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

August 14, 2023

Announced today that all 3 patients (100% response rate) treated with ELX-02 showed an improvement in podocyte foot process effacement post-treatment in kidney biopsies assessed by electron microscopy, demonstrating the disease-modifying effect of ELX-02

Announced achievement of remission in one patient in Phase 2 clinical study of ELX-02 for the treatment of Alport syndrome and decision to advance ELX-02 into a pivotal trial in Alport syndrome

Highlighted significant unmet need in the treatment of Alport syndrome and additional positive data from Phase 2 clinical study evaluating ELX-02 in KOL event

Received Food and Drug Administration (FDA) Investigational New Drug clearance to begin single ascending dose (SAD) study of ZKN-013; first subject intended to be dosed by the end of 2023

Raised $3.4M in net proceeds through "at-the-market" equity offering program as of August 11, 2023

Nasdaq granted Eloxx's request for an extension to regain compliance with the Market Value of Listed Securities continued listing requirement

WATERTOWN, Mass., Aug. 14, 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 June 30, 2023, and provided a business update.

"This is a transformative time at Eloxx. With today's confirmation of the disease modifying potential of ELX-02 in all three patient biopsies from the Alport syndrome trial, we look forward to advancing to a pivotal trial of ELX-02 for the treatment of Alport syndrome," said Sumit Aggarwal, President and Chief Executive Officer of Eloxx. "We also plan to initiate a clinical study for our lead TURBO-ZM™ based molecule, ZKN-013, for the potential treatment of recessive dystrophic epidermolysis bullosa (RDEB) with first patient dosing expected by the end of 2023."

Second Quarter 2023 and Subsequent Highlights

Alport Syndrome

- Eloxx intends to advance ELX-02 into pivotal trial for the treatment of Alport syndrome with nonsense mutations, pending obtaining the necessary capital. Alport syndrome is a rare genetic kidney disorder caused by mutations in COL4A3/4/5 genes, characterized by podocyte injury and impaired kidney filter function leading to proteinuria.
- In a separate press release, Eloxx today announced positive biopsy results from 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 three patients (100% response rate) treated with ELX-02 showed an improvement in podocyte foot process effacement post-treatment in kidney biopsies assessed by electron microscopy demonstrating the disease modifying effect of ELX-02 and potential for improvement in proteinuria with longer duration of treatment.
    - Podocytes are specialized cells that bind to the glomerular basement membrane and form finger-like extensions called foot processes that enable efficient ultrafiltration. Podocyte injury leads to the effacement (loss) of podocyte foot processes and proteinuria in nearly all cases of Alport syndrome.
    - In two patients, widespread foot process effacement was improved to segmental foot process effacement. In the third patient, moderate to severe foot process effacement was improved to moderate only.
    - Eloxx previously announced achievement of remission in one patient. One month after the end of treatment, the patient demonstrated a rapid increase in Urine Protein to Creatinine (UPCR), providing additional evidence of drug activity.
    - ELX-02 was well-tolerated in the study, with no discontinuations to date.
- An IND application for ELX-02 is expected to be submitted to the FDA in the third quarter of 2023.
- Additional data recently announced regarding the efficacy of ELX-02 in its cystic fibrosis study bolsters the strength of results in Phase 2 Alport syndrome trial, further supporting Eloxx's decision to advance into a pivotal trial in Alport syndrome.

- Alport syndrome RaDaR natural history data presented at the 60th European Renal Association Congress indicates that Alport syndrome patients with autosomal recessive COL4A4 mutations have severest disease, with a more rapid progression to kidney failure. The patient that achieved remission in Eloxx Phase 2 trial had autosomal recessive COL4A4 nonsense mutation resulting in a truncated protein.

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

- In May 2023, Eloxx announced that the FDA has cleared the IND application to initiate a 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 the Collagen 7 gene. Eloxx plans to initiate the Phase 1 SAD clinical study, assuming sufficient funding, with the first subject expected to be dosed by the end of 2023.
- Further SAD and multiple ascending dose (MAD) testing are 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 FDA.
- 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, assuming sufficient funding, 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.

TURBO-ZM Platform

- *Cancer Research Communications* published “A Novel Class of Ribosome Modulating Agents Exploits Cancer Ribosome Heterogeneity to Selectively Target the CMS2 Subtype of Colorectal Cancer.” The publication demonstrates the of potential the TURBO-ZM chemistry technology platform to develop novel Ribosome Modulating Agents (RMAs) and details preclinical data that demonstrate activity for ZKN-157 against subtypes of colorectal cancer.
    - Results suggest that MYC-overexpressing cancers can be targeted by exploiting ribosome heterogeneity in cancer, as preclinical data has demonstrated the activity of ZKN-157 against subtypes of colorectal cancer. This research potentially provides opportunities to selectively target MYC-driven cancers with a novel mechanism and possible synergy with existing cancer therapies.

Second Quarter 2023 Financial Results

For the three months ended June 30, 2023, we incurred a net loss of $4.3 million, or $1.96 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 $10.6 million, or $4.90 per share, which included $0.7 million in stock-based compensation.

R&D expenses were $2.3 million for the three months ended June 30, 2023, which included $0.3 million in stock-based compensation. For the same period in the prior year, R&D expenses were $7.7 million, which included $0.3 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 $1.8 million for the three months ended June 30, 2023, which included $0.3 million in stock-based compensation. For the same period in the prior year, G&A expenses were $2.6 million, which included $0.4 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 June 30, 2023, we had unrestricted cash and cash equivalents of $4.3 million, and subsequent to quarter end, as of August 11, 2023, the Company raised an additional $1.7 million in gross proceeds through our previously established “at-the-market” equity offering program (the “ATM Program”). Eloxx remains focused on its liquidity position and is committed to 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, which is subject to sufficient funding, in the third quarter of 2023 and that we maintain compliance with our debt covenants, we believe that our current cash position will be sufficient to fund our operations into the fourth quarter of 2023.

Eloxx received notice from the Nasdaq Listing Qualifications Panel (the “Hearings Panel”) of The Nasdaq Stock Market LLC (“Nasdaq”) that it has determined to extend the previously granted extension from July 30, 2023 until October 9, 2023 to allow the Company time to regain compliance with Listing Rule 5550(b)(2), which requires a listed company to have at least $35 million in market value of listed securities in order to qualify for continued listing on the Nasdaq Capital Market.

About Alport syndrome

Alport syndrome is a genetic disorder characterized by kidney disease with high levels of proteinuria, hearing loss and eye abnormalities caused by mutations in the genes (COL4A3, COL4A4, and COL4A5) needed for production of type 4 collagen. Approximately 6% to 7% of Alport syndrome patients, or approximately 9,400 to 12,750 individuals, are estimated to have nonsense mutations. These patients have significantly worse clinical outcomes than other patients with Alport syndrome and have no disease modifying 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, 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 regain and maintain compliance with 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 June 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)

	June 30, 2023	December 31, 2022
ASSETS		
Current assets:		
Cash and cash equivalents	$ 4,331	$ 19,207
Restricted cash	210	261
Prepaid expenses and other current assets	841	661
Total current assets	5,382	20,129
Property and equipment, net	130	169
Operating lease right-of-use asset	481	825
Total assets	**$ 5,993**	**$ 21,123**

LIABILITIES AND STOCKHOLDERS' DEFICIT		
Current liabilities:		
Accounts payable	$ 3,121	$ 3,020
Accrued expenses	2,752	2,799
Current portion of long-term debt	2,276	3,980
Advances from collaboration partners	12,535	12,535
Current portion of operating lease liability	492	712
Derivative liabilities	75	45
Total current liabilities	21,251	23,091
Long-term debt, net of current portion	3,334	8,557
Operating lease liability	4	135
Total liabilities	24,589	31,783
Total stockholders' deficit:	(18,596)	(10,660)
Total liabilities and stockholders' deficit	**$ 5,993**	**$ 21,123**

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,	
	2023	**2022**	**2023**	**2022**
Operating expenses:				
Research and development	$ 2,338	$ 7,651	$ 5,826	$ 15,550
General and administrative	1,802	2,645	3,797	5,699
Total operating expenses	4,140	10,296	9,623	21,249
Loss from operations	(4,140)	(10,296)	(9,623)	(21,249)
Other expense, net	201	322	948	989
Net loss	**$ (4,341)**	**$ (10,618)**	**$ (10,571)**	**$ (22,238)**
Net loss per share, basic and diluted	**$ (1.96)**	**$ (4.90)**	**$ (4.83)**	**$ (10.27)**
Weighted average number of shares of common stock used in computing net loss per share, basic and diluted	2,212,364	2,166,352	2,189,487	2,166,314

Source: Eloxx Pharmaceuticals