Eloxx Pharmaceuticals Reports Topline Results from Phase 2 Combination Clinical Trial of ELX-02 in Class 1 Cystic Fibrosis (CF) Patients

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Combination of subcutaneous ELX-02 with ivacaftor did not achieve statistical significance for efficacy endpoints in Phase 2 study in Class 1 CF

ELX-02 was well tolerated with no drug-related serious adverse events observed

Evidence of activity for ELX-02 observed; efficacy signal potentially confounded by variability due to low drug exposure

Path forward for ELX-02 for the treatment of Class 1 CF to be determined together with CF Foundation

Company to host conference call and webcast today, September 14, 2022, at 4:30 p.m. ET

WATERTOWN, Mass., Sept. 14, 2022 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (NASDAQ: ELOX), a leader in ribosomal RNA-targeted genetic therapies for rare diseases, today announced topline results from the Phase 2 clinical trial of ELX-02 in combination with ivacaftor in Class 1 cystic fibrosis (CF) patients with at least one nonsense mutation. The combination trial of ELX-02 with ivacaftor was well tolerated but did not achieve statistical significance for efficacy endpoints, including changes from baseline in sweat chloride concentration (SCC) and percent forced expiratory volume (FEV1).

“We are disappointed that ELX-02 failed to achieve statistical significance for its key efficacy endpoints in this Phase 2 trial in combination with ivacaftor for the treatment of Class 1 CF. Despite this setback, we were pleased to observe that ELX-02 was well tolerated and demonstrated additional evidence of activity in this underserved patient population. We will work closely with the CF Foundation, as it has generously supported this trial, to determine the next steps in the development of ELX-02 for CF,” said Sumit Aggarwal, President and Chief Executive Officer of Eloxx.

Mr. Aggarwal continued, “Given the safety and evidence of activity we have observed to date with ELX-02, including in this trial, we look forward to initiating a proof-of-concept trial for ELX-02 in Alport syndrome, a rare kidney disease, later this year. Given the likelihood of increased drug exposure, as ELX-02 is preferentially taken up in the kidneys, we believe ELX-02 is well suited to potentially deliver transformative results in these patients.”

Topline Results 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. The trial included a 1-week monotherapy period (1.5 mg/kg daily subcutaneous) followed by a four week combination period (1.5 mg/kg daily subcutaneous and 150 mg ivacaftor twice daily). Topline results are summarized below:

- ELX-02 was generally well tolerated in the trial, with no treatment-related serious adverse events noted.
- Overall, the study did not achieve statistical significance for efficacy endpoints in the Phase 2 study in Class 1 CF for efficacy endpoints, including changes from baseline in SCC and FEV1.
    - No incremental improvement was observed with ivacaftor combination.
- Evidence of activity for ELX-02 was observed, as patients with higher baseline sweat chloride levels demonstrated increased responses as indicated by SCC (p=0.00013 at Day 35).
- Trial results were potentially confounded by high variability in sweat chloride and lung function measurement.
- Eloxx believes this variability could have been caused by very low drug exposures in the lung. Steady state lung drug levels in patients from this trial were on average 20%, or 2μM, of the lowest levels at which drug activity has previously been seen in preclinical testing.
    - Lung drug exposure with inhaled delivery of ELX-02 expected to be at least 50-fold greater than with subcutaneous delivery.

About Class 1 CF

CF patients with a Class 1 nonsense mutation remain highly underserved with no approved disease modifying therapies. An estimated 10-12% of CF patients are Class 1 patients with one or both alleles harboring nonsense mutations, leading to less than full length CFTR proteins on the cell membrane in these patients.

Conference Call and Webcast

Eloxx’s management will host a conference call and webcast today at 4:30 p.m. ET. A live webcast of the conference call can be accessed through the “Investors” tab on the Eloxx website, and a replay will be available online after the call. For those planning to ask a question, the dial-in number for the conference call is (888) 672-2415 for domestic participants and (646) 307-1963 for international participants, with Conference ID # 7410846. Please dial in at least 15 minutes in advance to ensure a timely connection to the call.

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. The U.S. Food and Drug Administration (FDA) has granted Fast Track designation for ELX-02 for the treatment of CF patients with nonsense mutations. In addition, ELX-02 has also been granted Orphan Drug Designation for the treatment of CF patients with nonsense mutations by the FDA and orphan medicinal product designation by the European Commission. ELX-02 is in clinical development, focusing on cystic fibrosis (US Trial NCT04135495, EU/IL Trial NCT04126473). Eloxx also has preclinical programs focused on select rare diseases, including inherited diseases, cancer caused by nonsense mutations, kidney diseases, including autosomal dominant polycystic kidney disease, as well as rare ocular genetic disorders.

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, the expected timing of 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; 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 quarter ended June 30, 2022, 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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