Eloxx Pharmaceuticals Provides Program Updates on ELX-02 and ZKN-013

September 7, 2023

Investigational New Drug (IND) application submitted to U.S. Food and Drug Administration (FDA) for ELX-02 for the treatment of Alport syndrome with nonsense mutations

Rebound in average UPCR 3-months post treatment provides further evidence that proteinuria remission in one out of three patients was drug related

All 3 Alport patients (100% response rate) treated with ELX-02 had biopsy confirmed disease regression, suggesting clinical benefit likely with longer treatment duration

Significant strategic interest in ZKN-013 following FDA approval to start Phase 1 single ascending dose (SAD) trial

WATERTOWN, Mass., Sept. 07, 2023 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (NASDAQ: ELOX), a leader in ribosomal RNA-targeted genetic therapies for rare diseases, today provided an update on the continued advancement of ELX-02 for the treatment of Alport syndrome with nonsense mutations, including additional positive results from its Phase 2 clinical study evaluating ELX-02, as well as an update on ZKN-013.

Alport syndrome is a rare genetic kidney disorder caused by mutations in COL4A3/4/5 genes, characterized by podocyte injury and impaired kidney filter function leading to proteinuria.

"We have shared the results of our Phase 2 study with several key opinion leaders and key stakeholders within the Alport syndrome community. They have expressed uniform and overwhelming strong support for the potential of ELX-02 to treat this devasting indication and the need to advance into a pivotal study," said Sumit Aggarwal, President and Chief Executive Officer of Eloxx.

IND Submitted to U.S. FDA for Alport Syndrome

Based on the encouraging clinical results to date, Eloxx has submitted an IND application with the U.S. FDA for ELX-02 for the treatment of Alport syndrome with nonsense mutations. Allowance of the IND will allow for the inclusion of U.S.-based sites in the planned pivotal trial.

Biopsy Results from ELX-02 Phase 2 Alport Trial Support Protein Restoration

All three patients (100% response rate) treated with ELX-02 in its proof-of-concept Phase 2 open-label clinical trial (NCT05448755) showed an improvement in podocyte foot process effacement, a hallmark of proteinuric kidney diseases like Alport syndrome. Electron microscopy images of biopsy samples showed an improvement in the glomerular basement membrane and a re-attachment of podocyte foot processes in all treated patients. This confirms protein restoration and the disease modifying effect of ELX-02 and potential for improvement in proteinuria with longer duration of treatment. Podocytes are specialized cells that bind to the glomerular basement membrane and form finger-like extensions called foot processes that enable efficient ultrafiltration. Podocyte injury leads to the effacement (loss) of podocyte foot processes and proteinuria in nearly all cases of Alport syndrome. Eloxx previously announced achievement of remission in one patient

Eloxx is announcing today 3 month post treatment results. The increase in UPCR following withdrawal of therapy confirms likely on-treatment remission in one out of three patients, providing additional evidence of drug activity.

Patient	Average UPCR (mg/g) at baseline	Average UPCR (mg/g) during treatment over 8 weeks	Average UPCR (mg/g) 3 months post end of treatment
Patient 4401-01	1,299	1,799	2,328
Patient 4401-02*	**1,646**	**850**	**1,495**
Patient 4402-01	1,659	2,209	1,559

*Patient who achieved remission.

Strategic Interest in ZKN-013

As we previously announced, the FDA has cleared the company to begin a SAD trial in healthy volunteers for ZKN-013 for the treatment of recessive dystrophic epidermolysis bullosa (RDEB) and junctional epidermolysis bullosa (JEB).

Eloxx has since received significant strategic interest in ZKN-013, which the company is actively pursuing. Should these discussions lead to a transaction, it will allow Eloxx to remain focused on fully maximizing the potential of ELX-02 in rare kidney diseases and continue funded discovery efforts on our TURBO-ZM platform.

About Eloxx Pharmaceutical

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. 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 September 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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