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Alkeus Pharmaceuticals Presents Positive Gildeuretinol Trial Results at American Academy of Ophthalmology, Demonstrating Significant Slowing of Retinal Atrophic Lesions in Stargardt Disease

- Primary endpoint met, demonstrating a 21% slowing ($p<0.001$) in the growth rate of atrophic retinal lesions compared to the untreated arm; 28% reduction ($p<0.001$) using observed atrophic area.
- Patients receiving gildeuretinol showed 80% slower loss of retinal sensitivity compared to published natural history (post-hoc comparison).

SAN FRANCISCO, Nov. 03, 2023 (GLOBE NEWSWIRE) -- Alkeus Pharmaceuticals today announced positive data from its TEASE-1 study, a randomized, double-masked, placebo-controlled clinical trial investigating the effects of gildeuretinol (ALK-001) in patients with Stargardt disease. Christine Kay, M.D., of Vitreo Retinal Associates Florida, will present the trial results during the 2023 American Academy of Ophthalmology (AAO) Retina Subspecialty Day.

Dr. Kay's presentation is titled "Gildeuretinol (ALK-001) Slows the Growth of Atrophic Lesions in ABCA4-Related Stargardt Disease Results of a Randomized, Placebo-Controlled Clinical Trial ("TEASE-1" study)."

Gildeuretinol slowed the growth rate of atrophic retinal lesions by 21% during the two-year study (square root transformation analysis) or 28% using untransformed atrophic areas. The growth rates of atrophic retinal lesions were 0.19 mm/year (0.90 mm^2/year untransformed area) with gildeuretinol and 0.24 mm/year (1.25 mm^2/year) in the untreated arm, mean difference 0.05 mm/year (0.35 mm^2/year) (95% confidence interval, 0.03 to 0.07, $p<0.001$, $R^2 = 0.9996$). In a post-hoc comparison, gildeuretinol-treated patients had an 80% slower rate of retinal sensitivity loss compared to the 330 eyes ProgStar natural history (0.4 dB/year loss in gildeuretinol vs. 2.2 dB/year in ProgStar). In total, over 150 patients have been treated with gildeuretinol for over a year in several studies. The longest treatment duration with gildeuretinol has been five years. In these studies, gildeuretinol has been well tolerated.

"These results underscore the potential of gildeuretinol to transform the lives of patients living with Stargardt disease, a genetic eye condition that leads to significant vision loss and currently has no approved treatment," said Leonide Saad, Ph.D., Co-Founder, President and CEO of Alkeus Pharmaceuticals.

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. 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.

"I have been involved as a principal investigator with the Alkeus TEASE trials since their inception," said Dr. Kay. "It is extremely satisfying and humbling to have the opportunity to provide positive data at the Academy regarding the TEASE-1 trial. I think gildeuretinol has the capacity to benefit patients with Stargardt disease in a clinically-meaningful way. I am hopeful gildeuretinol will be our

first FDA-approved therapy for Stargardt disease, one of the most common inherited retinal diseases."

The TEASE trials consist of four independent clinical trials evaluating gildeuretinol in Stargardt disease. Gildeuretinol addresses the underlying cause of Stargardt disease, by selectively reducing the rate of vitamin A dimerization in the eye. In the TEASE-1 trial, 50 patients with Stargardt disease were randomized 3:2 to gildeuretinol or placebo once daily. After one year of treatment, 50% of placebo patients were randomly selected to cross over to gildeuretinol. The placebo arm was augmented with 59 natural history cases. The primary outcome measure was the growth rate of atrophic retinal lesions.

"BrightFocus Foundation provided early funding for this important research and is hopeful for the life-enhancing option it may provide for people living with Stargardt disease," said Stacy Pagos Haller, President and CEO of BrightFocus Foundation, a global nonprofit organization that funds eye and brain research.

About Alkeus Pharmaceuticals

Alkeus Pharmaceuticals is a biopharmaceutical company headquartered in Cambridge, Massachusetts, founded by Leonide Saad, Ph.D., and Ilyas Washington, Ph.D. Alkeus develops therapies for serious diseases of the eye with high unmet needs. Alkeus' lead candidate, gildeuretinol (ALK-001), is currently being evaluated in clinical trials for the treatment of Stargardt disease and geographic atrophy (GA) secondary to age-related macular degeneration (AMD).

About Gildeuretinol (ALK-001)

Gildeuretinol is a form of vitamin A specifically deuterated to prevent its dimerization in the eye, providing the potential to slow or halt progressive vision loss. In preclinical studies, gildeuretinol decreased vitamin A dimerization by more than 80% and prevented retinal degeneration and vision loss in animals with Stargardt disease.

Additional clinical trials of gildeuretinol are ongoing, including a fully-enrolled, randomized, placebo-controlled trial in 80 patients with intermediate Stargardt disease ("TEASE-2"). Alkeus has recently completed its Phase 3 study of gildeuretinol in 200 patients with geographic atrophy (GA) secondary to age-related macular degeneration (AMD); results are anticipated in the coming months. Gildeuretinol has received breakthrough therapy designation and orphan drug designation from the U.S. FDA.

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