Eloxx Pharmaceuticals Reports First Quarter 2019 Financial and Operating Results and Provides Business Update

May 9, 2019

Positive new preclinical data for Eloxx ERSG molecules presented at the Association for Research in Vision and Ophthalmology (ARVO) 2019 Annual Meeting May 2, 2019 demonstrated:

- *dose dependent restoration of missing protein of Usher Syndrome nonsense mutations,*
- *encouraging pharmacokinetics in the retina by intravitreal injection*
- *favorable tolerability profile*

On track to report top line data from Phase 2 clinical trials for ELX-02 in cystic fibrosis and cystinosis in the U.S. and Europe in 2019

Company to host webcast and conference call on Thursday, May 9, 2019 at 8:30 am ET

WALTHAM, Mass., May 09, 2019 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc., (NASDAQ: ELOX) a clinical-stage biopharmaceutical company dedicated to the discovery and development of novel therapeutics to treat cystic fibrosis, cystinosis, inherited retinal disorders, and other diseases caused by nonsense mutations limiting production of functional proteins, today reported its financial results for the three months ended March 31, 2019 and provided a business update.

"We are very pleased to be completing the final cohort of our multiple ascending dose study in the U.S. and rapidly advancing ELX-02 into Phase 2 clinical trials in cystic fibrosis and cystinosis. We look forward to reporting top line data in the U.S. and Europe in 2019 and we believe that the positive data we have generated for ELX-02 in cystic fibrosis patient-derived organoids substantially de-risk our Phase 2 program," said Robert E. Ward, Chairman and CEO of Eloxx Pharmaceuticals. "Eloxx had the opportunity to share new positive data for several of our investigational product candidates in inherited retinal diseases for the first time at ARVO, which was well received given the high unmet medical need across nonsense mutation-bearing patients."

Cystic Fibrosis Program Updates

- We initiated the seventh and final cohort of our multiple-ascending dose (MAD) study for ELX-02 and expect to complete the study in the U.S. in the next two weeks.

- We are on track to initiate a Phase 2 clinical trial this year in the U.S. and Europe in cystic fibrosis patients with the G542X CFTR mutation. We expect to report top line data from this trial in 2019. Our Clinical Trial Application has been approved and granted orphan drug designation by the European Medicines Agency.

- Three abstracts have been accepted for presentation at the **42nd European Cystic Fibrosis Society (ECFS) Conference June 5-8, 2019 titled:**

    - **"ELX-02 increases full length *CFTR m*RNA through nonsense mediated decay interruption"** June 6th 15:00-16:30, ORAL, Hall 2E**, Workshop: Abnormalities in cystic fibrosis cells and strategies to fix them**
    - **"ELX-02 Pharmacokinetic profile appropriate for CF patient use"** June 7th 14:00-15:00, POSTER**, Clinical Trials and New Therapies**
    - **"Administration of ELX-02 to Healthy Volunteers Demonstrates Dose-linearity and Proportionality as Well as Low Inter-subject Variability"** June 6th 17:00-18:30, ORAL, Hall 1A, **Workshop: Hot off the press; new data from drug trials**

- On March 27, 2019, Eloxx presented new positive data for ELX-02 at the European Cystic Fibrosis Basic Science Conference in Croatia. In a poster titled: "**CFTR protein detection in organoids from healthy and CF patients with nonsense mutations support using organoid model to test ELX-02 mediated CFTR read-through restoration",** Dr. Shira Landskroner-Eiger, Sr. Principal Scientist, Translational Sciences at Eloxx, reported that:

    - Consistent with increased CFTR activity observed in the organoid swelling assay, ELX-02 mediates a significant restoration of CFTR protein expression as measured via a capillary-based immunoassay approach in multiple *G542X* or *W1282X* nonsense carrying organoids.

- *G542X* organoids treated with ELX-02 demonstrate proper cell surface CFTR localization on the apical surface, which is consistent with increased CFTR, mRNA, and CFTR function in the swelling assay.

While ELX-02 mediated protein increases have been previously demonstrated, this is the first demonstration reported in cystic fibrosis patient organoids. Within this translational CF organoid model, ELX-02 dose-dependent increases in *CFTR* mRNA stability and function can now be extended to the demonstration of accompanying increases of CFTR protein.

Previously, Eloxx presented positive data for ELX-02 at the North American Cystic Fibrosis Conference on October 18th, 2018 in Denver, Colorado in a poster presentation titled: **"Measuring mRNA levels in cystic fibrosis organoids with nonsense mutations following treatment with ELX-02,"** which demonstrated ELX-02 mediated dose responsive increases in CFTR function, as measured by FIS swelling activity, which was found to correlate with increases in *CFTR* mRNA, as measured by nanoString™ technology, with elevations above wild-type. ELX-02 appears to increase the steady state concentrations of *CFTR* mRNA suggesting that ELX-02 may be modulating nonsense mediated decay. These data demonstrate that ELX-02 promotes translation of functional CFTR and we believe, de-risks our planned Phase 2 clinical trial of ELX-02 in cystic fibrosis patients with the G542X CFTR mutation on one or both alleles, which is the second most common mutation globally and accounts for about 5% of the cystic fibrosis population.

- Eloxx has continued to generate new data for ELX-02 activity from a growing number of patient-derived organoids which represent multiple nonsense mutations across a variety of genotypes representing the top 5 nonsense mutations in the cystic fibrosis population, which cover over 75% of the nonsense bearing cystic fibrosis patients.

- On February 26, 2019, Eloxx announced it joined the consortium agreement of the European HIT-CF project, a European Union funded preclinical and clinical research program evaluating the efficacy and safety of several disease modifying drug candidates in Cystic Fibrosis (CF) patients with rare genetic mutations. Eloxx's lead investigational drug candidate, ELX-02, a small molecule eukaryotic ribosomal selective glycoside (ERSG), will be evaluated in cystic fibrosis patients with nonsense mutations for whom there are few available treatment options.

    - The goal of the European HIT-CF project is to investigate whether a positive response to therapies in a patient derived organoid can be predictive of clinical response in a controlled trial. The project represents a new era in CF treatment and personalized medicine, as it has the potential to shift therapeutic trials from patients to the laboratory. The organoid model could be extended to all patients with CF and other rare genetic diseases to identify appropriate therapeutic options. The EU has granted EUR 6.7 million from the Horizon 2020 research program to HIT-CF.

- To support the Phase 2 clinical trial program for ELX-02, Eloxx has completed the manufacturing of a lyophilized clinical drug product. Eloxx has also identified a commercial manufacturer and is engaged in the process development work to scale up activities required to support Phase 3 clinical development.

ELX-02 is an investigational agent not approved by any regulatory agency for therapeutic use.

Inherited Retinal Disease Program Updates

- Eloxx has been advancing several new investigational product candidates from its library into IND enabling studies in ophthalmology. Currently available data for several of the molecules that have demonstrated positive activity on nonsense mutations across different inherited retinal disorders as well as a favorable tolerability profile.

- In a Poster presentation at the **ARVO Annual Meeting** on May 2, 2019 titled: "**Instituting a read-through therapeutic approach to nonsense-mutation based inherited retinal disorders, ELX-03, a translational nonsense mutation read-through agent demonstrates tolerability and activity for use in inherited retinal disorders**", presented by Dr. Matthew Goddeeris, Director of Research at Eloxx, it was reported that:

    - Eloxx has screened multiple compounds from its ERSG library of read-through agents for potential use in the treatment of inherited retinal disorders with an initial focus on Usher Syndrome, beginning with USH2A.

    - Multiple Eloxx compounds in preclinical studies have demonstrated:

        - Dose-responsive activity against Usher mutations.
        - Restoration of missing Usher Syndrome protein.
        - Favorable tolerability profile at high doses in sensitive species; preserved electroretinogram (ERG) and no compound-related histopathological changes.
        - Encouraging pharmacokinetics demonstrating retina exposure by intravitreal injection.

- IND-enabling studies are ongoing.

- Next steps include evaluation of patient-derived cell models and sustained release formulations.

At the request of the Foundation Fighting Blindness, Dr. Matthew Goddeeris, also made a presentation at the **Sixth Annual Retinal Cell and Gene Therapy Innovation Summit** in Vancouver, BC, on April 26, 2019, to review Eloxx Pharmaceuticals' read-through therapeutic approach to inherited retinal disorders with its library of ERSG molecules, which was very well received.

First Quarter 2019 Financial Results

As of March 31, 2019, we had cash and cash equivalents of $53.5 million. We expect our total cash and cash equivalents will be sufficient to fund our operations through top line data from our Phase 2 clinical trials in cystic fibrosis and cystinosis for our lead investigational product, ELX-02, and into the second quarter of 2020 based upon our current operating plans.

We incurred a loss for the three months ended March 31, 2019, of $11.9 million or $0.33 per share, which includes $2.7 million non-cash expense related to stock-based compensation. For the same period in the prior year, we incurred a net loss of $8.6 million, or $0.31 per share.

Our research and development expenses were $6.0 million for the three months ended March 31, 2019 which includes $0.5 million non-cash expense related to stock-based compensation. For the same period in the prior year, R&D expenses were $4.4 million. Quarter to quarter fluctuations were due to normal timing of R&D activities.

Our general and administrative expenses were approximately $6.0 million for the three months ended March 31, 2019 compared to approximately $3.4 million for the same period in the prior year, an increase of approximately $2.6 million. The increase in our general and administrative expenses was primarily related to non-cash expense related to stock-based compensation of $2.1 million in the 2019 period, and an increase in our headcount and related salaries, and professional service fees.

Conference Call Information:
Date: Thursday, May 9, 2019
Time: 8:30 a.m. ET
Domestic Dial-in Number: 866-913-8546
International Dial-in Number: 210-874-7715
Conference ID: 5198713
Live Webcast: accessible from the Company's website at www.eloxxpharma.com under Events and Presentations or with this link: https://edge.media-server.com/m6/p/ve82h2hr

About Eloxx Pharmaceuticals

Eloxx Pharmaceuticals, Inc. is a clinical-stage biopharmaceutical company developing novel RNA-modulating drug candidates (designed to be eukaryotic ribosomal selective glycosides) that are formulated to treat rare and ultra-rare premature stop codon diseases. Premature stop codons are point mutations that disrupt protein synthesis from messenger RNA. As a consequence, patients with premature stop codon diseases have reduced or eliminated protein production from the mutation bearing allele accounting for some of the most severe phenotypes in these genetic diseases. These premature stop codons have been identified in over 1,800 rare and ultra-rare diseases. Read-through therapeutic development is focused on extending mRNA half-life and increasing protein synthesis by enabling the cytoplasmic ribosome to read through premature stop codons to produce full-length proteins. 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 the early stages of clinical development focusing on cystic fibrosis and cystinosis. ELX-02 is an investigational drug that has not been approved by any global regulatory body. Eloxx's preclinical candidate pool consists of a library of novel drug candidates designed to be eukaryotic ribosomal selective glycosides identified based on read-through potential. Eloxx recently announced a new program focused on rare ocular genetic disorders. Eloxx is headquartered in Waltham, MA, with operations in Rehovot, Israel. For more information, please visit www.eloxxpharma.com.

Forward-Looking Statements

This press release contains forward-looking statements, which are generally statements that are not historical facts. Forward-looking statements can be identified by the words "expects," "anticipates," "believes," "intends," "estimates," "plans," "will," "outlook" and similar expressions. Forward-looking statements are based on management's current plans, estimates, assumptions and projections, and speak only as of the date they are made. We undertake no obligation to update any forward-looking statement in light of new information or future events, except as otherwise required by law. Forward-looking statements involve inherent risks and uncertainties, most of which are difficult to predict and are generally beyond our control. Actual results or outcomes may differ materially from those implied by the forward-looking statements as a result of the impact of a number of factors,including: the development of the Company's read-through technology; the approval of the Company's patent applications; the Company's ability to successfully defend its intellectual property or obtain necessary licenses at a cost acceptable to the Company, if at all; the successful implementation of the Company's research and development programs and collaborations; the Company's ability to obtain applicable regulatory approvals for its current and future product candidates; the acceptance by the market of the Company's products should they receive regulatory approval; the timing and success of the Company's preliminary studies, preclinical research, clinical trials, and related regulatory filings; the ability of the Company to consummate additional financings as needed; as well as those discussed in more detail in our Annual Report on Form 10-K and our other reports filed with the Securities and Exchange Commission.

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

	March 31, 2019	December 31, 2018
ASSETS		
Current assets:		
Cash and cash equivalents	$ 44,576	$ 48,606
Marketable securities	8,928	—
Restricted bank deposit	45	45
Prepaid expenses and other current assets	1,911	1,690
Total current assets	55,460	50,341
Property and equipment, net	231	248
Operating lease right-of-use asset	1,106	—
Other long-term assets	94	129
Total assets	$ 56,891	$ 50,718
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 2,240	$ 747
Accrued expenses	4,597	6,938
Current portion of long-term debt	984	—
Current portion of operating lease liability	481	—
Taxes payable	122	122
Total current liabilities	8,424	7,807
Long-term debt	13,409	—
Operating lease liability	625	—
Stockholders' equity:		
Common stock, $0.01 par value per share, 500,000,000 shares authorized, 36,047,498 and 35,951,537 shares issued and 35,945,608 and 35,860,114 shares outstanding as of March 31, 2019 and December 31, 2018, respectively	361	360
Common stock in treasury, at cost, 101,890 and 91,423 shares at March 31, 2019 and December 31, 2018, respectively	(1,250)	(1,129)
Additional paid in capital	133,383	129,825
Accumulated other comprehensive income	1	—
Accumulated deficit	(98,062)	(86,145)
Total stockholders' equity	34,433	42,911
Total liabilities and stockholders' equity	$ 56,891	$ 50,718

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

	Three Months Ended March 31,	
	2019	2018
Operating expenses:		
Research and development	$ 6,019	$ 4,394

General and administrative	5,958	3,393
Reverse merger related expenses	—	761
Total operating expenses	11,977	8,548
Loss from operations	(11,977)	(8,548)
Other (income) expense, net	(60)	43
Net loss	$ (11,917)	$ (8,591)
Basic and diluted net loss per share	$ (0.33)	$ (0.31)
Weighted average number of Common Shares used in computing basic and diluted net loss per share	35,910,270	27,527,738

SOURCE: Eloxx Pharamaceuticals, Inc.

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