MERIT
Your Clinical Endpoint Expert

Clinical Trial Endpoints in Stargardt Disease

Stargardt disease (STGD) is a rare genetic ocular disorder characterized by the accumulation of lipofuscin deposits on the macula, the critical region of the retina responsible for sharp, central vision. This condition is predominantly caused by mutations in the *ABCA4* gene, which disrupts the metabolism of vitamin A, essential for retinal cell function. Although vision loss in STGD typically manifests during childhood, some individuals may not exhibit symptoms until adulthood.

Currently there is no cure for the approximately 1 in 10,000 people worldwide living with STGD. However, researchers are exploring multiple strategies for potential treatments including gene therapy, stem cell therapy, and pharmacological treatments. These trials require reproducible and well-defined endpoints to measure the characteristics and progression of STGD.

MERIT's Inherited Retinal Disease (IRD) Experience

31 TRIALS

MERIT has provided clinical endpoint services for over 31 trials in IRD including STGD

EXPERT READERS

Standardized image analysis by expert readers

REAL-TIME DATA ACCESS

24/7 real-time data access through our cloud-based imaging platform, EXCELSIOR™

Clinical Trial Endpoints

The following endpoints can provide insight into the characteristics and progression of SGTD:

STRUCTURAL ASSESSMENTS

- Retinal atrophy and Ellipsoid Zone (EZ) loss measured quantitatively with Optical Coherence Tomography (OCT)
  - ✓ Length of EZ loss
  - ✓ Multilayer segmentation
  - ✓ Incomplete Retinal Pigment Epithelial and Outer Retinal Atrophy (iRORA)
  - ✓ Multimodal Definite Decreased Autofluorescence (DDAF) region confirmed with OCT

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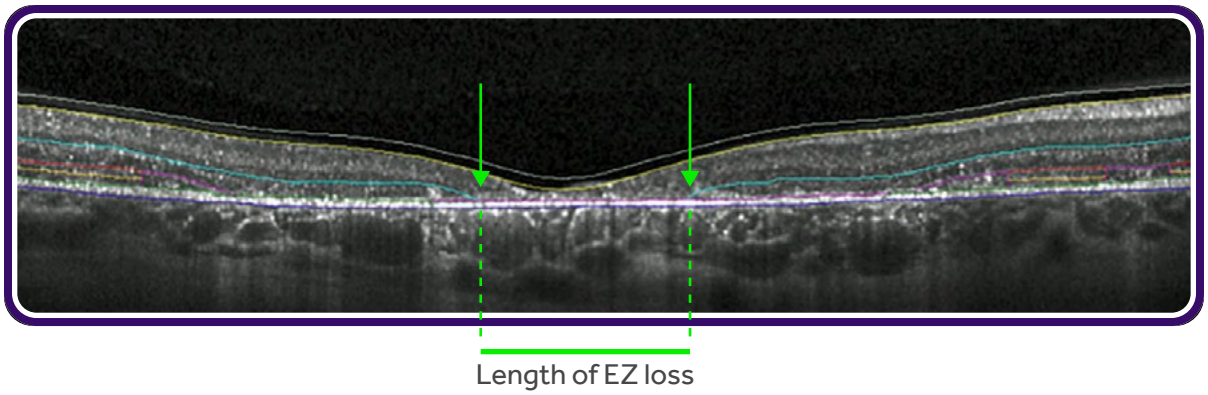

Retinal atrophy and Ellipsoid Zone (EZ) loss measured quantitatively with Optical Coherence Tomography (OCT). Strauss RW, Lang L, Ho A, et al. The Progression of Stargardt Disease as Determined by Spectral-Domain Optical Coherence Tomography over a 24-Month Period (ProgStar Report No. 18). *Ophthalmic Res*. 2024;67(1):435-447. doi:10.1159/000540028.

- Retinal atrophy and Retinal Pigment Epithelium (RPE) loss measured with Fundus Autofluorescence (FAF)
  - ✓ Questionably Decreased Autofluorescence (QDAF)
  - ✓ Definitely Decreased Autofluorescence (DDAF)
  - ✓ Area of a region or multiple regions
  - ✓ Presence of flecks
  - ✓ Hyper-fluorescence

VISUAL FUNCTION ASSESSMENTS

- Microperimetry (MP)
  - ✓ MP fixation stability (Bivariate Contour Ellipse Area, BCEA) and Peripheral Retinal Loci parameters
  - ✓ Photopic/Mesopic/Scotopic testing to isolate rod and cone function
- Visual Fields
  - ✓ Kinetic Perimetry
  - ✓ Static Visual Field
- Full-Field Stimulus Testing
- Electroretinography
- Best Corrected Visual Acuity

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Connect with us to learn more about how our expertise and approach can support bringing your product to market on-time and on-budget. Your success is our priority.

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