



Eloxx Pharmaceuticals Reports First Quarter 2020 Financial and Operating Results and Provides Business Update

May 7, 2020

Completing our Phase 2 clinical trial program for ELX-02 in Cystic Fibrosis remains our highest priority

Preclinical studies advancing in autosomal dominant polycystic kidney disease (ADPKD) and inherited retinal disorders

Strong balance sheet with \$44 million in cash and cash equivalents as of March 31, 2020 provides cash runway through the end of 2021

Company to host webcast and conference call today, Thursday, May 7, 2020, at 4:30 pm ET

WALTHAM, Mass., May 07, 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 months ended March 31, 2020 and provided a business update.

"Our highest priority is to resume and complete our Phase 2 proof of concept clinical trial program for ELX-02 in cystic fibrosis, as we believe these data represent a substantial value inflection point for the Company. As previously announced, these trials have been temporarily paused in response to the COVID-19 global pandemic in support of global healthcare providers and our shared commitment to ensure patient safety," said Dr. Gregory Williams, Chief Executive Officer of Eloxx Pharmaceuticals. "We are working very closely with our clinical investigators and study sites to ensure that we can resume and complete our Phase 2 trials as rapidly as possible and report top line results."

Company Updates

- We are pleased to announce today that our scientific manuscript titled: "**ELX-02 generates protein via premature stop codon read-through without inducing native stop codon read-through protein**" has been accepted for publication by the **Journal of Pharmacology and Experimental Therapeutics**. This manuscript demonstrates that while ELX-02 mediates read-through of premature stop codons, the fidelity of stop codons found at the end of healthy transcripts is maintained. This indicates that translation integrity is preserved with target-therapeutic exposure of ELX-02, consistent with the favorable tolerability profile across our preclinical and clinical datasets. The pre-publication version of the manuscript can be found within the "Fast Forward" section of the Journal's website.
- In April 2020, we applied for and received a loan of approximately \$800,000 through the U.S. SBA's "Paycheck Protection Program", which was a component of the CARES Act, signed into law in late March. PPP loans are eligible for partial forgiveness, which we will apply for, based on using the proceeds for payroll, maintaining headcount, and other specified costs. The remaining balance of the loan bears interest at the rate of 1% and is to be repaid commencing at the end of 2020.
- On March 25, 2020, we announced that enrollment in our Phase 2 clinical trials for ELX-02 in cystic fibrosis has been temporarily paused in response to the COVID-19 global pandemic. Our goals are to avoid unnecessary exposure in at-risk populations, to maintain the integrity of our study data and to support global healthcare providers in their commitment to ensure patient safety. Public health authorities worldwide have recommended that people at high risk stay at home as much as possible, cancel non-essential doctor's visits and avoid unnecessary exposure to people and public spaces. Cystic fibrosis patients, especially those with nonsense mutations, have compromised lung function and may be at increased risk of severe illness in the event of a COVID-19 infection.
- On February 24, 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.

Cystic Fibrosis Phase 2 Program

- Our Phase 2 program consists of two trials, one enrolling patients at sites in Europe and Israel and the second in the U.S.
 - o In the U.S., partial funding is being 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).

o 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.
- 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. 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.

ADPKD Kidney Program Update

- ELX-02 results from the first cohort of the Phase 2 study in the treatment of patients with nonsense mutation-mediated nephropathic cystinosis met the primary safety endpoint and the reductions in white blood cell cystine provided a clear indication of biologic activity. These data 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 provide the basis for expanding our studies to additional kidney diseases caused by nonsense mutations, such as ADPKD.
 - o 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 are pleased to report that using a reporter assay we have already observed dose-dependent read-through with our ERSGs across the most common PKD1 alleles and have now demonstrated dose-dependent read-through across the most common PKD2 alleles. We are now applying this information to our functional model efforts in order to confirm that the read-through we observe has an impact on cyst formation and growth. Our Cystic Fibrosis platform has highlighted the utility of organoid technology to assess function in a translational model. Similarly, for ADPKD, organoids derived from patient cells or induced pluripotent stem cells can be differentiated in a manner that recapitulates the cellular diversity of the kidney and generate the cysts characteristic of the disease state.
- Using a patient-derived organoid with the most common PKD2 nonsense allele, we have observed encouraging results of reduced cystogenesis.
- Results of our preclinical research to date demonstrate that a read-through approach can have a direct impact on a meaningful metric of ADPKD progression, cyst number. We intend to evaluate additional models of ADPKD and, with positive results, advance toward an IND submission.

Ocular Program Update

- In our ocular program, focusing on inherited retinal disorders, 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 disorder-relevant tissue layers beyond the photoreceptors.
- On May 6, 2020, we presented new preclinical data in a scientific presentation at the **Association for Research in Vision 2020 (ARVO 2020) Virtual Meeting** in a presentation entitled “**Intravitreal administration of small molecule read-through agents demonstrate functional activity in a nonsense mutation mouse model**”. This presentation described our studies in a mouse model with a naturally occurring nonsense mutation in the OCA2 gene which results in a form of albinism present in human 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 the organelle that produces pigment, the melanosome. The results showed a significant increase in melanin production which validates the potential to promote read-through activity in our target tissue via intravitreal injection.
- Our intravitreal read-through approach provides the opportunity to reach the totality of the retina. To extend the duration of

the delivery, our team is actively working to achieve a desired sustained release formulation. We are exploring several biodegradable, controlled release, polymer technologies and are encouraged by the in vitro release rates achieved to date, which are consistent with our target release profile of one to three months.

- When our tissue exposure data is coupled with our ongoing sustained release formulation efforts and the read-through potential we observe against nonsense mutations in disease causative genes such as USH2a, Myo7a, CEP290 and PDE6B, we are encouraged that the intravitreal ERSG approach could provide restoration of critical proteins to preserve or restore visual function across nonsense-related inherited retinal disorders.

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.

First Quarter 2020 Financial Results

As of March 31, 2020, we had cash, cash equivalents and marketable securities of \$44.0 million, which we expect will be sufficient to fund our operations through the end of 2021. The cash balance as of March 31, 2020 does not include the loan of \$800,000 which we received through the U.S. SBA's "Paycheck Protection Program."

For the three months ended March 31, 2020, we incurred a loss of \$13.9 million or \$0.35 per share, which includes \$4.0 million in restructuring charges associated with our realignment, \$2.1 million of which was non-cash stock-based compensation. Net loss also includes \$1.9 million in non-cash stock-based compensation from ongoing operations. For the same period in the prior year, we incurred a net loss of \$11.9 million, or \$0.33 per share.

Our research and development expenses were \$4.5 million for the three months ended March 31, 2020, which includes \$0.2 million in non-cash expense related to stock-based compensation. For the same period in the prior year, R&D expenses were \$6.0 million. The quarter to quarter decrease in R&D expenses of \$1.5 million was driven by reduced professional service fees and stock-based compensation, offset by an increase in headcount and related salaries for a portion of the 2020 period.

Our general and administrative expenses were \$5.2 million for the three months ended March 31, 2020, which includes \$1.7 million in non-cash expense related to stock-based compensation. For the same period in the prior year, G&A expenses were \$6.0 million. The decrease was primarily driven by lower non-cash stock-based compensation and other infrastructure-related costs.

Conference Call and Webcast Information:

Date: Thursday, May 7, 2020

Time: 4:30 p.m. ET

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

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

Conference ID: 7798542

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/mmc/p/tgendj2c>

Eloxx Pharmaceuticals

Eloxx Pharmaceuticals, Inc. is a clinical-stage biopharmaceutical company developing novel RNA-modulating drug candidates (each designed to be a eukaryotic ribosomal selective glycoside, or ERSG) 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, currently focusing on cystic fibrosis. 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 ERSG drug candidates 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 and Morristown, NJ. 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; the impact of global health concerns, such as the COVID-19 global pandemic, on our ability to continue our clinical and preclinical programs and otherwise operate our business effectively; 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:

SOURCE: Eloxx Pharmaceuticals, Inc.

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

	March 31, 2020	December 31, 2019
ASSETS		
Current assets:		
Cash and cash equivalents	\$ 25,875	\$ 22,493
Marketable securities	18,082	33,783
Restricted cash	43	43
Prepaid expenses and other current assets	1,927	1,390
Total current assets	45,927	57,709
Property and equipment, net	182	201
Operating lease right-of-use asset	822	924
Other long-term assets	110	113
Total assets	<u>\$ 47,041</u>	<u>\$ 58,947</u>
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	\$ 1,389	\$ 1,871
Accrued expenses	3,568	4,655
Current portion of long-term debt	4,772	4,336
Advances from collaboration partners	805	403
Current portion of operating lease liability	507	499
Taxes payable	43	43
Total current liabilities	11,084	11,807
Long-term debt	9,385	10,502
Operating lease liability	315	425
Total liabilities	20,784	22,734
Stockholders' equity:		
Preferred stock, \$0.01 par value per share, 5,000,000 shares authorized, no shares issued or outstanding as of March 31, 2020 or December 31, 2019	—	—
Common stock, \$0.01 par value per share, 500,000,000 shares authorized, 40,316,034 and 40,186,469 shares issued and 40,125,454 and 40,030,763 shares outstanding as of March 31, 2020 and December 31, 2019, respectively	403	402
Common stock in treasury, at cost, 190,580 and 155,706 shares as of March 31, 2020 and December 31, 2019, respectively	(1,819)	(1,703)
Additional paid-in capital	178,573	174,515
Accumulated other comprehensive income	65	18
Accumulated deficit	(150,965)	(137,019)
Total stockholders' equity	26,257	36,213
Total liabilities and stockholders' equity	<u>\$ 47,041</u>	<u>\$ 58,947</u>

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

	Three Months Ended March 31,	
	2020	2019
Operating expenses:		
Research and development	\$ 4,549	\$ 6,019
General and administrative	5,224	5,958
Restructuring charges	3,994	—
Total operating expenses	<u>13,767</u>	<u>11,977</u>
Loss from operations	(13,767)	(11,977)
Other expense (income), net	179	(60)
Net loss	<u>\$ (13,946)</u>	<u>\$ (11,917)</u>
Net loss per share, basic and diluted	<u>\$ (0.35)</u>	<u>\$ (0.33)</u>
Weighted average number of shares of common stock used in computing net loss per share, basic and diluted	40,074,275	35,910,270



Source: Eloxx Pharmaceuticals