

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

FORM 10-Q

(Mark One)

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

For the quarterly period ended March 31, 2023

OR

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
(State or Other Jurisdiction of Incorporation or Organization)

20-5894398
(I.R.S. Employer Identification No.)

1180 Celebration Boulevard, Suite 103, Celebration, FL
(Address of Principal Executive Offices)

34747
(Zip Code)

(321) 939-3416
(Registrant's Telephone Number, Including Area Code)

(Former Name, Former Address, and Former Fiscal Year if Changed Since Last Report)

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 per share	ZVRA	The Nasdaq Stock Market LLC (Nasdaq Global Select Market)

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 is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes No

As of May 8, 2023, the registrant had 33,881,804 shares of common stock outstanding.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q, 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 Part II, Item 1A. “Risk Factors” and elsewhere in this Quarterly Report on Form 10-Q and Part I, Item 1A. “Risk Factors” of our Annual Report on Form 10-K for the fiscal year ended December 31, 2022, filed with the SEC on March 7, 2023. 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;
- senior leadership and board member transitions and refreshments; 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 or made reference to important factors in the cautionary statements included in this report, particularly in the section entitled “Risk Factors” where we make reference to Part I, Item 1A. “Risk Factors” of our Annual Report on Form 10-K for the fiscal year ended December 31, 2022, filed with the SEC on March 7, 2023, 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.

NOTE REGARDING COMPANY REFERENCE

Unless the context otherwise requires, we use the terms “Zevra,” “Company,” “we,” “us” and “our” in this Quarterly Report on Form 10-Q 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 Quarterly Report on Form 10-Q that are important to our business, including LAT® and the Zevra logo. All other trademarks, trade names and service marks appearing in this Quarterly Report on Form 10-Q are the property of their respective owners. Solely for convenience, the trademarks and trade names in this Quarterly Report on Form 10-Q 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.

PART I — FINANCIAL INFORMATION

ITEM 1. UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

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

	March 31, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	\$ 40,181	\$ 65,466
Securities at fair value	34,403	16,900
Short-term investments - other	20,700	481
Accounts and other receivables	7,822	8,299
Prepaid expenses and other current assets	1,174	1,877
Total current assets	104,280	93,023
Inventories	620	671
Property and equipment, net	744	794
Operating lease right-of-use assets	898	988
Long-term investments - other	—	20,000
Other long-term assets	53	53
Total assets	\$ 106,595	\$ 115,529
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	\$ 10,098	\$ 6,169
Current portion of operating lease liabilities	470	480
Current portion of discount and rebate liabilities	4,746	4,655
Other current liabilities	302	422
Total current liabilities	15,616	11,726
Line of credit payable	12,914	12,800
Derivative and warrant liability	3	1
Operating lease liabilities, less current portion	736	843
Discount and rebate liabilities, less current portion	5,764	4,327
Other long-term liabilities	158	25
Total liabilities	35,191	29,722
Commitments and contingencies (Note D)		
Stockholders' equity:		
Preferred stock:		
Undesignated preferred stock, \$0.0001 par value, 10,000,000 shares authorized, no shares issued or outstanding as of March 31, 2023 or December 31, 2022	—	—
Common stock, \$0.0001 par value, 250,000,000 shares authorized, 35,457,496 shares issued and 33,881,804 shares outstanding as of March 31, 2023; 35,450,257 shares issued and 34,540,304 shares outstanding as of December 31, 2022	3	3
Additional paid-in capital	402,786	401,799
Treasury stock, at cost	(10,983)	(7,536)
Accumulated deficit	(320,339)	(308,572)
Accumulated other comprehensive (loss) income	(63)	113
Total stockholders' equity	71,404	85,807
Total liabilities and stockholders' equity	\$ 106,595	\$ 115,529

See accompanying notes to unaudited condensed consolidated financial statements.

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

	Three months ended March 31,	
	2023	2022
Revenue, net	\$ 2,879	\$ 3,965
Operating expenses:		
Cost of revenue	125	8
Research and development	8,844	3,082
Selling, general and administrative	6,834	2,734
Total operating expenses	<u>15,803</u>	<u>5,824</u>
Loss from operations	<u>(12,924)</u>	<u>(1,859)</u>
Other (expense) income:		
Interest expense	(182)	(5)
Fair value adjustment related to derivative and warrant liability	(2)	241
Fair value adjustment related to investments	196	(352)
Interest and other income, net	1,042	107
Total other (expense) income	<u>1,054</u>	<u>(9)</u>
Loss before income taxes	<u>(11,870)</u>	<u>(1,868)</u>
Income tax benefit	103	4
Net loss	<u>\$ (11,767)</u>	<u>\$ (1,864)</u>
Net loss attributable to common stockholders	<u>\$ (11,767)</u>	<u>\$ (1,864)</u>
Basic and diluted net loss per share of common stock:		
Net loss attributable to common stockholders	<u>\$ (0.34)</u>	<u>\$ (0.05)</u>
Weighted average number of shares of common stock outstanding:		
Basic and diluted	<u>34,466,542</u>	<u>34,506,597</u>

See accompanying notes to unaudited condensed consolidated financial statements.

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

	Three months ended March 31,	
	2023	2022
Net loss attributable to common stockholders	\$ (11,767)	\$ (1,864)
Other comprehensive (loss) income:		
Foreign currency translation adjustment	(176)	—
Other comprehensive (loss) income	(176)	—
Comprehensive loss	<u>\$ (11,943)</u>	<u>\$ (1,864)</u>

See accompanying notes to unaudited condensed consolidated financial statements.

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

	Common Stock	Additional Paid-in Capital	Treasury Stock, at cost	Accumulated Deficit	Other Comprehensive Income (Loss)	Total Stockholders' Equity
Balance as of January 1, 2023	\$ 3	\$ 401,799	\$ (7,536)	\$ (308,572)	\$ 113	\$ 85,807
Net loss	—	—	—	(11,767)	—	(11,767)
Stock-based compensation expense	—	591	—	—	—	591
Shares repurchased as part of the Share Repurchase Program	—	—	(3,447)	—	—	(3,447)
Issuance of common stock in exchange for consulting services	—	42	—	—	—	42
Severance expense	—	354	—	—	—	354
Other comprehensive loss	—	—	—	—	(176)	(176)
Balance as of March 31, 2023	<u>\$ 3</u>	<u>\$ 402,786</u>	<u>\$ (10,983)</u>	<u>\$ (320,339)</u>	<u>\$ (63)</u>	<u>\$ 71,404</u>

See accompanying notes to unaudited condensed consolidated financial statements.

ZEVRA THERAPEUTICS, INC.
UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY, CONTINUED
(in thousands)

	Common Stock	Additional Paid-in Capital	Treasury Stock, at cost	Accumulated Deficit	Total Stockholders' Equity
Balance as of January 1, 2022	\$ 4	\$ 396,957	\$ (2,814)	\$ (267,029)	\$ 127,118
Net loss	—	—	—	(1,864)	(1,864)
Stock-based compensation expense	—	918	—	—	918
Shares repurchased as part of the Share Repurchase Program	(1)	—	(4,722)	—	(4,723)
Issuance of common stock in exchange for consulting services	—	50	—	—	50
Balance as of March 31, 2022	<u>\$ 3</u>	<u>\$ 397,925</u>	<u>\$ (7,536)</u>	<u>\$ (268,893)</u>	<u>\$ 121,499</u>

See accompanying notes to unaudited condensed consolidated financial statements.

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

	Three months ended March 31,	
	2023	2022
Cash flows from operating activities:		
Net loss	\$ (11,767)	\$ (1,864)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	591	918
Non-cash severance expense	354	—
Depreciation and amortization expense	79	65
Fair value adjustment related to derivative and warrant liability	2	(241)
Fair value adjustment related to investments	(196)	352
Consulting fees paid in common stock	42	50
Gain on foreign currency exchange	(240)	—
Change in assets and liabilities:		
Accounts and other receivables	477	(1,792)
Prepaid expenses and other assets	703	303
Inventories	51	—
Operating lease right-of-use assets	80	51
Accounts payable and accrued expenses	3,929	(486)
Discount and rebate liability	1,528	—
Operating lease liabilities	(107)	(88)
Other liabilities	429	(821)
Net cash used in operating activities	<u>(4,045)</u>	<u>(3,553)</u>
Cash flows from investing activities:		
Purchases of property and equipment	(29)	(16)
Purchases of investments	(17,526)	(3,832)
Net cash used in investing activities	<u>(17,555)</u>	<u>(3,848)</u>
Cash flows from financing activities:		
Proceeds from issuance of debt	12,914	—
Repayment of debt	(12,800)	—
Payments of principal on insurance financing arrangements	(415)	—
Payment to repurchase shares as part of the share repurchase program	(3,447)	(4,723)
Repayment of principal on finance lease liabilities	(2)	(10)
Net cash used in financing activities	<u>(3,750)</u>	<u>(4,733)</u>
Effect of exchange rate changes on cash and cash equivalents	65	—
Net decrease in cash and cash equivalents	(25,285)	(12,134)
Cash and cash equivalents, beginning of period	65,466	112,346
Cash and cash equivalents, end of period	<u>\$ 40,181</u>	<u>\$ 100,212</u>
Supplemental cash flow information:		
Cash paid for interest	\$ 68	\$ 5

See accompanying notes to unaudited condensed consolidated financial statements.

ZEVRA THERAPEUTICS, INC.
NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

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

Organization

Zevra Therapeutics, Inc. (the "Company") is a rare disease company melding science, data and patient need to create transformational therapies for diseases with limited or no treatment options. The Company has 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 ("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 ("FDA") and orphan medical product designation for the treatment of NPC by the European Medicines Agency ("EMA"). KP1077 is the Company's lead clinical development product candidate which is being developed as a treatment for idiopathic hypersomnia ("IH"), a rare neurological sleep disorder, and narcolepsy. KP1077 is comprised solely of serdexmethylphenidate ("SDX"), the Company's proprietary prodrug of d-methylphenidate ("d-MPH"). The FDA has granted KP1077 orphan drug designation for the treatment of IH. The Company changed its name from KemPharm, Inc. to Zevra Therapeutics, Inc. effective as of February 21, 2023.

The accompanying unaudited condensed consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America ("U.S. GAAP") for interim financial information and with the instructions to Form 10-Q and Rule 8-03 of Regulation S-X. Accordingly, they do not include all of the information and related notes required by U.S. GAAP for complete financial statements. In the opinion of management, all adjustments (consisting of normal recurring adjustments) considered necessary for a fair presentation have been included in the accompanying consolidated financial statements. Operating results for the three months ended March 31, 2023, are not necessarily indicative of the results that may be expected for the full year ending December 31, 2023.

This interim information should be read in conjunction with the audited financial statements included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2022, filed with the United States Securities and Exchange Commission ("SEC") on March 7, 2023.

Basis of Presentation

The Company prepared the consolidated financial statements in accordance with U.S. GAAP and the rules and regulations of the SEC and, in the Company's opinion, reflect all adjustments, including normal recurring items that are necessary.

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 the Company's 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.

Amendment to Registration Statement on Form S-3

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 the Company's common stock issuable upon the exercise of the warrants issued in the Company's January 2021 underwritten public offering (the "Public Offering") and remaining unexercised as of the date of the amendment, which was declared effective on February 1, 2022.

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 March 31, 2023, 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 the Company's 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 March 31, 2023, the Company had repurchased 1,575,692 shares of its common stock for approximately \$11.0 million under the Share Repurchase Program.

Reclassifications

Certain reclassifications were made to the 2022 unaudited condensed consolidated financial statements to conform to the classifications used in 2023. 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 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 investments and 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.

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 unaudited condensed 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 unaudited condensed consolidated balance sheets. As of March 31, 2023, and December 31, 2022, the Company held securities with an aggregate fair value of \$34.4 million and \$16.9 million, respectively, that contained aggregate unrealized losses of approximately \$0.4 million and \$0.6 million, 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 operations. In addition, the Company holds certificates of deposit totaling \$20.7 million as of March 31, 2023, and December 31, 2022, and are included in short-term investments - other in the unaudited condensed 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 unaudited condensed consolidated statements of operations.

Revenue Recognition

The Company recognizes revenue in accordance with the provisions of 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 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 the 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 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 three months ended March 31, 2023, the Company recognized revenue related to the Arimoclomol EAP in France of \$2.0 million, which is net of a clawback liability of \$1.3 million and other gross to net adjustments. 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 March 31, 2023, including the additional clawback liability for the three months ended March 31, 2023, was \$10.5 million. The total reserve liability as of December 31, 2022, was \$9.0 million. As of March 31, 2023, and December 31, 2022, this estimated reserve liability is recorded as discount and rebate liabilities in the unaudited condensed consolidated balance sheets 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 Gurnet Point Capital ("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-MPH, including AZSTARYS, or any other product candidates containing SDX and developed to treat ADHD or any other central nervous system ("CNS") disease. Corium, Inc. ("Corium") was tasked by Commave, to lead all commercialization activities for AZSTARYS under the AZSTARYS License Agreement. Pursuant to the AZSTARYS License Agreement, Commave 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. The AZSTARYS Amendment increased the total remaining future regulatory and sales milestone payments related to AZSTARYS to 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 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 percentage 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 three months ended March 31, 2023 and 2022, the Company recognized \$0.6 million and \$0.1 million of revenue under the AZSTARYS License Agreement, respectively. There was no deferred revenue related to this agreement as of March 31, 2023, or December 31, 2022.

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 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 was 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 was recognized as the consulting services were performed and the services associated with the reimbursable third-party pass-through costs were 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.

For the three months ended March 31, 2023, and 2022, the Company recognized \$0.2 million and \$3.5 million of revenue, respectively, under the Corium Consulting Agreement. As of March 31, 2023, and December 31, 2022, the Company had no deferred revenue related to this agreement. The Corium Consulting Agreement expired on March 31, 2023.

Foreign currency

Assets and liabilities are translated into the reporting currency using the exchange rates in effect on the unaudited consolidated condensed 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 unaudited condensed consolidated statements of stockholders’ equity.

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. As of March 31, 2023, the Company had receivables related to the Arimoclomol EAP of \$5.3 million, AZSTARYS License Agreement of \$0.6 million, consulting arrangements of \$0.2 million, income tax receivables of \$1.0 million, and other receivables of \$0.7 million. As of December 31, 2022, the Company had receivables related to the Arimoclomol EAP of \$6.3 million, Corium Consulting Agreement of \$0.2 million, AZSTARYS License Agreement of \$0.5 million, income tax receivables of \$0.9 million and other receivables of \$0.4 million. As of March 31, 2023, and December 31, 2022, no reserve or allowance for doubtful accounts had been established.

C. Debt Obligations

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 was May 31, 2025. The Line of Credit contained customary events of default that could have led to an acceleration of the loans, including cross-default, bankruptcy and payment defaults. As of December 31, 2022, the Company had drawn \$12.8 million from the Line of Credit to finance the transactions under the Arimoclomol Purchase Agreement, and this amount was supported by a \$12.8 million certificate of deposit which was shown as long-term investments - others in the unaudited condensed consolidated balance sheet as of December 31, 2022. The remaining \$7.2 million under the Line of Credit was in a separate interest-bearing certificate of deposit and is also recorded as long-term investments - other in the unaudited condensed consolidated balance sheets as of December 31, 2022. These certificates of deposit are pledged as collateral against the Line of Credit and could not be redeemed so long as the \$20.0 million remained 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. On January 31, 2023, the Company repaid the \$12.8 million outstanding under the Line of Credit in full and closed the Line of Credit. In conjunction with closing the Line of Credit, the maturity dates of the certificates of deposit were modified to May 2023. As such, the certificates of deposit are classified as short-term investments - other in the unaudited condensed consolidated balance sheet as of March 31, 2023. No amounts are outstanding under the Line of Credit as of March 31, 2023.

On January 26, 2023, the Company and Wells Fargo, as lender, entered into a revolving margin account agreement under which the Company initially borrowed \$12.8 million. The Company's investments are used as collateral for the loan and the amount the Company is able to borrow is limited to 80-90% of its outstanding investment balance held with Wells Fargo. The margin account bears interest at the Prime rate minus 225 basis-points. As of March 31, 2023 \$12.9 million was outstanding under the margin account.

D. Commitments and Contingencies

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.

During the first quarter of 2023, the Company and KVK-Tech, Inc. ("KVK") began discussing a possible termination of the Collaboration and License Agreement (the "Agreement") that the parties entered into on October 25, 2018. Under the terms of the Agreement, each party has grounds to terminate the Agreement. While negotiations are on-going, in April 2023, the Company determined that it is probable that the Agreement will be terminated and accrued an estimated settlement to KVK of \$0.9 million, which is included in research and development in the unaudited condensed consolidated statement of operations for the three months ended March 31, 2023. As of December 31, 2022, no accruals have been made related to commitments and contingencies.

E. Stock and Warrants***Authorized, Issued, and Outstanding Common Shares***

As of March 31, 2023, and December 31, 2022, the Company had authorized shares of common stock of 250,000,000 shares. Of the authorized shares, 35,457,496 and 35,450,257 shares of common stock were issued as of March 31, 2023, and December 31, 2022, respectively, and 33,881,804 and 34,540,304 respectively shares of common stock were outstanding as of March 31, 2023, and December 31, 2022, respectively.

As of March 31, 2023 and December 31, 2022, the Company had reserved authorized shares of common stock for future issuance as follows:

	March 31, 2023	December 31, 2022
Outstanding awards under equity incentive plans	6,024,889	2,456,407
Outstanding common stock warrants	4,252,490	4,252,600
Possible future issuances under equity incentive plans	3,731,263	4,421,508
Possible future issuances under employee stock purchase plans	1,417,365	1,417,365
Total common shares reserved for future issuance	15,426,007	12,547,880

Common Stock Activity

The following table summarizes common stock activity for the three months ended March 31, 2023:

	Shares of Common Stock
Balance as of January 1, 2023	34,540,304
Common stock issued as compensation to third-parties	7,129
Common stock repurchased as a result of the Stock Repurchase Plan	(665,739)
Common stock issued as a result of stock warrants exercised	110
Balance as of March 31, 2023	33,881,804

Authorized, Issued, and Outstanding Preferred Stock

As of March 31, 2023, and December 31, 2022, the Company had 10,000,000 shares of authorized preferred stock, none of which were designated, issued, or outstanding.

Warrants

Warrants to Purchase Common Stock

In June 2014, the Company entered into a multi-tranche credit facility (the "Deerfield Facility Agreement") with Deerfield Private Design Fund III, LP ("Deerfield"). The Company issued to Deerfield a warrant to purchase 120,192 shares of the Company's common stock, which is exercisable at \$38.34 per share until June 2, 2024, and Deerfield assigned this warrant to OTA LLC in December of 2022 (the "OTA Warrant"). 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 H). 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 determined that the OTA Warrant should be recorded as a liability and stated at fair value at each reporting period. Changes to the fair value of the warrant liability are recorded through the unaudited condensed statements of operations as a fair value adjustment (Note G).

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 \$36.80 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. 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. 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 unaudited condensed consolidated statements of operations.

On January 12, 2021, the Company issued warrants to purchase 12,078,361 shares of the Company's common stock (collectively, the "Offering Warrants") in the Public Offering. 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 meet the equity classification requirements and thus are recorded in additional paid-in capital on the unaudited condensed consolidated balance sheets. As of March 31, 2023, 3,461,968 Offering Warrants have been exercised for 3,030,991 shares of common stock and gross proceeds of approximately \$16.9 million.

On January 12, 2021, the Company issued a warrant to Roth Capital Partners ("Roth") to purchase 806,932 shares of the Company's common stock and the exercise price is \$8.125 per share. In connection with the closing of the Roth's partial exercise of its over-allotment option, on February 3, 2021, Roth was issued an additional warrant to purchase 18,702 shares of common stock. As of March 31, 2023, 400,000 of these 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 (the "January 2021 Inducement Warrants") to purchase up to 7,944,430 shares of the Company's common stock (the "January 2021 Inducement Warrants"). The January 2021 Inducement 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 \$6.36 per share. As of March 31, 2023, 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.

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") 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 June 2021 Inducement Warrants were immediately exercisable and expire on December 31, 2026, at an exercise price per share of common stock equal to \$16.50 per share. As of March 31, 2023, no June 2021 Inducement Warrants have been exercised.

F. 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 8,271,497 as of March 31, 2023. 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 2014 Plan, on January 1, 2023, the common stock reserved for issuance under the 2014 Plan automatically increased by 1,381,612 shares.

During the three months ended March 31, 2023, and 2022, 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 March 31, 2023, 82,635 shares have been issued under the ESPP.

In January 2023, the Board approved the 2023 Employment Inducement Award Plan (the "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 unaudited condensed consolidated statements of operations (in thousands):

	Three months ended March 31,	
	2023	2022
Research and development	\$ 572	\$ 311
Selling, general and administrative	19	607
Total stock-based compensation expense	<u>\$ 591</u>	<u>\$ 918</u>

There was no stock-based compensation expense related to performance-based awards recognized during the three months ended March 31, 2023, or 2022.

As a result of the modification of certain stock options in connection with the Transition Agreement, as further discussed in Note J, the Company recaptured \$0.9 million in stock-compensation expense and recognized \$0.4 million in severance expense for the three months ended March 31, 2023.

G. 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, investments and accounts payable and accrued expenses, approximate their 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 March 31, 2023, and December 31, 2022 (in thousands):

	Balance as of March 31, 2023	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
OTA Warrant liability	\$ 3	\$ —	\$ —	\$ 3
Total liabilities	\$ 3	\$ —	\$ —	\$ 3

Securities:

U.S. government-sponsored agency securities	\$ 7,290	\$ —	\$ 7,290	\$ —
U.S. Treasury securities	27,113	27,113	—	—
Total assets	\$ 34,403	\$ 27,113	\$ 7,290	\$ —

	Balance as of 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	\$ —

The Company's OTA Warrant liability, embedded Warrant Put Option and securities are measured at fair value on a recurring basis. As of March 31, 2023, and December 31, 2022, the OTA Warrant liability and the embedded Warrant Put Option are reported on the unaudited condensed consolidated balance sheets in derivative and warrant liability. As of March 31, 2023, and December 31, 2022, the securities are reported on the unaudited condensed consolidated balance sheets in securities at fair value. 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 unaudited condensed consolidated statements of operations for the three months ended March 31, 2023, and 2022, as a fair value adjustment related to derivative and warrant liability.

The derivative liability for the OTA Warrant was \$3,000 and \$1,000 at March 31, 2023, and December 31, 2022, respectively. The derivative liability for the embedded Warrant Put Option was \$0 at March 31, 2023, and December 31, 2022. 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 48% with an estimated probability-weighted value of approximately \$38.5 million and a weighted-average probability of a fundamental change event occurring of approximately 30% with an estimated probability-weighted value of approximately \$463.8 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 March 31, 2023, and December 31, 2022, the KVK Warrant liability is reported on the unaudited condensed 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 unaudited condensed consolidated statements of operations for the three months ended March 31, 2023, and 2022, 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 significant unobservable inputs (Level 3) is as follows (in thousands):

	Three months ended March 31,	
	2023	2022
Balance as of beginning of period	\$ 1	\$ 306
Adjustment to fair value	2	(305)
Balance as of end of period	\$ 3	\$ 1

H. 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 shares of common stockholders by the weighted average number of shares of common stock outstanding during the period. Undistributed net income attributable to shares of 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, warrants and other outstanding convertible securities. 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 and other outstanding convertible securities under the if-converted method when calculating diluted income (loss) attributable to common stockholders per share of common stock, in which it is assumed that the stock options, warrants and other outstanding convertible securities convert into common stock at the beginning of the period or date of issuance, if the stock option, warrant or other outstanding convertible security 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 periods 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:

	Three months ended March 31,	
	2023	2022
Awards under equity incentive plans	6,024,889	2,186,769
Common stock warrants	4,252,490	4,252,600
Total securities excluded from the calculation of weighted average number of shares of common stock outstanding	<u>10,277,379</u>	<u>6,439,369</u>

A reconciliation from net loss to basic and diluted net loss attributable to common stockholders per share of common stock for the three months ended March 31, 2023, and 2022, is as follows (in thousands):

	Three months ended March 31,	
	2023	2022
Net loss per share of common stock:		
Net loss	\$ (11,767)	\$ (1,864)
Less: Dividends declared or accumulated	—	—
Undistributed net loss attributable to shares of common stockholders, basic and diluted	\$ (11,767)	\$ (1,864)
Weighted average number of shares of common stock outstanding, basic and diluted	34,467	34,507
Basic and diluted net loss attributable to common stockholders per share of common stock	<u>\$ (0.34)</u>	<u>\$ (0.05)</u>

I. 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 unaudited condensed 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 3 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):

Lease Cost	Three months ended March 31,	
	2023	2022
Finance lease cost:		
Amortization of right-of-use assets	\$ 32	\$ 32
Interest on lease liabilities	—	1
Total finance lease cost	32	33
Operating lease cost	113	91
Short-term lease cost	55	50
Variable lease cost	13	13
Less: sublease income	(39)	(39)
Total lease costs	\$ 174	\$ 148

Supplemental cash flow information related to leases was as follows (in thousands):

	Three months ended March 31,	
	2023	2022
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows from finance leases	\$ —	\$ 1
Financing cash flows from finance leases	2	10
Operating cash flows from operating leases	142	117
Operating cash flows from short-term leases	55	50
Operating cash flows from variable lease costs	13	13
Right-of-use assets obtained in exchange for lease liabilities:		
Finance leases	\$ —	\$ —
Operating leases	—	—

Supplemental balance sheet information related to leases was as follows (in thousands, except weighted average remaining lease term and weighted average discount rate):

	March 31, 2023	December 31, 2022
Finance Leases		
Property and equipment, at cost	\$ 1,031	\$ 1,031
less: accumulated depreciation and amortization	(812)	(780)
Property and equipment, net	<u>\$ 219</u>	<u>\$ 251</u>
Other current liabilities	\$ 5	\$ 6
Other long-term liabilities	—	—
Total finance lease liabilities	<u>\$ 5</u>	<u>\$ 6</u>
Operating Leases		
Operating lease right-of-use assets	\$ 898	\$ 988
Total operating lease right-of-use assets	<u>\$ 898</u>	<u>\$ 988</u>
Current portion of operating lease liabilities	\$ 470	\$ 480
Operating lease liabilities, less current portion	736	843
Total operating lease liabilities	<u>\$ 1,206</u>	<u>\$ 1,323</u>
Weighted Average Remaining Lease Term		
Finance leases	1 year	1 year
Operating leases	3 years	3 years
Weighted Average Discount Rate		
Finance leases	14.3%	14.3%
Operating leases	7.4%	7.3%

Maturities of lease liabilities were as follows (in thousands):

Year Ending December 31,	Finance Leases	Operating Leases
2023 (excluding the three months ended March 31, 2023)	\$ 5	\$ 419
2024	—	488
2025	—	390
2026	—	30
2027	—	—
Total lease payments	5	1,327
Less: future interest expense	0	(121)
Lease liabilities	<u>\$ 5</u>	<u>\$ 1,206</u>

J. 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 continued to serve as a member of the Board until April 25, 2023. Mr. Pascoe was 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 from 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, which was held on April 25, 2023. Additionally, on January 6, 2023, the Board 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, which was held on April 25, 2023. 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. The severance benefits consisted of personnel and other related charges of approximately \$1.0 million and stock compensation expense of approximately \$0.4 million related to the acceleration of vesting on unvested shares subject to certain stock options and the extension of the exercise period for certain stock options. These severance benefits are presented in selling, general and administrative expenses in the unaudited condensed consolidated statement of operations for the three months ended March 31, 2023. As of March 31, 2023, the Company had accrued severance expense recorded within accounts payable and accrued expenses of \$1.0 million.

At the Company's 2023 Annual Meeting of Stockholders (the "Annual Meeting"), which was held on April 25, 2023, each of John B. Bode, Douglas W. Calder, and Corey Watton was elected as a director of the Company and each of Richard W. Pascoe, Christopher A. Posner, and David S. Tierney ceased serving on the Company's Board of Directors. After the Annual Meeting, the Company's Board of Directors accepted the resignation of Richard W. Pascoe from his role as Chief Executive Officer on May 5, 2023, effective June 1, 2023, and appointed Tamara A. Favorito as the Chair of the Board of Directors. In addition, on May 3, 2023, Matthew R. Plooster and Joseph B. Saluri indicated to the Board of Directors that they do not intend to stand for re-election at the Company's 2024 Annual Meeting of Stockholders, and that they intend to step down from the Board of Directors as soon as replacements are found. In May 2023, the Board of Directors appointed Christal M. M. Mickle, Co-Founder and Chief Development Officer, to serve as interim President and Chief Executive Officer effective on June 1, 2023. The Board also intends to begin a search to identify a new Chief Executive Officer, as well as new Board members to replace Mr. Plooster and Mr. Saluri.

K. Subsequent Events

The Company evaluated events and transactions occurring subsequent to March 31, 2023, through May 15, 2023, the date the accompanying unaudited condensed consolidated financial statements were issued. During this period, other than the contingency discussed in Note D and the significant events disclosed in Note J, there were no subsequent events that required recognition in the accompanying unaudited condensed consolidated financial statements, nor were there any additional non-recognized subsequent events that required disclosure.

ITEM 2. 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 unaudited condensed consolidated financial statements and related notes thereto included elsewhere in this Quarterly Report on Form 10-Q. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report on Form 10-Q, 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 Part II, Item 1A. "Risk Factors" of this Quarterly Report on Form 10-Q and Part I, Item 1A. "Risk Factors" of our Annual Report on Form 10-K for the fiscal year ended December 31, 2022, filed with the SEC on March 7, 2023, 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®, 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 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 WorldSymposium™ 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. In May 2023, we announced that our Investigational New Drug (IND) application for KP1077 for the treatment of narcolepsy was accepted by the FDA to begin a Phase 1 clinical trial of KP1077 in narcolepsy. We plan to initiate the first of several Phase 1 clinical trials of KP1077 in narcolepsy as early as the end of 2023. These trials are expected to support both the narcolepsy and IH clinical development programs.

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 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 resubmission as early as Q3 2023
Methylphenidate (ER) - IH	KP1077IH*	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	Phase 1 Trial - initiation prior to year-end
Methylphenidate (ER) - ADHD	AZSTARYS	FDA Approved/Partnered	Tracking 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" of our Annual Report on Form 10-K for the fiscal year ended December 31, 2022, filed with the SEC on March 7, 2023.

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 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.

Results of Operations**Comparison of the three months ended March 31, 2023 and 2022 (in thousands):**

	Three months ended March 31,		Period-to- Period Change
	2023	2022	
Revenue, net	\$ 2,879	\$ 3,965	\$ (1,086)
Operating expenses:			
Cost of revenue	125	8	117
Research and development	8,844	3,082	5,762
Selling, general and administrative	6,834	2,734	4,100
Total operating expenses	15,803	5,824	9,979
Loss from operations	(12,924)	(1,859)	(11,065)
Other (expense) income:			
Interest expense	(182)	(5)	(177)
Fair value adjustment related to derivative and warrant liability	(2)	241	(243)
Fair value adjustment related to investments	196	(352)	548
Interest and other income, net	1,042	107	935
Total other (expense) income	1,054	(9)	1,063
Loss before income taxes	(11,870)	(1,868)	(10,002)
Income tax benefit	103	4	99
Net loss	\$ (11,767)	\$ (1,864)	\$ (9,903)
Net loss attributable to common stockholders	\$ (11,767)	\$ (1,864)	\$ (9,903)

Net Loss Attributable to Common Stockholders

Net loss attributable to common stockholders for the three months ended March 31, 2023, was \$11.8 million compared to net loss attributable to common stockholders of \$1.9 million for the three months ended March 31, 2022, a change of \$9.9 million. The change was primarily attributable to a change in loss from operations of \$11.1 million; partially offset by a change in other (expense) income of \$1.1 million.

Revenue

Revenue for the three months ended March 31, 2023, was \$2.9 million, a decrease of \$1.1 million compared to revenue of \$4.0 million for the three months ended March 31, 2022. The decrease was primarily attributable to a decrease in revenue of \$3.2 million from the Corium Consulting Agreement, partially offset by an increase in revenue of \$2.1 million under the Arimoclomol EAP.

Cost of Revenue

Cost of revenue for the three months ended March 31, 2023, was \$0.1 million, an increase of \$0.1 million compared to \$8,000 cost of revenue for the three months ended March 31, 2022. The increase was primarily attributable to cost of goods sold under the Arimoclomol EAP.

Research and Development

Research and development expenses increased by \$5.7 million, from \$3.1 million for the three months ended March 31, 2022, to \$8.8 million for the three months ended March 31, 2023. This increase was primarily attributable to an increase in third-party research and development costs of \$3.7 million, an increase in personnel-related costs of \$1.7 million and an increase in other research and development costs of \$0.3 million.

Selling, General and Administrative

Selling, general and administrative expenses increased by \$4.1 million, from \$2.7 million for the three months ended March 31, 2022, to \$6.8 million for the three months ended March 31, 2023. This increase was primarily attributable to an increase in personnel-related costs of \$1.2 million, an increase in professional fees of \$2.8 million and an increase in other selling, general and administrative costs of \$0.1 million.

Other (Expense) Income

Other (expense) income changed by \$1.1 million, from \$9,000 of expense for the three months ended March 31, 2022, to \$1.1 million of income for the three months ended March 31, 2023. This period-to-period change was primarily attributable to a change in net interest expense and other items of \$0.8 million and a change in fair value adjustment related to investments of \$0.5 million; partially offset by a change in fair value adjustment related to derivative and warrant liability of \$0.2 million.

Liquidity and Capital Resources

Sources of Liquidity

Through March 31, 2023, 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 March 31, 2023, we had cash, cash equivalents and investments of \$95.3 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.

We have had recurring negative net operating cash flows and we anticipate that we may continue to incur negative net cash flows or minimal positive 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, through the Arimoclomol EAP or through potential consulting arrangements and any other future arrangements related to one of our product candidates.

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 March 31, 2023, 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 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 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. During the three months ended March 31, 2023, we repurchased 665,739 shares of our common stock for approximately \$3.4 million under the Share Repurchase Program, and as of March 31, 2023, we had repurchased an aggregate of 1,575,692 shares of our common stock for approximately \$11.0 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 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 had drawn \$12.8 million from the Line of Credit to finance the transactions under the Arimoclomol Purchase Agreement, and this amount was supported by a \$12.8 million certificate of deposit which was shown as long-term investments - other in the unaudited condensed consolidated balance sheet as of December 31, 2022. The remaining \$7.2 million available under the Line of Credit was secured by a separate interest-bearing certificate of deposit and was also recorded as long-term investments - other in the unaudited condensed consolidated balance sheet as of December 31, 2022. These certificates of deposit were pledged as collateral against the Line of Credit and could not be redeemed so long as the \$20.0 million remained 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. On January 31, 2023, we repaid the \$12.8 million outstanding under the Line of Credit in full, and subsequently closed the Line of Credit during the first quarter of 2023.

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. As of March 31, 2023, \$12.9 million was outstanding under the margin account.

Cash Flows

The following table summarizes our cash flows for the three months ended March 31, 2023 and 2022 (in thousands):

	Three months ended March 31,	
	2023	2022
Net cash used in operating activities	\$ (4,045)	\$ (3,553)
Net cash used in investing activities	(17,555)	(3,848)
Net cash used in financing activities	(3,750)	(4,733)
Effect of exchange rates on cash and cash equivalents	65	—
Net decrease in cash and cash equivalents	<u>\$ (25,285)</u>	<u>\$ (12,134)</u>

Operating Activities

For the three months ended March 31, 2023, net cash used in operating activities of \$4.0 million consisted of a net loss of \$11.8 million, partially offset by \$0.6 million in adjustments for non-cash items and \$7.1 million in changes in working capital. 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, \$0.5 million related to a change in accounts and other receivables, \$1.5 million related to a change in discount and rebate liabilities, \$3.9 million related to a change in accounts payable and accrued expenses, \$0.1 million related to a change in operating lease right-of-use assets, \$0.1 million related to a change in inventories and \$0.4 million related to a change in other liabilities, partially offset by \$0.1 million related to a change in operating lease liabilities. The adjustments for non-cash items primarily consisted of stock-based compensation expense of \$0.6 million, non-cash severance expense of \$0.4 million and \$0.1 million related to depreciation, amortization and other items, partially offset by a change in the fair value adjustment related to investments of \$0.2 million and a gain on foreign currency exchange rates of \$0.2 million.

For the three months ended March 31, 2022, net cash used in operating activities of \$3.6 million consisted of a net loss of \$1.9 million and \$2.8 million in changes in working capital, partially offset by \$1.1 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 and the Corium Consulting Agreement. The changes in working capital consisted of \$1.8 million related to a change in accounts and other receivables, \$0.5 million related to a change in accounts payable and accrued expenses, \$0.1 million related to a change in operating lease liabilities and \$0.8 million related to a change in other liabilities, partially offset by \$0.3 million related to a change in prepaid expense and other assets and \$0.1 million related to a change in operating lease right-of-use assets. The adjustments for non-cash items primarily consisted of stock-based compensation expense of \$0.9 million, a change in the fair value adjustment related to investments of \$0.4 million and \$0.1 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 \$0.2 million.

Investing Activities

For the three months ended March 31, 2023, net cash used in investing activities was \$17.6 million, which was primarily attributable to purchases of investments.

For the three months ended March 31, 2022, net cash used in investing activities was \$3.8 million, which was primarily attributable to purchases of investments.

Financing Activities

For the three months ended March 31, 2023, net cash used in financing activities was \$3.8 million, which was primarily attributable to proceeds from the issuance of debt of \$12.9 million, partially offset by payments of principal on insurance financing arrangements of \$0.4 million, payments to repurchase shares as part of the Share Repurchase Program of \$3.4 million and payments of principal on debt of \$12.8 million.

For the three months ended March 31, 2022, net cash used in financing activities was \$4.7 million, which was primarily attributable to payments to repurchase shares as part of the Share Repurchase Program of \$4.7 million.

Future Funding Requirements

Based on our current operating forecast, we believe that our existing cash, cash equivalents and 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. In addition, we maintain the majority of our cash and cash equivalents in accounts with major U.S. and multi-national financial institutions, and our deposits at these institutions exceed insured limits. Market conditions can impact the viability of these institutions. In the event of a failure of any of the financial institutions where we maintain our cash and cash equivalents, there can be no assurance that we would be able to access uninsured funds in a timely manner or at all. Any inability to access or delay in accessing these funds could adversely affect our business and financial position.

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. 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.

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 unaudited condensed consolidated financial statements, which we have prepared in accordance with accounting principles generally accepted in the United States. The preparation of our unaudited condensed consolidated 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 unaudited condensed consolidated 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.

Our critical accounting policies have not changed materially from those described in *Item 7 - Management's Discussion and Analysis of Financial Condition and Results of Operations* of our Annual Report on Form 10-K for the fiscal year ended December 31, 2022, filed with the SEC on March 7, 2023.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Not applicable.

ITEM 4. 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 March 31, 2023. Based on the evaluation of our disclosure controls and procedures as of March 31, 2023, 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.

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 March 31, 2023, that materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II OTHER INFORMATION

ITEM 1. 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 1A. RISK FACTORS

In addition to the other information set forth in this Quarterly Report on Form 10-Q, you should carefully consider all the risk factors and uncertainties described in Part I, Item 1A. "Risk Factors" of our Annual Report on Form 10-K for the fiscal year ended December 31, 2022, filed with the SEC on March 7, 2023, before investing in our common stock. Except as discussed below, there have been no material changes to the risk factors described in that report. If any of those risks materialize, our business, financial condition and results of operations could be seriously harmed. This Quarterly Report on Form 10-Q 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 those risk factors and the other factors described in this Quarterly Report on Form 10-Q.

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 our senior leadership team, 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. Among other recent changes in our senior management team, our Chief Executive Officer has resigned effective as of June 1, 2023. Our future performance will depend, in part, on a successful transition period with our new Chief Executive Officer, including any interim Chief Executive Officer, the successful integration of these and any other new senior level executives into their roles, and the continuity of leadership among the larger workforce. If we do not successfully manage these transitions, it could be viewed negatively by our customers, employees, investors, and other third-party partners, and could have an adverse impact on our business and results of operations.

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.

We could be negatively affected as a result of the actions of activist stockholders, which could be disruptive and costly and may conflict with or disrupt the strategic direction of our business.

In January 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, which resulted in a contested election at the annual meeting at which such nominees were elected by our stockholders. Similar to the activist stockholder activities initiated in January 2023, activist stockholders may from time to time attempt to effect changes in our strategic direction and seek changes regarding our corporate governance or structure. Our board of directors and management team strive to maintain constructive, ongoing communications with all stockholders who wish to speak with us, including activist stockholders, and welcome their views and opinions with the goal of working together constructively to enhance value for all stockholders. Any future 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) actual or perceived uncertainties as to our future direction caused by activist activities may cause or appear to cause instability or lack of continuity, resulting in the loss of potential business opportunities, and potentially making 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. Activist stockholder activities may also cause significant fluctuations in our stock price based on temporary or speculative market perceptions, or other factors that do not necessarily reflect the fundamental underlying value of our business.

Concentration of Credit Risk

Financial instruments that potentially subject the Company to a concentration of credit risk consist primarily of cash, cash equivalents, and investments. The Company invests in money market funds, U.S. treasury securities, and U.S. government agency securities. The Company maintains bank deposits in federally insured financial institutions and these deposits may exceed federally insured limits. The Company is exposed to credit risk in the event of a default by the financial institutions holding its cash and cash equivalents to the extent recorded on the unaudited condensed consolidated balance sheets. The Company has not experienced any material losses on its deposits of cash, cash equivalents, and investments.

ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS**Recent Sales of Unregistered Securities**

None.

Purchases of Equity Securities By the Issuer and Affiliated Purchasers*Issuer Purchases of Equity Securities*

Period	Total Number of Shares Purchased	Average Price Paid per Share	Total Number of Shares Purchased as Part of Publicly Announced Plans or Programs(1)	Maximum Number (or Approximate Dollar Value) of Shares that May Yet Be Purchased Under the Plans or Programs
January 1, 2023, through January 31, 2023	—	\$ —	—	\$ 42,500,000
February 1, 2023, through February 28, 2023	—	—	—	42,500,000
March 1, 2023, through March 31, 2023	665,739	5.09	1,575,692	39,079,815
Total	665,739	\$ 5.09	1,575,692	\$ 39,079,815

(1) On December 20, 2021, the Company announced that it had 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. 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.

ITEM 3. DEFAULTS UPON SENIOR SECURITIES

Not applicable.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

ITEM 5. OTHER INFORMATION

None.

ITEM 6. EXHIBITS

The following is a list of exhibits filed as part of this Form 10-Q (the SEC file number for all items incorporated by reference herein from reports on Forms 10-K, 10-Q, and 8-K is 001-36913):

Exhibit No.	Description
3.1	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 April 21, 2015).
3.1.1	Certificate of Amendment of Amended and Restated Certificate of Incorporation of the Registrant, 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 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).
10.1	Amendment to Employment Agreement, dated as of January 6, 2023, between Zevra Therapeutics, Inc. and Richard W. Pascoe (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on January 9, 2023).
10.2	Transition Agreement, dated as of January 4, 2023, as amended, between Zevra Therapeutics, Inc. and Travis C. Mickle, Ph.D. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on January 9, 2023).
10.3	Consulting Agreement, dated as of January 6, 2023, between Zevra Therapeutics, Inc. and Travis C. Mickle, Ph.D. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on January 9, 2023).
10.4	2023 Employment Inducement Award Plan and forms of award agreements thereunder (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on March 7, 2023).
10.5	Transition Agreement, dated as of May 6, 2023, between Zevra Therapeutics, Inc. and Richard W. Pascoe (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on May 8, 2023).
10.6	Agreement Regarding Employment Terms, dated as of May 15, 2023, between Zevra Therapeutics, Inc. and Christal M.M. Mickle (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on May 15, 2023).
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.
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 combined in Exhibit 101)

* Filed herewith

** Furnished herewith

SIGNATURES

Pursuant to the requirements 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.

Date: May 15, 2023

By: /s/ Richard W. Pascoe
Richard W. Pascoe
Chief Executive Officer
(Principal Executive Officer)

Date: May 15, 2023

By: /s/ R. LaDuane Clifton
R. LaDuane Clifton, CPA
Chief Financial Officer, Secretary and Treasurer
(Principal Financial Officer)

CERTIFICATION

I, Richard W. Pascoe, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q 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.

May 15, 2023

/s/ Richard W. Pascoe

Name: Richard W. Pascoe

Title: Chief Executive Officer

(Principal Executive Officer)

CERTIFICATION

I, R. LaDuane Clifton, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q 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.

May 15, 2023

/s/ R. LaDuane Clifton

Name: R. LaDuane Clifton, CPA

Title: Chief Financial Officer, Secretary and Treasurer
(Principal Financial Officer)

**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 Quarterly Report on Form 10-Q of Zevra Therapeutics, Inc., (the "Company") for the quarterly period ended March 31, 2023, 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.

May 15, 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.

**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 Quarterly Report on Form 10-Q of Zevra Therapeutics, Inc., (the "Company") for the quarterly period ended March 31, 2023, 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.

May 15, 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.