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Phase 3 Study of ALK-001 in Geographic Atrophy (SAGA)

ClinicalTrials.gov ID  NCT03845582

Sponsor  Alkeus Pharmaceuticals, Inc.

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

Last Update Posted  2024-01-30

Study Details Tab

Study Overview

Brief Summary

This is a double-masked, multicenter, randomized, placebo-controlled clinical trial, evaluating the efficacy and safety of ALK-001 in participants with Geographic Atrophy (GA) secondary to age-related macular degeneration (AMD).

Up to 200 participants will receive ALK-001 while up to 100 participants will receive a placebo.

Detailed Description

There is no treatment available for Geographic Atrophy secondary to AMD. AMD is characterized by an age-related degeneration of the retina.

The root cause for this degeneration or why some people develop AMD while others do not, is unknown. Over 20 years ago, it was hypothesized that the dimerization of vitamin A may be a significant contributor to the etiology of AMD. The eye indeed uses vitamin A as a cofactor to sense light, and a striking chemical signature of the aging and degenerating retina is the accumulation of vitamin A



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dimers in the retinal pigment epithelium (RPE) and the underlying Bruch's membrane. In rodent models, high levels of vitamin A dimers correlate with poor retinal health, and a variety of mechanisms have been proposed by which vitamin A dimers may induce retinal toxicity. It has been argued that these mechanisms participate in the development and progression of AMD. ALK-001, the study drug, is a modified form of vitamin A. When taken once a day as a capsule, it replaces natural vitamin A in the body with one that forms vitamin A dimers more slowly. This study will measure the extent to which treatment with ALK-001 slows the progression of Geographic Atrophy.			
Official Title			
A Phase 2/3 Multicenter, Randomized, Double-masked, Parallel-group, Placebo-controlled Study to Investigate the Safety, Pharmacokinetics, Tolerability, and Efficacy of ALK-001 in Geographic Atrophy Secondary to Age-related Macular Degeneration			
Conditions ⓘ			
		Geographic Atrophy Age Related Macular Degeneration AMD Atrophy, Geographic	
Intervention / Treatment ⓘ			
		- Drug: ALK-001 oral capsule - Drug: Placebo oral capsule	
Other Study ID Numbers ⓘ			
		ALK001-P3001	
Study Start (Actual) ⓘ			
		2019-05-07	
Primary Completion (Actual) ⓘ			
		2023-07-25	
Study Completion (Estimated) ⓘ			
		2024-04-01	
Enrollment (Estimated) ⓘ			
		300	
Study Type ⓘ			
		Interventional	

Phase 1

Phase 3

Resource links provided by the National Library of Medicine

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

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

[MedlinePlus](https://medlineplus.gov/) (<https://medlineplus.gov/>) related topics: [Macular Degeneration](https://medlineplus.gov/maculardegeneration.html) (<https://medlineplus.gov/maculardegeneration.html>).

[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.

This study has 1 location

United States**Massachusetts Locations**

 **Somerville, Massachusetts, United States, 02144**

Coordinating Center

**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

Major Inclusion Criteria:

- At least one eye with geographic atrophy secondary to dry age-related macular degeneration (AMD)

Major Exclusion Criteria:

- Medical condition, which may interfere with the progression of GA, prevent performance of study procedures, compliance with protocol, or continuous participation in the study

Ages Eligible for Study ⓘ

60 Years and older (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 ⓘ	Intervention/Treatment ⓘ
Experimental: ALK-001 Capsule	Drug: ALK-001 oral capsule - Daily administration for 24 months - Other Names: - C20-D3-Retinyl Acetate - C20 Deuterated vitamin A
Placebo Comparator: Placebo Capsule	Drug: Placebo oral capsule Daily administration for 24 months

What is the study measuring?

Primary Outcome Measures ⓘ

Outcome Measure	Measure Description	Time Frame
Growth rate of GA lesions, as assessed by Fundus		Baseline to 24 months

Autofluorescence (FAF)		
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Secondary Outcome Measures

Outcome Measure	Measure Description	Time Frame
Safety and tolerability, as assessed by evaluation of adverse events		Baseline to 24 months
Pharmacokinetics, as assessed by plasma concentrations of ALK-001 and metabolites		Baseline to 24 months
Incidence of choroidal neovascularization (CNV)		Baseline to 24 months
Changes in Visual Acuity		Baseline to 24 months
Changes in Reading Speed		Baseline 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: 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.

- [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).
- [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).

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 ⓘ

2019-02-15

First Submitted that Met QC Criteria ⓘ

2019-02-18

First Posted ⓘ

2019-02-19

Study Record Updates

Last Update Submitted that met QC Criteria ⓘ

2024-01-29

Last Update Posted ⓘ

2024-01-30

Last Verified ⓘ

2024-01

More Information

Terms related to this study

Additional Relevant MeSH Terms

- Retinal Degeneration
- Retinal Diseases
- Eye Diseases
- Pathological Conditions, Anatomical
- Macular Degeneration
- Geographic Atrophy
- Atrophy
- Vitamins
- Micronutrients
- Physiological Effects of Drugs
- Protective Agents
- Anticarcinogenic Agents
- Antineoplastic Agents

Adjuvants, Immunologic
Immunologic Factors
Vitamin A
Retinol acetate

Plan for Individual Participant Data (IPD)

Plan to Share Individual Participant Data (IPD)?

Undecided

Drug and device information, study documents, and helpful links

Studies a U.S. FDA-Regulated Drug Product

Yes

Studies a U.S. FDA-Regulated Device Product

No