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

April 3, 2023

Three patients now dosed in Phase 2 clinical study evaluating ELX-02 for the treatment of Alport syndrome; encouraging initial reduction in proteinuria has been observed in one patient to date

Investigational New Drug (IND) application for ZKN-013 filed for treatment of recessive dystrophic epidermolysis bullosa (RDEB)

WATERTOWN, Mass., April 03, 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 December 31, 2022 and provided a business update.

“With topline data expected for ELX-02 in Alport syndrome in the first half of 2023, we believe we are approaching a significant milestone for the company, to advance into our first Phase 3 study, with the potential to create significant value for both patients and shareholders,” said Sumit Aggarwal, President and Chief Executive Officer of Eloxx. “Positive data from the trial would also be a validation of our potential to treat other rare kidney diseases and develop small molecule genetic therapy products.”

Fourth Quarter 2022 and Subsequent Highlights

Alport Syndrome

- Eloxx has now dosed three patients with ELX-02 in the ongoing 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). Encouraging initial reduction in proteinuria has been observed in one patient to date. Alport syndrome is a rare genetic disorder characterized by kidney disease with high levels of proteinuria, hearing loss and eye abnormalities. 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.
- 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)

- In March 2023, Eloxx announced the submission of an Investigational New Drug (“IND”) application with the U.S. Food and Drug Administration for ZKN-013 for the treatment of recessive Dystrophic Epidermolysis Bullosa (RDEB) with nonsense mutations. RDEB is a rare skin disease characterized by mutations in Collagen 7 gene.
- Recent preclinical results demonstrated read-through activity of ZKN-013 in multiple COL7 genotypes across multiple RDEB patient derived fibroblasts and keratinocytes. Read-through activity resulted in up to an 18-fold increase in full-length COL VII protein levels. Prolonged treatment with ZKN-013 further increased COL VII protein levels. Functionality of the restored full-length COL VII protein was confirmed. 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. An additional IND filing for ZKN-013 for treatment of FAP is planned in the first half of 2023.
- 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.

Fourth Quarter 2022 Financial Results

For the three months ended December 31, 2022, we incurred a net loss of $6.3 million, or $2.92 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 $12.1 million, or $5.60 per share, which included $1.6 million in stock-based compensation.

R&D expenses were $3.3 million for the three months ended December 31, 2022, which includes $0.3 million in stock-based compensation. For the same period in the prior year, R&D expenses were $7.9 million, which included $0.6 million of stock-based compensation. The decrease was related to a decrease in clinical trial expenses related primarily to Cystic Fibrosis Foundation funded activities, and a decrease in salaries and other personnel costs.

General and administrative (G&A) expenses were $2.7 million for the three months ended December 31, 2022, which includes $0.3 million in stock-based compensation. For the same period in the prior year, G&A expenses were $3.7 million, which included $1.0 million of stock-based compensation. The decrease was primarily related to a decrease in salaries and other personal related costs due to reduced headcount and outsourcing certain functions, a decrease in expenses attributable to professional and consulting fees, and a decrease in stock-based compensation expense.

As of December 31, 2022, we had unrestricted cash and cash equivalents of $19.2 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 Eloxx will be required to make principal payments, plus interest, beginning in September 2023. 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. 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 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 Annual Report on Form 10-K for the fiscal year ended December 31, 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/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.

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)

	December 31, 2022	December 31, 2021
ASSETS		
Current assets:		
Cash and cash equivalents	$ 19,207	$ 42,268
Restricted cash	261	299
Prepaid expenses and other current assets	661	913
Total current assets	20,129	43,480
Property and equipment, net	169	216
Operating lease right-of-use assets	825	1,443
Total assets	**$ 21,123**	**$ 45,139**
LIABILITIES AND STOCKHOLDERS' (DEFICIT) EQUITY		
Current liabilities:		
Accounts payable	$ 3,020	$ 1,379
Accrued expenses	2,799	4,196
Current portion of long-term debt	3,980	-
Advances from collaboration partners	12,535	3,723
Current portion of operating lease liabilities	712	657
Derivative liabilities	45	-
Total current liabilities	23,091	9,955
Long-term debt	8,557	11,996
Operating lease liabilities	135	804
Total liabilities	31,783	22,755
Total stockholders' (deficit) equity	(10,660)	22,384
Total liabilities and stockholders' (deficit) equity	**$ 21,123**	**$ 45,139**

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

	Three Months Ended December 31,		Year Ended December 31,	
	2022	2021	2022	2021
Operating expenses:				
Research and development	$ 3,297	$ 7,912	$ 23,727	$ 22,899
General and administrative	2,731	3,718	10,692	20,449
In process research and development	—	—	—	22,670
Total operating expenses	6,028	11,630	34,419	66,018
Loss from operations	(6,028)	(11,630)	(34,419)	(66,018)
Other expense, net	291	460	1,646	709
Net loss	**$ (6,319)**	**$ (12,090)**	**$ (36,065)**	**$ (66,727)**
Basic and diluted net loss per share	**$ (2.92)**	**$ (5.60)**	**$ (16.65)**	**$ (38.15)**
Weighted average number of common shares used in computing net loss per share, basic and diluted	2,166,356	2,159,658	2,166,311	1,749,071

SOURCE: Eloxx Pharmaceuticals, Inc.

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