Eloxx Pharmaceuticals Announces Open Investigational New Drug Application (IND) for a Phase 2 Clinical Trial of ELX-02 in Cystic Fibrosis Patients with the G542X Mutation in the US and Protocol Endorsement from the Cystic Fibrosis Foundation (CFF)

July 31, 2019

On track to report top line data from Phase 2 clinical trials for ELX-02 in cystic fibrosis and cystinosis in the U.S., Europe, Israel and Canada in 2019

Company to host webcast and conference call on August 7, 2019 at 8:30 am ET to report second quarter 2019 financial results and provide a business update

WALTHAM, Mass., July 31, 2019 (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, cystinosis, inherited retinal disorders, and other diseases caused by nonsense mutations limiting production of functional proteins, today announced that an IND for ELX-02 in cystic fibrosis is now open in the U.S. and the Phase 2 clinical trial has been endorsed by the CFF.

"We are very pleased that our IND is open in the U.S. and the protocol for our Phase 2 clinical trial in cystic fibrosis has been endorsed by the Cystic Fibrosis Foundation (CFF) in the U.S. Dr. Ahmet Uluer, Director of the Adult Cystic Fibrosis Program at the Boston Children's Hospital/Brigham and Women's Hospital CF Center, has agreed to be the lead study investigator in the U.S., and Professor Kerem Eitan, M.D., Head of the Division of Pediatrics, Children's Hospital, Hadassah Medical Center, will serve as the Global Lead Investigator. We are gratified by the participation of these two leading experts and we look forward to reporting top line data later this year," said Robert E. Ward, Chairman and CEO of Eloxx Pharmaceuticals. "We believe that the positive data we have generated for ELX-02 in cystic fibrosis patient-derived organoids substantially de-risk our Phase 2 program."

"I am very pleased to be leading the Phase 2 clinical trial of ELX-02 in cystic fibrosis patients with the G542X mutation on one or both alleles in the U.S. These patients have a high unmet medical need and few, if any, targeted treatment options. ELX-02 is the only therapy to have demonstrated positive results in organoids derived from cystic fibrosis patients across the majority of nonsense mutations and studies have shown the organoid model to be highly predictive of clinical benefit," said Ahmet Uluer, DO, MPH, Director, Adult Cystic Fibrosis Program, Boston Children's Hospital, Division of Pulmonary Medicine, and Brigham and Women's Hospital, Division of Pulmonary and Critical Care Medicine. "While important progress has been made in the development of disease modifying treatments for patients with cystic fibrosis, patients with nonsense mutations represent the most severe phenotypes and often do not respond to currently available therapies," said Dr. Kerem Eitan, Head of the Division of Pediatrics, Children's Hospital, Hadassah Medical Center. "I am excited to lead this clinical trial of ELX-02 which may provide a new therapeutic approach for these patients."

Conference Call Information:
Date: Wednesday, August 7, 2019
Time: 8:30 a.m. ET
Domestic Dial-in Number: (866) 913-8546
International Dial-in Number: (210) 874-7715
Conference ID: 1754316
Live Webcast: accessible from the Company's website at www.eloxxpharma.com under Events and Presentations or with this link:

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 and cystinosis. 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 recently announced a new program focused on rare ocular genetic disorders. Eloxx is headquartered in Waltham, MA, with operations in Rehovot, Israel. 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; 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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