



## Eloxx Pharmaceuticals Announces Initiation of Phase 2 Clinical Trial for ELX-02 in Cystinosis

August 5, 2019

*Dr. Paul Goodyer, Professor of Pediatrics at McGill University and recognized leader in hereditary renal disease, is the principal investigator in the Phase 2 clinical trial*

*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., Aug. 05, 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 it has initiated a Phase 2 clinical trial (Study 003) for ELX-02 in cystinosis in Canada.

Study 003 is a single arm, open label study designed to assess the safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of subcutaneous (SC) ELX-02 in patients with nephropathic cystinosis with at least 1 nonsense mutation in the cystinosis gene. Six patients will be enrolled in the trial, and will be treated with ELX-02 for four weeks with three escalating dose levels.

"I am excited to have the opportunity to offer ELX-02 to my cystinosis patients, a new potential treatment option which has exhibited positive results in cellular and animal models with dose-dependent reductions in white blood cell cystine levels, a biomarker used to monitor cysteamine therapy and to predict clinical benefit," said Dr. Paul Goodyer, Professor of Pediatrics at McGill University. "There remains a high unmet medical need in cystinosis, which is diagnosed in infancy, and new therapeutic options with the potential to alter the course of this progress disease could dramatically improve the lives of patients and their families."

"We are very pleased to have initiated a Phase 2 clinical trial for ELX-02 in cystinosis patients with nonsense mutations in Canada and we are grateful for the support and non-dilutive funding we have received for this trial from Genome Quebec and Genome Canada," said Dr. Greg Williams, COO of Eloxx Pharmaceuticals. "We look forward to reporting top line data from this trial early in the fourth quarter of this year. We believe that the results to date, as well as the preliminary results from our renal impairment study provide support for both continuing our clinical development programs and evaluating the suitability of our ERSG library for development in additional renal disorders, including autosomal dominant polycystic kidney disease and cystinuria."

Dr. Goodyer previously reported data at the WORLDSymposium which showed that ELX-02 decreases the cystine content in cellular and animal models. Dr. Goodyer extended his research to show ELX-02 cystine decreases when used in combination with cystamine. These data were presented in March at the Cystinosis Research Foundation "Day of Hope" meeting in Irvine, California.

In support of the cystinosis program, where preserving renal function is a major concern, Eloxx has completed a renal study in mild, moderate, and severe renal impairment cohorts. Previously, the U.S. Food and Drug Administration granted orphan drug designation for ELX-02 for the treatment of cystinosis and an IND is currently open. ELX-02 is an investigational drug that has not been approved by any global regulatory body.

### 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](http://www.eloxxpharma.com) under Events and Presentations or with this link: <https://edge.media-server.com/mmc/p/9axiqnvt>

### 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](http://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.*

### **Contact:**

Barbara Ryan  
203-274-2825  
[barbarar@eloxxpharma.com](mailto:barbarar@eloxxpharma.com)

SOURCE: Eloxx Pharmaceuticals, Inc.



Source: Eloxx Pharmaceuticals