FOR IMMEDIATE RELEASE

Alkeus Pharmaceuticals Announces Positive Gildeuretinol Data Will Be Presented During the 12th International FLORetina ICOOR Congress December 5–8 in Florence, Italy

CAMBRIDGE, Mass., December 5, 2024 – Alkeus Pharmaceuticals, Inc. today announced that results from its clinical studies of investigational oral gildeuretinol for the treatment of Stargardt disease (TEASE) will be presented at the 12th International FLORetina ICOOR Congress being held December 5-8 in Florence, Italy.

Oral Presentation:

- **Gildeuretinol Slows Progression of Stargardt Disease: The TEASE Program**
  RETINA FUTURA - SESSION 1
  Date: December 6, 2024
  Time: 12:10 - 12:14 GMT+1
  Presenter: Philip Ferrone, M.D., Vitreoretinal Consultants of New York

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).
For further information, please contact:

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