



Anti-tumor Activity Observed in Phase 1 Trial of Novel P53-Targeting Drug ALRN-6924

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First clinical data reported on a dual inhibitor of MDMX and MDM2, key suppressors of P53 function

Data reviewed in oral presentation at 2017 ASCO Annual Meeting

CHICAGO, IL—June 3, 2017—Aileron Therapeutics, a clinical-stage biopharmaceutical company developing a novel class of therapeutics called stapled peptides, today announced safety and clinical activity data, including complete responses, partial responses and evidence of stable disease, from its Phase 1, multi-center trial with ALRN-6924 in a variety of advanced cancers. ALRN-6924, a stapled peptide therapeutic, is believed to be the first product candidate shown to disrupt both MDMX- and MDM2-mediated inhibition of the wild type p53 tumor suppressor gene in clinical trials. The data were reviewed in an oral presentation at the 2017 ASCO Annual Meeting by Funda Meric-Bernstam, M.D., Chair of the Department of Investigational Cancer Therapeutics at The University of Texas MD Anderson Cancer Center.

Dr. Meric-Bernstam commented, "These results lend clinical support to the belief that the function of tumor suppressor gene p53, one of the most sought-after targets in oncology, is inhibited by MDMX and MDM2. What is encouraging about these early data with ALRN-6924 is the duration of clinical activity observed--some lasting more than one year--and that the compound appeared to be reasonably well tolerated in this population of previously treated patients with advanced solid tumors and lymphomas. Additional results from studies will look at ALRN-6924, both as a single agent therapy as well as in combination with established cancer agents."

Study Results

The data presented from the Phase 1, multi-center, dose escalation trial included 71 patients with a broad range of advanced blood and solid tumor cancers.

ALRN-6924 was well tolerated in patients in the study. The most common treatment-related, non-hematologic adverse events were GI symptoms, fatigue and headache. There was no Grade 3/4 thrombocytopenia, and Grade 3/4 neutropenia was reported in less than 5% of patients.

Efficacy data was presented on 41 patients in the trial with a broad range of advanced blood and solid tumor cancers who received a >3.2 mg/kg dose of ALRN-6924 per treatment cycle and who did not have a mutation of the p53 gene. The 30 patients who were not included in the efficacy analysis either had a mutation of the p53 gene, received lower drug doses, or discontinued the study prior to disease evaluation.

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Of the 41 patients, there were two complete responses (CR), two partial responses (PR), and 20 with stable disease (SD). The disease control rate was 59% (24/41). One CR patient, both PR patients and two SD patients have received ALRN-6924 for well over one year, the CRs and PRs are ongoing as of May 1, 2017 and patients continue to receive treatment with ALRN-6924.

The Aileron abstract on ALRN-6924 was also selected for the Best of ASCO® program, an educational initiative highlighting the year's most notable abstracts from the ASCO Annual Meeting at various meetings around the globe this summer.

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 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.

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