Eloxx Pharmaceuticals Intends to Advance ELX-02 into Pivotal Trial for the Treatment of Alport Syndrome with Nonsense Mutations Following Achievement of Remission in Patient in Phase 2 Study

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Patient treated in trial achieved a remission after eight weeks of treatment, demonstrating a significant reduction in proteinuria

ELX-02 well-tolerated in study, with no discontinuations to date

WATERTOWN, Mass., May 24, 2023 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (NASDAQ: ELOX), a leader in ribosomal RNA-targeted genetic therapies for rare diseases, today announced that the company intends to advance ELX-02 into a pivotal trial for the treatment of Alport syndrome with nonsense mutations, following the achievement of remission in one patient in its Phase 2 clinical study. The patient demonstrated a significant reduction in urine protein creatinine ratio (UPCR) from baseline. Consistent with prior clinical studies, ELX-02 was well tolerated in this trial.

“The achievement of a remission in one of the first two patients with Alport syndrome with nonsense mutations in this trial of ELX-02 is a very encouraging result, as patients with this rare disease rarely demonstrate a reduction in proteinuria,” said Detlef Bockenhauer, MD., Ph.D., Professor of Paediatric Nephrology at University College London and Honorary Consultant at Great Ormond Street Hospital NHS Foundation Trust, London. “Based on the efficacy and tolerability to date, additional clinical testing is warranted in this patient population in need of disease-modifying treatments.”

Topline Results of ELX-02 Phase 2 Trial for Alport Syndrome

- Eloxx has dosed three patients with ELX-02 in the ongoing proof-of-concept Phase 2 open-label clinical trial (NCT05448755). Two patients have now completed dosing and the third patient is still receiving ELX-02.
- One patient that has completed dosing achieved a remission, defined as a UPCR decline greater than or equal to 50%. For this patient, five out of eight UPCR readings produced an average of 53% below baseline. Spontaneous reductions in proteinuria are not expected in this population, providing added weight to this result.
- The patient achieving remission also demonstrated a significant reduction (-49%) (-p-value p=0.009) in UPCR from baseline versus values collected over the treatment period.
    - The UPCR value for week 6 was excluded from this analysis as it was deemed to be unreliable due to delayed processing over a holiday period, potentially resulting in protein degradation and inaccurate proteinuria readings.
- No significant change in UPCR was seen in the other patient that has completed 8 weeks of dosing.
- Both patients had the same Col4A4 mutation, but were being treated with substantially different levels of renin-angiotensin-aldosterone system (RAAS) inhibitors.
- Assessment of COL IV protein restoration remains ongoing as both patients had Col4A4 mutations and the kidney biopsy assay was designed to assess COL IVA5 protein expression.
- Consistent with previous clinical studies, ELX-02 has been generally well tolerated with no discontinuations to date.
    - Kidney biopsies from both patients after 8 weeks of dosing demonstrated no evidence of nephrotoxicity, further demonstrating tolerability.

“We are incredibly pleased with these initial findings from our Phase 2 trial of ELX-02 for the treatment of Alport syndrome in patients with nonsense mutations. These patients have significant unmet medical needs, with no disease modifying treatments currently available,” said Sumit Aggarwal, President and Chief Executive Officer of Eloxx. “Following completion of the third patient in the trial, we plan to discuss the findings with the FDA with the goal of advancing the program into a pivotal trial, pending obtaining the necessary capital.”

Eloxx intends to provide more detailed results from the trial at an upcoming medical meeting.

About the Phase 2 Clinical Study

This Phase 2 trial includes Alport syndrome patients with Col4A5 and Col4A3/4 nonsense mutations in the COL4 gene. Patients will be dosed with ELX-02 for two months with a three month follow-up. In addition to the primary endpoint of safety, the key secondary efficacy endpoint of change from baseline in proteinuria will be measured every two weeks. Treatment effect on proteinuria is a well-validated surrogate endpoint for several renal indications and may be a good predictor of treatment outcomes. For eligible patients, expression of COL4 will also be measured at the end of the two month treatment period.

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™ 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 our cash runway and our ability to comply with the covenants in our debt agreement, 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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