# Aileron Therapeutics Reports First Quarter 2018 Financial Results

May 9, 2018

CAMBRIDGE, Mass., May 09, 2018 (GLOBE NEWSWIRE) -- Aileron Therapeutics (Nasdaq:ALRN), the clinical-stage leader in the field of stapled peptide therapeutics for cancers and other diseases, today reported business highlights and financial results for the first quarter ended March 31, 2018.

“In the first quarter, we continued to advance our clinical and non-clinical stapled peptide programs as we pursue our mission to provide transformational outcomes for patients with life-threatening diseases,” said Joseph A. Yanchik III, President and Chief Executive Officer of Aileron. “The clinical activity and safety seen in our interim data from our ongoing Phase 2a clinical trial of ALRN-6924, in addition to our Phase 1 data, continue to support the clinical importance of p53 and the potential of ALRN-6924 in solid and liquid tumors where there is significant need for new treatment options. In addition, our research team continues to make progress in evaluating ALRN-6924 for development in combination studies, and to identify new targets and disease indications for clinical development.”

**ALRN-6924 Program Highlights**

- **Enrollment Ongoing in Phase 2a Trial with ALRN-6924 in Peripheral T-Cell Lymphoma**
  ALRN-6924 is a first-in-class stapled peptide designed to reactivate wild-type p53 tumor suppression in solid and liquid tumors. Aileron is conducting a Phase 2a open-label, multi-center trial of ALRN-6924 as a monotherapy in patients with relapsed or refractory peripheral T-cell lymphoma (PTCL). The Company believes that the preliminary overall response rate observed in the trial (as of February 26, 2018) is generally in line with the reported overall response rates for Romidepsin, the 2$^{nd}$ line PTCL market share leader. Given that ALRN-6924 continues to be well-tolerated, the Company is enrolling patients in an expansion cohort to determine if more frequent dosing can provide an increased benefit to certain patients. Aileron expects to report additional interim data from this trial in the second half of this year.

- **Company Continues to Advance Phase 1 and 1b Studies in AML and MDS**
  Aileron is conducting Phase 1 and 1b open-label, multi-center clinical trials of ALRN-6924 as a monotherapy and in combination with cytosine arabinoside (Ara-C) for the treatment of acute myeloid leukemia (AML) and myelodysplastic syndrome (MDS). The dose escalation studies are designed to establish the recommended Phase 2 dose of ALRN-6924 in patients with AML or MDS, and to evaluate its safety and to provide a preliminary assessment of anti-leukemic activity. In an interim cut of the data (as of February 26, 2018), the Company has observed encouraging evidence of clinical activity and a safety profile consistent with earlier studies. Aileron expects to report interim data from these trials, its dosing strategy, and its plans for a Phase 2 trial in the second half of this year.

- **Company Expands Non-Clinical Studies of ALRN-6924 Combinations**
  Based on ALRN-6924’s unique mechanism of action and safety profile, Aileron has expanded its non-clinical research to test a variety of approved drugs in combination with ALRN-6924, including immuno-oncology agents, cyclin-dependent kinase inhibitors and traditional chemotherapeutic agents for solid and liquid tumors. Aileron expects to provide an update on its non-clinical data and development plans for its combination studies during the second half of 2018.

- **Interim Data Review of Phase 1 All Comers Trial Demonstrates ALRN-6924 Potential**
  In April, Aileron provided interim data (as of February 26, 2018) from the 63 evaluable patients in the Phase 1 dose escalation trial of ALRN-6924 in patients with advanced solid tumors and lymphomas. As of the February 26$^{th}$ cut-off date, five patients, including two patients who achieved complete responses (CR) and one patient who achieved a partial response (PR), remain on treatment for an average treatment period of 685 days. This trial tested nine dose levels and two dosing regimens of once and twice weekly. Of the 63 evaluable patients, 30 patients, or 48%, demonstrated disease

control. This included two CRs, two PRs, and 26 with stable disease, with 42% of stable disease patients showing tumor shrinkage. In a subset of 41 patients whose cells did not contain mutated p53 and received a minimum dose of ALRN-6924, 24 patients (59%) demonstrated disease control, consisting of two CRs, two PRs, and 20 with stable disease.

- **ALRN-6924 Non-Clinical Results Published in *Science Translational Medicine***
  In April, *Science Translational Medicine* published non-clinical results demonstrating the anti-cancer potential of ALRN-6924 in models of AML. Conducted by researchers at Albert Einstein College of Medicine, the studies showed that treatment with ALRN-6924 increased the median survival rate in an animal model of human AML (mice transplanted with human leukemia cells) from 50 to approximately 150 days. In addition, about 40% of the animals were cured, meaning they were tumor-free at one year.

**Corporate Updates**

- **Aileron Expands Scientific Advisory Board**
  In March, Aileron expanded its Scientific Advisory Board with the additions of preeminent scientists Dr. Brian Druker (Knight Cancer Institute, Oregon Health & Science University), Dr. Alan List (Moffitt Cancer Center), and Dr. Carol Prives (Columbia University), all of whom have made groundbreaking contributions to the development of novel cancer therapies.

- **Company to Present at Upcoming Conferences**
  The Company plans to participate at upcoming investor conferences, including the Bank of America Merrill Lynch 2018 Health Care Conference (May 15-17, Las Vegas), the Jefferies Global Healthcare Conference (June 5-8, NYC), and the Canaccord Genuity 38th Annual Growth Conference (Aug. 8-9, Boston).

**First Quarter 2018 Financial Results**

- **Cash Position and Guidance:** Cash, cash equivalents and investments as of March 31, 2018 were $43.3 million, compared to $50.8 million as of December 31, 2017. The Company believes that its cash, cash equivalents and investments as of March 31, 2018 will enable the Company to fund its operating expenses and capital expenditure requirements into the second half of 2019.

- **R&D Expenses:** Research and development (R&D) expenses were $4.8 million for Q1 2018, compared to $2.9 million for the same period in 2017. The increase in R&D expense was primarily driven by increased activity in the Company's non-clinical research and increases in clinical and non-clinical personnel expense. Higher costs were attributable to research associated with expanded testing of a variety of approved drugs in combination with ALRN-6924 along with higher expenses as a result of hiring additional personnel to support ongoing clinical and non-clinical research programs. The Company expects R&D expenses to continue to increase as it continues to advance its ALRN-6924 program and hires additional R&D personnel.

- **G&A Expenses:** General and administrative (G&A) expenses were $2.9 million in Q1 2018, compared to $1.6 million for the same period in 2017. The increase in G&A was primarily due to new hires, increases in non-cash stock compensation costs, and professional fees related to the increased cost of being a public company, consisting mostly of legal and accounting fees. The Company expects G&A expenses to continue to increase as it hires additional personnel to support the Company's anticipated growth in its research and development activities and incurs increased expenses associated with being a public company.

- **Net Loss:** The Company reported a net loss attributable to common stockholders of $7.6 million in Q1 2018 compared to $4.6 million for the same period in 2017. Based on the Company's weighted average shares outstanding, the Company reported a net loss attributable to common stockholders of $0.52 per share in Q1 2018, compared to $10.58 per share for the same period in 2017.

Non-GAAP net loss attributable to common stockholders for Q1 2017 was $0.42 based on non-GAAP weighted-average common shares outstanding of 10.9 million shares. The non-GAAP weighted-average shares outstanding gives effect to the 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.

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 March 31, 2018, there were 14.7 million shares of common stock outstanding.

**About ALRN-6924**
ALRN-6924 is a first-in-class product candidate designed to reactivate wild type p53 tumor suppression by disrupting the interactions between the two primary p53 suppressor proteins, MDMX and MDM2. Aileron believes ALRN-6924 is the first and only product candidate in clinical development that can equipotently bind to and disrupt the interaction of MDMX and MDM2 with p53. Based on preclinical data and preliminary evidence of safety and anti-tumor activity in its ongoing clinical trials, there may be a significant opportunity to develop ALRN-6924 as a monotherapy or combination therapy

for a wide variety of solid and liquid tumors. ALRN-6924 is currently being evaluated in multiple clinical trials for the treatment of acute myeloid leukemia (AML), advanced myelodysplastic syndrome (MDS) and peripheral T-cell lymphoma (PTCL). For information about its clinical trials, please visit www.clinicaltrials.gov.

**About Aileron**

Aileron is a clinical-stage biopharmaceutical company advancing stapled peptides, a novel class of therapeutics for cancers and other diseases. Stapled peptides are chemically stabilized alpha-helical peptides that are modified to improve their stability and cell penetrability while maintaining high affinity for large protein surfaces. Our goal is to use our proprietary stapled peptide drug platform to create first-in-class therapeutics, like ALRN-6924, that may be able to address historically undruggable targets and complex mechanisms that underlie many diseases with high unmet medical need. Our platform enables us to chemically stabilize and improve the performance and activity of a broad range of alpha-helical peptides that we believe can potentially activate and inhibit key cellular functions that are otherwise difficult to target with existing drug technologies, including small molecules and monoclonal antibodies. For more information, visit www.aileronrx.com.

**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 periods 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 March 31, 2018, filed on May 9, 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 weighted-average shares outstanding to calculate non-GAAP net loss per share attributable to common stockholders. This non-GAAP financial measure 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.

For a reconciliation of historical 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.

**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 March 31,** | |
| | **2018** | **2017** |
| GAAP net loss per share attributable to common stockholders—basic and diluted | $ (0.52) | $ (10.58) |
| Numerator: | | |
| GAAP net loss | $ (7,588) | $ (4,557) |
| Accretion of redeemable convertible preferred stock to redemption value | - | (20) |
| GAAP net loss attributable to common stockholders | $ (7,588) | $ (4,577) |
| Denominator: | | |
| GAAP weighted average common shares outstanding — basic and diluted | 14,732,287 | 432,728 |
| Assumed conversion of redeemable convertible preferred stock to common stock[(1)] | - | 10,481,661 |
| Non-GAAP weighted average common shares outstanding - basic and diluted | 14,732,287 | 10,914,389 |
| Non-GAAP net loss per share attributable to common stockholders—basic and diluted | $ (0.52) | $ (0.42) |

(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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