Eloxx Pharmaceuticals Reports Second Quarter 2022 Financial and Operating Results and Provides Business Update

August 15, 2022

Topline data from cystic fibrosis (CF) Phase 2 expansion treatment arms evaluating combination with ivacaftor expected by the end of the third quarter of 2022

On track to initiate a proof-of-concept clinical trial with ELX-02 in up to eight Alport syndrome patients with nonsense mutations in the second half of 2022

On track to submit an Investigational New Drug (IND) application for the inhaled delivery of ELX-02 in the fourth quarter of 2022

On track to submit IND application for ZKN-013 for the treatment of recessive dystrophic epidermolysis bullosa (RDEB) patients with nonsense mutations cells in the fourth quarter of 2022

Announces results of ZKN-013 Dose Response Study in APC^{Min} Model that confirmed the results from prior study which concluded in March 2022 and expect to submit an IND application for Familial Adenomatous Polyposis (FAP) in the second half of 2023

Cash and equivalents expected to be sufficient to fund operations into the fourth quarter of 2023

WATERTOWN, Mass., Aug. 15, 2022 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (NASDAQ: ELOX), a leader in ribosomal RNA-targeted genetic therapies for rare diseases, reported its financial results for the three months ended June 30, 2022 and provided a business update.

“We continue to make significant progress advancing our pipeline of novel products for patients with substantial unmet medical needs. With multiple potential value-creating events expected over the next twelve months, including topline data readouts for ELX-02 in both CF and Alport syndrome, we are approaching an exciting and eventful period for the company,” said Sumit Aggarwal, President and Chief Executive Officer of Eloxx. “As further evidence of the potential of our technology platform, today we are pleased to announce results of a repeat study of ZKN-013 in a validated model, which provides further confirmation for the potential of ZKN-013 to treat FAP,” said Sumit Aggarwal, President and CEO of Eloxx Pharmaceuticals. “Based on the continuing positive preclinical results, we now expect to submit an IND to the U.S. Food and Drug Administration in the fourth quarter of 2023 for ZKN-013 for FAP.”

Second Quarter 2022 and Subsequent Highlights

Class 1 Cystic Fibrosis

- Phase 2 clinical trials in CF patients, with expansion arms designed to evaluate the safety of ELX-02 and assess short-term biological activity in patients, remains ongoing. Topline results are expected before the end of the third quarter of 2022 once complete patient data sets are received.
- A head-to-head preclinical study of inhaled (nebulizer-based) delivery of the current subcutaneous formulation of ELX-02 compared to subcutaneous dosing in a CF rodent model has been completed with data analysis ongoing. Human exposure, modeled based on previous animal pharmacokinetic studies, suggests that the lung drug exposure with inhaled delivery of ELX-02 is at least 50-fold greater than with subcutaneous delivery. Higher drug exposure, achievable with inhaled delivery, has been associated with 3 to 6 fold higher activity in CF models. We expect to report topline results before the end of the third quarter of 2022 and remain on track to submit an IND application in the fourth quarter of 2022.

Alport Syndrome

- Eloxx previously announced it has expanded its clinical development pipeline to include the potential treatment with ELX-02 of a subset of Alport Syndrome patients with nonsense mutations in the Collagen Type 4 genes, (COL4A3, COL4A4, and COL4A5). Alport syndrome is a rare genetic disorder characterized by kidney disease with high levels of proteinuria, hearing loss and eye abnormalities.
- Eloxx remains on track to initiate a proof-of-concept clinical trial in up to eight Alport syndrome patients with nonsense mutations in the second half of 2022. Patients will be dosed for two months with a three month follow-up. Trial primary endpoints include safety while secondary endpoints are reduction in proteinuria and induction of COL4A5 protein expression in the kidney. Initial topline results are expected in the first half of 2023.

Recessive Dystrophic Epidermolysis Bullosa and Junctional Epidermolysis Bullosa (JEB)

- Eloxx continues to expect to file an IND application in the fourth quarter of 2022 and be ready to start a First in Human (FIH) Phase 1 study with ZKN-013 after recently completing 28-day non Good Laboratory Practice (GLP) animal studies.

Familial Adenomatous Polyposis

- Eloxx today announced positive results from a repeat study in the APC^{Min} (multiple intestinal neoplasia) model evaluating the potential of ZKN-013 to treat FAP. These results provide further confirmation of ZKN-013's potential to treat FAP, following the previously reported single-dose data in the APC^{Min} model. The APC^{Min} mouse is a translationally validated model for drug development for FAP, a rare inherited disease, with no approved drug therapies, characterized by proliferation of colon polyps. Eloxx is targeting a subset of patients that have nonsense mutations in the Adenomatous Polyposis Coli (APC) gene that is truncated in these patients.
    - In the repeat study, APC^{Min} mice received daily oral doses of ZKN-013 at 50, 25, 12.5 mg/kg body weight for 8 weeks.
    - 100% survival was achieved at the lowest dose of ZKN-013 tested.
    - ZKN-013 significantly lessened anemic conditions, generally considered to be the major cause of animal death associated with APC^{Min} model.
- Eloxx intends to file an IND application to the U.S. Food and Drug Administration for ZKN-013 for the treatment of FAP in the second half of 2023.

Second Quarter 2022 Financial Results

For the three months ended June 30, 2022, we incurred a net loss of $10.6 million, or $0.12 per share, which included $0.7 million in stock-based compensation. For the same period in the prior year, we incurred a net loss of $36.1 million, or $0.54 per share. Since the closing date of Eloxx's acquisition of Zikani Therapeutics, Inc. in April 2021, the results of Zikani's operations have been included in the Eloxx' condensed consolidated financial statements. The second quarter of 2021 included a $22.7 million expense for in process research and development (R&D) related to the Zikani acquisition.

Our R&D expenses were $7.7 million for the three months ended June 30, 2022, which includes $0.3 million in stock-based compensation. For the same period in the prior year, R&D expenses were $5.7 million, which included $0.1 million of stock-based compensation. The increase was primarily related to increases in expenses related to preclinical and development activities, the continued development of ELX-02 related primarily to Cystic Fibrosis Foundation funded activities, facility and overhead expenses and salaries and stock-based compensation.

Our general and administrative (G&A) expenses were $2.6 million for the three months ended June 30, 2022, which includes $0.4 million in stock-based compensation. For the same period in the prior year, G&A expenses were $7.4 million, which included $3.9 million of stock-based compensation. The decrease was primarily related to decreases in salaries and other personnel related costs, stock-based compensation and facility and overhead expenses.

As of June 30, 2022, we had unrestricted cash and cash equivalents of $30.0 million. The Hercules Term Loan Agreement contains customary affirmative and negative covenants, which among others require the Company to maintain at all times a minimum qualified cash balance. As of June 30, 2022, the Company was in compliance with all debt covenants. If our lender does not accelerate our debt upon our failure to comply with the minimum cash covenant, we expect our current cash position will be sufficient to fund our operations into the fourth quarter of 2023.

About Eloxx Pharmaceuticals

Eloxx Pharmaceuticals, Inc. is engaged in the science of ribosome modulation, leveraging its innovative TURBO-ZM™ chemistry technology platform in an effort to develop novel Ribosome Modulating Agents (RMAs) and its library of Eukaryotic Ribosome Selective Glycosides (ERSGs). Eloxx's lead investigational product candidate, ELX-02, is a small molecule drug candidate designed to restore production of full-length functional proteins. The U.S. Food and Drug Administration (FDA) has granted Fast Track designation for ELX-02 for the treatment of CF patients with nonsense mutations. In addition, ELX-02 has also been granted Orphan Drug Designation for the treatment of CF patients with nonsense mutations by the FDA and orphan medicinal product designation by the European Commission. ELX-02 is in clinical development, focusing on cystic fibrosis (US Trial NCT04135495, EU/IL Trial NCT04126473). Eloxx also has preclinical programs focused on select rare diseases, including inherited diseases, cancer caused by nonsense mutations, kidney diseases, including autosomal dominant polycystic kidney disease, as well as rare ocular genetic disorders.

For more information, please visit www.eloxxpharma.com.

Forward-looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements other than statements of present and historical facts contained in this press release, including without limitation, statements regarding our future financial results, our cash runway, the expected timing of trials and results from clinical studies of our product candidates and the potential of our product candidate to treat nonsense mutations are forward-looking statements. Forward-looking statements can be identified by the words "aim," "may," "will," "would," "should," "expect," "explore," "plan," "anticipate," "could," "intend," "target," "project," "contemplate," "believe," "estimate," "predict," "potential," "seeks," or "continue" or the negative of these terms similar expressions, although not all forward-looking statements contain these words.

Forward-looking statements are based on management's current plans, estimates, assumptions and projections based on information currently available to us. Forward-looking statements are subject to known and unknown risks, uncertainties and assumptions, and actual results or outcomes

may differ materially from those expressed or implied in the forward-looking statements due to various important factors, including, but not limited to: our ability to progress any product candidates in preclinical or clinical trials; the uncertainty of clinical trial results and the fact that positive results from preclinical studies are not always indicative of positive clinical results; the scope, rate and progress of our preclinical studies and clinical trials and other research and development activities; the competition for patient enrollment from drug candidates in development; the impact of the global COVID-19 pandemic on our clinical trials, operations, vendors, suppliers, and employees; our ability to obtain the capital necessary to fund our operations; the cost of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights; our ability to obtain financial in the future through product licensing, public or private equity or debt financing or otherwise; general business conditions, regulatory environment, competition and market for our products; and business ability and judgment of personnel, and the availability of qualified personnel and other important factors discussed under the caption “Risk Factors” in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2022, as any such factors may be updated from time to time in our other filings with the SEC, accessible on the SEC’s website at www.sec.gov and the “Financials & Filings” page of our website at https://investors.eloxxpharma.com/financial-information/sec-filings.

All forward-looking statements speak only as of the date of this press release and, except as required by applicable law, we have no obligation to update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

Contact

Investors
John Woolford
john.woolford@westwicke.com
443.213.0506

Media
Laureen Cassidy
laureen@outcomescg.com

ELOXX PHARMACEUTICALS, INC. AND SUBSIDIARIES
UNAUDITED CONDENSED CONSOLIDATED BALANCE SHEETS
(Amounts in thousands, except share and per share data)

	June 30, 2022	December 31, 2021
ASSETS		
Current assets:		
Cash and cash equivalents	$ 30,048	$ 42,268
Restricted cash	263	299
Prepaid expenses and other current assets	1,281	913
Total current assets	31,592	43,480
Property and equipment, net	190	216
Operating lease right-of-use assets	1,159	1,443
Total assets	**$ 32,941**	**$ 45,139**
LIABILITIES AND STOCKHOLDERS’ EQUITY		
Current liabilities:		
Accounts payable	$ 1,934	$ 1,379
Accrued expenses	4,870	4,196
Current portion of long-term debt	1,125	-
Advances from collaboration partners	10,723	3,723
Derivative liabilities	166	-
Current portion of operating lease liabilities	686	657
Total current liabilities	19,504	9,955
Long-term debt, net of current portion	11,129	11,996
Operating lease liabilities	496	804
Total liabilities	31,129	22,755
Total stockholders’ equity	1,812	22,384
Total liabilities and stockholders' equity	**$ 32,941**	**$ 45,139**

ELOXX PHARMACEUTICALS, INC. AND SUBSIDIARIES
UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS
(Amounts in thousands, except share and per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Operating expenses:				
Research and development	$ 7,651	$ 5,704	$ 15,550	$ 9,777
General and administrative	2,645	7,355	5,699	11,696
In process research and development	—	22,670	—	22,670
Total operating expenses	10,296	35,729	21,249	44,143
Loss from operations	(10,296)	(35,729)	(21,249)	(44,143)
Other expense, net	322	329	989	609
Net loss	$ (10,618)	$ (36,058)	$ (22,238)	$ (44,752)
Net loss per share, basic and diluted	$ (0.12)	$ (0.54)	$ (0.26)	$ (0.84)
Weighted average number of shares of common stock used in computing net loss per share, basic and diluted	86,654,120	66,389,865	86,652,587	53,357,401

Source: Eloxx Pharmaceuticals

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