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

November 13, 2023

Kidney morphology improved in all three patients with protein re-expression consistent with disease regression in its Phase 2 open-label clinical trial of ELX-02 for the treatment of Alport Syndrome

ELX-02 improved podocyte foot process effacement in all three treated patients by an average of 60% based on blinded biopsy analysis by NIPOKA GmbH

Clinical data from Phase 2 study of ELX-02 for Alport Syndrome included in presentations at the American Society of Nephrology (ASN) Kidney Week 2023

Significant progress toward completion of a strategic partnership for ZKN-013

WATERTOWN, Mass., Nov. 13, 2023 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (OTC: ELOX), a leader in ribosomal RNA-targeted genetic therapies for rare diseases, today reported its financial results for the three months ended September 30, 2023, and provided a business update.

- Completed Phase 2 open label trial of ELX-02 for the treatment of Alport Syndrome patients with nonsense mutations.
  - Analysis of biopsy samples provided unequivocal clinical evidence of both morphology and ELX-02's disease modifying potential.
  - Collagen Alpha 5 protein expression observed in the glomerular basement membrane post treatment in all patients.
- Data from the Phase 2 study of ELX-02 for Alport Syndrome was included in two presentations at the American Society of Nephrology (ASN) Kidney Week 2023.
- Significant progress towards completing a strategic partnership for ZKN-013
- Raised additional cash in ongoing efforts to strengthen balance sheet.

"We are excited about the recent progress across our pipeline and remain committed to advancing our programs for the benefit of our patients and stakeholders," said Sumit Aggarwal, President and Chief Executive Officer of Eloxx.

Third Quarter 2023 and Subsequent Highlights

ELX-02 in Nonsense Mutation Alport Syndrome

- Confirmed that all three patients showed an improvement in morphology and proteinuria in its proof-of-concept Phase 2 open-label clinical trial (NCT05448755) of ELX-02 for the treatment of Alport Syndrome after eight weeks of treatment.
  - All patients had Autosomal Recessive Alport Syndrome with a nonsense mutation on one allele. No collagen 4 alpha 5 was expressed in the glomerular basement membrane (GBM) at baseline.
  - Collagen 4 Alpha 5 expression in the GBM post-treatment was reported post-treatment based on immunofluorescence staining of patient biopsies
  - ELX-02 increased the filtration slit density (FSD) by an average of 60% as compared to baseline levels, consistent with reduction of podocyte foot process effacement.
    - Biopsies were analyzed on a blinded basis by NIPOKA GmbH. FSD was estimated for 15 to 20 glomeruli per sample.
    - Data supporting FSD as a precise histopathological estimator of podocyte health and its inverse correlation with proteinuria was presented at the ASN meeting on November 2, 2023.
  - Visual assessment of improvement in foot process effacement in Transmission Electron Micrography (TEM) images was independently confirmed by highly regarded renal pathologist and TEM expert.
  - All three treated patients had reduced proteinuria variability and two patients had a reduction in proteinuria compared to baseline during or in 2 months post treatment consistent with improvement in kidney morphology.
  - ELX-02 was well-tolerated in the study with no discontinuations.

- Data from the Phase 2 study of ELX-02 for Alport Syndrome was included in two presentations at the ASN Kidney Week 2023.

ZKN-013 in nonsense mutation Recessive Dystrophic Epidermolysis Bullosa (RDEB), Junctional Epidermolysis Bullosa (JEB) and Familial Adenomatous Polyposis

- Eloxx made significant progress in completing a strategic transaction for ZKN-013.
    - Following FDA clearance to begin a single ascending dose trial in healthy volunteers for ZKN-013 for the treatment of recessive RDEB and JEB, Eloxx received significant strategic interest in ZKN-013.
    - Should these discussions lead to a transaction, it will allow Eloxx to remain focused on fully maximizing the potential of ELX-02 in rare kidney diseases and continue funded discovery efforts on our TURBO-ZM platform.

Third Quarter 2023 Financial Results

For the three months ended September 30, 2023, we incurred a net loss of $3.6 million, or $1.31 per share, which included $0.4 million in stock-based compensation. For the same period in the prior year, we incurred a net loss of $7.5 million, or $3.47 per share, which included $0.7 million in stock-based compensation.

R&D expenses were $1.3 million for the three months ended September 30, 2023, which included $0.1 million in stock-based compensation. For the same period in the prior year, R&D expenses were $4.9 million, which included $0.3 million of stock-based compensation. The decrease was primarily related to a decrease in expenses related to subcontractors, advisors, and laboratory supplies in connection with preclinical research and development activities related to inhaled delivery of ELX-02 in CF, a decrease in CFF funded activities, a decrease in salaries and other personnel costs, a decrease related to stock-based compensation, and a decrease in facility and overhead expenses.

General and administrative (G&A) expenses were $2.4 million for the three months ended September 30, 2023, which included $0.4 million in stock-based compensation. For the same period in the prior year, G&A expenses were $2.3 million, which included $0.4 million of stock-based compensation. The increase was primarily related to a $0.2 million increase in expenses attributable to professional and consulting fees, offset by a decrease of $0.1 million related to facility and overhead expenses.

As of September 30, 2023, we had unrestricted cash and cash equivalents of $4.8 million. During the quarter, we raised gross proceeds of $2.0 million from the sale of shares of common stock, pre-funded warrants and warrants. Additionally, we sold shares of common stock through our ATM program during the quarter for gross proceeds of $1.8 million. Eloxx remains focused on its liquidity position and raising additional capital in the near term in order to fund its operating plan through the end of 2023 and beyond. Assuming that we initiate Phase 3 clinical trial activities when sufficient funding allows, and that we maintain compliance with our debt covenants, we believe that our current cash position will be sufficient to fund our operations through the end of the fourth quarter of 2023.

As previously reported, we received a delisting determination from the Listing Qualifications Department of the Nasdaq Stock Market LLC as the company did not regain compliance with the Nasdaq Listing Rule 5550(b)(2), which requires a listed company to have at least $35 million in market value of listed securities. Effective October 16, 2023, trading of our common stock was suspended on the Nasdaq Capital Market and began trading on the OTC Pink Marketplace under the symbol “ELOX.” On October 26, 2023, we requested a review of the Nasdaq delisting determination by the Nasdaq Listing Council. Trading of or common stock on the Nasdaq Capital Market will remain suspended pending a decision by the Nasdaq Listing Council.

About Nonsense Mutation Alport Syndrome

Nonsense Mutation Alport syndrome is a rare Type IV Collagenopathy characterized by mutations in the genes (COL4A3, COL4A4, and COL4A5) that result in a less than full length (truncated) Type 4 Collagen. This disorder mostly affects children with a median age at diagnosis of 9 to 20 years. It is characterized by rapid and progressive damage to the kidneys, ear and eyes, starting with worsening of kidney morphology to proteinuria and finally kidney failure, hearing loss and eye abnormalities. It is estimated that there are approximately 7,500 patients in the US and 20,000 patients in US, Europe, Japan and China with Nonsense Mutation Alport Syndrome. These patients have no approved treatment options.

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 to fund our operating plan, our plans to raise additional capital, our ability to comply with the covenants in our debt agreement, the expected timing of and results from trials of our product candidates, the potential of our product candidate to treat nonsense mutations, the outcome of the Nasdaq Listing Council’s review of the delisting determination; the strategic partnership for the clinical development of ZKN-013 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 obtain the capital necessary to fund our operations; our ability to

regain and maintain compliance with the continued listing requirements of the Nasdaq Capital Market; 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;; 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 quarterly period ended September 30, 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 CONDENSED CONSOLIDATED BALANCE SHEETS
(Amounts in thousands, except share and per share data)

	September 30, 2023	December 31, 2022
ASSETS		
Current assets:		
Cash and cash equivalents	$ 4,775	$ 19,207
Restricted cash	219	261
Prepaid expenses and other current assets	635	661
Total current assets	5,629	20,129
Property and equipment, net	107	169
Operating lease right-of-use asset	306	825
Total assets	**$ 6,042**	**$ 21,123**
LIABILITIES AND STOCKHOLDERS’ DEFICIT		
Current liabilities:		
Accounts payable	$ 3,134	$ 3,020
Accrued expenses	2,442	2,799
Current portion of long-term debt	2,819	3,980
Advances from collaboration partners	12,966	12,535
Warrant liabilities	1,695	—
Current portion of operating lease liability	315	712
Derivative liabilities	95	45
Total current liabilities	23,466	23,091
Long-term debt, net of current portion	2,621	8,557
Operating lease liability	3	135
Total liabilities	26,090	31,783
Total stockholders’ deficit:	(20,048)	(10,660)
Total liabilities and stockholders’ deficit	**$ 6,042**	**$ 21,123**

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,	
	2023	**2022**	**2023**	**2022**
Operating expenses:				
Research and development	$ 1,250	$ 4,880	$ 7,076	$ 20,430
General and administrative	2,387	2,262	6,184	7,961
Total operating expenses	3,637	7,142	13,260	28,391
Loss from operations	(3,637)	(7,142)	(13,260)	(28,391)
Other (income) expense, net	(45)	366	903	1,355
Net loss	**$ (3,592)**	**$ (7,508)**	**$ (14,163)**	**$ (29,746)**
Net loss per share, basic and diluted	**$ (1.31)**	**$ (3.47)**	**$ (5.96)**	**$ (13.73)**
Weighted average number of shares of common stock used in computing net loss per share, basic and diluted	2,747,687	2,166,404	2,377,599	2,166,344

Source: Eloxx Pharmaceuticals