Eloxx Presents Two Preclinical Posters at the 2020 North American Cystic Fibrosis Virtual Conference

October 22, 2020

WALTHAM, Mass., Oct. 22, 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 announced that it 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 live sessions and discussions will take place through October 23rd, 2020. These virtual posters are available to registered attendees on the NACFC online conference platform.

"We were pleased to have the opportunity to present additional preclinical study results in cystic fibrosis at the 2020 NACFC virtual conference that demonstrate ELX-02's selectivity for read-through of premature stop codons versus native stop codons and its ability to restore production of functional *CFTR* in patient-derived organoids," said Dr. Gregory Williams, Chief Executive Officer of Eloxx Pharmaceuticals. "We believe that these results de-risk the current Phase 2 proof of concept clinical trials for ELX-02 in cystic fibrosis. We are continuing to advance our trials in Europe, Israel and the U.S., and we look forward to reporting top line data from the Phase 2 clinical trial program as quickly as possible."

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.

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. 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.

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