Aileron Therapeutics to Present Previously Announced Data from the Phase 1b Clinical Trial Evaluating Low-Dose LTI-03 in Idiopathic Pulmonary Fibrosis (IPF) at the 22nd International Colloquium on Lung and Airway Fibrosis

October 12, 2024

First scientific presentation of previously announced data from Cohort 1 of the ongoing Phase 1b clinical trial evaluating low-dose LTI-03 (2.5 mg BID) in IPF, affirms positive trends in seven of the eight biomarkers evaluated, suggesting potential therapeutic effect

Recently completed enrollment of Cohort 2 evaluating high-dose LTI-03 (5 mg BID) in mid-September; topline data expected in the near-term

AUSTIN, Texas, Oct. 12, 2024 /PRNewswire/ -- Aileron Therapeutics, Inc. ("Aileron") (NASDAQ: ALRN), a biopharmaceutical company advancing a novel pipeline of first-in-class medicines to address significant unmet medical needs in orphan pulmonary and fibrosis indications, today announced the presentation of two abstracts detailing LTI-03's pre-clinical and Phase 1b (NCT05954988) results in Idiopathic Pulmonary Fibrosis (IPF) at the 22nd International Colloquium on Lung and Airway Fibrosis (ICLAF).

The Company previously **announced positive data** from Cohort 1 of the ongoing Phase 1b clinical trial evaluating low-dose LTI-03 (2.5 mg BID) in patients with IPF. Following inhaled administration of low-dose LTI-03 in 12 patients over the course of 14 days, a positive trend was observed in seven out of eight biomarkers with evidence of reduced expression among multiple profibrotic proteins produced by basal-like cells and fibroblasts that contribute to the progression of IPF, including data from three biomarkers (collagen synthesis, inflammation, and fibrogenesis) that was statistically significant, reinforcing the potential of LTI-03 to improve lung function and reverse the course of IPF. The [poster][abstracts] being presented at ICLAF will summarize the previously disclosed data from Cohort 1.

Pre-clinical data presented at ICLAF further supports the potential therapeutic effectiveness of LTI-03 for IPF through precision cut lung slices (PCLS) performed *ex-vivo.* Pre-clinical studies demonstrated molecular activity in IPF PCLS explants indicative of fibrosis during five days in culture and LTI-03 broadly attenuated pro-fibrotic proteins and pathways.

Additionally, the Company recently announced completion of enrollment in Cohort 2 of the ongoing Phase 1b clinical trial evaluating high-dose LTI-03 (5 mg BID) in 12 patients with IPF. In the trial, eligible patients (n=24) are randomly assigned (3:1) to receive either inhaled LTI-03 or placebo. The primary objective of the trial is to evaluate the safety and tolerability of LTI-03 in patients with IPF after treatment for 14 consecutive days, with measurement of multiple protein biomarkers as exploratory endpoints. The Company expects to report topline data for this cohort in the near-term.

Details of the [poster] presentations are as follows:

Presentation Title: Anti-Fibrotic Activity of Caveolin-1 scaffolding domain Peptide LTI-03 in *Ex Vivo* Precision Cut Lung Slices from Patients with Idiopathic Pulmonary Fibrosis
Abstract #: 0186
Presenter: Professor Cory M. Hogaboam, Cedars Sinai Medical Center, Los Angeles, CA
Date & Time: Tuesday, October 15, 2024 at 3:30 pm EEST/ 8:30 am ET

Presentation Title: Inhalation of LTI-03 Modulates Multiple Targets in a Phase 1B Placebo Controlled Clinical Trial for IPF
Abstract #: 0183
Presenter: Professor Cory M. Hogaboam, Cedars Sinai Medical Center, Los Angeles, CA
Date & Time: Tuesday, October 15, 2024 at 3:30 pm EEST/ 8:30 am ET

About the Phase 1 Clinical Trial of LTI-03

The Phase 1b clinical trial of LTI-03 is a randomized, double-blind, placebo controlled, multi-center, dose escalation trial in patients recently diagnosed with IPF that have not received prior treatment with anti-fibrotic agents for at least two months (**NCT05954988**). Eligible patients are randomly assigned (3:1) to receive one of two doses of inhaled LTI-03 or placebo. The primary objective of the trial is to investigate the safety and tolerability of LTI-03 in patients with IPF after treatment for 14 consecutive days, with measurement of multiple protein biomarkers as exploratory endpoints.

About IPF

IPF is a chronic lung disease characterized by progressive tissue scarring that prevents proper lung function. It is a progressive, fatal, age-associated lung disease affecting approximately 100,000 people in the United States[1]. IPF typically presents in adults 65 or older and is usually fatal within two to five years after diagnosis[2].

About LTI-03 and Caveolin-1 (Cav1)

LTI-03 is a seven amino acid peptide, the sequence of which is derived from the caveolin scaffolding domain (CSD), an important binding region of the Cav1 protein. Cav1 normally serves a critical function in the prevention of fibrosis by maintaining a balance between pathways that both initiate and arrest lung repair and cell movement. Through the CSD, caveolin interacts with a large number of signaling molecules, all of which possess a caveolin binding domain region. Cav1 expression is decreased in IPF lung tissues and has been demonstrated to decrease during the fibrotic phase of bleomycin lung injury in mice. Restoring the balance of important biological signals in the lung may not only slow lung function decline but could also restore healthy lung function through the protection of healthy epithelial cells.

About Aileron Therapeutics

Aileron Therapeutics is a biopharmaceutical company advancing a novel pipeline of first-in-class medicines to address significant unmet medical needs in orphan pulmonary and fibrosis indications. Aileron's lead product candidate, LTI-03, is a novel, synthetic peptide with a dual mechanism targeting alveolar epithelial cell survival as well as inhibition of profibrotic signaling. Currently, LTI-03 is being evaluated in a Phase 1b clinical trial for the treatment of idiopathic pulmonary fibrosis. Aileron's second product candidate, LTI-01, is a proenzyme that has completed Phase 1b and Phase 2a clinical trials for the treatment of loculated pleural effusions. LTI-01 has received Orphan Drug Designation in the US and EU and Fast Track Designation in the US.

References

[1] Pergolizzi, Jr., J., LeQuang, J., Varrassi, M., Breve, F., Magnusson, P., Varrassi, G., (2023). What Do We Need to Know About Rising Rates of Idiopathic Pulmonary Fibrosis? A Narrative Review and Update. Springer Nature, Published online 2023 Jan 24. Doi: 10.1007/s12325-022-02395-9.
[2] Nathan et al. "Long-term Course and Prognosis of Idiopathic Pulmonary Fibrosis in the New Millennium". Chest Journal Volume 140, ISSUE 1, P221-229, July 2011.

Forward-Looking Statements

This press release may contain forward-looking statements of Aileron Therapeutics, Inc. ("Aileron", the "Company", "we", "our" or "us") within the meaning of the Private Securities Litigation Reform Act of 1995, including statements with respect to: the timing and expectation of the topline results of Cohort 2 of the Phase 1b clinical trial of LTI-03; future expectations, plans and prospects for the Company, the sufficiency of the Company's cash resources; the status and plans for clinical trials, including the timing of data; future product development; and the potential commercial opportunity of LTI-03 and LTI-01. We use words such as "anticipate," "believe," "estimate," "expect," "hope," "intend," "may," "plan," "predict," "project," "target," "potential," "would," "can," "could," "should," "continue," and other words and terms of similar meaning to help identify forward-looking statements, although not all forward-looking statements contain these identifying words. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including risks and uncertainties related to, changes in applicable laws or regulations, the possibility that the Company may be adversely affected by other economic, business, and/or competitive factors, including risks inherent in pharmaceutical research and development, such as: adverse results in the Company's drug discovery, preclinical and clinical development activities, the risk that the results of preclinical studies and early clinical trials may not be replicated in later clinical trials or that partial results of a trial such as the Cohort 1 results from the Company's ongoing Phase 1b clinical trial will be indicative of the full results of the trial, the Company's ability to enroll patients in

its clinical trials, and the risk that any of its clinical trials may not commence, continue or be completed on time, or at all; decisions made by the U.S. Food and Drug Administration and other regulatory authorities, investigational review boards at clinical trial sites and publication review bodies with respect to our development candidates; our ability to obtain, maintain and enforce intellectual property rights for our platform and development candidates; competition; uncertainties as to the sufficiency of the Company's cash resources to fund its planned activities for the periods anticipated and the Company's ability to manage unplanned cash requirements; and general economic and market conditions; as well as the risks and uncertainties discussed in the "Risk Factors" section of the Company's Annual Report on Form 10-K for the year ended December 31, 2023 and Quarterly Report on Form 10-Q for the quarter ended June 30, 2024, which are on file with the United States Securities and Exchange Commission (the "SEC"), and in subsequent filings that the Company makes with the SEC. These forward-looking statements should not be relied upon as representing the Company's view as of any date subsequent to the date of this press release, and we expressly disclaim any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

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