Aileron Therapeutics Reports Fourth Quarter and Full Year 2017 Financial Results

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Encouraging interim clinical results from studies of ALRN-6924 and progress against new programs support advancement and expansion of portfolio development plan

CAMBRIDGE, Mass., April 02, 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 fourth quarter and full year ended December 31, 2017.

“2017 was a pivotal year for Aileron, marked by our initial public offering, key additions to our leadership team, and important progress across our clinical and preclinical programs,” said Joseph A. Yanchik III, President and Chief Executive Officer. “As we are showing with our ALRN-6924 program for cancer, we are committed to bringing lifesaving medicines to patients through our scientific leadership in stapled peptides. We are encouraged by our interim clinical data and by both the progress we have made in the last year in understanding the application of ALRN-6924 and advancing our pipeline as we pursue our objective of substantially improving patient outcomes. This year we want to build on our momentum by focusing on three key priorities:

- Execute on our ongoing clinical plans in peripheral T-cell lymphoma (PTCL), and acute myeloid leukemia and myelodysplastic syndrome (AML/MDS),
- Build on our substantial non-clinical progress in the evaluation of ALRN-6924 in combination with anti-cancer agents, and
- Leverage our product platform to create stapled peptide development programs against new targets through continued expansion of our research and development capabilities.”

Program Highlights and Current Updates

- **Updated Report on ALRN-6924 Studies Shows Encouraging Results in Multiple Cancers**
  ALRN-6924 is Aileron’s lead stapled peptide therapeutic and is being evaluated in multiple clinical trials. It is, the Company believes, the only drug in clinical development capable of disrupting the interaction of both MDMX and MDM2 with p53. ALRN-6924 is designed to reactivate p53-mediated tumor suppression to restore p53’s function as the body’s first line of defense against cancer.

  Phase 1 All Comers Trial; Selected as Best of ASCO® at 2017 American Society of Clinical Oncology (ASCO) Annual Meeting – In an update of the ASCO data, as of February 26, 2018, there were 63 evaluable patients in the Phase 1 dose escalation trial of advanced solid tumors and lymphomas. 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 these, 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. Data from this trial were presented at ASCO in June 2017. The abstract on ALRN-6924 was selected as one of the meeting’s top abstracts in the Best of ASCO® program. In addition, the Company’s Phase 1 trial of ALRN-6924 was selected for inclusion in *Clinical Cancer Advances 2018*, the Society’s annual review of the year’s major achievements in cancer research and care.

  Phase 2a PTCL Trial – Aileron continues to advance ALRN-6924 as a monotherapy for the treatment of PTCL in a Phase 2a trial in wild-type p53 patients. This trial is designed to provide preliminary insight into the responsiveness of this patient population to ALRN-6924, to evaluate its safety and confirm the optimal dosing regimen. 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 2nd line PTCL market share leader. Additional data are described in more detail in the Annual Report on Form 10-K. Given that ALRN-6924 continues to be well-tolerated, the Company has commenced enrollment of an expansion cohort to determine if more frequent dosing provides an increased benefit to certain patients.

Phase 1 and 1b in AML/MDS Trial – Aileron continues to advance ALRN-6924 as a monotherapy and in combination with Ara-C for the treatment of AML and MDS. The Phase 1 and 1b 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 preliminary anti-leukemic activity. As of February 26, 2018, 33 patients have been enrolled across six cohorts representing two dosing regimens. The Company has observed evidence of clinical activity consisting of two marrow complete responses, which were observed in two patients in one of the combination cohorts in the trial in which five patients had been enrolled as of the cut-off date for the data. Six patients remain on treatment in the trials and the safety profile is consistent with earlier studies.

Non-clinical Combination Studies – Aileron has expanded and advanced 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. The Company believes the mechanism of action and safety profile of ALRN-6924 may provide the potential for its combination with a wide variety of conventional and novel therapies. Aileron currently expects to provide an update on its non-clinical data and development plans for its ALRN-6924 combination studies during the second half of 2018.

- **Anti-Cancer Effects of ALRN-6924 Highlighted in Oral Presentations at American Society of Hematology (ASH) Annual Meeting**
  Two oral presentations at ASH in December highlighted positive preclinical data of ALRN-6924 in PTCL and AML from its collaborations with the Dana-Farber Cancer Institute and Albert Einstein College of Medicine, respectively. In PTCL, the presented data showed that in T-cell lymphoma lines (TCL) ALRN-6924 induced apoptotic cell death and demonstrated superior efficacy across multiple TCL subtypes, compared to the current standard-of-care. In AML, the data presented showed that dual inhibition of MDMX and MDM2 by ALRN-6924 led to activation of p53-dependent pathways, resulting in strong anti-leukemic effects including complete remissions and prolonged overall survival.

Corporate Updates

- **Company Strengthens Executive Team and Board of Directors**
  Jeffrey A. Bailey, CEO of IlluminOss Medical, was recently appointed as Chairman of Aileron's Board of Directors, bringing 30 years' experience in key leadership roles at major pharmaceutical companies as well as in the creation of valuable, development stage healthcare companies. In June, Donald Dougherty joined the Aileron management team as Senior Vice President and Chief Financial Officer, bringing more than 30 years of financial leadership experience from his most recent role as Founder and President at Compound Capital Growth Investments, LLC.

- **Aileron Successfully Completed $56.25 Million Initial Public Offering**
  In an initial public offering (IPO) in early July, Aileron issued and sold 3,750,000 shares of common stock at an offering price of $15 per share, resulting in net proceeds of approximately $50 million after deducting underwriting discounts, commissions and expenses.

- **Three Leading Scientists Join Aileron's Scientific Advisory Board**
  Joining Aileron's development efforts are preeminent researchers Dr. Brian Druker, Dr. Alan List, and Dr. Carol Prives, all of whom have made groundbreaking contributions to the development of novel cancer therapies.

- **Aileron to Present at Upcoming Conferences**
  The Company will be participating at investor conferences throughout the year, including Bank of America Merrill Lynch Healthcare Conference (May 15-17, Las Vegas), Jefferies Global Healthcare Conference (June 5-8, NYC), and Canaccord Genuity 38th Annual Growth Conference (Aug. 8-9, Boston).

Fourth Quarter and Full Year 2017 Financial Results

- **Cash Position and Guidance:** Cash, cash equivalents and investments as of December 31, 2017 were $50.8 million, compared to $20.7 million as of December 31, 2016. The Company closed its initial public offering on July 5, 2017, resulting in net proceeds of $50.0 million. The Company believes that its cash, cash equivalents and investments as of December 31, 2017 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 $4.3 million for Q4 2017, compared to $3.1 million for

the same period in 2016 and $14.2 million for the full year 2017, compared to $10.3 million for the same period in 2016. The increase in R&D expense for both the fourth quarter of 2017 and the full year was primarily driven by increased activity in the Company's ALRN-6924 program and expenses related to the hiring of additional R&D personnel. 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 $2.7 million in Q4 2017, compared to $1.4 million for the same period in 2016 and $8.8 million for the full year 2017, compared to $7.9 million for the same period in 2016. The increase in G&A for both the fourth quarter of 2017 and the full year was primarily due to increases in non-cash stock compensation costs, the costs associated with being a public company and professional fees, 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 $6.9 million in Q4 2017 compared to $4.5 million for the same period in 2016 and $22.6 million for the full year 2017, compared to $18.2 million for the same period in 2016. Based on the Company's weighted average shares outstanding, the Company reported a net loss attributable to common stockholders of $0.47 per share in Q4 2017, compared to $10.31 per share for the same period in 2016 and a net loss attributable to common stockholders of $3.04 per share for the full year 2017, compared to $42.35 per share in the same period in 2016.

  Non-GAAP net loss attributable to common stockholders for Q4 2017 and Q4 2016 was $0.47, based on non-GAAP weighted-average common shares outstanding of 14.7 and 9.5 million shares, respectively, and non-GAAP net loss attributable to common stockholders for the full year 2017 and 2016 was $1.77 and $1.91, respectively, based on non-GAAP weighted-average common shares outstanding of 12.8 and 9.5 million shares, respectively. 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 December 31, 2017, subsequent to the closing of the IPO and the conversion of the convertible preferred stock, 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 annual report on Form 10-K for the period ended December 31, 2017, filed on April 2, 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 December 31,		Year Ended December 31,	
	2017	**2016**	**2017**	**2016**
GAAP net loss per share attributable to common stockholders—basic and diluted	$ (0.47)	$ (10.31)	$ (3.04)	$ (42.35)
Numerator:				
GAAP net loss	$ (6,865)	$ (4,442)	$ (22,604)	$ (18,123)
Accretion of redeemable convertible preferred stock to redemption value	-	(19)	(41)	(75)
GAAP net loss attributable to common stockholders	$ (6,865)	$ (4,461)	$ (22,645)	$ (18,198)
Denominator:				
GAAP weighted average common shares outstanding — basic and diluted	14,720,734	432,413	7,443,078	429,686
Assumed conversion of redeemable convertible preferred stock to common stock[(1)]	-	9,060,073	5,348,734	9,069,374
Non-GAAP weighted average common shares outstanding - basic and diluted	14,720,734	9,492,486	12,791,812	9,499,060

Non-GAAP net loss per share attributable to common stockholders—basic and diluted	$ (0.47)	$ (0.47)	$ (1.77)	$ (1.91)

(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.

Source: Aileron Therapeutics

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