Eloxx Pharmaceuticals Reports Independent Confirmation of Positive Biopsy Results in All Patients Treated with ELX-02 in Phase 2 Clinical Study for Alport Syndrome

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Highly regarded renal pathologist and transmission electron microscopy (TEM) expert independently confirms previously reported qualitative assessment by Mayo Clinic of TEM biopsy scans

All three patients treated with ELX-02 showed a visual improvement in podocyte foot process effacement post-treatment in kidney biopsies demonstrating the disease modifying effect of ELX-02

Podocyte foot process effacement is a hallmark of Alport syndrome

Eloxx intends to gain alignment with U.S. Food and Drug Administration (FDA) on design of pivotal trial and potential for seeking Breakthrough Therapy Designation

WATERTOWN, Mass., Sept. 18, 2023 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (NASDAQ: ELOX), a leader in ribosomal RNA-targeted genetic therapies for rare diseases, today reported additional independent confirmation on the positive TEM assessment results from its proof-of-concept Phase 2 open-label clinical trial (NCT05448755) of ELX-02 for the treatment of Alport syndrome after eight weeks of treatment. Visual assessment of TEM images from kidney biopsies showed an improvement in foot process effacement in all three treated patients consistent with disease regression. 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.

Additional independent confirmation has now been reported. The qualitative assessment of images of pre- and post-treatment kidney biopsies from the three patients in the Phase 2 clinical trial noted:

- **Patient 4401-01 and Patient 4401-02:** *All images showed more regions* of glomerular basement membranes in post-treatment biopsies *covered by intact foot processes, consistent with partial improvement of podocyte injury in those biopsies.* As previously announced, Patient 4401-02 achieved remission based on Urine-Protein Creatine ratio (UPCR).
- **Patient 4402-01:** Post-treatment images showed wider areas of intact foot processes compared with either pre- or post-treatment images for Patient 4401-01 and Patient 4401-02.

These findings are generally consistent with previous assessments, providing stronger evidence of protein restoration and the disease modifying effect of ELX-02 and potential for improvement in proteinuria with longer duration of treatment.

“We are incredibly pleased with this additional confirmation from a globally renowned expert in interpreting the findings from kidney biopsies,” said Sumit Aggarwal, President and Chief Executive Officer of Eloxx. “We look forward to initiating a pivotal trial as we believe ELX-02 has the potential to make a meaningful impact on the lives of patients with Alport syndrome.”

Eloxx previously announced achievement of a rapid and sustained remission in one patient in the Phase 2 clinical trial. Based on the results from the Phase 2 trial, Eloxx intends to gain alignment with the FDA on the design of pivotal trial for ELX-02 for the treatment of Alport syndrome with nonsense mutations and the potential for seeking Breakthrough Therapy Designation. Eloxx recently submitted an Investigational New Drug application (IND) to the FDA for ELX-02 for the treatment of Alport syndrome with nonsense mutations. The IND, if allowed, would enable the inclusion of U.S.-based sites in the planned pivotal 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 patients with Alport syndrome 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 historical facts contained in this press release, including without limitation, statements regarding the potential of our product

candidate to treat Alport syndrome and intentions regarding communications with FDA for alignment on a potential pivotal trial and a Breakthrough Therapy Designation submission 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 June 30, 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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