Zevra Therapeutics Reports Corporate Updates and Third Quarter 2023 Financial Results

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Arimoclomol NDA resubmission remains on track for end of year filing

Proposed acquisition of Acer Therapeutics on track to close Q4 2023 and will diversify Zevra's revenue with FDA-approved OLPRUVA® for urea cycle disorders (UCDs)

Net revenue of $2.9M for Q3 2023

Ended Q3 2023 with $83.4M in cash, cash equivalents, and investments, supporting our forecasted cash runway into 2026

Conference call and live audio webcast scheduled for today, Nov 7, 2023, 8:00 a.m. ET

CELEBRATION, Fla., Nov. 07, 2023 (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 quarter ended September 30, 2023.

"This is a pivotal time for Zevra as we advance our pipeline programs toward multiple potential value inflection points," said Neil F. McFarlane, President and Chief Executive Officer of Zevra. "As we look to the end of 2023 and into 2024, we're focused on three key priorities. First, to close the proposed Acer acquisition and, if consummated, deliver value to patients by commercializing OLPRUVA. Second, to resubmit the arimoclomol NDA. Third, to complete the Phase 2 trial in idiopathic hypersomnia and prepare to advance KP1077 into Phase 3. We are making solid progress towards achieving our mission of building a patient-focused leading rare disease company."

Recent Business and Corporate Highlights:

- Corporate: Neil F. McFarlane was named President and Chief Executive Officer of Zevra and appointed to the Board of Directors on October 10, 2023. Neil succeeds Christal M.M. Mickle who has been serving as interim CEO and President since June 2023, and who will continue serving in her role as Chief Development Officer. Joseph B. Saluri's previously announced retirement from the Board of Directors also became effective contemporaneous with Neil's appointment.
- Arimoclomol: The new drug application (NDA) for arimoclomol is on track to be resubmitted to the U.S. Food and Drug Administration (FDA) by the end of this year. Arimoclomol is an investigational therapeutic candidate for the treatment of Niemann-Pick disease type C (NPC), an ultra-rare, genetic, progressive and fatal neurological disease.
    - If approved, arimoclomol has the potential to be a first-in-class, orally-delivered treatment for NPC. Because a significant amount of new data has been added to the updated NDA, it is expected to be classified as a Class II submission, which will be subject to a six-month review period by the FDA.
    - Arimoclomol is eligible to receive a Priority Review Voucher, if approved.
    - In parallel with the ongoing regulatory work to resubmit the arimoclomol NDA, we are preparing for the potential commercial launch of arimoclomol in the U.S., if approved, with the goal of making this groundbreaking therapy accessible to patients as soon as possible.
    - The Company intends to complete the regulatory pathway for arimoclomol in the U.S., and then continue the evaluation and identification of the optimal regulatory pathway to approval for arimoclomol in the E.U.
- KP1077: On October 2, 2023, the Company reported interim data from the open-label dose titration phase of its Phase 2 clinical trial evaluating KP1077 in patients with idiopathic hypersomnia (IH).
    - Results demonstrated KP1077 was well-tolerated at all dose levels and both dosing regimens. The interim data related to the secondary and exploratory endpoints showed

marked improvements in patient-reported assessments of key IH symptoms from the open-label titration phase, including excessive daytime sleepiness (EDS), sleep inertia, and brain fog.
    - Top-line data from the completed trial are expected in the first half of 2024 after all patients have completed the double-blind withdrawal phase. The combined open-label interim and upcoming topline data are expected to also provide information related to secondary and exploratory endpoints, including excessive daytime sleepiness, sleep inertia, and brain fog. The results from the completed Phase 2 trial will inform the final design of the anticipated Phase 3 trial in IH expected to be initiated by the end of 2024.
    - Phase 1 clinical trial under the narcolepsy IND was completed for KP1077 in healthy volunteers. Data generated from this trial will be analyzed alongside the Phase 2 IH data to support clinical development of both narcolepsy and IH programs.
- Acer Therapeutics, Inc. (Acer) Acquisition: The acquisition of Acer, announced by Zevra on August 31, 2023, and subject to customary closing conditions including approval by Acer shareholders, would bring multiple rare disease assets that increase and diversify Zevra's revenue potential with the addition of OLPRUVA®, which is indicated for the treatment of urea cycle disorders (UCDs), and expand its clinical development portfolio with the addition of EDSIVO™, currently in Phase 3, for vascular Ehlers-Danlos syndrome (vEDS).
    - Acer's programs strategically complement Zevra's rare disease portfolio and commercial assets and increase and diversify revenues.
    - OLPRUVA's commercial operations and capabilities would provide scale and cost synergies to support and accelerate launch and commercialization of arimoclomol, if approved.
    - Acer's special meeting of shareholders to approve the transaction is scheduled for November 8, 2023.
- Cash Runway: Balance sheet remains strong with $83.4 million in cash, cash equivalents, and investments as of September 30, 2023, which supports our forecasted operating cash runway into 2026.
    - Cash runway forecast includes the ongoing reimbursements from the French expanded access program for arimoclomol, completion of the arimoclomol NDA resubmission, pull forward of commercial activities to support the launch of arimoclomol, if approved, and completion of the KP1077 development program for IH.
    - Cash runway forecast does not include commercial revenue from arimoclomol following potential FDA approval, or the potential sale of the Priority Review Voucher, which would be received upon approval, or any revenue from sales of OLPRUVA.

Overview of Q3 2023 Financial Results:

Net revenue for Q3 2023 was $2.9 million compared to prior year Q3 net revenue of $2.9 million. The components of revenue during the current quarter include ongoing royalties from AZSTARYS® and reimbursements from the French early access program for arimoclomol.

Research and development (R&D) expenses were $12.3 million for Q3 2023, compared to $5.4 million in Q3 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 $5.8 million for Q3 2023, compared to $4.0 million in Q3 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 Q3 2023 was ($14.0) million, or ($0.40) per basic and diluted share, compared to a net loss of ($6.6) million, or ($0.19) per basic and diluted share for the same period in 2022.

As of September 30, 2023, total cash, cash equivalents, and cash investments were $83.4 million, a decrease of ($4.0) million compared to $87.4 million as of June 30, 2023. The decrease was driven, in part, by increased third-party R&D costs related to the KP1077 clinical trial program, the arimoclomol program, and increased G&A expenses during the period.

Concomitant with the signing of the merger agreement and announcement of the Acer transaction, Zevra purchased Acer's outstanding senior secured debt for an amount totaling $28.6 million, and provided a bridge loan to of up to $16.5 million to Acer to provide funding for Acer's commercial efforts related to OLPRUVA and other corporate purposes. As of September 30, 2023, these assets are included in secured corporate notes in the unaudited condensed consolidated balance sheet and totaled $42.0 million as of September 30, 2023. Upon closing of the Acer transaction, if approved, these amounts are expected to be eliminated as an inter-company transaction.

As of September 30, 2023, total shares of common stock outstanding were 36,211,710, and fully diluted common shares outstanding were 51,598,902, which included 4,252,490 shares issuable upon exercise of warrants.

Conference Call Information:

Zevra will host a conference call and live audio webcast today at 8:00 a.m. ET, to discuss its corporate and financial results for Q3 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 9:00 a.m. ET, on November 7, 2023.

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

- (800) 343-4849 (U.S.)
- +1 (203) 518-9843 (International)
- Conference ID: ZVRAQ323

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®:

ACER-001 (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, 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 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, based on claims data, that approximately 37,000 patients in the United States are currently diagnosed with IH treatment, 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 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 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 for 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.

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.

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 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 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 potential launch or commercialization of any of product candidates or products, including in the event of the consummation of the proposed merger transaction, or the timing thereof, the sufficiency of our cash, cash equivalents and investments to fund our operating activities for any specific period of time, statements regarding the proposed merger transaction, its timing and its consummation, the anticipated financial performance of Zevra and Acer related thereto, including the anticipated closing of, benefits of, accounting impact of, and synergies related to the proposed merger transaction, potential strategic implications as a result of the proposed merger transaction, our plans to build out commercial teams for products or product candidates, our commercial infrastructure investments and the impact of the proposed transactions on them, 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 (the SEC). 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.

Additional Information about the Proposed Merger between Acer and Zevra, the Special Meeting and Where to Find It

In connection with the proposed merger, Zevra has filed a registration statement on Form S-4 with the SEC, including a proxy statement / prospectus. The registration statement was declared effective on October 10, 2023. Additionally, Acer's proxy statement was filed on October 10, 2023. On October 30, 2023, Acer filed a supplement to the proxy statement to make certain supplemental disclosures. Acer shareholders are urged to read these materials because they contain important information about Acer, Zevra and the proposed merger. The proxy statement / prospectus and other relevant materials, including any proxy supplements, and any other documents filed by Zevra and/or Acer with the SEC, may be obtained free of charge at the SEC website at www.sec.gov. In addition, Acer shareholders will be able to attend the Acer special meeting via the Internet at https://www.cstproxy.com/acertx/sm2023 and view the Acer 2023 Special Meeting Proxy Statement and the Zevra Therapeutics, Inc. Forms 10-K, 10-Qs and 8-Ks. Acer shareholders are urged to read the proxy statement / prospectus, including any supplements thereto, and the other relevant materials before making any voting or investment decision with respect to the proposed merger.

No Offer or Solicitation

This communication is for informational purposes only and not intended to and does not constitute an offer to subscribe for, buy or sell, the solicitation of an offer to subscribe for, buy or sell or an invitation to subscribe for, buy or sell any securities or the solicitation of any vote or approval in any jurisdiction pursuant to or in connection with the proposed transaction or otherwise, nor shall there be any sale, issuance or transfer of securities in any

jurisdiction in contravention of applicable law. No offer of securities shall be made except by means of a prospectus meeting the requirements of Section 10 of the Securities Act of 1933, as amended, and otherwise in accordance with applicable law.

Participants in the Solicitation

Zevra, Acer and their respective directors and executive officers may be considered participants in the solicitation of proxies in connection with the proposed transaction. Information about the directors and executive officers of Acer is set forth in its Annual Report on Form 10-K for the year ended December 31, 2022, which was filed with the SEC on March 27, 2023, and its proxy statement for its 2023 annual meeting of shareholders, which was filed with the SEC on April 14, 2023. Information about the directors and executive officers of Zevra is set forth in its Annual Report on Form 10-K for the year ended December 31, 2022, which was filed with the SEC on March 7, 2023, and its proxy statement for its 2023 annual meeting of stockholders, which was filed with the SEC on March 15, 2023, the definitive proxy statement filed by Daniel J. Mangless, together with the other participants named therein, which was filed with the SEC on March 17, 2023, and Zevra's Current Reports on Form 8-K, filed with the SEC on March 30, 2023, May 8, 2023, May 15, 2023, and August 7, 2023. Other information regarding the participants in the proxy solicitations and a description of their direct and indirect interests, by security holdings or otherwise, are contained in the proxy statement/prospectus and other relevant materials filed with the SEC.

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

	Three months ended September 30,		Six months ended September 30,	
	2023	2022	2023	2022
Revenue, net	$ 2,895	$ 2,874	$ 14,244	$ 8,139
Operating expenses:				
Cost of revenue	144	141	946	200
Research and development	12,297	5,385	28,574	13,262
Selling, general and administrative	5,818	3,974	19,657	10,266
Acquired in-process research and development	—	—	—	17,663
Total operating expenses	18,259	9,500	49,177	41,391
Loss from operations	(15,364)	(6,626)	(34,933)	(33,252)
Other (expense) income:				
Interest expense	(366)	(124)	(745)	(165)
Fair value adjustment related to derivative and warrant liability	—	22	—	295
Fair value adjustment related to investments	124	(139)	451	(634)
Interest and other income, net	1,738	218	4,331	482
Total other income (expense):	1,496	(23	4,037	(22)
Loss before income taxes	(13,868)	(6,649)	(30,896)	(33,274)
Income tax benefit	(177)	33	—	752
Net loss	$ (14,045)	$ (6,616)	$ (30,896)	$ (32,522)
Basic and diluted net loss per share of common stock:				
Net loss	$ (0.40)	$ (0.19)	$ (0.90)	$ (0.94)
Weighted average number of shares of common stock outstanding:				
Basic and diluted	34,724,614	34,494,702	34,364,075	34,482,791

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

	September 30, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 43,269	$ 65,466
Securities at fair value	39,672	16,900
Secured corporate notes	41,999	—
Short-term investments - other	485	481
Accounts and other receivables	9,927	8,299
Prepaid expenses and other current assets	1,661	1,877
Total current assets:	137,013	93,023

Inventories	481	671
Property and equipment, net	642	794
Operating lease right-of-use assets	698	988
Long-term investments - other	—	20,000
Other long-term assets	148	53
Total assets:	$ 139,982	$ 115,529
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 13,080	$ 6,169
Current portion of operating lease liabilities	433	480
Current portion of discount and rebate liabilities	7,890	4,655
Other current liabilities	311	422
Total current liabilities:	21,714	11,726
Line of credit payable	38,801	12,800
Secured promissory note	5,073	—
Operating lease liabilities, less current portion	517	843
Discount and rebate liabilities, less current portion	4,987	4,327
Other long-term liabilities	420	26
Total liabilities:	71,512	29,722
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 September 30, 2023, or December 31, 2022	—	—
Common stock, $0.0001 par value, 250,000,000 shares authorized, 37,787,402 shares issued and 36,211,710 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	418,138	401,799
Treasury stock, at cost	(10,983)	(7,536)
Accumulated deficit	(339,468)	(308,572)
Accumulated other comprehensive (loss) income	(220)	113
Total stockholders' equity:	67,470	85,807
Total liabilities and stockholders' equity:	$ 138,982	$ 115,529

[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