



Eloxx Pharmaceuticals Announces FDA Clearance to Begin Single Ascending Dose Study of ZKN-013

May 2, 2023

ZKN-013 is in development for potential treatment of recessive dystrophic and junctional epidermolysis bullosa (RDEB and JEB) and Familial Adenomatous Polyposis (FAP)

ZKN-013 is Eloxx's first drug candidate from its TURBO-ZM™ platform to gain clearance for clinical testing

WATERTOWN, Mass., May 02, 2023 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (NASDAQ: ELOX) ("Eloxx" or the "Company"), a leader in ribosomal RNA-targeted genetic therapies for rare diseases, today announced that the U.S. Food and Drug Administration (FDA) has cleared the Company's Investigational New Drug (IND) application to initiate a single ascending dose (SAD) clinical trial in healthy volunteers for ZKN-013 for the potential treatment of recessive Dystrophic Epidermolysis Bullosa (RDEB) with nonsense mutations. RDEB is a rare skin disease characterized by mutations in Collagen7 gene.

"FDA clearance to begin our planned single ascending dose trial is an important milestone towards providing a potential treatment option for patients with RDEB and JEB," said Sumit Aggarwal, President and Chief Executive Officer of Eloxx. "This update is also a momentous event for Eloxx, as ZKN-013, our lead TURBO-ZM™ based molecule, is the first program developed from hit to lead and is now poised to enter clinical development. Beyond ZKN-013, Eloxx has promising TURBO-ZM based discovery programs in cystic fibrosis (fully funded by the Cystic Fibrosis Foundation) and in certain cMYC-overexpressing cancers."

Further SAD and multiple ascending dose (MAD) testing is expected to be conducted following the completion of the planned dose cohorts in the SAD study and discussion with the FDA. The MAD testing could potentially include RDEB patients given the strong benefit/risk in patients cited by the FDA.

ZKN-013 Preclinical Activity in RDEB, JEB and FAP

ZKN-013 has demonstrated robust functional preclinical activity in RDEB and JEB patient cells and in APC^{min} (multiple intestinal neoplasia) mice.

Eloxx also plans to develop ZKN-013 for the treatment of FAP patients with nonsense mutations characterized by proliferation of colon polyps and progression to colon cancer. In January 2023, Eloxx published its preclinical results that showed treatment with ZKN-013 demonstrated a decrease in intestinal polyps and adenomas, resulting in increased survival.

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. 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, statements regarding clinical testing of ZKN-013 for the treatment of RDEB and JEB and the expected timing of trials for our other product candidates and the potential of our product candidates 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 Annual Report on Form 10-K for the fiscal year ended December 31, 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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