



## Eloxx Pharmaceuticals Reports Fourth Quarter and Full Year 2018 Financial and Operating Results and Provides Business Update

March 8, 2019

*Eloxx announces the hiring of Dr. Susan Schneider as SVP Ophthalmology to lead the development of its rare inherited retinal disease programs*

*Abstract accepted for presentation at European Cystic Fibrosis Society (ECFS) Basic Science Conference March 27-30, 2019 demonstrating ELX-02 activity in restoring CFTR protein*

*ELX-03 abstract accepted for presentation at the Association for Research in Vision and Ophthalmology (ARVO) 2019 Annual Meeting April 28-May 2, 2019*

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

*Company to host webcast and conference call on Friday, March 8, 2019 at 8:00 am ET*

WALTHAM, Mass., March 08, 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 and twelve months ended December 31, 2018 and provided a business update.

"We are very pleased to be advancing ELX-02 in cystic fibrosis and look forward to reporting top line data from our Phase 2 clinical trial in the U.S. and Europe in 2019. New positive data for ELX-02 in cystic fibrosis patient-derived organoids with nonsense mutations consistently demonstrate dose responsive increases in FIS swelling, and functional CFTR which correlate with *CFTR* mRNA elevations to levels at or above wild-type, and we believe substantially de-risk our Phase 2 program. We look forward to presenting new important data on ELX-02 activity in restoring the CFTR protein at ECFS later this month," said Robert E. Ward, Chairman and CEO of Eloxx Pharmaceuticals. "As we advance several new investigational product candidates from our library into development for inherited retinal diseases, we are grateful to have attracted Dr. Susan Schneider, a recognized expert in ophthalmology, to lead the team and also for the support of the Foundation Fighting Blindness."

### Cystic Fibrosis Program Updates

- An abstract reporting on new data demonstrating the ability of ELX-02 to restore production of the CFTR protein has been accepted for presentation at the ECFS Basic Science Meeting on May 25<sup>th</sup>-27<sup>th</sup> 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"**.
- Previously, Eloxx presented positive data for ELX-02 at the North American Cystic Fibrosis Conference on October 18<sup>th</sup>, 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 increased 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.

- The results from our Phase 1a single-ascending dose (SAD) study for ELX-02 have been published in the January 2019 edition of the **Journal of Clinical Pharmacology and Drug Development** titled: **“Safety, Tolerability, and Pharmacokinetics of Single Ascending Doses of ELX-02, a Potential Treatment for Genetic Disorders Caused by Nonsense Mutations. In Healthy Volunteers”**
- We have initiated the sixth cohort of our multiple-ascending dose (MAD) study for ELX-02 and expect to complete the final cohort in the U.S. in the first half of 2019.
- 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.
- 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.
- Eloxx announced that it has hired Dr. Kristie Kapinas to its Patient Advocacy team. Dr. Kapinas has deep cystic fibrosis experience and previously worked as a Medical Science Liaison and Disease Educator.

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

#### **Inherited Retinal Disease Program Updates**

- Eloxx today announced that it has hired a highly experienced ophthalmology executive, Dr. Susan Schneider, as Senior Vice President Ophthalmology to lead the clinical development team and will be responsible for strategic clinical oversight and the advancement of our inherited retinal disease programs across our library of eukaryotic ribosomal selective glycosides (ERSG). Dr. Schneider has extensive experience leading the clinical development and strategic planning efforts across a range of ocular indications at leading companies including most recently as Chief Medical Officer at ThromboGenics, Vice President and Therapeutic Head, Glaucoma & Retina, Glaucoma and wet AMD at Allergan, as well as leadership roles in Ophthalmology at GlaxoSmithKline, Bausch & Lomb, and Genentech.
- 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 have demonstrated positive activity on nonsense mutations across different inherited retinal disorders as well as a favorable tolerability profile. The preservation of the electroretinogram wave function and retinal histology are important safety considerations, and the preclinical data to date show that our investigational agent has an acceptable safety profile. We believe that these data are supportive of the use of these compounds for intravitreal injection with initial development focused on Usher's Syndrome. There are an estimated 4,000 individuals in the U.S. alone with nonsense mutations underlying Usher's Syndrome.
- An abstract has been accepted for presentation at the ARVO Meeting April 28-May 2, 2019, in Vancouver titled: **“ELX-03, a translational nonsense mutation read-through agent demonstrates tolerability and activity for use in inherited retinal disorders”**.

#### **Fourth Quarter 2018 Financial Results**

As of December 31, 2018, we had cash and cash equivalents of \$48.6 million, which does not include \$14.8 million in net proceeds received from a debt financing transaction completed in January 2019. We expect our total cash and cash equivalents, including the net debt proceeds, will be sufficient to fund our operations through top line data from our Phase 2 clinical trial in cystic fibrosis 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 December 31, 2018, of \$14.0 million or \$0.40 per share, which includes \$3.8 million non-cash expense related to stock-based compensation. For the same period in the prior year, we incurred a net loss of \$11.4 million, or \$0.52 per share.

Our research and development expenses were \$6.5 million for the three months ended December 31, 2018, which includes \$0.8 million non-cash expense related to stock-based compensation. For the same period in the prior year, R&D expenses were \$8.2 million. Quarter to quarter fluctuations were due to normal timing of R&D activities.

Our general and administrative expenses were approximately \$7.6 million for the three months ended December 31, 2018 compared to approximately \$2.4 million for the same period in the prior year, an increase of approximately \$5.2 million. The increase in our general and administrative expenses was primarily related to non-cash expense related to stock-based compensation of \$2.9 million in the 2018 period, and an increase in our headcount

and related salaries, and professional service fees.

## Full Year 2018 Financial Results

We incurred a loss for the twelve months ended December 31, 2018, of \$47.2 million or \$1.45 per share, which includes \$13.4 million non-cash expense related to stock-based compensation, versus a loss of \$23.6 million, or \$4.75 per share in the same prior year period.

Our research and development expenses were \$20.5 million for the twelve months ended December 31, 2018, which includes \$1.7 million non-cash expense related to stock-based compensation, and compares to \$16.4 million for the same period in the prior year. The year over year increase of \$4.1 million is in part due to increased fees and salaries and growth in clinical development.

Our general and administrative expenses were approximately \$27.1 million for the twelve months ended December 31, 2018, compared to approximately \$4.0 million for the same period in the prior year, an increase of approximately \$23.0 million. The increase in our general and administrative expenses was primarily related to non-cash stock-based compensation expense of \$11.6 million and an increase in our headcount and related salaries, as well as other personnel and professional service fees.

## Conference Call Information:

**Date:** Friday, March 8, 2019

**Time:** 8:00 a.m. ET

**Domestic Dial-in Number:** (866) 913-8546

**International Dial-in Number:** (210) 874-7715

**Conference ID:** 7877535

**Live Webcast:** accessible from the Company's website at [www.eloxxpharma.com](http://www.eloxxpharma.com) under Events and Presentations or with this link: <https://edge.media-server.com/m6/p/hcyzfmo>

## 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](http://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.*

## Contact:

Barbara Ryan  
203-274-2825

[barbarar@eloxxpharma.com](mailto:barbarar@eloxxpharma.com)

**ELOXX PHARMACEUTICALS, INC.**

**UNAUDITED CONSOLIDATED BALANCE SHEETS**  
**(Amounts in thousands, except share and per share data)**

	December 31,	
	2018	2017
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 48,606	\$ 24,049
Restricted bank deposit	45	102
Prepaid expenses and other current assets	1,690	355
Total current assets	50,341	24,506
Property and equipment, net	248	278
Other long-term assets	129	—
Total	<u>\$ 50,718</u>	<u>\$ 24,784</u>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable	\$ 747	\$ 1,530
Accrued expenses	6,938	1,893
Taxes payable	122	—
Total current liabilities	7,807	3,423
Stockholders' equity:		
Common stock, \$0.01 par value 500,000,000 and 500,000,000 shares authorized as of December 31, 2018 and 2017, respectively; 35,860,114 and 27,527,738 shares issued and outstanding as of December 31, 2018 and 2017, respectively	360	274
Common stock in treasury stock, at cost, 91,423 and 0 shares at December 31, 2018 and 2017, respectively	(1,129)	—
Additional paid in capital	129,825	60,047
Accumulated deficit	(86,145)	(38,960)
Total stockholders' equity	42,911	21,361
Total	<u>\$ 50,718</u>	<u>\$ 24,784</u>

**ELOXX PHARMACEUTICALS, INC.**

**UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS**  
(Amounts in thousands, except share and per share data)

	Year ended December 31,		
	2018	2017	2016
Operating expenses:			
Research and development	\$ 20,489	\$ 16,398	\$ 8,986
General and administrative	26,482	3,992	854
Reverse merger related expenses	594	—	—
Total operating expenses	47,565	20,390	9,840
Loss from operations	(47,565)	(20,390)	(9,840)
Other (income) expense, net	(502)	824	7
Loss before income taxes	(47,063)	(21,214)	(9,847)
Provision for income taxes	122	—	—
Net loss	<u>\$ (47,185)</u>	<u>\$ (21,214)</u>	<u>\$ (9,847)</u>

Less: Dividends accumulated for the period

Net loss available to common stockholders

	—	2,404	1,100
\$	(47,185)	\$ (23,618)	\$ (10,947)

Basic and diluted net loss per share

Weighted average number of Common Stock used in  
computing basic and diluted net loss per share

\$	(1.45)	\$ (4.75)	\$ (2.60)
	32,436,506	4,976,377	4,205,277



Source: Eloxx Pharmaceuticals