



Aileron Therapeutics Reports Third Quarter 2019 Results

November 7, 2019

Announced positive interim results from its ongoing Phase 2a clinical trial evaluating the combination of ALRN-6924 and Pfizer's IBRANCE® (palbociclib) for the treatment of patients with tumors harboring wild-type p53 and MDM2 amplification or MDM2/CDK4 co-amplification

Presented new nonclinical data showing ALRN-6924 reduced chemotherapy-related toxicities ("myelopreservation") in cellular studies and mouse models of cancer without limiting – and even enhancing – anti-cancer efficacy

Initiated patient treatment in its first clinical Phase 1b/2 trial of ALRN-6924 as a myelopreservation agent

WATERTOWN, Mass., Nov. 07, 2019 (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 third quarter ended September 30, 2019.

"We have generated positive data over the last few months for ALRN-6924, namely positive interim clinical trial results from our palbociclib combination therapy trial in MDM2-amplified cancers and compelling nonclinical data for our 2nd key program using ALRN-6924 as a myelopreservation agent," said Dr. Manuel Aivado, President and CEO of Aileron Therapeutics.

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

"In late September, we were pleased to announce positive interim results from our Phase 2a expansion cohort of ALRN-6924 in combination with palbociclib, which showed a median progression-free survival of 4.4 months in 17 patients with previously treated advanced MDM2-amplified liposarcoma," continued Dr. Aivado "There is a high unmet medical need for those patients as evidenced by a median progression-free survival of 3 months or less for FDA-approved drugs for the treatment of 2nd or 3rd line liposarcoma, such as trabectedin and eribulin, respectively. Although palbociclib monotherapy is not approved for the treatment of liposarcoma, it is recommended by the National Cancer Center Network guidelines as a treatment option. However, the reported progression-free survival rate for palbociclib monotherapy at 12 weeks was only 57%. Encouragingly, our combination therapy achieved a more favorable progression-free survival rate of 73% at 12 weeks in liposarcoma, despite the fact that our combination therapy was evaluated in a more pretreated patient population. These interim results have further encouraged us to expand enrollment from 26 to 35 patients, focusing on indications outside of liposarcoma to determine the broader applicability of the combination. These results have also helped drive enrollment for the trial, and we are on track to announce the final data readout in the second quarter of 2020."

ALRN-6924 as a myelopreservation agent

"We are also excited by the recent advancement of our myelopreservation development program for ALRN-6924, including the initiation of the dose-optimization Phase 1b portion of the Phase 1b/2 clinical trial, and we expect to present key findings from this trial in the second quarter of 2020. We are extremely pleased with our recent nonclinical data presented at the AACR-NCI-EORTC conference last month that showed ALRN-6924 reduced chemotherapy-related toxicities in cellular studies and mouse models of cancer without limiting – and even enhancing – anti-cancer efficacy, and we now plan to reallocate resources from our early-stage pipeline program to the clinical development of ALRN-6924 as a myelopreservation agent. Specifically, we expect to expand our myelopreservation program by adding a Phase 1b cohort in non-small cell lung cancer patients treated with docetaxel, and a randomized expansion cohort of the Phase 1b small cell lung cancer trial to treat patients with alternating cycles of chemotherapy with and without ALRN-6924," concluded Dr. Aivado.

Third Quarter 2019 Financial Results

- **Cash Position:** Cash, cash equivalents and investments as of September 30, 2019 were \$24.6 million, compared to \$20.7 million as of December 31, 2018. The Company believes that its cash, cash equivalents and investments as of September 30, 2019 will be sufficient to fund its operations and capital expenditure requirements into the fourth quarter of 2020.
- **R&D Expenses:** Research and development (R&D) expenses were \$4.5 million for the three months ended September 30, 2019 compared to \$4.3 million for the same period in 2018. The increase in R&D expense was primarily driven by clinical development of ALRN-6924 and partially offset by decreases to employee-related expenses.

Clinical development of ALRN-6924 during the three months ended September 30, 2019 primarily reflects costs associated with the Phase 2a expansion cohort of the combination of ALRN-6924 and palbociclib (Ibrance), for the treatment of MDM2-amplified advanced cancers and costs incurred in preparation for our Phase 1b/2 clinical trial to evaluate ALRN-6924 as a myelopreservation agent. Clinical development of ALRN-6924 during the three months ended September 30, 2018 primarily reflected costs incurred in connection with our PTCL trial, which we completed, and our AML/MDS trial, which we discontinued, in early 2019.

- **G&A Expenses:** General and administrative (G&A) expenses were \$3.4 million in the three months ended September 30, 2019, compared to \$3.2 million for the same period in 2018. The increase in G&A was primarily due to costs related to increased business development efforts.
- **Net Loss:** The Company reported a net loss of \$7.7 million or \$0.28 per share in the three months ended September 30, 2019 compared to \$7.4 million or \$0.50 per share for the same period in 2018. The Company's weighted average shares outstanding were 27.8 million and 14.7 million for the three months ended September 30, 2019 and 2018, respectively.
- **Shares Outstanding:** As of September 30, 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 nine 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 nine months ended September 30, 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."

Conference Call

A conference call to discuss these results has been scheduled for Thursday, November 7, 2019 at 8:30 a.m. EST. To access the conference call, investors are invited to dial 877-876-9176 (U.S. and Canada) or 785-424-1670 (international). The conference ID is "AILERON". A live audio webcast can be accessed by visiting the investor relations section of the Company's website, www.aileronrx.com. A replay of the webcast will be archived on Aileron's website for 90 days following the event.

About ALRN-6924

ALRN-6924 is a first-in-class dual MDM2/MDMX inhibitor that is currently being evaluated 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 cancers, and in a Phase 1b/2 clinical trial to evaluate ALRN-6924 as a myelopreservative agent to protect against chemotherapy-related toxicities.

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 and cancer supportive care treatments 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, including the additional planned myelopreservation trials; 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; and other factors discussed in the "Risk Factors" section of Aileron's quarterly report on Form 10-Q for the period ended September 30, 2019, filed on November 7, 2019, 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.

Non-GAAP Financial Measures

We report all financial information required in accordance with U.S. generally accepted accounting principles (GAAP). To supplement our unaudited condensed financial statements presented in accordance with GAAP, we use certain non-GAAP measures of financial performance. The presentation of these non-GAAP financial measures is not intended to be considered in isolation from, as a substitute for, or superior to, the financial information prepared and presented in accordance with GAAP and may be different from non-GAAP financial measures used by other companies. We use non-GAAP net loss to calculate non-GAAP net loss per share. This non-GAAP financial measure reflects charges incurred in connection with the separation agreement with our former Chief Executive Officer in 2018.

For a reconciliation of non-GAAP financial measures to the most directly comparable GAAP financial measures, please see the accompanying table titled "Reconciliation of Non-GAAP Financial Measures to GAAP Financial Measures."

We believe that these non-GAAP financial measures, when taken together with the corresponding GAAP financial measures, provide meaningful

supplemental information regarding our results. Management uses and believes that investors benefit from referring to these non-GAAP financial measures in assessing our operating results, as well as when planning, forecasting and analyzing future periods. For the nine months ended September 30, 2018, we reduced our net loss by the amount of charges incurred in connection with a separation agreement with our former Chief Executive Officer to calculate our non-GAAP net loss per share. We believe the quantification of these items will enable investors to more clearly understand the nature of our current expenses and increase the comparability of them to prior periods.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Numerator:				
GAAP net loss	\$ (7,749) \$ (7,434) \$ (22,134) \$ (24,513
Stock based compensation charge related to CEO separation agreement				612
Salary continuation charge related to CEO separation agreement				564
Non-GAAP net loss	\$ (7,749) \$ (7,434) \$ (22,134) \$ (23,337
Denominator				
GAAP weighted average common shares outstanding —basic and diluted	27,810,358	14,737,402	23,431,823	14,735,660
GAAP net loss per share —basic and diluted	\$ (0.28) \$ (0.50) \$ (0.95) \$ (1.66
Non-GAAP net loss per share —basic and diluted	\$ (0.28) \$ (0.50) \$ (0.95) \$ (1.58

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Source: Aileron Therapeutics, Inc.