FOR IMMEDIATE RELEASE

Alkeus Pharmaceuticals Announces First Patient Dosed in Pivotal Global Phase 3 NORTHSTAR Study of Oral Gildeuretinol in Stargardt Disease

- NORTHSTAR will evaluate the efficacy and safety of investigational oral gildeuretinol, designed to reduce the formation of toxic vitamin A dimers, a proven mechanism of Stargardt disease.

- The study will assess gildeuretinol's potential to reduce the growth rate of retinal atrophic lesions and to preserve visual acuity.

- Alkeus aims to enroll approximately 230 participants globally in the study between the ages of 8 and 45 living with advanced Stargardt disease, building on previously observed findings across more than 400 patients treated with gildeuretinol to date.

- Gildeuretinol has been well-tolerated in prior studies, and there were no reports of chromatopsia, dark adaptation delays, or night blindness, consistent with gildeuretinol's unique mechanism of action that does not disrupt the visual cycle.

CAMBRIDGE, Mass., June 8, 2026 – Alkeus Pharmaceuticals, Inc., a biopharmaceutical company dedicated to preserving the sight of individuals impacted by retinal diseases, today announced that the first patient has been dosed in the global Phase 3 NORTHSTAR Study of oral gildeuretinol (ALK-001) for the treatment of Stargardt disease, a rare, progressive inherited retinal disease that causes irreversible central vision loss in children and adults, and with no approved treatment option.

"We are tremendously excited to begin dosing in our pivotal global Phase 3 NORTHSTAR study evaluating oral gildeuretinol in Stargardt disease, which represents an important step forward in the development of a much-needed treatment for patients impacted by this severely debilitating disease," said Michel Dahan, President and CEO of Alkeus Pharmaceuticals. "We are particularly excited about the potential of gildeuretinol to preserve visual acuity as observed in our studies to date. This Phase 3 program is designed to demonstrate clinically meaningful outcomes for patients, retina specialists, and regulators that show preservation of vision."

The NORTHSTAR Study (NCT07419334) is a randomized, placebo-controlled, double-masked 24-month trial designed to evaluate efficacy, safety and pharmacokinetics of gildeuretinol in people living with advanced Stargardt disease and atrophic lesions at baseline. The primary endpoint is the rate of growth of atrophic lesions from months 6 to 24 comparing gildeuretinol to placebo. The key secondary endpoint is the preservation of visual acuity as measured by low luminance visual acuity (LLVA).

“We are excited to enroll the first participant in the NORTHSTAR Study, which marks an important milestone for those living with Stargardt disease who have no approved treatment options,” said David R.P. Almeida, M.D., Principal Investigator at Erie Retina Research, Erie, Pa., where the first participant in the global study received the first dose. “There remains a significant unmet need in Stargardt disease, and continued progress in research is important for patients and families affected by this condition. This study demonstrates a shared commitment to better understand the disease and to evaluate gildeuretinol’s potential as a treatment for Stargardt disease.”

Fundus autofluorescence (FAF) will be used to measure the growth of atrophic lesions, areas where retinal cells have been lost. Building on previously observed anatomical and functional findings across the approximately 400 patients that have received gildeuretinol to date, LLVA will be utilized to evaluate participants’ vision in dim lighting conditions, which is expected to be more sensitive than best-corrected visual acuity (BCVA) and has the potential to detect changes in visual acuity when vision has started declining. Gildeuretinol has been well-tolerated in prior studies, which have included patients as young as 8 years old and a treatment duration as long as seven plus years. In these trials, there were no reports of chromatopsia, dark adaptation delays, or night blindness, consistent with gildeuretinol’s unique mechanism of action that does not disrupt the visual cycle.

“In Stargardt disease, atrophic macular lesions enlarge over time, and measuring the rate of lesion expansion provides an objective and reproducible way to track disease progression,” said Charles C. Wykoff, M.D., Ph.D., retina specialist with Retina Consultants of Texas. “Slowing lesion growth reflects preservation of retinal tissue, which is important for maintaining visual function over time. The inclusion of LLVA as a secondary endpoint is also meaningful, as many patients with Stargardt disease report challenges functioning in low-light environments, even early in the disease course. LLVA may serve as a sensitive functional measure beyond high-contrast visual acuity testing and could provide additional insight into clinically meaningful changes that more closely reflect patients’ day-to-day experiences.”

Isabelle Audo, M.D., Ph.D., Professor of Ophthalmology, Coordinator of the Center for Rare Disease, National Hospital of Ophthalmology (CHNO) of Quinze-Vingts in Paris and in the Department of Genetics at the Institut de la Vision, France, added, “We are excited about the opportunity to investigate whether gildeuretinol may have a statistically significant, clinically meaningful visual acuity preservation in patients. In addition, the global nature of the NORTHSTAR study is particularly important in a rare inherited condition such as Stargardt disease, where international collaboration is necessary to advance research and broaden patient participation. Patients worldwide face substantial unmet needs related to progressive vision loss, and global trials are important to furthering scientific knowledge and therapeutic development in the field.”

The global Phase 3 study design was agreed to by the U.S. Food and Drug Administration and the European Medicine Agency with an estimated 55 sites planned in more than 11 countries.

About Stargardt Disease

Stargardt disease causes severe vision impairment and blindness, primarily in children and young adults, with an estimated 50,000 to 60,000 people affected 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 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. Alkeus' breakthrough-designated lead candidate, gildeuretinol acetate (ALK-001), currently is being evaluated in clinical trials for the treatment of Stargardt disease.

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 being evaluated in clinical trials for the treatment of Stargardt disease and has been studied 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 (FDA). The European Medicines Agency (EMA) has designated gildeuretinol as an orphan medicinal product for the treatment of non-syndromic inherited retinal dystrophies due to defects in the ABCA4 gene, which includes Stargardt disease.

For more information contact:
Media Relations
Media@alkeuspharma.com
Website: www.alkeuspharma.com