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COMPLETED ⓘ

Effect of DHA Supplements on Macular Function in Patients With Stargardt Macular Dystrophy and Stargardt-like Macular Dystrophy

ClinicalTrials.gov ID ⓘ NCT00060749

Sponsor ⓘ National Eye Institute (NEI)

Information provided by ⓘ National Institutes of Health Clinical Center (CC)

Last Update Posted ⓘ 2017-07-02

Study Details Tab

Study Overview

Brief Summary

This study will evaluate whether docosahexaenoic acid (DHA) dietary supplementation can improve macular function in patients with Stargardt macular dystrophy and Stargardt-like macular dystrophy. Stargardt macular dystrophy is a recessive inherited trait that causes a severe form of macular degeneration. (The macula is the center part of the retina in the back of the eye that is responsible for fine vision.) The disorder begins in late childhood and progresses to a significant decrease in central vision. One of the earliest signs of the disorder is accumulation in and under the macula of a fatty pigment called lipofuscin. Stargardt-like macular dystrophy is a dominant inherited trait involving loss of central vision, but it begins later than Stargardt macular dystrophy, and the accumulation of lipofuscin extends beyond the central region of the macula. DHA is a fatty acid that is essential for normal brain and eye development. It is normally found in the diet, but not in large amounts. Supplements may help prevent or slow the progression of some eye diseases.

Patients with autosomal dominant Stargardt-like macular dystrophy or autosomal recessive Stargardt macular dystrophy are eligible for this study. Candidates will be screened with the following tests and procedures:

- Medical history and physical examination.
- Blood test to measure levels of DHA and vitamins.
- Eye examination: The patient's vision and eye pressure are tested, then the pupils are dilated to examine structures inside the eye. Photographs are also taken.
- Visual field test: The patient looks at a tiny spot of light projected onto a white screen and is asked to note when other lights appear at other places on the screen.
- Electroretinogram (ERG): An electrode (small silver disk) is taped to the patient's forehead. Drops are given to numb the eyes and special contact lenses are inserted in the eyes. For the first part of the test, the patient looks at the center of a black and white

checkerboard screen that flickers for 30 seconds at a time. This is repeated 16 or more times. For the second part of the test, the patient looks inside a sphere, in which flashes of light flicker for 20 seconds at a time. This is repeated four or more times. The contact lenses sense small electrical signals generated by the retina during the tests.

Participants will begin taking DHA capsules or a placebo (look-alike capsules with no active ingredient) from 1 week to 3 months after enrolling in the study and will repeat several of the screening tests at follow-up visits scheduled 3, 6, 9, 12, and 15 months after they start taking the capsules. They will also be interviewed about any treatment side effects.

Detailed Description

We propose to undertake a double-masked, randomized, placebo-controlled, crossover study on the effect of docosahexaenoic acid (DHA) dietary supplementation in subjects with macular dystrophy to determine whether DHA can improve macular function. Subjects will receive either oral DHA supplementation (5x200 mg BID, 2,000 mg/day) or placebo. Subjects will 'crossover' to the opposite treatment twice during this study. Primary outcomes will measure the change in macular function during periods with and without DHA supplementation.

Zhang and colleagues found a mutation in the gene, ELOVL4 (elongation of the very long chain fatty acid-4), in individuals with Stargardt-like macular dystrophy. The gene is presumed to function in the pathway of synthesis of very long chain polyunsaturated fatty acids, including DHA. DHA is the major very long chain polyunsaturated fatty acid of the retina. As our North American diet is poor in DHA, we hypothesize that a DHA dietary supplement might improve macular function in individuals with the ELOVL4 mutation. Since the effect of DHA supplementation may be non-specific, we propose to study a second cohort with Stargardt macular dystrophy, which has a different genotype involving a different metabolic pathway in the eye, but presents with a similar phenotype. Two cohorts of up to 10 subjects for analysis will be recruited from patients with either Stargardt-like macular dystrophy or Stargardt macular dystrophy.

Official Title

Investigation of the Effect of Dietary Docosahexaenoic Acid (DHA) Supplementation on Macular Function in Subjects With Autosomal Dominant Stargardt-Like and Autosomal Recessive Stargardt Macular Dystrophy

Conditions

Macular Degeneration

Intervention / Treatment

- Drug: Docosahexaenoic Acid (DHA) Dietary Supplement

Other Study ID Numbers

- 030179
- 03-EI-0179

Study Start

2003-05-05

Study Completion

2007-12-10

Enrollment

22

Study Type

Interventional

Phase

Phase 1

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>).

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

[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.

This study has 1 location

United States

Maryland Locations

 **Bethesda, Maryland, United States, 20892**
National Institutes of Health Clinical Center, 9000 Rockville Pike



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) (<https://www.clinicaltrials.gov/study-basics/learn-about-studies>).

Eligibility Criteria

Description

- INCLUSION CRITERIA:

To be eligible to enroll in this study, a prospective participant must satisfy the following inclusion criteria.

1. Understand and sign the informed consent.
2. Able to comply with all study procedures (likely to exclude participants less than 10 years of age, but not necessarily).

Autosomal Recessive Stargardt Macular Dystrophy Participants (must be observed in at least one study eye):
3. Have a pattern of inheritance that indicates autosomal recessive inheritance.
4. Have a phenotype consistent with the diagnosis of autosomal recessive Stargardt macular dystrophy including the following clinical features: fundus examination showing bilateral central maculopathy and/or fundus flecks, or characteristic changes on an intravenous fluorescein angiogram.

Autosomal Dominant Stargardt-like Macular Dystrophy Participants (must be observed in at least one study eye):
5. Have a pattern of inheritance that indicates autosomal dominant inheritance.
6. Have a phenotype consistent with the diagnosis of Stargardt-like macular dystrophy that may include: fundus examination showing bilateral central maculopathy and fundus flecks confined to the central macula, or intravenous fluorescein angiogram.

EXCLUSION CRITERIA:

To be eligible to enroll in this study, a prospective participant must not satisfy any of the following exclusion criteria.

1. Have a non-recordable multi-focal ERG.

Ages Eligible for Study ⓘ

(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

Arms and Interventions

Intervention/Treatment ⓘ	
Drug: Docosahexaenoic Acid (DHA) Dietary Supplement	

Collaborators and Investigators

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

Sponsor ⓘ

National Eye Institute (NEI)

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.

- [Zhang K, Kniazeva M, Han M, Li W, Yu Z, Yang Z, Li Y, Metzker ML, Allikmets R, Zack DJ, Kakuk LE, Lagali PS, Wong PW, MacDonald IM, Sieving PA, Figueroa DJ, Austin CP, Gould RJ, Ayyagari R, Petrukhin K. A 5-bp deletion in ELOVL4 is associated with two related forms of autosomal dominant macular dystrophy. Nat Genet. 2001 Jan;27\(1\):89-93. doi: 10.1038/83817.](#) (<https://pubmed.ncbi.nlm.nih.gov/11138005>).

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 ⓘ
2003-05-09
First Submitted that Met QC Criteria ⓘ
2003-05-09
First Posted (Estimated) ⓘ
2003-05-12

Study Record Updates
Last Update Submitted that met QC Criteria ⓘ
2017-06-30
Last Update Posted ⓘ

2017-07-02

Last Verified ⓘ

2007-12-10

More Information

Terms related to this study

Keywords Provided by National Institutes of Health Clinical Center (CC)

Stargardt
Macular Dystrophy
ELOVL4
DHA
Stargardt's Disease
Autosomal Dominant Macular Dystrophy
Autosomal Recessive Macular Dystrophy

Additional Relevant MeSH Terms

Retinal Degeneration
Retinal Diseases
Eye Diseases
Eye Diseases, Hereditary
Genetic Diseases, Inborn
Macular Degeneration
Stargardt Disease