Aileron Therapeutics Reports Second Quarter 2020 Financial Results and Provides Business Update

August 5, 2020

- Company continues to develop ALRN-6924 as novel chemoprotective medicine to protect patients from chemotherapy-induced toxicities and side effects
  -- Positive interim dose optimization data from proof-of-concept Phase 1b study, reported in second quarter 2020
- Additional second quarter and recent highlights from Phase 1b proof-of-concept study include:
  -- Completed enrollment in dose optimization expansion cohort
  -- Initiated enrollment in schedule optimization part of the study
- Final dose optimization data and preliminary schedule optimization data anticipated in fourth quarter 2020
- Received $10.3 million in aggregate net proceeds in June 2020 from public offering of common stock

WATERTOWN, Mass., Aug. 05, 2020 (GLOBE NEWSWIRE) -- Aileron Therapeutics (NASDAQ:ALRN) today reported business highlights and financial results for the second quarter ended June 30, 2020.

“We continue to make important progress advancing our goal to deliver a novel chemoprotective medicine that can protect cancer patients against multiple serious, often life-threatening chemotherapy-induced side effects,” said Manuel Aivado, M.D., Ph.D., President and Chief Executive Officer of Aileron. “We’re pleased with the preliminary data we reported in June from the dose optimization part of our ongoing Phase 1b proof-of-concept clinical trial of ALRN-6924 to protect against chemotherapy-induced bone marrow toxicities in small cell lung cancer patients undergoing treatment with topotecan. These data showed clinically meaningful protection against severe chemotherapy-induced anemia and thrombocytopenia. In addition, a signal of protection against Grade 4 neutropenia in the first treatment cycle was observed at the 0.3 mg/kg dose level.”

Dr. Aivado further commented, “Key inflection points for Aileron in the coming months include final Phase 1b dose optimization data in addition to preliminary data from the recently initiated schedule optimization part of the trial, both in the fourth quarter, as well as the initiation of our healthy volunteer study of ALRN-6924 in the third quarter. These milestones will further support and de-risk our plans to expand development of ALRN-6924 for multiple cancer types and various chemotherapies, to ultimately deliver this unique chemoprotective medicine to as many patients with p53-mutated cancers as possible, which could translate to approximately half of all cancer patients.”

Key Second Quarter and Recent Highlights

Ongoing ALRN-6924 Proof-of-Concept Phase 1b Study

Part one of study: dose optimization

- **Announced positive interim dose optimization results.** In June 2020, Aileron announced positive interim data from the open-label dose optimization part of its ongoing Phase 1b clinical study of ALRN-6924. The study is evaluating ALRN-6924 as a therapeutic agent administered ahead of chemotherapy to protect against chemotherapy-induced bone marrow toxicities in patients with p53-mutated small cell lung cancer (SCLC) who are being treated with topotecan.

  In the dose optimization part of the study, ALRN-6924 was administered at three dose levels (0.3, 0.6, and 1.2 mg/kg) 24 hours before each dose of topotecan. Topotecan was administered days 1 through 5 every 21 days.

  As reported in June, treatment with ALRN-6924 resulted in a protective effect against severe chemotherapy-induced anemia and thrombocytopenia compared to historical controls. In addition, patients treated with 0.3 mg/kg ALRN-6924 met the protocol-defined criterion for reduction of NCI CTC Grade 3/4 neutropenia to ≤50% in the first treatment cycle, triggering a cohort expansion at this dose level, from six to 14 patients.

- **Completed enrollment in dose optimization expansion cohort.** Aileron recently announced completion of enrollment into the 0.3 mg/kg dose level cohort expansion, to reach a total of 14 patients treated at this dose and the 24-hour before topotecan schedule

Part 2 of study: schedule optimization

Initiated enrollment in schedule optimization part of study. In June 2020, Aileron announced that it began enrollment in the schedule optimization part of its ongoing Phase 1b clinical study. This part of the study is intended to determine whether ALRN-6924 given six hours before topotecan further enhances the protective effects observed when ALRN-6924 was given 24 hours before topotecan in the dose optimization part of the trial, as described above. Aileron plans to report final dose optimization data, including data from the dose optimization expansion cohort, in the fourth quarter of 2020. In addition, Aileron also plans to report preliminary schedule optimization data in the fourth quarter of 2020. Aileron will continue to carefully monitor the effect of the coronavirus pandemic on its clinical trial sites and the healthcare system, which may impact its planned data announcements.

Planned Healthy Volunteer Study

In the third quarter of 2020, Aileron plans to initiate enrollment in a healthy volunteer study to determine dosing schedules for ALRN-6924 that will support and further de-risk the company's long-term vision to provide a chemoprotective medicine for patients with p53-mutated cancer regardless of cancer type or chemotherapeutic drug.

Corporate

- **Completed public offering of common stock.** In June 2020, Aileron completed a public offering of common stock, priced at a public offering price of $1.10 per share, raising $10.3 million in aggregate net proceeds, including shares sold to the underwriter pursuant to its exercise of its option to purchase additional shares and after deducting underwriting discounts and commissions and offering expenses.

Second Quarter 2020 Financial Results

- **Cash Position:** Cash, cash equivalents and investments as of June 30, 2020 were $18.9 million, compared to $18.3 million as of December 31, 2019.

- **Research and Development Expenses:** Research and development expenses for the quarter ended June 30, 2020 were $2.5 million, compared to $4.3 million for the quarter ended June 30, 2019. The decrease is primarily a result of a $1.2 million decrease in clinical development expenses attributed to the completion of patient dosing in our Phase 2a expansion cohort of the combination of ALRN-6924 and IBRANCE® (palbociclib) for the treatment of MDM2-amplified advanced solid tumors during the first quarter of 2020 and the effect of cost-saving measures implemented in 2019 and 2020.

- **General and Administrative Expenses:** General and administrative expenses were $1.9 million for the quarter ended June 30, 2020, compared to $3.1 million for quarter ended June 30, 2019. The decreased expense in 2020 primarily reflects cost-saving initiatives that were implemented in March 2020 and lower administrative support-related costs in 2020 as compared to 2019.

- **Net Loss:** Net loss was $4.4 million for the quarter ended June 30, 2020, compared to $7.2 million for the corresponding period in 2019.

How ALRN-6924 Works to Protect Healthy Cells from Chemotherapy-Induced Damage

ALRN-6924 is being developed by Aileron as a novel chemoprotective medicine to treat and protect healthy cells in patients with cancer that harbors p53-mutations to reduce or eliminate chemotherapy-induced side effects.

Chemotherapy targets cells that are cycling, or undergoing the process of cell division. In cancer cells, the cell cycle is unchecked, which leads to uncontrolled cell proliferation, a hallmark of cancer. Certain types of healthy cells also naturally need to cycle, such as bone marrow cells (which give rise to red blood cells, white blood cells, and platelets), hair follicle cells, skin cells, and cells lining the oral cavity and the gastrointestinal tract. As a result, chemotherapy targets and kills both cycling healthy cells and cycling cancer cells. This, in turn, leads to a spectrum of chemotherapy-induced side effects, from unpleasant to life-threatening.

ALRN-6924, an investigational first-in-class MDM2/MDMX dual inhibitor, is administered to cancer patients shortly before chemotherapy. ALRN-6924 is designed to selectively activate normal p53 protein in patients' healthy cells, temporarily and reversibly pausing cell cycling to shield healthy cells from chemotherapy. The protection is limited to healthy cells, as ALRN-6924 cannot work in p53-mutated cancer cells given that p53 has lost function in those cells. Therefore, cancer cells continue to cycle uninterrupted, remaining fully susceptible to destruction by chemotherapy.

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. We are advancing ALRN-6924, our first-in-class dual MDM2/MDMX inhibitor currently in clinical development, to provide a single medicine to protect multiple healthy cell types throughout the body from chemotherapy while ensuring 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 provide chemoprotection for patients with p53-mutated cancers, which represents approximately 50% of cancer patients, regardless of cancer type or chemotherapeutic drug. 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 and/or trials anticipated; whether results obtained in preclinical and nonclinical studies and clinical trials will be indicative of results obtained in future clinical trials; whether preliminary or interim results from a clinical trial such as the interim data referenced in this release will be indicative of the final results of the trial; 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 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; whether the coronavirus pandemic will have an impact 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 June 30, 2020, filed on August 5, 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.

Investor Contacts:
Aileron Therapeutics
Richard Wanstall, SVP Chief Financial Officer
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.
Balance Sheet Data
(In thousands)

	June 30, 2020	December 31, 2019
Cash, cash equivalents and investments	$ 18,880	$ 18,278
Working capital	$ 14,744	$ 13,711
Total assets	$ 26,125	$ 26,473
Accumulated deficit	$ (209,273)	$ (198,135)
Total stockholders' equity	$ 16,654	$ 16,048

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,	
	2020	2019	2020	2019
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	2,488	4,304	6,557	8,478
General and administrative	1,912	3,075	4,719	6,214

Total Operating expenses	4,400	7,379	11,276	14,692
Loss from operations	(4,400)	(7,379)	(11,276)	(14,692)
Gain on sale of property and equipment	-	-	66	-
Interest income	10	207	72	307
Net loss	(4,390)	(7,172)	(11,138)	(14,385)
Net loss per share — basic and diluted	$ (0.14)	$ (0.26)	$ (0.38)	$ (0.68)
Weighted average common shares outstanding—basic and diluted	31,221,139	27,526,065	29,515,749	21,206,269