



Eloxx Pharmaceuticals Adds Additional Treatment Arm to Ongoing Phase 2 Clinical Studies for Cystic Fibrosis

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Fifth Treatment Arm to Evaluate Safety of ELX-02 in Combination with Kalydeco (ivacaftor)

Reaffirmed Data Readout for First Four Treatment Arms On Track for Second Half of 2021

WALTHAM, Mass., April 29, 2021 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (NASDAQ: ELOX) today announced the addition of a new study arm in 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. The Phase 2 clinical program now includes a fifth treatment arm to evaluate safety of ELX-02 in combination with Kalydeco (ivacaftor), an FDA-approved CFTR (CF transmembrane conductance regulator) potentiator for the treatment of cystic fibrosis in patients who have at least one mutation in their CF gene amenable to ivacaftor. The Phase 2 trials are designed to evaluate the safety of ELX-02 and assess its biological activity.

"We are extremely pleased to include this additional treatment arm in the ongoing Phase 2 clinical trial to further explore the potential of ELX-02 to treat cystic fibrosis," said Sumit Aggarwal, President and Chief Executive Officer. "Given ELX-02's potential to treat CFTR nonsense mutations and the synergistic effects of ELX-02 and ivacaftor seen to date in pre-clinical models, we believe that combination treatment with a CFTR potentiator, such as ivacaftor, provides the opportunity to improve clinical efficacy in the treatment of CF. We remain on track to present data from the first four treatment arms of the study in the second half of this year."

ELX-02 is currently in Phase 2 clinical trials in CF patients affected by nonsense mutations in the CFTR gene. The trial currently has sites in the U.S., Europe, Israel, Australia and Canada. Several planned Safety Review Committee meetings have occurred and allowed dose escalation up to the top dose level, and data have shown no drug-related serious adverse events reported to date. Multiple patients have progressed through the four-dose escalation treatment range. 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 Annual Report on Form 10-K for the fiscal year ended December 31, 2020, 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.

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