



Eloxx Pharmaceuticals Announces Opening of Clinical Trial Sites for Phase 2 Study of ELX-02 for the Treatment of Alport Syndrome

November 1, 2022

Trial sites now open in Australia and the United Kingdom

WATERTOWN, Mass., Nov. 01, 2022 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (NASDAQ: ELOX), a leader in ribosomal RNA-targeted genetic therapies for rare diseases, today announced the opening of clinical trial sites for its Phase 2 study of ELX-02 for the treatment of Alport syndrome in patients with nonsense mutations.

"Rare diseases with nonsense mutations, such as Alport syndrome, are particularly challenging to treat. We are incredibly pleased to open the first clinical trial sites for our Phase 2 study of ELX-02 for the treatment of Alport syndrome in patients with nonsense mutations, reflecting our commitment to advance our science in the interest of patients affected by rare disease. Additional sites are expected to be opened and actively recruiting Alport patients in the coming weeks," said Sumit Aggarwal, President and Chief Executive Officer of Eloxx. "Alport patients with nonsense mutations have significantly worse clinical outcomes than other Alport patients and have no disease modifying treatment options."

This proof-of-concept clinical trial will include up to eight Alport syndrome patients with nonsense mutations in the second half of 2022. Patients will be dosed for two months with a three month follow-up. Trial primary endpoints will include safety, while secondary endpoints will include reduction in proteinuria and induction of COL4A5 protein expression in the kidney. Topline results are expected in the first half of 2023.

Eloxx believes there is a strong rationale to pursue clinical development of ELX-02 in Alport syndrome, based on encouraging preclinical results demonstrating potentially therapeutic levels of readthrough, ability to deliver high drug concentrations in the kidney relative to plasma at clinically tolerable dose levels, and the clinical readthrough results seen in our Phase 2 cystic fibrosis trial.

About Alport syndrome

Alport syndrome is a genetic disorder characterized by kidney disease with high levels of proteinuria, hearing loss and eye abnormalities caused by mutations in the genes (COL4A3, COL4A4, and COL4A5) needed for production of type 4 collagen. Approximately 6% to 7% of Alport syndrome patients, or approximately 9,400 to 12,750 individuals, are estimated to have nonsense mutations. These patients have significantly worse clinical outcomes than other Alport patients and have no disease modifying treatment options.

About Nonsense Mutations

Nonsense mutations cause a premature stop codon in the mRNA resulting in less than full length or loss 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-ZMTM 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. The U.S. Food and Drug Administration (FDA) has granted Fast Track designation for ELX-02 for the treatment of CF patients with nonsense mutations. 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 Commission. 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, the expected timing of trials 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 June 30, 2022, 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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