Eloxx Pharmaceuticals Provides ELX-02 and ZKN-013 Program Updates

April 16, 2024

ELX-02 Granted Orphan Drug Designation (ODD) from U.S. Food and Drug Administration (FDA) for Treatment of Alport Syndrome

Pre-Investigational New Drug (IND) Meeting Requested with U.S. FDA for ELX-02 to Discuss Planned next Study In Nonsense Mutation Alport Syndrome

Signed Global Licensing Partnership for ZKN-013 with Almirall Pharmaceuticals with $3M upfront and up to $470M in milestones and Tiered Royalties on Global Sales

New Paper Published on Autosomal Dominant Polycystic Kidney Disease (ADPKD) shows that ELX-02 treatment prevents cyst formation in diseased PKD organoids with nonsense mutations and a single healthy gene copy

WATERTOWN, Mass., April 16, 2024 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (OTC: ELOX), a leader in ribosomal RNA-targeted genetic therapies for rare diseases, today provided program updates for ELX-02 and ZKN-013, including Orphan Drug Designation (ODD) for ELX-02.

Eloxx has significantly advanced the development of ELX-02 for the treatment of Alport syndrome with Nonsense Mutations (NMAS). Additionally, with the global licensing partnership with Almirall announced in March, the company has begun the development of ZKN-013 for the treatment of Recessive Dystrophic Epidermolysis Bullosa (RDDEB) and Familial Adenomatous Polyposis (FAP) patients with nonsense mutations. The publication of a recent paper on Autosomal Dominant Polycystic Kidney Disease (ADPKD), confirms the potential of ELX-02 for the treatment of all rare genetic kidney diseases with nonsense mutations in the disease causing genes.

“The recent ELX-02 program updates, including Orphan Drug Designation for ELX-02 for the treatment of Alport Syndrome highlights the significant unmet medical needs of Alport Syndrome patients with Nonsense Mutations,” said Sumit Aggarwal, President and Chief Executive Officer of Eloxx. “The designation further strengthens our belief that ELX-02 has the potential to be transformational in supporting the ultra-rare subset of Alport Syndrome patient population with nonsense mutations. We look forward to engaging the FDA on initiating the larger clinical trial to support the potential for regulatory marketing approval of ELX-02 in this patient population.”

ELX-02: Regulatory and Clinical Updates for Alport Syndrome Nonsense Mutation Program

- **ELX-02 Granted ODD from U.S. FDA:** In April, the FDA Office of Orphan Products Development (OOPD) granted ODD for ELX-02 for the treatment of Alport Syndrome.
    - The designation was based on a review of the prevalence of NMAS and the data from the Proof-of-concept Phase 2 Study that Eloxx announced top-line results for in 2023.
    - FDA ODD is granted to investigational therapies addressing rare medical diseases or conditions that affect fewer than 200,000 people in the United States with a reasonable clinical or preclinical hypothesis for efficacy in the target population.
- **Requested a Pre-Investigational New Drug (IND) meeting with U.S. FDA:** The request for a pre-IND meeting formally initiates communications with the FDA regarding development of ELX-02 for the treatment of NMAS.
    - The purpose of the pre-IND meeting will be to request feedback on the Eloxx’s planned clinical trial in nonsense mutation Alport syndrome patients.
- **Presented Clinical Data Updates at Alport Workshop Meeting in March:** Prof. Daniel Gale presented updated results from the Proof-of Concept Study in 3 NMAS patients treated with ELX-02
    - Meaningful reduction in Podocyte Foot Process Effacement after treatment measured as an average 60% increase in Filtration Slit Density in post treatment kidney biopsies in all 3 patients.
    - Results confirm the expression of functional collagen 4 protein in-line with the mechanism of protein induction of ELX-02.
    - Changes in UPCR noted to be consistent with biopsy results with reduction or stabilization of proteinuria during or up to 2 months post completion of dosing.

ZKN-013: Update on Global Licensing Partnership with Almirall

In March 2024, Eloxx announced an exclusive license agreement with Almirall to develop and commercialize ZKN-013 in orphan indications including RDEB and FAP with nonsense mutations.

- **Received $3 million in upfront payment**
- **Additional payments of ELX-02 of up to $470 million and tiered royalties on global sales:** Eloxx shall be eligible for additional payments throughout the potential development phases, including regulatory and sales milestones of up to $470 million as well as tiered royalties on any potential global sales
- **Initiated Phase 1 Single Ascending Dose (SAD) study:** Initiated Phase 1 SAD study in Australia with initial results expected in 2nd half of 2024

Recently Published Paper on Autosomal Dominant Polycystic Kidney Disease (ADPKD) may Have Other Rare Kidney Disease Applications

A paper was published in April 2024, on ADPKD titled *Genetics of cystogenesis in base-edited human organoids reveal therapeutic strategies for polycystic kidney disease*, authored by Courtney E. Vishy, Chardai Thomas, Thomas Vincent, Daniel K. Crawford, Matthew M. Goddeeris, and Benjamin S. Freedman. Freedman and colleagues engineered base-edited human iPS cells to resolve the functional genetics of human PKD. They discovered that cyst formation in diseased organoids could be prevented with a single healthy gene copy. This insight enables the demonstration of both genetic and pharmacological therapeutic approaches for clinically relevant nonsense mutations and may have the potential to support other rare kidney diseases.

About Nonsense Mutation Alport Syndrome

Nonsense Mutation Alport syndrome (NMAS) is an ultra-rare Type IV Collagenopathy characterized by nonsense mutations in the genes (*COL4A3, COL4A4, and COL4A5*) that result in a less than full length (truncated) Type 4 Collagen. This disorder mostly affects children with a median age at diagnosis of 9 to 20 years. It is characterized by rapid and progressive damage to the kidneys, ear, and eyes, starting with worsening of kidney morphology to proteinuria and finally kidney failure, hearing loss and eye abnormalities. 90% NMAS patients progress to kidney failure and hearing loss before the age of 30. There are no approved therapies. It is estimated that there are approximately 11,000 NMAS patients in the US and >20,000 patients in US, Europe, Japan and China.

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 future payments and future benefits under the license agreement, achievement of key milestones under the license agreement and the expected timeline for clinical development and efficacy of ZKN-013 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, or our licensees' 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, and our licensees' preclinical studies and clinical trials and other research and development activities; the competition for patient enrollment from drug candidates in development;; 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 financing 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 September 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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