

ALK-001 (C20-D3-Vitamin A) Slows the Growth of Atrophic Lesions in ABCA4-related Stargardt Disease: Results of a Randomized Placebo-Controlled Clinical Trial (TEASE study)

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Topic: RETINA, VITREOUS
Genetic Diseases

Abstract Body:

Purpose: A chemically-modified form of vitamin A, C20-D3-vitamin A (ALK-001), is being investigated to treat Stargardt disease (STGD1), the most common form of juvenile macular dystrophy. **Methods:** In a multicenter, 24-month, double-masked, randomized, placebo-controlled trial, 50 patients with atrophic Stargardt Disease (STGD1) were randomized 30:20 to ALK-001:placebo as a once-a-day pill. After one year, 10 randomly chosen placebo patients crossed over to ALK-001. The prespecified primary efficacy endpoint was the growth rate of retinal atrophic lesions. **Results:** The mean growth rate of retinal atrophic lesions was 0.90 mm²/year (0.19 mm/year in square root units) in the ALK-001 and 1.25 mm²/year (0.24 mm/year) in the untreated arm (p<0.001). ALK-001 was well-tolerated, with no reports of night blindness or impaired dark adaptation, and no clinically-relevant changes in liver enzymes. **Conclusion:** In patients with atrophic STGD1, oral administration of ALK-001 safely slowed the growth rate of retinal atrophic lesions.

Course Format: Paper Only

Financial Interest: No

Industry Employed: No

Study Design: Randomized Controlled Trial

Approved by IRB: Yes

Précis: In a 24-month, double-masked, randomized, placebo-controlled trial evaluating the safety and efficacy of daily, oral administration of ALK-001 (C20-D3-vitamin A) in patients with atrophic Stargardt disease, ALK-001 safely slowed the growth rate of atrophic lesions.

Background Statement: Stargardt disease is the most common inherited juvenile macular dystrophy. No treatment exists. We present results suggesting that daily administration of ALK-001 (C20-D3-vitamin A) may slow the progression of Stargardt disease.

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Other Information

Is the Presenting Author of this submission in a residency program (formal training) or subspecialty training for ophthalmology?

No

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Yes

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