For patients with Stargardt's disease

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What is Stargardt disease

Stargardt disease is one of the prevalent macular dystrophy, which is characterized by atrophy (loss of photoreceptor cells) in the macula. The major causative gene of Stargardt disease is the ABCA4 gene, illustrating autosomal recessive inheritance trait.
In childhood-onset group patients, visual acuity loss in both eyes is noticed at around 10 years of age. The disease progression in childhood is relatively rapid, with macular atrophy enlargement over several years, often resulting in legal blindness. It is also known that the disease course is diverse, ranging from a type in which the retinal damage is confined to the macular area to a type in which the damage expands to the periphery and causes extensive visual field abnormalities. ※

Study introduction

The most common cause of Stargardt disease is pathogenic variants/mutations in the ABCA4 gene which leads to an excessive accumulation of certain lipids in retina that causes degeneration (loss of function) of photoreceptors. Tinlarebant is a retinol binding protein 4 (RBP4) antagonist (inhibits function). RBP4 carries retinol, which is an essential nutrient for vision. Tinlarebant interferes with the physiological action of RBP4 by inhibiting its interaction to form complex structure. This reduces retinol concentration in blood which further inhibits formation of cytotoxic substance, thus helps in slowing down the progression of Stargardt disease.

The study comprises of a Phase 1b part conducted in Japan and a Phase 2/3 part.

<Phase 1b>
Phase 1b will investigate how Tinlarebant move into, through and out of the body in Japanese patients with Stargardt disease.
Starting on Day 1, subjects will receive 5 mg of tinlarebant once daily for 7 days and will attend the clinic for visits on Days 1, 2, 7, 10, 14, and 21. (Completed)

<Phase 2/3>
For approximately 60 subjects in Japan, UK and US) with Stargardt disease (with ABCA4 mutations), the efficacy of 5 mg tinlarebant administered once daily will be investigated. There are no restrictions of nationality/ethnicity for each clinical trial site. Eligible patients oversea are recruited at the Japanese study site.
Subjects will receive a treatment of tinlarebant or placebo. Starting on Day 1 (Month 1), subjects will receive 5 mg of tinlarebant or placebo once daily for 24 months. Subjects will return to the study site once every 3 months for safety and efficacy assessments.

Eligibility criteria

1. Males or females between 12 and 20 years of age,
2. Must have clinically diagnosed Stargardt disease with at least one mutation identified in the ABCA4 gene.
3. Subject and/or their parent(s) or legal representative are willing to provide their consent on an ICF prior to participating in any study related procedures.

There are other criteria and it is possible you don't meet them.

Potential Benefits

There is no guarantee that you will receive any benefit from taking part in this study. Your condition may remain the same, improve or could get worse.
Information obtained from the study may help in the development of treatments for Stargardt disease.

Who to Contact with Question

If you would like to know more about this study or if you are eligible to participate, please contact us below.

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