Eloxx Pharmaceuticals Reports Third Quarter 2021 Financial and Operating Results and Provides Business Update

November 9, 2021

Data from Monotherapy Arms of Ongoing Phase 2 ELX-02 Cystic Fibrosis (CF) Clinical Trials Expected in Fourth Quarter of 2021

Received FDA Fast Track Designation for ELX-02 for the Treatment of CF Patients with Nonsense Mutations

Hosted Call and Webcast on CF Programs Featuring CF Expert Dr. Eitan Kerem

Appointed Ali Hariri, M.D. as Chief Medical Officer

Entered into Senior Debt Facility from Hercules Capital for Borrowings of Up to $30 Million

WATERTOWN, Mass., Nov. 09, 2021 (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 and nine months ended September 30, 2021 and provided a business update.

“We continue to make significant progress across our portfolio of therapeutic programs,” said Sumit Aggarwal, President and Chief Executive Officer of Eloxx. “We are on track to report data from the ongoing Phase 2 clinical trials for ELX-02 in the fourth quarter of 2021. In addition, our preclinical programs continue to advance demonstrating the potential of our oral RMAs. We intend to provide additional updates on these programs in the coming quarters.”

Third Quarter 2021 and Subsequent Highlights

Class 1 Cystic Fibrosis

- Ongoing ELX-02 Phase 2 clinical trials in CF patients affected by nonsense mutations in the CFTR (CF transmembrane conductance regulator) gene are designed to evaluate the safety of ELX-02 and assess short-term biological activity in patients.
    - We remain on track to report data from the monotherapy arms of the ELX-02 clinical trials in the fourth quarter of 2021. In addition, we are continuing to enroll additional patients to support Phase 3 clinical trial planning.
    - As previously announced, the U.S. Food and Drug Administration granted Fast Track designation for ELX-02. Fast Track Designation is granted to drugs being developed for the treatment of serious or life-threatening diseases or conditions where there is an unmet medical need.
    - Biological activity is being assessed by changes in sweat chloride, a surrogate marker for restoring CFTR activity. For comparison, Orkambi, an FDA-approved combination CF agent (Lumacaftor/Ivacaftor), demonstrated mid-single digit reductions in sweat chloride over one- to two-week study durations in a similar trial setting. In addition, Symdeko, another FDA-approved combination agent, demonstrated low- to mid-single digit reductions in sweat chloride over 28 days.
    - Patient dosing in the expansion arm, which includes a combination of ELX-02 and the CFTR protein potentiator, Kalydeco (ivacaftor), is expected to begin by the end of 2021, with topline results expected in the first half of 2022.
- Began evaluation of inhaled (nebulizer-based) delivery of the current subcutaneous formulation of ELX-02. This has the

potential to further improve the activity of ELX-02 as a single agent and in combination with other drugs given potential for increased drug exposure in the lung versus plasma. An Investigational New Drug (IND) application is expected in 2H 2022.

- Presented preclinical data at the 2021 North American Cystic Fibrosis Conference demonstrating that significantly greater than 75% of patient derived organoids with ultra rare premature termination codon (PTC) mutations and with no residual CFTR function show swelling response to treatment with ELX-02.

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

- ZKN-013 is advancing into IND-enabling studies for the treatment of RDEB and JEB. Good Laboratory Practice (GLP) safety studies are expected to begin by the end of 2021. We remain on track to file an IND in 2022.

Rare Inherited and Targeted Oncology

- Continued to advance our preclinical oncology pipeline, which is focused on rare inherited cancers with driver nonsense mutations and cancers with driver mutations in the ribosome.

- Our lead program is for the treatment of patients with Familial Adenomatous Polyposis (FAP).
    - An 8-week study in APC^{Min} (multiple intestinal neoplasia) mice to evaluate the potential of RMAs to treat FAP. The APC^{Min} mouse is a translationally validated model for drug development for FAP. This study in APC^{Min} will evaluate polyp number and size in ZKN013 treated mice versus control mice. We expect to report results from this study in the fourth quarter of 2021.

- Cancer cell line and xenograft mouse studies ongoing to evaluate the response to treatment with RMAs to advance first-in-class onco-ribosome targeted inhibitors for oncology therapy.

Corporate

- Eloxx entered into a debt facility with Hercules Capital, Inc., a specialty financing lender for life science and technology companies, that provides for borrowings of up to $30.0 million. Eloxx has drawn the initial tranche of $12.5 million and used the proceeds to repay in full amounts outstanding under the Eloxx' existing debt facility. The remaining funds under the new facility will be available in additional tranches upon the achievement of specific clinical milestones or other terms and conditions.

- Ali Hariri, M.D., joined Eloxx as Chief Medical Officer, overseeing clinical development efforts for the company's promising pipeline of therapies to treat rare diseases.

Third Quarter 2021 Financial Results

For the three months ended September 30, 2021, we incurred a net loss of $9.9 million, or $0.11 per share, which includes $2.2 million in stock-based compensation. For the same period in the prior year, we incurred a net loss of $6.6 million, or $0.16 per share. Results for the third quarter of 2021 included costs related to the acquisition of Zikani. Since the closing date of the acquisition, the results of Zikani's operations have been included in the Eloxx' condensed consolidated financial statements.

Our research and development expenses (R&D) were $5.2 million for the three months ended September 30, 2021, which includes $0.4 million in stock-based compensation. For the same period in the prior year, R&D expenses were $3.4 million. The increase was primarily related to expenses related to the continued development of ELX-02 due to the impact of the COVID-19 pandemic in the prior year period, an increase in salaries and other personnel related costs, and an increase in operational facilities.

Our general and administrative (G&A) expenses were $5.0 million for the three months ended September 30, 2021, which includes $1.9 million in stock-based compensation. For the same period in the prior year, G&A expenses were $2.9 million. The increase was primarily related to an increase in stock-based compensation expense, an increase in salaries and other personnel related costs, as well as an increase in legal, accounting and other professional fees.

As of September 30, 2021, we had cash and cash equivalents of $52.4 million, which we expect will be sufficient to fund our operations into the first 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 Ribsome 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 clinical development, focusing on cystic fibrosis. 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 expected cash burn and future financial results, 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 quarter ended September 30, 2021, 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/financial-information/sec-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)

	September 30, 2021	December 31, 2020
ASSETS		
Current assets:		
Cash and cash equivalents	$ 52,432	$ 24,668
Restricted cash	246	56
Prepaid expenses and other current assets	864	1,169
Total current assets	53,542	25,893
Property and equipment, net	224	133
Operating lease right-of-use assets	1,617	421
Other long-term assets	—	30
Total assets	$ 55,383	$ 26,477
LIABILITIES AND STOCKHOLDERS’ EQUITY		
Current liabilities:		
Accounts payable	$ 2,509	$ 481
Accrued expenses	2,611	2,924
Current portion of long-term debt	—	5,239
Advances from collaboration partners	3,723	805
Current portion of operating lease liabilities	667	389

Total current liabilities	9,510	9,838
Long-term debt	11,911	6,376
Operating lease liabilities	964	33
Total liabilities	22,385	16,247
Total stockholders' equity	32,998	10,230
Total liabilities and stockholders' equity	$ 55,383	$ 26,477

UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Operating expenses:				
Research and development	$ 5,210	$ 3,445	$ 14,987	$ 11,950
General and administrative	5,035	2,851	16,731	11,705
In process research and development	—	—	22,670	—
Restructuring charges	—	—	—	3,994
Total operating expenses	10,245	6,296	54,388	27,649
Loss from operations	(10,245)	(6,296)	(54,388)	(27,649)
Other income (expense), net	360	(321)	(249)	(801)
Net loss	$ (9,885)	$ (6,617)	$ (54,637)	$ (28,450)
Net loss per share, basic and diluted	$ (0.11)	$ (0.16)	$ (0.85)	$ (0.71)
Weighted average number of shares of common stock used in computing net loss per share, basic and diluted	86,208,754	40,142,178	64,428,187	40,115,351

Source: Eloxx Pharmaceuticals

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