FOR IMMEDIATE RELEASE

Alkeus Pharmaceuticals Presents Gildeuretinol Data During the Association for Research in Vision and Ophthalmology (ARVO) 2025 Annual Meeting May 4-8, 2025

Participants treated with gildeuretinol in the SAGA study in geographic atrophy (GA) secondary to age-related macular degeneration (AMD) showed a slower decline in vision-related quality of life and functional reading scores compared to placebo, demonstrating functional benefit

CAMBRIDGE, Mass., May 7, 2025 – Alkeus Pharmaceuticals, Inc., a biopharmaceutical company dedicated to preserving the sight of individuals impacted by retinal diseases, today announced additional data from its Phase 2 clinical study (SAGA) in geographic atrophy (GA) secondary to age-related macular degeneration (AMD) in which patients treated with oral gildeuretinol had a slower decline in VFQ-25 (vision-related quality of life) and FRI (functional reading independence) index scores compared to placebo at month 24. These data were presented at the Association for Research in Vision and Ophthalmology (ARVO) 2025 Annual Meeting being held May 4-8, in Salt Lake City.

“We are pleased with these results from the SAGA study which showed that gildeuretinol provides meaningful functional benefits to people living with GA,” said Seemi Khan, M.D., M.P.H., M.B.A., Chief Medical Officer of Alkeus Pharmaceuticals. “GA secondary to AMD and Stargardt disease share a common pathophysiology, the accumulation of vitamin A dimers that are toxic to the retina. Importantly, these data, along with the positive results reported from our TEASE clinical program evaluating gildeuretinol for Stargardt disease, provide additional clinical evidence supporting the unique mechanism of gildeuretinol.”

In SAGA, a placebo-controlled, double masked Phase 2 study of gildeuretinol in GA secondary to AMD, gildeuretinol showed a clinically meaningful trend in slowing GA growth rate of 13.4% from 0 to 24 months (p=0.075), the study’s primary endpoint. In a sensitivity analysis, gildeuretinol showed a statistically significant reduction in the GA lesion growth rate of 15.3% vs. placebo from 6 to 24 months (p=0.047). Gildeuretinol also demonstrated a statistically significant visual function improvement, showing 4.4 fewer letters lost (p=0.031) in low luminance visual acuity (LLVA) over 24 months, the first key secondary endpoint. A trend toward functional benefit in best corrected visual acuity (BCVA) was demonstrated at 24 months with 3.3 fewer letters lost (p=0.099), also a key secondary endpoint.

The majority of adverse events were mild or moderate. The safety profile demonstrated no reports of delayed dark adaptation, chromatopsia, or vasculitis.

“For my patients living with GA secondary to AMD, the symptoms can have a profound impact on their daily activities and their independence,” said David Boyer, M.D., Retina-Vitreous Associates Medical Group in Los Angeles. “I’m very encouraged by these results which show meaningful trends in improvement when treated with investigational oral gildeuretinol. These

results demonstrate that gildeuretinol should be further evaluated as a potential systemic treatment of GA secondary to AMD."

In addition, data from the TEASE-1 study of gildereutinol for Stargardt disease was presented on May 5 by Benjamin Bakall, M.D., Ph.D., Associated Retina Consultants of Phoenix. The study showed daily oral gildeuretinol significantly slowed the growth of atrophic retinal lesions in Stargardt disease by a 21.6% reduction versus the untreated group ($p<0.001$). This treatment effect was maintained across all pre-specified sensitivity analyses. Gildeuretinol was well tolerated and demonstrated a consistent safety profile across all studies to date.

"As a physician, I find it frustrating to be without effective options for patients who are losing their central vision due to Stargardt disease, which currently has no approved treatment," said Dr. Bakall, a primary investigator for the study. "I'm extremely encouraged by these data showing that gildeuretinol significantly slowed the disease progression and was generally well-tolerated for the two-year treatment period."

About Alkeus Pharmaceuticals

Alkeus Pharmaceuticals, Inc. is a private biopharmaceutical company dedicated to preserving the sight of individuals impacted by retinal diseases. Based in Cambridge, Mass., Alkeus is backed by institutional investors led by Bain Capital Life Sciences. Alkeus is developing therapies for serious diseases of the eye with high unmet need, with the purpose to preserve the sight of individuals impacted by retinal diseases. Alkeus' breakthrough-designated lead candidate, gildeuretinol acetate (ALK-001), is a new molecular entity currently being evaluated in clinical trials for the treatment of Stargardt disease and for geographic atrophy secondary to age-related macular degeneration.

About Gildeuretinol Acetate (ALK-001)

Oral gildeuretinol acetate (ALK-001) is a new molecular entity designed to reduce the dimerization of vitamin A without modulating the visual cycle. Gildeuretinol is currently being evaluated in clinical trials for the treatment of Stargardt disease and for geographic atrophy secondary to age-related macular degeneration. 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.

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, and is complete. The TEASE-2 trial is an ongoing, fully enrolled, randomized, double-masked, placebo-controlled trial in 80 patients with moderate Stargardt disease, and is expected to read out topline data in 2025. TEASE-3, the 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 the SAGA study

The Study of ALK-001 in GA secondary to age-related macular degeneration (SAGA) was a 24-month, double-masked, randomized, placebo-controlled trial to investigate safety, pharmacokinetics, tolerability and efficacy in 198 patients with geographic atrophy secondary to age-related macular degeneration and is complete.

For further information, contact:

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