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

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of Earliest Event Reported): March 7, 2023

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

Delaware	**001-36913**	**20-5894398**
(State or Other Jurisdiction of Incorporation)	**(Commission File Number)**	**(IRS Employer Identification No.)**
1180 Celebration Boulevard, Suite 103, Celebration, FL		**34747**
(Address of Principal Executive Offices)		**(Zip Code)**

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

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instructions A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock	ZVRA	The Nasdaq Stock Market LLC (Nasdaq Global Select Market)

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

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. ☐

Item 2.02 Results of Operations and Financial Condition.

On March 7, 2023, Zevra Therapeutics, Inc., a Delaware corporation, or Zevra, issued a press release announcing its financial results for the fourth quarter and fiscal year ended December 31, 2022, as well as information regarding a conference call and live audio webcast with slide presentation to discuss its financial results and corporate updates scheduled for Tuesday, March 7, 2023 at 8:30 a.m. ET. A copy of the press release and presentation are furnished as Exhibit 99.1 and Exhibit 99.2, respectively, to this Current Report on Form 8-K. The information contained in the press release and presentation, furnished as Exhibit 99.1 and Exhibit 99.2, respectively, shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or the Exchange Act, and is not incorporated by reference into any of Zevra's filings under the Securities Act of 1933, as amended, or the Securities Act, whether made before or after the date hereof, except as shall be expressly set forth by specific reference in any such filing.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Press Release dated March 7, 2023.
99.2	Presentation dated March 7, 2023.
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

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: March 7, 2023

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

Zevra Therapeutics Reports Fourth Quarter and Fiscal Year 2022 Financial Results and Corporate Updates

Conference Call and Live Audio Webcast with Slide Presentation Scheduled for Today, March 7, 2023, 8:30 a.m. ET

Newly rebranded Zevra Therapeutics well positioned to become a commercially focused rare disease company

Arimoclomol NDA resubmission on track to be filed as early as Q3 2023

KP1077 Phase 2 Trial on track to deliver preliminary data as early as Q3 2023 and top-line data as early as year-end 2023

AZSTARYS® royalty revenue growing with one or more revenue milestones expected to be achieved in 2023

Strong balance sheet with cash, cash equivalents and investments of $102.9 million which extends the cash runway into 2026

Celebration, FL – **March 7, 2023** –Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) ("Zevra," or "the Company," formerly "KemPharm, Inc."), a rare disease therapeutics company, today reported its financial results for the fourth quarter and year ended December 31, 2022.

"Zevra is a company transformed, with a new strategic purpose to deliver novel therapeutics to patients with rare diseases," said Richard W. Pascoe, Chief Executive Officer of Zevra Therapeutics. "The Company is guided by a world-class leadership team and board of directors, and we are in a strong position to execute on multiple anticipated milestones during 2023 that should provide a springboard for continued value creation. The Company's future is more promising than at any time in our history as we are primed to advance arimoclomol, our NDA-stage investigational product candidate for Niemann-Pick disease Type C ("NPC"), and KP1077, our lead clinical candidate for treatment for idiopathic hypersomnia (IH), towards key regulatory and data events during the year. As we move Zevra forward, we will continue to leverage our legacy platform to target internally discovered rare disease product opportunities and to extend exclusivity of our pipeline assets through life cycle management."

Mr. Pascoe continued, "We believe the numerous clinical, regulatory and revenue milestone opportunities anticipated for 2023 and beyond will position Zevra for continued growth as we focus on bringing much-needed therapies to patients with rare diseases. Our strategic and pipeline development efforts are supported by a strong financial foundation with $102.9 million in cash, cash equivalents, and investments as of December 31, 2022, and with a growing revenue stream from AZSTARYS® royalties and anticipated milestone payments, and ongoing revenue from the arimoclomol early access program in France. Based on our current operating forecast, we expect available capital will allow us to pursue our development plans and extend our cash runway into 2026, positioning us to build a fully integrated commercial company."

Recent Business and Corporate Highlights:

- Renamed company Zevra Therapeutics to reflect strategic transformation into a commercially focused rare disease company.

- Announced Board and senior management enhancements to support strategic efforts:
  - Christopher Posner appointed to Board of Directors;
  - Matthew Plooster appointed as Board Chairman;
  - Richard W. Pascoe appointed Chief Executive Officer;
  - Joshua Schafer appointed as Chief Commercial Officer and Executive Vice President of Business Development;
  - Daniel Gallo, Ph.D., appointed as Senior Vice President of Medical Affairs and Advocacy, and
  - Abbi Maher, J.D., named Vice President of Legal Affairs.

- Advancing toward resubmission of arimoclomol New Drug Application ("NDA") to the U.S. Food and Drug Administration ("FDA"):
  - Working to amass and characterize safety and efficacy data from the four-year open-label extension of the Phase 2/3 clinical trial of arimoclomol in NPC to include as part of the updated arimoclomol NDA.
    - Research presented at the recent 19th Annual WORLD*Symposium*™ 2023 from four-year open-label extension trial suggested that arimoclomol may reduce the long-term progression of NPC.
  - Resubmission of the updated NDA anticipated as early as Q3 2023.

- Initiated Phase 2 clinical trial in December 2022 evaluating KP1077 as a treatment for idiopathic hypersomnia ("IH"):
  - Trial designed as a multi-center, dose-optimizing, double-blind, placebo-controlled, randomized-withdrawal study to evaluate safety and efficacy of KP1077 for the treatment of IH, as well as to assess its impact on the symptoms and severity of "brain fog"
  - Interim efficacy and safety data expected as early as Q3 2023 with potential for topline Phase 2 data by year-end 2023

- Strong balance sheet, with $102.9 million in cash, cash equivalents and investments as of December 31, 2022:
  - Current operating forecast projects cash runway to extend into 2026
  - The potential to realize milestone and royalty revenue from AZSTARYS® as Corium executes its commercialization strategy could provide further capital flexibility and extend the operating cash runway.

Overview of Fourth Quarter (Q4) and Full-Year (FY) 2022 Financial Results:
Net revenue for Q4 2022 was $2.3 million, as compared to Q4 2021 net revenue of $2.6 million. Q4 2022 net revenue was primarily driven by ongoing reimbursements received from the French early access program for arimoclomol, AZSTARYS® royalty revenues and consulting fees.

Research and development (R&D) expenses were $6.4 million for Q4 2022, as compared to $2.8 million in Q4 2021. The period-over-period increase was primarily driven by the KP1077 clinical development program and the addition of the arimoclomol program in Q2 2022.

General and administrative (G&A) expenses were $5.1 million for Q4 2022, as compared to $2.6 million in Q4 2021. The period-over-period increase was primarily related to the arimoclomol acquisition, which led to an increase in compensation costs, including non-cash stock-based compensation expense, as well as increased professional fees and depreciation and amortization expenses beginning in Q2 2022.

Net loss attributable to common stockholders for Q4 2022 was ($9.0) million, or ($0.26) per basic and diluted share, compared to a net loss attributable to common stockholders of ($2.7) million, or ($0.08) per basic and diluted share for the same period in 2021. Net loss for Q4 2022 was driven primarily by R&D expenses of $6.4 million and G&A expenses of $5.1 million, partially offset by net revenue of $2.3 million and other income of $0.2 million.

Net revenue for FY 2022 was $10.5 million, as compared to FY 2021 net revenue of $28.7 million. The period-over-period decrease was primarily attributed to the non-recurrence of the one-time regulatory milestone payments totaling $20 million for the approval of AZSTARYS and the subsequent scheduling of SDX by the U.S. Drug Enforcement Agency as a C-IV scheduled controlled substance.

For FY 2022, R&D expenses were $19.6 million, as compared to $10.2 million in FY 2021. The period-over-period increase was primarily driven by increased investments in the KP1077 clinical development program, and the addition of the arimoclomol program.

G&A expenses were $15.3 million for FY 2022, as compared to $8.7 million in FY 2021. The period-over-period increase was primarily driven by the acquisition of arimoclomol in Q2 2022.

Net loss attributable to common stockholders for FY 2022 was ($41.5) million, or ($1.20) per basic and diluted share, compared to a net loss attributable to common stockholders of ($62.9) million, or ($2.11) per basic and diluted share in 2021. Net loss for FY 2022 was driven primarily by R&D expenses of $19.6 million, G&A expenses of $15.3 million, and a one-time non-cash charge of $17.7 million for acquired in-process R&D related to the arimoclomol asset acquisition in Q2 2022, partially offset by net revenues of $10.5 million. Non-GAAP FY 2022 net loss, excluding the one-time non-cash charge of $17.7M, was ($23.9M), or ($0.69) per basic and diluted share.

As of December 31, 2022, total cash, cash equivalents, and investments were $102.9 million, which was a decrease of $4.5 million compared to $107.4 million as of September 30, 2022. The decrease was driven, in part, by increased third-party R&D costs related to the KP1077 clinical trial program, the arimoclomol program, and investment of working capital related to the collection of accounts receivable due from French EAP reimbursements.

Based on the Company's current operating forecast, which includes approximately $2.0M in net revenue per quarter from the French early access program reimbursements, existing cash, cash equivalents, and investments are expected to be sufficient to continue operations into 2026, including activities required to submit an NDA for KP1077 and its potential PDUFA date, and to fund commercial preparation for the potential launch of arimoclomol in the U.S., if approved.

As of December 31, 2022, the total shares of common stock outstanding was 34,540,304 shares, and the fully diluted common shares outstanding was 47,088,184 shares, which included 4,252,600 shares issuable upon exercise of warrants.

Conference Call Information:
Zevra will host a conference call and live audio webcast with a slide presentation today at 8:30 a.m. ET, to discuss its corporate and financial results for the fourth quarter and full-year 2022.

The audio webcast with a slide presentation will be accessible via the Investor Relations section of the Company's website, http://investors.zevra.com/. An archive of the webcast and presentation will be available for 90 days beginning at approximately 9:30 a.m. ET, on March 7, 2023.

Additionally, interested participants and investors may access the conference call by dialing either:

- (800) 343-4849 (U.S.)
- (203) 518-9848 (International)
- Conference ID: ZVRAQ422

About Zevra:
Zevra Therapeutics is 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, the Company is overcoming complex drug development challenges to bring much-needed therapies to patients.

Arimoclomol, Zevra's orally-delivered, first-in-class investigational product candidate for the treatment of Niemann-Pick disease type C ("NPC"), 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 medicinal product designation for the treatment of NPC by the European Medicines Agency ("EMA").

KP1077 is Zevra's lead clinical candidate being developed to treat idiopathic hypersomnia ("IH") and narcolepsy. KP1077 is comprised solely of serdexmethylphenidate ("SDX"), Zevra's proprietary prodrug of d-methylphenidate ("d-MPH"). The FDA has granted KP1077 orphan drug designation for the treatment of IH, and the U.S. Drug Enforcement Agency ("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.

Early access programs are made available by Zevra Therapeutics, Inc. and its affiliates and are subject to the Company's Early Access Program ("EAP") policy as published on its website at zevra.com. Participation in these programs is subject to the laws and regulations of each jurisdiction under which each respective program is operated. Eligibility for participation in any such program is at the treating physician's discretion.

Caution Concerning Forward-Looking Statements:
This press release may contain forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements include all statements that do not relate solely to historical or current facts, and which can be identified by the use of words such as "may," "will," "expect," "project," "estimate," "anticipate," "plan," "believe," "potential," "should," "continue," "could," "intend," "target," "predict," or the negative versions of those words or other comparable words or expressions, although not all forward-looking statements contain these identifying words or expressions. Forward-looking statements are not guarantees of future actions or performance. These forward-looking statements include without limitation statements regarding: the promise and potential impact of our preclinical or clinical trial data, including without limitation the initiation, timing, and results of any clinical trials or readouts, the timing or results of any Investigational New Drug ("IND") applications and New Drug Application ("NDA") submissions or resubmissions for arimoclomol, KP1077, or any other product candidates for any specific disease indication or at any dosage, the potential achievement of commercial sales or revenue milestones for AZSTARYS and timing thereof, the sufficiency of our cash, cash equivalents and investments to fund our operating activities for any specific period of time, expected net revenue from the French EAP program, and our strategic and product development objectives, including with respect to becoming a leading, commercially focused rare disease company. These forward-looking statements are based on information currently available to Zevra and its current plans or expectations. They are subject to several known and unknown uncertainties, risks, and other important factors that may cause our actual results, performance, or achievements to be materially different from any future results, performance, or achievements expressed or implied by the forward-looking statements. These and other important factors are described in detail in the "Risk Factors" section of Zevra's (formerly KemPharm) Annual Report on Form 10-K for the year ended December 31, 2022, and Zevra's (formerly KemPharm) other filings with the Securities and Exchange Commission. While we may elect to update such forward-looking statements at some point in the future, except as required by law, we disclaim any obligation to do so, even if subsequent events cause our views to change. Although we believe the expectations reflected in such forward-looking statements are reasonable, we cannot assure that such expectations will prove correct. These forward-looking statements should not be relied upon as representing our views as of any date after the date of this press release.

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

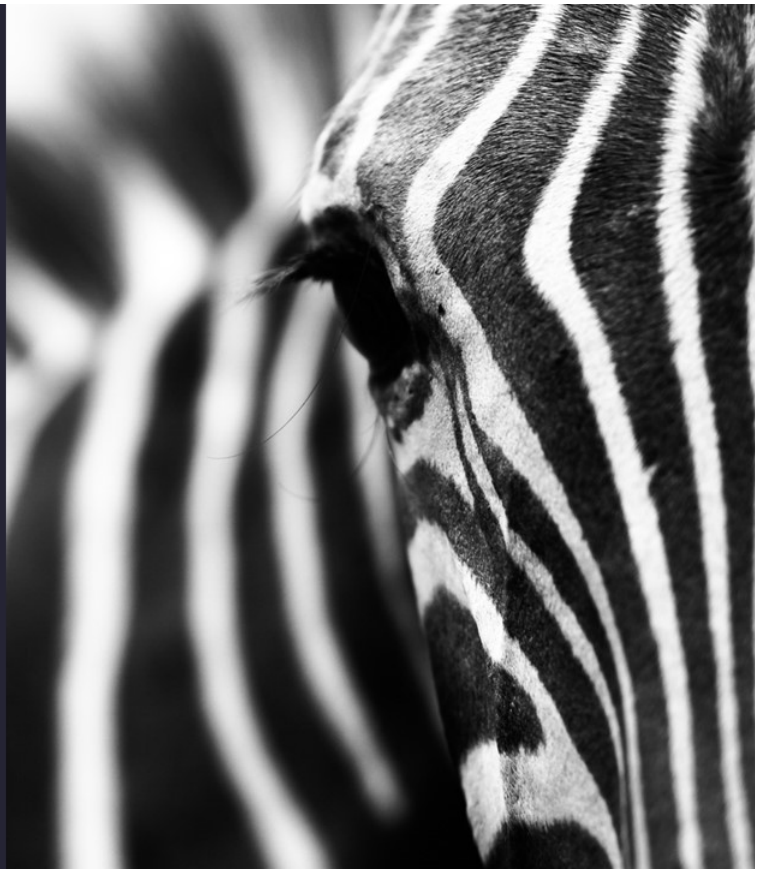
	Year Ended December 31,	
	2022	**2021**
Revenue, net	$ 10,458	$ 28,650
Operating expenses:		
Cost of revenue	343	2,059
Research and development	19,614	10,161
General and administrative	15,343	8,701
Acquired in-process research and development	17,663	—
Total operating expenses	52,963	20,921
(Loss) income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

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

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

Q4 and FY 2022 Results

March 7, 2023



ZEVRA THERAPEUTICS

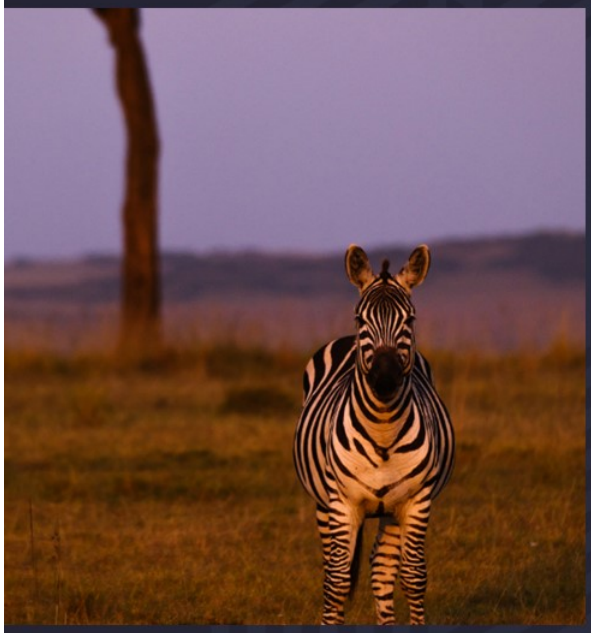
Cautionary Note Regarding Presentation Information

This presentation may contain forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements include all statements that do not relate solely to historical or current facts, and can be identified by the use of words such as "may," "will," "expect," "project," "estimate," "anticipate," "plan," "believe," "potential," "should," "continue," "could," "intend," "target," "predict," or the negative versions of those words or other comparable words or expressions, although not all forward-looking statements contain these identifying words or expressions. Forward-looking statements are not guarantees of future actions or performance. These forward-looking statements include statements regarding the promise and potential impact of our preclinical or clinical trial data, including without limitation the timing and results of any clinical trials or readouts, the timing or results of any Investigational New Drug applications and NDA submissions, including the resubmission of the NDA for arimoclomol, communications with the FDA, the potential uses or benefits of arimoclomol, KP1077, SDX or any other product candidates for any specific disease indication or at any dosage, the potential benefits of any of Zevra's product candidates, the success or timing of the launch or commercialization of AZSTARYS® or any other products or related sales milestones, the sufficiency of cash to fund operations, expected net revenue from the French EAP program, our plans or ability to seek funding, our plans with respect to our share repurchase program, and our strategic and product development objectives. These forward-looking statements are based on information currently available to Zevra and its current plans or expectations and are subject to a number of known and unknown uncertainties, risks and other important factors that may cause our actual results, performance or achievements expressed or implied by the forward-looking statements. These and other important factors are described in detail in the "Risk Factors" section of Zevra's Annual Report on Form 10-K for the year ended December 31, 2022, and Zevra's other filings with the Securities and Exchange Commission.

While we may elect to update such forward-looking statements at some point in the future, except as required by law, we disclaim any obligation to do so, even if subsequent events cause our views to change. Although we believe the expectations reflected in such forward-looking statements are reasonable, we can give no assurance that such expectations will prove to be correct. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to this presentation.

This presentation also may contain estimates and other statistical data made by independent parties and by us relating to market size and other data about our industry. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, projections, assumptions and estimates of our future performance and the future performance of the markets in which we operate are necessarily subject to a high degree of uncertainty and risk.

Agenda



Strategic Overview, 2022 Highlights & Program Updates

Richard W. Pascoe, CEO

AZSTARYS® Commercial Update, 2022 Financial Results & 2023 Financial Guidance

R. LaDuane Clifton, CFO, Secretary & Treasurer

Upcoming Milestones & 2023 Outlook

Richard W. Pascoe, CEO

Q&A

Richard W. Pascoe, CEO
R. LaDuane Clifton, CFO, Secretary & Treasurer
Travis C. Mickle, President & Co-Founder
Christal Mickle, Chief Product Development Officer
Joshua Schafer, Chief Commercial Officer & EVP, BD

We are Zevra

ZEVRA THERAPEUTICS

Nimble and focused rare disease company with track record of success in overcoming drug development & regulatory challenges

Leveraging unique insights and regulatory strategies to forge pathways to success for transformational rare disease therapies

Well positioned for success with robust pipeline, world-class management and board and strong balance sheet

Our Strategic Evolution

ZEVRA THERAPEUTICS

EVALUATE » STRATEGIZE » EVOLVE

Responding to market dynamics, stakeholder demand, and changing technologies and circumstances to ensure **relevance, progress and prosperity**

Significant Value-Creation Opportunity in Rare Disease for Patients and Shareholders

ZEVRA THERAPEUTICS

- Unique partnerships with patient communities
- Longer market exclusivity and less generic competition
- Lower cost of R&D
- Regulatory and financial incentives
- Shorter development timelines & smaller studies
- Small patient population served by specialist clinicians can be addressed with in-house commercial team

Why Zevra Therapeutics?

Zevra, Greek for zebra, is the internationally recognized symbol for the rare disease community

ZEVRA THERAPEUTICS

Positioned for Success

Strategic focus on rare diseases	• Build a highly differentiated pipeline of development assets with multiple clinical and regulatory milestones • Focus on high-value areas with significant unmet needs in rare disease with potential to internally commercialize
Arimoclomol: treatment of Niemann-Pick disease Type C (NPC)	• NDA-stage drug candidate being developed for the treatment of NPC • "Capital efficient" financial structure; potential for positive cash flow; no shareholder dilution • NDA resubmission expected as early as Q3 2023; potential Zevra commercial candidate
KP1077: treatment of idiopathic hypersomnia (IH) and narcolepsy	• High-value opportunity with significant unmet need • Initiated IH Phase 2 Trial in December 2022 • Initiate Narcolepsy Phase 3 Trial post IH Phase 2 results
Other product opportunities	• Leverage prodrug platform to internally develop product candidates with significant potential value • Business development activities focused on complimentary clinical-stage rare disease assets
AZSTARYS® license	• Expanding launch of AZSTARYS provides ongoing revenue potential from royalties and milestones
Strong balance sheet	• Cash, cash equivalents and investments of **$102.9 M** as of December 31, 2022 • Strong cash position supports development plan and other opportunities • Based on operating forecast, **cash runway expected to extend into 2026**

2022 Highlights

ZEVRA THERAPEUTICS

Arimoclomol for Niemann-Pick Type C

- **5/15:** Arimoclomol acquisition
- Completion of 4-year safety trial
- Ongoing collaborative dialogue and periodic meetings with the FDA

KP1077 for Rare Sleep Disorders

- **4/19:** Phase 1 SDX cardiovascular safety trial initiation
- **5/5:** IND submitted for Phase 2 study in idiopathic hypersomnia
- **9/28:** Phase 1 positive topline cardiovascular trial data
- **11/18:** Orphan drug designation in IH
- **12/21:** Phase 2 trial initiation in idiopathic hypersomnia

Financial

- **3/16:** One-time fee of $1.975M fee from Corium following FDA approval of ADLARITY®
- Net revenue of $10.5M for full year 2022
- Cash, cash equivalents and investments of $102.9M as of Dec. 31, 2022
- Available capital expected to extend cash runway into 2026

Leadership Appointments

- 11/28: Christopher Posner appointed independent director
- 8/9: Nichol Ochsner appointed VP, IR and Corp Comms

Q4 2022 and Recent Highlights

KP1077 for Rare Sleep Disorders
November 18, 2022
Orphan Drug Designation received for treatment of IH

KP1077 for Rare Sleep Disorders
December 21, 2022
Initiation of Phase 2 study in IH

Leadership Appointment
January 9, 2023
Matthew Plooster
Board Chairman
Richard W. Pascoe
Chief Executive Officer
Joshua Schafer,
Chief Commercial Officer and EVP of BD

Leadership Appointment
January 31, 2023
Daniel Gallo, Ph.D.
SVP Medical Affairs & Advocacy
Abbi Maher, J.D.
VP of Legal Affairs

Corporate
February 22, 2023
Company renamed Zevra Therapeutics, Inc.

Arimoclomol for Niemann-Pick Disease Type C
February 24, 2023
Interim analysis of four-year safety study presented at WORLDSymposium suggest arimoclomol may reduce disease progression

Corporate
February 28, 2023
Joined NORD Corporate Council

Corporate
March 1, 2023
Began trading as ZVRA

*Dates of corporate announcements

ZEVRA THERAPEUTICS

Financial

Net revenue of **$2.3M** for Q4

Ended the year with **$102.9 million** of capital available on the balance sheet

World Class Leadership Team and Board

Decades of R&D and Commercialization Experience Driving Excellence in Rare Disease

Richard Pascoe
CEO

Travis Mickle, Ph.D.
President & Co-Founder

R. LaDuane Clifton
CFO, Secretary & Treasurer

Sven Guenther, Ph.D.
Chief Scientific Officer

Christal Mickle
Chief Product Development Officer & Co-Founder

Joshua Schafer
Chief Commercial Officer & EVP of BD

Board of Directors

Matthew R. Plooster
Chairman

Richard Pascoe

Travis Mickle, Ph.D.

Tamara A. (Seymour) Favorito

Joseph B. Saluri

David S. Tierney, M.D.

Christopher Posner

ZEVRA THERAPEUTICS

ARIMOCLOMOL

For the Treatment of Niemann-Pick Disease Type C (NPC)

Arimoclomol – Innovative Product for a High Unmet Need

ZEVRA THERAPEUTICS

FIRST-IN-CLASS, ORAL TREATMENT INTENDED FOR NPC

- Capsule formulation designed to be swallowed whole, opened to allow contents to be mixed with soft foods/liquids or delivered through a gastric feeding tube
- Nonclinical and clinical evidence demonstrated significantly improved lysosomal and cellular function with arimoclomol treatment

EXTENSIVELY RESEARCHED

- Studied in ten Phase 1, four Phase 2, and three Phase 2/3 trials
- No significant safety findings identified to date (500+ patients treated)
- Positive efficacy results from NPC trial (NPC-002) and Phase 2 trial in Gaucher Disease (GD), both of which are lysosomal storage disorders

BENEFICIAL REGULATORY POSITIONING

- Orphan Drug Designation for NPC in U.S. and EU
- Fast-Track Designation, Breakthrough Therapy Designation, and Rare Pediatric Disease Designation from the FDA for NPC
- Eligible to receive Rare Pediatric Disease Priority Review Voucher if approved by FDA

Zevra expects to resubmit the NDA for arimoclomol in NPC as early as Q3 2023

Near-Term Opportunity to Commercialize and Retain Full Market Value

ZEVRA THERAPEUTICS

Launch arimoclomol with a small, focused commercialization effort which can be foundation for future rare disease products, including KP1077

1. Small & nimble commercial team
2. Lower marketing spend
3. Patient advocacy relationships support adoption
4. Market entry through U.S. and E.U. EAPs
5. Commercial opportunities outside the U.S.

ZEVRA THERAPEUTICS

SDX PRODUCT CANDIDATE: KP1077

For the Treatment of Idiopathic Hypersomnia (IH)

KP1077 – Multiple Clinical Programs Targeting Rare Sleep Indications

ZEVRA THERAPEUTICS

KP1077 Represents a Potential "Portfolio in a Pill" Opportunity

IDIOPATHIC HYPERSOMNIA	NARCOLEPSY
• Lead KP1077 indication • Investigational New Drug (IND) application cleared by FDA • Ongoing phase 2 clinical trial was initiated in December 2022 • Interim data from Phase 2 clinical trial expected as early as Q3 2023 • Top-line data expected by EOY 2023	• Second KP1077 indication would allow Zevra to address two rare sleep indications that are underserved by currently available medications • Evaluate the potential to initiate narcolepsy Phase 3 trial based on IH phase 2 results • Seek to leverage key data points from IH program to expedite narcolepsy development

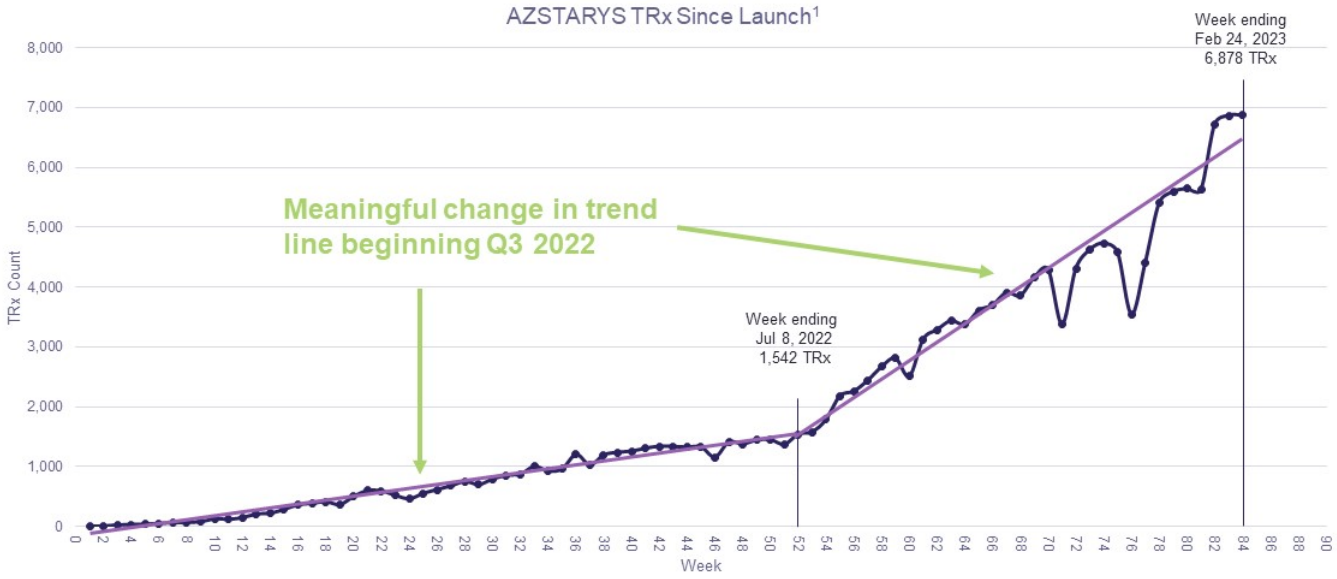
IH Phase 2 results may support advancement into Phase 3 in narcolepsy

AZSTARYS® Commercialization Update

d-Methylphenidate Prodrug Product for the Treatment of ADHD

AZSTARYS® Prescription Trends Are Encouraging

Potential to achieve one or more sales milestones based on current trend



[1] Source: Symphony Health, Metys™ Version 5.8.1, 2023

AZSTARYS® Provides a Foundation

Royalties from legacy develop/out-license model may not be a strong growth driver in short or medium term

	Quarterly TRx[1]	Cumulative TRx	Actual Royalties
Q3 2021	497	497	$0.03M
Q4 2021	5,189	5,686	$0.06M
Q1 2022	8,710	14,396	$0.08M
Q2 2022	15,130	29,526	$0.13M
Q3 2022	25,418	54,944	$0.24M
Q4 2022	53,811	108,755	$0.40M
Q1 2023 (thru Feb 24)	55,272	164,027	TBD

- Trend line improvement noted in Q3 2022, with coverage at three largest PBMs and ~175 field reps in the field (as reported by Corium, Inc.)
- Potential for FY 2023 to benefit from added focus on adult ADHD market
- Royalties are meaningful, but modest
- Sales milestones, if achieved, improve return

[1] Source: Symphony Health, Metys™ Version 5.8.1, 2023

ZEVRA THERAPEUTICS

FINANCIAL UPDATE

Financial Position is a Source of Strength

Q4 and FY 2022 Results:

- Net Revenue:
  - Q4 2022 was $2.3M; FY 2022 was $10.5M, derived primarily from the French EAP program, royalties and consulting service fees
- Net Loss Attributable to Common Stockholders:
  - Q4 2022 was ($9.0M), or ($0.26) per basic and diluted share, driven primarily by R&D expense of $6.4M, and G&A expense of $5.1M, partially offset by net revenue of $2.3M
  - FY 2022 was ($41.5M), or ($1.20) per basic and diluted share, driven primarily by R&D expense of $19.6M, G&A expense of $15.3M, and a one-time non-cash charge of $17.7M for in-process R&D from the arimoclomol acquisition, partially offset by net revenue of $10.5M
    - Non-GAAP FY 2022 net loss excluding the one-time non-cash charge of $17.7M was ($23.9M), or ($0.69) per basic and diluted share

Balance Sheet as of Dec 31, 2022:

- Cash, cash equivalents and investments was $102.9M, a decrease of $4.5M vs. Sep 30, 2022
- Common shares outstanding of 34,540,304, fully diluted shares outstanding of 47,088,184

2023 Financial Guidance

Cash balance remains strong, with potential to realize milestone revenue

- Available cash, cash equivalents and investments expected to extend cash runway into 2026
  - Current operating plan includes the expected reimbursements from the French arimoclomol EAP, the full development of KP1077 through NDA submission and potential PDUFA, as well as investments needed to prepare for the potential U.S. launch of arimoclomol, if approved.
- Based on current prescription trend for AZSTARYS®, we expect to achieve at least the first net sales milestone under the license agreement with Commave Therapeutics, SA
- Net revenue from French EAP program expected to continue at approximately $2.0M per quarter throughout FY 2023 and beyond
- R&D investments for KP1077 will be higher during FY 2023 due to the ongoing Phase 2 trial, and the preparation for the potential initiation of a Phase 3 Trial.

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Outlook for 2023 and Beyond

Multiple Growth Catalysts in 2023

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Arimoclomol

- Potential to re-file NDA as early as Q3 2023
- Anticipate ongoing quarterly revenue from French EAP reimbursements

KP1077 Development Program

- Interim data from Phase 2 clinical trial expected as early as Q3 2023
- Top-line data expected by EOY 2023
- Potential to initiate phase 3 trial in narcolepsy following IH phase 2 trial results
- Expect to file IND in Q2 2023 for narcolepsy

Strong Balance Sheet to Support Value Creation

- Solid balance sheet supports development efforts and other pipeline expansion activities
- Available capital extends cash runway into 2026

AZSTARYS®

- Potential to realize sales milestones and continued royalty revenue from AZSTARYS®
- Adds capital flexibility and potential to further extend cash runway

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Q&A

Thank You.

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