Eloxx Pharmaceuticals Announces Final Data Assessment from Phase 2 Combination Clinical Trial of ELX-02 in Class 1 Cystic Fibrosis (CF) Patients

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ELX-02 trial results demonstrated clinically relevant improvement in percent predicted forced expiratory volume (ppFEV1) based on final data assessment; initial topline results were previously reported in September 2022

Efficacy of ELX-02 in CF study bolsters strength of recently announced results in Phase 2 Alport syndrome trial, further supporting Eloxx's decision to advance into a pivotal trial in Alport syndrome

WATERTOWN, Mass., June 14, 2023 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (NASDAQ: ELOX), a leader in ribosomal RNA-targeted genetic therapies for rare diseases, today announced the final data assessment from the Phase 2 clinical trial of ELX-02 in combination with ivacaftor in Class 1 CF patients with at least one nonsense mutation. In the final assessment, ELX-02 demonstrated clinically relevant improvement in ppFEV1. The final data assessment includes a reanalysis using change in ppFEV1 from Day 1 instead of baseline, as multiple patients experienced disease progression between screening and treatment. Initial topline results from this trial were reported in September 2022.

"We believe that the clinical improvements with ELX-02 observed in a trial of CF patients with severe illness, coupled with remission observed in one patient in the ongoing Phase 2 trial of ELX-02 in Alport syndrome patients with nonsense mutations, reinforces our belief in the disease-modifying potential of ELX-02 and warrants further development. Similar to the patients in this CF trial, participants in our Phase 2 Alport syndrome trial also had highly progressive autosomally recessive disease, making a remission in even one patient highly clinically significant. The body of data strongly validate our decision to advance ELX-02 into a pivotal study in Alport syndrome," said Sumit Aggarwal, President and CEO of Eloxx.

Mr. Aggarwal continued, "The high concentrations of ELX-02 observed in the kidney in those with Alport Syndrome make the disease an ideal target for further study, as the ELX-02 exposure in the Alport study is at least 25-fold higher than in the CF trial."

Final Assessment of ELX-02 Phase 2 Combination Trial in Class 1 CF Patients

The Phase 2 combination clinical trial of ELX-02 was designed to evaluate safety and assess biological activity in G542X nonsense mutation Class 1 CF patients as monotherapy and in combination with ivacaftor after 5 weeks of treatment. Results from the final analysis of 13 patients evaluable, versus 11 at time of initial assessment are summarized below:

- 6 of 13 patients entered trial from monotherapy arm (after average 463 days) and had a decrease in lung function (annualized -4.26% reduction in ppFEV1) due to disease progression.
- Treatment with ELX-02 stabilized disease overall and resulted in a clinically relevant increase in ppFEV1 in six of thirteen patients based on change in ppFEV1 at the end of treatment at Day 35 compared to the start of treatment at Day 1.

Number or patients	Change in ppFEV1 (%) at end of treatment at Day 35 vs Day 1	Change in ppFEV1 (%) at Safety follow up vs Day 35
Overall, n=13	-0.31%	-2.69%
Responders, n=6	+2.83%	-5.83%

- Topline data previously confirmed biological activity. Patients with higher baseline sweat chloride levels had increased responses to treatment as indicated by sweat chloride concentration (p=0.00013 at Day 35).
- ELX-02 was generally well tolerated in the trial, with no treatment-related serious adverse events noted.

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 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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