Eloxx Pharmaceuticals Announces Fast Track Designation for ELX-02 for the Treatment of Cystic Fibrosis Patients with Nonsense Mutations

September 9, 2021

WATERTOWN, Mass., Sept. 09, 2021 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (Nasdaq: ELOX), today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for ELX-02, a drug candidate intended to treat cystic fibrosis patients with nonsense mutations. ELX-02 is currently in Phase 2 clinical trials in CF patients affected by nonsense mutations in the CFTR (CF transmembrane conductance regulator) gene for whom there are no effective disease modifying therapies. The Phase 2 trials are designed to evaluate the safety of ELX-02 and assess its biological activity, and Eloxx expects to present data from the first four treatment arms in the fourth quarter of 2021.

"We are delighted to receive Fast Track Designation from the FDA for ELX-02 as the need for patients remains significant. The ability to have greater access to the FDA and their guidance on the regulatory pathway for ELX-02 can help provide the ability to work with the urgency needed on behalf of CF patients with nonsense mutations," said Sumit Aggarwal, President and CEO of Eloxx.

Fast Track Designation is granted to drugs being developed for the treatment of serious or life-threatening diseases or conditions where there is an unmet medical need. The purpose of the provision is to help facilitate development and expedite the review of drugs to treat serious or life-threatening conditions so that an approved product can reach the market expeditiously. Sponsors of drugs that receive Fast Track Designation have the opportunity for more frequent interactions with the FDA review team throughout the development program.

ELX-02 has previously been granted orphan drug designation by the FDA and orphan medicinal product designation by the European Medicines Agency.

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 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 June 30, 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.

Contact

Investors
John Woolford
john.woolford@westwicke.com
443.213.0506

Media
Laureen Cassidy
laureen@outcomescg.com

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