# Aileron Therapeutics Reports Fourth Quarter and Full Year 2021 Financial Results and Provides Business Highlights

March 28, 2022

- Several anticipated catalysts in 2022 aim to advance Aileron's vision to bring chemoprotection against multiple toxicities to all patients with p53-mutated cancer regardless of type of cancer or chemotherapy
  - Expected readouts for Phase 1b trial in p53-mutated non-small cell lung cancer (NSCLC): Interim data in 2Q22; topline results in 4Q22
  - Anticipated milestones for planned Phase 1b clinical trial in p53-mutated neoadjuvant breast cancer: Initiation in 1H22; interim results in 4Q22
- Cash, cash equivalents and investments as of December 31, 2021 expected to fund operations into the fourth quarter of 2023

BOSTON, Mass., March 28, 2022 (GLOBE NEWSWIRE) -- Aileron Therapeutics (Nasdaq: ALRN), a chemoprotection oncology company that aspires to make chemotherapy safer and thereby more effective to save more patients' lives, today reported business highlights and financial results for the fourth quarter and year ended December 31, 2021.

"We're incredibly proud of the progress we've made to date advancing our mission to develop a targeted drug that prevents chemotherapy-induced side effects in patients with p53-mutated cancer," said Manuel Aivado, M.D., Ph.D., President and Chief Executive Officer of Aileron Therapeutics. "Given proof of concept of ALRN-6924's ability to protect against chemotherapy-induced bone marrow toxicities and its potential to also protect against multiple other chemotherapy-induced toxicities, our vision is to redefine care for millions of cancer patients, regardless of the type of p53-mutated cancer they have or the type of chemotherapy they receive. We look forward to several anticipated catalysts in 2022 that could further propel us toward this vision."

Aileron is developing ALRN-6924 to 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, and Aileron is pioneering a precision medicine-based approach known as selective chemoprotection to exclusively treat patients with p53-mutated cancers who are receiving chemotherapy. ALRN-6924 is designed to selectively protect these patients' healthy cells from chemotherapy without interfering with chemotherapy's effects on cancer cells. The reduction or elimination of multiple chemotherapy-induced side effects is expected to enhance tolerability of chemotherapy, which is expected to result in fewer dose reductions and delays of chemotherapy, and thus expected to improve efficacy of chemotherapy.

**2021 and Recent Highlights**

- **NSCLC clinical trial start and blinded safety evaluation.**
  - Initiated a Phase 1b randomized, double-blind, placebo-controlled clinical trial to evaluate ALRN-6924 to protect against chemotherapy-induced bone marrow toxicities in patients with p53-mutated NSCLC undergoing first-line carboplatin plus pemetrexed with or without immune checkpoint inhibitors.
  - Conducted a blinded safety evaluation of the first ten patients enrolled in the trial who completed the first cycle of treatment with ALRN-6924 and chemotherapy. The evaluation did not identify any safety concern, consistent with ALRN-6924's previously demonstrated safety and tolerability profile.
  - The Phase 1b trial is anticipated to enroll 60 patients. Patients are randomized 1:1 to receive carboplatin/pemetrexed plus 0.3 mg/kg ALRN-6924 or placebo for at least four 21-day treatment cycles.

- **Upcoming breast cancer clinical trial.** Announced plans to initiate a new clinical trial in 1H22 to evaluate ALRN-6924 against chemotherapy-induced bone marrow and other toxicities in patients with p53-mutated ER+/HER2- breast cancer treated with a doxorubicin + cyclophosphamide and docetaxel chemotherapy regimen, also known as 'AC-D'. The Phase

1b trial is anticipated to enroll up to 30 patients in a parallel group design trial with a dose expansion cohort.

- **Small cell lung cancer (SCLC) clinical trial final data presentation.** Presented final results from a completed Phase 1b trial in patients with p53-mutated SCLC receiving second-line topotecan. Following interim proof-of-concept data presented in October 2020 from this trial, the final results reinforced ALRN-6924's 'triple-play' reduction in chemotherapy-induced neutropenia, thrombocytopenia and anemia.

- **Healthy volunteer study mechanism of action (MOA) data presentation.** Presented initial data from company's ongoing Phase 1 pharmacology 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. The presented data confirmed ALRN-6924's novel p53 biomarker-driven MOA, as well as its pharmacodynamic effects, including time to onset, magnitude and duration.

- **Patent portfolio expansion.** Aileron was issued seven new international patents and four U.S. patents, including a new patent for ALRN-6924 in China. The newly issued patents support Aileron's robust intellectual property portfolio, which includes nearly 160 U.S. and foreign patents, with more than 40 additional applications in prosecution.

**2022 Anticipated Milestones**

*Ongoing NSCLC Clinical Trial*

- 2Q22: Announce results from an interim analysis of the first 20 patients enrolled in the NSCLC trial. The interim analysis will include an initial evaluation of the total number of completed treatment cycles free of hematological toxicity and free of chemotherapy or immunochemotherapy dose reductions, dose delays, use of growth factors and transfusions for the treatment arm compared to placebo. We expect these interim data will determine a refinement to the primary and secondary endpoints for the study.

- 4Q22: Announce topline results of full anticipated trial enrollment of 60 patients.

*Planned Breast Cancer Clinical Trial*

- 1H22: Initiate enrollment of patients with p53-mutated, ER+/HER2- breast cancer; provide more details on the planned neoadjuvant breast cancer trial design at time of trial initiation.

- 4Q22: Announce interim results from breast cancer trial.

*Phase 1 Pharmacology Study in Healthy Volunteers*

- 2H22: Report additional findings from healthy volunteer study.

*CMC Activities*

- Advance validation studies for ALRN-6924 drug substance and drug product manufacturing processes to support a potential future NDA filing for ALRN-6924.

**Fourth Quarter and Full Year 2021 Financial Results**

- **Cash Position:** As of December 31, 2021, cash, cash equivalents and investments were $45.9 million, compared to $13.8 million as of 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 fourth quarter of 2023.

- **Research and Development (R&D) Expenses:** R&D expenses for the fourth quarter of 2021 were $4.6 million, compared to $1.9 million for the fourth quarter of 2020. The increase in R&D expenses was primarily due to clinical activities related to our NSCLC trial and our healthy volunteer study and increased spend on CMC development for ALRN-6924 in 2021 as compared to 2020. R&D expenses for the full-year 2021 were $17.0 million, compared to $11.2 million for the prior year.

- **General and Administrative (G&A) Expenses:** G&A expenses for the fourth quarter of 2021 were $2.3 million, compared to $2.3 million for the fourth quarter of 2020. G&A expenses for the full-year 2021 were $9.6 million, compared to $9.3 million for the prior year.

- **Net Loss:** Net loss for the fourth quarter of 2021 was $6.8 million, compared to $4.9 million for the fourth quarter of 2020. Net loss for the full-year 2021 was $26.2 million, compared to a net loss of $21.2 million for the prior year. The basic and diluted net loss per share for the fourth quarter of 2021 was $0.08 compared to $0.12 for the fourth quarter of 2020. The basic and diluted net loss per share for the full-year 2021 was $0.29 compared to $0.61 for the full-year 2020.

**About Aileron Therapeutics**

Aileron is a clinical stage chemoprotection oncology company that aspires to make chemotherapy safer and thereby more effective to save more patients' lives. 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 all 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 potential of ALRN-6924 as a chemoprotective agent, 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-K for the year ended December 31, 2021, filed on March 28, 2022, 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.
Selected Balance Sheet Data
(Unaudited)
(In thousands)

| | December 31, 2021 | December 31, 2020 |
|---|---|---|
| Cash, cash equivalents and investments | $ 45,933 | $ 13,805 |
| Working capital | 43,669 | 12,366 |
| Total assets | 48,481 | 16,341 |
| Accumulated deficit | (245,456) | (219,292) |
| Total stockholders' equity | $ 43,904 | $ 12,162 |

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

| | Three Months Ended | Year Ended |
|---|---|---|

| | December 31, | | December 31, | |
|---|---|---|---|---|
| | 2021 | 2020 | 2021 | 2020 |
| Revenue | $ - | $ - | $ - | $ - |
| Operating expenses: | | | | |
| Research and development | 4,561 | 1,925 | 17,008 | 11,166 |
| General and administrative | 2,255 | 2,267 | 9,597 | 9,330 |
| Total Operating expenses | 6,816 | 4,192 | 26,605 | 20,496 |
| Loss from operations | (6,816) | (4,192) | (26,605) | (20,496) |
| Other income (expense), net | 17 | (738) | 441 | (661) |
| Net loss | (6,799) | (4,930) | (26,164) | (21,157) |
| Net loss per share — basic and diluted | $ (0.08) | $ (0.12) | $ (0.29) | $ (0.61) |
| Weighted average common shares outstanding—basic and diluted | 90,573,552 | 40,997,759 | 88,806,763 | 34,866,690 |