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COMPLETED ⓘ

Phase 1 Pilot Study of 4-MP to Treat Stargardt Macular Dystrophy

ClinicalTrials.gov ID ⓘ NCT00346853

Sponsor ⓘ University of Utah

Information provided by ⓘ University of Utah

Last Update Posted ⓘ 2020-08-05

Study Details Tab

Study Overview

Brief Summary

The purpose of this study is to investigate whether taking 4-methylpyrazole (4-MP, fomepizole, Antizol™) inhibits dark adaptation of the eye. In other words, we are testing if 4-MP slows the processing of vitamin A derivatives in the eye. By slowing down these processes, individuals with Stargardt disease may have better chances of saving their remaining vision. 4-MP has been shown to slow dark adaptation in animals, and is FDA approved for human use to treat individuals with methanol or ethylene glycol (antifreeze) poisoning by shutting down the body's ability to process alcohols. This medication has an excellent safety profile and has been reported to have no short-term or long-term side effects, as long as patients refrain from any alcohol while the medication is in the body. A single dose of 4-MP remains in the body for about 12 hours, and therefore, it may inhibit dark adaptation of your eyes for up to 12 hours. Studying the effects of 4-MP may lead to effective medical treatment to save Stargardt patients' vision, and may also have similar effects in other macular degenerative diseases.

Official Title

Clinical Interventions Against Stargardt Macular Dystrophy: Phase 1 Pilot Study of 4-MP as an Inhibitor of Dark Adaptation

Conditions ⓘ

Macular Dystrophy, Corneal

Intervention / Treatment ⓘ

- Drug: 4-Methylpyrazole
- Other: saline

Feedback

Other Study ID Numbers ⓘ

- 4-MP Dark Adaptation Inhib.

Study Start ⓘ

2005-11

Primary Completion (Actual) ⓘ

2006-05

Study Completion (Actual) ⓘ

2006-09

Enrollment (Actual) ⓘ

10

Study Type ⓘ

Interventional

Phase ⓘ

Phase 1

Resource links provided by the National Library of Medicine

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

[Drug Information](https://dailymed.nlm.nih.gov/dailymed/) (<https://dailymed.nlm.nih.gov/dailymed/>), available for: [Fomepizole](https://dailymed.nlm.nih.gov/dailymed/search.cfm?labeltype=human&query=Fomepizole) (<https://dailymed.nlm.nih.gov/dailymed/search.cfm?labeltype=human&query=Fomepizole>).

[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

Utah Locations

-  Salt Lake City, Utah, United States, 84132
Moran Eye Center, University of Utah



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:

- All nonpregnant, nonlactating adults with normal vision in both eyes

Exclusion Criteria:

- Previous ocular pathologies

Ages Eligible for Study ⓘ

18 Years to 65 Years (Adult, Older Adult)

Sexes Eligible for Study ⓘ

All

Accepts Healthy Volunteers ⓘ

Yes

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 ⓘ : Triple (Participant, Investigator, Outcomes Assessor)

Arms and Interventions

Participant Group/Arm ⓘ	Intervention/Treatment ⓘ	
Experimental: 1	Drug: 4-Methylpyrazole 15 mg/kg dose	
Placebo Comparator: 2 saline	Other: saline	

What is the study measuring?

Primary Outcome Measures ⓘ

Outcome Measure	Measure Description	Time Frame
Dark adaptation inhibition measured 30 minutes after drug infusion using Goldman-Weeker adaptometer.		6 weeks

Collaborators and Investigators

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

Sponsor ⓘ

University of Utah

Investigators ⓘ

- Principal Investigator: Paul S Bernstein, M.D., Ph.D., University of Utah

Publications

Study Results

These publications are provided voluntarily by the person who enters information about the study and are about the study results.

- [Jurgensmeier C, Bhosale P, Bernstein PS. Evaluation of 4-methylpyrazole as a potential therapeutic dark adaptation inhibitor. Curr Eye Res. 2007 Oct;32\(10\):911-5. doi: 10.1080/02713680701616156.](#) (<https://pubmed.ncbi.nlm.nih.gov/17963111>)

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 ⓘ	2006-06-28
First Submitted that Met QC Criteria ⓘ	2006-06-28
First Posted (Estimated) ⓘ	2006-06-30

Study Record Updates	
Last Update Submitted that met QC Criteria ⓘ	2020-08-03
Last Update Posted ⓘ	2020-08-05
Last Verified ⓘ	2020-08

More Information

Terms related to this study	
Keywords Provided by University of Utah	Dark adaptation inhibition Stargardt macular dystrophy 4-Methylpyrazole (4-MP) Antizol Fomepizole
Additional Relevant MeSH Terms	Retinal Degeneration Retinal Diseases Eye Diseases Eye Diseases, Hereditary Genetic Diseases, Inborn Corneal Diseases Macular Degeneration Stargardt Disease Corneal Dystrophies, Hereditary Antidotes Protective Agents Physiological Effects of Drugs Enzyme Inhibitors

Molecular Mechanisms of Pharmacological Action

Fomepizole