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

November 5, 2020

Expect to report top line data from our Phase 2 cystic fibrosis clinical trials in the first half of 2021

The independent Safety Review Committees for our Phase 2 cystic fibrosis clinical trial program have allowed dose escalation up to the highest dose level

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

Cash and cash equivalents of $30.6 million as of September 30, 2020 provides cash runway through the end of 2021

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

WALTHAM, Mass., Nov. 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 nine months ended September 30, 2020 and provided a business update.

"Having resumed our Phase 2 cystic fibrosis clinical trials in Europe, Israel and the U.S., we now expect to report top line data in the first half of 2021, contingent on no further disruptions due to the COVID-19 pandemic. It is our highest priority 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," said Dr. Gregory Williams, Chief Executive Officer of Eloxx Pharmaceuticals. "We are pleased that, following several planned meetings, the independent Safety Review Committees for this clinical trial program have allowed dose escalation up to the highest dose level and, to date, no drug related serious adverse events have been reported."

Company Updates

- On October 27, 2020, we announced the publication of two manuscripts on ELX-02 and our ERSG library in two leading journals.
    - A publication entitled: "ELX-02: an investigational read-through agent for the treatment of nonsense mutation-related genetic disease" in the *Expert Opinion on Investigational Drugs Journal.*
        - The review by Professor Eitan Kerem M.D., Senior Attending Physician at the Hadassah CF Center in Jerusalem, Israel and Senior Medical Consultant for Eloxx, details the development of ELX-02 for the restoration of functional protein in nonsense-mediated disease in support of our ongoing Phase 2 clinical trials in cystic fibrosis.
    - A publication entitled: "Intravitreal administration of small molecule read-through agents demonstrate functional activity in a nonsense mutation mouse model" was published in the *Journal of Experimental Eye Research.*
        - Multiple small molecules in our ERSG library mediate dose-dependent read-through at the back of the eye after a single intravitreal injection.
    - Collectively, these manuscripts demonstrate the wide-ranging potential of our small molecule read-through approach to rare genetic disorders mediated by nonsense mutations, from targeted delivery for inherited retinal disorders to systemic delivery for multi-system disorders like cystic fibrosis. IND enabling studies are also underway for our library of ERSG compounds in ADPKD.
- On October 22, 2020, we announced that we had presented data from two scientific abstracts at the North American Cystic Fibrosis Virtual Conference (NACFC). The two abstracts were also showcased in the NACFC virtual poster gallery and electronically published as a supplement to *Pediatric Pulmonology*.

  The details for the two ELX-02 poster presentations are:

  Poster Session Presentation Title: "**ELX-02 Generates Protein Via Premature Stop Codon Read-through Without**

Inducing Native Stop Codon Read-through Proteins"
Poster #: 433
Presenter: Dr. Dan Crawford, Eloxx Pharmaceuticals

○ ELX-02 produces significant read-through of premature stop codons leading to full length proteins, demonstrated using DMS-114 cells with the *R213X* nonsense mutation in the *TP53* gene.
○ Using three complementary techniques, no evidence of native stop codon read-through products could be detected. These data suggest that ELX-02 does not promote native stop codon read-through at concentrations relevant to premature stop codon read-though.
○ The results of studies are consistent with the acceptable tolerability profile of ELX-02 across preclinical and clinical studies to date.

Poster Session Presentation Title: "**CFTR Restoration By ELX-02 Across CF Nonsense Genotypes: Utilizing Patient-Derived Organoids to Survey Responsive Alleles**"
Poster #: 383
Presenter: Dr. Matthew Goddeeris, Eloxx Pharmaceuticals

○ The patient derived organoid *CFTR* FIS assay has enabled the screening of a wide selection of cystic fibrosis nonsense alleles representing >75% of the cystic fibrosis nonsense population. Using this method, we continue to identify new responsive genotypes.
○ The response of *W1282X* patient-derived organoids to ELX-02 mediated through read-through positively correlates with *CFTR* mRNA expression.
○ Increasing the available *CFTR* mRNA pool through inhibition of nonsense mediated decay has a synergistic effect on ELX-02 mediated functional *CFTR* read-through.
○ These results help guide the interpretation of the patient-derived organoid *CFTR* FIS assay data by highlighting the importance of considering *CFTR* expression differences across patient-derived organoids for the applicability of ELX-02 as a potential therapeutic option for cystic fibrosis patients with nonsense alleles.

- On September 14, 2020, we announced that Professor Eitan Kerem, M.D. had joined the Company as a Senior Consultant. Dr. Kerem will continue to advise on our cystic fibrosis program focusing on strategic leadership, cystic fibrosis medical safety review and medical communications, as well as liaising with patient advocacy groups and regulatory authorities. Dr. Kerem joined Eloxx upon his retirement from Hadassah Medical Center where he most recently served as Head of Pediatrics and Professor of Pediatrics at Hebrew University Hadassah Medical School. Dr. Kerem was a board member of the European Cystic Fibrosis Society where he contributed to the development of the European Cystic Fibrosis Registry and was the President of CIPP, the Annual **International Congress on Pediatric Pulmonology**. Dr. Kerem was previously a member of the editorial boards of the leading journals in the field of pulmonology, "**Pediatric Pulmonology**", "**Chest**" and the "**American Journal of Respiratory and Critical Care Medicine**".

- 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**" was published in the August 2020 edition of 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.

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.
    - 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 CFF's Therapeutics Development Network.

    - In Europe, the European Cystic Fibrosis Society Clinical Trial Network has given our trial a “high priority” ranking.
- 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.
- The independent Safety Review Committees have held several planned meetings and approved dose escalation up to the highest dose level. To date, no drug related serious adverse events have been reported.
- We expect to report top line data from our proof of concept Phase 2 program in the first half of 2021, which is contingent on no further disruptions due to COVID-19.

ADPKD Kidney Program

- 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 efforts 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.

Ocular Program

- 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.

- 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.

Third Quarter 2020 Financial Results

As of September 30, 2020, we had cash and cash equivalents of $30.6 million, which we expect will be sufficient to fund our operations through the end of 2021.

For the three months ended September 30, 2020, we incurred a net loss of $6.6 million or $0.16 per share, which includes $1.4 million in non-cash stock-based compensation. For the same period in the prior year, we incurred a net loss of $12.9 million, or $0.32 per share.

Our research and development expenses were $3.2 million for the three months ended September 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 $6.8 million. The quarter to quarter decrease in R&D expenses of $3.6 million was driven by reduced professional service fees primarily the result of the temporary pause in our clinical trials due to COVID-19, and reduced headcount and related salaries for the 2020 period.

Our general and administrative expenses were $3.1 million for the three months ended June 30, 2020, which includes $1.1 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 headcount and professional services costs.

First Nine Months 2020 Financial Results

For the nine months ended September 30, 2020, we incurred a net loss of $28.5 million, or $0.71 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 $5.3 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 $39.2 million, or $1.05 per share.

Our research and development expenses were $11.3 million for the nine months ended September 30, 2020, which includes $0.8 million in non-cash expense related to stock-based compensation. For the same period in the prior year, R&D expenses were $20.2 million. The year over year decrease

in R&D expenses of $8.9 million was driven by reduced professional service fees primarily the result of the temporary 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 $12.3 million for the nine months ended September 30, 2020, which includes $4.5 million in non-cash expense related to stock-based compensation. For the same period in the prior year, G&A expenses were $18.9 million. The decrease of $6.6 million was primarily driven by lower headcount and professional services costs.

Conference Call and Webcast Information:
Date: Thursday, November 5, 2020
Time: 4:30 p.m. ET
Domestic Dial-in Number: (866) 913-8546
International Dial-in Number: (210) 874-7715
Conference ID: 9467336
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/gucnkjge.

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. 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 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)

	September 30, 2020	December 31, 2019
ASSETS		
Current assets:		
Cash and cash equivalents	$ 30,592	$ 22,493
Marketable securities	—	33,783
Restricted cash	52	43
Prepaid expenses and other current assets	1,568	1,390

Total current assets	32,212	57,709
Property and equipment, net	149	201
Operating lease right-of-use asset	551	924
Other long-term assets	30	113
Total assets	$ 32,942	$ 58,947
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 638	$ 1,871
Accrued expenses	3,091	4,655
Current portion of long-term debt	4,917	4,336
Advances from collaboration partners	805	403
Current portion of operating lease liability	496	499
Taxes payable	38	43
Total current liabilities	9,985	11,807
Long-term debt	7,823	10,502
Operating lease liability	56	425
Total liabilities	17,864	22,734
Stockholders' equity:		
Preferred stock, $0.01 par value per share, 5,000,000 shares authorized, no shares issued or outstanding as of September 30, 2020 or December 31, 2019	—	—
Common stock, $0.01 par value per share, 500,000,000 shares authorized, 40,343,181 and 40,186,469 shares issued, and 40,150,530 and 40,030,763 shares outstanding as of September 30, 2020 and December 31, 2019, respectively	403	402
Common stock in treasury, at cost, 192,651 and 155,706 shares as of September 30, 2020 and December 31, 2019, respectively	(1,825)	(1,703)
Additional paid-in capital	181,969	174,515
Accumulated other comprehensive income	—	18
Accumulated deficit	(165,469)	(137,019)
Total stockholders' equity	15,078	36,213
Total liabilities and stockholders' equity	$ 32,942	$ 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 September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Operating expenses:				
Research and development	$ 3,231	$ 6,801	$ 11,308	$ 20,160
General and administrative	3,065	5,978	12,347	18,907
Restructuring charges	—	—	3,994	—
Total operating expenses	6,296	12,779	27,649	39,067
Loss from operations	(6,296)	(12,779)	(27,649)	(39,067)
Other expense, net	321	96	801	174
Net loss	$ (6,617)	$ (12,875)	$ (28,450)	$ (39,241)
Net loss per share, basic and diluted	$ (0.16)	$ (0.32)	$ (0.71)	$ (1.05)

Weighted average number of shares of common stock used in computing net loss per share, basic and diluted	40,142,178	39,944,324	40,115,351	37,394,310

Source: Eloxx Pharmaceuticals