**Alkeus Pharmaceuticals Announces Presentation of a TEASE-3 Study Update Showing Progression Stalled in Early-Stage Stargardt Disease Patients Treated with Gildeuretinol**

***Data in early-stage patients presented at the 42nd ASRS Annual Scientific Meeting***

**CAMBRIDGE, Mass.**, July 18, 2024 – Alkeus Pharmaceuticals, Inc. today announced the presentation of interim data from its TEASE-3 study demonstrating that early-stage Stargardt disease patients treated with gildeuretinol acetate showed no disease progression and remained asymptomatic while on therapy ranging between two and six years. The study update was presented during the 42nd American Society of Retina Specialists (ASRS) Annual Scientific Meeting, being held July 17-20 in Stockholm, Sweden.

“Interim results from the TEASE-3 study of gildeuretinol in early-stage Stargardt patients indicate the potential value of treating patients with confirmed disease-causing ABCA4 genetic mutations as early as possible before the disease causes progressive loss of central vision,” said Seemi Khan, M.D., M.P.H., M.B.A., Chief Medical Officer of Alkeus Pharmaceuticals. “There is high unmet need in this disease area with no existing treatment, and we look forward to sharing additional results from this encouraging study in the future as we work to advance our development program, which has the potential to be the first therapy for Stargardt disease.”

TEASE-3, the first clinical trial in early-stage Stargardt disease, is an open-label study of gildeuretinol in genetically confirmed patients with early signs of disease visible on retinal imaging, but who have not begun experiencing symptoms of vision loss. Each TEASE-3 study participant has a sibling who was previously diagnosed with Stargardt disease, has identical gene mutations and has experienced irreversible vision loss. The primary endpoint of disease progression over two years is assessed by retinal imaging and functional outcome measures. After the initial two-year treatment period, patients continue to receive gildeuretinol while enrolled in an open label long-term extension study. The TEASE-3 study has enrolled a total of six patients, who receive gildeuretinol as a once-a-day pill.

“The TEASE-3 study has been a unique opportunity to evaluate gildeuretinol in a small group of presymptomatic children for whom we had clinical history information regarding the course of Stargardt disease in older siblings,” said Michael B. Gorin, M.D., Ph.D., of the Departments of Ophthalmology and of Human Genetics at the David Geffen School of Medicine at UCLA. “We were able to assess the ability of the medication to stall vision loss in these children as compared to the age-matched data of their genetically-matched Stargardt siblings. The dramatic delay of vision and photoreceptor loss in this small cohort is an exciting advancement in the therapy for ABCA4-related Stargardt disease and suggests that the clinical benefits may be greater at the earliest stages of this condition.”

Stargardt disease is a serious cause of blindness in children and young adults, with an estimated 30,000 people affected in the U.S. and more than 150,000 worldwide. There is no approved treatment. In individuals with Stargardt disease, the ABCA4 protein is defective. This

defect in the protein results in the accelerated dimerization of vitamin A, forming toxic by-products that irreversibly damage the retina, resulting in progressive vision loss.

**About the TEASE Trials**

The TEASE trials consist of four clinical studies of gildeuretinol acetate (ALK-001) in Stargardt disease, denoted as TEASE-1, TEASE-2, TEASE-3 and TEASE-4. The TEASE-1 study was a randomized, double-masked, placebo-controlled trial in 50 patients with Stargardt disease. Gildeuretinol met its prespecified primary efficacy endpoint showing a 21% reduction in the growth rate of retinal atrophic lesions ($p<0.001$, square root units, 28% reduction for untransformed areas of retinal atrophic lesions) against untreated patients. Gildeuretinol was well-tolerated. The TEASE-2 trial is an ongoing, fully enrolled, randomized, double-masked, placebo-controlled trial in 80 patients with Stargardt disease, expected to read out topline data in 2025. TEASE-3 is an open-label study designed to assess gildeuretinol in early-stage Stargardt patients. TEASE-4 is an open-label extension study.

**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 to the normal rate seen in unaffected individuals and prevented retinal degeneration and loss of visual function in animals with Stargardt disease. Gildeuretinol has received breakthrough therapy designation and orphan drug designation from the U.S. Food and Drug Administration. In addition to the TEASE trials, a Phase 3 (SAGA) study of gildeuretinol in 200 patients with geographic atrophy (GA) secondary to age-related macular degeneration (AMD) is expected to read out topline data in 2024.

**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).

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