FOR IMMEDIATE RELEASE

Alkeus Pharmaceuticals Announces Japan Ministry of Health, Labour and Welfare (MHLW) Has Granted Orphan Drug Designation to Gildeuretinol for Stargardt Disease

CAMBRIDGE, Mass., August 24, 2026 – Alkeus Pharmaceuticals, Inc., a biopharmaceutical company dedicated to preserving the sight of individuals impacted by retinal diseases, today announced that the Ministry of Health, Labour and Welfare (MHLW) in Japan has granted Orphan Drug Designation for oral gildeuretinol for the treatment of Stargardt disease. Stargardt disease is an inherited retinal disease that often begins in childhood or adolescence, and progressively and irreversibly damages the central vision patients rely on to read, recognize faces, drive and live independently. Gildeuretinol currently is being evaluated in the global Phase 3 NORTHSTAR Study.

“Receiving orphan designation from the Japan Ministry of Health, Labour and Welfare reinforces the significant unmet need for people living with Stargardt disease and the potential of gildeuretinol (ALK-001) to be an important new treatment option," said Michel Dahan, President and CEO of Alkeus Pharmaceuticals. “This designation adds to the growing global momentum behind our program as our pivotal global Phase 3 NORTHSTAR study continues to enroll patients. We remain focused on advancing gildeuretinol with urgency and scientific rigor and are working closely with regulators and the global retina community to advance gildeuretinol as quickly and responsibly as possible.”

In Japan, Orphan Drug Designation is granted to drugs intended for the treatment of rare diseases affecting fewer than 50,000 patients in the country and for which there is a high medical need. Benefits include eligibility for subsidies for development costs and potential market exclusivity for up to 10 years if approved.

Alkeus recently announced dosing of the first patient and is actively enrolling its global Phase 3 NORTHSTAR Study of gildeuretinol as a potential treatment for patients with Stargardt disease. The study is evaluating gildeuretinol’s potential to reduce the growth rate of retinal atrophic lesions and to preserve visual acuity.

About the NORTHSTAR Study

The NORTHSTAR Study (NCT07419334) is a Phase 3 randomized, placebo-controlled, double-masked 24-month trial designed to evaluate the efficacy and safety of investigational gildeuretinol in people living with advanced Stargardt disease. 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). Alkeus aims to enroll approximately 230 participants globally in the study between the ages of 8 and 45, building on previously observed findings across more than 400 patients treated with gildeuretinol to date.

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. The Ministry of Health, Labour and Welfare (MHLW) in Japan has granted Orphan Drug Designation for gildeuretinol for the treatment of Stargardt disease.

About Stargardt Disease

Stargardt disease is a rare, inherited and progressive retinal disease that often begins in childhood or early adulthood. It is the most common form of inherited juvenile macular degeneration and is estimated to affect approximately 1 in 8,000 to 10,000 individuals worldwide. The disease damages the macula, the central portion of the retina responsible for detailed vision, and can progressively affect patients' ability to read, recognize faces, drive and live independently. There are currently no FDA-approved therapies for Stargardt disease.

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. Tarsus Pharmaceuticals, Inc. (Nasdaq: TARS) announced on August 6, 2026, that it has entered into a definitive agreement to acquire Alkeus. The pending transaction is expected to close in 2026, subject to the expiration or termination of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act and the satisfaction of other customary closing conditions. There can be no assurances that the pending acquisition of Alkeus will be consummated on the terms and in the timing described herein or at all. 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 a Phase 3 clinical trial for the treatment of Stargardt disease.

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