Alkeus Announces $150 Million Series B Financing, Supporting Rapid Registration Path for gildeuretinol (ALK-001) in the Treatment of Stargardt Disease

Vertex Founder and Industry Pioneer Joshua Boger, Ph.D. named as Executive Chairman of Alkeus

June 5, 2023, Cambridge MA – Alkeus Pharmaceuticals announced today that it has raised a $150 million Series B financing to support the registration and launch of gildeuretinol (ALK-001), a potential disease-modifying, precision medicine for the treatment of Stargardt disease, a leading genetic cause of blindness in children and young adults. The financing was led by Bain Capital Life Sciences, with additional participation by TCGX, Wellington Management and Sofinnova Investments. Alkeus also announced today that Joshua Boger, Ph.D., a biotech industry pioneer and the founder of Vertex Pharmaceuticals, has joined Alkeus as Executive Chairman.

"The proceeds from this $150 million financing will allow Alkeus to expand our team and drive gildeuretinol towards NDA submission and FDA approval as rapidly as possible," said Leonide Saad, Ph.D., CEO, President, and Co-Founder of Alkeus. "I am thrilled to work hand in hand with Joshua as Executive Chairman as we build a patient-focused company. Joshua's deep experience leading the successful development and commercialization of transformative medicines will be of tremendous value to Alkeus during this period of organizational growth, in preparation for the launch of gildeuretinol."

Stargardt disease is a leading cause of blindness in children and young adults, affecting more than 30,000 people in the U.S. and more than 150,000 worldwide. Patients with Stargardt disease are typically born with normal vision, but mutations in the ABCA4 gene lead to accelerated dimerization — or clumping — of vitamin A in the eye, causing damage to the retina and subsequent progressive vision loss, starting as early as five years of age. Gildeuretinol is the first and only medicine in clinical development to address the underlying mechanism of Stargardt

disease by substantially reducing vitamin A dimerization in the eye, without any impact on normal vision. Phase 2 clinical data has shown a statistically and clinically meaningful slowing of retinal damage in Stargardt patients. The U.S. FDA has granted Breakthrough Therapy Designation and Orphan Drug Designation to gildeuretinol (ALK-001). Alkeus plans to submit an NDA for approval of gildeuretinol in 2024.

“Stargardt is a progressive, debilitating disease that leads inevitably to devastating vision loss. The genetic cause is well characterized, but to date there is no effective treatment,” said Joshua Boger, Ph.D., Executive Chairman of Alkeus. “Leonide and the Alkeus team have brought gildeuretinol from the lab all the way to positive and compelling clinical data across multiple stages of the disease, including a Phase 2 placebo-controlled trial in patients with advanced disease, where we have reported a statistically- and clinically-significant slowing of disease. Additional clinical data show halting of disease in patients, including children.”

Dr. Boger continued, “We are now poised to make this transformative medicine a reality for all Stargardt patients.”

About Gildeuretinol (ALK-001)

Gildeuretinol was designed by researchers as a novel, specifically deuterated form of vitamin A that greatly reduces vitamin A dimerization, with the potential to slow or halt the progression of vision loss in Stargardt patients without disrupting the visual cycle. In preclinical studies, gildeuretinol decreased vitamin A dimerization by more than 80% and prevented development of blindness in a genetic animal model of the disease. In a 2-year, double-blind, placebo-controlled Phase 2 study (n = 50) in Stargardt patients with advanced disease, treatment with gildeuretinol once a day as a pill resulted in slowing of retinal damage as compared to placebo, based on observed growth rates in atrophic lesions as measured by fundus autofluorescence (FAF). The drug was well tolerated, with safety consistent with the well-characterized profile of vitamin A. In children and young adults with earlier stage Stargardt disease, gildeuretinol appeared to halt the disease process, preventing any further retinal damage and vision loss. Additional clinical trials of gildeuretinol in Stargardt disease are ongoing, including a fully-enrolled, randomized, placebo-controlled clinical trial (n = 80) in Stargardt patients with intermediate disease, expected

to read out top line data in 2025. In addition, a Phase 3 study (n = 200) of gildeuretinol in patients with geographic atrophy (GA) secondary to dry age-related macular degeneration (AMD) is now fully enrolled and is expected to read out later in 2023.

About Alkeus Pharmaceuticals

Alkeus Pharmaceuticals is a biopharmaceutical company with headquarters in Cambridge, MA. Co-founded by Leonide Saad, Ph.D. and Ilyas Washington, Ph.D., Alkeus is focused on developing therapies for serious diseases of the eye with high unmet need. Alkeus' lead candidate, gildeuretinol (ALK-001), is currently being evaluated in clinical trials for the treatment of Stargardt disease and for geographic atrophy secondary to dry age-related macular degeneration (AMD). Stargardt disease is a leading mono-genetic cause of blindness in children and young adults, with more than 30,000 people affected in the U.S. and more than 150,000 worldwide. Geographic atrophy is the chronic progressive degeneration of the macula in the eye, characteristic of the late stage of age-related macular degeneration (AMD), with more than one million people affected in the U.S. and more than five million worldwide.

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