A Study to Learn How Stargardt-type Eye Conditions Progress in Children and Adults

NCT07425574

Status	RECRUITING
Sponsor	Astellas Pharma Global Development, Inc.
Enrollment	90 participants

Key Eligibility Criteria

Inclusion (14)

• Participant has a documented clinical diagnosis of macular dystrophy expressing a STGD-type clinical presentation and molecular confirmation, defined as either:

• ABCA4-associated disease: presence of biallelic (pathogenic or likely pathogenic) ABCA4 variants, or one definite disease-causing ABCA4 variant together with a typical phenotype consistent with STGD.

• STGD-like macular dystrophy: presence of one or more pathogenic variants in a gene known to cause macular dystrophy, as appropriate for its expected inheritance mode. Note: All genetic testing should be performed by a Clinical Laboratory Improvement Amendments (CLIA)-certified laboratory or equivalent whenever possible. Acceptable documentation includes a copy of the genetic test report, laboratory certification statement, or clinical notes explicitly referencing CLIA certification. Predicted pathogenic variant of uncertain significance (VUS) that cannot be confirmed by standard laboratory criteria as certainly disease-causing (novel mutations) will be considered on a case by-case basis.

• Participant has sufficiently clear ocular media and adequate pupillary dilation to allow for all imaging procedures.

• Participant has intraocular pressure (IOP) both at screening and baseline of d 21 mmHg measured by applanation tonometry. Note: Participant who is on topical IOP lowering treatment may also be included.

... and 9 more (see full listing online)

Exclusion (39)

• Participant has a known history of significant systemic disease (e.g., uncontrolled hepatitis, pancreatitis, cirrhosis, liver failure, uncontrolled thyroid disease or immunocompromising conditions such as human immunodeficiency virus \[HIV\]) that could impact ocular health or confound study assessments, based on medical history or prior clinical documentation.

• Participant has an autoimmune condition that requires treatment with immunomodulatory therapy and/or biologics that cause immunosuppression.

• Participant has a known diagnosis of diabetes mellitus with a documented hemoglobin A1c (HbA1c) value e 7% 3 months prior to screening and based on available medical records. If the documented HbA1c is e 7% and there is no clinical history of diabetic symptoms, diabetic retinopathy, abnormal renal function (e.g., elevated creatinine), or glycosuria noted in medical records, the participant may be enrolled.

• Participant has a known history of any systemic or metabolic condition, or physical examination finding that may significantly affect ocular health or interfere with the interpretation of study assessments.

• Participant has a history or evidence of severe cardiac disease (e.g., New York Heart Association Functional Class III or IV), clinical evidence of unstable angina, acute coronary syndrome, myocardial infarction or revascularization within 6 months prior to screening.

... and 34 more (see full listing online)

Locations (1 total)

Retina Foundation of the Southwest, Dallas, Texas, United States