



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

August 7, 2019

Phase 2 cystic fibrosis clinical trial IND is open in the U.S., protocol endorsed by the Cystic Fibrosis Foundation (CFF)

Initiated a Phase 2 clinical trial for ELX-02 in cystinosis in Canada and received non-dilutive funding from Genome Quebec and Genome Canada

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

Three abstracts for ELX-02 were accepted for presentation at the North American Cystic Fibrosis Conference, October 31-November 2, 2019

Company to host webcast and conference call on Wednesday, August 7, 2019 at 8:30 am ET

WALTHAM, Mass., Aug. 07, 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 six months ended June 30, 2019 and provided a business update.

"We are pleased to be rapidly advancing our Phase 2 programs for ELX-02. Our IND for a Phase 2 clinical trial in cystic fibrosis is open in the U.S. and the protocol has been endorsed by the Cystic Fibrosis Foundation (CFF). 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. "We have initiated a Phase 2 clinical trial in Canada in cystinosis and we look forward to reporting top line data from both of these programs this year."

"We are gratified to have two leading CF experts leading our Phase 2 clinical trial of ELX-02 in cystic fibrosis patients: Dr. Ahmet Uluer, Director of the Adult Cystic Fibrosis Program at Boston Children's Hospital/ Brigham and Women's Hospital CF Center, has agreed to be 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, said Dr. Greg Williams, Chief Operating Officer of Eloxx. "ELX-02 is the only read-through agent to have demonstrated positive results in organoids derived from cystic fibrosis patients across the majority of nonsense mutations and studies have shown the organoid model to be highly predictive of clinical benefit. We believe these data de-risk our Phase 2 clinical trial program in cystic fibrosis."

Cystic Fibrosis Program Updates

- Our IND in the U.S. is open and the CFF has endorsed the protocol for our Phase 2 clinical in cystic fibrosis. We are on track to report top line data from a Phase 2 clinical trial in cystic fibrosis patients with the G542X CFTR mutation in the U.S. and Europe this year.
- Dr. Ahmet Uluer, Director of the Adult Cystic Fibrosis Program at the Boston Children's Hospital/ Brigham and Women's Hospital CF Center, will be 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.
- Our Phase 2 program will include up to 24 patients in the U.S., Europe and Israel. The protocol calls for 4 increasing doses of ELX-02 ranging from 0.3 up to 3.0 mg/kg/day in order to identify an optimal dose to carry into further development. While patient safety is the primary endpoint in Phase 2, we will be evaluating changes in sweat chloride at multiple ascending doses of ELX-02 as the primary biomarker, which is consistent with other successful Phase 2 programs for approved drugs to measure CFTR activity. We will also be evaluating changes in FEV1.
- We are pleased with our participation in the European HIT-CF program and the progress being made. The program is now in the screening phase and has already identified around 40 cystic fibrosis patients with nonsense mutations who are participating in organoid development and further testing.
- Three abstracts have been accepted for presentation at the North American Cystic Fibrosis Conference on October 31st through November 2nd, 2019 in Nashville, Tennessee:
 - **"Pharmacokinetics, Safety, and Tolerability of Single Ascending Doses of ELX-02 in Healthy Volunteers, a Potential Treatment for Cystic Fibrosis Caused by Nonsense Mutations"** Thursday, October 31, 2019 11:15 a.m. – 1:45 p.m. CDT, Poster Session 1
 - **"Pharmacokinetics, Safety, and Tolerability of Multiple Ascending Doses of ELX-02 in Healthy Volunteers, a**

Potential Treatment for Cystic Fibrosis Caused by Nonsense Mutations”Thursday, October 31, 201911:15 a.m. – 1:45 p.m. CDT, Poster Session 1

- **“Investigational Drug ELX-02 Mediates CFTR Nonsense Mutation Read-through to Increase CFTR MRNA, CFTR Protein Translation and CFTR Function” Friday, November 1, 20192:15 p.m. – 3:50 p.m. CDT, Oral Workshop “New & Emerging Therapies to Correct the Basic Defect” Workshop**

Cystinosis Program Updates

- We have initiated a Phase 2 clinical trial in cystinosis in Canada. Dr. Paul Goodyer, a Professor of Pediatrics at McGill University and recognized leader in hereditary renal disease, is the principal investigator. The Phase 2 clinical trial is a single arm, open label study designed to assess the safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of subcutaneous (SC) ELX-02 in 6 patients with nephropathic cystinosis with at least 1 nonsense mutation in the cystinosis gene. The study will measure the dose-dependent effect of ELX-02 on cysteine levels in white blood cells, the biomarker used in the development of the most recently approved drugs for cystinosis. The study will include three nominal doses ranging from 0.5 to 2.0 mg/kg/day in order to identify an optimal dose to carry into further development.
- Genome Quebec and Genome Canada are providing 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.
- We expect to report top line data in cystinosis early in the fourth quarter of 2019.
- 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. To date, the preliminary results support continuing our existing clinical programs and expanding our research in other kidney disorders such as autosomal dominant polycystic kidney disease. We expect to present the results from the completed renal impairment study at a scientific meeting later this year.

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

Second Quarter 2019 Financial Results

As of June 30, 2019, we had cash, cash equivalents and marketable securities of \$76.3 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 in 2019 and our current and planned operations into the first quarter of 2021.

For the three months ended June 30, 2019, we incurred a loss of \$14.4 million or \$0.40 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 \$13.4 million, or \$0.42 per share.

Our research and development expenses were \$7.3 million for the three months ended June 30, 2019 which includes \$2.3 million non-cash expense related to stock-based compensation. For the same period in the prior year, R&D expenses were \$4.2 million. Quarter over quarter increases in R&D expenditures were driven by our Phase 1 clinical studies, the renal impairment study, and preparations for our multiple Phase 2 clinical trials, along with pre-clinical and CMC operations.

Our general and administrative expenses were \$7.0 million for the three months ended June 30, 2019 which includes \$0.7 million in non-cash expense related to stock-based compensation. For the same period in the prior year, G&A expenses were \$9.6 million. The year over year decrease in our G&A expenses was primarily related to lower non-cash expense related to stock-based compensation in the 2019 period, partially offset by an increase in salary related costs reflective of our year over year headcount growth and increases in professional service fees.

First Half 2019 Financial Results

For the six months ended June 30, 2019, we incurred a loss of \$26.4 million or \$0.73 per share, which includes \$5.7 million in non-cash expense related to stock-based compensation. For the same period in the prior year, we incurred a net loss of \$22.0 million, or \$0.74 per share.

Our research and development expenses were \$13.3 million for the six months ended June 30, 2019 which includes \$4.4 million in non-cash expense related to stock-based compensation. For the same period in the prior year, R&D expenses were \$8.5 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 \$12.9 million for the six months ended June 30, 2019 which includes \$1.3 million in non-cash stock-based compensation. For the same period in the prior year, G&A expenses were \$13.0 million. Lower non-cash expense related to stock-based compensation million in the 2019 period, was partially offset by an increase in salary related costs reflective of our year over year headcount growth and increases in professional service fees.

Conference Call Information:

Date: Wednesday, August 7, 2019

Time: 8:30 a.m. ET

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

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

Conference ID: 1754316

Live Webcast: accessible from the Company's website at www.eloxpharma.com under Events and Presentations or with this

link: <https://edge.media-server.com/mmc/p/9axiqnvt>

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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SOURCE: Eloxx Pharmaceuticals, Inc.

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

	June 30, 2019	December 31, 2018
ASSETS		
Current assets:		
Cash and cash equivalents	\$ 49,326	\$ 48,606
Marketable securities	26,973	—
Restricted bank deposit	45	45
Prepaid expenses and other current assets	1,392	1,690
Total current assets	77,736	50,341
Property and equipment, net	214	248
Operating lease right-of-use asset	989	—
Other long-term assets	100	129
Total assets	\$ 79,039	\$ 50,718
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	\$ 2,473	\$ 747
Accrued expenses	5,773	6,938
Current portion of long-term debt	2,231	—

Current portion of operating lease liability	473	—
Taxes payable	43	122
Total current liabilities	<u>10,993</u>	<u>7,807</u>
Long-term debt	12,306	—
Operating lease liability	516	—
Stockholders' equity:		
Common stock, \$0.01 par value per share, 500,000,000 shares authorized, 40,043,365 and 35,951,537 shares issued and 39,914,668 and 35,860,114 shares outstanding as of June 30, 2019 and December 31, 2018, respectively	402	360
Common stock in treasury, at cost, 128,697 and 91,423 shares at June 30, 2019 and December 31, 2018, respectively	(1,539)	(1,129)
Additional paid in capital	168,848	129,825
Accumulated other comprehensive income	24	—
Accumulated deficit	(112,511)	(86,145)
Total stockholders' equity	<u>55,224</u>	<u>42,911</u>
Total liabilities and stockholders' equity	<u>\$ 79,039</u>	<u>\$ 50,718</u>

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Operating expenses:				
Research and development	\$ 7,340	\$ 4,150	\$ 13,359	\$ 8,544
General and administrative	6,971	9,560	12,929	12,953
Reverse merger related expenses	—	(167)	—	594
Total operating expenses	<u>14,311</u>	<u>13,543</u>	<u>26,288</u>	<u>22,091</u>
Loss from operations	(14,311)	(13,543)	(26,288)	(22,091)
Other expense (income), net	138	(137)	78	(94)
Net loss	<u>\$ (14,449)</u>	<u>\$ (13,406)</u>	<u>\$ (26,366)</u>	<u>\$ (21,997)</u>
Basic and diluted net loss per share	<u>\$ (0.40)</u>	<u>\$ (0.42)</u>	<u>\$ (0.73)</u>	<u>\$ (0.74)</u>
Weighted average number of Common Shares used in computing basic and diluted net loss per share	36,278,567	31,839,303	36,098,171	29,695,430



Source: Eloxx Pharmaceuticals