Eloxx Pharmaceuticals Presents Positive Data for Lead Investigational Drug, ELX-02, at the North American Cystic Fibrosis Conference (NACFC)

October 31, 2019

Eloxx presented findings from the Phase 1 single ascending dose (SAD) and multiple ascending dose (MAD) studies and additional preclinical data for ELX-02

Eloxx will provide an update on the Phase 2 programs for ELX-02 in cystic fibrosis and cystinosis and provide a business update on November 5th, 2019 with the release of third quarter 2019 financial results

Eloxx to host webcast and conference call on November 5, 2019 at 4:30 p.m. ET

WALTHAM, Mass., Oct. 31, 2019 (GLOBE NEWSWIRE) -- **Eloxx Pharmaceuticals, Inc. ("Eloxx"),** (NASDAQ: ELOX), a clinical-stage biopharmaceutical company dedicated to the discovery and development of novel therapeutics to treat cystic fibrosis, cystinosis and other diseases caused by nonsense mutations limiting production of functional proteins, today announced positive data demonstrating that ELX-02 results in a pronounced increase in both CFTR protein expression and mRNA stability further supporting its proposed mechanism of action, and that it was well tolerated with no serious adverse events in the Phase 1 program. These data were presented at the North American Cystic Fibrosis Foundation Conference on October 31-November 2, 2019 in Nashville, Tennessee.

"We are extremely pleased with the emerging profile of ELX-02 and believe that the consistency of our data with those from previous studies with already approved drugs for cystic fibrosis demonstrating that functional increases in CFTR protein across a growing list of *CFTR* genotypes were correlated with improvements in FEV1, and lung function derisk our Phase 2 studies," said Dr. Matthew Goddeeris, Executive Director of Research, Eloxx Pharmaceuticals.

"With the successful completion of our Phase 1 programs, we are pleased to have advanced ELX-02 into Phase 2 clinical trials, and we look forward to providing an update on the programs on November 5th, 2019 during our third quarter 2019 financial results webcast and conference call," said Dr. Gregory Williams, Chief Operating Officer of Pharmaceuticals. "We would like to express our gratitude to the Cystic Fibrosis Foundation for their continued support of our work in cystic fibrosis."

The data presented for ELX-02 at the North American Cystic Fibrosis Conference in Nashville, Tennessee are summarized below.

In a Poster presentation titled: "Pharmacokinetics, Safety, and Tolerability of Multiple Ascending Doses of ELX-02 in Healthy Volunteers, a Potential Treatment for Cystic Fibrosis Caused by Nonsense Mutations" presented Dr. Gregory Williams, COO of Eloxx, reported that:

- ELX-02 shows linear and dose proportional PK following subcutaneous administration twice weekly.
- There were consistent and dose proportional increases in C_{max}, AUC_t, and AUC_{inf} across the dose range on Day 1 and Day 29, with no accumulation.
- Elimination is primarily renal as parent compound and is essentially complete within 24 hours post-dose.
- To date, ELX-02 has been generally well tolerated in clinical studies, with 105 volunteers exposed, no reported SAEs or renal findings.
- Collectively, these data support the future evaluation of ELX-02 in Phase 2 trials with nonsense mediated CF.
- Phase 2 trials will use the 50 mg/mL concentration with daily administration which achieves comparable exposure.

In a Poster presented by Dr. Gregory Williams, COO of Eloxx, titled "Pharmacokinetics, Safety, and Tolerability of Single Ascending Doses of ELX-02 in Healthy Volunteers, a Potential Treatment for Cystic Fibrosis Caused by Nonsense Mutations" Eloxx reported that:

- Single subcutaneously injected doses of ELX-02 between 0.3 mg/kg and 7.5 mg/kg showed an acceptable safety profile without severe or serious drug-related adverse events, including a lack of renal and ototoxicity events.
- ELX-02 shows rapid absorption, high bioavailability (98%) and linear and dose proportional PK following subcutaneous administration.
- Elimination is primarily renal as parent compound.
- To date, ELX-02 has been generally well tolerated in clinical studies, with 105 volunteers exposed, no reported SAEs or renal findings.

- The tolerability of the 7.5 mg/kg single dose supports the safety of the planned exposures and dose range being used in Phase 2.
- Collectively, these data support the future evaluation of ELX-02 in Phase 2 trials with nonsense mediated diseases.

In a Poster presentation titled: “Investigational Drug ELX-02 Mediates CFTR Nonsense Mutation Read-through to Increase CFTR MRNA, CFTR Protein Translation and CFTR Function”, which was also included in an Oral Workshop titled: “New and Emerging Therapies Correcting the Basic Defect”, both presented by Dr. Matthew Goddeeris, Executive Director of Eloxx Pharmaceuticals, Eloxx reported that:

- Pronounced ELX-02 mediated CFTR read-through is demonstrated in FRT, transgenic mice, and patient-derived HBE cells and organoids. Significant and meaningful activity is observed against the top 5 most prevalent nonsense alleles, representing >75% of the CF nonsense population. We continue to identify new responsive genotypes.
- ELX-02 results in a pronounced increase in both CFTR protein expression and mRNA stability further supporting proposed mechanism of action.
- ELX-02 preclinical efficacy-associated exposures translate to the selected Phase 2 clinical trial ascending dose ranges and exposures.

ELX-02 is an investigational agent not approved by any regulatory agency for therapeutic use and is currently in Phase 2 clinical trials for cystic fibrosis and cystinosis.

Conference Call and Webcast Information:

Date: Tuesday, November 5, 2019

Time: 4:30 p.m. ET

Domestic Dial-in Number: (866) 754-6374

International Dial-in Number: (210) 874-7715

Conference ID: 8339658

Live Webcast: accessible from the Company's website at www.eloxxpharma.com under Events and Presentations or with this link: https://edge.media-server.com/mmc/p/ni34pajh

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 designed 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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