on the Date of Grant and there is no other impermissible deferral of compensation associated with the option.

14. NOTICES. Any notices provided for in your option or the Plan will be given in writing (including electronically) and will be deemed effectively given upon receipt or, in the case of notices delivered by mail by the Company to you, five (5) days after deposit in the United States mail, postage prepaid, addressed to you at the last address you provided to the Company. The Company may, in its sole discretion, decide to deliver any documents related to participation in the Plan and this option by electronic means or to request your consent to participate in the Plan by electronic means. By accepting this option, you consent to receive such documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

15. GOVERNING PLAN DOCUMENT. Your option is subject to all the provisions of the Plan, the provisions of which are hereby made a part of your option, and is further subject to all interpretations, amendments, rules and regulations, which may from time to time be promulgated and adopted pursuant to the Plan. If there is any conflict between the provisions of your option and those of the Plan, the provisions of the Plan will control. In addition, your option (and any compensation paid or shares issued under your option) is subject to recoupment in accordance with The Dodd–Frank Wall Street Reform and Consumer Protection Act and any implementing regulations thereunder, any clawback policy adopted by the Company and any compensation recovery policy otherwise required by applicable law.

16. OTHER DOCUMENTS. You hereby acknowledge receipt of and the right to receive a document providing the information required by Rule 428(b)(1) promulgated under the Securities Act, which includes the Plan prospectus.

In addition, you acknowledge receipt of the Company's policy permitting certain individuals to sell shares only during certain "window" periods and the Company's insider trading policy, in effect from time to time.

17. EFFECT ON OTHER EMPLOYEE BENEFIT PLANS. The value of this option will not be included as compensation, earnings, salaries, or other similar terms used when calculating your benefits under any employee benefit plan sponsored by the Company or any Affiliate, except as such plan otherwise expressly provides. The Company expressly reserves its rights to amend, modify, or terminate any of the Company's or any Affiliate's employee benefit plans.

18. VOTING RIGHTS. You will not have voting or any other rights as a stockholder of the Company with respect to the shares to be issued pursuant to this option until such shares are issued to you. Upon such issuance, you will obtain full voting and other rights as a stockholder of the Company. Nothing contained in this option, and no action taken pursuant to its provisions, will create or be construed to create a trust of any kind or a fiduciary relationship between you and the Company or any other person.

19. SEVERABILITY. If all or any part of this Option Agreement or the Plan is declared by any court or governmental authority to be unlawful or invalid, such unlawfulness or invalidity will not invalidate any portion of this Option Agreement or the Plan not declared to be unlawful or invalid. Any Section of this Option Agreement (or part of such a Section) so declared to be unlawful or invalid shall, if possible, be construed in a manner which will give effect to the terms of such Section or part of a Section to the fullest extent possible while remaining lawful and valid.

20. MISCELLANEOUS.

(a) The rights and obligations of the Company under your option will be transferable to any one or more persons or entities, and all covenants and agreements hereunder will inure to the benefit of, and be enforceable by the Company's successors and assigns.

(b) You agree upon request to execute any further documents or instruments necessary or desirable in the sole determination of the Company to carry out the purposes or intent of your option.

(c) You acknowledge and agree that you have reviewed your option in its entirety, have had an opportunity to obtain the advice of counsel prior to executing and accepting your option, and fully understand all provisions of your option.

(d) This Option Agreement will be subject to all applicable laws, rules, and regulations, and to such approvals by any governmental agencies or national securities exchanges as may be required.

(e) All obligations of the Company under the Plan and this Option Agreement will be binding on any successor to the Company, whether the existence of such successor is the result of a direct or indirect purchase, merger, consolidation, or otherwise, of all or substantially all of the business and/or assets of the Company.

21. EMPLOYMENT INDUCEMENT AWARD. THIS AWARD IS INTENDED TO CONSTITUTE AN "EMPLOYMENT INDUCEMENT AWARD" UNDER NASDAQ RULE 5635(C)(4) THAT IS EXEMPTED FROM THE REQUIREMENTS OF SHAREHOLDER APPROVAL OF EQUITY COMPENSATION PLANS UNDER NASDAQ RULE 5635(C)(4). THIS AGREEMENT AND THE TERMS AND CONDITIONS OF THIS AWARD WILL BE INTERPRETED CONSISTENT WITH SUCH INTENT.

* * *

This Option Agreement will be deemed to be signed by you upon the signing by you of the Stock Option Grant Notice to which it is attached.

ATTACHMENT III
NOTICE OF EXERCISE

Zevra Therapeutics, Inc.

1180 Celebration Blvd, Suite 103

Celebration, FL 34747

Date of Exercise: [_____]

Ladies and Gentlemen:

This constitutes notice to Zevra Therapeutics, Inc. (the "***Company***") under my stock option described below (the "***Option***") granted pursuant to the Company's 2023 Employment Inducement Award Plan (as it may be amended from time to time) as evidenced by the Stock Option Grant Notice and Option Agreement thereunder, that I elect to purchase the below number of shares of Common Stock of the Company (the "***Shares***") for the price set forth below.

Option Grant Date:	
Number of Shares as to which the Option is exercised:	
Certificates to be issued in name of:	
Total exercise price:	$
Cash payment delivered herewith:	$
[Value of Shares delivered herewith][1]:	$
[Value of Shares pursuant to net exercise][2]:	$
[Regulation T Program (cashless exercise)][3]:	$

By this exercise, I agree (i) to provide such additional documents as you may require pursuant to the terms of the Company's 2023 Employment Inducement Award Plan (as it may be amended from time to time) and (ii) to provide for the payment by me to the Company (in the manner designated by the Company) of the Company's withholding obligation, if any, relating to the exercise of the Option.

Very truly yours,

Signature

Print Name

ZEVRA THERAPEUTICS, INC.
RESTRICTED STOCK AWARD GRANT NOTICE
(2023 EMPLOYMENT INDUCEMENT AWARD PLAN)

Zevra Therapeutics, Inc. (the "***Company***"), pursuant to its 2023 Employment Inducement Award Plan (the "***Plan***"), hereby awards to Participant the number of shares of the Company's Common Stock set forth below ("***Award***"). This Award is subject to all of the terms and conditions as set forth herein and in the Restricted Stock Award Agreement and the Plan, all of which are attached hereto and incorporated herein in their entirety. Capitalized terms not otherwise defined herein shall have the meanings set forth in the Plan or the Restricted Stock Award Agreement. In the event of any conflict between the terms in the Award and the Plan, the terms of the Plan shall control.

Participant:[1]	
Date of Grant:	
Number of Shares Subject to Award:	
Consideration:	Participant's Services

Vesting Schedule: [As determined by the Board of Directors]

Additional Terms/Acknowledgements: The undersigned Participant acknowledges receipt of, and understands and agrees to, this Restricted Stock Award Grant Notice, the Restricted Stock Award Agreement and the Plan. Participant acknowledges and agrees that this Restricted Stock Award Grant Notice and the Restricted Stock Award Agreement may not be modified, amended or revised except as provided therein or in the Plan. Participant further acknowledges that as of the Date of Grant, this Restricted Stock Award Grant Notice, the Restricted Stock Award Agreement and the Plan set forth the entire understanding between Participant and the Company regarding the acquisition of the Common Stock pursuant to the Award specified above and supersede all prior oral and written agreements on that subject with the exception, if applicable, of (i) the written employment agreement, offer letter agreement entered into between the Company and Participant specifying the terms that should govern this specific Award, if any, and (ii) any compensation recovery policy that is adopted by the Company or is otherwise required by applicable law.

Participant consents to receive Plan documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

ZEVRA THERAPEUTICS, INC.
By: _____
Signature
Title: _____
Date: _____

PARTICIPANT:
_____
Signature
Date: _____

ATTACHMENTS:

Attachment I: Restricted Stock Award Agreement

Attachment II: 2023 Employment Inducement Award Plan

ATTACHMENT I
ZEVRA THERAPEUTICS, INC.
2023 EMPLOYMENT INDUCEMENT AWARD PLAN
RESTRICTED STOCK AWARD AGREEMENT

Pursuant to the Restricted Stock Award Grant Notice ("***Grant Notice***") and this Stock Award Agreement (collectively, the "***Award***") and in consideration of your services, Zevra Therapeutics, Inc. (the "***Company***") has awarded you ("***Participant***") a Restricted Stock Award under its 2023 Employment Inducement Award Plan (the "***Plan***") for the number of shares of the Company's Common Stock subject to the Award as indicated in the Grant Notice. Capitalized terms not explicitly defined in this Restricted Stock Award Agreement but defined in the Plan shall have the same definitions as in the Plan. The details of your Award, in addition to those set forth in the Grant Notice and the Plan, are as follows.

1. Vesting. The shares of Common Stock subject to your Award are fully vested upon issuance. This means that you are not required to perform additional services on behalf of the Company or any of its Affiliates, and no other performance conditions or other contingencies must be satisfied, in order for you to retain the shares.

2. Number of Shares. The number of shares subject to your Award may be adjusted from time to time for Capitalization Adjustments, as provided in the Plan.

3. Securities Law Compliance. Notwithstanding anything to the contrary contained herein, you may not be issued any shares of Common Stock under your Award unless the shares are either (i) then registered under the Securities Act or (ii) the Company has determined that such issuance would be exempt from the registration requirements of the Securities Act. Your Award must also comply with other applicable laws and regulations governing the Award, and you will not receive such shares if the Company determines that such receipt would not be in material compliance with such laws and regulations.

4. Restrictive Legends. The shares issued under your Award shall be endorsed with appropriate legends determined by the Company.

5. Award not a Service Contract. Your Award is not an employment or service contract, and nothing in your Award shall be deemed to create in any way whatsoever any obligation on your part to continue in the employ of the Company or an Affiliate, or on the part of the Company or an Affiliate to continue your employment. In addition, nothing in your Award shall obligate the Company or an Affiliate, their respective stockholders, boards of directors, Officers or Employees to continue any relationship that you might have as a Director or Consultant for the Company or an Affiliate.

6. WITHHOLDING OBLIGATIONS.

(a) At the time your Award is made, or at any time thereafter as requested by the Company, you hereby authorize withholding from payroll and any amounts payable to you, and otherwise agree to make adequate provision for any sums required to satisfy the federal, state, local and foreign tax withholding obligations of the Company or an Affiliate, if any, which arise in connection with your Award.

(b) Unless the tax withholding obligations of the Company and/or any Affiliate are satisfied, the Company shall have no obligation to issue a certificate for such shares or release such shares from any escrow provided for herein.

7. Notices. Any notices provided for in your Award or the Plan shall be given in writing and shall be deemed effectively given upon receipt or, in the case of notices delivered by the Company to you, five (5) days after deposit in the United States mail, postage prepaid, addressed to you at the last address you provided to the Company.

8. MISCELLANEOUS.

(a) The rights and obligations of the Company under your Award shall be transferable to any one or more persons or entities, and all covenants and agreements hereunder shall inure to the benefit of, and be enforceable by the Company's successors and assigns. Your rights and obligations under your Award may only be assigned with the prior written consent of the Board in its sole discretion.

(b) You agree upon request to execute any further documents or instruments necessary or desirable in the sole determination of the Company to carry out the purposes or intent of your Award.

(c) You acknowledge and agree that you have reviewed your Award in its entirety, have had an opportunity to obtain the advice of counsel prior to executing and accepting your Award and fully understand all provisions of your Award.

9. Employment Inducement Award. This Award is intended to constitute an "employment inducement award" under NASDAQ Rule 5635(c)(4) that is exempted from the requirements of shareholder approval of equity compensation plans under NASDAQ Rule 5635(c)(4). This agreement and the terms and conditions of this Award will be interpreted consistent with such intent.

10. Governing Plan Document. Your Award is subject to all the provisions of the Plan, the provisions of which are hereby made a part of your Award, and is further subject to all interpretations, amendments, rules and regulations which may from time to time be promulgated and adopted pursuant to the Plan. In the event of any conflict between the provisions of your Award and those of the Plan, the provisions of the Plan shall control.

*****

This Restricted Stock Award Agreement shall be deemed to be signed by the Company and Participant upon the acceptance by the Participant of the Restricted Stock Award Grant Notice.

ZEVRA THERAPEUTICS, INC.
RESTRICTED STOCK UNIT GRANT NOTICE
(2023 EMPLOYMENT INDUCEMENT AWARD PLAN)

Zevra Therapeutics, Inc. (the "***Company***"), pursuant to the Company's 2023 Employment Inducement Award Plan (the "***Plan***"), hereby awards to Participant a Restricted Stock Unit Award for the number of shares of the Company's Common Stock ("***Restricted Stock Units***") set forth below (the "***Award***"). The Award is subject to all of the terms and conditions as set forth in this notice of grant (this "***Restricted Stock Unit Grant Notice***") and in the Plan and the Restricted Stock Unit Award Agreement (the "***Award Agreement***"), both of which are attached hereto and incorporated herein in their entirety. Capitalized terms not otherwise defined herein shall have the meanings set forth in the Plan or the Award Agreement. In the event of any conflict between the terms in the Award and the Plan, the terms of the Plan shall control.

Participant:[1]
ID:
Date of Grant:
Grant Number:
Vesting Commencement Date:
Number of Restricted Stock Units/Shares:

Vesting Schedule: The shares subject to the Award shall vest as follows: [To be Determined by the Board of Directors].

Issuance Schedule: Subject to any change on a Capitalization Adjustment, one share of Common Stock will be issued for each Restricted Stock Unit that vests at the time set forth in Section 6 of the Award Agreement.

Additional Terms/Acknowledgements: Participant acknowledges receipt of, and understands and agrees to, this Restricted Stock Unit Grant Notice, the Award Agreement and the Plan. Participant further acknowledges that as of the Date of Grant, this Restricted Stock Unit Grant Notice, the Award Agreement and the Plan set forth the entire understanding between Participant and the Company regarding the acquisition of the Common Stock pursuant to the Award specified above and supersede all prior oral and written agreements on the terms of this Award with the exception, if applicable, of (i) the written employment agreement or offer letter agreement entered into between the Company and Participant specifying the terms that should govern this specific Award, and (ii) any compensation recovery policy that is adopted by the Company or is otherwise required by applicable law.

By accepting this Award, Participant acknowledges having received and read the Restricted Stock Unit Grant Notice, the Award Agreement and the Plan and agrees to all of the terms and conditions set forth in these documents. Participant consents to receive Plan documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

Other Agreements:

ZEVRA THERAPEUTICS, INC.	**PARTICIPANT**
By:	
Signature	Signature
Title:	Date:
Date:	

ATTACHMENTS: Award Agreement and 2023 Employment Inducement Award Plan

ZEVRA THERAPEUTICS, INC.
2023 EMPLOYMENT INDUCEMENT AWARD PLAN
RESTRICTED STOCK UNIT AWARD AGREEMENT

Pursuant to the Restricted Stock Unit Grant Notice (the "***Grant Notice***") and this Restricted Stock Unit Award Agreement (the "***Agreement***"), Zevra Therapeutics, Inc. (the "***Company***") has awarded you ("***Participant***") a Restricted Stock Unit Award (the "***Award***") pursuant to the Company's 2023 Employment Inducement Award Plan (the "***Plan***") for the number of Restricted Stock Units/shares indicated in the Grant Notice. Capitalized terms not explicitly defined in this Agreement or the Grant Notice shall have the same meanings given to them in the Plan. The terms of your Award, in addition to those set forth in the Grant Notice, are as follows.

1. GRANT OF THE AWARD. This Award represents the right to be issued on a future date one (1) share of Common Stock for each Restricted Stock Unit that vests on the applicable vesting date(s) (subject to any adjustment under Section 3 below) as indicated in the Grant Notice. As of the Date of Grant, the Company will credit to a bookkeeping account maintained by the Company for your benefit (the "***Account***") the number of Restricted Stock Units/shares of Common Stock subject to the Award. This Award was granted in consideration of your services to the Company.

2. VESTING. Subject to the limitations contained herein, your Award will vest, if at all, in accordance with the vesting schedule provided in the Grant Notice, provided that vesting will cease upon the termination of your Continuous Service. Upon such termination of your Continuous Service, the Restricted Stock Units/shares of Common Stock credited to the Account that were not vested on the date of such termination will be forfeited at no cost to the Company and you will have no further right, title or interest in or to such underlying shares of Common Stock.

3. NUMBER OF SHARES. The number of Restricted Stock Units/shares subject to your Award may be adjusted from time to time for Capitalization Adjustments, as provided in the Plan. Any additional Restricted Stock Units, shares, cash or other property that becomes subject to the Award pursuant to this Section 3, if any, shall be subject, in a manner determined by the Board, to the same forfeiture restrictions, restrictions on transferability, and time and manner of delivery as applicable to the other Restricted Stock Units and shares covered by your Award. Notwithstanding the provisions of this Section 3, no fractional shares or rights for fractional shares of Common Stock shall be created pursuant to this Section 3. Any fraction of a share will be rounded down to the nearest whole share.

4. SECURITIES LAW COMPLIANCE. You may not be issued any Common Stock under your Award unless the shares of Common Stock underlying the Restricted Stock Units are either (i) then registered under the Securities Act, or (ii) the Company has determined that such issuance would be exempt from the registration requirements of the Securities Act. Your Award must also comply with other applicable laws and regulations governing the Award, and you shall not receive such Common Stock if the Company determines that such receipt would not be in material compliance with such laws and regulations.

5. TRANSFER RESTRICTIONS. Prior to the time that shares of Common Stock have been delivered to you, you may not transfer, pledge, sell or otherwise dispose of this Award or the shares issuable in respect of your Award, except as expressly provided in this Section 5. For example, you may not use shares that may be issued in respect of your Restricted Stock Units as security for a loan. The restrictions on transfer set forth herein will lapse upon delivery to you of shares in respect of your vested Restricted Stock Units.

(a) Death. Your Award is transferable by will and by the laws of descent and distribution. At your death, vesting of your Award will cease and your executor or administrator of your estate shall be entitled to receive, on behalf of your estate, any Common Stock or other consideration that vested but was not issued before your death.

(b) Domestic Relations Orders. Upon receiving written permission from the Board or its duly authorized designee, and provided that you and the designated transferee enter into transfer and other agreements required by the Company, you may transfer your right to receive the distribution of Common Stock or other consideration hereunder, pursuant to a domestic relations order or marital settlement agreement that contains the information required by the Company to effectuate the transfer. You are encouraged to discuss the proposed terms of any

division of this Award with the Company General Counsel prior to finalizing the domestic relations order or marital settlement agreement to verify that you may make such transfer, and if so, to help ensure the required information is contained within the domestic relations order or marital settlement agreement.

6. Date of Issuance

(a) The issuance of shares in respect of the Restricted Stock Units is intended to comply with Treasury Regulations Section 1.409A-1(b)(4) and will be construed and administered in such a manner. Subject to the satisfaction of the withholding obligations set forth in this Agreement, in the event one or more Restricted Stock Units vests, the Company shall issue to you one (1) share of Common Stock for each Restricted Stock Unit that vests on the applicable vesting date(s) (subject to any adjustment under Section 3 above). The issuance date determined by this paragraph is referred to as the "***Original Issuance Date***".

(b) If the Original Issuance Date falls on a date that is not a business day, delivery shall instead occur on the next following business day. In addition, if:

(i) the Original Issuance Date does not occur (1) during an "open window period" applicable to you, as determined by the Company in accordance with the Company's then-effective policy on trading in Company securities, or (2) on a date when you are otherwise permitted to sell shares of Common Stock on an established stock exchange or stock market, *and*

(ii) either (1) Withholding Taxes do not apply, or (2) the Company decides, prior to the Original Issuance Date, (A) not to satisfy the Withholding Taxes by withholding shares of Common Stock from the shares otherwise due, on the Original Issuance Date, to you under this Award, and (B) not to permit you to pay your Withholding Taxes in cash,

then the shares that would otherwise be issued to you on the Original Issuance Date will not be delivered on such Original Issuance Date and will instead be delivered on the first business day when you are not prohibited from selling shares of the Company's Common Stock in the open public market, but in no event later than December 31 of the calendar year in which the Original Issuance Date occurs (that is, the last day of your taxable year in which the Original Issuance Date occurs), or, if and only if permitted in a manner that complies with Treasury Regulations Section 1.409A-1(b)(4), no later than the date that is the 15th day of the third calendar month of the applicable year following the year in which the shares of Common Stock under this Award are no longer subject to a "substantial risk of forfeiture" within the meaning of Treasury Regulations Section 1.409A-1(d).

(c) The form of delivery (e.g., a stock certificate or electronic entry evidencing such shares) shall be determined by the Company.

7. DIVIDENDS. You shall receive no benefit or adjustment to your Award with respect to any cash dividend, stock dividend or other distribution that does not result from a Capitalization Adjustment.

8. RESTRICTIVE LEGENDS. The shares of Common Stock issued under your Award shall be endorsed with appropriate legends as determined by the Company.

9. EXECUTION OF DOCUMENTS. You hereby acknowledge and agree that the manner selected by the Company by which you indicate your consent to your Grant Notice is also deemed to be your execution of your Grant Notice and of this Agreement. You further agree that such manner of indicating consent may be relied upon as your signature for establishing your execution of any documents to be executed in the future in connection with your Award.

10. Award Not a Service Contract.

(a) Nothing in this Agreement (including, but not limited to, the vesting of your Award or the issuance of the shares subject to your Award), the Plan or any covenant of good faith and fair dealing that may be found implicit in this Agreement or the Plan shall: (i) confer upon you any right to continue in the employ of, or affiliation with, the Company or an Affiliate; (ii) constitute any promise or commitment by the Company or an Affiliate regarding the fact or nature of future positions, future work assignments, future compensation or any other term or condition of employment or affiliation; (iii) confer any right or benefit under this Agreement or the Plan unless such right or benefit has specifically accrued under the terms of this Agreement or Plan; or (iv) deprive the Company of the right to terminate you at will and without regard to any future vesting opportunity that you may have.

(b) The Company has the right to reorganize, sell, spin-out or otherwise restructure one or more of its businesses or Affiliates at any time or from time to time, as it deems appropriate (a "***reorganization***"). Such a reorganization could result in the termination of your Continuous Service, or the termination of Affiliate status of your employer and the loss of benefits available to you under this Agreement, including but not limited to, the termination of the right to continue vesting in the Award. This Agreement, the Plan, the transactions contemplated hereunder and the vesting schedule set forth herein or any covenant of good faith and fair dealing that may be found implicit in any of them do not constitute an express or implied promise of continued engagement as an employee or consultant for the term of this Agreement, for any period, or at all, and shall not interfere in any way with the Company's right to conduct a reorganization.

11. Withholding Obligations

(a) On each vesting date, and on or before the time you receive a distribution of the shares underlying your Restricted Stock Units, and at any other time as reasonably requested by the Company in accordance with applicable tax laws, you hereby authorize any required withholding from the Common Stock issuable to you and/or otherwise agree to make adequate provision in cash for any sums required to satisfy the federal, state, local and foreign tax withholding obligations of the Company or any Affiliate that arise in connection with your Award (the "***Withholding Taxes***"). Additionally, the Company or any Affiliate may, in its sole discretion, satisfy all or any portion of the Withholding Taxes obligation relating to your Award by any of the following means or by a combination of such means: (i) withholding from any compensation otherwise payable to you by the Company; (ii) causing you to tender a cash payment; (iii) permitting or requiring you to enter into a "same day sale" commitment, if applicable, with a broker-dealer that is a member of the Financial Industry Regulatory Authority (a "***FINRA Dealer***") whereby you irrevocably elect to sell a portion of the shares to be delivered in connection with your Restricted Stock Units to satisfy the Withholding Taxes and whereby the FINRA Dealer irrevocably commits to forward the proceeds necessary to satisfy the Withholding Taxes directly to the Company and/or its Affiliates; or (iv) withholding shares of Common Stock from the shares of Common Stock issued or otherwise issuable to you in connection with the Award with a Fair Market Value (measured as of the date shares of Common Stock are issued to pursuant to Section 6) equal to the amount of such Withholding Taxes; *provided*, *however*, that the number of such shares of Common Stock so withheld will not exceed the amount necessary to satisfy the Company's required tax withholding obligations using the minimum statutory withholding rates for federal, state, local and foreign tax purposes, including payroll taxes, that are applicable to supplemental taxable income; and *provided*, further, that to the extent necessary to qualify for an exemption from application of Section 16(b) of the Exchange Act, if applicable, such share withholding procedure will be subject to the express prior approval of the Company's Compensation Committee.

(b) Unless the tax withholding obligations of the Company and/or any Affiliate are satisfied, the Company shall have no obligation to deliver to you any Common Stock.

(c) In the event the Company's obligation to withhold arises prior to the delivery to you of Common Stock or it is determined after the delivery of Common Stock to you that the amount of the Company's withholding obligation was greater than the amount withheld by the Company, you agree to indemnify and hold the Company harmless from any failure by the Company to withhold the proper amount.

12. TAX CONSEQUENCES. The Company has no duty or obligation to minimize the tax consequences to you of this Award and shall not be liable to you for any adverse tax consequences to you arising in connection with this Award. You are hereby advised to consult with your own personal tax, financial and/or legal advisors regarding the tax consequences of this Award and by signing the Grant Notice, you have agreed that you have done so or knowingly and voluntarily declined to do so. You understand that you (and not the Company) shall be responsible for your own tax liability that may arise as a result of this investment or the transactions contemplated by this Agreement.

13. UNSECURED OBLIGATION. Your Award is unfunded, and as a holder of a vested Award, you shall be considered an unsecured creditor of the Company with respect to the Company's obligation, if any, to issue shares or other property pursuant to this Agreement. You shall not have voting or any other rights as a stockholder of the Company with respect to the shares to be issued pursuant to this Agreement until such shares are issued to you pursuant to Section 6 of this Agreement. Upon such issuance, you will obtain full voting and other rights as a stockholder of the Company. Nothing contained in this Agreement, and no action taken pursuant to its provisions,

shall create or be construed to create a trust of any kind or a fiduciary relationship between you and the Company or any other person.

14. NOTICES. Any notice or request required or permitted hereunder shall be given in writing to each of the other parties hereto and shall be deemed effectively given on the earlier of (i) the date of personal delivery, including delivery by express courier, or delivery via electronic means, or (ii) the date that is five (5) days after deposit in the United States Post Office (whether or not actually received by the addressee), by registered or certified mail with postage and fees prepaid, addressed at the following addresses, or at such other address(es) as a party may designate by ten (10) days' advance written notice to each of the other parties hereto:

COMPANY:	Zevra Therapeutics, Inc. Attn: Stock Administrator 1180 Celebration Blvd, Suite 103 Celebration, FL 34747
PARTICIPANT:	Your address as on file with the Company at the time notice is given

15. HEADINGS. The headings of the Sections in this Agreement are inserted for convenience only and shall not be deemed to constitute a part of this Agreement or to affect the meaning of this Agreement.

16. Miscellaneous.

(a) The rights and obligations of the Company under your Award shall be transferable by the Company to any one or more persons or entities, and all covenants and agreements hereunder shall inure to the benefit of, and be enforceable by, the Company's successors and assigns.

(b) You agree upon request to execute any further documents or instruments necessary or desirable in the sole determination of the Company to carry out the purposes or intent of your Award.

(c) You acknowledge and agree that you have reviewed your Award in its entirety, have had an opportunity to obtain the advice of counsel prior to executing and accepting your Award and fully understand all provisions of your Award.

(d) This Agreement shall be subject to all applicable laws, rules, and regulations, and to such approvals by any governmental agencies or national securities exchanges as may be required.

(e) All obligations of the Company under the Plan and this Agreement shall be binding on any successor to the Company, whether the existence of such successor is the result of a direct or indirect purchase, merger, consolidation, or otherwise, of all or substantially all of the business and/or assets of the Company.

17. GOVERNING PLAN DOCUMENT. Your Award is subject to all the provisions of the Plan, the provisions of which are hereby made a part of your Award, and is further subject to all interpretations, amendments, rules and regulations which may from time to time be promulgated and adopted pursuant to the Plan. Your Award (and any compensation paid or shares issued under your Award) is subject to recoupment in accordance with The Dodd–Frank Wall Street Reform and Consumer Protection Act and any implementing regulations thereunder, any clawback policy adopted by the Company and any compensation recovery policy otherwise required by applicable law. No recovery of compensation under such a clawback policy will be an event giving rise to a right to voluntarily terminate employment upon a resignation for "good reason," or for a "constructive termination" or any similar term under any plan of or agreement with the Company.

18. EFFECT ON OTHER EMPLOYEE BENEFIT PLANS. The value of the Award subject to this Agreement shall not be included as compensation, earnings, salaries, or other similar terms used when calculating benefits under any employee benefit plan (other than the Plan) sponsored by the Company or any Affiliate except as such plan otherwise expressly provides. The Company expressly reserves its rights to amend, modify, or terminate any or all of the employee benefit plans of the Company or any Affiliate.

19. CHOICE OF LAW. The interpretation, performance and enforcement of this Agreement shall be governed by the law of the State of Delaware without regard to that state's conflicts of laws rules.

20. SEVERABILITY. If all or any part of this Agreement or the Plan is declared by any court or governmental authority to be unlawful or invalid, such unlawfulness or invalidity shall not invalidate any portion of this Agreement or the Plan not declared to be unlawful or invalid. Any Section of this Agreement (or part of such a Section) so declared to be unlawful or invalid shall, if possible, be construed in a manner which will give effect to the terms of such Section or part of a Section to the fullest extent possible while remaining lawful and valid.

21. OTHER DOCUMENTS. You hereby acknowledge receipt or the right to receive a document providing the information required by Rule 428(b)(1) promulgated under the Securities Act. In addition, you acknowledge receipt of the Company's *Insider Trading and Trading Window Policy*.

22. AMENDMENT. This Agreement may not be modified, amended or terminated except by an instrument in writing, signed by you and by a duly authorized representative of the Company. Notwithstanding the foregoing, this Agreement may be amended solely by the Board by a writing which specifically states that it is amending this Agreement, so long as a copy of such amendment is delivered to you, and provided that, except as otherwise expressly provided in the Plan, no such amendment materially adversely affecting your rights hereunder may be made without your written consent. Without limiting the foregoing, the Board reserves the right to change, by written notice to you, the provisions of this Agreement in any way it may deem necessary or advisable to carry out the purpose of the Award as a result of any change in applicable laws or regulations or any future law, regulation, ruling, or judicial decision, provided that any such change shall be applicable only to rights relating to that portion of the Award which is then subject to restrictions as provided herein.

23. COMPLIANCE WITH SECTION 409A OF THE CODE. This Award is intended to comply with the "short-term deferral" rule set forth in Treasury Regulation Section 1.409A-1(b)(4). Notwithstanding the foregoing, if it is determined that the Award fails to satisfy the requirements of the short-term deferral rule and is otherwise deferred compensation subject to Section 409A, and if you are a "Specified Employee" (within the meaning set forth in Section 409A(a)(2)(B)(i) of the Code) as of the date of your "separation from service" (within the meaning of Treasury Regulation Section 1.409A-1(h) and without regard to any alternative definition thereunder), then the issuance of any shares that would otherwise be made upon the date of the separation from service or within the first six (6) months thereafter will not be made on the originally scheduled date(s) and will instead be issued in a lump sum on the date that is six (6) months and one day after the date of the separation from service, with the balance of the shares issued thereafter in accordance with the original vesting and issuance schedule set forth above, but if and only if such delay in the issuance of the shares is necessary to avoid the imposition of adverse taxation on you in respect of the shares under Section 409A of the Code. Each installment of shares that vests is intended to constitute a "separate payment" for purposes of Treasury Regulation
Section 1.409A-2(b)(2).

24. Employment Inducement Award. This Award is intended to constitute an "employment inducement award" under NASDAQ Rule 5635(c)(4) that is exempted from the requirements of shareholder approval of equity compensation plans under NASDAQ Rule 5635(c)(4). This agreement and the terms and conditions of this Award will be interpreted consistent with such intent.

* * * * *

This Restricted Stock Unit Award Agreement shall be deemed to be signed by the Company and the Participant upon the signing by the Participant of the Restricted Stock Unit Grant Notice to which it is attached.

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the Registration Statements (No. 333-234235, No. 333-252078, No. 333-252903, No. 333-257433, No. 333-257661 and No. 333-250945) on Form S-3 and Registration Statements (No. 333-203703, No. 333-210369, No. 333-216858, No. 333-224062, No. 333-230041, No. 333-236794, No. 333-252743 and No. 333-257429) on Form S-8 of Zevra Therapeutics, Inc. (f/k/a KemPharm, Inc.) of our report dated March 30, 2022 relating to the financial statements of KemPharm, Inc. for the year ended December 31, 2021, appearing in this Annual Report on Form 10-K of Zevra Therapeutics, Inc. for the year ended December 31, 2022.

/s/ RSM US LLP

Orlando, Florida
March 7, 2023

Exhibit 23.2

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the following Registration Statements:

1. Registration Statements (Form S-3 Nos. 333-234235, 333-252078, 333-252903, 333-257433, 333-257661 and 333-250945) of Zevra Therapeutics, Inc.,

2. Registration Statement (Form S-8 No. 333-203703) pertaining to the Incentive Stock Plan, as amended, and the 2014 Equity Incentive Plan of Zevra Therapeutics, Inc.,

3. Registration Statements (Form S-8 Nos. 333-210369, 333-216858, 333-224062, 333-230041, 333-236794 and 333-252743) pertaining to the 2014 Equity Incentive Plan of Zevra Therapeutics, Inc., and

4. Registration Statement (Form S-8 No. 333-257429) pertaining to the Amended and Restated 2014 Equity Incentive Plan, and the 2021 Employee Stock Purchase Plan of Zevra Therapeutics, Inc.;

of our report dated March 7, 2023, with respect to the financial statements of Zevra Therapeutics, Inc. included in this Annual Report (Form 10-K) of Zevra Therapeutics, Inc. for the year ended December 31, 2022.

/s/ Ernst & Young LLP

Orlando, Florida

March 7, 2023

Exhibit 31.1

CERTIFICATIONS

I, Richard W. Pascoe, certify that:

1. I have reviewed this Annual Report on Form 10-K of Zevra Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

March 7, 2023

/s/ Richard W. Pascoe

Name: Richard W. Pascoe
Title: Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, R. LaDuane Clifton, certify that:

1. I have reviewed this Annual Report on Form 10-K of Zevra Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

March 7, 2023

/s/ R. LaDuane Clifton

Name: R. LaDuane Clifton, CPA
Title: Chief Financial Officer, Secretary and Treasurer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION OF THE PRINCIPAL EXECUTIVE OFFICER
PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report on Form 10-K of Zevra Therapeutics, Inc., (the "Company") for the fiscal year ended December 31, 2022, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Richard W. Pascoe, Principal Executive Officer of the Company, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge:

1. The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
2. The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

March 7, 2023

/s/ Richard W. Pascoe

Name: Richard W. Pascoe
Title: Chief Executive Officer
(Principal Executive Officer)

The foregoing certification is being furnished solely pursuant to 18 U.S.C. Section 1350, is not being "filed" by the Company as part of the Report or as a separate disclosure document and is not to be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Report), irrespective of any general incorporation language contained in such filing.

Exhibit 32.2

CERTIFICATION OF THE PRINCIPAL FINANCIAL OFFICER
PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report on Form 10-K of Zevra Therapeutics, Inc., (the "Company") for the fiscal year ended December 31, 2022, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, R. LaDuane Clifton, Principal Financial Officer of the Company, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge:

1. The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
2. The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

March 7, 2023

/s/ R. LaDuane Clifton

Name: R. LaDuane Clifton, CPA
Title: Chief Financial Officer, Secretary and Treasurer
(Principal Financial Officer)

The foregoing certification is being furnished solely pursuant to 18 U.S.C. Section 1350, is not being "filed" by the Company as part of the Report or as a separate disclosure document and is not to be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Report), irrespective of any general incorporation language contained in such filing.