Aileron Therapeutics Reports Third Quarter 2021 Financial Results and Business Highlights

November 12, 2021

- Continues to progress Phase 1b randomized, double-blind, placebo-controlled trial of ALRN-6924 in patients with p53-mutated non-small cell lung cancer (NSCLC) undergoing chemotherapy
    - Updated planned NSCLC study data readouts include interim results in 2Q22; topline full results in 4Q22

- Reported preliminary data on ALRN-6924's p21-mediated cell cycle arrest in healthy humans
    - Confirmed biomarker-driven mechanism of action
    - Characterized time to onset, magnitude and duration of cell cycle arrest, informing potential universal dose and schedule across distinct chemotherapy regimens

- $52.2 million cash and cash equivalents as of September 30, 2021, expected to fund operations into second half of 2023

BOSTON, Nov. 12, 2021 (GLOBE NEWSWIRE) -- Aileron Therapeutics (NASDAQ:ALRN), a chemoprotection oncology company focused on fundamentally transforming the experience of chemotherapy for cancer patients, today reported financial results and business highlights for the third quarter ended September 30, 2021.

"In the third quarter, we continued our clinical progress, reporting final positive results from our chemoprotection proof-of-concept SCLC study and confirming ALRN-6924's p21-mediated cell cycle arrest in our healthy volunteer study. We also reported healthy volunteer data on time to onset, magnitude and duration of ALRN-6924's cell cycle arrest, which provides support for a universal dose and schedule across distinct chemotherapy regimens," said Manuel Aivado, M.D., Ph.D., President and Chief Executive Officer at Aileron. "In the year ahead, we anticipate key data readouts from our NSCLC trial, as we continue to advance toward our vision to deliver selective chemoprotection to patients with p53-mutant cancers regardless of type of chemotherapy or cancer."

Dr. Aivado continued, "We recently presented preclinical data demonstrating cell cycle arrest in healthy tissue outside of the bone marrow – namely, epithelial mucosa cells in the GI tract. This is another important strategic building block, as a key characteristic of ALRN-6924's differentiated profile is the potential to selectively protect multiple tissues and organs from chemotherapy-induced toxicities with a single medicine – representing a potential paradigm shift in the care of millions of cancer patients."

Despite the rise of immunotherapies and targeted therapies, chemotherapy continues to play a central role, at some point in the treatment continuum, for nearly all cancer patients, bringing with it significant toxicities. Aileron is developing ALRN-6924 to selectively protect healthy cells in patients with p53-mutated cancers to reduce or eliminate chemotherapy-induced side effects. Nearly 1 million patients each year are diagnosed with a p53-mutated cancer in the US alone. Aileron employs a precision medicine approach, exclusively treating patients with p53-mutated cancers; ALRN-6924 is designed to selectively protect these patients' healthy cells from chemotherapy without protecting cancer cells. This novel concept is known as selective chemoprotection. The reduction or elimination of multiple chemotherapy-induced side effects may enhance tolerability of chemotherapy, may result in fewer dose reductions or delays of chemotherapy, and the potential for improved efficacy of chemotherapy. Aileron's 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.

Third Quarter 2021 Highlights and Recent Updates

- **Continued to progress randomized, double-blind, placebo-controlled Phase 1b clinical trial of ALRN-6924 for the prevention of chemotherapy-induced side effects in patients with advanced p53-mutated NSCLC.** Aileron plans to enroll 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 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.

  Currently, 12 trial sites are open for enrollment in the United States and Europe, and Aileron anticipates opening additional

sites. Due to the impact of the COVID-19 Delta variant in Eastern Europe, several sites in this region began screening two months later than planned. With those sites now activated, there has been an associated increase in patient enrollment. As a result of the delay in site activation and patient enrollment, Aileron has updated its guidance for planned data readouts as follows: interim results on 20 patients with four cycles in 2Q22, and topline results on 60 patients with four cycles in 4Q22. The company also plans to conduct a blinded safety evaluation on 10 patients after one cycle in 1Q22 to determine whether the safety and tolerability profile of ALRN-6924 in NSCLC patients is consistent with the safety and tolerability profile observed in previous clinical trials.

For more information, please visit the NSCLC trial listing on clinicaltrials.gov.

- **Presented new preclinical data at AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics 2021 supporting potential of chemoprotection in healthy cells beyond bone marrow cells.** Aileron presented new preclinical data that demonstrated ALRN-6924's activity as a radioprotective agent in preclinical mouse models of acute radiation-induced toxicity, leveraging the same mechanism of action – p53 activation and subsequent p21 upregulation as well as p21-induced cell cycle arrest – that has clinically shown protection against chemotherapy-induced bone marrow toxicities.

  As part of this poster presentation, Aileron presented preclinical data demonstrating ALRN-6924's activation of p21-induced cell cycle arrest in murine bone marrow cells as well as in epithelial mucosa cells in the GI tract. ALRN-6924 is the first chemoprotective agent, either in clinical development or marketed, to demonstrate the potential of cell cycle arrest-mediated chemoprotection of bone marrow cells as well as cells outside of bone marrow. These findings are consistent with Aileron's belief that ALRN-6924 may also selectively protect additional organs and tissues from chemotherapy-induced toxicities.

- **Presented final data from Phase 1b trial of ALRN-6924 in SCLC patients at the European Society for Medical Oncology (ESMO) Congress 2021.** Aileron presented final results from its completed Phase 1b trial in patients with p53-mutated SCLC receiving second-line topotecan. Following the interim proof-of-concept data presented in October 2020 from this study, these final results reinforced ALRN-6924's 'triple-play' reduction in neutropenia, thrombocytopenia and anemia caused by chemotherapy, as well as a reduction of platelet and red blood cell transfusions, reported in October 2020.

- **Presented preliminary data from ALRN-6924 Healthy Volunteer Study at International Society for Experimental Hematology (ISEH) 2021 Scientific Meeting and ESMO 2021.** Aileron presented preliminary results from its ongoing Phase 1 pharmacology study of ALRN-6924 in healthy volunteers, which confirmed 0.3 mg/kg as the optimal dose for ALRN-6924 and confirmed the drug's novel p53 biomarker-driven mechanism of action, as well as its pharmacodynamic effects, including time to onset, magnitude and duration.

  The healthy volunteer study 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. The 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. The study is ongoing, and Aileron anticipates presenting additional findings at a later date.

Third Quarter 2021 Financial Results

- **Cash Position:** Cash, cash equivalents and investments on September 30, 2021 were $52.2 million. 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 September 30, 2021 were $4.3 million, compared to $2.7 million for the quarter ended September 30, 2020. The increase of $1.6 million primarily resulted from increased spending of $0.5 million related to clinical development of ALRN-6924 for the company's Phase 1b clinical trial in patients with advanced NSCLC and the company's Phase 1 trial of ALRN-6924 in healthy human volunteers, $0.7 million of ALRN-6924 manufacturing costs, $0.3 million of higher employee-related costs, and $0.2 million in non-clinical research costs, all partially offset by lower facility costs.

- **General and Administrative (G&A) Expenses:** G&A expenses for the quarter ended September 30, 2021 were $2.5 million compared to $2.3 million for the quarter ended September 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 September 30, 2021 was $6.7 million, compared to $5.0 million for the corresponding quarter in 2020. The basic and diluted net loss per share for the third quarter of 2021 was $0.07 compared

to $0.13 for the third quarter of 2020. The change in basic and diluted net loss per share is primarily a result of increased shares outstanding as a result of sales of common stock during the first quarter of 2021 partially offset by higher operating expense.

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 without protecting 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 and the Company's cash runway. 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 or in which territories or indications ALRN-6924 may receive approval; 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 September 30, 2021, filed on November 12, 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)

	September 30, 2021	December 31, 2020
Cash, cash equivalents and investments	$ 52,232	$ 13,805
Working capital	49,863	12,366
Total assets	54,768	16,341
Accumulated deficit	(238,657)	(219,292)
Total stockholders' equity	50,109	12,162

Condensed Statement of Operations
(In thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	4,278	2,684	12,447	9,241
General and administrative	2,513	2,344	7,342	7,063
Total operating expenses	6,791	5,028	19,789	16,304
Loss from operations	(6,791)	(5,028)	(19,789)	(16,304)
Interest and other income, net	87	5	424	143
Net loss	(6,704)	(5,023)	(19,365)	(16,161)
Net loss per share — basic and diluted	$ (0.07)	$ (0.13)	$ (0.22)	$ (0.49)
Weighted average common shares outstanding — basic and diluted	90,548,972	39,321,177	88,211,362	32,808,082