Aileron Therapeutics Reports Third Quarter 2020 Financial Results and Business Highlights

November 13, 2020

- Presented positive clinical proof-of-concept data from ongoing ALRN-6924 Phase 1b trial in patients with p53-mutated small cell lung cancer (SCLC) treated with topotecan in late-breaking presentation at 2020 EORTC-NCI-AACR Annual Symposium
- Phase 1b randomized, controlled trial in patients with p53-mutated advanced non-small cell lung cancer receiving first-line platinum-based chemotherapy projected to start in the second quarter of 2021
- Advancing long-term vision of bringing chemoprotection to all patients with p53-mutated cancers regardless of cancer type or chemotherapy
- Novel precision medicine strategy delivers chemoprotective agent with anticancer efficacy of chemotherapy fully intact

WATERTOWN, Mass., Nov. 12, 2020 (GLOBE NEWSWIRE) -- Aileron Therapeutics (NASDAQ:ALRN) today reported business highlights and financial results for the third quarter ended September 30, 2020.

“We recently achieved the critical milestone of clinical proof of concept of ALRN-6924, demonstrating a protective effect against severe anemia, thrombocytopenia and neutropenia in our ongoing Phase 1b trial of patients with p53-mutated small cell lung cancer undergoing treatment with topotecan,” said Manuel Aivado, M.D., Ph.D., President and Chief Executive Officer of Aileron. “These positive findings help inform our future clinical development strategy, which involves advancing randomized, controlled studies of ALRN-6924 in large cancer indications, including, non-small cell lung cancer, gastrointestinal cancers such as colorectal cancer, and other cancers.”

Dr. Aivado continued, “We are laying the foundation to advance our broad vision to bring chemoprotection to patients with p53-mutated cancers, which represent approximately 50% of cancer patients, regardless of cancer type or chemotherapy. We believe our approach, grounded on the principle of chemoprotection without the potential to interfere with chemotherapy’s anticancer activity, may establish ALRN-6924 as standard of care for chemoprotection among patients with p53-mutated cancers who are undergoing chemotherapy.”

Key Third Quarter and Recent Highlights

- **Announced proof-of-concept data from ongoing Phase 1b trial of ALRN-6924.** In October 2020, Aileron announced new positive clinical data from its ongoing Phase 1b trial demonstrating clinical proof of concept that treatment with ALRN-6924 given 24 hours prior to second-line topotecan administration resulted in a protective effect against severe chemotherapy-induced bone marrow toxicities – anemia, thrombocytopenia and neutropenia – in patients with p53-mutated small cell lung cancer (SCLC). Robust and clinically meaningful protection against toxicities were observed with the 0.3 mg/kg dose of ALRN-6924. The findings were featured in a late-breaking poster presentation at the 32nd EORTC-NCI-AACR Annual (ENA 2020) Symposium on Molecular Targets and Cancer Therapeutics. The poster presentation and associated data press release can be viewed here and here, respectively. An archived webcast with company management and the study’s principal investor, Bojan Zaric, M.D., Ph.D., discussing the findings, can be found on Aileron’s website here.

  Chemotherapy is unselective, meaning it cannot distinguish between cancer cells and healthy cells. As a result, chemotherapy destroys both cancer cells and rapidly dividing healthy cells, such as bone marrow cells, hair follicle cells and skin cells, among others. ALRN-6924 is a cell-permeating peptide drug designed to work intracellularly, activating wild-type p53 to arrest cell cycling in normal, healthy cells and thereby selectively shield these cells from chemotherapy in patients who harbor p53-mutant tumors, without interrupting chemotherapy’s targeting of cancer cells.

“Chemotherapy-induced toxicities are a long-overlooked and unaddressed area of significant unmet need among the millions of cancer patients undergoing chemotherapy,” said Dr. Aivado. “For our ongoing Phase 1b trial we have focused on chemotherapy-induced bone marrow toxicities

because of their severe, often life-threatening consequences for patients and also because they are the most objectively quantifiable to evaluate chemoprotection. Biologically, we believe that ALRN-6924's ability to arrest cell cycling in normal, healthy cells has the potential to protect patients undergoing chemotherapy against a spectrum of side effects beyond bone marrow toxicities, such as hair loss, nausea, vomiting, diarrhea and fatigue."

Upcoming Milestones

Following the achievement of clinical proof-of-concept for ALRN-6924, Aileron anticipates undertaking the following next steps to progress and expand clinical development of ALRN-6924.

- **Initiate Phase 1b randomized, controlled chemoprotection trial in patients with advanced non-small cell lung cancer.** Aileron is planning to start a Phase 1b randomized, controlled trial of ALRN-6924 in patients with p53-mutated advanced non-small cell lung cancer who are receiving first-line platinum-based chemotherapy in the second quarter of 2021, subject to additional funding.

- **Complete schedule optimization part of the Phase 1b trial in small cell lung cancer to inform potential alternative dosing schedule for additional flexibility.** Aileron continues to enroll patients in the schedule optimization part of the Phase 1b trial in small cell lung cancer, which is intended to determine whether ALRN-6924 given six hours prior to topotecan ("6h-schedule part") could be an alternative dosing schedule that could provide patients and healthcare providers with additional flexibility of when to administer ALRN-6924 before topotecan. Aileron expects to report final data from the Phase 1b trial, including data from the 6h-schedule part, in the first quarter of 2021.

- **Undertake healthy volunteer study to support long-term clinical development strategy for ALRN-6924.** In the fourth quarter of 2020, Aileron plans to initiate a study of ALRN-6924 in healthy volunteers to gather data to support the company's long-term strategy to bring chemoprotection to all patients with p53-mutated cancer regardless of cancer type or chemotherapy. Specifically, the study is being conducted to characterize the time to onset, and the magnitude and duration of cell cycle arrest in human bone marrow relative to ALRN-6924 administration. This study is designed to further support Aileron's design of future randomized, controlled studies of ALRN-6924 when given prior to various chemotherapies.

Third Quarter 2020 Financial Results

- **Cash Position:** Cash, cash equivalents and investments as of September 30, 2020 were $14.1 million, compared to $18.3 million as of December 31, 2019. We expect, based on our current operating plan, that our cash, cash equivalents and investments will fund operations into the fourth quarter of 2021.

- **Research and Development Expenses:** Research and development expenses for the quarter ended September 30, 2020 were $2.7 million, compared to $4.5 million for the quarter ended September 30, 2019.

- **General and Administrative Expenses:** General and administrative expenses were $2.3 million for the quarter ended September 30, 2020, compared to $3.4 million for quarter ended September 30, 2019.

- **Net Loss:** Net loss was $5.0 million for the quarter ended September 30, 2020, compared to $7.7 million for the corresponding period in 2019.

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. ALRN-6924, our first-in-class MDM2/MDMX dual inhibitor activating p53, is the only reported therapeutic agent in clinical development to employ a biomarker strategy, in which we exclusively focus on treating patients with p53-mutated cancers. With this unique, targeted strategy, ALRN-6924 is designed to protect multiple healthy cell types throughout the body from chemotherapy while 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 bring chemoprotection to patients with p53-mutated cancers – approximately 50% of cancer patients – regardless of cancer type 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 the Company will obtain sufficient cash resources to conduct its planned clinical trials; whether results obtained in clinical trials will be indicative of results obtained in future clinical trials; 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 be accepted by and 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; 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.

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

	September 30, 2020	December 31, 2019
Cash, cash equivalents and investments	$ 14,121	$ 18,278
Working capital	$ 10,725	$ 13,711
Total assets	$ 22,239	$ 26,473
Accumulated deficit	$ (214,296)	$ (198,135)
Total stockholders' equity	$ 12,458	$ 16,048

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	2,684	4,475	9,241	12,953
General and administrative	2,344	3,440	7,063	9,654
Total Operating expenses	5,028	7,915	16,304	22,607
Loss from operations	(5,028)	(7,915)	(16,304)	(22,607)
Gain on sale of property and equipment	-	-	66	-
Interest income	5	166	77	473
Net loss	(5,023)	(7,749)	(16,161)	(22,134)
Net loss per share — basic and diluted	$ (0.13)	$ (0.28)	$ (0.49)	$ (0.95)
Weighted average common shares outstanding—basic and diluted	39,321,177	27,810,358	32,808,082	23,431,823