Eloxx Pharmaceuticals Reports First Quarter 2023 Financial and Operating Results and Provides Business Update

May 15, 2023

Topline results to include biopsy results for the Phase 2 clinical study evaluating ELX-02 for the potential treatment of Alport syndrome expected in first half of 2023; meaningful protein reduction has been observed in one patient to date

Received U.S. Food and Drug Administration (FDA) clearance to begin a single ascending dose (SAD) clinical trial for ZKN-013 for the potential treatment of recessive Dystrophic Epidermolysis Bullosa (RDEB) with nonsense mutations

WATERTOWN, Mass., May 15, 2023 (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 March 31, 2023 and provided a business update.

“We are approaching a significant milestone for Eloxx, with topline data, including kidney biopsy results, expected for ELX-02 in Alport syndrome in the coming weeks,” said Sumit Aggarwal, President and Chief Executive Officer of Eloxx. “In addition, ZKN-013, our lead TURBO-ZM™ based molecule, is the first program developed from hit to lead and our excitement for the program continues to grow. Eloxx has additional promising TURBO-ZM based discovery programs in cystic fibrosis (fully funded by the Cystic Fibrosis Foundation) and in certain cMYC-overexpressing cancers.”

First Quarter 2023 and Subsequent Highlights

Alport Syndrome

- Alport syndrome is a rare genetic disorder characterized by kidney disease with high levels of proteinuria, hearing loss and eye abnormalities.
- Eloxx has dosed three patients with ELX-02 in the ongoing proof-of-concept Phase 2 open-label clinical trial (NCT05448755). Meaningful protein reduction has been observed in one patient to date. We will be evaluating expression of COL IV protein in these three patients at the end of dosing and measuring proteinuria every two weeks. Topline results are expected in the first half of 2023.

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

- In May 2023, Eloxx announced that the U.S. Food and Drug Administration (FDA) has cleared the Company’s Investigational New Drug application to initiate a single ascending dose (SAD) clinical trial in healthy volunteers for ZKN-013 for the potential treatment of RDEB with nonsense mutations. RDEB is a rare skin disease characterized by mutations in Collagen 7 gene.
- Further SAD and multiple ascending dose (MAD) testing is expected to be conducted following the completion of the planned dose cohorts in the SAD study and discussion with the FDA. The MAD testing could potentially include RDEB patients given the strong benefit/risk in patients cited by the FDA.
- Recent preclinical results demonstrated read-through activity of ZKN-013 in multiple COL7 genotypes across multiple RDEB patient derived fibroblasts and keratinocytes. In this trial, read-through activity resulted in up to an 18-fold increase in full-length COL VII protein levels. Prolonged treatment with ZKN-013 was shown to further increased COL VII protein levels. Functionality of the restored full-length COL VII protein was observed. These results have been accepted for presentation at an upcoming medical conference.

Familial Adenomatous Polyposis (FAP)

- Eloxx also plans to develop 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.
- In January 2023, Eloxx published positive results from a study in the APC^{Min} (multiple intestinal neoplasia) model evaluating the potential of ZKN-013 to treat FAP. The APC^{Min} mouse is a translationally validated model for drug development for FAP. In the APC^{Min} model, treatment with ZKN-013 demonstrated a decrease in intestinal polyps and adenomas, resulting in increased survival. The publication also included *in vitro* and *in vivo* results demonstrating that ZKN-013 inhibited the growth of human colon carcinoma cells with APC nonsense mutations, and promoted read through of premature stop codons in the APC gene, leading to functional restoration of full-length APC protein.

First Quarter 2023 Financial Results

For the three months ended March 31, 2023, we incurred a net loss of $6.2 million, or $2.88 per share, which included $0.6 million in stock-based compensation. For the same period in the prior year, we incurred a net loss of $11.6 million, or $5.36 per share, which included $0.9 million in stock-based compensation.

R&D expenses were $3.5 million for the three months ended March 31, 2023, which included $0.3 million in stock-based compensation. For the same period in the prior year, R&D expenses were $7.9 million, which included $0.4 million of stock-based compensation. The decrease was primarily related to a decrease in clinical trial expenses for activities related to inhaled delivery of ELX-02 in cystic fibrosis and a decrease in clinical trial expenses related to a decrease in Cystic Fibrosis Foundation funded activities.

General and administrative (G&A) expenses were $2.0 million for the three months ended March 31, 2023, which included $0.3 million in stock-based compensation. For the same period in the prior year, G&A expenses were $3.1 million, which included $0.6 million of stock-based compensation. The decrease was primarily related to a decrease in salaries and other personal related costs, a decrease in expenses attributable to professional and consulting fees, and a decrease in facility and overhead expenses.

As of March 31, 2023, we had unrestricted cash and cash equivalents of $4.9 million. In March 2023, we amended the terms of the Hercules Term Loan Agreement and repaid $7.5 million of the outstanding principal. The minimum qualified cash balance requirement was reduced to $2.25 million and the interest only payment period was extended to September 1, 2023, at which time the Company will be required to begin making principal payments. Assuming that we initiate Phase 3 clinical trial activities in the third quarter of 2023, our expectation is that our current cash position and assuming maintaining compliance with our debt covenants, will be sufficient to fund our operations into the third 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. ELX-02 is in Phase 2 clinical development for the treatment of Alport syndrome in patients with nonsense mutations. 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 cash runway and our ability to comply with the covenants in our debt agreement, the expected timing of and results from trials 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; our ability to meet the continued listing requirements of the Nasdaq Capital Market; 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 quarterly period ended March 31, 2023, 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/financials-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 CONSOLIDATED STATEMENTS OF OPERATIONS
(Amounts in thousands, except share and per share data)

	Three Months Ended March 31,	
	2023	2022
Operating expenses:		
Research and development	$ 3,488	$ 7,899
General and administrative	1,995	3,054
Total operating expenses	5,483	10,953
Loss from operations	(5,483)	(10,953)
Other expense, net	747	667
Net loss	**$ (6,230)**	**$ (11,620)**
Net loss per share, basic and diluted	**$ (2.88)**	**$ (5.36)**
Weighted average number of shares of common stock used in computing net loss per share, basic and diluted	2,166,356	2,166,275

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

	March 31, 2023	December 31, 2022
ASSETS		
Current assets:		
Cash and cash equivalents	$ 4,904	$ 19,207
Restricted cash	261	261
Prepaid expenses and other current assets	1,238	661
Total current assets	6,403	20,129
Property and equipment, net	143	169
Operating lease right-of-use asset	654	825
Total assets	**$ 7,200**	**$ 21,123**
LIABILITIES AND STOCKHOLDERS' DEFICIT		
Current liabilities:		
Accounts payable	$ 2,434	$ 3,020
Accrued expenses	2,230	2,799
Current portion of long-term debt	1,536	3,980
Advances from collaboration partners	12,535	12,535
Current portion of operating lease liability	667	712
Derivative liabilities	58	45
Total current liabilities	19,460	23,091
Long-term debt, net of current portion	4,027	8,557
Operating lease liability	6	135
Total liabilities	23,493	31,783
Total stockholders' deficit:	(16,293)	(10,660)
Total liabilities and stockholders' deficit	**$ 7,200**	**$ 21,123**

SOURCE: Eloxx Pharmaceuticals, Inc.

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