Aileron Therapeutics Reports Fourth Quarter and Full Year 2019 Financial Results

March 30, 2020

Phase 1b/2 clinical trial of ALRN-6924 as a myelopreservation agent for the protection against chemotherapy-induced toxicity in small cell lung cancer started dosing patients September 2019; interim data suggests reduction in severe anemia and thrombocytopenia; encouraging results led to the plan to test second dosing schedule to assess if effects can be extended to reduction in severe neutropenia

Due to uncertainty and enrollment delays associated with the coronavirus pandemic, efforts are now focused solely on the development of ALRN-6924 as a myelopreservation agent; the company is planning to implement measures to reduce operating expenses to extend its cash runway to first quarter 2021

WATERTOWN, Mass., March 30, 2020 (GLOBE NEWSWIRE) -- Aileron Therapeutics (NASDAQ:ALRN), the clinical-stage leader in the field of stabilized, cell-permeating peptides, today reported business highlights and financial results for the fourth quarter and full year ended December 31, 2019.

"We continue to advance our lead clinical program evaluating ALRN-6924 as an agent that protects patients with p53-mutant cancers against chemotherapy-induced toxicity, which is a novel treatment concept known as 'myelopreservation'. The dose-optimization Phase 1b portion of the Phase 1b/2 clinical trial started dosing small cell lung cancer (SCLC) patients in September 2019. After observing encouraging interim data from the initial cohorts showing a protective effect against severe anemia and thrombocytopenia, we decided to test a second dosing schedule to assess whether the myelopreservation effect may also be extended to include severe neutropenia. However, enrollment has recently been slowed by the coronavirus pandemic. Subject to potential further delays from the global impact of this pandemic, we plan to present proof of concept data from the Phase 1b portion of the trial in the fourth quarter of 2020," said Dr. Manuel Aivado, President and CEO of Aileron Therapeutics. "In light of our cash resources and the uncertainties associated with the coronavirus pandemic, we have decided to focus our efforts solely on the development of ALRN-6924 as a myelopreservation agent and plan to reduce our operating expenses in order to extend our cash runway from the fourth quarter of 2020 to the first quarter of 2021."

ALRN-6924 as a myelopreservation agent

In September 2019, the Company initiated the Phase 1b portion of the Phase 1b/2 clinical trial evaluating ALRN-6924 as a myelopreservation agent in patients with SCLC, being treated with the chemotherapy topotecan. The phase 1b portion of the trial is designed to identify a recommended phase 2 dose and schedule for ALRN-6924 and to evaluate the safety and efficacy of ALRN-6924 when administered to protect SCLC patients from toxicities caused by treatment with topotecan. The Phase 1b portion is expected to enroll approximately 40 patients with tumors harboring p53-mutations; this biomarker-based patient selection strategy is designed to achieve selective protection of normal tissues from the effects of chemotherapy. The Company currently plans to report proof of concept data from the Phase 1b portion of the trial in the fourth quarter of 2020. However, the effect of the coronavirus pandemic on the healthcare system may continue to delay enrollment and delay these data.

While still preliminary, the Company is encouraged by the interim results of a data cutoff on January 22, 2020. In the first eleven patients from the first two patient cohorts, a protective effect of ALRN-6924 was observed for severe thrombocytopenia and anemia. Based on the preliminary data from the Phase 1b trial, and historical rates of these toxicities in SCLC patients treated with topotecan Aileron believes that ALRN-6924, when dosed before chemotherapy may have a myelopreservation effect on severe thrombocytopenia and anemia. Enrollment is currently proceeding with a third patient cohort of ALRN-6924. Aileron is encouraged with the data that is emerging from this cohort as it supports the trends observed with the previous two cohorts.

In the first three cohorts of the trial, ALRN-6924 was administered 24 hours before administration of topotecan. The Company plans to assess a second dosing schedule of ALRN-6924 administered six hours before administration of topotecan in the ongoing clinical trial. The objective of the second dosing schedule is to determine if ALRN-6924 dosed six hours before topotecan will achieve protection from severe neutropenia in addition to the protection from severe anemia and thrombocytopenia observed in the first dosing schedule.

Clinical trial of ALRN-6924 in combination with Pfizer's palbociclib

As of March 15, 2020, seven out of 34 patients remained on treatment, and the Company anticipates the final PFS analysis and presentation of the results from this Phase 2a trial in the second half of 2020. The Company believes that there is a commercial opportunity to bring this drug combination to patients with MDM2-amplified solid tumors. However, in light of its cash resources and the uncertainties associated with the coronavirus pandemic,

Aileron has determined to suspend further development of the combination of ALRN-6924 and palbociclib, as it focuses its R&D efforts solely on the development of ALRN-6924 as a myelopreservation agent.

Impact of the coronavirus pandemic on our corporate strategy

The recent coronavirus pandemic has created substantial uncertainties in the United States and throughout the world, including in the financial markets and in the biopharmaceutical industry. In response, Aileron is in the process of implementing a plan to reduce operating expenses, which may include a reduction in personnel as well as the elimination of previously planned research studies and advisory services. The Company has also determined to delay the initiation of two previously planned cohorts in the development of ALRN-6924 for myelopreservation. However, the Company's plans to continue its Phase 1b trial and identify a recommended phase 2 dose for myelopreservation in SCLC is unaffected by this cash preservation plan. The decision to initiate the delayed myelopreservation cohorts will be subject to obtaining sufficient additional funding and contingent on assessing the full impact of the coronavirus pandemic to the Company.

Fourth Quarter and Full Year 2019 Financial Results

Cash Position: Cash, cash equivalents and investments as of December 31, 2019 were $18.3 million. The Company believes that its cash, cash equivalents and investments as of December 31, 2019 along with the impact of its plan to reduce operating expenses, will be sufficient to fund its operations and capital expenditure requirements into the first quarter of 2021.

R&D Expenses:

Research and development expenses for the three months ended December 31, 2019 were $4.7 million, compared to $4.0 million for the three months ended December 31, 2018. The increase of $0.7 million was primarily due to costs incurred of $0.8 million for the manufacture of ALRN-6924 to support the myelopreservation clinical program, and increased clinical development cost of $0.7 million, partially offset by lower headcount-related costs of $0.5 million and lower spending on research of $0.2 million.

Research and development expenses for the year ended December 31, 2019 were $17.7 million, compared to $18.4 million for the year ended December 31, 2018. The decrease of $0.8 million was primarily due to a $1.6 million decrease in employee, facility, and other development expenses in 2019 when compared to 2018. Research and development headcount was seven on December 31, 2019 compared to sixteen on December 31, 2018. Lower employee cost was partially offset by an increase in clinical trial costs of ALRN-6924 associated with the Phase 2a expansion cohort of the combination of ALRN-6924 and palbociclib (Ibrance), for the treatment of MDM2-amplified advanced solid tumors and costs incurred for the Phase 1b clinical trial to evaluate ALRN-6924 as a myelopreservation agent.

G&A Expenses:

General and administrative expenses were $2.6 million for the three months ended December 31, 2019, compared to $3.0 million for the three months ended December 31, 2018. The decrease in general and administrative expense is attributed to lower legal support fees and decreased consulting fees.

General and administrative expenses were $12.3 million for the year ended December 31, 2019, compared to $13.5 million for the year ended December 31, 2018. General and administrative expenses in 2018 include $1.1 million in expense related to a separation agreement with the Company’s former chief executive officer. Of this $1.1 million, approximately $0.5 million related to salary continuation payments to the former chief executive officer and $0.6 million resulted from modifications to his stock options in 2018. Legal expenses also decreased in 2019 as compared to 2018. This decrease was partially offset by an increase in business development related costs and facilities costs in 2019 when compared to 2018.

Net Loss: Net loss was $7.2 million for the three months ended December 31, 2019, compared to $7.0 million for the same period in 2018; and $29.4 million for the year ended December 31, 2019, compared to $30.4 million for the year ended December 31, 2018.

Shares Outstanding: As of December 31, 2019, there were 27.8 million shares of common stock outstanding.

Non-GAAP net loss: Non-GAAP net loss and Non-GAAP net loss per share for the twelve months ended September 30, 2018 excludes costs incurred under the 2018 separation agreement with our former chief executive officer. There were no Non-GAAP adjustments in the three or twelve months ended December 31, 2019.

A reconciliation of GAAP to non-GAAP financial measures has been provided in the table included below in this press release. An explanation of these measures is also included below under the heading “Non-GAAP Financial Measures.”

About ALRN-6924
ALRN-6924 is a first-in-class dual MDM2/MDMX inhibitor that is currently being evaluated in a Phase 1b/2 clinical trial to evaluate ALRN-6924 as a myelopreservation agent to protect against chemotherapy-related toxicities and as an anti-cancer agent in a Phase 2a clinical trial in combination with Pfizer’s palbociclib (Ibrance®) for the treatment of MDM2-amplified advanced solid tumors.

About Aileron
Aileron is a clinical-stage biopharmaceutical company advancing a proprietary platform of cell-permeating alpha-helical peptides. The stabilized helical structure of our peptides allows the design of cell-permeating therapeutic agents with large molecular surfaces for optimal target binding properties, resulting in drug candidates like ALRN-6924. Our current focus is to improve the standard of care for patients with cancer by developing safe and effective therapies that leverage our proprietary peptide platform. For more information, visit www.aileronrx.com, and for more information about our clinical trials please visit www.clinicaltrials.gov.

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 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 and our operations; and other factors discussed in the "Risk Factors" section of Aileron's quarterly report on Form 10-K for the period ended December 31, 2019, filed on March 30, 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.

Reconciliation of Non-GAAP Financial Measures to GAAP Financial Measures
Aileron Therapeutics, Inc.
(In thousands, except per share data)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2019	2018	2019	2018
Numerator:				
GAAP net loss	$ (7,235)	$ (7,034)	$ (29,369)	$ (31,547)
Stock based compensation charge related to CEO separation agreement				612
Salary continuation charge related to CEO separation agreement				564
Non-GAAP net loss	$ (7,235)	$ (7,034)	$ (29,369)	$ (30,371)
Denominator				
GAAP weighted average common shares outstanding —basic and diluted	27,810,358	14,745,707	24,535,454	14,738,193
GAAP net loss per share —basic and diluted	$ (0.26)	$ (0.48)	$ (1.20)	$ (2.14)
Non-GAAP net loss per share —basic and diluted	$ (0.26)	$ (0.48)	$ (1.20)	$ (2.06)

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Source: Aileron Therapeutics, Inc.