Alkeus Pharmaceuticals Announces Results from the SAGA Study of Oral Gildeuretinol in Patients with Geographic Atrophy Secondary to Age-Related Macular Degeneration

- Oral gildeuretinol demonstrated a clinically meaningful reduction in the geographic atrophy (GA) lesion growth rate at 24 months, supporting further clinical development.

- Gildeuretinol showed a statistically significant slowing of the rate of decline in low luminance visual acuity (LLVA) at 24 months, a key secondary endpoint. It is the first oral therapy to demonstrate an impact on a functional endpoint in GA.

- A favorable safety and tolerability profile was demonstrated by gildeuretinol, consistent with prior clinical studies in Alkeus' Stargardt disease program.

- SAGA topline data has been accepted as a late breaker at the 128th Annual Meeting of the American Academy of Ophthalmology (AAO) being held October 18-21 in Chicago.

CAMBRIDGE, Mass., September 17, 2024 – Alkeus Pharmaceuticals, Inc. today announced that the SAGA study of oral gildeuretinol acetate (ALK-001) in patients with geographic atrophy (GA) secondary to age-related macular degeneration (AMD) demonstrated a reduction of 0.25 sqmm/year vs. placebo (p=0.07) in the primary endpoint of GA lesion growth rate at 24 months. Gildeuretinol demonstrated a statistically significant reduction in the loss of low luminance visual acuity (LLVA) at 24 months (p=0.03). Gildeuretinol showed a favorable safety profile consistent with other studies of gildeuretinol in Stargardt disease. The topline results will be presented as a late breaker at the 128th Annual Meeting of the American Academy of Ophthalmology (AAO) during Retina Subspecialty Day on Friday, October 18, in Chicago.

"These data clearly indicate a clinically meaningful trend in slowing the growth rate of GA lesions, which is extremely encouraging," said Seemi Khan, M.D., M.P.H., M.B.A., Chief Medical Officer of Alkeus Pharmaceuticals. "The SAGA data represent the first clinical demonstration that slowing vitamin A dimerization could be beneficial in the treatment of GA secondary to AMD. Results from SAGA build upon the positive data from TEASE-1, a study of gildeuretinol in Stargardt disease. We look forward to discussing these results with the U.S. Food and Drug Administration to determine the optimal path forward. We extend our gratitude to the patients, investigators and trial sites for their participation in this study."

SAGA was a 24-month, double-masked, randomized, placebo-controlled trial to investigate the safety, pharmacokinetics, tolerability and efficacy in patients with GA secondary to AMD. The study enrolled 198 patients. The primary efficacy endpoint was the growth rate of GA lesions from baseline to 24 months as assessed by Fundus Autofluorescence (FAF). The first key secondary endpoint was the change in LLVA at 24 months.

"I am highly encouraged by the results of an oral treatment that showed a significant reduction of the growth rate of GA, as well as its effect on visual acuity," said David S. Boyer, M.D., a retina specialist with Retina-Vitreous Associates Medical Group of Los Angeles, and a principal

investigator in SAGA. “The patient population afflicted with GA is in desperate need of an oral treatment to slow disease progression. I’m extremely excited by these data and believe this is a significant advancement of our scientific understanding of the GA disease mechanism.”

GA is a serious, progressive condition that causes irreversible loss of central vision. It is estimated that the median time of progression to legal blindness is slightly over six years. There is no oral therapy approved by the FDA to treat GA. The prevalence of GA in the United States is estimated to be over 1 million people, with 160,000 new cases occurring each year.

“These results reinforce the potential of gildeuretinol as an oral therapy for the treatment of macular degenerative diseases,” said Michel Dahan, President and CEO of Alkeus Pharmaceuticals. “We are driven by our mission to positively impact the lives of the many people around the world living with degenerative eye diseases. Patients, parents and children, and their treating physicians are waiting for treatment options to preserve sight. We will continue to work diligently to bring oral gildeuretinol to those in need starting with individuals impacted by Stargardt disease, subject to regulatory approval.”

About Gildeuretinol Acetate (ALK-001)

Gildeuretinol acetate (ALK-001) is a novel molecule created as a specialized form of deuterated vitamin A designed to reduce the dimerization of vitamin A without disrupting vision. In preclinical studies, gildeuretinol decreased vitamin A dimerization down to the normal rate and prevented retinal degeneration and loss of visual function in animals with Stargardt disease. A randomized, placebo-controlled, double-masked clinical trial of gildeuretinol in late-stage Stargardt patients (TEASE-1) showed clinically and statistically significant slowing of the growth of retinal lesions over two years of treatment. Additional clinical trials of gildeuretinol in Stargardt disease are ongoing. Gildeuretinol has received breakthrough therapy designation and orphan drug designation for Stargardt disease from the U.S. Food and Drug Administration. A Phase 2/3 study (SAGA) of gildeuretinol in approximately 200 patients with geographic atrophy (GA) secondary to age-related macular degeneration (AMD) has been completed and topline data has been accepted as a late-breaking abstract at the 128th Annual Meeting of the American Academy of Ophthalmology (AAO) being held October 18-21 in Chicago.

About Alkeus Pharmaceuticals

Alkeus Pharmaceuticals, Inc. is a private biopharmaceutical company with headquarters in Cambridge, Mass., backed by institutional investors led by Bain Capital Life Sciences. Founded in 2010, Alkeus is developing therapies for serious diseases of the eye with high unmet need. Alkeus’ breakthrough-designated lead candidate, gildeuretinol acetate (ALK-001), is currently being evaluated in clinical trials for the treatment of Stargardt disease and for geographic atrophy (GA) secondary to age-related macular degeneration (AMD).

For further information, please contact:

Media Relations
media@alkeuspharma.com
Website: www.alkeuspharma.com