Eloxx Pharmaceuticals Highlights Recent Alport Syndrome Natural History Data Presented at 60th ERA Congress

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Data from RaDaR natural history study indicates that Alport syndrome patients with autosomal recessive COL4A4 mutations have severest disease, with a more rapid progression to kidney failure

Patient that achieved remission in Eloxx Phase 2 trial had autosomal recessive COL4A4 nonsense mutation resulting in a truncated protein

RaDaR registry data further supports decision to advance into a pivotal trial in Alport syndrome with nonsense mutations

WATERTOWN, Mass., June 21, 2023 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (NASDAQ: ELOX), a leader in ribosomal RNA-targeted genetic therapies for rare diseases, today highlighted recent Alport syndrome natural history data presented at the 60th ERA (European Renal Association) Congress.

Katie Wong, Clinical Research Fellow at University College London Department of Renal Medicine, presented “Alport Syndrome Natural History from the RaDaR Registry: Associations with gene, variant type and sex”, a natural history study that aims to describe demographics and investigate renal outcomes associated with pathogenic mutations in Alport syndrome patients. Longitudinal data is collected from the National Registry of Rare Kidney Diseases (RaDaR) which recruits patients at 108 UK renal clinics. Eloxx’s Ali Hariri, M.D., Chief Medical Officer of Eloxx, was involved in the study.

Overall, data from RaDaR natural history study, which included 920 patients in the analysis, demonstrated that the observed effect of mutation type on renal outcomes varied by gene affected, number of mutations, and gender. One key finding indicates that approximately 11% of Alport syndrome patients have autosomal recessive COL4A4 mutations and have severest disease. The subset of these patients with truncated COL4A4 proteins, had a 2- to 3-fold more rapid progression to kidney failure compared to patients with truncated COL4A5 proteins with male patients having the worst outcomes.

In the current Phase 2 study in patients with Alport syndrome, the first two patients for which the company has provided data were both males with autosomal recessive COL4A4 nonsense mutations resulting in a truncated COL4A4 protein. As these patients have this highly progressive autosomal recessive disease, Eloxx believes a remission in even one patient is highly clinically significant.

“Rare diseases benefit from natural history studies to help researchers understand disease progression and help inform meaningful clinical intervention. Our work to advance ELX-02 for Alport patients has been targeted to those with nonsense mutations. We now know that patients with the COL4A4 mutation are the most at risk for rapid advancement to kidney failure. This RaDaR registry data, coupled with recently announced results from our Phase 2 trial, give us even greater confidence in our decision to advance into a pivotal trial in Alport syndrome with nonsense mutations,” said Sumit Aggarwal, President and CEO of Eloxx. “We are grateful to the patients who participated in the natural history study, honored to participate in the research, and look forward to continuing our support of the Alport syndrome community.”

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 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 Phase 2 clinical development for the treatment of Alport syndrome in patients with nonsense mutations. 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 and results from 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; our ability to meet the continued listing requirements of the Nasdaq Capital Market; 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 quarterly period ended March 31, 2023, 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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