Eloxx Pharmaceuticals Announces New Development Program for ELX-02 for the Treatment of Alport Syndrome

March 30, 2022

Strong rationale for development for Alport syndrome as ELOX-02 has demonstrated significant readthrough in COL4A5 nonsense mutations in preclinical studies and is preferentially transported to kidney

Initiation of proof-of-concept clinical trial expected in second half of 2022; topline results expected in first half of 2023

WATERTOWN, Mass., March 30, 2022 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (NASDAQ: ELOX), a leader in ribosomal RNA-targeted genetic therapies for rare diseases, today announced it has expanded the ELX-02 development program to include the treatment of Alport syndrome, a rare kidney disease. Clinical testing of ELX-02 in Alport syndrome is expected to initiate in the second half of 2022, with topline results expected in the first half of 2023.

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.

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.

“We are beginning to fully capture the potential of ELX-02 as a novel readthrough agent in rare diseases with significant need with the addition of a new program in Alport syndrome,” said Sumit Aggarwal, President and Chief Executive Officer of Eloxx.

"For the global patient community, this study represents a groundbreaking and hopeful step as the first to explore a potential curative therapy in a specific genetic mutation of our rare disease,” notes Lisa Bonebrake, Executive Director of the U.S. based patient support group, Alport Syndrome Foundation.

ELX-02 is preferentially taken up in the kidney, resulting in an expected greater than 50-fold exposure in the kidneys compared to plasma. In recently published preclinical studies, ELX-02 has demonstrated readthrough in COL4A5 mutations, which represent 85% of nonsense mutations in this population. We believe that the results of the treatment of Class 1 CF patients with ELX-02 monotherapy and its high local kidney drug levels make it well suited to potentially deliver transformative results in these patients.

Eloxx intends to initiate a proof-of-concept clinical trial in 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.

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™ 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 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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