Eloxx Announces Publication of Scientific Manuscript on ELX-02 in the Journal of Clinical Pharmacology

January 20, 2021

Publication titled: “Phase 1 Renal Impairment Trial Results Enable Targeted Individualized Dosing of ELX-02 in Nephropathic Cystinosis Patients”

WALTHAM, Mass., Jan. 20, 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 that a scientific manuscript titled: “Phase 1 Renal Impairment Trial Results Enable Targeted Individualized Dosing of ELX-02 in Nephropathic Cystinosis Patients,” has been published in the **Journal of Clinical Pharmacology.**

“We are pleased that our scientific manuscript on the results of our Phase 1 renal impairment trial which defined the relationship of eGFR and drug exposure and urinary clearance in patients with renal impairment has been published in the **Journal of Clinical Pharmacology,”** said Dr. Thomas Haverty, Chief Medical Officer at Eloxx Pharmaceuticals. “ELX-02 was well tolerated by patients with renal insufficiency and nephropathic cystinosis patients and exhibits a consistent pharmacokinetic profile across increasing degrees of renal insufficiency with reduced clearance, increased exposure, and prolonged renal elimination proportional to eGFR. The data from the renal impairment study enabled the development of an eGFR-PK model of ELX-02 which was successfully used to implement individualized daily dosing of ELX-02 in a Phase 2a study in patients with nephropathic cystinosis to achieve a weekly targeted exposure.” These results further support our ongoing Phase 2 program in cystic fibrosis and IND-enabling studies in autosomal dominant polycystic kidney disease,”

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.

Contact:

Barbara Ryan
203-274-2825
barbarar@eloxxpharma.com

SOURCE: Eloxx Pharmaceuticals, Inc.

Source: Eloxx Pharmaceuticals