Aileron Therapeutics Reports Fourth Quarter and Full Year 2020 Financial Results and Provides Business Highlights

March 24, 2021

- *2020 marked a year of successful evolution to a chemoprotection oncology company focused on fundamentally transforming the experience of chemotherapy for cancer patients*

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- *Advancing selective chemoprotection via p53 biomarker strategy; 50% of all cancer patients across multiple tumor types have a p53 mutation*
  - *Achieved clinical proof-of-concept in Phase 1b study of ALRN-6924 in p53-mutated small cell lung cancer (SCLC)*
  - *Aileron's strategy is to ultimately pursue a tumor-agnostic indication*

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- *On track to initiate new Phase 1b placebo-controlled trial of ALRN-6924 in patients with advanced p53-mutated non-small cell lung cancer (NSCLC) in second quarter 2021*
  - *Planned data readouts: Interim data end of 2021; Topline results mid-2022*

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- *Aggregate proceeds of $55.7 million from equity fundraises in the first quarter of 2021, together with cash and investments of $13.8 million as of December 31, 2020, expected to support operations into second half of 2023*
  - *Strategically investing in CMC, p53 companion diagnostic development and team scale-up to accelerate entry into late-stage development of ALRN-6924 in NSCLC*

WATERTOWN, Mass., March 24, 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 fourth quarter and year ended December 31, 2020.

"In the fourth quarter of 2020, our Phase 1b trial in SCLC showed that ALRN-6924 has the potential for best-in-class protection against thrombocytopenia, neutropenia and anemia in SCLC patients receiving second-line topotecan chemotherapy. Bolstered by these robust proof-of-concept data, we are on the threshold of initiating our first randomized, double-blind placebo-controlled trial with ALRN-6924 to 'chemoprotect' patients with NSCLC who are receiving first-line chemotherapy," said Manuel Aivado, M.D., Ph.D., President and Chief Executive Officer.

Dr. Aivado further commented, "Given our significant capital raises in early 2021, we now believe that we have the resources to strategically invest in CMC, p53 companion diagnostic development and team scale-up, with the explicit goal of rapidly advancing ALRN-6924 into late-stage development for NSCLC following the Phase 1b trial. Bringing selective chemoprotection to NSCLC patients, approximately 50% of whom have a p53 mutation, will be a critical step toward achieving our vision of bringing selective chemoprotection to patients with p53-mutated cancers across all types of cancers and chemotherapies."

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, a novel concept 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. The regulatory pathway has been clarified for chemoprotection with the recent U.S. Food and Drug Administration (FDA) approval of the first myeloprotective drug for patients with extensive-stage SCLC.[1] Given Aileron's biomarker approach, designed to ensure selective chemoprotection, coupled with the high prevalence of p53-mutated cancers, the company's strategy is to ultimately pursue a tumor-agnostic

indication for ALRN-6924.

Recent and Fourth Quarter and Full Year 2020 Highlights

Recent Highlights

- **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, increased from the original target of 40 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 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 begin enrolling patients in the NSCLC trial in the second quarter of 2021 and anticipates reporting interim data at the end of 2021 and topline results in mid-2022.

- **Completed Phase 1b trial of ALRN-6924 in patients with SCLC receiving ALRN-6924 prior to topotecan and conducted preliminary evaluation of data from additional cohorts.** Since Aileron's late-breaking presentation of proof-of-concept data from the Phase 1b SCLC trial at the 32nd EORTC-NCI-AACR Annual Symposium on Molecular Targets and Cancer Therapeutics (ENA 2020), the company has completed the trial, including the enrollment of additional cohorts that allowed further analysis of the ALRN-6924/topotecan dosing interval and dose-response relationship. A preliminary evaluation of data 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) was in line with the data presented in 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 submit final results of the Phase 1b SCLC trial for presentation at a medical 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 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 has sold an aggregate of 13,775,399 shares of its common stock 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.

Fourth Quarter and Full Year 2020 Highlights

- **Initiated healthy volunteer study to support long-term clinical development strategy for ALRN-6924.** In November 2020, Aileron initiated a study of ALRN-6924 in healthy volunteers to gather data to support its goal to bring chemoprotection to patients with p53-mutated cancer who are undergoing chemotherapy regardless of type of cancer or chemotherapy. The ongoing study is designed to characterize the time to onset, magnitude, and duration of cell cycle arrest in human bone marrow relative to ALRN-6924 administration, with a goal of developing a universal dosing regimen for ALRN-6924 for use as a chemoprotection agent across a range of chemotherapies and p53-mutated tumor indications. Aileron plans to submit results from the healthy volunteer study for presentation at a medical conference in the second half of 2021.

- **Presented proof-of-concept data from Phase 1b SCLC trial of ALRN-6924 in late-breaking presentation at ENA 2020.** In October 2020, Aileron presented new positive clinical data from its Phase 1b SCLC trial at the ENA 2020 symposium 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 SCLC. The most robust and clinically meaningful protection against toxicities was observed with the 0.3 mg/kg dose of ALRN-6924.

Fourth Quarter and Full Year 2020 Financial Results

- **Cash Position:** Cash, cash equivalents and investments as of December 31, 2020 were $13.8 million, compared to $18.3 million as of December 31, 2019. 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 fourth quarter of 2020 were $1.9 million, compared to $4.7 million for the fourth quarter of 2019. The decrease in R&D expenses was primarily due to the completion of the company's anti-cancer trials in late 2019 and lower spend on the manufacture of ALRN-6924 and research related activities in 2020 as compared to 2019. R&D expenses for the full-year 2020 were $11.2 million, compared to $17.7 million for the prior year.

- **General and Administrative (G&A) Expenses:** G&A expenses for the fourth quarter of 2020 were $2.3 million, compared to $2.6 million for the fourth quarter of 2019. The decrease in G&A expenses was largely due to lower spending on employees and facilities in 2020 as compared to 2019. G&A expenses for the full-year 2020 were $9.3 million, compared to $12.3 million for the prior year.

- **Net Loss:** Net loss for the fourth quarter of 2020 was $4.9 million, compared to $7.2 million for the fourth quarter of 2019. Net loss for the full-year 2020 was $21.2 million, compared to a net loss of $29.4 million for the prior year. The basic and diluted net loss per share for the fourth quarter of 2020 was $0.12 compared to $0.26 for the fourth quarter of 2019. The basic and diluted net loss per share for the full-year 2020 was $0.61 compared to $1.20 for the full-year 2019.

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-K for the year ended December 31, 2020, filed on March 24, 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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[1] Now FDA approved, G1 Therapeutics' COSELA™ (trilaciclib) is intended to help protect bone marrow in patients with extensive-stage SCLC when administered prior to chemotherapy.

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

	December 31, 2020	December 31, 2019
Cash, cash equivalents and investments	$ 13,805	$ 18,278
Working capital	$ 12,366	$ 13,711
Total assets	$ 16,341	$ 26,473
Accumulated deficit	$ (219,292)	$ (198,135)
Total stockholders' equity	$ 12,162	$ 16,048

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2020	2019	2020	2019
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	1,925	4,709	11,166	17,663
General and administrative	2,267	2,639	9,330	12,293
Total Operating expenses	4,192	7,348	20,496	29,956
Loss from operations	(4,192)	(7,348)	(20,496)	(29,956)
Interest income	(738)	112	(661)	587
Net loss	(4,930)	(7,236)	(21,157)	(29,369)
Net loss per share — basic and diluted	$ (0.12)	$ (0.26)	$ (0.61)	$ (1.20)
Weighted average common shares outstanding—basic and diluted	40,997,759	27,810,358	34,866,690	24,535,454