Eloxx Pharmaceuticals Announces Submission of Investigational New Drug (IND) Application for ZKN-013

March 28, 2023

ZKN-013 in development for treatment of recessive dystrophic epidermolysis bullosa (RDEB) and junctional epidermolysis bullosa (JEB)

Additional IND filing for ZKN-013 for treatment of familial adenomatous polyposis (FAP) expected in first half of 2023

Recent preclinical results have provided additional support for ZKN-013 in RDEB and FAP

WATERTOWN, Mass., March 28, 2023 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (NASDAQ: ELOX), a leader in ribosomal RNA-targeted genetic therapies for rare diseases, today announced the submission of an Investigational New Drug (IND) application with the U.S. Food and Drug Administration for ZKN-013 for the treatment of recessive Dystrophic Epidermolysis Bullosa (RDEB) with nonsense mutations. RDEB is a rare skin disease characterized by mutations in Collagen7 gene.

“This IND application is an important milestone towards providing a treatment option for patients with RDEB and JEB, as there are currently no approved disease-modifying treatments,” said Sumit Aggarwal, President and Chief Executive Officer of Eloxx. “We look forward to advancing ZKN-013, our lead TURBO-ZM™ based molecule, into clinical development, as we believe it has the potential to have a positive impact on the treatment of these two painful and debilitating diseases.”

Recent preclinical results demonstrated read-through activity of ZKN-013 in multiple COL7 genotypes across multiple RDEB patient derived fibroblasts and keratinocytes. Read-through activity resulted in up to an 18-fold increase in full-length COL VII protein levels. Prolonged treatment with ZKN-013 further increased COL VII protein levels. Functionality of the restored full-length COL VII protein was confirmed. These results have been accepted for presentation at an upcoming medical conference.

An additional IND filing for ZKN-013 for the treatment of familial adenomatous polyposis (FAP) is planned in the first half of 2023. FAP, a rare inherited disease with no approved drug therapies, is characterized by proliferation of colon polyps. Eloxx is targeting a subset of patients that have nonsense mutations in the Adenomatous Polyposis Coli (APC) gene that is truncated in these patients.

In January 2023, Eloxx published positive results from a study in the APC^{Min} (multiple intestinal neoplasia) model evaluating the potential of ZKN-013 to treat FAP. The APC^{Min} mouse is a translationally validated model for drug development for FAP. In the APC^{Min} model, treatment with ZKN-013 demonstrated a decrease in intestinal polyps and adenomas, resulting in increased survival. The publication also included *in vitro* and *in vivo* demonstrating that ZKN-013 inhibited the growth of human colon carcinoma cells with APC nonsense mutations, and promoted read through of premature stop codons in the APC gene, leading to functional restoration of full-length APC protein.

About Eloxx Pharmaceuticals

Eloxx Pharmaceuticals, Inc. is engaged in the science of ribosome modulation, leveraging its innovative TURBO-ZMTM 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 September 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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