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): August 14, 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, par value $0.0001 per share	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 August 14, 2023, Zevra Therapeutics, Inc., a Delaware corporation ("Zevra" or "the Company"), issued a press release announcing its financial results for the second quarter ended June 30, 2023, 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 Monday, August 14, 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, and is not incorporated by reference into any of Zevra's filings under the Securities Act of 1933, as amended, 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 August 14, 2023.
99.2	Presentation dated August 14, 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: August 14, 2023

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

Zevra Therapeutics Reports Corporate Updates and Second Quarter 2023 Financial Results

Completed collaborative and productive pre-submission meeting with FDA for arimoclomol NDA in August 2023; filing expected in Q4 2023

Net revenue of $8.5M for Q2 2023, which includes $5 million milestone payment earned under the AZSTARYS® license agreement

Ended Q2 2023 with $87.4 million in cash, cash equivalents, and investments, supporting our forecasted cash runway into 2026

Conference call and live audio webcast with slide presentation scheduled for today, August 14, 2023, 8:30 a.m. ET

Celebration, FL – August 14, 2023 – Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) (Zevra, or the Company), a rare disease therapeutics company, today provided corporate updates and reported its financial results for the quarter ended June 30, 2023.

Recent Business and Corporate Highlights:

- Continued advancement of the arimoclomol New Drug Application (NDA) for resubmission to the U.S. Food and Drug Administration (FDA):
    - Completed a productive and collaborative pre-submission meeting with the FDA in August 2023, receiving important information that will be used to finalize the NDA for resubmission.
    - NDA package is anticipated to be submitted in Q4 2023.
- Year-to-date net sales of AZSTARYS® surpassed $25 million, triggering the first net sales milestone payment of $5 million, which was earned and recognized in Q2 2023 revenue, and was received after quarter-end; net sales trend supports the potential to earn a second net sales milestone during 2023.
- Continued advancement of a Phase 2 clinical trial evaluating KP1077 as an investigational treatment for IH:
    - Phase 2 IH trial is actively enrolling 48 adult patients with IH at more than 30 sites in the U.S.

- Interim Phase 2 data for the open-label titration phase of the trial are expected by the end of Q3 2023.
- Topline Phase 2 data in IH is expected to be reported in the first half of 2024 based on the pace of enrollment.

- Expanded the clinical program for KP1077 by opening an Investigational New Drug Application (IND) for narcolepsy, extending its potential to address multiple rare sleep disorders.
  - Phase 1 clinical trial in healthy volunteers initiated during Q2 2023 and is currently enrolling.
  - By leveraging the robust data from the IH program and the existing dataset generated as part of the AZSTARYS development program for serdexmethylphenidate (SDX), the sole active pharmaceutical ingredient in KP1077, Zevra can potentially initiate a pivotal Phase 3 trial in narcolepsy sometime next year.

- Strong balance sheet, with $87.4 million in cash, cash equivalents, and investments as of June 30, 2023, which supports our forecasted operating cash runway into 2026.
  - Forecast includes the ongoing reimbursements from the French early access program for arimoclomol, completion of the arimoclomol NDA resubmission, commercial activities to support the launch of arimoclomol, if approved, and completion of the KP1077 development program for IH up to NDA submission.
  - Forecast does not include revenue from arimoclomol after potential FDA approval, or the potential sale of the Priority Review Voucher, which would be received at that time, as well.

- Thomas Anderson was appointed to the Board of Directors on August 7, 2023 as part of an ongoing plan of Board refreshment first announced on May 8, 2023.

“The first half of 2023 has been a time of dynamic change for Zevra, and we are pleased with our progress in executing on the key priorities for our programs in Niemann-Pick Disease type C (NPC) and Idiopathic Hypersomnia (IH),” said Christal Mickle, interim Chief Executive Officer and Chief Development Officer at Zevra. “We have made meaningful progress in our preparation of the arimoclomol NDA, including a productive and collaborative pre-submission meeting with the FDA earlier this month, which provides confidence as we anticipate re-submission of the NDA package by the end of this year. In addition, the KP1077 program is on track, and we are pleased with progress toward the AZSTARYS® net sales milestones and the momentum toward the possibility of earning a second net sales milestone in 2023. Zevra has several upcoming catalysts for value creation, and we believe our focus on developing and commercializing therapies for rare diseases with a patients-first approach will lead to better therapies for the communities we serve.”

Tamara A. Favorito, Zevra's Board Chair added, "Just last week, we announced the addition of Thomas Anderson to our Board of Directors, which was an important step in executing the plan of Board refreshment we announced on May 8, 2023. Tom brings to Zevra relevant experience in managing successful commercial teams, and in navigating complex drug development challenges, including a track record of success in strategic roles within the rare disease space. Our search to identify both a new chief executive officer and an additional replacement Board member continues to progress. There are excellent candidates available, and I am confident that we will fill these roles in the near-term."

Overview of Q2 2023 Financial Results:

Net revenue for Q2 2023 was $8.5 million compared to Q2 2022 net revenue of $1.3 million. AZSTARYS milestone revenues, ongoing royalties from AZSTARYS, and the French early access program for arimoclomol primarily drove Q2 2023 net revenue.

Research and development (R&D) expenses were $7.4 million for Q2 2023, compared to $4.8 million in Q2 2022. The increase in R&D expenses were primarily driven by the ongoing Phase 2 clinical trial in KP1077, along with the ongoing work to prepare the arimoclomol NDA for resubmission.

General and administrative (G&A) expenses were $7.0 million for Q2 2023, compared to $3.6 million in Q2 2022. The period-over-period increase was primarily related to an increase in personnel costs and professional fees.

Net loss for Q2 2023 was ($5.1) million, or ($0.15) per basic and diluted share, compared to a net loss of ($24.0) million, or ($0.70) per basic and diluted share for the same period in 2022. The net loss during Q2 2022 included recognition of $17.7 million of expense related to acquired in-process research and development from the arimoclomol asset acquisition which was immediately expensed.

As of June 30, 2023, total cash, cash equivalents, and investments were $87.4 million, a decrease of $7.9 million compared to $95.3 million as of March 31, 2023. The decrease was driven, in part, by increased third-party R&D costs related to the KP1077 clinical trial program and the arimoclomol program and increased G&A expenses during the period. The balance as of June 30, 2023, does not include the cash payment of the $5 million net sales milestone earned under the AZSTARYS license agreement which was received after quarter-end.

Based on the Company's current operating forecast, existing cash, cash equivalents, and investments are expected to be sufficient to continue operations into 2026.

As of June 30, 2023, total shares of common stock outstanding were 33,928,005, and fully diluted common shares outstanding were 49,315,197, which included 4,252,490 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 Q2 2023.

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 August 14, 2023.

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

- (800) 267-6316 (U.S.)
- +1 (203) 518-9783 (International)
- Conference ID: ZVRAQ223

About Niemann-Pick disease type C (NPC):

Niemann-Pick disease type C (NPC) is an ultra-rare and progressive, neurodegenerative lysosomal storage disorder characterized by an inability of the body to transport cholesterol and other lipids within the cell, leading to an accumulation of these substances in various tissue areas, including brain tissue. The disease is caused by mutations in the NPC1 or NPC2 genes which are responsible for making lysosomal proteins and is an autosomal recessive trait. Both children and adults can be affected by NPC with varying clinical presentations. Those living with NPC lose independence due to physical and cognitive limitations, with key neurological impairments presenting in speech, cognition, swallowing, ambulation, and fine motor skills. Disease progression is irreversible and can be fatal within months or take years to be diagnosed and advance in severity.

About Arimoclomol:

Arimoclomol, Zevra's orally-delivered, first-in-class investigational product candidate for the treatment of NPC, has been granted orphan drug designation, Fast Track designation, Breakthrough Therapy designation and rare pediatric disease designation for NPC by the FDA, and orphan medicinal product designation for the treatment of NPC by the European Medicines Agency (EMA). The arimoclomol NDA is currently being prepared for resubmission to the FDA.

About Idiopathic Hypersomnia (IH):

Idiopathic hypersomnia (IH) is a rare sleep disorder characterized by excessive daytime sleepiness. Patients with IH experience daytime lapses into sleep, or an irrepressible need to sleep that persists even with adequate or prolonged nighttime sleep. Additionally, those with IH have extreme difficulty waking, otherwise known as “sleep inertia,” severe “brain fog”, and often fall asleep unintentionally or at inappropriate times. These symptoms of IH often lead to further, even more debilitating problems such as memory lapses, difficulty maintaining focus, and depression.

It is estimated that approximately 37,000 patients in the United States are currently diagnosed with IH and seeking treatment, although the total patient population may be much larger due to some patients not seeking treatment or being undiagnosed or misdiagnosed.

About Narcolepsy:

Narcolepsy is a chronic debilitating central disorder of hypersomnolence. The primary symptom of narcolepsy is excessive daytime sleepiness characterized by daily episodes of an irrepressible need to sleep or daytime lapses into sleep. Patients with narcolepsy have an abnormal rapid eye movement (REM) sleep phase which can cause disrupted nighttime sleep, sleep paralysis and sleep-related hallucinations during sleep-wake transitions. Narcolepsy has severe personal, social, and economic consequences. Patients with narcolepsy experience substantial impairment of their mental and physical wellbeing, and depression and anxiety are common. Cognitive dysfunctions such as difficulty to focus and memory lapses (also referred to as ‘brain fog’) are frequently reported. The many symptoms experienced by patients with narcolepsy result in a high disease burden and poor quality of life.

Narcolepsy is categorized in to two types: narcolepsy type 1 (NT1) and type 2 (NT2). NT1 is considered a distinct disease entity characterized in part by loss of hypocretin neurons and symptoms of cataplexy (sudden, brief attacks of muscle weakness sometimes resulting in the body to fall uncontrollably, often triggered by strong emotions). When narcolepsy presents without cataplexy and with normal hypocretin-1 concentrations in the cerebrospinal fluid (CSF), it is categorized as NT2 (Hypocretin-1 is also known as orexin-A, a neuropeptide involved in regulating sleep-wake cycles).

The combined worldwide prevalence of both types of narcolepsy has been estimated to be 25-50 per 100,000 people. Epidemiological studies using well-defined criteria for assessing the prevalence of narcolepsy (both NT1 and NT2) estimate incidence rates ranging from 31 to 79 per 100,000 people corresponding to approximately 100,000 to 260,000 total patients in the United States.

About SDX and KP1077:

Serdexmethylphenidate (SDX) is Zevra’s proprietary prodrug of d-methylphenidate (d-MPH) and the sole active pharmaceutical ingredient (API) in KP1077, Zevra’s lead clinical candidate being developed as a treatment for idiopathic hypersomnia (IH) and narcolepsy. Zevra is currently enrolling a multicenter, dose-optimizing, double-blind, placebo-controlled, randomized-withdrawal Phase 2 clinical trial to evaluate safety and efficacy of KP1077 as a treatment for IH. For more information regarding the Phase 2 trial, visit www.clinicaltrials.gov.

SDX is also the primary API in AZSTARYS®, a once-daily product for the treatment of attention deficit hyperactivity disorder (ADHD) in patients ages six and older being commercialized in the U.S. by Corium, Inc.

KP1077 has been granted Orphan Drug Designation by the U.S. Food and Drug Administration (FDA) for the treatment of IH, and the U.S. Drug Enforcement Agency (DEA) has classified SDX, the sole API in KP1077, 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.

About Zevra Therapeutics:

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. With both regulatory and clinical stage product candidates, the Company is building its commercial capability to make new therapies available to the rare disease community.

Early access programs are made available by Zevra Therapeutics 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.

Cautionary Note 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 senior leadership and board member transitions and refreshment, or the timing thereof, and our strategic and product development objectives, the potential sale of the Priority Review Voucher, 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 content, information used for, timing or results of any IND applications and 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 the timing thereof, the sufficiency of our cash, cash equivalents and investments to fund our operating activities for any specific period of time, and our strategic and product development objectives, including with respect to becoming a leading, commercially-focused rare disease company. 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 Annual Report on Form 10-K for the year ended December 31, 2022, as updated in Zevra’s Quarterly Report on Form 10-Q for the quarter ended June 30, 2023, 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 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.
UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(in thousands, except share and per share amounts)

	Three months ended June 30,		Six months ended June 30,	
	2023	2022	2023	2022
Revenue, net	$ 8,470	$ 1,300	$ 11,349	$ 5,265
Operating expenses:				
Cost of revenue	677	51	802	59
Research and development	7,433	4,795	16,277	7,877
Selling, general and administrative	7,005	3,558	13,839	6,292
Acquired in-process research and development	—	17,663	—	17,663
Total operating expenses	15,115	26,067	30,918	31,891
Loss from operations	(6,645)	(24,767)	(19,569)	(26,626)
Other (expense) income:				
Interest expense	(197)	(36)	(379)	(41)
Fair value adjustment related to derivative and warrant liability	—	32	—	273
Fair value adjustment related to investments	131	(352)	327	(495)
Interest and other income, net	1,553	366	2,593	264
Total other income	1,487	10	2,541	1
Loss before income taxes	(5,158)	(24,757)	(17,028)	(26,625)
Income tax benefit	74	715	177	719
Net loss	$ (5,084)	$ (24,042)	$ (16,851)	$ (25,906)
Basic and diluted net loss per share of common stock:				
Net loss	$ (0.15)	$ (0.70)	$ (0.49)	$ (0.75)
Weighted average number of shares of common stock outstanding:				
Basic and diluted	33,898,233	34,447,206	34,180,818	34,476,737

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

	June 30, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 66,196	$ 65,466
Securities at fair value	20,696	16,900
Short-term investments - other	479	481
Accounts and other receivables	14,033	8,299
Prepaid expenses and other current assets	2,023	1,877
Total current assets	103,427	93,023
Inventories	546	671
Property and equipment, net	689	794
Operating lease right-of-use assets	803	988
Long-term investments - other	—	20,000
Other long-term assets	53	53
Total assets	$ 105,518	$ 115,529
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 10,510	$ 6,169
Current portion of operating lease liabilities	456	480
Current portion of discount and rebate liabilities	6,965	4,655
Other current liabilities	321	422
Total current liabilities	18,252	11,726
Line of credit payable	12,709	12,800
Operating lease liabilities, less current portion	627	843
Discount and rebate liabilities, less current portion	5,114	4,327
Other long-term liabilities	317	26
Total liabilities	37,019	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 June 30, 2023 or December 31, 2022	—	—
Common stock, $0.0001 par value, 250,000,000 shares authorized, 35,503,697 shares issued and 33,928,005 shares outstanding as of June 30, 2023; 35,450,257 shares issued and 34,540,304 shares outstanding as of December 31, 2022	3	3
Additional paid-in capital	405,127	401,799
Treasury stock, at cost	(10,983)	(7,536)
Accumulated deficit	(325,423)	(308,572)
Accumulated other comprehensive (loss) income	(225)	113
Total stockholders' equity	68,499	85,807
Total liabilities and stockholders' equity	$ 105,518	$ 115,529

ZEVRA THERAPEUTICS

Q2 2023 Results

August 14, 2023

NasdaqGS: ZVRA

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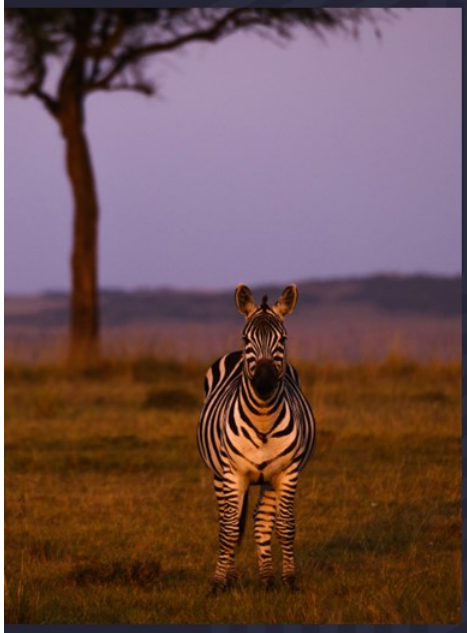
While we may elect to update such forward-looking statements at some point in the future, except as required by law, we disclaim any obligation to do so, even if subsequent events cause our views to change. Although we believe the expectations reflected in such forward-looking statements are reasonable, 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



Second Quarter 2023 Overview

- Christal Mickle, Chief Development Officer, Co-Founder, interim CEO and President

Highlights and Program Updates

- Christal Mickle

Second Quarter 2023 Financial Results and 2023 Financial Guidance

- R. LaDuane Clifton, Chief Financial Officer

Q&A Session

- Christal Mickle
- R. LaDuane Clifton
- Joshua Schafer, Chief Commercial Officer and Executive Vice President, Business Development

Q2 2023 Overview

Positioned for Success

- Multiple near-term clinical and regulatory catalysts for value creation
- Building key functions and capabilities to support successful arimoclomol launch, if approved

Creating and Retaining Value

- In-house commercial team provides foundation to bring therapies to patients
- EAPs and patient advocacy relationships support product adoption at launch

Arimoclomol for Niemann-Pick disease Type C (NPC)

- Orally-delivered, first-in-class investigational product candidate
- Pre-submission meeting with FDA was productive and collaborative; no change in strategy
- Full NDA package expected to be re-submitted in Q4 2023

KP1077 for Idiopathic Hypersomnia (IH) and Narcolepsy

- Lead prodrug candidate for idiopathic hypersomnia (IH) and narcolepsy type I & II
- Interim Phase 2 IH data readout expected by end of Q3 2023; top-line data expected H1 2024
- Enrollment for Phase 1 study for the narcolepsy program underway

Unified Board of Directors

- Thomas Anderson, 35-year industry veteran with rare disease experience, joined Zevra's Board of Directors as part of continuing plan for Board refreshment

Strong Financial Position

$87.4M of capital available on the balance sheet as of 6/30/2023

- Cash runway expected to extend into 2026
- $5M AZSTARYS® milestone payment received Aug 2023
- AZSTARYS trend gives confidence in earning second milestone by YE 2023

Pre-Submission Meeting with FDA was Productive and Collaborative

Complete Response Letter (CRL) and FDA feedback gathered through multiple interactions/meetings has provided added clarity on resubmission package.

COMPLETE RESPONSE LETTER	ZEVRA'S ONGOING RESPONSE
1 Sufficiency of validation and reliability of the Niemann-Pick type C Clinical Severity Scale (NPCCSS) instrument	Additional evidence being provided to support use of the NPCCSS as the primary instrument in measuring NPC disease progression
2 Appropriateness of how to handle data affected by certain patient events and method of primary endpoint analysis	Using FDA preferred primary analysis and supportive additional analyses
3 Robustness of confirmatory evidence to support single efficacy trial	Additional data from multiple new nonclinical studies being provided, as well as data from the 4-year open label extension of the Ph2/3 clinical trial

Strategic Imperatives to Ensure Successful Launch of Arimoclomol

Patient advocacy relationships support adoption with a small and focused commercial team

Develop NPC market through disease awareness and patient identification

Establish arimoclomol as foundational treatment for NPC

Decrease time from diagnosis to treatment through market access

Establish Zevra as a committed partner to patients and caregivers

Phase 2 Clinical Trial of KP1077 in IH

Multi-center, dose-optimizing, double-blind, placebo-controlled, randomized-withdrawal study to evaluate safety of KP1077, as well as potential efficacy endpoints

PHASE 2 TRIAL (N=48)

Part 1:

- Five-week open-label titration phase
- Patients optimized to one of the four doses of SDX (80, 160, 240, or 320 mg/day)

Part 2:

- Two-week randomized, double-blind, withdrawal phase
- 2/3 receive active; 1/3 placebo
- 50% receive single daily dose; 50% receive half daily dose upon awakening and at bedtime

INTERIM DATA:

To inform the design of the Phase 3 trial

Potential key differentiators:

1. Alignment of peak efficacy with patient need through dose optimized timing
2. Expanded exposure range through unique PK

PRIMARY ENDPOINT

- Safety and tolerability of SDX

MAJOR SECONDARY ENDPOINT

- Change in Epworth Sleepiness Scale (ESS) total score

ADDITIONAL EXPLORATORY ENDPOINTS

- Patient Global Impression of Severity (PGI-S)
- Clinical Global Impression of Severity (CGI-S)
- Change in total score on the Idiopathic Hypersomnia Severity Scale (IHSS)
- Sleep Inertia at 1 hour after awakening
- New scale to assess the symptoms and severity of “Brain Fog”

KP1077 Opportunity in Rare Sleep Disorders

ZEVRA THERAPEUTICS

Lead clinical program in idiopathic hypersomnia (IH) with potential to expand into narcolepsy

Idiopathic Hypersomnia

- Ongoing Phase 2 clinical trial was initiated in December 2022
- Designated by the FDA as an orphan drug, and potentially eligible for fast-track review status, as well as designation as a breakthrough treatment
- Interim data from Phase 2 clinical trial expected end of Q3 2023
- Top-line data expected H1 2024

Narcolepsy

- Expanded clinical program for KP1077 by opening IND in April 2023
- Phase 1 trial enrollment underway
- 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 program

Update on Partnered Asset: AZSTARYS®

ZEVRA THERAPEUTICS

Commercial Product Delivering Growing Value

Surpassed $25 million in annual net sales; Zevra earned $5 million net sales milestone

- Q2 2023 royalties were $0.8M
- Based on net sales trend, Zevra expected to earn second net sales milestone payment by the end of 2023

INDICATED FOR TREATMENT OF ADHD
IN PATIENTS 6 YEARS OF AGE AND OLDER

APPROVED BY U.S. FDA
IN MARCH 2021

COMMERCIALIZED IN U.S. BY **CORIUM INC**

Financial Position is a Source of Strength

Q2 2023 Financial Results

- Net Revenue:
  - Q2 2023 was $8.5M; derived primarily from AZSTARYS® milestone payment of $5M, French EAP reimbursements of $2.8M and AZSTARYS royalties of $0.8M
- Net Loss:
  - Q2 2023 was ($5.1M), or ($0.15) per basic and diluted share, driven primarily by R&D expense of $7.4M, and G&A expense of $7.0M, partially offset by net revenue of $8.5M

Balance Sheet as of June 30, 2023

- Cash, cash equivalents and investments was $87.4M, a decrease of $7.9M vs. Mar 31, 2023
- $5M AZSTARYS milestone payment received after quarter-end further bolstering cash balance
- 33,928,005 shares of common stock outstanding, fully diluted shares outstanding of 49,315,197

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 for IH through NDA submission, and commercial investments to prepare for the U.S. launch of arimoclomol, if approved.
  - *Forecast does not include any commercial revenue from arimoclomol, or the potential sale of the Pediatric Review Voucher which would be granted with approval.*
- Based on current AZSTARYS® prescription trends, second net sales milestone may be earned in 2023.
- Net revenue from French EAP program reimbursements for arimoclomol expected to continue at approximately $2.0M per quarter until at least FY 2025.
- R&D investments for KP1077 expected to be higher during FY 2023 due to the ongoing Phase 2 trial in IH, Phase 1 trial in narcolepsy, and ongoing commercial activities supporting Zevra's pipeline.

Q&A Session

ZEVRA THERAPEUTICS

Significant Value Creation through Continued Execution

ZEVRA THERAPEUTICS

Arimoclomol for Niemann-Pick Type C

- Pre-submission meeting with FDA was productive and collaborative; no change in strategy
- Full NDA package expected to be submitted in Q4 2023
- Building key functions and capabilities to support successful arimoclomol launch, if approved
- EAPs and patient advocacy relationships to support product adoption at launch

KP1077 for Rare Sleep Disorders

- Phase 2 IH trial is actively enrolling in the U.S.
- Interim Phase 2 data are expected by end of Q3 2023
- Topline Phase 2 data in IH are expected to be reported in the H1 2024 based on the pace of enrollment
- Phase 1 clinical trial in narcolepsy program initiated during Q2 2023 and currently enrolling

Financial

- Net revenue of $8.5M for Q2 2023
- AZSTARYS® $25M net sales milestone reached; Zevra earned $5M milestone payment
- Cash, cash equivalents and investments of $87.4M as of June 30, 2023
- Available capital expected extends cash runway into 2026

Leadership Updates

- Thomas Anderson joined Board of Directors
- Matthew R. Plooster retired from Board