Eloxx Pharmaceuticals Presents Positive New Data at the Association for Research in Vision and Ophthalmology “ARVO” 2019 Annual Meeting

May 2, 2019

Eloxx is evaluating several of its eukaryotic ribosomal selective glycoside (ERSG) molecules in IND-enabling studies for use in the treatment of inherited retinal diseases with an initial focus on Usher Syndrome

Preclinical studies have demonstrated:
dose-dependent restoration of missing protein of Usher Syndrome nonsense mutations;
encouraging pharmacokinetics in the retina by intravitreal injection;
a favorable tolerability profile at high doses in sensitive species

Eloxx also made a presentation at the **Sixth Annual Retinal Cell and Gene Therapy Innovation Summit** on April 26, 2019

WALTHAM, Mass., May 02, 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, inherited retinal disorders, and other diseases caused by nonsense mutations limiting production of functional proteins, today presented new findings related to preclinical inherited retinal disease therapeutic development at the **ARVO 2019 Annual Meeting** in Vancouver, BC on May 2, 2019.

“We are very pleased with the preclinical results for several of our ERSG compounds demonstrating that they are suitable for intravitreal injection, well-tolerated in a sensitive model species, active against Usher Syndrome mutations and capable of reaching the retina. We look forward to advancing IND-enabling studies in inherited retinal diseases, with an initial focus on Usher Syndrome,” said Dr. Matthew Goddeeris, Director of Research, Eloxx Pharmaceuticals. Dr. Susan Schneider, Eloxx’s SVP, Clinical Development, Ophthalmology added, “There is a significant unmet medical need and high prevalence of nonsense mutations across inherited retinal diseases, including Usher Syndrome, and we look forward to working closely with key opinion leaders in ophthalmology and the Foundation Fighting Blindness to develop potential treatments for these patients.”

In a Poster presentation at the **ARVO Annual Meeting** on May 2, 2019 titled: “**Instituting a read-through therapeutic approach to nonsense-mutation based inherited retinal disorders, ELX-03, a translational nonsense mutation read-through agent demonstrates tolerability and activity for use in inherited retinal disorders**”, presented by Dr. Matthew Goddeeris, Director of Research, at Eloxx, it was reported that:

- Eloxx has screened multiple compounds from its ERSG library of read-through agents for potential use in the treatment of inherited retinal disorders with an initial focus on Usher Syndrome, beginning with USH2A.
- Multiple Eloxx compounds in preclinical studies have demonstrated:
  - Dose-dependent activity against Usher mutations.
  - Restoration of missing Usher Syndrome protein.
  - Favorable tolerability profile at high doses in sensitive species; preserved electroretinogram (ERG) and no compound-related histopathological changes.
  - Encouraging pharmacokinetics demonstrating retina exposure by intravitreal injection.
- IND-enabling studies ongoing.
- Next steps include evaluation of patient-derived cell models and sustained release formulations.

At the request of the Foundation Fighting Blindness, Dr. Matthew Goddeeris, also made a presentation at the **Sixth Annual Retinal Cell and Gene Therapy Innovation Summit** in Vancouver, BC., on April 26, 2019, to review Eloxx Pharmaceuticals’ read-through therapeutic approach to inherited retinal disorders with its library of ERSG molecules.

Eloxx compounds are investigational agents not approved by any regulatory agency for therapeutic use.

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 moving to Phase 2 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 is also advancing 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.

Contact:
Barbara Ryan
Barbarar@eloxxpharma.com
(203) 274-2825

SOURCE: Eloxx Pharmaceuticals, Inc.

Source: Eloxx Pharmaceuticals