Zevra Therapeutics Reports Corporate Updates and Second Quarter 2023 Financial Results

August 14, 2023

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

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

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

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

CELEBRATION, Fla., Aug. 14, 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 June 30, 2023.

Recent Business and Corporate Highlights:

- Continued advancement of the arimoclomol New Drug Application (NDA) for resubmission to the U.S. Food and Drug Administration (FDA):
    - Completed a productive and collaborative pre-submission meeting with the FDA in August 2023, receiving important information that will be used to finalize the NDA for resubmission.
    - NDA package is anticipated to be submitted in Q4 2023.
- Year-to-date net sales of AZSTARYS® surpassed $25 million, triggering the first net sales milestone payment of $5 million, which was earned and recognized in Q2 2023 revenue, and was received after quarter-end; net sales trend supports the potential to earn a second net sales milestone during 2023.
- Continued advancement of a Phase 2 clinical trial evaluating KP1077 as an investigational treatment for IH:
    - Phase 2 IH trial is actively enrolling 48 adult patients with IH at more than 30 sites in the U.S.
    - Interim Phase 2 data for the open-label titration phase of the trial are expected by the end of Q3 2023.
    - Topline Phase 2 data in IH is expected to be reported in the first half of 2024 based on the pace of enrollment.
- Expanded the clinical program for KP1077 by opening an Investigational New Drug Application (IND) for narcolepsy, extending its potential to address multiple rare sleep disorders.
    - Phase 1 clinical trial in healthy volunteers initiated during Q2 2023 and is currently enrolling.
    - By leveraging the data from the IH program and the existing dataset generated as part of the AZSTARYS development program for serdexmethylphenidate (SDX), the sole active pharmaceutical ingredient in KP1077, Zevra can potentially initiate a pivotal Phase 3 trial in narcolepsy sometime next year.
- Strong balance sheet, with $87.4 million in cash, cash equivalents, and investments as of June 30, 2023, which supports our forecasted operating cash runway into 2026.
    - Forecast includes the ongoing reimbursements from the French early access program for arimoclomol, completion of the arimoclomol NDA resubmission, commercial activities to support the launch of arimoclomol, if approved, and completion of the KP1077 development program for IH up to NDA submission.
    - Forecast does not include revenue from arimoclomol after potential FDA approval, or the potential sale of the Priority Review Voucher, which would be received at that time, as well.

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

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

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

Overview of Q2 2023 Financial Results:

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

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

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

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

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

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

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

Conference Call Information:

Zevra will host a conference call and live audio webcast with a slide presentation today at 8:30 a.m. ET, to discuss its corporate and financial results for Q2 2023.

The audio webcast with a slide presentation will be accessible via the Investor Relations section of the Company's website, http://investors.zevra.com/. An archive of the webcast and presentation will be available for 90 days beginning at approximately 9:30 a.m. ET, on August 14, 2023.

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

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

About Niemann-Pick disease type C (NPC):

Niemann-Pick disease type C (NPC) is an ultra-rare and progressive, neurodegenerative lysosomal storage disorder characterized by an inability of the body to transport cholesterol and other lipids within the cell, leading to an accumulation of these substances

in various tissue areas, including brain tissue. The disease is caused by mutations in the NPC1 or NPC2 genes which are responsible for making lysosomal proteins and is an autosomal recessive trait. Both children and adults can be affected by NPC with varying clinical presentations. Those living with NPC lose independence due to physical and cognitive limitations, with key neurological impairments presenting in speech, cognition, swallowing, ambulation, and fine motor skills. Disease progression is irreversible and can be fatal within months or take years to be diagnosed and advance in severity.

About Arimoclomol:

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

About Idiopathic Hypersomnia (IH):

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

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

About Narcolepsy:

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

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

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

About SDX and KP1077:

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

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

KP1077 has been granted Orphan Drug Designation by the U.S. Food and Drug Administration (FDA) for the treatment of IH, and the U.S. Drug Enforcement Agency (DEA) has classified SDX, the sole API in KP1077, as a Schedule IV controlled substance based on evidence suggesting SDX has a lower potential for abuse when compared to d-MPH, a Schedule II controlled substance.

About Zevra Therapeutics:

Zevra Therapeutics is a rare disease company melding science, data, and patient need to create transformational therapies for diseases with limited or no treatment options. With unique, data-driven clinical, regulatory, and commercialization strategies, the Company is overcoming complex drug development challenges to bring much-needed therapies to patients. With both regulatory and clinical stage product candidates, the Company is building its commercial capability to make new therapies available to the rare disease community.

Early access programs are made available by Zevra Therapeutics and its affiliates and are subject to the Company's Early Access Program (EAP) policy as published on its website at zevra.com. Participation in these programs is subject to the laws and regulations of each jurisdiction under which each respective program is operated. Eligibility for participation in any such program is at the treating physician's discretion.

Cautionary Note Concerning Forward Looking Statements:

This press release may contain forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements include all statements that do not relate solely to historical or current facts, and which can be identified by the use of words such as "may," "will," "expect," "project," "estimate," "anticipate," "plan," "believe," "potential," "should," "continue," "could," "intend," "target," "predict," or the negative versions of those words or other comparable words or expressions, although not all forward-looking statements contain these identifying words or expressions. Forward-looking statements are not guarantees of future actions or performance. These forward-looking statements include without limitation statements regarding senior leadership and board member transitions and refreshment, or the timing thereof, and our strategic and product development objectives, the potential sale of the Priority Review Voucher, the promise and potential impact of our preclinical or clinical trial data, including without limitation the initiation, timing and results of any clinical trials or readouts, the content, information used for, timing or results of any IND applications and NDA submissions or resubmissions for arimoclomol, KP1077, or any other product candidates for any specific disease indication or at any dosage, the potential achievement of commercial sales or revenue milestones for AZSTARYS and the timing thereof, the sufficiency of our cash, cash equivalents and investments to fund our operating activities for any specific period of time, and our strategic and product development objectives, including with respect to becoming a leading, commercially-focused rare disease company. Forward-looking statements are based on information currently available to Zevra and its current plans or expectations. They are subject to several known and unknown uncertainties, risks, and other important factors that may cause our actual results, performance, or achievements to be materially different from any future results, performance, or achievements expressed or implied by the forward-looking statements. These and other important factors are described in detail in the “Risk Factors” section of Zevra’s Annual Report on Form 10-K for the year ended December 31, 2022, as updated in Zevra’s Quarterly Report on Form 10-Q for the quarter ended June 30, 2023, and Zevra’s other filings with the Securities and Exchange Commission. While we may elect to update such forward-looking statements at some point in the future, except as required by law, we disclaim any obligation to do so, even if subsequent events cause our views to change. Although we believe the expectations reflected in such forward-looking statements are reasonable, we cannot assure that such expectations will prove correct. These forward-looking statements should not be relied upon as representing our views as of any date after the date of this press release.

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

	Three months ended June 30,		Six months ended June 30,	
	2023	2022	2023	2022
Revenue, net	$ 8,470	$ 1,300	$ 11,349	$ 5,265
Operating expenses:				
Cost of revenue	677	51	802	59
Research and development	7,433	4,795	16,277	7,877
Selling, general and administrative	7,005	3,558	13,839	6,292
Acquired in-process research and development	—	17,663	—	17,663
Total operating expenses	15,115	26,067	30,918	31,891
Loss from operations	(6,645)	(24,767)	(19,569)	(26,626)
Other (expense) income:				
Interest expense	(197)	(36)	(379)	(41)
Fair value adjustment related to derivative and warrant liability	—	32	—	273
Fair value adjustment related to investments	131	(352)	327	(495)
Interest and other income, net	1,553	366	2,593	264
Total other income	1,487	10	2,541	1

Loss before income taxes	(5,158)	(24,757)	(17,028)	(26,625)
Income tax benefit	74	715	177	719
Net loss	$ (5,084)	$ (24,042)	$ (16,851)	$ (25,906)
Basic and diluted net loss per share of common stock:				
Net loss	$ (0.15)	$ (0.70)	$ (0.49)	$ (0.75)
Weighted average number of shares of common stock outstanding:				
Basic and diluted	33,898,233	34,447,206	34,180,818	34,476,737

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

	June, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 66,196	$ 65,466
Securities at fair value	20,696	16,900
Short-term investments - other	479	481
Accounts and other receivables	14,033	8,299
Prepaid expenses and other current assets	2,023	1,877
Total current assets:	103,427	93,023
Inventories	546	671
Property and equipment, net	689	794
Operating lease right-of-use assets	803	988
Long-term investments - other	—	20,000
Other long-term assets	53	53
Total assets:	$ 105,518	$ 115,529
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 10,510	$ 6,169
Current portion of operating lease liabilities	456	480
Current portion of discount and rebate liabilities	6,965	4,655
Other current liabilities	321	422
Total current liabilities:	18,252	11,726
Line of credit payable	12,709	12,800
Operating lease liabilities, less current portion	627	843
Discount and rebate liabilities, less current portion	5,114	4,327
Other long-term liabilities	317	25
Total liabilities:	37,019	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 June 30, 2023, or December 31, 2022	—	—
Common stock, $0.0001 par value, 250,000,000 shares authorized, 35,503,697 shares issued and 33,928,005 shares outstanding as of June 30, 2023; 35,450,257 shares issued and 34,540,304 shares outstanding as of December 31, 2022	3	3
Additional paid-in capital	405,127	401,799
Treasury stock, at cost	(10,983)	(7,536)
Accumulated deficit	(325,423)	(308,572)

Accumulated other comprehensive (loss) income	(225)	113
Total stockholders' equity:	68,499	85,807
Total liabilities and stockholders' equity:	$ 105,518	$ 115,529

ZEVRA THERAPEUTICS