



Eloxx Pharmaceuticals Announces Cystic Fibrosis Foundation (CF Foundation) To Provide Funding and Support for ELX-02 U.S. Phase 2 Clinical Trial

September 23, 2019

CF Foundation and Eloxx to form joint program advisory group focused on the development of ELX-02 for cystic fibrosis

Eloxx plans to conduct the U.S. portion of the Phase 2 clinical trial at Cystic Fibrosis Therapeutics Development Network (TDN) member sites

On track to report top line Phase 2 data for ELX-02 in cystic fibrosis and cystinosis in 2019

Eloxx to host one-on-one meetings with institutional investors at the Oppenheimer Fall Summit focused on Specialty Pharma & Rare Diseases (September 24, 2019) and to present and host one-on-one meetings at the 2019 Cantor Global Healthcare Conference (October 3, 2019)

WALTHAM, Mass., Sept. 23, 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 the Cystic Fibrosis Therapeutics Development Network has sanctioned its Phase 2 clinical trial protocol in the US for ELX-02 in cystic fibrosis, and the CF Foundation will provide funding and additional resources for the program.

"We are very gratified by the CF Foundation's support as we advance our development program for ELX-02 in cystic fibrosis. The CF Foundation's funding will accelerate the program in the US," said Robert E. Ward, Chairman and Chief Executive Officer of Eloxx Pharmaceuticals. "We are also pleased that Dr. Ahmet Uluer, Director of the Adult Cystic Fibrosis Program, Boston Children's Hospital has agreed to be the lead investigator in the U.S. and we look forward to reporting top line data from this trial later this year."

Eloxx's Phase 2 clinical trial program will consist of two open label, dose escalation studies to evaluate the safety, tolerability, PK and PD of multiple doses of ELX-02 in patients with CF with at least one G542X allele. The U.S. study, EL-012, will enroll up to eight patients, and the CF Foundation has provided a financial award of up to \$1.6 million to support this study. The European study, EL-004, will enroll up to 16 patients. Eloxx anticipates reporting top line data in the fourth quarter of 2019.

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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SOURCE: Eloxx Pharmaceuticals, Inc.



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