Zevra Therapeutics Reports Second Quarter 2024 Financial Results and Corporate Highlights

August 13, 2024

FDA advisory committee voted favorably that the data support arimoclomol as effective treatment for patients with NPC; PDUFA date of September 21, 2024

Pro forma June 30, 2024, cash, cash equivalents, investments and net proceeds from underwritten public offering total $113.8 million following the closing on August 12, 2024

Conference call scheduled for today, August 13, 2024, at 4:30 p.m. ET

CELEBRATION, Fla., Aug. 13, 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 second quarter ended June 30, 2024.

"During the second quarter, we made steady progress executing on our strategic objectives," **said Neil F. McFarlane, President and Chief Executive Officer of Zevra**. "We are encouraged by favorable vote from the FDA's Genetic Metabolic Diseases Advisory Committee, that data presented support that arimoclomol is effective in the treatment in patients with Niemann Pick disease type C. While the vote is non-binding, we believe it is an important factor as the FDA completes its consideration for approval. Additionally, we continue the launch of OLPRUVA with a keen focus on driving patient awareness and intend to use the same commercial team to launch arimoclomol, if approved. Additionally, we expect to meet with the FDA to discuss the design of a pivotal Phase 3 trial to study KP1077 in idiopathic hypersomnia at the end of third quarter."

"We continue to be prudent in our capital allocation as we focus on creating long-term value for stockholders," **said R. LaDuane Clifton, Zevra's Chief Financial Officer and Treasurer.** "Our recent underwritten offering provided net proceeds to the Company of $64.5 million, along with attracting a cadre of institutional investors well known as long term supporters of innovation and building momentum as we lean into our near-term catalysts while also extending our cash runway."

Q2 2024 Corporate Highlights:

- **Arimoclomol**
  - On August 2, 2024, the U.S. Food and Drug Administration's (FDA) newly formed Genetic Metabolic Diseases Advisory Committee (the "GeMDAC") convened for the first time and discussed the benefits and risks of arimoclomol.
  - The GeMDAC reviewed comments received from independent experts, NPC patients, and patient advocacy group representatives, and voted favorably that arimoclomol is effective in the treatment of NPC.
  - On Friday, August 9, 2024, the Company received the first round of labeling comments, and is working closely with the FDA.
- **OLPRUVA**
  - On June 18, 2024, announced transition of specialty pharmacy partner to Orsini.
  - Increased OLPRUVA reimbursement coverage to 75% of covered lives and improved preferred status on formulary plans.
  - Added nine (9) new patient enrollments during Q2 2024, which is defined as a prescription for a patient on the Quick Start program or receiving a paid dispense of OLPRUVA.
- **KP1077**
  - On June 3, 2024, presented positive topline data from its Phase 2 study in patients with idiopathic hypersomnia (IH) at the SLEEP 2024 Annual Meeting.
  - The Company submitted a briefing book to the FDA for an end-of-Phase 2 meeting to be held at the end of Q3

2024.

- **Celiprolol**
  - Restarted recruitment for the Phase 3 DiSCOVER trial of celiprolol in patients with Vascular Ehler-Danlos Syndrome (VEDS).
- **Corporate**
  - On April 5, 2024, the Company entered into a $100 million credit facility with leading biotech investors, Perceptive Advisors and Healthcare Royalty Partners.
  - On June 25, 2024, the Company announced the appointment of Rahsaan Thompson as Chief Legal Officer, Secretary and Compliance Officer, and Alison Peters as Chief People Officer, bringing expertise that will support the next phase of the Company's growth.

Overview of Q2 2024 Financial Results:

Net revenue for Q2 2024 was $4.4 million, compared to net revenue of $8.5 million in Q2 2023. The components of revenue during the second quarter included $3.1 million in net reimbursements from the French EAP for arimoclomol, $1.3 million of royalties and other reimbursements under the AZSTARYS® License Agreement, and de minimis OLPRUVA revenue via sales to the new specialty pharmacy, Orsini, were offset by returns from the prior pharmacy in Q2 2024. In addition, cost of goods sold was inflated during the quarter due to recognition of a $3.2 million obsolescence reserve against OLPRUVA inventory which is nearing expiration. This excess inventory was ordered prior to our acquisition of Acer, and the delayed launch impacted the rate of usage, leading to the need for this reserve to be recognized in the quarter.

Research and development (R&D) expenses were $10.5 million for Q2 2024, compared to $7.4 million in Q2 2023. The increase in R&D expenses was primarily driven by an increase in spending for the KP1077 Phase 2 clinical trial and an increase in personnel-related costs, partially offset by a decrease in third-party costs related to arimoclomol.

Selling, general and administrative (SG&A) expenses were $12.6 million for Q2 2024, compared to $6.6 million in Q2 2023. The period-over-period increase reflects the commercial team in place for the entire quarter and actively engaged in activities to build awareness and provide patient services related to OLPRUVA, leading to an increase in personnel costs due to the additional headcount and an increase in other expenses related primarily to the launch of OLPRUVA.

Net loss for Q2 2024 was ($19.9) million, or ($0.48) per basic and diluted share, compared to a net loss of ($2.6) million, or ($0.08) per basic and diluted share for Q2 2023.

As of June 30, 2024, total cash, cash equivalents, and investments were $49.3 million, a decrease of $3.4 million compared to $52.7 million as of March 31, 2024. The decrease was driven, in part, by increased third-party R&D costs related to the KP1077 clinical development program and increased SG&A expenses during the period as the Company invested in its commercial infrastructure.

As of June 30, 2024, total shares of common stock outstanding were 41,991,464, and fully diluted common shares were 57,324,496, which included 5,483,537 shares issuable upon exercise of warrants.

On April 5, 2024, the Company announced the refinancing of its existing debt with up to $100 million in committed capital, which strengthened its balance sheet, simplified its debt structure, and provided non-dilutive capital flexibility. The refinancing was led by Perceptive Advisors and HealthCare Royalty Partners, premier biotech investors. From the initial draw of $60 million at closing, the Company refinanced its existing debt of $43 million and added an incremental $14 million in net cash proceeds to the cash balance after fees and discounts. A second tranche of up to $20 million is available at the Company's discretion until October 5, 2025, and a third tranche of up to $20 million will become available upon approval of arimoclomol, in each case subject to certain terms and conditions.

On August 12, 2024, the Company completed an underwritten public offering, further strengthening its balance sheet. The underwriters fully exercised their overallotment option, resulting in the issuance of approximately 10.6 million shares at a price of $6.50 per share, raising net proceeds of $64.5 million after fees and expenses. Combining the net proceeds from this offering with our existing resources, pro forma June 30, 2024 total cash, cash equivalents and investments was $113.8 million.

Following the closing of the underwritten public offering on August 12, 2024, pro forma June 30, 2024, common shares and fully diluted shares outstanding were 52,606,849 and 67,939,881, respectively. No warrants were issued as part of the underwritten public offering.

Based on our current operating plan, available cash, cash equivalents, and investments, including proceeds from the underwritten offering closed in August, are expected to extend our cash runway into the first quarter of 2027, subject to continuing compliance with our debt covenants.

- Cash runway forecast includes: revenue from the expected sales of OLPRUVA, ongoing reimbursements from the French

EAP for arimoclomol, ongoing royalties under the AZSTARYS license agreement, and investments into the incremental commercial activities needed to support the launch of arimoclomol, if approved.

- Cash runway forecast does not include 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.

Conference Call Information

Zevra will host a conference call and audio webcast today at 4:30 p.m. ET, to discuss its corporate and financial results for Q2 2024.

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 ninety (90) days beginning at approximately 5:30 p.m. ET, on August 13, 2024.

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

- (800) 225-9448 (United States)
- +1 (203) 518- 9708 (International)
- Conference ID: ZVRAQ224

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 therapies, 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 UCDs, involving deficiencies of carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC), or argininosuccinic acid synthetase (AS). OLPRUVA is not used to treat rapid increase of ammonia in the blood (acute hyperammonemia), which can be life-threatening and requires emergency medical treatment. For more information, please visit www.OLPRUVA.com.

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, investigational drug 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 action 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 FDA, and by the European Commission, for the treatment of IH. 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. In addition, KP1077 has intellectual property protection though 2037 and potentially beyond.

About Celiprolol

Celiprolol is an investigational clinical candidate for the treatment of Vascular Ehlers-Danlos Syndrome (VEDS). Celiprolol has been granted Orphan Drug and Breakthrough Therapy Designations by the FDA. Zevra recently restarted enrollment in the Phase 3 trial, known as the DiSCOVER trial being conducted under a Special Protocol Assessment (SPA) agreement with the FDA. Celiprolol's mechanism of action is designed to reduce the mechanical stress on collagen fibers within the arterial wall through vascular dilation and smooth muscle relaxation.

About Vascular Ehlers-Danlos Syndrome

Vascular Ehlers-Danlos syndrome is a rare genetic cardiovascular disorder which impairs collagen 3 rich connective tissue and leads to vascular and hollow organ ruptures.

It is estimated that approximately 7,500 patients in the United States are currently diagnosed patients with VEDS. There remains an unmet need with no approved treatment in the United States. Celiprolol is currently the standard of care in Europe.

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.

For more information, please visit www.zevra.com or follow us on X 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 benefits of any of our products or product candidates for any specific disease or at any dosage; the impact of meetings or communications with the FDA or any advisory committee; decisions by the FDA or any other entity for arimoclomol or any other product candidates; our strategic and product development objectives, including with respect to becoming a leading, commercially focused rare disease company; the potential benefits of our debt facility;, our our financial position, including our cash balance: our corporate governance objectives; potential revenues from our arimoclomol expanded access program; the potential for royalty and milestone contributions, the presentation of data at conferences; and the timing of any of the foregoing. 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, 2023, Zevra's Quarterly Report on Form 10-Q for the three months ended June 30, 2024, 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.

[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.

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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,	
	2024	2023	2024	2023
Revenue, net	$ 4,449	$ 8,470	$ 7,874	$ 11,646
Cost of product revenue (excluding $1,546 and $3,074 in intangible asset amortization for the three and six months ended June 30, 2024, respectively shown separately below)	3,573	677	3,748	802
Intangible asset amortization	1,546	—	3,074	—
Operating expenses:				
Research and development	10,521	7,433	22,798	16,088
Selling, general and administrative	12,604	6,612	22,535	13,839
Total operating expenses	23,125	14,045	45,333	29,927
Loss from operations	(23,795)	(6,252)	(44,281)	(19,083)
Other income (expense):				
Interest expense	(2,110)	(197)	(2,845)	(379)
Fair value adjustment related to warrant and CVR liability	5,779	2,118	9,406	575
Fair value adjustment related to investments	1	131	(26)	327
Interest and other income (expense), net	270	1,553	1,199	2,593
Total other income	3,940	3,605	7,734	3,116
Loss before income taxes	(19,855)	(2,647)	(36,547)	(15,967)
Income tax benefit	(70)	74	—	177
Net loss	$ (19,925)	$ (2,573)	$ (36,547)	$ (15,790)
Basic and diluted net loss per share of common stock:				
Net loss	$ (0.48)	$ (0.08)	$ (0.87)	$ (0.46)
Weighted average number of shares of common stock outstanding:				
Basic and diluted	41,899,087	33,898,233	41,839,582	34,180,818

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

	June 30, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 39,260	$ 43,049
Securities at fair value	9,998	24,688
Accounts and other receivables	8,947	17,377
Prepaid expenses and other current assets	2,686	1,824
Total current assets	60,891	86,938
Inventories	10,198	9,841
Property and equipment, net	678	736
Operating lease right-of-use assets	911	790
Goodwill	4,701	4,701
Intangible assets, net	66,154	69,227
Other long-term assets	875	94
Total assets	$ 144,408	$ 172,327
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 20,452	$ 28,403
Line of credit payable	—	37,700
Current portion of operating lease liabilities	596	543
Current portion of discount and rebate liabilities	6,768	4,550
Other current liabilities	2,633	2,524
Total current liabilities	30,449	73,720
Long-term debt	58,328	5,066
Warrant liability	7,856	16,100
Operating lease liabilities, less current portion	544	456
Discount and rebate liabilities, less current portion	8,115	7,663
Other long-term liabilities	6,638	7,458
Total liabilities	111,930	110,463
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 June 30, 2024, or December 31, 2023	—	—
Common stock, $0.0001 par value, 250,000,000 shares authorized, 43,567,156 shares issued and 41,991,464 shares outstanding as of June 30, 2024; 43,110,360 shares issued and 41,534,668 shares outstanding as of December 31, 2023	4	4
Additional paid-in capital	479,361	472,664
Treasury stock, at cost	(10,983)	(10,983)
Accumulated deficit	(436,325)	(399,778)
Accumulated other comprehensive income (loss)	421	(43)
Total stockholders' equity	32,478	61,864
Total liabilities and stockholders' equity	$ 144,408	$ 172,327