



Eloxx Pharmaceuticals Provides Enrollment Update for Ongoing Phase 2 Clinical Studies for Cystic Fibrosis

July 6, 2021

Data Readout for First Four Treatment Arms Expected in the Fourth Quarter of 2021

WATERTOWN, Mass., July 06, 2021 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (NASDAQ: ELOX) today provided an update on enrollment for the ongoing global Phase 2 clinical program for ELX-02 for the treatment of cystic fibrosis (CF) in patients with at least one G542X allele. Based on enrollment to date, Eloxx expects to present data from the first four treatment arms of the study in the fourth quarter of this year. As of the end of June 2021, Eloxx has enrolled a sufficient number of patients to assess biological activity of ELX-02. Eloxx will continue to enroll additional patients to support Phase 3 clinical trial planning. The Phase 2 trials are designed to evaluate the safety of ELX-02 and assess its biological activity.

"Given the substantial efforts of our clinical team, we have made considerable progress in enrolling patients in our ongoing Phase 2 clinical program to explore the potential of ELX-02 to treat cystic fibrosis," said Sumit Aggarwal, President and Chief Executive Officer. "Given this progress, we are positioned to present data from the first four treatment arms of the study in the fourth quarter of this year."

ELX-02 is currently in Phase 2 clinical trials in CF patients affected by nonsense mutations in the CFTR (CF transmembrane conductance regulator) gene. The trial currently has sites in the U.S., Europe, Israel, Australia and Canada. Following several planned Safety Review Committee meetings, dose escalation has proceeded up to the top dose level, and data have shown no drug-related serious adverse events reported to date. Recently, a fifth treatment arm was added to the program to evaluate the safety of ELX-02 in combination with Kalydeco (ivacaftor), an FDA-approved CFTR potentiator for the treatment of cystic fibrosis in patients who have at least one mutation in their CF gene amenable to ivacaftor.

The program is partially funded by the Cystic Fibrosis Foundation (CFF).

The U.S. Food and Drug Administration has granted orphan drug designation for ELX-02 for the treatment of CF.

About Eloxx Pharmaceuticals

Eloxx Pharmaceuticals, Inc. is engaged in the science of ribosome modulation, leveraging both its innovative TURBO-ZM™ chemistry technology platform in an effort to develop novel Ribosome Modulating Agents (RMAs) and its library of Eukaryotic Ribosome Selective Glycosides (ERSGs). 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 clinical development focusing on cystic fibrosis. ELX-02 is an investigational drug that has not been approved by any global regulatory body. Eloxx also has preclinical programs focused on select rare diseases including inherited diseases, cancer caused by nonsense mutations, kidney diseases, including autosomal dominant polycystic kidney disease, as well as rare ocular genetic disorders.

For more information, please visit www.eloxxpharma.com.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements other than statements of present and historical facts contained in this press release, including without limitation, statements regarding the expected timing of trials and results from clinical studies of our product candidate, the expansion of our clinical trial sites and the potential of our product candidate to treat nonsense mutations are forward-looking statements. Forward-looking statements can be identified by the words "aim," "may," "will," "would," "should," "expect," "explore," "plan," "anticipate," "could," "intend," "target," "project," "contemplate," "believe," "estimate," "predict," "potential," "seeks," or "continue" or the negative of these terms similar expressions, although not all forward-looking statements contain these words.

Forward-looking statements are based on management's current plans, estimates, assumptions and projections based on information currently available to us. Forward-looking statements are subject to known and unknown risks, uncertainties and assumptions, and actual results or outcomes may differ materially from those expressed or implied in the forward-looking statements due to various important factors, including, but not limited to: our ability to progress any product candidates in preclinical or clinical trials; the uncertainty of clinical trial results and the fact that positive results from preclinical studies are not always indicative of positive clinical results; the scope, rate and progress of our preclinical studies and clinical trials and other research and development activities; the competition for patient enrollment from drug candidates in development; the impact of the global COVID-19 pandemic on our clinical trials, operations, vendors, suppliers, and employees; our ability to obtain the capital necessary to fund our operations; the cost of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights; our ability to obtain financial in the future through product licensing, public or private equity or debt financing or otherwise; general business conditions, regulatory environment, competition and market for our products; and business ability and judgment of personnel, and the availability of qualified personnel and other important factors discussed under the caption "Risk Factors" in our Quarterly Report on Form 10-Q for the quarterly period ended March 31, 2021, as any such factors may be updated from time to time in our other filings with the SEC, accessible on the SEC's website at www.sec.gov and the "Financials & Filings" page of our website at <https://investors.eloxxpharma.com/financial-information/sec-filings>

All forward-looking statements speak only as of the date of this press release and, except as required by applicable law, we have no obligation to update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

SOURCE: Eloxx Pharmaceuticals, Inc.

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