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RECRUITING 

Phase 2 Tolerability and Effects of ALK-001 on Stargardt Disease (TEASE)

ClinicalTrials.gov ID  NCT02402660

Sponsor  Alkeus Pharmaceuticals, Inc.

Information provided by  Alkeus Pharmaceuticals, Inc. (Responsible Party)

Last Update Posted  2024-06-21

Study Details Tab

Study Overview

Brief Summary

The purpose of this study is to determine the long term safety and tolerability of ALK-001 (C20-D3-retinyl acetate), and to explore the effects of ALK-001 on the progression of Stargardt disease in patients between the ages of 8 and 70 years old.

Funding Source - FDA OOPD

Detailed Description

This study evaluates the effects of orally-administered ALK-001 on the progression of Stargardt disease (ABCA4-related). Stargardt disease is a rare genetic disorder that leads to damage to the retina and results in legal blindness. Stargardt disease is caused by a defective ABCA4 gene, which affects the processing of vitamin A in the eye and leads to the formation of toxic vitamin A aggregates (called "vitamin A dimers") in the eye. Vitamin A dimers are thought to contribute to vision loss in Stargardt disease. ALK-001, the investigational drug, is a chemically-modified vitamin A designed as a replacement of vitamin A to prevent the formation of toxic vitamin A dimers in the eye. Trial participants



will receive either ALK-001 or placebo, and follow-up visits will take place periodically for up to 24 months. There is currently no treatment for Stargardt disease.

Official Title

A Phase 2 Multicenter, Double-Masked, Randomized, Placebo-Controlled Study to Investigate the Long Term Safety, Tolerability, Pharmacokinetics and Effects of ALK-001 on the Progression of Stargardt Disease

Conditions ⓘ

- Stargardt Disease
- Stargardt Macular Degeneration
- Stargardt Macular Dystrophy
- Autosomal Recessive Stargardt Disease 1 (ABCA4-related)

Intervention / Treatment ⓘ

- Drug: ALK-001
- Drug: Placebo

Other Study ID Numbers ⓘ

- ALK001-P1002
- [R01FD004098 \(U.S. FDA Grant/Contract \)](https://reporter.nih.gov/quickSearch/R01FD004098) (<https://reporter.nih.gov/quickSearch/R01FD004098>)

Study Start (Actual) ⓘ

2015-08

Primary Completion (Estimated) ⓘ

2024-12

Study Completion (Estimated) ⓘ

2025-03

Enrollment (Estimated) ⓘ

140

Study Type ⓘ

Interventional

Phase ⓘ

Phase 2

Resource links provided by the National Library of Medicine

[MedlinePlus Genetics](https://medlineplus.gov/genetics/) (<https://medlineplus.gov/genetics/>), related topics:

[Stargardt macular degeneration](https://medlineplus.gov/genetics/condition/stargardt-macular-degeneration) (<https://medlineplus.gov/genetics/condition/stargardt-macular-degeneration>), [Age-related macular degeneration](https://medlineplus.gov/genetics/condition/age-related-macular-degeneration) (<https://medlineplus.gov/genetics/condition/age-related-macular-degeneration>).

[Genetic and Rare Diseases Information Center](https://rarediseases.info.nih.gov/gard) (<https://rarediseases.info.nih.gov/gard>), resources:
[Stargardt Disease](https://rarediseases.info.nih.gov/diseases/181/stargardt-disease) (<https://rarediseases.info.nih.gov/diseases/181/stargardt-disease>).

[FDA Drug and Device Resources](https://www.clinicaltrials.gov/fda-links) (<https://www.clinicaltrials.gov/fda-links>).

Contacts and Locations

This section provides the contact details for those conducting the study, and information on where this study is being conducted.

Study Contact

Name: Leonide Saad, PhD

Phone Number:

800-287-2755

Email: trials@alkeus.com

This study has 8 locations

United States

California Locations

 **Los Angeles, California, United States, 90095**

Recruiting

University of California Los Angeles -

Jules Stein Eye Institute

Principal Investigator: Michael Gorin, MD,
PhD

Florida Locations



Participation Criteria

Researchers look for people who fit a certain description, called [eligibility criteria](#). Some examples of these criteria are a person's general health condition or prior treatments.

For general information about clinical research, read [Learn About Studies](#) (<https://www.clinicaltrials.gov/study-basics/learn-about-studies>).

Eligibility Criteria

Description

Simplified Inclusion Criteria:

- Male or female between 8 and 70 years old (inclusive), with any visual acuity
- Has a clinical diagnosis of typical autosomal recessive Stargardt macular dystrophy (STGD1)
- Has provided a genetic report indicating at least two ABCA4 disease-causing mutations. When only one ABCA4 disease-causing mutation is reported, sponsor's permission will be required.
- At least one eye (called the "primary study eye") must have at least one well-demarcated area of significantly reduced autofluorescence as imaged by fundus autofluorescence (FAF), have decreased retinal sensitivity as measured by microperimetry, or have maculopathy expected to progress over the duration of the study
- Primary study eye must have clear ocular media and adequate pupillary dilation, including no allergy to dilating eyedrops, to permit good quality retinal imaging
- Healthy as judged by investigator
- Able and willing to comply with study requirements, restrictions and instructions and is likely to complete the 24-month study
- Has signed and dated the informed consent forms (or assent where appropriate) to participate
- Female of childbearing potential has signed the informed consent about birth defects or attestation on contraception requirements

Main Exclusion Criteria:

- Has taken disallowed items (supplement containing vitamin A or beta-carotene, liver-based products, or prescription oral retinoid medications) over the past 30 days
- Is lactating, pregnant, or has a positive serum or urine pregnancy test at screening or at randomization
- Has concurrent medical condition or history, which in the opinion of the investigator, is likely to prevent compliance with the protocol and/or interfere with absorption of ALK-001 or study procedures
- Has clinically significant abnormal laboratory result(s) at screening
- Has active or historical acute or chronic liver disorder
- Has active or historical ocular disorder in the primary study eye that, in the opinion of the investigator, may confound assessment of the retina morphologically or functionally (this could include for example cataract surgery within the past 6 months, choroidal neovascularization (CNV), glaucoma, recurring uveitis, diabetic retinopathy, other retinal disease, etc.)
- Has had intraocular surgery or injections in the primary study eye within 90 days of the screening visit

- Has a clinically significant abnormal electrocardiogram (ECG), or has a corrected QT interval (QTc) that is 450 ms or greater

Ages Eligible for Study ⓘ

8 Years to 70 Years (Child, Adult, Older Adult)

Sexes Eligible for Study ⓘ

All

Accepts Healthy Volunteers ⓘ

No

Study Plan

This section provides details of the study plan, including how the study is designed and what the study is measuring.

How is the study designed?

Design Details

Primary Purpose ⓘ : Treatment

Allocation ⓘ : Randomized

Interventional Model ⓘ : Parallel Assignment

Masking ⓘ : Quadruple (Participant, Care Provider, Investigator, Outcomes Assessor)

Arms and Interventions

Participant Group/Arm i	Intervention/Treatment i
Experimental: ALK-001 Daily, oral administration of one capsule. See details below.	Drug: ALK-001 - Daily, oral administration for 24 months - Other Names: - C20-D3-Retinyl Acetate - C20 Deuterated vitamin A
Placebo Comparator: Placebo Daily, oral administration of one capsule. See details below.	Drug: Placebo Daily, oral administration for 24 months

What is the study measuring?

Primary Outcome Measures i

Outcome Measure	Measure Description	Time Frame
Safety and tolerability of 24 months of daily dosing of ALK-001 assessed by Incidence and/or clinically significant		From baseline to 24 months

changes of a combination of ocular and non-ocular adverse events		
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Secondary Outcome Measures ⓘ

Outcome Measure	Measure Description	Time Frame
Effects of ALK-001 on the progression of Stargardt disease	Combination of changes in atrophic lesion size, best corrected visual acuity (BCVA), and ocular assessments.	From baseline to 24 months
Pharmacokinetic profile of ALK-001 derived from the concentrations of ALK-001 and metabolites in plasma		Up to 24 months

Collaborators and Investigators

This is where you will find people and organizations involved with this study.

Sponsor ⓘ

Alkeus Pharmaceuticals, Inc.

Investigators ⓘ

- Study Director: Hendrik Scholl, MD, University of Basel

- Study Director: Leonide Saad, PhD, Alkeus Pharmaceuticals, Inc.

Publications

General

These publications are provided voluntarily by the person who enters information about the study and may be about anything related to the study.

- [Ma L, Kaufman Y, Zhang J, Washington I. C20-D3-vitamin A slows lipofuscin accumulation and electrophysiological retinal degeneration in a mouse model of Stargardt disease. J Biol Chem. 2011 Mar 11;286\(10\):7966-7974. doi: 10.1074/jbc.M110.178657. Epub 2010 Dec 14.](https://pubmed.ncbi.nlm.nih.gov/2156790/) (https://pubmed.ncbi.nlm.nih.gov/2156790/)
- [Kaufman Y, Ma L, Washington I. Deuterium enrichment of vitamin A at the C20 position slows the formation of detrimental vitamin A dimers in wild-type rodents. J Biol Chem. 2011 Mar 11;286\(10\):7958-7965. doi: 10.1074/jbc.M110.178640. Epub 2010 Nov 12.](https://pubmed.ncbi.nlm.nih.gov/21075840/) (https://pubmed.ncbi.nlm.nih.gov/21075840/)
- [Mihai DM, Jiang H, Blaner WS, Romanov A, Washington I. The retina rapidly incorporates ingested C20-D\(3\)-vitamin A in a swine model. Mol Vis. 2013 Jul 25;19:1677-83. Print 2013.](https://pubmed.ncbi.nlm.nih.gov/23914132/) (https://pubmed.ncbi.nlm.nih.gov/23914132/)
- [Charbel Issa P, Barnard AR, Herrmann P, Washington I, MacLaren RE. Rescue of the Stargardt phenotype in Abca4 knockout mice through inhibition of vitamin A dimerization. Proc Natl Acad Sci U S A. 2015 Jul 7;112\(27\):8415-20. doi: 10.1073/pnas.1506960112. Epub 2015 Jun 23.](https://pubmed.ncbi.nlm.nih.gov/26106163/) (https://pubmed.ncbi.nlm.nih.gov/26106163/)
- [Saad L, Washington I. Can Vitamin A be Improved to Prevent Blindness due to Age-Related Macular Degeneration, Stargardt Disease and Other Retinal Dystrophies? Adv Exp Med Biol. 2016;854:355-61. doi: 10.1007/978-3-319-17121-0_47.](https://pubmed.ncbi.nlm.nih.gov/26427432/) (https://pubmed.ncbi.nlm.nih.gov/26427432/)
- [Zhang D, Robinson K, Washington I. C20D3-Vitamin A Prevents Retinal Pigment Epithelium Atrophic Changes in a Mouse Model. Transl Vis Sci Technol. 2021 Dec 1;10\(14\):8. doi: 10.1167/tvst.10.14.8.](https://pubmed.ncbi.nlm.nih.gov/34878528/) (https://pubmed.ncbi.nlm.nih.gov/34878528/)

Study Record Dates

These dates track the progress of study record and summary results submissions to ClinicalTrials.gov. Study records and reported results are reviewed by the National Library of Medicine (NLM) to make sure they meet specific quality control standards before being posted on the public website.

Study Registration Dates
First Submitted ⓘ 2015-03-09
First Submitted that Met QC Criteria ⓘ 2015-03-27
First Posted (Estimated) ⓘ 2015-03-30

Study Record Updates
Last Update Submitted that met QC Criteria ⓘ 2024-06-18
Last Update Posted ⓘ 2024-06-21
Last Verified ⓘ 2024-06

More Information

Terms related to this study
Additional Relevant MeSH Terms Retinal Degeneration Retinal Diseases Eye Diseases Eye Diseases, Hereditary Genetic Diseases, Inborn Macular Degeneration Stargardt Disease Vitamins Micronutrients

Physiological Effects of Drugs

Protective Agents

Anticarcinogenic Agents

Antineoplastic Agents

Adjuvants, Immunologic

Immunologic Factors

Vitamin A

Retinol acetate