Eloxx Pharmaceuticals Announces Therapeutic Development Award from Cystic Fibrosis Foundation

March 29, 2022

Cystic Fibrosis Foundation (CF Foundation) to provide an award of up to $15.9 million for the ongoing ELX-02 clinical program

Expect topline data from cystic fibrosis Phase 2 expansion treatment arms evaluating combination with ivacaftor by the end of the first half of 2022

WATERTOWN, Mass., March 29, 2022 (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 additional funding of a Therapeutic Development Award of up to $15.9 million from the CF Foundation to support the ongoing ELX-02 clinical program. This is in addition to the previously announced partial funding of the global clinical trial program.

“We are incredibly grateful for this significant level of financial and scientific support from the CF Foundation, which will enable us to build uponour clinical results announced in November 2021 and the potential of ELX-02 to bring forward a treatment for Class 1 CF patients with nonsense mutations,” said Sumit Aggarwal, President and Chief Executive Officer of Eloxx. “Class 1 CF patients with mutations do not have any available treatment options, and so our ability to work urgently is critical on behalf of patients. Furthermore, this additional award from the CF Foundation extends our cash runway into the second quarter of 2023.”

The CF Foundation has awarded funding of up to $15.9 million to support the ongoing ELX-02 global Phase 2 clinical program. Following an upfront funding of $7.0 million, the funding will be tranched based on the achievement of certain clinical milestones. Eloxx will pay the CF Foundation royalties tiered to the actual level of funding from the CF Foundation.

Patient dosing is ongoing in the expansion arm of the Phase 2 trial, which includes a combination of ELX-02 and Kalydeco (ivacaftor), a CFTR protein potentiator. In preclinical studies, Class 1 CF patient organoids had a 2- to 3-fold higher swelling response with a combination of ELX-02 and Kalydeco than with ELX-02 as a monotherapy. Topline results are expected by the end of the first half of 2022.

The U.S. Food and Drug Administration (FDA) has granted Fast Track designation for ELX-02. In addition, ELX-02 has also been granted Orphan Drug Designation for the treatment of CF patients with nonsense mutations by the FDA and orphan medicinal product designation by the European Medicines Agency.

About nonsense mutations

Nonsense mutations cause a premature stop codon in the mRNA resulting in less than full length or los of function proteins. These remain highly underserved with no approved disease modifying therapies. An estimated 10-12% patients across over 8,000 inherited genetic rare diseases harbor nonsense mutations in one or both alleles harboring nonsense mutations.

About Eloxx Pharmaceuticals

Eloxx Pharmaceuticals, Inc. is engaged in the science of ribosome modulation, leveraging its innovative TURBO-ZM^{TM} 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 (US Trial NCT04135495, EU/IL Trial NCT04126473). 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 our expected cash burn and future financial results, the expected timing of trials and results from clinical studies of our product candidates 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 quarter ended September 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/financials-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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