# Eloxx Pharmaceuticals Reports Additional Confirmation that All Nonsense Mutation Alport Syndrome Patients Treated with ELX-02 in Phase 2 Study had Improvement in Kidney Morphology and Clinical Benefit of Reduction or Stabilization of Proteinuria

October 9, 2023

*ELX-02 treatment improved podocyte foot process effacement in all three patients by an average of 60% based on a blinded kidney biopsy analysis by NIPOKA GmbH*

*Biopsy results support clinical benefit in all three patients as improvement of kidney morphology is consistent with reduction or stabilization of proteinuria during or up to 2 months post completion of dosing*

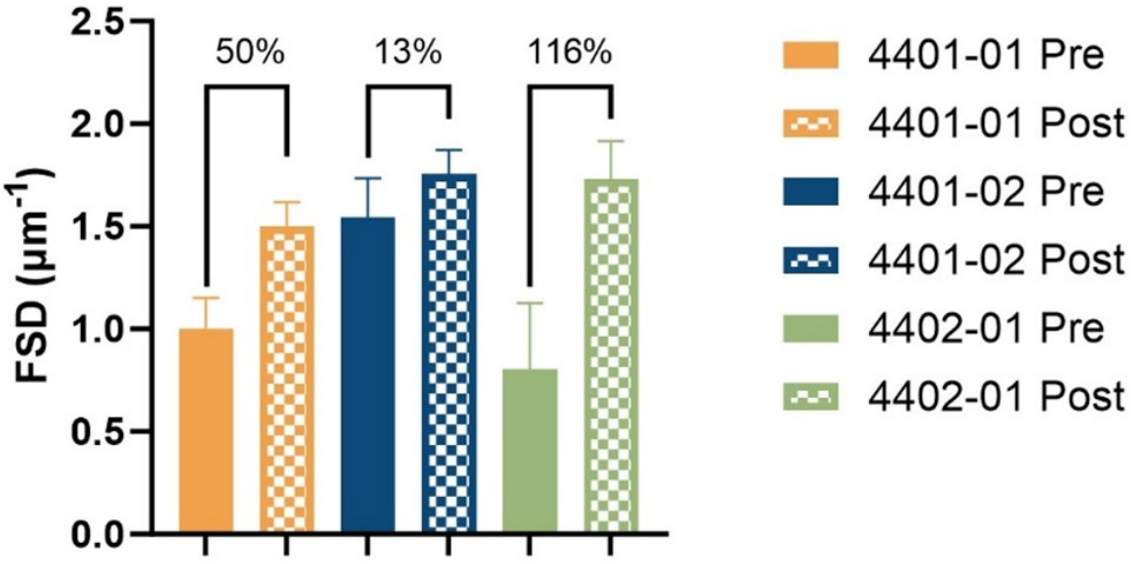
*Renowned key opinion leaders recommend continued development following review of clinical and biopsy results*

WATERTOWN, Mass., Oct. 09, 2023 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (NASDAQ: ELOX), a leader in ribosomal RNA-targeted genetic therapies for rare diseases, today reported results from an assessment of patient biopsies by NIPOKA GmbH (Nipoka). They have developed a highly accurate method for the quantification of podocyte foot process morphology. These results confirm previously reported positive biopsy results from the proof-of-concept Phase 2 open-label clinical trial (NCT05448755) of ELX-02 for the treatment of Nonsense Mutation Alport syndrome patients. Analysis of formalin-fixed paraffin-embedded (FFPE) biopsy samples by Nipoka show ELX-02 treatment improved podocyte foot process morphology with lower effacement in all three patients at the end of the 8-week study period.

“With this accurate analysis of the patient biopsies and quantification of changes, we now have unequivocal evidence of morphology and clinical improvement in all three Nonsense Mutation Alport patients treated with ELX-02. Improvement in kidney morphology drives clinical benefit in this devastating rare disease,” said Sumit Aggarwal, President and Chief Executive Officer of Eloxx. “We believe that our proteinuria data, during and after treatment, in the context of this improvement in kidney morphology, confirms clinical benefit in all three patients.”

NIPOKA GmbH have developed a *Podocyte Exact Morphology Procedure (*PEMP*)* to quantify podocyte foot process morphology accurately and precisely in an unbiased and reproducible manner. PEMP utilizes immunostaining for foot-process specific protein markers followed by 3D-SIM imaging to quantify Filtration Slit Density (FSD) for 15 to 20 glomeruli per sample. FSD is a quantitative measure of the degree of podocyte foot process effacement. Higher FSD correlates with better podocyte health and lower podocyte foot process effacement. Healthy patients have an FSD of approximately 3.0 to 4.0. This analysis has been validated in multiple glomerular diseases.

PEMP analysis confirmed that ELX-02 treatment improved podocyte foot process effacement in all three patients with an average post-treatment increase in FSD of 60% as compared to baseline levels. These findings are also consistent with previous Transmission Electron Micrograph (TEM) image assessments.




Differences in Urine Protein-Creatinine ratio (UPCR) changes across patients during treatment were correlated to severity of disease (lower vs. higher

FSD) at baseline. Therefore, improvement in UPCR was assessed both during and 2 months after treatment to evaluate clinical benefit and capture the full effect of the 45-day protein half-life.

| Patient | FSD at end of treatment (% change vs. baseline) | Average change in UPCR during treatment vs. baseline | Average change in UPCR 2 months after end of treatment vs baseline | UPCR variability change vs baseline (Standard deviation 2 months after end of treatment vs baseline) |
|---|---|---|---|---|
| **4401 – 01** | **1.50 (50%)** | No change | No Change | **-32%** |
| **4401 – 02** | **1.75 (13%)** | **-49%;** | No Change | **-46%** |
| **4402 – 01** | **1.73 (118%)** | No change | **-25%** | **-68%** |

As shown in the table above, all patients had proteinuria stabilization (lower variability vs. baseline) or improvement (reduction during or 2-months after treatment). This is consistent with clinical benefit and with the improvement in kidney morphology.

Renowned key opinion leaders have reviewed these data and overwhelmingly believe that they provide strong evidence of the potential of the disease modifying effect of ELX-02 and warrant advancement into a pivotal trial.

**About Nonsense Mutation Alport Syndrome**

Nonsense Mutation Alport syndrome is a rare Type IV Collagenopathy characterized by mutations in the genes (COL4A3, COL4A4, and COL4A5) that result in a less than full length (truncated) Type 4 Collagen. This disorder mostly affects children with a median age at diagnosis of 9 to 20 years. It is characterized by rapid and progressive damage to the kidneys, ear and eyes, starting with worsening of kidney morphology to proteinuria and finally kidney failure, hearing loss and eye abnormalities. It is estimated that there are approximately 7,500 patients in the US and 20,000 patients in US, Europe, Japan and China with Nonsense Mutation Alport Syndrome. These patients have no approved treatment options.

**About Eloxx Pharmaceuticals**

Eloxx Pharmaceuticals, Inc. is engaged in the science of ribosome modulation, leveraging its innovative TURBO-ZM™ chemistry technology platform in an effort to develop novel Ribosome Modulating Agents (RMAs) and its library of Eukaryotic Ribosome Selective Glycosides (ERSGs). 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 Phase 2 clinical development for the treatment of Alport syndrome in patients with nonsense mutations. For more information, please visit www.eloxxpharma.com.

**Forward-looking Statements**

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements other than statements of historical facts contained in this press release, including without limitation, statements regarding the potential of our product candidate to treat Alport syndrome, the Company's intentions to advance ELX-02 into a global pivotal confirmatory trial for the potential treatment of patients with Alport Syndrome, to advance its clinical study of ZKN-013 and the exploration of strategic alternatives with potential development partners are forward-looking statements. Forward-looking statements can be identified by the words "aim," "may," "will," "would," "should," "expect," "explore," "plan," "anticipate," "could," "intend," "target," "project," "contemplate," "believe," "estimate," "predict," "potential," "seeks," or "continue" or the negative of these terms similar expressions, although not all forward-looking statements contain these words. Forward-looking statements are based on management's current plans, estimates, assumptions and projections based on information currently available to us. Forward-looking statements are subject to known and unknown risks, uncertainties and assumptions, and actual results or outcomes may differ materially from those expressed or implied in the forward-looking statements due to various important factors, including, but not limited to: our ability to progress any product candidates in preclinical or clinical trials; the uncertainty of clinical trial results and the fact that positive results from preclinical studies are not always indicative of positive clinical results; the scope, rate and progress of our preclinical studies and clinical trials and other research and development activities; the competition for patient enrollment from drug candidates in development; the impact of the global COVID-19 pandemic on our clinical trials, operations, vendors, suppliers, and employees; our ability to obtain the capital necessary to fund our operations; the cost of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights; our ability to obtain financial in the future through product licensing, public or private equity or debt financing or otherwise; our ability to meet the continued listing requirements of, and remain listed on, the Nasdaq Capital Market; general business conditions, regulatory environment, competition and market for our products; and business ability and judgment of personnel, and the availability of qualified personnel and other important factors discussed under the caption "Risk Factors" in our Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2023, as any such factors may be updated from time to time in our other filings with the SEC, accessible on the SEC's website at www.sec.gov and the "Financials & Filings" page of our website at https://investors.eloxxpharma.com/financials-filings.

All forward-looking statements speak only as of the date of this press release and, except as required by applicable law, we have no obligation to update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

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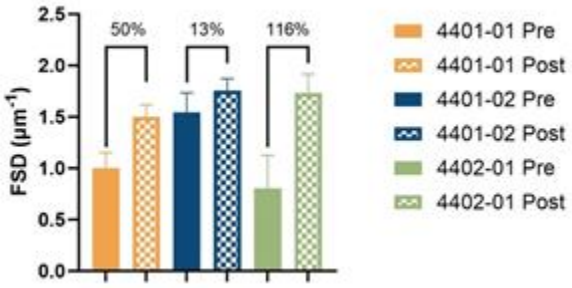
SOURCE: Eloxx Pharmaceuticals, Inc.

A photo accompanying this announcement is available at https://www.globenewswire.com/NewsRoom/AttachmentNg/5e112bd4-b39a-4356-8e29-2ceb97ffbac4

Source: Eloxx Pharmaceuticals

**Podocyte Exact Morphology Procedure Results**




**Podocyte Exact Morphology Procedure Results**