# Aileron Therapeutics Announces Completion of Enrollment in Dose Optimization Expansion Cohort of Proof-of-Concept Phase 1b Study of ALRN-6924

August 3, 2020

- Study is evaluating ALRN-6924 to protect against chemotherapy-induced bone marrow toxicities in patients with p53-mutated small cell lung cancer (SCLC) treated with topotecan
- Previously reported positive interim data from dose optimization part of study demonstrated 0.3 mg/kg dose of ALRN-6924, now being evaluated in expansion cohort, resulted in most robust chemoprotective effects
- Data readouts anticipated in Q4 2020: Final dose optimization data, including data from expansion cohort as well as pharmacodynamic biomarker and tumor efficacy data; preliminary data from recently initiated schedule optimization part of study

WATERTOWN, Mass., Aug. 03, 2020 (GLOBE NEWSWIRE) -- Aileron Therapeutics (NASDAQ:ALRN) announced today that it has completed enrollment in the dose optimization expansion cohort of its ongoing open-label Phase 1b clinical study of ALRN-6924. Aileron is focused on transforming the experience of chemotherapy for cancer patients by developing and delivering a novel chemoprotective medicine to protect against multiple chemotherapy-induced side effects. The proof-of-concept Phase 1b study is evaluating ALRN-6924 as a therapeutic agent administered ahead of chemotherapy to prevent chemotherapy-induced bone marrow toxicities, such as severe anemia, thrombocytopenia and neutropenia, in patients with p53-mutated small cell lung cancer (SCLC) who are being treated with topotecan. The protocol for this study consists of a phase 1b and a randomized, controlled phase 2; the Phase 1b has two parts: dose optimization and schedule optimization.

“We’re very encouraged by the data we reported from the dose optimization part of the Phase 1b in June demonstrating ALRN-6924’s potential to shield multiple healthy cell types from chemotherapy-induced damage without limiting the effect of chemotherapy on cancer cells,” said Manuel Aivado, M.D., Ph.D., President and Chief Executive Officer at Aileron. “Per the interim findings, the 0.3 mg/kg dose of ALRN-6924 achieved the most robust and consistent chemoprotective effects of the three dose levels evaluated. The chemoprotection results were further supported by pharmacodynamic biomarker results also observed in the interim analysis.”

Dr. Aivado continued, “We look forward to reporting the final results of the dose optimization part of the Phase 1b, including findings from the 0.3 mg/kg expansion cohort, as well as preliminary results from the recently initiated schedule optimization part of the Phase 1b, in Q4 of this year. We believe that the results from the Phase 1b, along with results from a healthy volunteer study that we plan to initiate in Q3 of this year, will further support and de-risk our plans to develop ALRN-6924 as a chemoprotective agent for patients with multiple cancer types who are treated with various chemotherapies.”

In June 2020, Aileron announced positive interim data from the dose optimization part of the Phase 1b study demonstrating that treatment with ALRN-6924 resulted in protective effects against severe chemotherapy-induced anemia and thrombocytopenia in patients across three dose levels (0.3, 0.6, and 1.2 mg/kg) relative to historical controls. In addition, patients treated with 0.3 mg/kg ALRN-6924 also met the protocol-defined criterion for reduction of NCI CTC Grade 3/4 neutropenia to ≤50% in the first treatment cycle, triggering a cohort expansion at this dose level, from six to 14 patients.

As previously guided, in the fourth quarter of 2020, Aileron plans to report final Phase 1b dose optimization data, including data from the dose optimization expansion cohort as well as pharmacodynamic biomarker and tumor efficacy data, in addition to preliminary Phase 1b schedule optimization data. Additionally, in the third quarter or 2020, Aileron plans to initiate enrollment in a healthy volunteer study to determine dosing schedules for ALRN-6924 that will support and further de-risk the company’s long-term vision to provide a chemoprotective medicine for patients with a p53-mutation regardless of cancer type or chemotherapeutic drug. Aileron will continue to carefully monitor the effect of the coronavirus pandemic on its clinical trial sites and the healthcare system, which may impact its planned data announcements.

**About the Dose Optimization Expansion Cohort Design**

In the dose optimization expansion cohort, which has completed planned enrollment of eight patients, ALRN-6924 is being administered at a 0.3

mg/kg dose level 24 hours before each dose of topotecan. Topotecan is being administered days 1 through 5 every 21 days.

**How ALRN-6924 Works to Protect Healthy Cells from Chemotherapy-Induced Damage**

ALRN-6924 is being developed by Aileron as a novel chemoprotective medicine to treat and protect healthy cells in patients with cancer that harbors p53-mutations to reduce or eliminate chemotherapy-induced side effects.

Chemotherapy targets cells that are cycling, or 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 (which give rise to red blood cells, white blood cells, and platelets), hair follicle cells, skin cells, and cells lining the oral cavity and the gastrointestinal tract. As a result, chemotherapy targets and kills both cycling healthy cells and cycling cancer cells. This, in turn, leads to a spectrum of chemotherapy-induced side effects, from unpleasant to life-threatening.

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

**About Aileron Therapeutics**

At Aileron, we are focused on transforming the experience of chemotherapy for cancer patients, enabling them to fight cancer without the fear or burden of chemotherapy-induced side effects. We are advancing ALRN-6924, our first-in-class dual MDM2/MDMX inhibitor currently in clinical development, to provide a single medicine to protect multiple healthy cell types throughout the body from chemotherapy while ensuring chemotherapy continues to destroy cancer cells.

In addition to potentially reducing or eliminating multiple side effects, ALRN-6924 may also improve patients' quality of life and help them better tolerate chemotherapy, potentially allowing patients to complete their treatment without dose reductions or delays. Our long-term vision is to provide chemoprotection for patients with p53-mutated cancers, which represents approximately 50% of cancer patients, regardless of cancer type or chemotherapeutic drug. 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 and/or trials anticipated; whether results obtained in preclinical and nonclinical studies and clinical trials will be indicative of results obtained in future clinical trials; whether preliminary or interim results from a clinical trial such as the interim data referenced in this release will be indicative of the final results of the trial; whether Aileron's product candidates will advance through the clinical trial process on a timely basis, or at all; whether the results of such trials will warrant submission for approval from the United States Food and Drug Administration or equivalent foreign regulatory agencies; whether Aileron's product candidates will receive approval from regulatory agencies on a timely basis or at all; whether, if product candidates obtain approval, they will be successfully distributed and marketed; whether the coronavirus pandemic will have an impact 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 March 31, 2020, filed on May 11, 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.

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