Source: *Alkeus Pharmaceuticals*

July 14, 2021 07:00 ET

FDA Grants Alkeus Pharmaceuticals Breakthrough Therapy Designation for ALK-001 (C20-D3-vitamin A) for the Treatment of Stargardt Disease

- Only drug to receive Breakthrough Therapy Designation for Stargardt Disease
- Alkeus Pharmaceuticals to discuss next steps toward filing an NDA with the FDA
- Ongoing clinical trial of ALK-001 in dry age-related macular degeneration

SOMERVILLE, Mass., July 14, 2021 (GLOBE NEWSWIRE) -- Alkeus Pharmaceuticals, Inc., a private, late-stage biopharmaceutical company, today announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation to ALK-001 for the treatment of Stargardt Disease.

ALK-001 (C20-D3-vitamin A) is a chemically-modified form of vitamin A developed to treat multiple retinal degenerative diseases. The investigational treatment is taken as a once-a-day. Clinical data indicates that ALK-001 safely slows the progression of Stargardt while preserving the normal visual cycle. ALK-001 is the only drug to receive Breakthrough Therapy Designation for Stargardt Disease.

Stargardt disease is a progressive inherited retinal degenerative disease that causes irreversible vision loss leading to blindness. An estimated 40 to 60,000 people in the United States have this rare and serious condition. Symptoms typically begin in childhood or adolescence. There is currently no approved therapy for Stargardt disease. Almost everyone diagnosed with the disease will become legally blind.

“Obtaining Breakthrough Therapy Designation is a transformative milestone in Stargardt disease,” said Leonide Saad, PhD, CEO of Alkeus Pharmaceuticals. “The results from our Phase 2 trial provide a strong basis for regulatory filing and approval of ALK-001 for the treatment of Stargardt disease. We look forward to working with the FDA and other regulatory agencies so that we can bring ALK-001 to patients as quickly as possible.”

The FDA granted ALK-001 Breakthrough Therapy Designation after reviewing data from Alkeus Pharmaceuticals’ Phase 2, double-masked, randomized, placebo-controlled trial in Stargardt disease. Data from the trial and other ongoing studies are expected to be reported later in the year. Breakthrough Therapy Designation is a process designed by the FDA to expedite the development and review of promising experimental drugs. Previously, the FDA granted ALK-001 Orphan Drug Designation.

Clinical trials for ALK-001 are also underway for patients with dry age-related macular degeneration (AMD). AMD is the number one cause of unpreventable blindness in the USA. Information about clinical trials and expanded access (compassionate use) can be found on the company’s website at www.alkeuspharma.com.

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