Eloxx Pharmaceuticals Announces Cystic Fibrosis Foundation (CF Foundation) To Provide Increased Funding and Support for ELX-02 Global Phase 2 Clinical Trial Program

January 13, 2021

The CF Foundation's partial funding of the U.S. trial has been extended to partial funding of the global clinical trial program which includes Europe and Israel

Expect to report top line data from our Phase 2 cystic fibrosis clinical trial program for ELX-02 in the first half of 2021

Eloxx had previously reported the formation of a joint program advisory group with the CF Foundation focused on the development of ELX-02 in cystic fibrosis

WALTHAM, Mass., Jan. 13, 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 announced an expansion of its agreement with the CF Foundation beyond the U.S. portion of the trial to include additional funding for the global clinical trial program which includes Europe and Israel.

"We are gratified by the CF Foundation's expanded support for our Phase 2 proof of concept global clinical trial program for ELX-02 for the treatment of patients with nonsense mediated cystic fibrosis, for whom there remains a high unmet medical need," said Dr. Gregory Williams, Chief Executive Officer of Eloxx Pharmaceuticals. "We Look forward to reporting top line data from this program in the first half of this year and are pleased that ELX-02 has been granted an orphan drug designation by the FDA and orphan medicinal product designation by the EMA."

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., in addition to the partial funding being provided by the CF Foundation, our protocol has been sanctioned by the Cystic Fibrosis Therapeutics Development Network (TDN).
    - In Europe, the European Cystic Fibrosis Society Clinical Trial Network (ECFS-CTN) 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.
- 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.
- The Safety Review Committee has held several planned meetings and approved dose escalation up to the highest dose level. To date, we have had no reported serious adverse events in the clinical trial.
- We are participating in the European HIT-CF consortium to support the collection of cystic fibrosis patient-derived organoids and the initiative to conduct a prospective clinical trial to confirm the translational potential of the organoid model. The intent of the program is to use these positive results to enroll patients with responsive organoids in a prospective trial with ELX-02. We believe this program will continue to expand the application of organoid technology from drug discovery through drug approval, and also offers possible label expansion opportunities.

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.

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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