



Eloxx Pharmaceuticals Receives U.S. Orphan Drug Designation for ELX-02 for the Treatment of Cystic Fibrosis

August 4, 2020

ELX-02 had previously been granted orphan medicinal product designation for the treatment of Cystic Fibrosis by the European Medicines Agency

WALTHAM, Mass., Aug. 04, 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 the U.S. Food and Drug Administration (FDA) has granted the Company orphan drug designation for ELX-02 for the treatment of Cystic Fibrosis.

The FDA's Office of Orphan Drug Products grants orphan status to support the development of medicines for underserved patient populations, or rare disorders, that affect fewer than 200,000 people in the U.S. Orphan-drug designation qualifies Eloxx for certain benefits, including seven years of market exclusivity upon regulatory approval if received, exemption of FDA application fees, tax credits on qualified U.S. clinical trials and eligibility for grant funding opportunities that can be used for clinical trial costs.

"We are pleased to have received orphan drug designation from the FDA for ELX-02 in the U.S. Combined with our previous EMA orphan medicinal product designation in Europe, and upon approvals in the U.S. and the E.U., we will be able to obtain significant periods of marketing exclusivity in these important markets and provide treatment options to patients with high unmet medical needs, particularly in patients with nonsense mutations for whom there are few, if any, treatments available," said Dr. Gregory Williams, Chief Executive Officer of Eloxx Pharmaceuticals. "As previously announced, we have resumed enrollment in our Phase 2 proof of concept clinical trials in Israel and Europe, with our trial in the U.S. continuing to be temporarily paused due to the COVID-19 pandemic. We look forward to reporting top line results as soon as feasible, and believe that this will be a major value inflection point for our Company."

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; 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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