Zevra Therapeutics Reports Corporate Updates and First Quarter 2023 Financial Results

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Christal Mickle, Co-Founder and Chief Development Officer, appointed to serve as interim President and CEO effective June 1, 2023

Conference call and live audio webcast with slide presentation scheduled for today, May 15, 2023, 4:30 p.m. ET

CELEBRATION, Fla., May 15, 2023 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) (Zevra, or the Company, formerly KemPharm, Inc.), a rare disease therapeutics company, today provided corporate updates and reported its financial results for the quarter ended March 31, 2023.

“Zevra is moving at full speed as a company strategically focused on developing and commercializing novel therapeutics designed to address the unmet needs of people with rare diseases,” said Christal Mickle, Chief Development Officer of Zevra Therapeutics. “To this end, we continue to advance arimoclomol, our NDA-stage investigational product candidate for Niemann-Pick disease type C (NPC) as we complete our preparation of the NDA for resubmission. KP1077, our lead clinical candidate for treating idiopathic hypersomnia (IH), is advancing with plans to expand into narcolepsy.”

LaDuane Clifton, Chief Financial Officer, added, “Our pipeline development efforts are supported by a strong financial foundation with growing revenues from AZSTARYS® royalties and potential milestone payments, as well as from the arimoclomol early access program in France. With $95.3 million in cash, cash equivalents, and investments as of March 31, 2023, our cash runway is expected to extend into 2026."

Recent Business and Corporate Highlights:

- Rebranded Zevra Therapeutics, reinforcing the Company's strategic transformation into a commercially focused rare disease company.
- Continued advancement of arimoclomol toward resubmission of its New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) anticipated as early as Q3 2023:
    - Safety and efficacy data from the four-year open-label extension of the Phase 2/3 clinical trial of arimoclomol in NPC were publicly announced and will be included as part of the updated arimoclomol NDA.

- Continued advancement of a Phase 2 clinical trial evaluating KP1077 as a treatment for IH:

    - Interim efficacy and safety data are expected as early as Q3 2023, with the potential for topline Phase 2 data as early as year-end 2023.
    - Data gathered from the Phase 2 trial in IH will support the advancement of KP1077 into a pivotal Phase 3 trial in patients with IH, as well as inform the decision of whether to initiate a future Phase 3 trial in patients with narcolepsy.

- Expanded the clinical program for KP1077 by opening an IND for narcolepsy, extending its potential to address multiple rare sleep disorders.

- Strong balance sheet, with $95.3 million in cash, cash equivalents, and investments as of March 31, 2023, which supports our operating cash runway into 2026.

- Announced Board and Chief Executive Officer (CEO) changes:

    - John B. Bode, Douglas E. Calder, Wendy Dixon and Corey Watton joined the Board of Directors at the 2023 Annual Meeting of Stockholders.
    - Tamara A. Favorito was unanimously appointed Chair of the Board of Directors.
    - Richard W. Pascoe resigned from his role as CEO, effective June 1, 2023.
    - Christal Mickle, Co-Founder and Chief Development Officer, appointed to serve as interim CEO and President effective June 1, 2023.

- The Board will begin a search for both a new CEO and replacement Board members for Matthew Plooster and Joseph B. Saluri who have announced their intention to not stand for re-election at the Company's 2024 Annual Meeting of Stockholders, and to retire from the Board as soon as replacements are found.

Overview of Q1 2023 Financial Results:

Net revenue for Q1 2023 was $2.9 million compared to Q1 2022 net revenue of $4.0 million. Ongoing reimbursements from the French early access program for arimoclomol, AZSTARYS® royalty revenues, and consulting fees primarily drove Q1 2023 net revenue.

Research and development (R&D) expenses were $8.8 million for Q1 2023, compared to $3.1 million in Q1 2022. The increase in R&D expenses were primarily driven by expanding activity for the KP1077 clinical development program and the addition of the arimoclomol program in Q2 2022.

General and administrative (G&A) expenses were $6.8 million for Q1 2023, compared to $2.7 million in Q1 2022. The period-over-period increase was primarily related to the arimoclomol acquisition, which led to an increase in compensation costs, as well as increased professional fees and depreciation and amortization expenses beginning in Q2 2022.

Net loss attributable to common stockholders for Q1 2023 was ($11.8) million, or ($0.34) per basic and diluted share, compared to a net loss attributable to common stockholders of ($1.9) million, or ($0.05) per basic and diluted share for the same period in 2022.

During Q1 2023, the Company repurchased 665,739 shares of the Company's common stock for approximately $3.4 million, at an average price of $5.09 per share. Approximately $39.1 million remains available under the Share Repurchase Program as of March 31, 2023.

As of March 31, 2023, total cash, cash equivalents, and investments were $95.3 million, a decrease of $7.6 million compared to $102.9 million as of December 31, 2022. The decrease was driven, in part, by increased third-party R&D costs related to the KP1077 clinical trial program and the arimoclomol program, increased G&A expenses, and share repurchases during the period.

Based on the Company's current operating forecast, existing cash, cash equivalents, and investments are expected to be sufficient to continue operations into 2026. The operating forecast includes the remaining activities required to resubmit the arimoclomol NDA, funding of the commercial preparation for the potential launch of arimoclomol in the U.S., if approved, completion of the development program for KP1077 in IH, including preparation and submission of the NDA and up to the potential PDUFA date.

As of March 31, 2023, the total shares of common stock outstanding were 33,881,804, and the fully diluted common shares outstanding were 49,307,811, 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 4:30 p.m. ET, to discuss its corporate and financial results for Q1 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 5:30 p.m. ET, on May 15, 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: ZVRAQ123

About Zevra:

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.

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

KP1077 is Zevra's lead clinical candidate being developed to treat idiopathic hypersomnia (IH) and narcolepsy. KP1077 is comprised solely of serdexmethylphenidate (SDX), Zevra's proprietary prodrug of d-methylphenidate ("d-MPH"). The FDA has granted KP1077 orphan drug designation for the treatment of IH, and the U.S. Drug Enforcement Agency (DEA) has classified SDX 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.

Early access programs are made available by Zevra Therapeutics, Inc. 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.

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 senior leadership and board member transitions and refreshment, or the timing thereof, our strategic and product development objectives, 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, timing or results of any Investigational New Drug ("IND") applications and New Drug Application ("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, expected net revenue from the French EAP program, 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 (formerly KemPharm) Annual Report on Form 10-K for the year ended December 31, 2022, as updated in Zevra's (formerly KemPharm) Quarterly Report on Form 10-Q for the quarter ended March 31, 2023, and Zevra's (formerly KemPharm) 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 March 31,	
	2023	**2022**
Revenue, net	$ 2,879	$ 3,965
Operating expenses:		
Cost of revenue	125	8
Research and development	8,844	3,082
General and administrative	6,834	2,734
Total operating expenses:	15,803	5,824
(Loss) income from operations	(12,924)	(1,859)
Other (expense) income:		
Interest expense	(182)	(5)
Fair value adjustment related to derivative and warrant liability	(2)	241
Fair value adjustment related to investments	196	(352)
Interest and other income, net	1,042	107
Total other income (expense):	1,054	(9)
Loss before income taxes:	(11,870)	(1,868)
Income tax benefit	103	4
Net loss:	(11,767)	(1,864)
Net loss attributable to common stockholders:	$ (11,767)	$ (1,864)
Basic and diluted net loss per share of common stock:		
Net loss attributable to common stockholders	$ (0.34)	$ (0.05)
Weighted average number of shares of common stock outstanding:		
Basic and diluted	34,466,542	34,506,597

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

	March 31, 2023	**December 31, 2022**
Assets		
Current assets:		
Cash and cash equivalents	$ 40,181	$ 65,466
Securities at fair value	34,403	16,900
Short-term investments - other	20,700	481
Accounts and other receivables	7,822	8,299
Prepaid expenses and other current assets	1,174	1,877

Total current assets:	104,280	93,023
Inventories	620	671
Property and equipment, net	744	794
Operating lease right-of-use assets	898	988
Long-term investments - other	—	20,000
Other long-term assets	53	53
Total assets:	$ 106,595	$ 115,529
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 10,098	$ 6,169
Current portion of operating lease liabilities	470	480
Current portion of discount and rebate liabilities	4,746	4,655
Other current liabilities	302	422
Total current liabilities:	15,616	11,726
Line of credit payable	12,914	12,800
Derivative and warrant liability	3	1
Operating lease liabilities, less current portion	736	843
Discount and rebate liabilities, less current portion	5,764	4,327
Other long-term liabilities	158	25
Total liabilities:	35,191	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 March 31, 2023 or December 31, 2022	—	—
Common stock, $0.0001 par value, 250,000,000 shares authorized, 35,457,496 shares issued and 33,881,804 shares outstanding as of March 31, 2023; 35,450,257 shares issued and 34,540,304 shares outstanding as of December 31, 2022	3	3
Additional paid-in capital	402,786	401,799
Treasury stock, at cost	(10,983)	(7,536)
Accumulated deficit	(320,339)	(308,572)
Accumulated other comprehensive (loss) income	(63)	113
Total stockholders' equity:	71,404	85,807
Total liabilities and stockholders' equity:	$ 106,595	$ 115,529

ZEVRA THERAPEUTICS