QR-110 Development Program Update

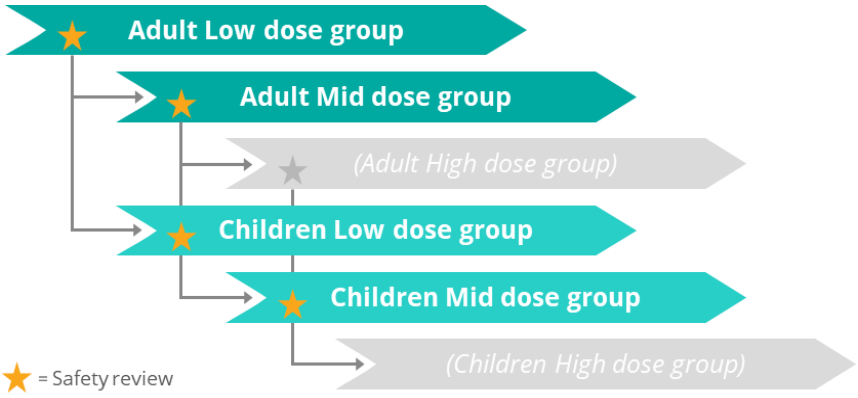
September 2018

ProQR has announced interim results of a QR-110 trial (PQ-110-001) in patients who have Leber's congenital amaurosis 10 (LCA10) due to one or two copies of the p.Cys998X mutation in the CEP290 gene.

Summary of the objectives and design of the QR-110 phase 1/2 trial (PQ-110-001)

The purpose of this ongoing trial is to find out if different dose levels of QR-110 are safe and whether QR-110 restores/improves vision (efficacy). QR-110 is injected into the eye (intravitreal injection) with the worst vision. The trial was designed to study up to three dose levels of QR-110 in adults and children in a step-wise way. Safety was reviewed before treating the next dose group. Trial participants in each group receive QR-110 every 3 months for a maximum of 4 times. The highest dose level was not given, as efficacy was observed with the low and mid doses. Enrollment is now competed, with 11 patients participating.

Clinical trial design



The trial is being conducted at three specialized centers with significant expertise in genetic retinal disease: the University of Iowa, Iowa City, Iowa, U.S., the Scheie Eye Institute at the University of Pennsylvania, Philadelphia, U.S., and the Ghent University Hospital, Ghent, Belgium.

You can find more information about the QR-110 ongoing study at ClinicalTrials.gov

Interim results of the QR-110 phase 1/2 trial (PQ-110-001)

The results of the interim analysis are encouraging. Approximately 60% of participants showed improvements in visual acuity and functional vision after three months. In the trial, visual acuity is assessed using different eye charts and functional vision is assessed using a series of mobility courses with increasing difficulty and multiple light levels. Improvements in visual function were supported by a significant increase in the ability to detect flashes of red or blue light as determined by the full field stimulus test (FST). Additionally, the majority of patients improved on nystagmus (wandering eye movements in low vision patients).

So far, QR-110 was well tolerated with no serious adverse events related to treatment or the intravitreal injection procedure.

Enrollment of the trial is now completed. Patients in the trial will continue the 12-month treatment and observation period. Eligible patients that complete the trial, will be offered the option to continue treatment in the open-label extension trial called “INSIGHT”.

Next steps for the QR-110 development program

Pending completing discussions with the regulatory authorities in the coming months, ProQR expects to start a Phase 2/3 trial called "ILLUMINATE" in 2019. The trial is expected to initially enroll 30-40 patients with LCA10 due to one or two copies of the p.Cys998X mutation in the CEP290 gene. The trial will study the efficacy and safety of several doses of QR-110 and is expected to be conducted at centers in North America and European countries. As well as ILLUMINATE, ProQR plans to conduct trials in children younger than 6 years old.

The ProQR team would like to thank the study participants, their caregivers, and the investigators and their staff for the support in the development of QR-110 in this trial.

ProQR remains committed to making a significant and positive impact on the lives of those affected by LCA10. We look forward to continued collaboration and support from the LCA community. As the QR-110 development program advances, we will keep the community updated on our progress. Please visit our website for the latest news and future study participation opportunities.

If you have any questions, please consult your treating physician or you can contact ProQR at patientinfo@proqr.com