Aileron Therapeutics Announces Enrollment Expansion for Upcoming Phase 1b Clinical Trial of ALRN-6924 in Patients with Advanced Non-Small Cell Lung Cancer (NSCLC)

March 2, 2021

-- Placebo-controlled trial anticipated to enroll 60 patients with advanced p53-mutated NSCLC undergoing treatment with first-line carboplatin plus pemetrexed --

-- Trial anticipated to begin in second quarter 2021 with initial data anticipated at the end of 2021 and final results mid-2022 --

-- Regulatory pathway now clarified for chemoprotection with recent U.S. FDA approval of first-generation chemoprotective drug for patients with extensive-stage small cell lung cancer[1] --

-- ALRN-6924 is the first and only reported chemoprotective agent in clinical development to use a biomarker to ensure selective chemoprotection of healthy cells without protecting cancer cells --

WATERTOWN, Mass., March 02, 2021 (GLOBE NEWSWIRE) -- Aileron Therapeutics, Inc. (Nasdaq: ALRN) today announced that it has expanded the enrollment target for its upcoming Phase 1b clinical trial of ALRN-6924 in patients with non-small cell lung cancer (NSCLC) undergoing chemotherapy. Aileron plans to enroll 60 patients, increased from the original target of 40 patients, with advanced p53-mutated NSCLC undergoing treatment with first-line carboplatin plus pemetrexed (with or without immune checkpoint inhibitors). The company anticipates beginning to enroll patients in the trial in the second quarter of 2021. Aileron is developing ALRN-6924 to selectively protect healthy cells in patients with cancers that harbor p53 mutations to reduce or eliminate chemotherapy-induced side effects while preserving chemotherapy's attack on cancer cells, a novel concept known as chemoprotection.

"Last month, the field of chemoprotection took a giant step forward with the approval of the industry's first chemoprotection agent, which is approved to decrease the incidence of chemotherapy-induced myelosuppression in patients with extensive small-cell lung cancer[1]. With this milestone, the regulatory path for chemoprotective agents like ALRN-6924 has now been clarified. ALRN-6924 is the first and only reported chemoprotective agent in clinical development to employ a biomarker strategy, in which we exclusively focus on treating patients with p53-mutated cancers. This strategy is designed to selectively protect healthy cells in the body from chemotherapy while ensuring we do not protect cancer cells. As a result, healthy cells are spared from chemotherapeutic destruction while chemotherapy continues to kill cancer cells. Given the high prevalence of p53-mutated cancers, we believe ALRN-6924 could potentially benefit millions of patients worldwide," said Manuel Aivado, M.D., Ph.D., President and CEO of Aileron.

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[1] On February 12, 2021, the U.S. Food & Drug Administration approved G1 Therapeutics, Inc.'s COSELA™ (trilaciclib). COSELA is intended to help protect bone marrow in patients with extensive-stage small cell lung cancer when administered prior to chemotherapy.

Dr. Aivado continued, "Our recent equity fundraises in January of this year enabled us to enrich our ALRN-6924 clinical development planning, including a 50% increase in the planned enrollment target for our upcoming Phase 1b clinical trial of ALRN-6924 in patients with advanced p53-mutated NSCLC. We believe this enrollment expansion will enable a more robust exploration of ALRN-6924 as a novel chemoprotective agent to prevent toxicities in the NSCLC patient population and potentially better positions us to more rapidly reach late-stage clinical development."

In the planned Phase 1b NSCLC trial, patients will be randomized 1:1 to receive either carboplatin/pemetrexed plus 0.3 mg/kg ALRN-6924 or carboplatin/pemetrexed plus placebo for at least four 21-day treatment cycles. Evaluations will include the proportion of treatment cycles free of severe hematological and other toxicities, transfusions and the use of growth factors, as well as the impact on quality of life. Aileron anticipates initial data from the trial late in the fourth quarter of 2021 and full results mid-2022.

The planned Phase 1b NSCLC trial follows Aileron's presentation in October 2020 of clinical data from its ongoing Phase 1b clinical trial of ALRN-6924 in small cell lung cancer (SCLC) demonstrating clinical proof-of-concept that treatment with ALRN-6924 resulted in a protective effect against severe anemia, thrombocytopenia and neutropenia in patients with p53-mutated SCLC treated with topotecan.

About ALRN-6924

Aileron is developing ALRN-6924, a novel chemoprotective medicine, to selectively protect healthy cells in patients with cancers that harbor p53 mutations to reduce or eliminate chemotherapy-induced side effects.

Chemotherapy preferentially acts on cells that are cycling, i.e. undergoing the process of cell division. In cancer cells, the cell cycle is unchecked, which leads to uncontrolled cell proliferation, a hallmark of cancer. Certain types of healthy cells also naturally need to cycle, such as bone marrow cells, hair follicle cells, skin cells, and cells lining the oral cavity and the gastrointestinal tract. As a result, chemotherapy preferentially targets and kills both cycling healthy cells and cycling cancer cells. This, in turn, can lead to a spectrum of chemotherapy-induced side effects, from unpleasant to life-threatening and fatal.

ALRN-6924, an investigational first-in-class MDM2/MDMX dual inhibitor, is administered prior to chemotherapy to patients with p53-mutant cancers. ALRN-6924 is designed to activate normal p53 protein in patients' healthy cells, temporarily and reversibly pausing cell cycling to selectively shield the patients' healthy cells from chemotherapy. The protection is limited to healthy cells, as ALRN-6924 cannot work in p53-mutated cancer cells given that mutated p53 has lost its function in those cells. Therefore, p53-mutated cancer cells continue to cycle uninterrupted and remain fully susceptible to being killed by chemotherapy.

About Aileron Therapeutics

Aileron is a clinical-stage biopharmaceutical company that is focused on enabling patients to fight cancer without the fear or burden of chemotherapy-induced side effects. We believe selective chemoprotection has the potential to fundamentally transform chemotherapy like anesthesia transformed surgery.

ALRN-6924, our first-in-class MDM2/MDMX dual inhibitor activating p53, is the only reported chemoprotective agent in clinical development to employ a biomarker strategy, in which we exclusively focus on treating patients with p53-mutated cancers. Our targeted strategy is designed to selectively protect multiple healthy cell types throughout the body from chemotherapy while ensuring we do not protect cancer cells. As a result, healthy cells are spared from chemotherapeutic destruction while chemotherapy continues to kill cancer cells. In addition to reducing or eliminating multiple chemotherapy-induced side effects, ALRN-6924 may also improve patients' quality of life and help them better tolerate chemotherapy. Enhanced tolerability may result in fewer dose reductions or delays of chemotherapy and the potential for improved efficacy.

Our vision is to bring chemoprotection to patients with p53-mutated cancers, which represent approximately 50% of cancer patients, regardless of type of cancer or chemotherapy. Visit us at aileronrx.com to learn more.

Forward-Looking Statements

Statements in this press release about Aileron's future expectations, plans and prospects, as well as any other statements regarding matters that are not historical facts, may constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. These statements include, but are not limited to, statements about the Company's strategy and clinical development plans. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would" and similar expressions are intended to 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 whether Aileron's cash resources will be sufficient to fund its continuing operations for the periods anticipated; whether initial results of clinical trials will be indicative of final results of those trials or results obtained in future clinical trials, including trials in different indications; whether ALRN-6924 will advance through the clinical trial process on a timely basis, or at all; whether the results of such trials will be accepted by and warrant submission for approval from the United States Food and Drug Administration or equivalent foreign regulatory agencies; whether ALRN-6924 will receive approval from regulatory agencies on a timely basis or at all; whether, if ALRN-6924 obtains approval, it will be successfully distributed and marketed; what impact the coronavirus pandemic may have on the timing of our clinical development, clinical supply and our operations; and other factors discussed in the "Risk Factors" section of Aileron's quarterly report on Form 10-Q for the period ended September 30, 2020, filed on November 12, 2020, and risks described in other filings that Aileron may make with the Securities and Exchange Commission. Any forward-looking statements contained in this press release speak only as of the date hereof, and Aileron specifically disclaims any obligation to update any forward-looking statement, whether because of new information, future events or otherwise.

Investor Contacts:
Richard Wanstall, SVP Chief Financial Officer
Aileron Therapeutics
617-995-0900
rwanstall@aileronrx.com

Hans C. Vitzthum
LifeSci Advisors, LLC.
617-430-7578
hans@lifesciadvisors.com

Media Contact:
Liz Melone
617-256-6622
lmelone@aileronrx.com