



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

November 5, 2019

*-Presented Positive Phase 1 Data from SAD and MAD Studies at North American Cystic Fibrosis Conference*

*-On track to report top line Phase 2 data in nephropathic cystinosis this quarter, preliminary results show cystine reduction with 1mg/kg dose*

*-Actively enrolling Phase 2 cystic fibrosis trial in IL with additional sites in EU and US to open this quarter. Expect fully enrolled Phase 2 cystic fibrosis trials in the first quarter of 2020*

*-Company to present Phase 1 Renal Impairment Study results and additional preclinical data at Kidney Week*

*-Maintained Strong Balance Sheet with \$64.9 Million in Cash and Equivalents as of September 30, 2019*

*-Company to host webcast and conference call today, Tuesday, November 5, 2019, at 4:30 pm ET*

WALTHAM, Mass., Nov. 05, 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 nine months ended September 30, 2019 and provided a business update.

"We are very pleased to have presented the results of our MAD study at NACFC which reinforced the overall favorable tolerability profile and pharmacokinetics of ELX-02, with no serious adverse events or nephrotoxicity. To date, ELX-02 safety and tolerability has been evaluated in 105 healthy volunteers," said Dr. Gregory Williams, Chief Operating Officer of Eloxx Pharmaceuticals. "We are encouraged by the preliminary results of our phase 2 clinical trial in nephropathic cystinosis, as ELX-02 has demonstrated a statistically significant reduction in white blood cell cystine levels at the second dose level of 1.0 mg/kg. We are on track to complete this cohort and report top line data this quarter. We believe that achievement of proof of concept for ELX-02 in cystinosis will provide a basis for expansion to studies of additional kidney diseases, as well as increasing our probability of success in other clinical uses across this dose range."

"This week the Eloxx team will be presenting the results from our completed Phase 1 Renal Impairment Study at Kidney Week. These data enable dose adjustment based on individual patient's kidney function," said Robert Ward, Chairman and CEO of Eloxx Pharmaceuticals. "We are pleased to be actively enrolling patients in our Cystic Fibrosis program, and we expect additional clinical trial sites to open in the US and EU this quarter and full enrollment to be achieved during the first quarter of 2020. With this change, we expect to report top line data from these trials in the first half of 2020."

During the quarter ended September 30, 2019, Eloxx appointed Dr. Thomas Haverty as our Chief Medical Officer. Dr. Haverty has over 30 years of pharmaceutical and biotechnology experience leading clinical research and operations teams with responsibility for large development portfolios in virtually all classes of molecules and indications. Prior to joining us in his current capacity, Dr. Haverty, a Board-certified Nephrologist, served as a consultant to us following a long tenured career at Johnson & Johnson, Schering Plough and Merck Research Labs. Dr. Haverty has successfully led the development and approval of over 20 leading drugs.

### Cystic Fibrosis Program Updates

- Our Phase 2 program consists of two trials, one for clinical investigators enrolling patients at sites in Europe and Israel. We are actively enrolling patients in Israel and expect to open European sites this quarter. Our second Phase 2 trial is in the U.S., where today, Boston Children's Hospital is open for enrollment and we are expecting additional U.S. clinical trial sites to open this quarter. The expansion of our cystic fibrosis program to the U.S. has been made possible in part by the support provided by the Cystic Fibrosis Foundation and by the sanctioning of our protocol by the Cystic Fibrosis Therapeutics Development Network (TDN).
- We are aware that there are available patients who have expressed interest in the trials and the extended period for opening clinical sites will mean that our cystic fibrosis trials will be fully enrolled during the first quarter of 2020. As a result, we are changing our guidance for top line data in cystic fibrosis to the first half of 2020.
- During October 2019, we completed an interim CMC review meeting with the U.S. Food and Drug Administration, and we have gained alignment with the agency on our manufacturing formulation process, which we believe will be suitable for our expected drug supply needs through completion of our pivotal trials.
- 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., and Dr. Eitan Kerem, Head of the Division of Pediatrics, Children's Hospital, Hadassah Medical Center, will serve as the Global Lead Investigator.

- We are pleased with our participation in the European HIT-CF program and the progress being made. The program will conduct experiments with ELX-02 in organoids from cystic fibrosis patients with nonsense mutations who are participating in the program.
- We presented positive data in three scientific presentations at the North American Cystic Fibrosis Conference on October 31-November 2, 2019 in Nashville, Tennessee, from our completed Phase 1 clinical trial program for ELX-02, including results from the SAD study, the MAD study, and additional preclinical data for ELX-02. These data demonstrated that ELX-02 increases functional CFTR protein in patient-derived organoids and human bronchial epithelial cells, using chamber systems, FRT and transgenic mice, and that it restores CFTR mRNA to healthy control levels. ELX-02 results in a pronounced increase in both CFTR protein expression and mRNA stability and the preclinical efficacy-associated exposures from the completed MAD study translate to the selected Phase 2 clinical trial ascending dose ranges and exposures. ELX-02 shows linear and dose proportional PK following subcutaneous administration twice weekly. To date, ELX-02 has been generally well tolerated in clinical studies, with 105 volunteers exposed, no reported SAEs or renal findings.

#### Cystinosis Program Updates

- Our Phase 2 cystinosis trial involves two sequential cohorts of three patients with three escalating doses. A cohort is complete when each patient has escalated through each of the three dosing levels or if the Safety Review Committee (SRC) recommends halting escalation. Following the completion of each dose, the SRC meets to review the patient safety data prior to escalation to the next dose level. The first two doses in the first cohort are complete and the SRC has authorized us to start the final dose in this first cohort which is currently ongoing. To date, based upon preliminary results, ELX-02 has been well tolerated through the first two dose levels, and at the second 1 mg/kg dose level, ELX-02 demonstrated a statistically significant reduction in white blood cell cystine levels. Upon completion of the first cohort, we will review with the Principal Investigator and conduct a separate review with a panel of cystinosis scientific and clinical experts before reporting top line data later this year.
- We believe that the emerging profile of ELX-02 is suitable for continued development and we intend to seek regulatory advice, following top line results, on initiating an extension study for patients in the first cohort and to expand the trial to include sites and patients in the United States. We believe that proof of concept for ELX-02 in cystinosis will provide a basis for expansion to studies of additional kidney diseases caused by nonsense mutations, as well as increase our probability of success in other clinical uses of this dosage range.
- Genome Quebec and Genome Canada have provided non-dilutive funding for the Phase 2 clinical trial of ELX-02 in cystinosis. The Cystinosis Research Foundation provided non-dilutive funding for the preclinical phase of the program.
- In support of the cystinosis program where many patients have impaired renal function, we have successfully completed a renal impairment study with ELX-02 in subjects with mild, moderate, and severe renal impairment. The results of the renal impairment study and additional preclinical data in cystinosis will be presented on Thursday, November 7, 2019, at the American Society of Nephrology (ASN) Kidney Week Conference in Washington, DC November 5-10, 2019 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”** - November 7, 2019 10:00 am – 12:00 pm
  - **“Cystinosis nonsense mutation read-through mediated by ELX-02 restores protein function using in vitro and in vivo models”** – November 7, 2019 10:00 am – 12:00 pm

#### 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 or by addressing new therapeutic indications. This quarter, we evaluated the three most prevalent autosomal dominant polycystic kidney disease (ADPKD) nonsense mutations in an in vitro read-through assay and have demonstrated significant levels of read-through for ELX-02 and several library compounds, which is the first step in our preclinical development toward IND. We intend to evaluate additional cellular and/or animal models for ADPKD and with positive results, advance towards IND.
- In our inherited retinal disease program, we are conducting feasibility and IND enabling studies for several ERSG compounds from our library. We believe that our intravitreal ERSG approach could provide restoration of critical proteins to

preserve or restore visual function across the nonsense related inherited retinal disorder landscape. We have demonstrated dose-dependent read-through for our compounds using our in vitro assay platform. During the quarter, we achieved another important milestone by demonstrating that several of our compounds successfully restored protein production in an animal model. We evaluated a mouse model with a naturally occurring nonsense mutation in the OCA2 gene which results in type 2 oculocutaneous albinism. In this model the R262X mutation results in a lack of OCA2 channel protein which is needed to establish the pH of an organelle that produces pigment, the melanocyte. Loss of the functional OCA2 protein results in a lack of pigment in the retinal pigment epithelium and the underlying choroid layers.

- Multiple Eloxx ERSG compounds have demonstrated an increase in pigment, an indication of functional restoration of OCA2, after a single intravitreal injection of Eloxx ERSGs. We have used a dose range of 50 to 200 nanograms per mouse eye which we believe provides a window for efficacy based on our current tolerability profile. This outcome demonstrates that ERSG compounds can reach inherited retinal disease-relevant tissue layers beyond the photoreceptors. These data support that ERSG compounds may be applicable in a wider range of inherited retinal disease that impact cells deep to the neurosensory retina, including the retinal pigment epithelium and choroid such as nonsense mediated Best and choroideremia. Furthermore, we are encouraged that this is possible through intravitreal delivery, which is the most common and least technical administration and provides a global distribution of the compound to the retina.
- This work has helped us progress our efforts to produce a sustained release formulation for the program, and we are currently evaluating sustained release technologies and potential partners.
- We continue to collaborate and engage in our multi-year partnership with the Foundation Fighting Blindness (FFB) to support its inherited retinal degenerative disease registry and educational programs. In October 2019, we presented as part of the FFB New York Vision Seminar and we believe that the ongoing research and development consultation and support provided by the FFB will accelerate our development programs that seek to support patients with ocular disorders associated with nonsense mutations, an area of high unmet medical need. At the FFB New York Vision Seminar, we presented some of our *in vivo* proof-of-concept data in a nonsense model of oculocutaneous albinism type 2.

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.

### Third Quarter 2019 Financial Results

As of September 30, 2019, we had cash, cash equivalents and marketable securities of \$64.9 million, which we expect will be sufficient to fund our operations through top line data from our Phase 2 clinical trials in cystic fibrosis and cystinosis and our current and planned operations into the first quarter of 2021.

For the three months ended September 30, 2019, we incurred a loss of \$12.9 million or \$0.32 per share, which includes \$3.0 million non-cash expense related to stock-based compensation. For the same period in the prior year, we incurred a net loss of \$11.2 million, or \$0.32 per share.

Our research and development expenses were \$6.8 million for the three months ended September 30, 2019 which includes \$0.7 million non-cash expense related to stock-based compensation. For the same period in the prior year, R&D expenses were \$5.4 million. Quarter to quarter increases in R&D expenditures were largely driven by preparations for our multiple Phase 2 clinical trials, along with pre-clinical and CMC operations.

Our general and administrative expenses were \$6.0 million for the three months ended September 30, 2019 which includes \$2.3 million in non-cash expense related to stock-based compensation. For the same period in the prior year, G&A expenses were \$5.9 million. Our G&A expenses were relatively flat year over year with higher professional service fees offset by a decrease in salary related costs.

### First Nine Months 2019 Financial Results

For the nine months ended September 30, 2019, we incurred a loss of \$39.2 million or \$1.05 per share, which includes \$8.6 million in non-cash expense related to stock-based compensation. For the same period in the prior year, we incurred a net loss of \$33.2 million, or \$1.05 per share.

Our research and development expenses were \$20.2 million for the nine months ended September 30, 2019 which includes \$2.0 million in non-cash expense related to stock-based compensation. For the same period in the prior year, R&D expenses were \$14.0 million. The year over year increase in R&D expenditures was driven primarily by growth in our clinical and preclinical operations.

Our general and administrative expenses were \$18.9 million for the nine months ended September 30, 2019 which includes \$6.6 million in non-cash stock-based compensation. For the same period in the prior year, G&A expenses were \$18.9 million. Our G&A expenses were relatively flat year over year despite increases in salary related costs reflective of our year over year headcount growth and increases in professional service fees, due to lower non-cash expense related to stock-based compensation in the 2019 period.

### Conference Call and Webcast Information:

**Date:** Tuesday, November 5, 2019

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

**Domestic Dial-in Number:** (866) 754-6374

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

**Conference ID:** 8339658

**Live Webcast:** accessible from the Company's website at [www.eloxxpharma.com](http://www.eloxxpharma.com) under Events and Presentations or with this link:

## 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 CONDENSED CONSOLIDATED BALANCE SHEETS

(Amounts in thousands, except share and per share data)

	September 30, 2019	December 31, 2018
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 22,155	\$ 48,606
Marketable securities	42,781	—
Restricted bank deposit	40	45
Prepaid expenses and other current assets	1,878	1,690
Total current assets	66,854	50,341
Property and equipment, net	220	248
Operating lease right-of-use asset	870	—
Other long-term assets	113	129
Total assets	<u>\$ 68,057</u>	<u>\$ 50,718</u>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable	\$ 1,687	\$ 747

Accrued expenses	5,145	6,938
Current portion of long-term debt	3,488	—
Advances from collaboration partners	403	—
Current portion of operating lease liability	461	—
Taxes payable	43	122
Total current liabilities	11,227	7,807
Long-term debt	11,193	—
Operating lease liability	409	—
Stockholders' equity:		
Preferred stock, \$0.01 par value per share, 5,000,000 shares authorized, no shares issued and outstanding at September 30, 2019 and December 31, 2018	—	—
Common stock, \$0.01 par value per share, 500,000,000 shares authorized, 40,117,186 and 35,951,537 shares issued and 39,977,654 and 35,860,114 shares outstanding as of September 30, 2019 and December 31, 2018, respectively	404	360
Common stock in treasury, at cost, 139,532 and 91,423 shares at September 30, 2019 and December 31, 2018, respectively	(1,585)	(1,129)
Additional paid-in capital	171,783	129,825
Accumulated other comprehensive income	12	—
Accumulated deficit	(125,386)	(86,145)
Total stockholders' equity	45,228	42,911
Total liabilities and stockholders' equity	\$ 68,057	\$ 50,718

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Operating expenses:				
Research and development	\$ 6,801	\$ 5,415	\$ 20,160	\$ 13,959
General and administrative	5,978	5,945	18,907	18,898
Reverse merger related expenses	—	—	—	594
Total operating expenses	12,779	11,360	39,067	33,451
Loss from operations	(12,779)	(11,360)	(39,067)	(33,451)
Other expense (income), net	96	(199)	174	(293)
Net loss	\$ (12,875)	\$ (11,161)	\$ (39,241)	\$ (33,158)
Basic and diluted net loss per share	\$ (0.32)	\$ (0.32)	\$ (1.05)	\$ (1.05)
Weighted average number of Common Shares used in computing basic and diluted net loss per share	39,944,324	35,005,979	37,394,310	31,485,067



Source: Eloxx Pharmaceuticals