Eloxx Pharmaceuticals Announces Participation in the HIT-CF Project on Cystic Fibrosis

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Eloxx is evaluating ELX-02, its lead investigational compound, for the treatment of Cystic Fibrosis in patients with nonsense mutations

WALTHAM, Mass., Feb. 26, 2019 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc., (NASDAQ: ELOX) a clinical-stage biopharmaceutical company dedicated to the discovery and development of novel therapeutics to treat cystic fibrosis, inherited retinal diseases and other diseases caused by nonsense mutations limiting production of functional proteins, today announced it joined the consortium agreement of the European HIT-CF project, a European Union funded preclinical and clinical research program evaluating the efficacy and safety of several disease modifying drug candidates in Cystic Fibrosis (CF) patients with rare genetic mutations. Eloxx's lead investigational drug candidate, ELX-02, a small molecule eukaryotic ribosomal selective glycoside (ERSG), will be evaluated in cystic fibrosis patients with nonsense mutations for whom there are few available treatment options.

The goal of the European HIT-CF project is to investigate whether a positive response to therapies in a patient derived organoid can be predictive of clinical response in a controlled trial. The project represents a new era in CF treatment and personalized medicine, as it has the potential to shift therapeutic trials from patients to the laboratory. The organoid model could be extended to all patients with CF and other rare genetic diseases to identify appropriate therapeutic options. The EU has granted EUR 6.7 million from the Horizon 2020 research program to HIT-CF.

"We are extremely excited to be participating in the HIT-CF research project which has the potential to lead to treatment options for the many CF patients based on their functional responses in the lab," said Dr. Gregory Williams, Eloxx Pharmaceutical's Chief Operating Officer. "and this important project will now include CF patients with rare nonsense mutations, often representing the most severe and underserved phenotypes. We believe the results of this project may extend the use of organoid response data to the regulatory process for drug approval or label expansion as well as for treatment/reimbursement decision making."

About HIT-CF (www.hitcf.org)

HIT-CF is a European research project (H2020 - No 755021) which aims to provide better treatment and better lives for people with CF and rare mutations. The project is coordinated by Prof Dr Kors van der Ent of the University Medical Center Utrecht, The Netherlands. Drug candidates of several companies will first be tested in the laboratory on patient-derived mini-intestines (organoids). Secondly, based on the reaction in the organoids, a smaller group of patients will be assigned to studies (clinical trials) with one of the drug candidates. All participating centers are part of the ECFS-CTN.

Adults with CF and a rare CF genotype reported to the European Cystic Fibrosis Society Patient Registry (ECFSPR), will be approached by their doctors and encouraged by the patient organizations (united in CF-Europe) to participate in the studies within the scope of this project. Firstly, stem cells from the patients' own intestine will be grown into "mini guts" (termed organoids) by the foundation Hubrecht Organoid Technology (HUB) and distributed to laboratories in the Netherlands (Utrecht), Belgium (Leuven) and Portugal (Lisboa). CF drug candidates will be tested in these organoids to check for their efficacy in the tissue of the individual patient. This requires taking a few millimeters of tissue via a tiny biopsy, a painless procedure.

Secondly, based on the effects in organoids, selected patients will be invited to participate in drug trials (organized within one of 43 CF-centers of the ECFS Clinical Trial Network and assisted by Julius Clinical) to evaluate the real life benefit of these compounds for these patients. HIT-CF aims to enable access to the most relevant drugs in development, and each trial group will test a drug candidate from one of the pharmaceutical consortium partners.

In parallel with this project, the pharmaceutical partners will work towards market approval of their drug candidates for the larger group of patients with more common genetic profiles.

The ultimate goal of this project is to develop a path for access to therapies for patient groups or individuals who show positive response to the therapy in an organoid test. One of the major impacts of this project will be the innovative methodologies used to acquire approval (and reimbursement. This will represent a new era in CF treatment as it implements a new type of personalized medicine based on organoids, by shifting therapeutic trials from patients to the laboratory.

About Organoids

Organoids are cell cultures that grow in a culture dish, and look similar to the organ from which they are derived. Intestinal organoids can therefore also be called mini-intestines. To make intestinal organoids for the HIT-CF project, rectal tissue samples (biopsies) will be obtained. This procedure is not painful and takes 5-10 minutes. Because organoids are made from stem cells, they contain the same mutations as the person from whom the biopsies are derived. The drug candidates target the basic defect of CF, and the organoids will be used to test on which mutations the drugs have a positive effect.

About Eloxx Pharmaceuticals

Eloxx Pharmaceuticals, Inc. is a clinical-stage biopharmaceutical company developing novel RNA-modulating drug candidates (designed to be eukaryotic ribosomal selective glycosides) that are formulated to treat rare and ultra-rare premature stop codon diseases. Premature stop codons are point mutations that disrupt protein synthesis from messenger RNA. As a consequence, patients with premature stop codon diseases have reduced or

eliminated protein production from the mutation bearing allele accounting for some of the most severe phenotypes in these genetic diseases. These premature stop codons have been identified in over 1,800 rare and ultra-rare diseases. Read-through therapeutic development is focused on extending mRNA half-life and increasing protein synthesis by enabling the cytoplasmic ribosome to read through premature stop codons to produce full-length proteins. 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 the early stages of clinical development focusing on cystic fibrosis and cystinosis. ELX-02 is an investigational drug that has not been approved by any global regulatory body. Eloxx's preclinical candidate pool consists of a library of novel drug candidates designed to be eukaryotic ribosomal selective glycosides identified based on read-through potential. Eloxx recently announced a new program focused on rare ocular genetic disorders. Eloxx is headquartered in Waltham, MA, with R&D operations in Rehovot, Israel. For more information, please visit www.eloxxpharma.com.

Forward-Looking Statements

This press release contains forward-looking statements, which are generally statements that are not historical facts. Forward-looking statements can be identified by the words "expects," "anticipates," "believes," "intends," "estimates," "plans," "will," "outlook" and similar expressions. Forward-looking statements are based on management's current plans, estimates, assumptions and projections, and speak only as of the date they are made. We undertake no obligation to update any forward-looking statement in light of new information or future events, except as otherwise required by law. Forward-looking statements involve inherent risks and uncertainties, most of which are difficult to predict and are generally beyond our control. Actual results or outcomes may differ materially from those implied by the forward-looking statements as a result of the impact of a number of factors,including: the development of the Company's read-through technology; the approval of the Company's patent applications; the Company's ability to successfully defend its intellectual property or obtain necessary licenses at a cost acceptable to the Company, if at all; the successful implementation of the Company's research and development programs and collaborations; the Company's ability to obtain applicable regulatory approvals for its current and future product candidates; the acceptance by the market of the Company's products should they receive regulatory approval; the timing and success of the Company's preliminary studies, preclinical research, clinical trials, and related regulatory filings; the ability of the Company to consummate additional financings as needed; as well as those discussed in more detail in our Annual Report on Form 10-K and our other reports filed with the Securities and Exchange Commission.

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