Eloxx Pharmaceuticals Reports Fourth Quarter and Full Year 2020 Financial and Operating Results and Provides Business Update

March 11, 2021

We expect to report top line data from our Phase 2 cystic fibrosis clinical trials in the first half of this year

We have expanded our global Phase 2 clinical trial beyond the U.S., Europe and Israel; opening additional clinical sites in Australia and Canada

Cystic Fibrosis Foundation (CF Foundation) has increased its partial funding of our clinical trial program beyond the U.S. portion to include Europe and Israel

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

Cash and cash equivalents of $24.7 million as of December 31, 2020

We will host a webcast and conference call today, Thursday, March 11, 2021 at 4:30 pm ET

WALTHAM, Mass., March 11, 2021 (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 twelve months ended December 31, 2020 and provided a business update.

"We are on track to report top line data from our global Phase 2 cystic fibrosis clinical trials in the first half of this year. In addition to Europe, Israel and the U.S., we are opening additional clinical sites in Australia and Canada. 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 the CF Foundation has increased its financial support for the global clinical trial program and that the independent Safety Review Committees 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 February 9, 2021, we announced that a scientific manuscript titled: "**Targeting *G542X CFTR* Nonsense Alleles With ELX-02 Restores CFTR Function in Human-Derived Intestinal Organoids**" was published in the ***Journal of Cystic Fibrosis*.**
    - Our evaluation of ELX-02 mediated read-through, using the CFTR-dependent, Forskolin-induced swelling (FIS) assay across a selection of *G542X* homozygous and heterozygous patient-derived organoids, demonstrated that ELX-02 increased CFTR activity in a dose-dependent fashion across a variety of forskolin induction concentrations. These functional increases are similar to those obtained with tezacaftor/ivacaftor in a *F508del* homozygous organoid. Additionally, in some cases, ELX-02 treatment of these patient-derived organoids resulted in about a 5-fold increase in *CFTR mRNA* when compared with vehicle treated as measured using Nanostring. These data support our Phase 2 clinical trial for ELX-02 in cystic fibrosis patients with *G542X* alleles.
- On January 26, 2021, we announced that a scientific manuscript titled: "**A Randomized, Double-Blind, Placebo-Controlled, Multiple Dose Escalation Study to Evaluate the Safety and Pharmacokinetics of ELX-02 in Healthy Subjects**" was published in the journal ***Clinical Pharmacology in Drug Development***.
    - In this Phase 1B multiple-ascending dose trial evaluating the safety and pharmacokinetics of ELX-02 in healthy volunteers there were no severe or serious side effects, which is consistent with the favorable tolerability profile demonstrated across our preclinical and clinical datasets.
- On January 20, 2021, we announced that a scientific manuscript titled: "**Phase 1 Renal Impairment Trial Results Enable Targeted Individualized Dosing of ELX-02 in Nephropathic Cystinosis Patients**" was published in the ***Journal of Clinical Pharmacology***.
    - This study was designed to define the relationship of eGFR and drug exposure and urinary clearance in patients with renal impairment. The results demonstrated that ELX-02 was well tolerated by patients with renal insufficiency

and nephropathic cystinosis patients. Across increasing degrees of renal insufficiency with reduced clearance, ELX-02 pharmacokinetics demonstrated increased exposure and prolonged renal elimination proportional to eGFR. The data from the renal impairment study enabled the development of an eGFR-PK model of ELX-02 which was successfully used to implement individualized daily dosing of ELX-02 in a Phase 2 study in patients with nephropathic cystinosis to achieve the targeted exposure.

- Collectively, our published scientific manuscripts support our current Phase 2 global clinical trial for ELX-02 in cystic fibrosis patients with nonsense alleles. Additionally, they 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 January 13, 2021, we announced that the CF Foundation extended its partial funding for our clinical trial program beyond the U.S. portion of the trial to include Europe and Israel.

Cystic Fibrosis Phase 2 Program

- We are on track to report top line data from our proof-of-concept Phase 2 program in the first half of this year.

- Our global Phase 2 program consists of two trials, one currently enrolling patients at sites in Europe, Israel and opening in Australia, and the second in the U.S. and opening in Canada.
    - The Cystic Fibrosis Foundation's (CFF) partial funding of the U.S. trial has been extended to include Europe and Israel and our protocol has been sanctioned by CFF's Therapeutics Development Network.
    - Opening additional clinical trial sites in Australia and Canada.
    - 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 fourth and highest dose level. To date, no drug related serious adverse events have been reported.

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 building the models to evaluate the ADPKD nonsense patient population, we enlisted the support of Dr. Benjamin Freedman, Associate Professor of the Division of Nephrology at the University of Washington. Dr. Freedman is an expert in differentiating induced pluripotent stem cells into 3-dimensional kidney organoids, capable of modeling cyst formation observed in ADPKD. We have modeled the most prevalent ADPKD nonsense mutations in these cells, and we anticipate providing updates on ELX-02's ability to prevent or reduce cysts in these organoids along with other program progress over the coming year.

- In our preclinical studies in ADPKD, we have observed dose-dependent read-through with our ERSG compounds across the most common *PKD2* alleles and have expanded our studies to include *PKD1*. Using a patient-derived organoid with the most common *PKD2* nonsense allele, we have reproducibly observed reduced cystogenesis and cyst size with administration of Eloxx ERSGs.

Ocular Program

- In order to expand our ocular research footprint and ensure we are evaluating the most relevant cellular and animal models of nonsense-mediated blindness we have established research collaborations with ocular disease experts at the University of Maryland, University of Wisconsin and UCLA, and look forward to sharing more results as these programs progress.

- 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 last 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 developing 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.

Fourth Quarter 2020 Financial Results

As of December 31, 2020, we had cash and cash equivalents of $24.7 million, which we expect will be sufficient to fund our operations though top line data and into the fourth quarter.

For the three months ended December 31, 2020, we incurred a net loss of $6.1 million or $0.15 per share, which includes $1.3 million in non-cash stock-based compensation. For the same period in the prior year, we incurred a net loss of $11.6 million, or $0.29 per share.

Our research and development expenses were $2.6 million for the three months ended December 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 $5.9 million. The quarter-to-quarter decrease in R&D expenses of $3.3 million was driven by reduced headcount and related salaries as well as decreases in certain clinical and pre-clinical research costs for the 2020 period.

Our general and administrative expenses were $3.1 million for the three months ended December 31, 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 $5.6 million. The decrease was primarily driven by lower headcount and professional services costs for the 2020 period.

Full Year 2020 Financial Results

For the twelve months ended December 31, 2020, we incurred a net loss of $34.6 million, or $0.86 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 $6.6 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 $50.9 million, or $1.34 per share.

Our research and development expenses were $14.6 million for the twelve months ended December 31, 2020, which includes $1.0 million in non-cash expense related to stock-based compensation. For the same period in the prior year, R&D expenses were $26.3 million. The year over year decrease in R&D expenses of $11.7 million was driven by reduced headcount and related salaries for a portion of the 2020 period as well as reduced costs relating to certain clinical and preclinical activities.

Our general and administrative expenses were $14.8 million for the twelve months ended December 31, 2020, which includes $5.6 million in non-cash expense related to stock-based compensation. For the same period in the prior year, G&A expenses were $24.2 million. The decrease of $9.4 million was primarily driven by lower headcount and professional services costs.

Conference Call Information:
Date: Thursday, March 11, 2021
Time: 4:30 p.m. ET
Domestic Dial-in Number: (866) 913-8546
International Dial-in Number: (210) 874-7715
Conference ID: 4772135
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/m65hdyjn.

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)

	December 31,	
	2020	**2019**
ASSETS		
Current assets:		
Cash and cash equivalents	$ 24,668	$ 22,493
Marketable securities	—	33,783
Restricted cash	56	43
Prepaid expenses and other current assets	1,169	1,390
Total current assets	25,893	57,709
Property and equipment, net	133	201
Operating lease right-of-use asset	421	924
Other long-term assets	30	113
Total assets	**$ 26,477**	**$ 58,947**
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 481	$ 1,871
Accrued expenses	2,886	4,655
Current portion of long-term debt	5,239	4,336
Advances from collaboration partners	805	403
Current portion of operating lease liability	389	499
Taxes payable	38	43
Total current liabilities	9,838	11,807
Long-term debt	6,376	10,502
Operating lease liability	33	425
Total liabilities	16,247	22,734
Total stockholders' equity	10,230	36,213
Total liabilities and stockholders' equity	**$ 26,477**	**$ 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 December 31,		Year Ended December 31,	
	2020	**2019**	**2020**	**2019**

Operating expenses:				
Research and development	$ 2,640	$ 5,855	$ 14,590	$ 26,349
General and administrative	3,142	5,633	14,847	24,206
Restructuring charges	24	—	4,018	—
Total operating expenses	5,806	11,488	33,455	50,555
Loss from operations	(5,806)	(11,488)	(33,455)	(50,555)
Other expense, net	321	145	1,122	319
Net loss	$ (6,127)	$ (11,633)	$ (34,577)	$ (50,874)
Net loss per share, basic and diluted	$ (0.15)	$ (0.29)	$ (0.86)	$ (1.34)
Weighted average number of shares of common stock used in computing net loss per share, basic and diluted	40,153,552	39,981,335	40,124,953	38,063,173

Source: Eloxx Pharmaceuticals