Eloxx Pharmaceuticals Presents New Positive Data for ELX-02 at the American Society of Nephrology (ASN) Kidney Week 2019

November 7, 2019

Results of our renal impairment study for ELX-02 support the expansion of our research in the kidney beyond nephropathic cystinosis into other areas such as autosomal dominant polycystic kidney disease

WALTHAM, Mass., Nov. 07, 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, cystinosis, inherited retinal disorders, and other diseases caused by nonsense mutations limiting production of functional proteins, today announced new data from two abstracts presented at the **American Society of Nephrology (ASN) Kidney Week 2019 in Washington, D.C. November 5 -10, 2019.**

"We are pleased to have presented the results of our completed renal impairment study at Kidney Week, which have been important in defining the appropriate doses for patients with renal insufficiency. These data support our current clinical programs as well as the expansion of our research in the kidney beyond nephropathic cystinosis into other areas such as autosomal dominant polycystic kidney disease where there is a high prevalence of nonsense mutation patients," said Dr. Gregory Williams, Chief Operating Officer of Eloxx Pharmaceuticals. "We are advancing our Phase 2 clinical trials for ELX-02 in cystic fibrosis and cystinosis and we look forward to providing an update on our cystic fibrosis clinical trials and reporting additional top line cystinosis data later this quarter."

In a poster session titled **"An open label-single dose, parallel-group study to evaluate the effects of renal impairment on the pharmacokinetics of ELX-02: Results from subjects with mild and moderate renal impairment,"** Dr. Gregory Williams, Chief Operating Officer, Eloxx Pharmaceuticals reported that:

- As degree of renal impairment increased, the exposure to ELX-02 increased and its clearance decreased.
- There were no significant differences in plasma ELX-02 concentrations between the control group and the mildly impaired renal groups. AUC_{0-24} was higher in the moderate and severe groups relative to the control group.
- The observed changes in plasma concentrations enable dose adjustment based on eGFR/renal function.
- Urinary ELX-02 clearance was similar to plasma clearance, with decreased rate in subjects with more severe renal impairment.
- 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 diseases.

In a poster session titled **"Cystinosis nonsense mutation read-through mediated by ELX-02 restores protein function using in vitro and in vivo models,"** Dr. Matthew Goddeeris, Executive Director of Research, Eloxx Pharmaceuticals reported that:

- ELX-02 read-through is sufficient to produce functional CTNS protein and increase *CTNS* mRNA.
- ELX-02 demonstrated that the expressed cystinosin protein reduced the accumulated lysosomal cystine by one third in the time frame of the experiment at the given dose of 10 mg/kg.
- Kidney exposure and demonstration of efficacy *in vivo* support dose-range selection for a Phase 2 clinical trial of ELX-02 in Nephropathic Cystinosis.
- Completion of a Phase 1 study in renal insufficient participants provides modeling necessary for dose adjustments based on renal function.
- These results support the continued development of ELX-02 for the potential treatment of nephropathic cystinosis and other nonsense mutation mediated diseases of the kidney, such as Autosomal Dominant Polycystic Kidney Disease (ADPDK).

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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