Aileron Therapeutics Reports Third Quarter 2018 Financial Results

November 7, 2018

- *Manuel Aivado, MD, PhD, promoted to President and Chief Executive Officer*
- *Vojislav Vukovic, MD, appointed Senior Vice President, Chief Medical Officer, and Allen Annis, PhD, promoted to Senior Vice President, Research*
- *Interim ALRN-6924 clinical data for PTCL, AML, and MDS patients to be presented at ASH in December 2018*
- *Preclinical data for ALRN-6924 to be presented at SITC and EORTC/NCI/AACR conferences in November 2018*
- *Initiation of clinical trials of ALRN-6924 in combination with approved anti-cancer drugs planned over the next three to six months*

WATERTOWN, Mass., Nov. 07, 2018 (GLOBE NEWSWIRE) -- Aileron Therapeutics (NASDAQ:ALRN), the clinical-stage leader in the field of stabilized, cell-permeating peptides to treat cancer and other diseases, today reported business highlights and financial results for the third quarter ended September 30, 2018. Aileron's lead development product ALRN-6924 is a first-in-class, stabilized alpha-helical peptide that mimics the p53 tumor suppressor protein to disrupt its interactions with both its endogenous inhibitors, MDMX and MDM2. For p53 wild-type tumors, ALRN-6924 can restore p53-dependent tumor suppression.

"The new executive team is taking the helm at an important time for the organization and positions Aileron well to capitalize on the opportunity represented by our technology platform and ALRN-6924, our lead development product," said Manuel Aivado, President and CEO of Aileron. "Over the next several weeks, Aileron is scheduled to present new nonclinical data, as well as new clinical results from its phase 2a PTCL trial and phase 1 and 1b trials in AML and MDS at several conferences, including SITC, EORTC and ASH. We look forward to sharing these results, which we believe support our enthusiasm for ALRN-6924 and continued investment in the development of these programs," concluded Dr. Aivado.

ALRN-6924 Program Updates

- **Phase 2a Expansion Cohorts with ALRN-6924 in Peripheral T-Cell Lymphoma**
  Aileron is conducting a Phase 2a expansion of ALRN-6924 as a monotherapy in cohorts of patients with relapsed or refractory peripheral T-cell lymphoma (PTCL). Previous interim data from an expansion cohort testing a once-weekly dosing schedule (days 1, 8, and 15 every 28 days) showed response rates of approximately 25% in these patients with relapsed or refractory PTCL. Updated interim results from both the once-weekly cohort and a three-times-weekly cohort (days 1, 3, and 5 every 21 days) will be presented at the 60th Annual Meeting of the American Society of Hematology (ASH) in December 2018.

- **Phase 1 and 1b Trials with ALRN-6924 in AML and MDS**
  Aileron is conducting Phase 1 and 1b open-label, multi-center dose-escalation trials of ALRN-6924 as a monotherapy and in combination with cytosine arabinoside (Ara-C) for the treatment of patients with relapsed or refractory acute myeloid leukemia (AML) and patients with myelodysplastic syndrome (MDS) who failed hypomethylating agents. In the monotherapy arm, patients receive ALRN-6924 once per week for three weeks every 28 days or three times per week for two weeks, every 21 days. In the combination arm, MDS patients receive ALRN-6924 plus low-dose Ara-C on days 1, 8, and 15 every 28 days. Based on the data in dose-escalation, we have initiated an expansion cohort in patients with advanced MDS who are relapsed or refractory to hypomethylating agents. In this expansion cohort, we are testing a combination of ALRN-6924 at a dose of 4.4 mg/kg and Ara-C at a dose of 200 mg/m^2. The company will report interim data from these trials at the ASH meeting in December 2018.

- **Initiation of New ALRN-6924 Clinical Trials**
  We have recently announced a collaboration with the Dana-Farber Cancer Institute to support an investigator-initiated

open-label, multi-center Phase 1 clinical trial in which they will enroll pediatric patients with acute leukemia and solid tumors. In this trial, pediatric patients with solid tumors will receive ALRN-6924 as a single agent, while pediatric patients with acute leukemia will be treated with a combination of ALRN-6924 and Ara-C. We expect that a third cohort of this trial will be biomarker-enriched with the intent of improving response rates with this precision-medicine approach.

Over the next six months, and subject to obtaining the necessary funding, we also plan to initiate clinical trials or support investigator-initiated trials evaluating ALRN-6924 in combination with approved therapies, including one or more trials testing combinations of ALRN-6924 with targeted therapies such as cyclin-dependent-kinase 4/6 inhibitors, and one or more trials testing ALRN-6924 in combination with chemotherapies.

- **Preclinical Research Testing ALRN-6924 as Combination Therapy**
  Aileron has recently completed preclinical testing of ALRN-6924 as combination therapy with a variety of approved drugs. The results will be presented at two upcoming conferences this month, including the Society for Immunotherapy of Cancer (SITC) Annual Meeting in Washington, D.C. on November 9th and 10th and the 2018 EORTC/NCI/AACR Molecular Targets and Cancer Therapeutics Symposium in Dublin, Ireland on November 16th.

  Aileron is scheduled to present a poster titled “The stapled peptide ALRN-6924, a dual inhibitor of MDMX and MDM2, displays immunomodulatory activity and enhances immune checkpoint blockade in syngeneic mouse models” at SITC.

  Additionally, the Company is scheduled to present a poster titled “Harnessing the anticancer activity of the stapled peptide ALRN-6924, a dual inhibitor of MDMX and MDM2, using rational combination strategies for breast cancer and other malignancies” at the EORTC/NCI/AACR symposium.

- **Pipeline Research**
  Aileron is performing additional discovery work to demonstrate that its proprietary cell-permeating peptide technology is well-suited to address heretofore undruggable targets, within and outside of the therapeutic area of oncology. The company is committed to expanding the potential of its cell-permeating peptide technology.

Corporate Update

- **Appointed Dr. Aivado as CEO**
  Manuel Aivado, MD, PhD, was named President and Chief Executive Officer and elected to Aileron’s Board of Directors in September 2018. Dr. Aivado previously served as Aileron’s Senior Vice President and Chief Medical and Scientific Officer since 2014. He succeeds John P. Longenecker, PhD, who was appointed interim CEO on May 15, 2018.

- **Aileron Strengthens Management Team**
  Vojislav (Vojo) Vukovic, MD, PhD, has been named Chief Medical Officer, succeeding Dr. Aivado. Dr. Vukovic’s prior experience includes CMO roles at Taiho Oncology and Synta Pharmaceuticals, and the Medical Director role at Pfizer. In addition to leading key clinical development programs, Dr. Vukovic has also led translational research components within his studies, including biomarker discovery and validation. Additionally, Aileron has promoted Allen Annis, PhD, to Senior Vice President, Research. Dr. Annis has led Aileron’s internal and collaborative research activities for many years, and his work has been instrumental in the clinical development of ALRN-6924. Dr. Annis received his PhD from Harvard University and has over 20 years of experience in the pharmaceutical industry.

Third Quarter 2018 Financial Results

Cash Position: Cash, cash equivalents and investments as of September 30, 2018 were $27.9 million, compared to $50.8 million as of December 31, 2017.

R&D Expenses: Research and development (R&D) expenses were $4.3 million for the three months ended September 30, 2018, compared to $3.8 million for the same period in 2017. The Company reported R&D expenses of $14.5 million for the nine months ended September 30, 2018 compared to $9.9 million for the same period in 2017. The increase in R&D expense for both the three and nine months ended September 30, 2018 was primarily driven by increased activity in the Company’s non-clinical research and increases in clinical personnel expense.

G&A Expenses: General and administrative (G&A) expenses were $3.2 million in the three months ended September 30, 2018, compared to $2.6 million for the same period in 2017. The Company reported G&A expenses of $10.4 million for the nine months ended September 30, 2018 compared to $6.0 million for the same period in 2017. The increase in G&A expenses for both the three and nine-month periods ended September 30, 2018 was primarily due to increases in personnel related costs, higher legal fees in connection with the Company’s facility relocation in third quarter 2018, increased insurance cost associated with being a public company, and increased non-cash stock compensation costs. Further, approximately $1.1 million of the increased expense during the nine months ended September 30, 2018 was due to charges in connection with a separation agreement with the Company’s former Chief Executive Officer. Of this $1.1 million charge, approximately $0.5 million is a salary continuation charge and $0.6 million resulted from a non-cash charge for stock option modifications.

Net Loss: The Company reported a net loss attributable to common stockholders of $7.4 million in the three months ended September 30, 2018 compared to $6.3 million for the same period in 2017. The Company reported a net loss of $24.5 million and $15.8 million for the nine month periods

ended September 30, 2018 and 2017, respectively.

Based on the Company's weighted average shares outstanding, the Company reported a net loss attributable to common stockholders of $0.50 per share in the three months ended September 30, 2018, compared to $0.45 per share for the same period in 2017, and a net loss of $1.66 per share for the nine months ended September 30, 2018 compared to $3.16 per share for the same period in 2017.

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."

Shares Outstanding: As of September 30, 2018, there were 14.7 million shares of common stock outstanding.

About ALRN-6924

ALRN-6924 is a first-in-class, stabilized cell-permeating alpha-helical peptide that mimics the p53 tumor suppressor protein to disrupt its interactions with both its endogenous inhibitors, MDMX and MDM2. For p53 wild-type tumors, ALRN-6924 can restore p53-dependent tumor suppression. ALRN-6924 is currently being evaluated in multiple clinical trials for the treatment of solid and hematological cancers, including acute myeloid leukemia (AML), myelodysplastic syndrome (MDS), and peripheral T-cell lymphoma (PTCL). For information about Aileron's clinical trials, please visit www.clinicaltrials.gov.

About Aileron

Aileron is a clinical-stage biopharmaceutical company advancing a proprietary platform of cell-permeating alpha-helical peptides that address the most important intracellular targets in oncology and other therapeutic areas. 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 unique drugs like ALRN-6924. Our current focus is to improve the standard of care for patients with solid tumors and hematological malignancies by developing safe and effective therapies that leverage our proprietary peptide platform. For more information, visit www.aileronrx.com, and for more information about its 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 cash forecast, the sufficiency of the Company's cash resources and the timing of clinical trial enrollments and data. 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 period and/or trials anticipated; whether results obtained in preclinical studies and clinical trials will be indicative of results obtained in future clinical trials; 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, 2018, filed on November 7, 2018, 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 attributable to common stockholders and non-GAAP weighted-average shares outstanding to calculate non-GAAP net loss per share attributable to common stockholders. This non-GAAP financial measure reflects charges incurred in connection with the separation agreement with our former Chief Executive Officer in 2018, and gives effect to the conversion of all outstanding shares of preferred stock to common stock, as if such conversion had occurred at the beginning of the period in 2017.

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 periods prior to the closing of our initial public offering on July 5, 2017, we give effect to the automatic conversion of all outstanding shares of redeemable convertible preferred stock to common stock, as if such conversion had occurred at the beginning of the period, in our calculations of non-GAAP weighted-average common shares, basic and diluted, and non-GAAP net loss per share attributable to common stockholders, basic and diluted. The inclusion of these shares facilitates the comparison of results and business outlook for future periods with results for prior periods in order to better understand the long-term performance of our business. For the 2018 nine month period, 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.
Reconciliation of non-GAAP net loss per share, basic and diluted

(in thousands, except per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
GAAP net loss per share attributable to common stockholders—basic and diluted	$ (0.50)	$ (0.45)	$ (1.66)	$ (3.16)
Numerator:				
GAAP net loss	$ (7,434)	$ (6,259)	$ (24,513)	$ (15,739)
Stock based compensation charge related to CEO separation agreement	-	-	612	-
Salary continuation charge related to CEO separation agreement	-	-	564	-
Accretion of redeemable convertible preferred stock to redemption value	-	-	-	(41)
GAAP net loss attributable to common stockholders	$ (7,434)	$ (6,259)	$ (23,337)	$ (15,780)
Denominator:				
GAAP weighted average common shares outstanding — basic and diluted	14,737,402	13,939,950	14,735,660	4,990,535
Assumed conversion of redeemable convertible preferred stock to common stock[1]	-	571,183	-	7,146,719
Non-GAAP weighted average common shares outstanding - basic and diluted	14,737,402	14,511,133	14,735,660	12,137,254
Non-GAAP net loss per share attributable to common stockholders—basic and diluted	$ (0.50)	$ (0.43)	$ (1.58)	$ (1.30)

(1) All redeemable convertible preferred stock converted to common stock upon the settlement of the IPO on July 5th. Conversion of preferred stock into common stock is presumed to have occurred at the beginning of each of the periods presented.

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