

Eloxx Pharmaceuticals Announces FDA Grant of Orphan-Drug Designation for Investigational Drug ELX-02 for Treatment of Cystinosis

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WALTHAM, Mass., April 27, 2018 (GLOBE NEWSWIRE) -- **Eloxx Pharmaceuticals, Inc.** (“Eloxx”), ([ELOX](#)), a clinical-stage biopharmaceutical company dedicated to the discovery and development of novel therapeutics to treat cystic fibrosis, cystinosis and other diseases caused by nonsense mutations limiting production of functional proteins, today announced that it has received orphan-drug designation from the Food and Drug Administration (FDA) for ELX-02 for treatment of cystinosis from the FDA and that it will ring the Nasdaq Closing Bell on Monday, April 30, 2018.

“We are pleased to have received an orphan-drug designation for ELX-02 for treatment of cystinosis and for the opportunity to ring the NASDAQ Closing Bell in celebration of our recent listing on the Nasdaq coincident with our successful \$50 million public offering,” said Robert E. Ward, Chairman and CEO of Eloxx Pharmaceuticals. “As we ring the closing bell, I would like to acknowledge and thank the employees, investors, patients, and partners who have supported Eloxx in our journey thus far. Given the clinical progress in 2017 for our lead product candidate, ELX-02, we are poised to seek regulatory clearance to initiate Phase 2 clinical trials in cystic fibrosis and cystinosis this year in Belgium and the United States, respectively. Our listing on the Nasdaq and capital raise position us well to advance these and other future clinical programs and pursue our mission, which is to transform the lives of patients with rare and ultra rare diseases.”

Eloxx’ s common stock trades on the Nasdaq Global Market and began trading under the symbol “ELOX” on Thursday, April 26, 2018.

A live webcast of the honorary opening bell ceremony, courtesy of Nasdaq, will begin at 3:45 am ET on Monday, April 30, 2018, and will be

available at <https://new.livestream.com/nasdaq/live> and also on the Investor Relations portion of Eloxx' s website at www.eloxxpharma.com.

About Eloxx Pharmaceuticals

Eloxx Pharmaceuticals, Inc. ([ELOX](#)) is a clinical-stage biopharmaceutical company developing novel RNA-modulating drug candidates that are designed to treat rare and ultra-rare premature stop codon diseases. Premature stop codons are point mutations that disrupt protein synthesis from messenger RNA. As a consequence, patients with premature stop codon diseases have reduced or eliminated protein production from the mutation bearing allele accounting for some of the most severe phenotypes in these genetic diseases. These premature stop codons have been identified in over 1,800 rare and ultra-rare diseases. Read-through therapeutic development is focused on extending mRNA half-life and increasing protein synthesis by enabling the cytoplasmic ribosome to read through premature stop codons to produce full-length proteins. Eloxx' s lead product candidate, ELX-02, is a small molecule drug candidate designed to restore production of full-length functional proteins. ELX-02 is in the early stages of clinical development focusing on cystic fibrosis and cystinosis. ELX-02 is an investigational drug that has not been approved by any global regulatory body. Eloxx is headquartered in Waltham, MA, with R&D operations in Rehovot, Israel.

Forward-Looking Statements

Certain statements included in this press release are forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995 and involve a number of risks and uncertainties. These include statements of management' s intentions, beliefs, plans and future expectations and, therefore, you are cautioned not to place undue reliance on them. Such forward-looking statements involve risks and uncertainties and actual results could differ materially from any forward-looking statements expressed or implied herein. The risks and uncertainties that could result in actual results to differ materially from these forward-looking statements expressed or implied herein include, but are not limited to: the development of the Company' s read-through technology; the approval of the Company' s patent applications; the Company' s ability to successfully defend its intellectual property or obtain the necessary licenses at a cost acceptable to the Company, if at all; the successful implementation of the Company' s research and development programs and collaborations; the success of the Company' s license agreements; the acceptance by the market of the Company' s products should they receive regulatory approval; the timing and success of the Company' s preliminary studies, preclinical research, clinical trials, and related regulatory filings; the ability of the Company to consummate additional financings; as well as other factors expressed

from time to time in the Company' s periodic filings with the Securities and Exchange Commission (the "SEC"). As a result, this press release should be read in conjunction with the Company' s 10-K, 10-Qs and other periodic filings with the SEC. The forward looking statements contained herein are made only as of the date of this press release, and the Company undertakes no obligation to publicly update or revise such forward-looking statements to reflect subsequent events or circumstances.