Alkeus Pharmaceuticals Announces New Positive Interim TEASE-3 Study Results Showing Gildeuretinol Prevented Disease Progression in Early-Stage Stargardt Patients

- Two additional TEASE-3 participants who recently completed 24 months of therapy showed no disease progression, consistent with prior results.

- A total of five patients have completed the 24-month study. Following the study, these patients have remained on treatment and continue to show no disease progression, with the longest duration of therapy to date being more than seven years.

- TEASE-3 is the first clinical trial in early-stage Stargardt patients to show no disease progression over multiple years, including preservation of visual acuity.

- These data will be presented January 14 at 4:30 p.m. PST during the J.P. Morgan Healthcare Conference at The Westin St. Francis San Francisco, Mission Bay room, 32nd floor of the Tower Building.

CAMBRIDGE, Mass., January 9, 2025 – Alkeus Pharmaceuticals, Inc., a biopharmaceutical company dedicated to preserving the sight of individuals impacted by retinal diseases, today announced positive interim data from its TEASE-3 study demonstrating that additional early-stage Stargardt disease patients treated with oral gildeuretinol acetate showed no disease progression, including stable visual acuity, over multiple years.

“These dramatic results showing preservation of vision in early-stage patients highlight the potential of gildeuretinol to prevent children diagnosed with Stargardt disease from progressing to severe vision loss when therapy is started early,” said Michel Dahan, President and CEO of Alkeus Pharmaceuticals. “Alkeus is poised to transform the treatment of Stargardt disease, a rare and relentlessly progressive condition that leads to irreversible blindness in children and adults. With no approved treatment currently available, there is an urgent need to bring an option to patients. We plan to submit an NDA to the U.S. Food and Drug Administration for gildeuretinol as a treatment for Stargardt disease as soon as possible in 2025 based on compelling data generated to date from our multi-study clinical program.”

Alkeus will present the data at 4:30 p.m. PST on January 14 during the J.P. Morgan Healthcare Conference at The Westin St. Francis San Francisco, Mission Bay room, 32nd floor of the Tower Building.

“Two additional patients have completed 24 months of treatment in the TEASE-3 study, and the results demonstrated that once-daily oral gildeuretinol prevented progression and vision remained stable with a consistent, well-tolerated safety profile,” said Seemi Khan, MD, MBA, Chief Medical Officer at Alkeus Pharmaceuticals. “In addition, the initial three participants who completed the study and remained on treatment for multiple years have continued to show no progression with the longest duration of therapy to date of more than seven years. These are extremely encouraging results that provide increased understanding of gildeuretinol’s potential

to preserve sight. It is challenging to identify and enroll early-stage patients before they develop symptoms, and we are grateful to these patients and their families for participating in this study."

TEASE-3, the first clinical trial in early-stage Stargardt disease, is an open-label study of gildeuretinol. Participants have early signs of disease visible on retinal imaging but have not begun experiencing symptoms of vision loss. Fundus autofluorescence (FAF) imaging and other outcome measures are used to assess the extent to which gildeuretinol affects disease progression. Year-over-year progression is assessed, and the primary endpoint is a measure of progression after the first two years of treatment. Following the initial two-year treatment, patients can continue to receive gildeuretinol as part of an extension study. TEASE-3 has enrolled a total of seven patients to date.

Stargardt disease is a serious cause of severe vision impairment in children and young adults, with an estimated 30,000 people diagnosed in the U.S. There is no FDA-approved treatment. In individuals with Stargardt disease, the ABCA4 protein is defective. This defect in the protein results in the accumulation of toxic vitamin A dimers that irreversibly damage the retina, resulting in progressive vision loss.

About the TEASE Program

The Tolerability and Effects of ALK-001 on Stargardt diseasE (TEASE) studies consist of four independent clinical studies of oral gildeuretinol (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 21.6% reduction in the growth rate of retinal atrophic lesions area (square root) ($p<0.001$), and a 29.5% 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 moderate Stargardt disease, expected to read out topline data in 2025. 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. TEASE-4 is an open-label extension study.

About Gildeuretinol Acetate (ALK-001)

Oral gildeuretinol acetate (ALK-001) is a new chemical entity designed to reduce the dimerization of vitamin A without modulating the visual cycle. 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, Rare Pediatric Disease, Fast Track and Orphan Drug designations for Stargardt disease from the U.S. Food and Drug Administration. A study (SAGA) of gildeuretinol in 198 patients with geographic atrophy (GA) secondary to age-related macular degeneration (AMD) demonstrated a meaningful trend in the reduction of lesion growth rate and

demonstrated a functional benefit in low luminance visual acuity (LLVA). In studies, gildeuretinol demonstrated a favorable safety and tolerability profile.

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, with the purpose to protect the sight of individuals impacted by retinal diseases. Alkeus' breakthrough-designated lead candidate, gildeuretinol acetate (ALK-001), is a new chemical entity 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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