Eloxx Pharmaceuticals Key Opinion Leader Event Highlights Significant Unmet Need in Treatment of Alport Syndrome Patients with Nonsense Mutations and Additional Positive Results from Phase 2 Clinical Study Evaluating ELX-02

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Alport syndrome is a rare progressive hereditary glomerular kidney disease caused by variants in COL4A and patients have significant unmet medical needs, with no disease modifying treatments currently available

Eloxx announced additional results from its Phase 2 ELX-02 trial showing the patient that achieved a remission rebounded one month after withdrawing treatment, providing additional evidence of drug activity

Eloxx intends to advance into a pivotal trial of ELX-02 for the treatment of Alport syndrome with nonsense mutations

WATERTOWN, Mass., June 28, 2023 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (NASDAQ: ELOX), a leader in ribosomal RNA-targeted genetic therapies for rare diseases, today highlighted its recently held key opinion leader (KOL) event discussing the significant unmet medical need of patients with Alport syndrome and presented additional topline results from its Phase 2 clinical study (NCT05448755) evaluating ELX-02 for the treatment of Alport syndrome.

The event featured two globally renowned Alport syndrome experts:

- Detlef Bockenhauer, MD., Ph.D., Professor of Paediatric Nephrology, University Hospital and KU Leuven
- Professor Rachel Lennon, Ph.D., Professor of Nephrology, Consultant Paediatric Nephrologist at the Royal Manchester Children's Hospital, Director of the Wellcome Centre for Cell-Matrix Research at the University of Manchester, Director of the Stoneygate and Kidney Research UK Alport Research Hub

“Alport Syndrome is a progressive disease caused by a genetic defect in Collagen Type IV protein. There's no approved therapy currently and our current standard of care is supportive care,” said Professor Rachel Lennon, Ph.D. “Once we see persistent proteinuria, our objective is to reduce that to a lower level. Ultimately, these individuals will progress with our best standard of care right now to requiring dialysis or a kidney transplant.”

“In one of these three patients, there has been a substantial reduction in the proteinuria, pretty much half of it. So it is consistent with the remission of proteinuria,” said Detlef Bockenhauer, MD., Ph.D. “Spontaneous remission in this disease has not been described. It's possible in some other glomerular diseases, but this is a chronic progressive disease, so we do not see spontaneous remission. If there is an improvement in the proteinuria than this really has to do with the effect of the drug.”

“The two drugs that were previously tested but failed in Alport did not have the biological plausibility that is as strong as we have here [with ELX-02],” added Professor Lennon. “If we can increase the amount of Type IV Collagen, there’s already strong animal data that we can extend kidney survival.”

Eloxx provided topline data for 8-weeks of treatment for the third patient in the study and data after 4, and 8-weeks after end of treatment in all three patients that have completed treatment.

Patient	Average change in Urine Protein to Creatinine (UPCR) during treatment over 8 weeks	UPCR levels in patients at 4 weeks and 8 weeks after end of treatment
Patient 4401-01	-49% (Achieved remission) (p=0.009)	Regressed to baseline (+97% vs. end of treatment)
Patients 4401-01 and 4402-01	No change	No change

The rapid increase in UPCR after end of treatment in one patient who achieved remission during the trial provides additional evidence of biological activity of ELX-02 in this population. As observed in prior clinical studies, ELOX-02 was well tolerated in this trial. Based on these results, Eloxx intends to advance ELX-02 into a pivotal trial for the treatment of Alport syndrome with nonsense mutations.

“Eloxx’s recent announcement that one Alport syndrome patient in its Phase 2 trial of ELX-02 achieved remission is an extremely important milestone in addressing the needs of these patients have significantly worse clinical outcomes than other Alport patients and have no disease modifying treatment options,” said Professor Bockenhauer.

A replay of the webcast and presentation is available on www.eloxxpharma.com under "Events & Presentations" in the Investors section of the website for 30 days.

“We were delighted to bring more attention to the debilitating unmet medical needs of Alport syndrome patients during this event,” said Sumit Aggarwal, President and Chief Executive Officer of Eloxx. “Further, we are extremely encouraged by the findings from our Phase 2 trial of ELOX-02 for the treatment of Alport syndrome in patients with nonsense mutations and look forward to advancing this program into a pivotal open label study,

pending obtaining the necessary capital."

About the Phase 2 Clinical Study

This Phase 2 trial included Alport syndrome patients with nonsense mutations in the COL4 gene. Patients were 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 proteinuria was measured every two weeks. Treatment effect on proteinuria is a well-validated endpoint for several renal indications and a good predictor of treatment outcomes. For eligible patients, induction of COL4 was also measured at the end of two months.

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.

Contact

Investors
John Woolford
john.woolford@westwicke.com
443.213.0506

Media
Laureen Cassidy
laureen@outcomescg.com

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