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

November 10, 2022

Started proof-of-concept Phase 2 clinical trial with ELX-02 in up to eight Alport syndrome patients with nonsense mutations; topline results expected in the first half of 2023

On track to submit IND application for ZKN-013 for the treatment of recessive dystrophic epidermolysis bullosa (RDEB) patients with nonsense mutations cells by the end of 2022 or early 2023

WATERTOWN, Mass., Nov. 10, 2022 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (NASDAQ: ELOX), a leader in ribosomal RNA-targeted genetic therapies for rare diseases, today reported its financial results for the three months ended September 30, 2022 and provided a business update.

“With the recent start of our Phase 2 proof-of-concept trial for ELX-02 for the treatment of Alport Syndrome, we are on track to deliver topline results for the program in the first half of 2023. Alport patients with nonsense mutations have significantly worse clinical outcomes than other Alport patients and have no disease modifying treatment options,” said Sumit Aggarwal, President and Chief Executive Officer of Eloxx. “We have also made significant advancements in our Ribosome Modulating Agents programs, approaching clinical development for ZKN-013 for the treatment of RDEB and generating encouraging preclinical data in multiple indications.”

Third Quarter 2022 and Subsequent Highlights

Alport Syndrome

- Eloxx started a proof-of-concept Phase 2 open-label clinical trial (NCT05448755) in up to eight 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. 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.
- Eloxx presented a poster highlighting the activity of ELX-02 across a range of COL4A5 mutations in preclinical models at the American Society of Nephrology (ASN) Kidney Week 2022 Conference in early November 2022.

Recessive Dystrophic Epidermolysis Bullosa (RDEB) and Junctional Epidermolysis Bullosa (JEB)

- Eloxx continues to expect to file an Investigational New Drug (IND) application to start a First in Human (FIH) Phase 1 study of ZKN-013 by the end of 2022 or early 2023.

Familial Adenomatous Polyposis (FAP)

- Eloxx continues to evaluate the potential of ZKN-013 to treat FAP, targeting a subset of patients that have nonsense mutations in the Adenomatous Polyposis Coli (APC) gene that is truncated in these patients. Eloxx is currently preparing an IND application.

Class 1 Cystic Fibrosis (CF)

- A Phase 2 combination study data readout of ELX-02 with ivacaftor in CF patients with nonsense mutations did not meet efficacy endpoints but did confirm drug activity for ELX-02 and demonstrated need for higher drug exposure in lung to generate therapeutic benefit. Eloxx aims to complete IND enabling studies for inhaled ELX-02. The path forward in CF is to be determined together with the CF Foundation.

TURBO-ZM™ Platform

- Recent advancements have demonstrated the potential of Eloxx’s TURBO-ZM chemistry technology platform to develop novel Ribosome Modulating Agents (RMAs).
    - Two posters were presented at the 34th EORTC-NCI-AACR Symposium in late October 2022 demonstrating the potential of RMAs to treat MYC-driven cancers. Preclinical data demonstrated activity against subtypes of colorectal cancer (ZKN-157) and small cell lung cancer (ZKN-217).

- A poster was presented at the International Symposium on Medicinal Chemistry in early September. A phenotypic screen of our RMA library identified showed selective hits for cancer cell lines and demonstrated responsive structure-activity relationship.

- In Eloxx's program to identify RMAs for further development in the treatment of Class 1 CF, which is supported by the CF Foundation, screening has identified approximately 50 oral RMAs with promising functional CFTR activity.

Presentations are available on Eloxx's website at link.

Third Quarter 2022 Financial Results

For the three months ended September 30, 2022, we incurred a net loss of $7.5 million, or $0.09 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 $9.9 million, or $0.11 per share, which included $2.2 million in stock-based compensation.

Our R&D expenses were $4.9 million for the three months ended September 30, 2022, which includes $0.3 million in stock-based compensation. For the same period in the prior year, R&D expenses were $5.2 million, which included $0.4 million of stock based compensation. The decrease was related to a decrease in clinical trial expenses related primarily to CF Foundation funded activities, and a decrease in salaries and other personnel costs.

Our general and administrative (G&A) expenses were $2.3 million for the three months ended September 30, 2022, which includes $0.4 million in stock-based compensation. For the same period in the prior year, G&A expenses were $5.0 million, which included $1.9 million of stock-based compensation. The decrease was primarily related to a decrease in salaries and other personal related costs due to reduced headcount, a decrease in expenses attributable to professional and consulting fees, and a decrease in stock-based compensation expense due primarily to option modifications of former executives in 2021.

As of September 30, 2022, we had unrestricted cash and cash equivalents of $24.6 million. The Hercules Term Loan Agreement contains customary affirmative and negative covenants, which among others require us to maintain a minimum qualified cash balance. As of September 30, 2022, we were in compliance with all debt covenants. Our expectation is that our current cash position will be sufficient to fund our operations into the fourth quarter of 2023 assuming that we can comply with our lender's minimum cash covenant by either raising additional capital or working with our lender to restructure the covenants.

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 and ability to comply with the covenants in our debt agreement, 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 September 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.

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

	September 30, 2022	December 31, 2021
ASSETS		
Current assets:		
Cash and cash equivalents	$ 24,554	$ 42,268
Restricted cash	263	299
Prepaid expenses and other current assets	854	913
Total current assets	25,671	43,480
Property and equipment, net	215	216
Operating lease right-of-use assets	993	1,443
Total assets	**$ 26,879**	**$ 45,139**
LIABILITIES AND STOCKHOLDERS' (DEFICIT) EQUITY		
Current liabilities:		
Accounts payable	$ 1,595	$ 1,379
Accrued expenses	4,518	4,196
Current portion of long-term debt	2,530	-
Advances from collaboration partners	12,223	3,723
Derivative liabilities	116	-
Current portion of operating lease liabilities	698	657
Total current liabilities	21,680	9,955
Long-term debt, net of current portion	9,863	11,996
Operating lease liabilities	318	804
Total liabilities	31,861	22,755
Total stockholders' (deficit) equity	(4,982)	22,384
Total liabilities and stockholders' (deficit) equity	**$ 26,879**	**$ 45,139**

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
Operating expenses:				
Research and development	$ 4,880	$ 5,210	$ 20,430	$ 14,987
General and administrative	2,262	5,035	7,961	16,731
In process research and development	—	—	—	22,670
Total operating expenses	7,142	10,245	28,391	54,388
Loss from operations	(7,142)	(10,245)	(28,391)	(54,388)
Other expense (income), net	366	(360)	1,355	249
Net loss	$ (7,508)	$ (9,885)	$ (29,746)	$ (54,637)
Net loss per share, basic and diluted	$ (0.09)	$ (0.11)	$ (0.34)	$ (0.85)

Weighted average number of shares of common stock used in computing net loss per share, basic and diluted	86,656,221	86,208,754	86,653,811	64,428,187

Source: Eloxx Pharmaceuticals

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