Aileron Therapeutics Reports First Quarter 2021 Financial Results and Provides Business Update

May 11, 2021

- *Progressing ALRN-6924: on track to initiate Phase 1b randomized placebo-controlled trial in patients with p53-mutated non-small cell lung cancer in 2nd quarter 2021 with early interim read-out expected at end of 2021*

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- *ALRN-6924 is designed to deliver selective chemoprotection against chemotherapy-induced toxicities via p53 biomarker strategy*
    - *50% of all cancer patients across multiple tumor types have a p53 mutation*

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- *Abstract co-authored with Foundation Medicine evaluating frequency of longitudinal changes in TP53 mutation status accepted for presentation at 2021 ASCO Annual Meeting*

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- *Aileron to host KOL Fireside Chat moderated by Soumit Roy, Ph.D., Vice President, Healthcare Analyst with JonesTrading, on May 26, 2021*
    - *Event will feature renowned hematologist Alan List, M.D. and prominent geriatric oncologist Lodovico Balducci, M.D.*

BOSTON, May 11, 2021 (GLOBE NEWSWIRE) -- Aileron Therapeutics (NASDAQ:ALRN), a chemoprotection oncology company focused on fundamentally transforming the experience of chemotherapy for cancer patients, today reported business highlights and financial results for the first quarter ended March 31, 2021.

“We continue to work diligently to initiate our first randomized, double-blind, placebo-controlled clinical trial of ALRN-6924 as a novel therapeutic to protect non-small cell lung cancer (NSCLC) patients receiving first-line chemotherapy against various chemotherapy-induced toxicities. Approximately 50% of NSCLC patients have a p53 mutation, suggesting the broad potential impact of ALRN-6924 in this indication alone,” said Manuel Aivado, M.D., Ph.D., President and Chief Executive Officer.

Dr. Aivado further commented, “Simultaneously, we are actively planning for the rapid advancement of ALRN-6924 into late-stage development for patients with p53-mutated NSCLC pending the outcome of the Phase 1b trial. Bolstered by our successful capital raises in the first quarter of 2021, we are making near-term strategic investments in CMC, p53 companion diagnostic development, and team scale-up which we expect will not only enable the future advancement of our NSCLC clinical program, but also will provide foundational support for future clinical programs of ALRN-6924 across multiple p53-mutated cancer indications.”

Aileron is developing ALRN-6924 to selectively protect healthy cells in patients with cancers that harbor p53 mutations, which are present in over half of all cancer patients, to reduce or eliminate chemotherapy-induced side effects while not interfering with chemotherapy’s attack on cancer cells. This novel concept is known as chemoprotection. By reducing or eliminating multiple chemotherapy-induced side effects, ALRN-6924 may 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 as a result. Given Aileron’s p53 biomarker approach, designed to ensure selective chemoprotection only in healthy cells, coupled with the high prevalence of p53-mutated cancers, the company’s strategy is to ultimately pursue a tumor-agnostic label for ALRN-6924 as a chemoprotective agent in p53-mutated cancers.

First Quarter 2021 Highlights and Recent Updates

- **Aileron to host upcoming KOL Fireside Chat, “Protecting Cancer Patients from Chemotherapy-Induced Toxicities -**

A New Paradigm”. Moderated by Soumit Roy, Ph.D., Vice President, Healthcare Analyst with JonesTrading Institutional Services LLC, the event will include a discussion on the unmet need and potential for chemoprotection with Alan List, M.D. and Lodovico Balducci, M.D. Dr. List, who recently was appointed Chief Medical Officer of Precision Biosciences, is a renowned hematologist with extensive academic and clinical experience in the research and development of hematology and oncology products. Dr. List is also a member of Aileron’s Scientific Advisory Board. Dr. Balducci, a prominent geriatric oncologist, previously served as Senior Member of the Senior Adult Oncology Program and Medical Director of Affiliates and Referring Physician Relations at the Moffitt Cancer Center. Link here to register for the event, which will take place on Wednesday, May 26, 2021.

- **Abstract co-authored by Aileron and Foundation Medicine, Inc. to be presented at 2021 ASCO Annual Meeting.** The abstract, titled, “Gene Sequencing of Serial Tumor Biopsies from a Large Cohort of Cancer Patients Shows Longitudinal Changes in *TP53* Mutation Status Are Uncommon” (Abstract #3124), was accepted as a poster presentation at the meeting. Additional details will be announced upon ASCO’s online publication of this year’s abstracts later this month.

- **Announced enrollment expansion for upcoming Phase 1b clinical trial of ALRN-6924 in patients with advanced NSCLC.** In February 2021, Aileron announced a 50% expansion of its enrollment target for its upcoming Phase 1b clinical trial of ALRN-6924 in patients with NSCLC undergoing chemotherapy. Aileron plans to enroll 60 patients with advanced p53-mutated NSCLC undergoing treatment with first-line carboplatin plus pemetrexed (with or without immune checkpoint inhibitors). Aileron anticipates this enrollment expansion will enable a more robust exploration of ALRN-6924 as a novel chemoprotective agent to prevent chemotherapy-induced toxicities in the NSCLC patient population and better position the company to rapidly advance ALRN-6924 into late-stage clinical development for NSCLC following the Phase 1b trial.

  In the 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 plans to initiate the NSCLC trial in the second quarter of 2021 and anticipates reporting early interim data at the end of 2021 and topline results in mid-2022.

- **During the first quarter, Aileron completed its Phase 1b evaluation of ALRN-6924 at the recommended phase 2 dose of 0.3 mg/kg in patients with SCLC receiving ALRN-6924 24-hours prior to topotecan and conducted preliminary evaluation of data from additional cohorts.** These preliminary findings from 11 additional patients (n=7 patients receiving 0.3 mg/kg ALRN-6924 six hours before topotecan and n=4 patients receiving 0.2 mg/kg ALRN-6924 twenty-four hours before topotecan) were in line with data presented last October and with Aileron’s expectation that administering ALRN-6924 at 0.3mg/kg and 24 hours before topotecan remains the optimal dose and schedule in this patient population. Aileron expects to present results of the Phase 1b SCLC trial at a scientific conference in the second half of 2021.

- **Raised $55.7 million in aggregate proceeds from the sale of common stock during first quarter 2021, which based on Aileron’s current operating plan, is expected to provide funding into the second half of 2023 and approximately 12 months beyond the anticipated topline results from the NSCLC trial.** In January 2021, Aileron completed a registered direct offering of common stock, for a purchase price of $1.10 per share, raising $33.1 million in aggregate net proceeds, after deducting placement agent fees and other offering expenses payable by Aileron. New fundamental and institutional investors, including Acorn Bioventures, BVF Partners, L.P., Maven Investment Partners and Grand Oaks Capital, participated in the offering, in addition to several existing Aileron investors, including Satter Medical Technology Partners and Lincoln Park Capital Fund, LLC. In addition to the registered direct offering, since January 1, 2021, Aileron sold an aggregate of 13,775,399 shares of its common stock for an average purchase price of $1.64 per share, for aggregate net proceeds of $22.6 million, after deducting fees and offering expenses, in “at the market” offerings and under its structured equity line with Lincoln Park Capital Fund, LLC.

- **Continuing healthy volunteer study to support long-term clinical development strategy for ALRN-6924.** The ongoing study, initiated in November 2020, is designed to characterize the time to onset, magnitude, and duration of cell cycle arrest in human bone marrow relative to ALRN-6924 administration. Another goal of the study is to develop a universal dosing regimen for ALRN-6924 for use as a chemoprotection agent across a range of chemotherapies and p53-mutated tumor indications. Aileron expects to present results from the healthy volunteer study at a scientific conference in the second half of 2021.

First Quarter 2021 Financial Results

- **Cash Position:** Cash, cash equivalents and investments at March 31, 2021 were $63.4 million, compared to $13.8 million at December 31, 2020. The company expects, based on its current operating plan, that its existing cash, cash equivalents and investments will fund operations into the second half of 2023.

- **Research and Development (R&D) Expenses:** R&D expenses for the quarter ended March 31, 2021 were $4.3 million, compared to $4.1 million for the corresponding quarter in 2020. The increase of $0.2 million is primarily a result of increased spending on clinical development of ALRN-6924 in connection with the preparation for the upcoming Phase 1b clinical trial in patients with advanced NSCLC patients being treated with first-line chemotherapy of carboplatin and pemetrexed and is partially offset by the effect of cost savings measures implemented in 2020 resulting in decreased spending on employee, facility and other development expenses.

- **General and Administrative (G&A) Expenses:** G&A expenses for the quarter ended March 31, 2021 were $2.7 million, compared to $2.8 million for the corresponding quarter in 2020. The decrease in general and administrative expense is the result of lower headcount and facility costs during the three months ended March 31, 2021 as a result of cost savings measures as compared to the three months ended March 31, 2020 and is partially offset by increased spending on professional services.

- **Net Loss:** Net loss for the quarter ended March 31, 2021 was $7.0 million, compared to $6.7 million for the corresponding quarter in 2020. The basic and diluted net loss per share for the first quarter of 2021 was $0.08 compared to $0.24 for the first quarter of 2020. The change in basic and diluted net loss per share is primarily a result of increased shares outstanding in connection with sales of common stock during the first quarter of 2021.

About Aileron Therapeutics

Aileron is a clinical stage chemoprotection oncology company focused on fundamentally transforming the experience of chemotherapy for cancer patients. 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. By reducing or eliminating multiple chemotherapy-induced side effects, ALRN-6924 may 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 or with respect to the matters 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 annual report on Form 10-Q for the quarter ended March 31, 2021, filed on May 11, 2021, 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

Aileron Therapeutics, Inc.
Balance Sheet Data
(In thousands)

	March 31, 2021	December 31, 2020
Cash, cash equivalents and investments	$ 63,351	$ 13,805
Working capital	61,251	12,366
Total assets	64,671	16,341
Accumulated deficit	(226,267)	(219,292)
Total stockholders' equity	61,419	12,162

Aileron Therapeutics, Inc.
Condensed Statement of Operations
(In thousands, except share and per share data)

	Three Months Ended March 31,	
	2021	2020
Revenue	$ -	$ -
Operating expenses:		
Research and development	4,316	4,069
General and administrative	2,673	2,807
Total Operating expenses	6,989	6,876
Loss from operations	(6,989)	(6,876)
Interest and other income	14	128
Net loss	(6,975)	(6,748)
Net loss per share — basic and diluted	$ (0.08)	$ (0.24)
Weighted average common shares outstanding—basic and diluted	83,384,371	27,810,358