Aileron Therapeutics Announces Publication by Researchers at Albert Einstein College of Medicine of ALRN-6924 Nonclinical Data in Acute Myeloid Leukemia

April 11, 2018

Nonclinical data featured in Science Translational Medicine show dual inhibition of MDM2 and MDMX restored p53 function and demonstrated strong response rates in AML model

CAMBRIDGE, Mass., April 11, 2018 (GLOBE NEWSWIRE) -- Aileron Therapeutics (NASDAQ:ALRN), the clinical stage leader in the field of stapled peptide therapeutics for cancers and other diseases, today announced the publication of nonclinical results in *Science Translational Medicine* demonstrating the anti-cancer potential of ALRN-6924 in models of Acute Myeloid Leukemia (AML). ALRN-6924 is designed to reactivate p53-mediated tumor suppression by targeting the two primary p53 suppressor proteins, MDM2 and MDMX. ALRN-6924 is being evaluated in Phase 1 and Phase 2 clinical trials in patients with AML, myelodysplastic syndrome (MDS) and peripheral T-cell lymphoma (PTCL).

In AML, blood-forming stem cells in the bone marrow produce abnormal red and white blood cells as a result of damage to DNA. P53, a natural tumor suppressor, is inactive in AML, allowing cancer cells to grow unimpeded. Reactivating p53 with ALRN-6924 appears to slow or stop the growth of both mature and immature cancer cells. As demonstrated by the researchers at Albert Einstein in their nonclinical studies, 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 about 150 days. In addition, about 40% of the animals were cured, meaning they were tumor-free at one year.

“These data further support our belief that p53’s function may be more effectively restored when both MDMX and MDM2 are blocked. ALRN-6924, a stapled peptide therapeutic shown to inhibit both protein targets, has the potential to deliver on the long-held promise that restoring apoptosis through the p53 pathway may be critical in treating certain cancers,” said Manuel Aivado, M.D., Ph.D., Chief Medical and Scientific Officer of Aileron.

“This is a very striking response. Most experimental drugs for leukemia in our experience achieve an increase in survival of only a few days in these preclinical models. Even more importantly, ALRN-6924 effectively cured about 40 percent of the treated mice,” said study leader Ulrich Steidl, M.D., Ph.D., Professor of the Departments of Cell Biology and of Medicine and the Diane and Arthur B. Belfer Faculty Scholar in Cancer Research at Albert Einstein College of Medicine, and Associate Chair for Translational Research in Oncology at Montefiore.

The study in *Science Translational Medicine* is titled, “Dual inhibition of MDMX and MDM2 as a Therapeutic Strategy in Leukemia.”

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.

Source: Aileron Therapeutics

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