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

August 11, 2021

- *Initiated Phase 1b randomized, double-blind, placebo-controlled trial of ALRN-6924 in patients with advanced p53-mutated non-small cell lung cancer (NSCLC); first interim safety data expected at end of 2021 and topline results mid-2022*

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- *Announces upcoming presentations to highlight final data from Aileron's completed Phase 1b trial of ALRN-6924 in patients with small cell lung cancer (SCLC) and initial findings from ongoing Healthy Volunteer Study of ALRN-6924*
  - *ISEH 2021 Virtual Scientific Meeting (August 25-28, 2021)*
  - *ESMO Congress 2021 (September 16-21, 2021)*

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- *ALRN-6924 is designed to deliver selective chemoprotection against chemotherapy-induced toxicities via p53 biomarker strategy*
  - *Nearly 1 million patients in the U.S. across all cancer types are diagnosed annually with p53-mutated cancer*

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- *$59.5 million cash and cash equivalents as of June 30, 2021, expected to fund operations into second half of 2023*

BOSTON, Aug. 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 second quarter ended June 30, 2021.

"Bolstered by a strong balance sheet and a healthy cash runway, we continue to execute against our clinical development strategy to advance selective chemoprotection for patients with p53-mutated cancer, including our initiation in the second quarter of our first randomized, double-blind, placebo-controlled clinical trial of ALRN-6924 for patients with p53-mutated non-small cell lung cancer (NSCLC)," said Manuel Aivado, M.D., Ph.D., President and Chief Executive Officer at Aileron.

Dr. Aivado continued, "The findings from this NSCLC trial will inform our late-stage clinical development strategy for ALRN-6924. Nearly 1 million cancer patients across all cancer types each year are diagnosed with p53-mutated cancer in the United States alone, and chemotherapy continues to be a cornerstone of cancer treatment for nearly every patient. As such, we believe that ALRN-6924 has the potential to protect many patients from chemotherapy-induced effects, thereby transforming their quality of life while they undergo chemotherapy and without reducing the effects of chemotherapy against cancer."

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 lead to fewer dose reductions or delays of chemotherapy, which could result in an improved efficacy of chemotherapy. Given Aileron's p53 biomarker approach, designed to ensure selective chemoprotection only of healthy cells, coupled with the 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.

Second Quarter 2021 Highlights and Recent Updates

- **Initiated randomized, double-blind, placebo-controlled Phase 1b clinical trial of ALRN-6924 in patients with advanced NSCLC undergoing chemotherapy.** Aileron anticipates enrolling a total of 60 patients with advanced

p53-mutated NSCLC undergoing treatment with first-line carboplatin plus pemetrexed with or without immune checkpoint inhibitors. Patients enrolled in the NSCLC trial will be randomized 1:1 to receive carboplatin/pemetrexed plus 0.3 mg/kg ALRN-6924 or placebo for at least four 21-day treatment cycles. Components of the primary endpoint are the proportion of treatment cycles free of severe hematological and other toxicities, including Grade ≥ 3 neutropenia, Grade ≥ 3 thrombocytopenia, Grade ≥ 3 anemia, Grade 4 neutropenia and febrile neutropenia, as well as duration of Grade 4 neutropenia. An additional component of the primary endpoint is the proportion of completed treatment cycles without chemotherapy dose reduction or without the use of growth factors or transfusions. Other endpoints include the proportion of patients with National Cancer Institute Common Terminology Criteria Adverse Events Grade 3/4 treatment-emergent adverse events, quality of life, overall response rate, and progression-free survival.

Aileron anticipates reporting first interim safety data from the trial late in the fourth quarter of 2021 and topline results in mid-2022.

- **Company to present final data from ALRN-6924 SCLC study and initial data from Healthy Volunteer Study at two upcoming scientific congresses.** Aileron has had abstracts accepted for presentation at the International Society for Experimental Hematology (ISEH) 2021 Virtual Scientific Meeting, being held August 25-28, 2021, and the European Society for Medical Oncology (ESMO) Congress 2021, being held September 16-21, 2021. At these congresses, Aileron will present final data from its Phase 1b study of ALRN-6924 in patients with SCLC undergoing chemotherapy with second-line topotecan. This presentation follows Aileron's October 2020 presentation of data from its Phase 1b clinical trial of ALRN-6924 in SCLC demonstrating clinical proof-of-concept that treatment with ALRN-6924 resulted in a protective effect against neutropenia, thrombocytopenia and anemia in patients with p53-mutated SCLC treated with topotecan. A link to the SCLC presentation from October 2020 can be found here.

  Aileron will also present initial data at both meetings from its ongoing Healthy Volunteer Study, which is evaluating ALRN-6924's induction of p21-induced cell cycle arrest in healthy, normal bone marrow cells and other cell types in healthy volunteers receiving ALRN-6924. p53 upregulates the expression of p21, a known inhibitor of cell cycle. The Healthy Volunteer Study, therefore, is designed to characterize the time to onset, magnitude and duration of p21-induced cell cycle arrest in human bone marrow relative to ALRN-6924. The ultimate aim 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.

- **Presented poster at 2021 ASCO Annual Meeting, co-authored by Foundation Medicine, Inc., demonstrating rarity of longitudinal changes in p53 mutation status.** In June 2021, Aileron presented a poster co-authored by Foundation Medicine, Inc., entitled, "Frequency of longitudinal changes in TP53 mutation status from gene sequencing of serial tumor biopsies from a large cohort of cancer patients," at the 2021 ASCO Annual Meeting. The companies examined approximately 16,500 samples arising from repeat biopsies from more than 7,800 patients across a spectrum of cancer types and over an average interval of 11 months and up to 23 months between biopsies. The analysis found that in nearly all of the biopsies evaluated (97%), an initial p53-mutant diagnosis was maintained in subsequent biopsies, regardless of cancer type, primary tumor versus metastases, or time period between subsequent biopsies.

Second Quarter 2021 Financial Results

- **Cash Position:** Cash, cash equivalents and investments on June 30, 2021 were $59.5 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 June 30, 2021 were $3.9 million, compared to $2.5 million for the quarter ended June 30, 2020. The increase of $1.4 million primarily results from increased spending for clinical development of ALRN-6924 for the company's Phase 1b clinical trial in patients with advanced NSCLC and the study of ALRN-6924 in healthy volunteers. The company also increased its spending on ALRN-6924 manufacturing costs, employee-related costs and non-clinical research costs while decreasing facility-related spending.

- **General and Administrative (G&A) Expenses:** G&A expenses for the quarter ended June 30, 2021 were $2.2 million compared to $1.9 million for the quarter ended June 30, 2020. Increased G&A expenses were primarily the result of higher employee-related costs partially offset by lower facility costs.

- **Net Loss:** Net loss for the quarter ended June 30, 2021 was $5.7 million, compared to $4.4 million for the corresponding quarter in 2020. The basic and diluted net loss per share for the second quarter of 2021 was $0.06 compared to $0.14 for the second 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, is designed to activate p53, which in turn upregulates p21, a known inhibitor of the cell replication cycle. ALRN-6924 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 June 30, 2021, filed on August 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.

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

	June 30, 2021	December 31, 2020
Cash, cash equivalents and investments	$ 59,479	$ 13,805
Working capital	55,879	12,366
Total assets	60,531	16,341
Accumulated deficit	(231,953)	(219,292)
Total stockholders' equity	56,130	12,162

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

	Three Months Ended June 30,	Six Months Ended June 30,

	2021	2020	2021	2020
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	3,853	2,488	8,169	6,557
General and administrative	2,156	1,912	4,829	4,719
Total Operating expenses	6,009	4,400	12,998	11,276
Loss from operations	(6,009)	(4,400)	(12,998)	(11,276)
Interest and other income, net	323	10	337	138
Net loss	(5,686)	(4,390)	(12,661)	(11,138)
Net loss per share — basic and diluted	$ (0.06)	$ (0.14)	$ (0.15)	$ (0.38)
Weighted average common shares outstanding — basic and diluted	90,458,550	31,221,139	86,941,002	29,515,749