



## **Eloxx Pharmaceuticals Reports Fourth Quarter and Full Year 2019 Financial and Operating Results and Provides Business Update**

March 5, 2020

*Topline data from Phase 2 Cystic Fibrosis clinical trials for ELX-02 in H1 2020*

*Realignment strengthens commitment to Cystic Fibrosis and strategic flexibility by extending cash runway through the end of 2021*

*Strong balance sheet with \$56.3 million in cash and cash equivalents as of December 31, 2019*

*Company to host webcast and conference call today, Thursday, March 5, 2020, at 4:30 pm ET*

WALTHAM, Mass., March 05, 2020 (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 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, 2019 and provided a business update.

"I am highly confident that we have the right team and resources in place to achieve our clinical and portfolio objectives. Our highest priority is to complete enrollment of our Phase 2 clinical trials for Cystic Fibrosis and report topline data in the first half of this year," said Dr. Gregory Williams, Chief Executive Officer of Eloxx Pharmaceuticals. "We believe that proof of concept data in cystic fibrosis will be a substantial value inflection point for the Company. Our recent organizational realignment extends our cash runway through the end of 2021 and strengthens our strategic flexibility."

### **Organizational Realignment**

- On February 26, 2020, our Board of Directors approved a leadership and organizational realignment intended to reduce operating expenses and extend the Company's cash runway to the end of 2021.
  - The realignment strengthens our commitment to cystic fibrosis by ensuring that we have the appropriate resources and the strategic flexibility to accomplish our key objectives, which includes delivering topline Phase 2 proof of concept data for ELX-02 in cystic fibrosis in the first half of 2020.
  - These actions underscore our commitment to ensuring that we deliver value to investors and fulfill our mission to provide treatment options to patients with unmet medical needs in the most safe and expeditious manner possible.
  - The realignment included the succession of Dr. Gregory Williams to the position of CEO and Neil Belloff, Esq. to COO in addition to his role as General Counsel. Dr. Williams has a long track record of success in developing and gaining the approval of new drugs at companies including the Medicines Company, and most recently Tymlos at Radius Health, which is now the number one prescribed anabolic for postmenopausal osteoporosis.

### **Cystic Fibrosis Program Updates**

- We expect to complete enrollment and report topline data from our open-label Phase 2 clinical trial program for ELX-02 in cystic fibrosis in the first half of 2020.
- Our Phase 2 program consists of two trials, one enrolling patients at sites in Europe and Israel and the second in the U.S. The expansion of our cystic fibrosis program to the U.S. has been made possible in part by the funding provided by the Cystic Fibrosis Foundation (CFF) for a portion of the trial and our protocol has been sanctioned by the Cystic Fibrosis Therapeutics Development Network (TDN). In Europe, the European Cystic Fibrosis Society Clinical Trial Network (ECFS-CTN) has given our trial a "high priority" ranking.
- Professor Eitan Kerem, M.D., Head of the Division of Pediatrics, Children's Hospital, Hadassah Medical Center, is the Global Lead Investigator and Dr. Ahmet Uluer, Director of the Adult Cystic Fibrosis Program at the Boston Children's Hospital/Brigham and Women's Hospital CF Center, is the lead study investigator in the U.S.
- In a recent discussion with the members of the Cystic Fibrosis Program Advisory Group, a joint advisory group with CFF, we had the opportunity to share the positive results of the completed first cohort of the Phase 2 clinical trial for ELX-02 in nephropathic cystinosis. The Advisory Group viewed the safety and efficacy data as exciting for cystinosis patients and was pleased that the trial met the primary endpoint of safety. Together with the supportive pharmacokinetics from this trial, these data further de-risk the Phase 2 cystic fibrosis clinical trials.



- A scientific abstract for our Phase 2 clinical trial for ELX-02 in cystic fibrosis has been accepted for an Oral Presentation in a Workshop titled: **“Beyond modulators: approaches involving gene editing or alternative channels”** at the 43<sup>rd</sup> European Cystic Fibrosis Conference June 3<sup>rd</sup> through the 6<sup>th</sup>, 2020 in Lyon France.
- As previously reported, we completed an interim CMC review meeting with the U.S. Food and Drug Administration and gained alignment with the agency on manufacturing formulation process, which we believe will be suitable for our expected drug supply needs through completion of pivotal trials.
- We are pleased with our participation in the European HIT-CF consortium to support the collection of cystic fibrosis patient-derived organoids and the initiative to conduct a prospective clinical trial to confirm the translational potential of the organoid model. HIT-CF recently announced completion of the first phase of the program with the collection of organoids from patients at 47 of the biggest cystic fibrosis centers in 16 countries throughout Europe. Organoids from over 100 individuals bearing rare nonsense mutations in the CFTR have been collected and are being tested for responsiveness to ELX-02 in the laboratory. The first dataset evaluated ELX-02 in 31 patient-derived organoids and demonstrated activity in the majority of them. The intent of the program is to use these positive results to enroll patients with responsive organoids in a prospective trial with ELX-02. We believe this program will continue to expand the application of organoid technology from drug discovery through drug approval, and also offers possible label expansion opportunities.

#### Kidney Program Updates

- In January 2020, we announced positive data from the first cohort of the Phase 2 study of ELX-02 in the treatment of patients with nonsense mutation-mediated nephropathic cystinosis. The results of the first cohort met the primary safety endpoint and the reductions in white blood cell cystine provided a clear indication of biologic activity in these patients at nominal doses > 0.5 mg/kg/day. Following review of the safety and pharmacokinetic data by an independent Safety Review Committee (SRC), the SRC approved progressing to the second cohort that would enable enrolling patients ages 12 and older. Due to study design limitations, patients across all dose groups in cohort 1 had elevated and uncontrolled pretreatment white blood cell cystine levels which made it difficult to fully evaluate ELX-02-mediated white blood cell cystine reductions. Therefore, we have discontinued this study and will not proceed with the second cohort as contemplated in the original protocol. We will continue to review these data with a panel of scientific and clinical experts to determine appropriate modifications for a possible new study design.
- The clear indications of biologic activity in the first cohort of the Phase 2 study of ELX-02 in the treatment of patients with nonsense mutation-mediated nephropathic cystinosis provide human clinical proof of concept for ELX-02 and de-risk other clinical applications of our ERSG library using this dosage range. These encouraging results also provide a basis for expansion to studies of additional kidney diseases caused by nonsense mutations such as autosomal dominant polycystic kidney disease (ADPKD).
- ADPKD is a relatively common inherited genetic kidney disease, which in the U.S. affects between 300,000 and 600,000 individuals and is the leading cause of end stage renal disease. In our preclinical studies in ADPKD, we have observed dose-dependent read-through with our ERSG compounds across the most common PKD1 alleles and have expanded our studies to include PKD2. We are working on this program with Dr. Benjamin Freedman, a Professor in the Division of Nephrology, Department of Medicine, University of Washington, and a pioneer in ADPKD organoid technology. We intend to evaluate additional cellular and/or animal models of ADPKD and, with positive results, continue to advance this program toward an IND submission.
- The results of the completed renal impairment study and additional preclinical data in cystinosis were presented on November 7, 2019, at the American Society of Nephrology (ASN) Kidney Week Conference in Washington, DC in two posters titled:
  - **“An open label-single dose, parallel-group study to evaluate the effects of renal impairment on the pharmacokinetics of ELX-02: Results from subjects with mild and moderate renal impairment”**
  - **“Cystinosis nonsense mutation read-through mediated by ELX-02 restores protein function using in vitro and in vivo models”**

#### Additional Development Programs

- We have continued to develop our library of molecules and believe that there are multiple opportunities to expand our pipeline by advancing these novel molecules in new routes of administration and by addressing new therapeutic indications.



- In our inherited retinal disease program, we have reported that multiple ERSG compounds have demonstrated dose-dependent read-through using our in vitro assay platform, and an acceptable intravitreal tolerability in animal models. We have achieved an important preclinical milestone demonstrating an increase in pigment, an indication of functional restoration of OCA2, after a single intravitreal injection of Eloxx ERSGs. This outcome demonstrates that ERSG compounds can reach inherited retinal disease-relevant tissue layers beyond the photoreceptors.
- We will present preclinical data in a scientific presentation at the **Association for Research in Vision 2020 (ARVO 2020)** Meeting on May 3 – 7, 2020 in Baltimore, MD.

ELX-02 is an investigational agent not approved by any regulatory agency for therapeutic use which is currently in Phase 2 clinical trials in cystic fibrosis and cystinosis.

#### Fourth Quarter 2019 Financial Results

As of December 31, 2019, we had cash, cash equivalents and marketable securities of \$56.3 million, which we expect will be sufficient to fund our operations through the end of 2021, well beyond topline data from our Phase 2 clinical trials in cystic fibrosis.

For the three months ended December 31, 2019, we incurred a loss of \$11.6 million or \$0.29 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 \$14.0 million, or \$0.40 per share.

Our research and development expenses were \$5.7 million for the three months ended December 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 \$6.5 million. The quarter to quarter decrease in R&D expenditures was driven by the timing of significant activities in our Phase 2 clinical trials, along with pre-clinical and CMC costs, and lower non-cash stock compensation.

Our general and administrative expenses were \$5.8 million for the three months ended December 31, 2019 which includes \$2.2 million in non-cash expense related to stock-based compensation. For the same period in the prior year, G&A expenses were \$7.6 million. The change was primarily driven by decreases in professional service fees and non-cash stock compensation expense.

#### Full Year 2019 Financial Results

For the twelve months ended December 31, 2019, we incurred a loss of \$50.9 million or \$1.34 per share, which includes \$11.3 million in non-cash expense related to stock-based compensation. For the prior year, we incurred a net loss of \$47.2 million, or \$1.45 per share.

Our research and development expenses were \$25.8 million for the twelve months ended December 31, 2019 which includes \$2.5 million in non-cash expense related to stock-based compensation. For the same period in the prior year, R&D expenses were \$20.5 million. The year over year increase in R&D expenditures was driven primarily by growth in salaries and other personnel costs and increases in clinical operations costs related to our Phase 2 clinical trials.

Our general and administrative expenses were \$24.7 million for the twelve months ended December 31, 2019 which includes \$8.9 million in non-cash stock-based compensation. For the prior year, G&A expenses were \$26.5 million. The year over year decline in our G&A expenses was primarily related to lower non-cash expense related to stock-based compensation and a decrease in infrastructure-related costs offset by increases in salaries and other personnel costs.

#### Conference Call and Webcast Information:

**Date:** Thursday, March 5, 2020

**Time:** 4:30 p.m. ET

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

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

**Conference ID:** 3149964

**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/mmc/p/8d96keug>

#### 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)

SOURCE: Eloxx Pharmaceuticals, Inc.

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

	December 31,	
	2019	2018
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 22,493	\$ 48,606
Marketable securities	33,783	—
Restricted bank deposit	43	45
Prepaid expenses and other current assets	1,390	1,690
Total current assets	57,709	50,341
Property and equipment, net	201	248
Operating lease right-of-use asset	924	—
Other long-term assets	113	129
Total assets	<u>\$ 58,947</u>	<u>\$ 50,718</u>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable	\$ 1,871	\$ 747
Accrued expenses	4,655	6,938
Current portion of long-term debt	4,336	—
Advances from collaboration partners	403	—
Current portion of operating lease liability	499	—
Taxes payable	43	122
Total current liabilities	11,807	7,807
Long-term debt	10,502	—
Operating lease liability	425	—
Total liabilities	<u>22,734</u>	<u>7,807</u>
Stockholders' equity:		
Preferred stock, \$0.01 par value per share, 5,000,000 shares authorized, no shares issued or outstanding at December 31, 2019 and 2018	—	—
Common stock, \$0.01 par value per share, 500,000,000 shares authorized, 40,186,469 and 35,951,537 shares issued and 40,030,763 and 35,860,114 shares outstanding as of December 31, 2019 and 2018, respectively	402	360
Common stock in treasury, at cost, 155,706 and 91,423 shares as of December 31, 2019 and 2018, respectively	(1,703)	(1,129)
Additional paid-in capital	174,515	129,825



Accumulated other comprehensive income	18	—
Accumulated deficit	(137,019)	(86,145)
Total stockholders' equity	36,213	42,911
Total liabilities and stockholders' equity	<u>\$ 58,947</u>	<u>\$ 50,718</u>

**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,	
	2019	2018	2019	2018
Operating expenses:				
Research and development	\$ 5,682	\$ 6,530	\$ 25,842	\$ 20,489
General and administrative	5,806	7,584	24,713	26,482
Reverse merger related expenses	—	—	—	594
Total operating expenses	<u>11,488</u>	<u>14,114</u>	<u>50,555</u>	<u>47,565</u>
Loss from operations	(11,488)	(14,114)	(50,555)	(47,565)
Other expense (income), net	145	(209)	319	(502)
Loss before income taxes	(11,633)	(13,905)	(50,874)	(47,063)
Provision for income taxes	—	122	—	122
Net loss	<u>\$ (11,633)</u>	<u>\$ (14,027)</u>	<u>\$ (50,874)</u>	<u>\$ (47,185)</u>
Net loss per share, basic and diluted	<u>\$ (0.29)</u>	<u>\$ (0.40)</u>	<u>\$ (1.34)</u>	<u>\$ (1.45)</u>
Weighted-average number of shares of common stock used in computing net loss per share, basic and diluted	39,981,335	35,259,810	38,063,173	32,436,506



Source: Eloxx Pharmaceuticals