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

May 10, 2022

Cystic Fibrosis Foundation (CF Foundation) awarded up to $15.9 million for the ongoing ELX-02 clinical program

Topline data from cystic fibrosis (CF) Phase 2 expansion treatment arms evaluating combination with ivacaftor expected at the end of the first half of 2022

Expanded development of ELX-02 for the treatment of Alport syndrome, a rare kidney genetic disorder, with Phase 2 expected to start in second half of 2022

Expect to submit an Investigational New Drug (IND) application for the inhaled delivery of ELX-02 in the second half of 2022

On track to start First in Human Phase 1 study in 2022 with ZKN-013 for the treatment of recessive dystrophic epidermolysis bullosa (RDEB) patients with nonsense mutations cells

Cash and equivalents expected to be sufficient to fund operations into the second quarter of 2023

WATERTOWN, Mass., May 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 March 31, 2022 and provided a business update.

“During the first quarter, we made significant progress, highlighted by the funding award from the CF Foundation and the expansion of our development program for ELX-02 in Alport syndrome, as we begin to fully capture the potential of ELX-02 as a novel readthrough agent,” said Sumit Aggarwal, President and Chief Executive Officer of Eloxx. “Combined with the continuing progress for our novel Ribosome Modulating Agents in RDEB and familial adenomatous polyposis, we believe we are poised to deliver on multiple potential value-creating events over the next twelve months.”

First Quarter 2022 and Subsequent Highlights

Class 1 Cystic Fibrosis

- In March 2022, Eloxx announced additional funding from a Therapeutic Development Award of up to $15.9 million from the CF Foundation to support the ongoing global Phase 2 ELX-02 clinical development of ELX-02 in Class 1 CF. Following an upfront funding of $7.0 million, the funding will be tranched based on the achievement of certain clinical milestones.

- Phase 2 clinical trials in CF patients, with expansion arms designed to evaluate the safety of ELX-02 and assess short-term biological activity in patients, remains ongoing. Topline results are expected at the end of the first half of 2022.

- Evaluation of inhaled (nebulizer-based) delivery of the current subcutaneous formulation of ELX-02 remains ongoing. We believe that the increased drug exposure in the lung versus plasma with inhaled delivery has the potential to further improve the activity of ELX-02. We remain on track to submit an Investigational New Drug (IND) application in the second half of 2022.

Alport Syndrome

- In March 2022, Eloxx announced it has expanded its clinical development pipeline to include the potential treatment with ELX-02 of a subset of 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.

- Eloxx believes there is a strong rationale to pursue clinical development of ELX-02 in Alport syndrome based on encouraging preclinical results and clinical results.

    - Clinical readthrough results in our Phase 2 cystic fibrosis trial at well tolerated dose levels confirm activity of ELX-02 to restore proteins.

    - In recently published preclinical studies, ELX-02 has demonstrated significant readthrough in COL4A5 mutations,

which represent 85% of nonsense mutations in this population. Previously published in vivo studies have shown that even low levels of Collagen IV restoration may result in significant reduction in proteinuria.

- ELX-02 is preferentially taken up in the kidney with an expected greater than 50-fold exposure in the kidneys compared to plasma. As a result, we expect that a low dose of 0.75mg/kg/day of ELX-02 could restore therapeutically relevant levels of Collagen IV.

- Eloxx intends to initiate a proof-of-concept clinical trial in up to eight Alport syndrome patients with nonsense mutations in the second half of 2022. 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.

Recessive Dystrophic Epidermolysis Bullosa and Junctional Epidermolysis Bullosa (JEB)

- ZKN-013 continues to demonstrate dose dependent inducement of functional Collagen VIIA protein (truncated in patients with nonsense mutations patients), in RDEB patient cells.

- Eloxx continues to expect to file an IND application to start a First in Human (FIH) Phase 1 study in 2022 with ZKN-013 after recently completing 28-day non Good Laboratory Practice (GLP) animal studies.

Familial Adenomatous Polyposis

- FAP is a rare inherited disease characterized by proliferation of colon polyps with no approved drug therapies. Eloxx is targeting a subset of patients that have nonsense mutations in the Adenomatous Polyposis Coli (APC) gene that is truncated in these patients.

- As previously announced, Eloxx reported observed encouraging results from an 8-week treatment study for ZKN-013 in the APC^{Min} (multiple intestinal neoplasia) model to evaluate the potential of ZKN-013 to treat FAP. The APC^{Min} mouse is a translationally validated model for drug development for FAP.

    - 10-week old APC^{Min} mice were randomized for treatment with ZKN-013 for 8 weeks (n=10 in each group)

    - Treatment with ZKN-013 resulted in a significant 39% reduction in the number of colon polyps and an approximately 50% reduction in polyp burden. There were substantial reductions in both lesion area and area of adenoma with no progression to carcinomas

    - This led to an observed 50% survival benefit with no deaths in mice treated with ZKN-013.

First Quarter 2022 Financial Results

For the three months ended March 31, 2022, we incurred a net loss of $11.6 million, or $0.13 per share, which included $0.9 million in stock-based compensation. For the same period in the prior year, we incurred a net loss of $8.7 million, or $0.22 per share. Since the closing date of Eloxx's acquisition of Zikani Therapeutics, Inc. in April 2021, the results of Zikani's operations have been included in the Eloxx' condensed consolidated financial statements.

Our research and development expenses (R&D) were $7.9 million for the three months ended March 31, 2022, which includes $0.4 million in stock-based compensation. For the same period in the prior year, R&D expenses were $4.1 million. The increase was primarily related to increases in expenses related to preclinical activities, the continued development of ELX-02, salaries and other personnel related costs, stock-based compensation and operational facilities.

Our general and administrative (G&A) expenses were $3.1 million for the three months ended March 31, 2022, which includes $0.6 million in stock-based compensation. For the same period in the prior year, G&A expenses were $4.3 million. The decrease was primarily related to decreases in salaries and other personnel related costs, stock-based compensation expense, consultant and professional fees, and operational facilities.

As of March 31, 2022, we had cash and cash equivalents of $39.8 million (inclusive of the $7.0 million from the CF Foundation), which we expect will be sufficient to fund our operations into the second quarter of 2023.

About Nonsense Mutations

Nonsense mutations cause a premature stop codon in the mRNA resulting in less than full length or loss of function proteins. These remain highly underserved with no approved disease modifying therapies. An estimated 10-12% patients across over 8,000 inherited genetic rare diseases harbor nonsense mutations in one or both alleles harboring nonsense mutations.

About Eloxx Pharmaceuticals

Eloxx Pharmaceuticals, Inc. is engaged in the science of ribosome modulation, leveraging its innovative TURBO-ZM^{TM} 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, the sufficiency of our cash and cash equivalents to fund our operations, 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 quarterly period ended March 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.

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

	March 31, 2022	December 31, 2021
ASSETS		
Current assets:		
Cash and cash equivalents	$ 39,768	$ 42,268
Restricted cash	297	299
Prepaid expenses and other current assets	2,082	913
Total current assets	42,147	43,480
Property and equipment, net	206	216
Operating lease right-of-use assets	1,265	1,443
Total assets	**$ 43,618**	**$ 45,139**
LIABILITIES AND STOCKHOLDERS’ EQUITY		
Current liabilities:		
Accounts payable	$ 2,903	$ 1,379
Accrued expenses	4,631	4,196
Advances from collaboration partners	10,723	3,723

Derivative liabilities	270	-
Current portion of operating lease liabilities	647	657
Total current liabilities	19,174	9,955
Long-term debt	12,120	11,996
Operating lease liabilities	638	804
Total liabilities	31,932	22,755
Total stockholders' equity	11,686	22,384
Total liabilities and stockholders' equity	**$ 43,618**	**$ 45,139**

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

	Three Months Ended March 31,	
	2022	**2021**
Operating expenses:		
Research and development	$ 7,899	$ 4,073
General and administrative	3,054	4,341
Total operating expenses	10,953	8,414
Loss from operations	(10,953)	(8,414)
Other expense, net	667	280
Net loss	**$ (11,620)**	**$ (8,694)**
Basic and diluted net loss per share	**$ 0.13**	**$ 0.22**
Weighted average number of common shares used in computing net loss per share, basic and diluted	86,651,036	40,180,131

Source: Eloxx Pharmaceuticals, Inc.

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