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Gene Therapy Candidate Granted Fast Track Designation for Treatment of Achromatopsia

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The US Food and Drug Administration (FDA) has granted a fast track designation to MeiraGTx Holdings Plc's gene therapy product candidate—AAV-CNGB3—for the treatment of patients with achromatopsia caused by mutations in the *CNGB3* gene.

Currently, no effective treatments are available to combat the disease.

"We are thrilled by this designation and the continued recognition by the FDA of AAV-CNGB's potential to become a much-needed treatment option for achromatopsia patients suffering from this debilitating disease," MeiraGTx's president and chief executive officer Alexandria Forbes, PhD, said in a recent statement.

Achromatopsia, according to the National Institutes of Health's Genetics Home Reference, is characterized by either a partial or total absence of color vision; those with the rare, inherited retinal disease only see in black, white, and shades of gray. Many individuals with the disease are legally blind from birth and suffer from extreme sensitivity to light and involuntary eye movements, as cone photoreceptors in the eye are prevented from functioning properly. The investigational gene therapy works to restore cone function via subretinal injection.

MeiraGTx is currently in the process of conducting a phase 1/2 clinical trial in adult and pediatric patients designed to assess the safety and effectiveness of AAV-CNGB.

For the dose escalation phase of the trial, 18 adult participants will be given 1 of 3 different escalating doses of the therapy—a single low dose, a single medium dose, or a single high dose of range AAV-CNGB. After investigators are able to establish an acceptable dose in adult participants, they will then treat up to 9 children.

The primary outcome measure for the trial is the incidence of adverse events associated with the treatment, with safety defined as the absence of related safety events, while the secondary outcome measures include improvement of visual and retinal function and improvement in quality of life at 6 months.

To date, 14 patients have been treated; eleven adult patients have been treated with the therapy in the 3 dose escalation cohorts and 3 pediatric patients have received the treatment in the extension phase of the study. By the second half of 2018, MeiraGTx anticipates that they will complete dosing of up to 8 pediatric patients.

Additionally, investigators are also conducting an ongoing natural history study, in which they will follow upwards of 90 patients with achromatopsia for the duration of 5 years. During this time, they will be collecting data pertaining to the structure and function of the participants' eyes.

Previously, the gene therapy has received an orphan drug designation from the FDA as well as an orphan medicinal product designation from the European Medicines Agency (EMA); furthermore, AAV-CNGB3 was also granted a rare pediatric disease designation by the FDA and PRIME designation by the EMA for treatment of patients with achromatopsia caused by mutations in the *CNGB3* gene.
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