

## Eloxx Pharmaceuticals' Phase 2 Study of ELX-02 in Cystinosis Receives Authorization of Clinical Trial Application (CTA) by Health Canada and Funding from Genome Quebec and Genome Canada

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

*Genome Quebec and Genome Canada provides non-dilutive funding for the Phase 2 clinical trial of ELX-02*

*Cystinosis Research Foundation provided non-dilutive funding for the preclinical phase of the program*

*Eloxx to report top line data from Phase 2 cystinosis clinical trial in 2019*

WALTHAM, Mass., June 10, 2019 (GLOBE NEWSWIRE) -- **Eloxx Pharmaceuticals, Inc. ("Eloxx")** (ELOX:Nasdaq), 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 received authorization of its CTA from Health Canada for a Phase 2 clinical trial of ELX-02 in cystinosis. Dr. Paul Goodyer, Professor of Pediatrics at McGill University, a recognized leader in the care of cystinosis patients will be the principal investigator of the Phase 2 trial and Genome Quebec and Genome Canada will provide non-dilutive funding of \$1.1M CAD. The Cystinosis Research Foundation (CRF) provided financial support for the preclinical phase of the program.

"I look forward to advancing the Phase 2 clinical trial of ELX-02 in cystinosis and I'm excited about its potential to improve the lives of cystinosis patients. ELX-02 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.

"We are pleased to be authorized by Health Canada to proceed with a Phase 2 clinical trial in cystinosis and grateful to Genome Quebec, Genome Canada, and the Cystinosis Research Foundation for their engagement and support. The non-dilutive financing, additional resources and expertise have allowed us to move our Phase 2 study forward quickly," said Dr. Gregory Williams, Chief Operating Officer, Eloxx Pharmaceuticals. "Dr. Goodyer has a substantial Quebec-based cystinosis patient group, and we expect to report top line data early in the fourth quarter of 2019. We believe the results to date, as well as the successfully completed mild to moderate renal study cohorts, provide support for expanding our research into other inherited renal disorders such as 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 cysteamine. 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 initiated a renal study with successful results already demonstrated in the mild and moderate renal impairment cohorts. To date, the pharmacokinetic results were as expected with no adverse events reported.

Genome Quebec's mission is to catalyze development and excellence of genomics research and promote its integration and democratization. Genome Quebec is helping to accelerate the discovery of new applications for genomics in strategic areas such as health, agrifood, forestry and the environment. The funds invested by Genome Quebec are provided by the ministere de l'Economie et de innovation du Quebec (MEI). (<http://www.genomequebec.com/en/home/>)

Genome Canada makes strategic investments in large-scale initiatives to ensure leading genomics research is applied to maximum benefit for Canada. Genome Canada programs are designed based on engagement with a broad range of stakeholders and are implemented through a rigorous international peer-review system. Genome Canada acknowledges the financial support of the Government of Canada. (<https://www.genomecanada.ca/en>)

Currently, in the U.S., the Food and Drug Administration has granted orphan drug designation for ELX-02 for the treatment of cystinosis and an IND is open. ELX-02 is an investigational drug that has not been approved by any global regulatory body.

### **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

## 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. Hyperlinks are provided as a convenience and for informational purposes only. Eloxx bears no responsibility for the security or content of external websites.*

### Contact:

Barbara Ryan

[barbarar@eloxxpharma.com](mailto:barbarar@eloxxpharma.com)

(203) 274-2825

SOURCE: Eloxx Pharmaceuticals, Inc.