Zevra Therapeutics Reports Fourth Quarter and Full Year 2023 Financial Results and Corporate Updates

March 28, 2024

Launch of OLPRUVA® underway

Topline results from KP1077 phase 2 study demonstrate clinically meaningful benefits for key IH symptoms

Q4 2023 net revenue of $13.2M, and FY 2023 net revenue of $27.5M

Conference call scheduled for today, March 28, 2024, at 4:30 p.m. ET

CELEBRATION, Fla., March 28, 2024 (GLOBE NEWSWIRE) -- 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 fourth quarter and year ended December 31, 2023.

"We made solid progress on our key priorities in 2023," **said Neil F. McFarlane, President and Chief Executive Officer of Zevra.** "As we look to 2024, our strategic priorities are clear; first, successfully launch OLPRUVA® and ensure access for patients; second, prepare for the launch of arimoclomol; and third, advance the KP1077 development program in sleep disorders.

Mr. McFarlane continued, "We are encouraged by the positive data from our Phase 2 study of KP1077 in patients with idiopathic hypersomnia. KP1077 has been well tolerated while demonstrating early signs of differentiated clinical benefits. These data will help inform our registrational study, which we will discuss with FDA at an end of phase 2 meeting."

Recent Business and Corporate Highlights:

- Completion of the Acquisition of Acer Therapeutics, Inc. (Acer): On November 20, 2023, the Company announced the completion of its acquisition of Acer. The acquisition brought multiple complementary rare disease assets, increased Zevra's revenue potential, and bolstered Zevra's commercial and development capabilities.

- OLPRUVA, an FDA-approved treatment indicated for certain urea cycle disorders (UCDs):
  - To support the commercial launch, the Company accelerated the build-out of a focused and effective customer-facing team with decades of experience in rare diseases. The team includes Rare Disease Sales Specialists, Marketers, Patient Services and Market Access professionals, as well as Medical Science Liaisons and Patient Advocates who are in the field engaging with key customers, with our efforts largely focused on approximately 40 centers of excellence across the United States.
  - To raise awareness of the benefits of OLPRUVA, the Company has established *Quick Start*, which is a 30-day free trial to allow patients and physicians to gain experience with the treatment. Zevra continues to partner with the patient community and UCD treatment centers of excellence to drive brand recognition, while also working with payors to ensure access to OLPRUVA.
  - The Company has seen a meaningful increase in reimbursement coverage since the acquisition, and currently there is more than 70% overall coverage for OLPRUVA with commercial and government payors.
  - The Company expects that OLPRUVA's commercial operations and capabilities will provide scale and cost synergies to support and accelerate the launch and commercialization of arimoclomol, if approved.

- Arimoclomol, an investigational therapeutic candidate for the treatment of Niemann-Pick disease type C (NPC), an ultra-rare, genetic, progressive and fatal neurological disease:
  - During Q4 2023, the Company submitted a comprehensive data set to the U.S. Food and Drug Administration (FDA) supporting its resubmission of the New Drug Application (NDA) for arimoclomol.
  - On March 4, 2024, the Company announced that the FDA had extended the review period for the NDA, resulting in a revised Prescription Drug User Fee Act (PDUFA) date of September 21, 2024. The FDA re-affirmed its intent to present the resubmission for discussion at an advisory committee meeting to be scheduled.
  - If approved, arimoclomol will be a first-in-class, orally delivered treatment for NPC, and the Company will be eligible to receive a Priority Review Voucher.
  - Zevra is preparing for the commercial launch of arimoclomol in the U.S., if approved, and will leverage the commercial infrastructure that is in place for OLPRUVA.
  - The Company plans to publish real-world evidence data from its Expanded Access Program (EAP) and additional data from clinical studies regarding safety and efficacy, to inform physician treatment decision-making and support market access.

- The Company intends to complete the regulatory filing for arimoclomol in the U.S., and then continue its evaluation of optimal regulatory pathways to approval in the E.U. and other parts of the world.

- KP1077 (serdexmethylphenidate, or SDX), an investigational therapeutic candidate both for the treatment of idiopathic hypersomnia (IH), a rare sleep disorder characterized by excessive daytime sleepiness, and for the treatment of narcolepsy:
  - On March 26, 2024, the Company reported positive topline data from its placebo-controlled, double-blind, proof-of-concept Phase 2 study of KP1077 in patients with IH. KP1077 was well-tolerated at all dose levels evaluated in the trial, including the highest dose of 320 mg daily, regardless of dosing regimen (once or twice daily), supporting the study's primary endpoint of safety and tolerability.
  - KP1077 produced clinically meaningful improvement in excessive daytime sleepiness, as assessed by change from baseline in the Epworth Sleepiness Scale. This improvement was maintained during both the five-week open-label titration period and throughout the 2-week double-blind withdrawal period for both dosing regimens.
  - Patients administered KP1077 showed benefits in change from baseline for the IH Severity Scale, Sleep Inertia Visual Analog Scale and Brain Fog severity Scale at the end of the open-label dose titration, and at the end of the double-blind withdrawal period.
  - The study successfully fulfilled the objectives of providing key information for the design of a potentially pivotal efficacy trial, and the results of the secondary efficacy endpoints are supportive of initiating a Phase 3 trial of KP1077. The Company plans to request an end-of-Phase 2 (EOP2) meeting with the U.S. Food and Drug Administration to seek guidance on the Phase 3 clinical trial design.
  - The Company will present new data from a Phase 1 study completed under the narcolepsy IND, and the final results from its Phase 2 study of KP1077 in IH at the upcoming SLEEP 2024 conference.

Overview of Q4 2023 and FY 2023 Financial Results:

Net revenue for Q4 2023 was $13.2 million, compared to prior year Q4 net revenue of $2.2 million. The components of revenue during the current quarter include ongoing royalties from AZSTARYS®, reimbursements from the French EAP for arimoclomol, and some initial sales of OLPRUVA.

Research and development (R&D) expenses were $11.4 million for Q4 2023, compared to $6.5 million in Q4 2022. The increase in R&D expenses was primarily driven by the ongoing Phase 2 clinical study in KP1077, and ongoing work supporting to support the arimoclomol NDA which was resubmitted to the FDA in December 2023.

General and administrative (G&A) expenses were $14.7 million for Q4 2023, compared to $4.7 million in Q4 2022. The period-over-period increase was primarily related to an increase in personnel costs and professional fees associated with our commercial and business development activities.

Net loss for Q4 2023 was ($19.6) million, or ($0.51) per basic and diluted share, compared to a net loss of ($3.0) million, or ($0.09) per basic and diluted share for the same period in 2022.

Net revenue for FY 2023 was $27.5 million compared to prior year net revenue of $10.2 million. The period-over-period increase was primarily attributed to an increase of $18.5 in royalties and milestones received under the AZSTARYS License Agreement, which includes $15.0 million in one-time net sales milestone payments earned during FY 2023, an increase in sales of arimoclomol of $3.3 million, partially offset by a decrease in consulting revenue of $4.5 million.

R&D expenses were $39.8 million for FY 2023, compared to $19.8 million for FY 2022. The increase in R&D expenses was primarily driven by the ongoing KP1077 Phase 2 clinical study in IH, along with work to prepare the arimoclomol NDA for resubmission.

G&A expenses were $34.3 million for FY 2023, compared to $15.0 million in FY 2022. The period-over-period increase was primarily related to an increase in personnel costs and professional fees associated with our commercial and business development activities.

Net loss for FY 2023 was ($46.0) million, or ($1.30) per basic and diluted share, which includes the non-cash impact of the change in fair value adjustment for the warrant liability of ($1.4) million, or ($0.04) per basic and diluted share. Net loss for FY 2022 was ($26.8) million, or ($0.78) per basic and diluted share.

As of December 31, 2023, total cash, cash equivalents, and cash investments were $67.7 million, a decrease of $15.7 million compared to $83.4 million as of September 30, 2023. The decrease was driven, in part, by increased third-party R&D costs related to the KP1077 clinical development program, the arimoclomol program, and increased G&A expenses during the period as the Company invested in its commercial infrastructure. Based on our current operating forecast and available resources, our cash runway is expected to extend into 2026.

- Our cash runway forecast includes revenue from the expected sales of OLPRUVA, ongoing reimbursements from the French EAP for arimoclomol, and investments into the incremental commercial activities needed to support the launch of arimoclomol, if approved, and completion of the KP1077 development program for IH.

- Our cash runway forecast does not include any commercial revenue from arimoclomol which could follow a potential FDA

approval, or the potential sale of the Priority Review Voucher which would be received upon approval.

On November 17, 2023, Zevra completed the acquisition of Acer. Pursuant to the Merger Agreement, Acer continues as a wholly owned subsidiary of Zevra. The Merger included the acquisition of OLPRUVA® (sodium phenylbutyrate) for oral suspension, which was approved by the FDA on December 27, 2022, for the treatment of urea cycle disorders. Acer also has a pipeline of investigational product candidates, including celiprolol for the treatment of vascular Ehlers-Danlos syndrome, patients with a confirmed type III collagen (COL3A1) mutation. At the effective time of the Merger (the "Effective Time"), each share of common stock of Acer, par value $0.0001 per share, issued and outstanding immediately prior to the Effective Time (excluding cancelled shares and any shares held by holders who have exercised their appraisal rights) were converted into the right to receive (i) 0.1210 fully paid and non-assessable shares of common stock of Zevra, par value $0.0001 per share, and (ii) one non-transferable contingent value right ("CVR") issued by Zevra, which represents the right to receive one or more contingent payments up to an additional $76.0 million upon the achievement, if any, of certain commercial and regulatory milestones for Acer's OLPRUVA and celiprolol products within specified time periods. Certain additional cash payments are also possible pursuant to the CVRs with respect to milestones involving Acer's early-stage program ACER-2820 (emetine).

As of December 31, 2023, total shares of common stock outstanding were 41,534,668, and fully diluted common shares outstanding were 58,230,596, which included 5,603,729 shares issuable upon exercise of warrants.

On March 25, 2024, the Audit Committee (the "Audit Committee") of the Company's Board of Directors, after discussion with senior management and the Company's independent registered public accountants, concluded that the Company's previously issued audited consolidated financial statements as of and for the fiscal years ended December 31, 2022 and December 31, 2021, included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2022, (collectively, the "Prior Financial Statements") should no longer be relied upon.

In connection with the preparation of the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2023 (the "2023 Form 10-K"), the Audit Committee concluded that, in prior years it had not appropriately accounted for certain common stock warrants as liabilities. These errors led to understatements of derivative and warrant liability and additional paid-in capital and fluctuations in fair value adjustment related to derivative and warrant liability during the impacted periods.

In addition, in connection with the restatements, the Company has concluded that the previously disclosed errors led to misstatements of fair value adjustment related to derivative and warrant liability, derivative and warrant liability, additional paid-in capital, and retained earnings/(accumulated deficit) that were previously disclosed in the unaudited condensed consolidated balance sheets and statements of operations included in the Company's Quarterly Reports on Form 10-Q for the quarterly periods ended March 31, 2022, June 30, 2022, September 30, 2022, March 31, 2023, June 30, 2023 and September 30, 2023 (collectively, the "Prior Interim Financial Statements"). On March 25, 2024, the Audit Committee, after discussion with senior management and the Company's independent registered public accountants, concluded that the Prior Interim Financial Statements should no longer be relied upon.

The errors and corrective adjustments identified by the Company are non-cash in nature; and they do not impact results of operations or key metrics used by the Company in managing operations, such as revenue, operating expenses, and loss from operations.

Conference Call Information

Zevra will host a conference call and live audio webcast today at 4:30 p.m. ET, to discuss its corporate and financial results for Q4 and FY 2023.

The audio webcast will be accessible via the Investor Relations section of the Company's website, http://investors.zevra.com/. An archive of the audio webcast will be available for 90 days beginning at approximately 5:30 p.m. ET, on March 28, 2024.

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

- (800) 245-3047 (U.S.)
- +1 (203) 518- 9765 (International)
- Conference ID: ZVRAQ423

About Urea Cycle Disorders

UCDs are a group of rare, genetic disorders that can cause harmful ammonia to build up in the blood, potentially resulting in brain damage and neurocognitive impairments if ammonia levels are not controlled.[i] Any increase in ammonia over time is serious. Therefore, it is important to adhere to any dietary protein restrictions and have alternative medication options to help control ammonia levels.

About OLPRUVA®

OLPRUVA (sodium phenylbutyrate) was approved for the treatment of certain UCDs in December 2022 and has recently been marketed under the brand name, OLPRUVA®. OLPRUVA (sodium phenylbutyrate) for oral suspension is a prescription medicine used along with certain therapy, including changes in diet, for the long-term management of adults and children weighing 44

pounds (20 kg) or greater and with a body surface area (BSA) of 1.2 m^2 or greater, with urea cycle disorders (UCDs), involving deficiencies of carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC), or argininosuccinic acid synthetase (AS). Please see Important Safety Information and full Prescribing Information, including Patient Information.

Important Safety Information

Certain medicines may increase the level of ammonia in your blood or cause serious side effects when taken during treatment with OLPRUVA. Tell your doctor about all the medicines you or your child take, especially if you or your child take corticosteroids, valproic acid, haloperidol, and/or probenecid.

OLPRUVA can cause serious side effects, including: 1) nervous system problems (neurotoxicity). Symptoms include sleepiness, tiredness, lightheadedness, vomiting, nausea, headache, confusion, 2) low potassium levels in your blood (hypokalemia) and 3) conditions related to swelling (edema). OLPRUVA contains salt (sodium), which can cause swelling from salt and water retention. Tell your doctor right away if you or your child get any of these symptoms. Your doctor may do certain blood tests to check for side effects during treatment with OLPRUVA. If you have certain medical conditions such as heart, liver or kidney problems, are pregnant/planning to get pregnant or breast-feeding, your doctor will decide if OLPRUVA is right for you.

The most common side effects of OLPRUVA include absent or irregular menstrual periods, decreased appetite, body odor, bad taste or avoiding foods you ate prior to getting sick (taste aversion). These are not all of the possible side effects of OLPRUVA. Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

About Niemann-Pick Disease Type C (NPC)

Niemann-Pick disease type C (NPC) is an ultra-rare, progressive, and 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. 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 by the FDA, and Orphan Medicinal Product designation for the treatment of NPC by the European Medicines Agency (EMA). The FDA has accepted the resubmission of the NDA for arimoclomol and has set a user fee goal date (PDUFA date) of September 21, 2024.

About Idiopathic Hypersomnia (IH)

Idiopathic hypersomnia (IH) is a rare sleep disorder characterized by excessive daytime sleepiness (EDS). 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, based on claims data, that approximately 37,000 patients in the United States are currently diagnosed with IH, although the total patient population may be much larger due to some patients who have not yet been diagnosed, have been misdiagnosed, or are not currently seeking treatment.

About KP1077

KP1077 (serdexmethylphenidate or SDX) is Zevra's proprietary prodrug of d-methylphenidate (d-MPH) and its sole active pharmaceutical ingredient (API). 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 combining science, data, and patient needs to create transformational therapies for diseases with limited or no treatment options. Our mission is to bring life-changing therapeutics to people living with rare diseases. With unique, data-driven development and commercialization strategies, the Company is overcoming complex drug development challenges to make new therapies available to the rare disease community.

Expanded access programs are made available by Zevra Therapeutics and its affiliates and are subject to the Company's Expanded Access Program (EAP) policy as published on its website at www.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.

For more information, please visit www.zevra.com or follow us on X (formerly Twitter) and LinkedIn.

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, including without limitation statements regarding the promise and potential impact of our preclinical or clinical trial data, the initiation, timing and results of any clinical trials or readouts, the content, information used for, timing or results of any NDA submissions or resubmissions for arimoclomol or any other product candidates for any specific disease indication or at any dosage, the potential launch or commercialization of any of product candidates or products, the Company's estimated extent of and impacts of the restatements and the timing of the filing of the 2023 Form 10-K, the sufficiency of our cash, cash equivalents and investments to fund our operating activities for any specific period of time, our plans to build out commercial teams for products or product candidates, 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 September 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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[i] Ah Mew N, et al. Urea cycle disorders overview [updated June 22, 2017]. In: Adam MP, Ardinger HH, Pagon RA, et al, eds. GeneReviews® [Internet]. University of Washington; 1993-2022. Accessed March 20, 2022.

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

	Year Ended December 31, 2023	2022 (As Restated)
Revenue, net	$ 27,461	$ 10,161
Operating expenses:		
Cost of revenue	2,945	222
Research and development	39,806	19,803
Selling, general and administrative	34,314	15,038
Acquired in-process research and development	-	17,663
Total operating expenses	77,065	52,726
Loss from operations	(49,604)	(42,565)
Other (expense) income:		
Interest expense	(1,501)	(335)
Fair value adjustment related to derivative and warrant liability	(98)	15,159
Fair value adjustment related to investments	613	(577)
Interest and other income, net	4,541	1,513
Total other income	3,555	15,760
Loss before income taxes	(46,049)	(26,805)
Income tax (expense) benefit	-	33
Net loss	$ (46,049)	$ (26,772)
Basic and diluted net loss per share of common stock:		
Net loss	$ (1.30)	$ (0.78)
Weighted average number of shares of common stock outstanding:		
Basic and diluted	35,452,460	34,488,800

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

	December 31, 2023	December 31, 2022 (As Restated)
Assets		
Current assets:		
Cash and cash equivalents	$ 43,049	$ 65,466
Securities at fair value	24,688	16,900
Short-term investments - other	-	481
Accounts and other receivables	17,377	8,299
Prepaid expenses and other current assets	1,824	1,688
Total current assets	86,938	92,834
Inventories	9,841	671
Property and equipment, net	736	794
Operating lease right-of-use assets	790	988
Goodwill	4,701	-
Long-term investments - other	-	20,000
Intangible assets, net	69,227	-
Other long-term assets	94	53
Total assets	$ 172,327	$ 115,340
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 28,403	$ 6,169
Line of credit payable	37,700	12,800
Current portion of operating lease liabilities	543	480
Current portion of discount and rebate liabilities	4,550	4,655
Other current liabilities	2,524	719
Total current liabilities	73,720	24,823
Secured promissory note	5,066	-
Derivative and warrant liability	16,100	10,202
Operating lease liabilities, less current portion	456	843
Discount and rebate liabilities, less current portion	7,663	4,327
Other long-term liabilities	7,458	25
Total liabilities	110,463	40,220
Commitments and contingencies		
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, 2023 or December 31, 2022	-	-
Common stock, $0.0001 par value, 250,000,000 shares authorized, 43,110,360 shares issued and 41,534,668 shares outstanding as of December 31, 2023; 35,450,257 shares issued and 34,540,304 shares outstanding as of December 31, 2022	4	3
Additional paid-in capital	472,664	436,269
Treasury stock, at cost	(10,983)	(7,536)
Accumulated deficit	(399,778)	(353,729)
Accumulated other comprehensive income	(43)	113
Total stockholders' equity	61,864	75,120
Total liabilities and stockholders' equity	$ 172,327	$ 115,340

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