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

August 6, 2020

Enrollment in our Phase 2 clinical trial program for ELX-02 in cystic fibrosis has been resumed in Europe and Israel after being temporarily paused due to the COVID-19 pandemic

U.S. FDA has granted orphan drug designation for ELX-02 for the treatment of cystic fibrosis

Completing enrollment in our Phase 2 cystic fibrosis clinical trials and reporting top line data remain our highest priorities

Preclinical studies continuing to advance in autosomal dominant polycystic kidney disease (ADPKD) and inherited retinal disorders

Cash and cash equivalents of $37.1 million as of June 30, 2020 provides cash runway through the end of 2021

Company to host webcast and conference call today, Thursday, August 6, 2020, at 2:00 pm ET

WALTHAM, Mass., Aug. 06, 2020 (GLOBE NEWSWIRE) -- August 6, 2020 – 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 six months ended June 30, 2020 and provided a business update.

"We are pleased that enrollment in our Phase 2 cystic fibrosis clinical trial has been resumed in Europe and Israel after having been temporarily paused in response to the COVID-19 pandemic in support of global healthcare providers and our shared commitment to ensure patient safety. In the U.S., we are gratified to have received orphan drug designation for ELX-02 for cystic fibrosis from the FDA which confers substantial benefits to the program including seven years of exclusivity upon marketing approval and a potential waiver of the PDUFA application fee," said Dr. Gregory Williams, Chief Executive Officer of Eloxx Pharmaceuticals. "We are working very closely with our clinical investigators and study sites and evaluating additional sites where patient enrollment may be feasible. Our highest priority is to complete our Phase 2 proof of concept clinical trial program for ELX-02 in cystic fibrosis as soon as possible, as we believe the data from these trials will represent a substantial value inflection point for the Company."

Company Updates

- On August 4, 2020, we announced that the U.S. FDA had granted orphan drug designation for ELX-02 for the treatment of cystic fibrosis. The orphan drug designation confers several important benefits to support development for medicines for underserved patient populations, or rare disorders, that affect fewer than 200,000 people in the U.S. Orphan drug designation qualifies Eloxx for certain benefits, including eligibility for marketing exclusivity for seven years post approval, tax credits on qualified U.S. clinical trial expenses, potential grant funding opportunities that can be used for clinical trials and a potential waiver of the PDUFA application fee, which is currently set at just under $3 million dollars. ELX-02 had previously been granted orphan medicinal product designation by the European Medicines Agency for the treatment of cystic fibrosis.

- Our scientific manuscript titled: "**ELX-02 generates protein via premature stop codon read-through without inducing native stop codon read-through protein**" has been published in the August 2020 edition of 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.

- On June 17, 2020, we announced that enrollment in our cystic fibrosis Phase 2 clinical trial in Europe and Israel had been resumed, while the trial in the U.S. remains paused due to the COVID-19 pandemic.

- On May 6, 2020, we presented new preclinical data in a scientific presentation at the virtual **Association for Research in Vision 2020 (ARVO 2020) Annual Meeting** in a presentation entitled "**Intravitreal administration of small molecule read-through agents demonstrate functional activity in a nonsense mutation mouse model**". The studies demonstrated restoration of melanin production in a nonsense mouse model and helped validate the potential to promote read-through activity in ocular tissues via intravitreal injection.

- 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.

Cystic Fibrosis Phase 2 Program

- Our Phase 2 program consists of two trials, one currently enrolling patients at sites in Europe and Israel and the second in the U.S., where enrollment remains temporarily paused due to the COVID-19 pandemic.
    - 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).
    - 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 participating 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

- 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. Using a patient-derived organoid with the most common PKD2 nonsense allele, we have repeated encouraging results of reduced cystogenesis and also observe a reduction in cyst size. These results demonstrate that a read-through approach potentially can have a direct impact on meaningful metrics of ADPKD progression, cyst number and size.
- We continue our effort in establishing and evaluating functional models of ADPKD in order to confirm that the read-through we observe has an impact on cyst formation and growth. 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.

Ocular Program Update

- In our ocular program focusing on inherited retinal disorders, our library of compounds has demonstrated dose-dependent read-through using our in vitro assay platform, acceptable intravitreal tolerability and restored protein production in an animal model via ERSG intravitreal injection. Our intravitreal read-through approach provides the opportunity to reach the totality of the retina. We achieved an important preclinical milestone which we reported on at this year's virtual ARVO Meeting by showing an increase in pigment, an indication of functional restoration of OCA2, after a single intravitreal injection of Eloxx ERSG compounds. This outcome demonstrates that ERSG compounds can reach inherited retinal disorder-relevant tissue layers beyond the photoreceptors.
- While we continue in our ocular program efforts, we are gratified to observe that other researchers are also reporting on the exciting potential of Eloxx compounds in this area. For example, the group of Bikash Pattnaik at the University of Wisconsin-Madison recently published in the American Journal of Human Genetics that ELX-03 (also known as NB84) was sufficient to rescue Kir7.1 channel function in a nonsense allele cellular model derived from an individual with a form of pediatric blindness, Lebers congenital amaurosis. These promising results represent another example of how intravitreal delivery of an ERSG may have broad application across nonsense-related inherited retinal disorders through restoring production of essential proteins.
- We are actively working to develop a sustained release formulation for intravitreal injection and are exploring several biodegradable, controlled release technologies. We 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, 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 authority for therapeutic use, which is currently in Phase 2 clinical trials in cystic fibrosis.

Second Quarter 2020 Financial Results

As of June 30, 2020, we had cash, cash equivalents and marketable securities of $37.1 million, which we expect will be sufficient to fund our operations through the end of 2021.

For the three months ended June 30, 2020, we incurred a loss of $7.9 million or $0.20 per share, which includes $2.0 million in non-cash stock-based compensation. For the same period in the prior year, we incurred a net loss of $14.4 million, or $0.40 per share.

Our research and development expenses were $3.5 million for the three months ended June 30, 2020, which includes $0.3 million in non-cash expense related to stock-based compensation. For the same period in the prior year, R&D expenses were $7.3 million. The quarter to quarter decrease in R&D expenses of $3.8 million was driven by reduced professional service fees largely due to the pause in our clinical trials due to COVID-19, and reduced headcount and related salaries for a portion of the 2020 period.

Our general and administrative expenses were $4.1 million for the three months ended June 30, 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 $7.0 million. The decrease was primarily driven by lower headcount and professional services costs.

First Half 2020 Financial Results

For the six months ended June 30, 2020, we incurred a loss of $21.8 million or $0.54 per share, which includes a one-time restructuring charge of $4.0 million associated with our realignment in the first quarter (comprised of $2.1 in non-cash stock based compensation and $1.9 million in cash severance) and $3.8 million in non-cash stock-based compensation associated with ongoing operations. For the same period in the prior year, we incurred a net loss of $26.4 million, or $0.73 per share.

Our research and development expenses were $8.1 million for the six months ended June 30, 2020, which includes $0.5 million in non-cash expense related to stock-based compensation. For the same period in the prior year, R&D expenses were $13.4 million. The year over year decrease in R&D expenses of $5.3 million was driven by reduced professional service fees largely due to the pause in our clinical trials due to COVID-19, and reduced headcount and related salaries for a portion of the 2020 period.

Our general and administrative expenses were $9.3 million for the six months ended June 30, 2020, which includes $3.4 million in non-cash expense related to stock-based compensation. For the same period in the prior year, G&A expenses were $12.9 million. The decrease of $3.6 million was primarily driven by lower headcount and professional services costs.

Conference Call and Webcast Information:

Date: Thursday, August 6, 2020

Time: 2:00 p.m. ET

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

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

Conference ID: 2191126

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/nxdhnad8.

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 authority. Eloxx's preclinical candidate pool consists of a library of novel ERSG drug candidates identified based on read-through potential. Eloxx also has preclinical programs focused on kidney diseases including autosomal dominant polycystic kidney disease, as well as 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.

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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, 2020	December 31, 2019
ASSETS		
Current assets:		
Cash and cash equivalents	$ 30,347	$ 22,493
Marketable securities	6,769	33,783
Restricted cash	52	43
Prepaid expenses and other current assets	1,800	1,390
Total current assets	38,968	57,709
Property and equipment, net	165	201
Operating lease right-of-use asset	678	924
Other long-term assets	110	113
Total assets	$ 39,921	$ 58,947
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 770	$ 1,871
Accrued expenses	3,485	4,655
Current portion of long-term debt	5,149	4,336
Advances from collaboration partners	805	403
Current portion of operating lease liability	524	499
Taxes payable	38	43
Total current liabilities	10,771	11,807
Long-term debt	8,704	10,502
Operating lease liability	155	425
Total liabilities	19,630	22,734
Stockholders' equity:		
Preferred stock, $0.01 par value per share, 5,000,000 shares authorized, no shares issued or outstanding as of June 30, 2020 or December 31, 2019	—	—
Common stock, $0.01 par value per share, 500,000,000 shares authorized, 40,326,906 and 40,186,469 shares issued, and 40,135,290 and 40,030,763 shares outstanding as of June 30, 2020 and December 31, 2019, respectively	403	402

Common stock in treasury, at cost, 191,616 and 155,706 shares as of June 30, 2020 and December 31, 2019, respectively	(1,822)	(1,703)
Additional paid-in capital	180,549	174,515
Accumulated other comprehensive income	13	18
Accumulated deficit	(158,852)	(137,019)
Total stockholders' equity	20,291	36,213
Total liabilities and stockholders' equity	$ 39,921	$ 58,947

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Operating expenses:				
Research and development	$ 3,528	$ 7,340	$ 8,077	$ 13,359
General and administrative	4,058	6,971	9,282	12,929
Restructuring charges	—	—	3,994	—
Total operating expenses	7,586	14,311	21,353	26,288
Loss from operations	(7,586)	(14,311)	(21,353)	(26,288)
Other expense, net	301	138	480	78
Net loss	$ (7,887)	$ (14,449)	$ (21,833)	$ (26,366)
Net loss per share, basic and diluted	$ (0.20)	$ (0.40)	$ (0.54)	$ (0.73)
Weighted average number of shares of common stock used in computing net loss per share, basic and diluted	40,129,304	36,278,567	40,101,789	36,098,171

Source: Eloxx Pharmaceuticals