



**Source:** Alkeus Pharmaceuticals

January 10, 2024 06:00 ET

## **Alkeus Pharmaceuticals Presents Positive Interim Data Showing Gildeuretinol Halted Stargardt Disease Progression**

- All three patients treated for two years or more have remained free of symptoms and disease progression while taking gildeuretinol.
- TEASE-3 is the first clinical trial in early-stage Stargardt disease, a leading cause of inherited blindness in children and young adults.
- Data to be presented today at 11:30 a.m. PST during the J.P. Morgan Healthcare Conference at The Westin St. Francis San Francisco, Golden Gate room, 32nd floor of the Tower Building.

CAMBRIDGE, Mass., Jan. 10, 2024 (GLOBE NEWSWIRE) -- Alkeus Pharmaceuticals, Inc., today announced positive interim data showing gildeuretinol halted Stargardt disease progression for up to six years. In its ongoing TEASE-3 clinical trial in early-stage Stargardt disease, the first three teenage patients enrolled in TEASE-3 and treated with oral gildeuretinol acetate remained asymptomatic and free of disease progression for their treatment duration ranging between two (one patient) and six years (two patients). In the absence of treatment, the patients were projected to begin experiencing vision loss within two years, following the same disease trajectory as that of their respective older siblings with identical genetic mutations who had not been treated with gildeuretinol.

"The data show the potential of gildeuretinol acetate to arrest Stargardt disease when treatment is started early," said Leonide Saad, Ph.D., President and CEO of Alkeus Pharmaceuticals. "These early results of TEASE-3, together with the results of TEASE-1 in more advanced disease, recently presented at the American Academy of Ophthalmology, show great promise for all patients. We look forward to submitting a new drug application for gildeuretinol acetate in the treatment of Stargardt disease."

Alkeus will present an update at 11:30 a.m. PST today during the J.P. Morgan Healthcare Conference at The Westin St. Francis San Francisco, Golden Gate room, 32nd floor of the Tower Building.

"I am encouraged by the results of the different TEASE trials," said Michael B. Gorin, M.D., Ph.D., Departments of Ophthalmology and Human Genetics at the David Geffen School of Medicine at UCLA. "Both the initial TEASE-1 study and this smaller cohort of early-stage Stargardt patients show clinical benefits from gildeuretinol. However, the dramatic preservation of vision in these younger patients highlights the need to consider the impact of therapies at different stages of the condition. Usually, such asymptomatic or minimally symptomatic individuals are excluded from clinical trials. Being able to compare even this small number of individuals with the severity of disease in their older, untreated siblings who share the same genetic etiology, as well as the same dietary and environmental exposures, allows us to draw meaningful insights into the potential of this medication to preserve sight."

TEASE-3, the first clinical trial in early-stage Stargardt disease, is an open-label study of gildeuretinol. Participants have early signs of disease visible on retinal imaging, but have not begun experiencing symptoms of vision loss. Fundus autofluorescence (FAF) imaging and other outcome measures were used to assess the extent to which gildeuretinol affects disease progression. Year-over-year progression was assessed, as well as age-matched comparison to each participant's untreated sibling with Stargardt disease and identical mutations. The primary endpoint is a measure of progression after the first two years of treatment. After the initial two-year treatment,

patients can continue to receive gildeuretinol for extended periods. TEASE-3 has enrolled a total of five patients to date with no signs of disease progression while on treatment.

Stargardt disease is a leading genetic cause of blindness in children and young adults, with an estimated 30,000 people affected in the U.S. and more than 150,000 worldwide. There is no approved treatment. In individuals with Stargardt disease, the ABCA4 protein is defective. Loss of the protein results in the accelerated dimerization of vitamin A, forming toxic by-products that irreversibly damage the retina, resulting in progressive vision loss.

"Stargardt is a very serious blinding disease that progressively robs the patient of their central vision and unfortunately has no approved treatment," said Jason Menzo, Chief Executive Officer at the Foundation Fighting Blindness. "An efficacious and safe oral therapy that could slow or perhaps stop disease progression for this patient population would be welcomed by those living with Stargardt disease."

### **About the TEASE Trials**

The TEASE trials consist of four independent clinical studies of gildeuretinol (ALK-001) in Stargardt disease, denoted as TEASE-1, TEASE-2, TEASE-3 and TEASE-4. The TEASE-1 study was a randomized, triple-masked, placebo-controlled trial in 50 randomized patients with Stargardt disease. Gildeuretinol met its prespecified primary efficacy endpoint showing a 21% reduction in the growth rate of retinal atrophic lesions ( $p < 0.001$ , square root units, 28% effect for untransformed areas) against untreated patients. Gildeuretinol was well-tolerated. The TEASE-2 trial is an ongoing, fully enrolled, randomized, triple-masked, placebo-controlled trial in 80 patients with Stargardt disease, expected to read out topline data in 2025. TEASE-3 is an open-label study designed to assess gildeuretinol in early-stage Stargardt patients. TEASE-4 is an open-label extension study.

### **About Gildeuretinol Acetate (ALK-001)**

Gildeuretinol acetate (ALK-001) is a novel molecule created as a specialized form of deuterated vitamin A designed to reduce the dimerization of vitamin A without disrupting vision. In preclinical studies, gildeuretinol decreased vitamin A dimerization to the normal rate seen in unaffected individuals and prevented retinal degeneration and blindness in animals with Stargardt disease. In addition to the TEASE trials, a Phase 3 study of gildeuretinol in 200 patients with geographic atrophy (GA) secondary to age-related macular degeneration (AMD) has recently been completed and is expected to read out topline data in 2024. Gildeuretinol has received breakthrough therapy designation and orphan drug designation by the U.S. Food and Drug Administration.

### **About Alkeus Pharmaceuticals**

Alkeus Pharmaceuticals is a private biopharmaceutical company with headquarters in Cambridge, Mass., backed by institutional investors led by Bain Capital. Founded in 2010, Alkeus is developing therapies for serious diseases of the eye with high unmet need. Alkeus' breakthrough-designated lead candidate, gildeuretinol acetate (ALK-001), is 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:

David Setboun, Chief Operating Officer  
Email: [media01@alkeuspharma.com](mailto:media01@alkeuspharma.com)  
Website: [www.alkeuspharma.com](http://www.alkeuspharma.com)