Location: Lakeland and Lake Worth

Ionis 696844CS5

GOLDEN STUDY: A Study to Assess Safety and Efficacy of Multiple Doses of IONIS-FB-LRx in Participants With Geographic Atrophy Secondary to Age-Related Macular Degeneration (AMD)

Sponsor: Ionis Pharmaceuticals, Inc.
Information provided by (Responsible Party): Ionis Pharmaceuticals, Inc.

Study Description

Brief Summary:
The purpose of the study is to evaluate the effect of **IONIS-**FB-LRx on the rate of change of the area of geographic atrophy (GA) secondary to age-related macular degeneration (AMD) measured by fundus autofluorescence (FAF).

Condition or disease	Intervention/treatment	Phase
Macular Degeneration Geographic Atrophy	Drug: **IONIS-**FB-LRxDrug: Placebo	Phase 2

Detailed Description:
The study will assess the rate of change of the area of GA secondary to AMD by measuring FAF in up to 330 participants with GA due to AMD being treated with IONIS-FB-LRx. The study is a Phase 2, double-masked, randomized, stratified, placebo-controlled study conducted at multiple centers. It is an adaptive design in which three dose levels will be evaluated in a subset of patients (Stage 1) and, following an interim analysis, the number of patients in two of the dose cohorts will be expanded (Stage 2).

Ages Eligible for Study:	50 Years and older (Adult, Older Adult)
Sexes Eligible for Study:	All
Accepts Healthy Volunteers:	No

Criteria

Inclusion Criteria:

- Females must be non-pregnant and non-lactating, and either surgically sterile or post-menopausal.
- Vaccination against Neisseria meningitidis and Streptococcus pneumoniae received at least 2 weeks prior to first dose of investigational product
- Well-demarcated geographic atrophy (GA) due to AMD

- Best-corrected visual acuity (BCVA) letter score of 35 letters (approx. 20/200 Snellen equivalent) or better on the ETDRS chart
- Must have clear ocular media and adequate pupillary dilation in the study eye to permit high-quality fundus imaging

Exclusion Criteria:

- Clinically-significant abnormalities in medical history
- A lack of full recovery from any infection for at least 14 days prior to the Study Drug administration
- Chronic treatment with steroids, including topically or intravitreally administered
- History or presence of diabetic retinopathy or diabetic macular edema (DME)
- History or presence of a disease other than AMD that could affect vision or safety assessments
- Prior treatment with another investigational drug, biological agent, or device
- Other protocol-specified inclusion/exclusion criteria may apply

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