Eloxx Pharmaceuticals Reports Drug Response in All Patients Treated with ELX-02 in Phase 2 Clinical Study for Alport Syndrome

August 14, 2023

All three patients (100% response rate) treated with ELX-02 showed an improvement in podocyte foot process effacement post-treatment in kidney biopsies assessed by electron microscopy demonstrating the disease modifying effect of ELX-02

Podocyte foot process effacement is a hallmark of Alport syndrome

Together with previously announced rapid and sustained proteinuria remission in one patient in Phase 2 clinical study, these biopsy results strongly support decision to advance ELX-02 into a pivotal trial in Alport syndrome

WATERTOWN, Mass., Aug. 14, 2023 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (NASDAQ: ELOX), a leader in ribosomal RNA-targeted genetic therapies for rare diseases, today reported additional positive 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. Electron microscopy assessment of kidney biopsies demonstrated an improvement in foot process effacement in all three treated patients. These results are consistent with the disease modifying effect of restoration of COL4A4 protein with ELX-02 and with previously reported data from preclinical studies.

Alport syndrome, a rare genetic kidney disorder caused by mutations in COL4A3/4/5 genes, is characterized by podocyte injury and impaired kidney filter function leading to proteinuria. 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. This is the first potential therapy for Alport syndrome that has shown an improvement of podocyte foot process effacement.

“These are profound results. The improvement in podocyte foot process effacement in all three patients treated confirms the disease modifying potential of ELX-02. Combined with the reduction of proteinuria achieved in one patient, these data strengthen our confidence in ELX-02’s potential to treat Alport syndrome patients with nonsense mutations,” 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 be transformational in this underserved patient population.”

Electron microscopy assessment of the kidney biopsies in the Phase 2 study showed a reduction in podocyte foot process effacement, indicative of the disease modifying impact of ELX-02 all three patients. In two patients, widespread foot process effacement was improved to segmental foot process effacement. In the third patient, moderate to severe foot process effacement was improved to moderate only. Representative TEM images from Patient 4401-02 can be accessed here

Patient	Pre-treatment	Post -treatment
4401-01	Widespread foot process effacement present	Segmental foot process effacement present
4401-02*	Widespread foot process effacement present	Segmental foot process effacement present
4402-01	Moderate to severe foot process effacement present	Moderate segmental foot process effacement present

*Patient that achieved remission.

“As a physician scientist with a longtime focus on podocytes, I find these electron microscopy results compelling since the demonstrated improvement in podocyte foot process effacement shows that ELX-02 has substantial potential to treat Alport syndrome,” said Dr. Peter Mundel, a renowned expert in kidney diseases. “With longer treatment duration, I expect additional clinical benefit beyond the results seen in the Phase 2 trial.”

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 advance ELX-02 into pivotal trial for the treatment of Alport syndrome with nonsense mutations.

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 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 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.

Contact

Investors
John Woolford
john.woolford@westwicke.com
443.213.0506

Media
Laureen Cassidy
laureen@outcomescg.com

SOURCE: Eloxx Pharmaceuticals, Inc.

Source: Eloxx Pharmaceuticals