Zevra Therapeutics Reports First Quarter 2024 Financial Results and Corporate Updates

May 8, 2024

Full commercial launch of OLPRUVA at the end of January 2024, with market access growing to ~75% of covered lives as of May 1, 2024

Arimoclomol NDA review underway with PDUFA date of September 21, 2024

Reported positive topline results from KP1077 Phase 2 Trial in idiopathic hypersomnia, with full data to be presented at SLEEP 2024 in early June 2024

Conference call scheduled for today, May 8, 2024, at 8:00 a.m. ET

CELEBRATION, Fla., May 08, 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 first quarter ended March 31, 2024.

"During the first quarter, we made steady progress in executing on our strategic objectives: to successfully launch OLPRUVA and ensure access for patients, to prepare for the potential launch of arimoclomol, and to advance the KP1077 program in sleep disorders," **said Neil F. McFarlane, President and Chief Executive Officer of Zevra**.

"Our recent debt refinancing allowed us to simplify our capital structure while providing additional flexibility to support our strategic priorities," **said R. LaDuane Clifton, Zevra's Chief Financial Officer, Secretary and Treasurer.** "With a solid balance sheet, we are focused on creating long-term value for shareholders by executing against our plan of becoming a leading rare disease company."

Recent Business and Corporate Highlights:

- **OLPRUVA**
  - The full commercial launch of OLPRUVA was initiated at the end of January 2024, and the team has engaged with more than 90% of the specialists at the 40 centers of excellence that treat people with UCD. During the quarter, Zevra had four new patient enrollments, defined as a prescription for a patient eligible for either benefits investigation or the Company's *Quick Start* program.
  - Zevra has seen a meaningful increase in reimbursement coverage, reaching nearly 75% of covered lives as of May 1, 2024.
- **Arimoclomol**
  - During the quarter, the Company announced the FDA's extension of arimoclomol's Prescription Drug User Fee Act (PDUFA) date to September 21, 2024, and reaffirmed its intent to present the resubmission for discussion at an advisory committee meeting to be scheduled.
  - The National Niemann-Pick Disease Foundation, along with six other NPC advocacy and research organizations, submitted a petition in support of arimoclomol with nearly 1,000 signatures from NPC patients, caregivers and physicians.
  - In April 2024, the Company presented new long-term, real-world data from the expanded access program (EAP: NCT04316637) for the treatment of NPC at the *Society for Inherited Metabolic Disorders*.
  - Zevra is preparing for the U.S. commercial launch of arimoclomol, if approved, and intends to leverage the commercial infrastructure that is in place for OLPRUVA to accelerate patient access.
- **KP1077**
  - The Company reported positive topline data from its placebo-controlled, double-blind, proof-of-concept Phase 2 study of KP1077 in patients with IH which showed that it was well tolerated and demonstrated early signs of potentially differentiated and clinically meaningful benefits. The full data package from our completed Phase 2 study will be presented at the upcoming SLEEP 2024 conference in early June.
  - The Company is planning for an end-of-Phase 2 (EOP2) meeting with the FDA in the third quarter.
- **Celiprolol**
  - The Company recently restarted recruitment of the celiprolol Phase 3 trial in VEDS, also known as the DiSCOVER trial, to support patients currently enrolled and to preserve the value of the program while conducting our portfolio review.

Overview of Q1 2024 Financial Results:

Net revenue for Q1 2024 was $3.4 million, compared to net revenue of $3.2 million in Q1 2023. The components of revenue during the first quarter included $2.2 million in net reimbursements from the French EAP for arimoclomol, $1.2 million of royalties and other reimbursements under the AZSTARYS® License Agreement, and de minimis sales of OLPRUVA due to limited shipments to our specialty pharmacy in Q1 2024.

Research and development (R&D) expenses were $12.3 million for Q1 2024, compared to $8.7 million in Q1 2023. The increase in R&D expenses was primarily driven by an increase in spending for the KP1077 Phase 2 clinical trial that has since been completed, as well as ongoing support of the arimoclomol NDA.

Selling, general and administrative (SG&A) expenses were $9.9 million for Q1 2024, compared to $7.2 million in Q1 2023. The period-over-period increase was primarily related to an increase in personnel costs and professional fees associated with the Company's investments in its commercial infrastructure.

Net loss for Q1 2024 was ($16.6) million, or ($0.40) per basic and diluted share, compared to a net loss of ($13.2) million, or ($0.38) per basic and diluted share for Q1 2023.

As of March 31, 2024, total shares of common stock outstanding were 41,850,494, and fully diluted common shares outstanding decreased by 1,451,966 to 56,778,630, which included 5,603,729 shares issuable upon exercise of warrants.

As of March 31, 2024, total cash, cash equivalents, and investments were $52.7 million, a decrease of $15.0 million compared to $67.7 million as of December 31, 2023. The decrease was driven, in part, by increased third-party R&D costs related to the KP1077 clinical development program, reduction in accounts payable and accrued liabilities associated with the Company's acquisition of Acer Therapeutics, and increased SG&A expenses during the period as the Company invested in its commercial infrastructure.

On April 5, 2024, the Company announced the refinancing of our existing debt with up to $100 million in committed capital, which strengthened our balance sheet, simplified our 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, we have refinanced our existing debt of $43 million and added an incremental $14 million in net cash proceeds to the cash balance after fees and discounts. The facility also provides a second tranche of $20 million which can be drawn at the Company's discretion until October 5, 2025, and a third tranche of $20 million which may be drawn upon the approval of arimoclomol, with each of the additional tranches subject to terms and conditions.

Based on our current operating plan, available cash, cash equivalents and investments are expected to extend our cash runway into 2026, subject to continuing compliance with our debt covenants.

- The Company's 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, investments into the incremental commercial activities needed to support the launch of arimoclomol, if approved, and completion of the KP1077 development program for IH.
- The Company's 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.

Conference Call Information

Zevra will host a conference call and live audio webcast today at 8 a.m. ET, to discuss its corporate and financial results for Q1 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 90 days beginning at approximately 9 a.m. ET, on May 8, 2024.

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

- 800-343-5419 (U.S.)
- +1 203-518-9731 (International)
- Conference ID: ZVRAQ124

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). 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 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 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 DiSCOVER trial being conducted under a Special Protocol Assessment (SPA) agreement with the U.S. 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 U.S. and 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.

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 potential benefits of our debt facility, 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, 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, the potential launch or commercialization of any of product candidates or products, personnel needs and growth, including 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, 2023, Zevra’s quarterly report on Form 10-Q for the three months ended March 31, 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 March 31,	
	2024	**2023**
Revenue, net	$ 3,425	$ 3,176

Cost of product revenue (excluding $1,528 in intangible assets amortization for the three months ended March 31, 2024, shown separately below)	175	125
Intangible asset amortization	1,528	-
Operating expenses:		
Research and development	12,277	8,655
Selling, general and administrative	9,931	7,227
Total operating expenses	22,208	15,882
Loss from operations	(20,486)	(12,831)
Other income (expense):		
Interest expense	(735)	(182)
Fair value adjustment related to warrant and CVR liability	3,627	(1,545)
Fair value adjustment related to investments	(27)	196
Interest and other income, net	929	1,042
Total other income (expense)	3,794	(489)
Loss before income taxes	(16,692)	(13,320)
Income tax benefit	70	103
Net loss	$ (16,622)	$ (13,217)
Basic and diluted net loss per share of common stock:		
Net loss	$ (0.40)	$ (0.38)
Weighted average number of shares of common stock outstanding:		
Basic and diluted	41,778,774	34,466,542

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

	March 31, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 42,849	$ 43,049
Securities at fair value	9,868	24,688
Accounts and other receivables	8,305	17,377
Prepaid expenses and other current assets	1,868	1,824
Total current assets	62,890	86,938
Inventories	12,426	9,841
Property and equipment, net	695	736
Operating lease right-of-use assets	1,086	790
Goodwill	4,701	4,701
Intangible assets, net	67,699	69,227
Other long-term assets	1,786	94
Total assets	$ 151,283	$ 172,327
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 22,785	$ 28,403
Line of credit payable	-	37,700
Current portion of operating lease liabilities	583	543
Current portion of discount and rebate liabilities	4,452	4,550
Other current liabilities	2,089	2,524
Total current liabilities	29,909	73,720
Secured promissory note	5,059	5,066
Line of credit payable	37,939	-

Warrant liability	11,535	16,100
Operating lease liabilities, less current portion	705	456
Discount and rebate liabilities, less current portion	8,781	7,663
Other long-term liabilities	8,537	7,458
Total liabilities	102,465	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 March 31, 2024, or December 31, 2023	-	-
Common stock, $0.0001 par value, 250,000,000 shares authorized, 43,426,186 shares issued and 41,850,494 shares outstanding as of March 31, 2024; 43,110,360 shares issued and 41,534,668 shares outstanding as of December 31, 2023	4	4
Additional paid-in capital	476,056	472,664
Treasury stock, at cost	(10,983)	(10,983)
Accumulated deficit	(416,400)	(399,778)
Accumulated other comprehensive income (loss)	141	(43)
Total stockholders' equity	48,818	61,864
Total liabilities and stockholders' equity	$ 151,283	$ 172,327

ZEVRA THERAPEUTICS