Eledon Announces Positive Topline Results from Phase 2a Trial of Tegoprubart Demonstrating Safety, Target Engagement, and Biomarker Response in Patients Living with Amyotrophic Lateral Sclerosis

May 31, 2022

- *Tegoprubart was well-tolerated, with no drug-related serious adverse events*
- *Dose dependent target engagement was demonstrated, and ALS associated pro-inflammatory biomarkers were both observed and significantly reduced in a dose dependent manner*
- *Target engagement and level of pro-inflammatory biomarker reduction were associated with a trend in the slowing of disease progression as measured by ALSFRS slope when compared to a cohort from the ALS PRO-ACT database*
- *Biomarkers significantly reduced included biomarkers also associated with IgA nephropathy and kidney allograft transplant rejection*
- *Eledon management will host a webcast and conference call regarding these clinical results at 8:00 a.m. ET today, May 31, 2022*

IRVINE, Calif., May 31, 2022 (GLOBE NEWSWIRE) -- Eledon Pharmaceuticals, Inc. (“Eledon”) (Nasdaq: ELDN), a patient-focused clinical stage biopharmaceutical company committed to the development of innovative and impactful treatments for organ and cell transplantation, autoimmune conditions, and neurodegenerative disease, today announced topline results from a Phase 2a clinical trial of tegoprubart (formerly AT-1501) in patients with amyotrophic lateral sclerosis (ALS).[1]

Tegoprubart is an investigational humanized monoclonal antibody that inhibits CD40 Ligand (CD40L), a membrane protein linked to increased peripheral immune responses and neuroinflammation in ALS. The 12-week trial included 54 patients with ALS at 13 treatment sites in the United States and Canada. The primary objectives of the study were to assess the safety and tolerability of multiple doses of tegoprubart in four sequential, ascending dose cohorts (1, 2, 4, and 8 mg/kg). Secondary outcome measures included pharmacokinetic assessment of multiple intravenous doses of tegoprubart on target engagement and on pro-inflammatory biomarkers associated with ALS. Each subject served as their own control, with changes being compared to baseline.

Tegoprubart successfully met the primary endpoints of safety and tolerability. Adverse events were equally distributed across dose levels. Tegoprubart was well-tolerated, and no drug-related serious adverse events were observed. Anti-drug antibodies (ADAs) were present in less than 5 percent of samples. All ADAs were of low titer and did not impact tegoprubart drug levels.

Tegoprubart target engagement was demonstrated at the 4 and 8 mg / kg dosing levels using CD40L and CXCL13 biomarkers related to T cell and B cell function, respectively. A pro-inflammatory ALS signature was identified, consisting of 32 different inflammatory biomarkers in the tested population, including TNF-α, MCP1, EN-RAGE, C-Reactive Protein (CRP), and IL-6. IL-1 was not significantly detected in the study patient population. Dose dependent, significant reductions were observed in up to 23 of these biomarkers, including TNF-α, MCP1, EN-RAGE, and CRP. Other pro-inflammatory biomarkers significantly reduced included biomarkers also associated with IgA nephropathy and kidney transplant rejection, such as IgA, IgE, IgM, C3, CXCL9, and CXCL10. While the study was neither primarily designed nor powered to assess the effect of tegoprubart on ALS Functional Rating Scale (“ALSFRS”), both target engagement and level of pro-inflammatory biomarker reduction were associated with a trend in the slowing of disease progression as measured by ALSFRS slope when compared to a cohort from the ALS PRO-ACT database[2].

“Neuroinflammation is a driving force in the pathogenesis and progression of ALS. The ability to suppress inflammatory responses may translate into clinical benefit,” said Stanley H. Appel, MD, Co-Director of the Houston Methodist Neurological Institute and Chair of the Stanley H. Appel Department of Neurology at Houston Methodist. “These results reinforce the exciting potential of tegoprubart as a promising therapy for patients with ALS.”

“These positive Phase 2a topline trial results demonstrated target engagement and a reduction in key inflammatory biomarkers in patients living with ALS,” said Merit Cudkowicz, MD, Chief of the Neurology Department at Massachusetts General Hospital; Director of the Sean M. Healey & AMG Center for ALS; and Julieanne Dorn Professor of Neurology at Harvard Medical School. “A key goal for Phase 2 trials is confirming that a drug hits the intended targets. These encouraging data support advancing tegoprubart into larger clinical ALS studies.”

“There would be no tegoprubart without the resolve of so many ALS patients and their families who supported the early drug discovery efforts for tegoprubart, and who continue to champion the scientific advancement of ALS research,” said David-Alexandre C. Gros, MD, Chief Executive Officer of Eledon. “Our new data are helping elucidate the role of inflammation in ALS and tegoprubart. In addition to ALS, tegoprubart is in clinical-stage development for IgA nephropathy, renal transplantation, and islet cell transplantation. We look forward to presenting and reporting additional data from this and from our other clinical trials later this year.”

Conference Call and Webcast Details

Eledon management will host a webcast and conference call regarding these clinical results at 8:00 a.m. ET today, May 31, 2022. The live call may be accessed by dialing (877) 407-9039 for domestic callers and +1 (201) 689-8470 for international callers with conference ID code number: 13730346. A live webcast of the call will be available online in the investor relations section of Eledon’s website at www.eledon.com or by registering here, and will be archived there for one year.

About ALS

ALS, commonly known as Lou Gehrig's disease, is a progressive neuromuscular disease, and there is a critical need for more effective therapies to improve outcomes for patients. Currently, there are two drugs approved by the U.S. Food and Drug Administration (FDA) for ALS; however, the average life expectancy remains only three to five years. In the United States, ALS affects approximately 30,000 persons, and approximately 5,000

new cases are diagnosed each year.[3]

About the Phase 2a Study of Tegoprubart

This Phase 2a, multi-center, open label, multiple dose study of tegoprubart enrolled 54 adults with ALS at treatment sites in the United States and Canada. Four ascending doses of tegoprubart were administered as an IV infusion to sequentially enrolling cohorts of up to 18 subjects. Each participant received six bi-weekly (every other week) infusions of tegoprubart and served as their own control by comparing changes to baseline. Outcome measures included safety and tolerability, assessment of biomarkers of CD40L target engagement, and changes in pro-inflammatory chemokines and cytokines upregulated in ALS.

About Eledon Pharmaceuticals and tegoprubart (formerly AT-1501)

Eledon Pharmaceuticals is a clinical stage biotechnology company using its expertise in targeting the CD40 Ligand (CD40L, also called CD154) pathway to develop potential treatments for persons requiring an organ or cell-based transplant, living with autoimmune disease, or living with ALS. The company's lead compound in development is tegoprubart, an anti-CD40L antibody with high affinity for CD40 Ligand, a well-validated biological target with broad therapeutic potential. Eledon is headquartered in Irvine, Calif. For more information, please visit the company's website at www.eledon.com.

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Forward-Looking Statements

This press release contains forward-looking statements that involve substantial risks and uncertainties. Any statements about the company's pre-clinical and clinical trial results, data releases, future expectations, plans and prospects, including statements about planned clinical trials, the tolerability, safety profile, development and potential of product candidates, expected timing for initiation of future clinical trials, and the expected timing for receipt of data from clinical trials, as well as other statements containing the words "believes," "anticipates," "plans," "expects," "estimates," "intends," "predicts," "projects," "targets," "looks forward," "could," "may," and similar expressions, constitute forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements are inherently uncertain and are subject to numerous risks and uncertainties, including: risks relating to the safety and efficacy of our drug candidates; risks relating to clinical development timelines, including interactions with regulators and clinical sites, as well as patient enrollment; risks relating to costs of clinical trials and the sufficiency of the company's capital resources to fund planned clinical trials; and risks associated with the impact of the ongoing coronavirus pandemic. Actual results may differ materially from those indicated by such forward-looking statements as a result of various factors. These risks and uncertainties, as well as other risks and uncertainties that could cause the company's actual results to differ significantly from the forward-looking statements contained herein, are discussed in our quarterly 10-Q, annual 10-K, and other filings with the U.S. Securities and Exchange Commission, which can be found at www.sec.gov.

In addition, the results of nonclinical studies and early clinical trials of the company's product candidates may not be predictive of the results of later-stage clinical trials. Interim results of a clinical trial do not necessarily predict final results. Moreover, nonclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in nonclinical and clinical trials have nonetheless failed to obtain marketing approval of their products. There is a risk that additional nonclinical and/or clinical safety studies will be required by the FDA or similar regulatory authorities outside the United States and/or that subsequent studies will not match results seen in prior studies. As a result, topline data should be viewed with caution until the final data are available.

Any forward-looking statements contained in this press release speak only as of the date hereof and not of any future date, and the company expressly disclaims any intent to update any forward-looking statements, whether as a result of new information, future events or otherwise.

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Source: Eledon Pharmaceuticals

[1] ClinicalTrials.gov Identifier: NCT04322149. https://clinicaltrials.gov/ct2/show/NCT04322149?term=at-1501&draw=2&rank=4

[2] PRO-ACT (Pooled Resource Open-access ALS Clinical Trials) Database

[3] *10 Facts About ALS*. https://www.research.va.gov/programs/tissue_banking/als_caregivers/ALSFactSheet_NAR.pdf

Source: Eledon Pharmaceuticals, Inc.