Aileron Therapeutics and Advancium Health Network Announce an Exclusive Option Agreement for the Acquisition of ALRN-6924 for Retinoblastoma

October 31, 2024

Agreement marks first of its kind for Advancium, a public charity launched by Deerfield Management and the Deerfield Foundation

Advancium will evaluate ALRN-6924 as a potential therapy for retinoblastoma, a rare but devastating pediatric cancer of the eye

AUSTIN, Texas and NEW YORK, Oct. 31, 2024 /PRNewswire/ -- Aileron Therapeutics, Inc. ("Aileron") (NASDAQ: ALRN), a biopharmaceutical company advancing a novel pipeline of first-in-class medicines to address significant unmet medical needs in orphan pulmonary and fibrosis indications, and Advancium Health Network ("Advancium"), a public charity founded by Deerfield Management and the Deerfield Foundation to meet the needs of underserved patient populations, today announced entry into an exclusive option agreement for the acquisition of ALRN-6924, a clinical-stage oncology agent developed by Aileron prior to its 2023 merger with Lung Therapeutics, Inc.

During the option period, Advancium intends to evaluate ALRN-6924 as a potential therapy for retinoblastoma (RB), a rare but devastating cancer of the eye, with around 300 cases diagnosed in the United States and 9,000 cases worldwide each year, nearly all in children [1]. With early intervention the disease is rarely fatal; however, non-selective chemotherapy with its attendant side effects remains the usual treatment strategy, and removal of the affected eye(s) is a frequent outcome. In contrast to chemotherapy, ALRN-6924 selectively targets MDM2 and MDMX, the endogenous inhibitors of the regulatory protein p53, to activate p53-mediated tumor suppression in cancer cells [2]. Previous studies have implicated the MDMX protein as a key driver of RB tumorigenesis [3], and ALRN-6924 is the first and only clinical-stage drug that acts on MDMX. ALRN-6924 has previously been studied in preclinical models of RB, where it showed potent, on-mechanism anti-proliferative activity in RB cell lines and was found to be highly soluble and compatible with intraocular injection [4].

Under the terms of the option agreement, Advancium paid Aileron a non-refundable fee for the exclusive option to acquire ALRN-6924 and related assets. If Advancium exercises its option, Aileron will receive an exercise payment with potential for additional development, regulatory and commercial milestone payments and sales royalties.

"This agreement is a significant landmark for our work at Advancium, as it is the first drug opportunity we are pursuing in our mission as a public charity to develop much-needed therapies for children with cancer," said Mark Veich, Chief Executive Officer of Advancium and Vice President of Philanthropy and Executive Director of the Deerfield Foundation. "We look forward to evaluating ALRN-6924 as a potential treatment for children with

retinoblastoma who currently have limited care options."

"Aileron is a strong supporter of Advancium's cause," said Brian Windsor, Ph.D., President and Chief Executive Officer of Aileron. "Although we remain focused on developing novel therapies for the treatment of orphan pulmonary and fibrosis indications, we are honored that they have chosen to partner with us in this important step towards their goal of delivering therapies to pediatric patients with high unmet need."

References

[1] Cobrinik, D. "Retinoblastoma Origins and Destinations" N Engl J Med. 2024 Apr 18;390(15):1408-1419

[2] Guerlavais, V. et al, "Discovery of Sulanemadlin (ALRN-6924), the First Cell-Permeating, Stabilized α-Helical Peptide in Clinical Development" J Med Chem, 2023; 66:9401–9417

[3] Laurie, N. A. et al, "Inactivation of the p53 pathway in retinoblastoma" Nature. 2006 Nov 2;444(7115):61-6

[4] Wilson, M. W. et al, "Intraocular MDMX Inhibition by ALRN-6924: A Targeted Strategy to Reactivate p53 in Retinoblastoma." Investigative Ophthalmology & Visual Science, 2023 June;64(8): 1272

About Aileron Therapeutics

Aileron Therapeutics is a biopharmaceutical company advancing a novel pipeline of first-in-class medicines to address significant unmet medical needs in orphan pulmonary and fibrosis indications. Aileron's lead product candidate, LTI-03, is a novel, synthetic peptide with a dual mechanism targeting alveolar epithelial cell survival as well as inhibition of profibrotic signaling. Currently, LTI-03 is being evaluated in a Phase 1b clinical trial for the treatment of idiopathic pulmonary fibrosis. Aileron's second product candidate, LTI-01, is a proenzyme that has completed Phase 1b and Phase 2a clinical trials for the treatment of loculated pleural effusions. LTI-01 has received Orphan Drug Designation in the US and EU and Fast Track Designation in the US.

About Advancium Health Network

Advancium Health Network is a mission-driven public charity pioneering ways to overcome barriers in healthcare through a unique model that combines for-profit infrastructure with non-profit ideals. Advancium forges bonds between a diverse body of industry partners, experts, and philanthropic support to leverage its collective expertise and resources. From early-stage innovation to pediatric MedTech and rare diseases, we are confronting healthcare's most daunting hurdles for underserved patients and catalyzing a positive impact on human health.

Forward-Looking Statements

This press release may contain forward-looking statements of Aileron Therapeutics, Inc. ("Aileron", the "Company", "we", "our" or "us") within the meaning of the Private Securities Litigation Reform Act of 1995, including statements with respect to: the terms of the option agreement between Advancium and the Company; the exercise of the option to acquire ALRN-6924 pursuant to the option agreement; and the potential of the Company to receive a future option exercise payment, milestone payments and royalties pursuant to the option agreement with Advancium. We use words such as "anticipate," "believe," "estimate," "expect," "hope," "intend," "may," "plan," "predict," "project," "target," "potential," "would," "can," "could," "should," "continue," and other words and terms of similar meaning to help 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 risks and uncertainties related to, changes in applicable laws or regulations, the possibility that the Company may be adversely affected by other economic, business, and/or competitive factors, including risks inherent in pharmaceutical research and development, such as: adverse results in the Company's drug discovery, preclinical and clinical development activities, the risk that the results of preclinical studies and early clinical trials may not be replicated in later clinical trials or that partial results of a trial such as the Cohort 1 results from the Company's ongoing Phase 1b clinical trial will be indicative of the full results of the trial, the Company's ability to enroll patients in its clinical trials, and the risk that any of its clinical trials may not commence, continue or be completed on time, or at all; decisions made by the U.S. Food and Drug Administration and other regulatory authorities, investigational review boards at clinical trial sites and publication review bodies with respect to our development candidates; uncertainty as to whether or when the option will be exercised, whether the development of ALRN-6924 as a potential therapy for retinoblastoma will be successful, and whether the Company will receive any meaningful payments under the Company's option agreement with Advancium ; as well as the risks and uncertainties discussed in the "Risk Factors" section of the Company's Annual Report on Form 10-K for the year ended December 31, 2023 and Quarterly Report on Form 10-Q for the quarter ended June 30, 2024, which are on file with the United States Securities and Exchange Commission (the "SEC"), and in subsequent filings that the Company makes with the SEC. These forward-looking statements should not be relied upon as representing the Company's view as of any date subsequent to the date of this press release, and we expressly disclaim any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

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