Zevra Therapeutics Reports Fourth Quarter and Fiscal Year 2022 Financial Results and Corporate Updates

March 7, 2023 12:00 PM EST

Conference Call and Live Audio Webcast with Slide Presentation Scheduled for Today, March 7, 2023, 8:30 a.m. ET

Newly rebranded Zevra Therapeutics well positioned to become a commercially focused rare disease company

Arimoclomol NDA resubmission on track to be filed as early as Q3 2023

KP1077 Phase 2 Trial on track to deliver preliminary data as early as Q3 2023 and top-line data as early as year-end 2023

AZSTARYS® royalty revenue growing with one or more revenue milestones expected to be achieved in 2023

Strong balance sheet with cash, cash equivalents and investments of $102.9 million which extends the cash runway into 2026

CELEBRATION, Fla., March 07, 2023 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) ("Zevra," or "the Company," formerly "KemPharm, Inc."), a rare disease therapeutics company, today reported its financial results for the fourth quarter and year ended December 31, 2022.

"Zevra is a company transformed, with a new strategic purpose to deliver novel therapeutics to patients with rare diseases," said Richard W. Pascoe, Chief Executive Officer of Zevra Therapeutics. "The Company is guided by a world-class leadership team and board of directors, and we are in a strong position to execute on multiple anticipated milestones during 2023 that should provide a springboard for continued value creation. The Company's future is more promising than at any time in our history as we are primed to advance arimoclomol, our NDA-stage investigational product candidate for Niemann-Pick disease Type C ("NPC"), and KP1077, our lead clinical candidate for treatment for idiopathic hypersomnia (IH), towards key regulatory and data events during the year. As we move Zevra forward, we will continue to leverage our legacy platform to target internally discovered rare disease product opportunities and to extend exclusivity of our pipeline assets through life cycle management."

Mr. Pascoe continued, "We believe the numerous clinical, regulatory and revenue milestone opportunities anticipated for 2023 and beyond will position Zevra for continued growth as we focus on bringing much-needed therapies to patients with rare diseases. Our strategic and pipeline development efforts are supported by a strong financial foundation with $102.9 million in cash, cash equivalents, and investments as of December 31, 2022, and with a growing revenue stream from AZSTARYS® royalties and anticipated milestone payments, and ongoing revenue from the arimoclomol early access program in France. Based on our current operating forecast, we expect available capital will allow us to pursue our development plans and extend our cash runway into 2026, positioning us to build a fully integrated commercial company."

Recent Business and Corporate Highlights:

- Renamed company Zevra Therapeutics to reflect strategic transformation into a commercially focused rare disease company.
- Announced Board and senior management enhancements to support strategic efforts:
  - Christopher Posner appointed to Board of Directors;
  - Matthew Plooster appointed as Board Chairman;
  - Richard W. Pascoe appointed Chief Executive Officer;
  - Joshua Schafer appointed as Chief Commercial Officer and Executive Vice President of Business Development;
  - Daniel Gallo, Ph.D., appointed as Senior Vice President of Medical Affairs and Advocacy, and
  - Abbi Maher, J.D., named Vice President of Legal Affairs.
- Advancing toward resubmission of arimoclomol New Drug Application ("NDA") to the U.S. Food and Drug Administration ("FDA"):
  - Working to amass and characterize safety and efficacy data from the four-year open-label extension of the Phase 2/3 clinical trial of arimoclomol in NPC to include as part of the updated arimoclomol NDA.
    - Research presented at the recent 19th Annual WORLD*Symposium* ™ 2023 from four-year open-label extension trial suggested that arimoclomol may reduce the

long-term progression of NPC.

- Resubmission of the updated NDA anticipated as early as Q3 2023.

- Initiated Phase 2 clinical trial in December 2022 evaluating KP1077 as a treatment for idiopathic hypersomnia ("IH"):
  - Trial designed as a multi-center, dose-optimizing, double-blind, placebo-controlled, randomized-withdrawal study to evaluate safety and efficacy of KP1077 for the treatment of IH, as well as to assess its impact on the symptoms and severity of "brain fog"
  - Interim efficacy and safety data expected as early as Q3 2023 with potential for topline Phase 2 data by year-end 2023

- Strong balance sheet, with $102.9 million in cash, cash equivalents and investments as of December 31, 2022:
  - Current operating forecast projects cash runway to extend into 2026
  - The potential to realize milestone and royalty revenue from AZSTARYS® as Corium executes its commercialization strategy could provide further capital flexibility and extend the operating cash runway.

Overview of Fourth Quarter (Q4) and Full-Year (FY) 2022 Financial Results:
Net revenue for Q4 2022 was $2.3 million, as compared to Q4 2021 net revenue of $2.6 million. Q4 2022 net revenue was primarily driven by ongoing reimbursements received from the French early access program for arimoclomol, AZSTARYS® royalty revenues and consulting fees.

Research and development (R&D) expenses were $6.4 million for Q4 2022, as compared to $2.8 million in Q4 2021. The period-over-period increase was primarily driven by the KP1077 clinical development program and the addition of the arimoclomol program in Q2 2022.

General and administrative (G&A) expenses were $5.1 million for Q4 2022, as compared to $2.6 million in Q4 2021. The period-over-period increase was primarily related to the arimoclomol acquisition, which led to an increase in compensation costs, including non-cash stock-based compensation expense, as well as increased professional fees and depreciation and amortization expenses beginning in Q2 2022.

Net loss attributable to common stockholders for Q4 2022 was ($9.0) million, or ($0.26) per basic and diluted share, compared to a net loss attributable to common stockholders of ($2.7) million, or ($0.08) per basic and diluted share for the same period in 2021. Net loss for Q4 2022 was driven primarily by R&D expenses of $6.4 million and G&A expenses of $5.1 million, partially offset by net revenue of $2.3 million and other income of $0.2 million.

Net revenue for FY 2022 was $10.5 million, as compared to FY 2021 net revenue of $28.7 million. The period-over-period decrease was primarily attributed to the non-recurrence of the one-time regulatory milestone payments totaling $20 million for the approval of AZSTARYS and the subsequent scheduling of SDX by the U.S. Drug Enforcement Agency as a C-IV scheduled controlled substance.

For FY 2022, R&D expenses were $19.6 million, as compared to $10.2 million in FY 2021. The period-over-period increase was primarily driven by increased investments in the KP1077 clinical development program, and the addition of the arimoclomol program.

G&A expenses were $15.3 million for FY 2022, as compared to $8.7 million in FY 2021. The period-over-period increase was primarily driven by the acquisition of arimoclomol in Q2 2022.

Net loss attributable to common stockholders for FY 2022 was ($41.5) million, or ($1.20) per basic and diluted share, compared to a net loss attributable to common stockholders of ($62.9) million, or ($2.11) per basic and diluted share in 2021. Net loss for FY 2022 was driven primarily by R&D expenses of $19.6 million, G&A expenses of $15.3 million, and a one-time non-cash charge of $17.7 million for acquired in-process R&D related to the arimoclomol asset acquisition in Q2 2022, partially offset by net revenues of $10.5 million. Non-GAAP FY 2022 net loss, excluding the one-time non-cash charge of $17.7M, was ($23.9M), or ($0.69) per basic and diluted share.

As of December 31, 2022, total cash, cash equivalents, and investments were $102.9 million, which was a decrease of $4.5 million compared to $107.4 million as of September 30, 2022. The decrease was driven, in part, by increased third-party R&D costs related to the KP1077 clinical trial program, the arimoclomol program, and investment of working capital related to the collection of accounts receivable due from French EAP reimbursements.

Based on the Company's current operating forecast, which includes approximately $2.0M in net revenue per quarter from the French early access program reimbursements, existing cash, cash equivalents, and investments are expected to be sufficient to continue operations into 2026, including activities required to submit an NDA for KP1077 and its potential PDUFA date, and to fund commercial preparation for the potential launch of arimoclomol in the U.S., if approved.

As of December 31, 2022, the total shares of common stock outstanding was 34,540,304 shares, and the fully diluted common shares outstanding was 47,088,184 shares, which included 4,252,600 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 the fourth quarter and full-year 2022.

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 March 7, 2023.

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

- (800) 343-4849 (U.S.)
- (203) 518-9848 (International)
- Conference ID: ZVRAQ422

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 the treatment of 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").

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: 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 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 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. These 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, 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.
CONSOLIDATED STATEMENTS OF OPERATIONS
(in thousands, except share and per share amounts)

	Year Ended December 31,	
	2022	**2021**
Revenue, net	$ 10,458	$ 28,650
Operating expenses:		
Cost of revenue	343	2,059
Research and development	19,614	10,161
General and administrative	15,343	8,701
Acquired in-process research and development	17,663	-
Total operating expenses	52,963	20,921
(Loss) income from operations	(42,505)	7,729
Other (expense) income:		

Loss on extinguishment of debt	-	(16,096)
Interest expense related to amortization of debt issuance costs and discount	-	(150)
Interest expense	(335)	(226)
Fair value adjustment related to derivative and warrant liability	328	(26)
Fair value adjustment related to investments	(577)	(13)
Interest and other income, net	760	261
Total other income (expense)	176	(16,250)
Loss before income taxes	(42,329)	(8,521)
Income tax benefit (expense)	786	(34)
Net loss	(41,543)	(8,555)
Deemed dividend	-	(54,342)
Net loss attributable to common stockholders	$ (41,543)	$ (62,897)
Basic and diluted net loss per share of common stock:		
Net loss attributable to common stockholders	$ (1.20)	$ (2.11)
Weighted average number of shares of common stock outstanding:		
Basic and diluted	34,488,800	29,766,347

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

	December 31,	
	2022	**2021**
Assets		
Current assets:		
Cash and cash equivalents	$ 65,466	$ 112,346
Securities at fair value	16,900	-
Short-term investments - other	481	-
Accounts and other receivables	8,299	1,528
Prepaid expenses and other current assets	1,877	1,182
Total current assets	93,023	115,056
Inventories	671	-
Property and equipment, net	794	884
Operating lease right-of-use assets	988	1,141
Securities at fair value	-	14,932
Long-term investments - other	20,000	490
Other long-term assets	53	438
Total assets	$ 115,529	$ 132,941
Liabilities and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable and accrued expenses	$ 6,169	$ 3,038
Current portion of operating lease liabilities	480	356
Current portion of discount and rebate liabilities	4,655	-
Other current liabilities	422	836
Total current liabilities	11,726	4,230
Line of credit payable	12,800	-
Derivative and warrant liability	1	330
Operating lease liabilities, less current portion	843	1,232
Discount and rebate liabilities, less current portion	4,327	-
Other long-term liabilities	25	31
Total liabilities	29,722	5,823
Commitments and contingencies (Note H)		
Stockholders' equity:		
Preferred stock:		
Undesignated preferred stock, $0.0001 par value, 10,000,000 shares authorized, no shares issued or outstanding as of December 31, 2022 or December 31, 2021	-	-
Common stock, $0.0001 par value, 250,000,000 shares authorized, 35,450,257 shares issued and 34,540,304 shares outstanding as of December 31, 2022; 35,325,801 shares issued and 35,005,640 shares outstanding as of December 31, 2021	3	4
Additional paid-in capital	401,799	396,957
Treasury stock, at cost	(7,536)	(2,814)

Accumulated deficit	(308,572)	(267,029)
Accumulated other comprehensive income	113	-
Total stockholders' equity	85,807	127,118
Total liabilities and stockholders' equity	$ 115,529	$ 132,941

ZEVRA THERAPEUTICS