



Eloxx Pharmaceuticals Announces Funding Award from Cystic Fibrosis Foundation for Development of Ribosome Modulating Agents

May 27, 2021

*Leverages TURBO-ZM™ Synthetic Chemistry Platform for Designing Oral Macrolide-based Ribosome Modulating Agents (RMAs)
Complements ELX-02 program in Phase 2 Clinical Trials for the Treatment of Cystic Fibrosis*

WALTHAM, Mass., May 27, 2021 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (NASDAQ: ELOX), a leader in ribosomal RNA-targeted genetic therapies for rare diseases, today announced that the Company has received an award of up to \$2.6M from the Cystic Fibrosis Foundation. This award will help to identify optimized oral RMAs for further development in the treatment of cystic fibrosis (CF) patients with nonsense mutations.

"We are honored to receive this funding from the Cystic Fibrosis Foundation in our ongoing commitment to identify and develop new treatments for CF. Their support of novel technologies enables critical research to advance and further evaluate the potential of the TURBO-ZM™ synthetic chemistry platform to develop a new class of therapies. We have tremendous confidence in the potential of RMAs designed with TURBO-ZM™ to treat rare genetic diseases," said Sumit Aggarwal, President and Chief Executive Officer. "In preclinical models, novel RMAs have demonstrated the potential for significant readthrough and functional activity."

Mr. Aggarwal continued, "In addition, the development of a Ribosome Modulating Agent for the treatment of CF with support from the Cystic Fibrosis Foundation complements our ongoing ELX-02 program, currently in Phase 2 clinical trials. Data from these Phase 2 trials is expected in the second half of this year."

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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