



Eloxx Pharmaceuticals Presents Positive New Data for Lead Investigational Drug, ELX-02, at the 42nd European Cystic Fibrosis Society Conference (ECFS)

June 6, 2019

ELX-02 demonstrates dose-responsive pronounced increases in functional CFTR and read-through in organoids, human bronchial epithelial (HBE), and Ussing chamber systems

ELX-02 increases CFTR mRNA to healthy control levels

ELX-02 has shown increased CFTR function in organoids bearing nonsense alleles representing >75% of the cystic fibrosis nonsense genotype population

Eloxx to report top line data from Phase 2 clinical trial in cystic fibrosis in 2019

Phase 2 protocol has been reviewed and approved by the European Cystic Fibrosis Society-Clinical Trial Network (ECFS-CTN) and given a score of "high priority"

WALTHAM, Mass., June 06, 2019 (GLOBE NEWSWIRE) -- **Eloxx Pharmaceuticals, Inc. ("Eloxx")**, (ELOX:Nasdaq), a clinical-stage biopharmaceutical company dedicated to the discovery and development of novel therapeutics to treat cystic fibrosis, cystinosis, inherited retinal disorders, and other diseases caused by nonsense mutations limiting production of functional proteins, today announced positive data demonstrating that ELX-02 increases functional CFTR protein in organoid, human bronchial epithelial, and Ussing chamber systems and restores CFTR mRNA to healthy control levels. These data were presented at the 42nd European Cystic Fibrosis Society Conference on June 5-8, 2019 in Liverpool, UK.

"We are extremely pleased with the emerging profile of ELX-02 and believe that previous studies with already approved drugs for cystic fibrosis demonstrating that functional increases in CFTR protein have been highly correlated with improvements in FEV1, and lung function derisk our Phase 2 studies," said Dr. Matthew Goddeeris, Director of Research, Eloxx Pharmaceuticals. Dr. Gregory Williams stated, "We expect to report top line data from a Phase 2 study in cystic fibrosis patients carrying at least one G542X allele later this year and are committed to developing new potential treatment options for the approximately 13% of patients with Cystic Fibrosis who carry a nonsense mutation on at least one CFTR allele. These patients have a high burden of disease, and few, if any, available treatment options."

In an oral presentation titled: **"ELX-02 increases full length CFTR mRNA through nonsense mediated decay interruption"** presented by Dr. Matthew Goddeeris, Director of Research, Eloxx reported that:

- ELX-02 demonstrates dose-responsive pronounced increases in functional CFTR and read-through in plasmid, HBES, Fisher rat thyroid epithelia, transgenic mice and patient-derived organoids.
- ELX-02 dose-dependently increases CFTR mRNA stability.
- ELX-02 increases CFTR mRNA to healthy control levels.
- ELX-02 increases CFTR function in organoids bearing nonsense alleles representing >75% of the cystic fibrosis nonsense genotype population.

In a second oral presentation titled: **"Administration of ELX-02 to Healthy Volunteers Demonstrates Dose Linearity and Proportionality as well as Low Inter-subject Variability"** presented by Dr. Gregory Williams, Chief Operating Officer, Eloxx reported that:

- ELX-02 has been generally well tolerated in clinical studies to date, supporting future development in cystic fibrosis patients with nonsense genotypes.
- ELX-02 shows high bioavailability (98%) upon subcutaneous administration and highly reproducible pharmacokinetics.
- ELX-02 administered SC shows linear and dose proportional AUC₀₋₁₂ and C_{max} over the dosage studied (0.3 to 7.5 mg/kg).
- Consistent results were observed across single and multiple dose studies, with no accumulation.
- Elimination is primarily renal as parent compound.

In a poster presentation titled: **"ELX-02 Pharmacokinetic profile appropriate for CF patient use"** presented by Dr. Matthew Goddeeris, Director of Research, Eloxx reported that:

- ELX-02 demonstrated pronounced restoration of CFTR activity in organoid, HBE, and Ussing chamber systems.
- Pharmacokinetic nonclinical and clinical results along with physiologically based modeling data support the use of ELX-02 in cystic fibrosis patients with twice weekly or daily dosing.
- Preliminary multiple ascending dose study results are consistent with single ascending dose study results.

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 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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SOURCE: Eloxx Pharmaceuticals, Inc.



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