QR-010 Development Program Update

November 2017

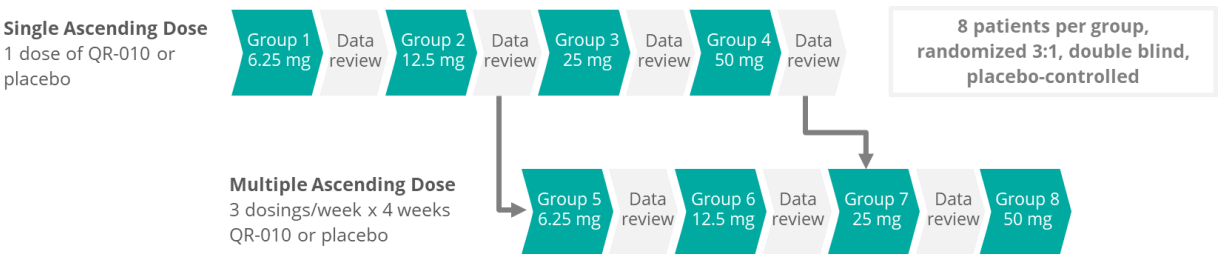
ProQR has announced results of a QR-010 Phase 1b clinical trial with CF patients that are homozygous- or carrying two copies- for the F508del mutation. This QR-010 program update was developed as a way to keep the CF community updated.

Summary of the objectives and design of the QR-010 phase 1b trial (PQ-010-001)

This international Phase 1b trial was primarily designed to look at safety of different doses of inhaled QR-010 in people with CF that are homozygous for the F508del mutation. Other exploratory measurements were also recorded that might indicate early evidence of efficacy of QR-010.

As part of the study the validated patient reported outcome measure Cystic Fibrosis Questionnaire-Revised Respiratory Symptom Score (CFQ-R RSS) was included, as it is important to assess clinical benefit of QR-010 from the patient's perspective. The CFQ-R RSS is used in other CF clinical trials and the results are considered important indicators of patient benefit in clinical trials both by the medical community and regulators.

The effects of different doses of inhaled QR-010 were examined by dividing the clinical trial into two parts (please refer to the infographic). 4 groups of patients each received 1 assigned dose of QR-010 or placebo. Another 4 groups received an assigned dose of QR-010 or placebo, 3 times a week over 4 weeks. To ensure patient safety, the data from each group was reviewed by experts before QR-010 dosing was increased for the next group. This was a double blind trial, which meant that neither the study doctor nor patient knew if they were allocated QR-010 or placebo.



Clinical Trial Design of PQ-010-001

Results QR-010 phase 1b trial (PQ-010-001)

The trial was completed in September 2017 and ProQR is pleased to share that this trial met all its goals. The results show that QR-010 was safe and well tolerated across all dose levels. There are encouraging signals that people with CF may benefit from taking QR-010. Most participants that received QR-010 in the trial reported a reduction in CF symptoms as measured by an increase in the CFQ-R RSS which was not seen in the placebo group. A supportive trend was observed in improved lung function (as measured by percent predicted forced expiratory volume in 1 second, or ppFEV1) compared to placebo. No change was observed on sweat chloride and weight gain.

The results of the QR-010 phase 1b trial were presented at the 2017 North American CF Conference (NACFC).

The ProQR team would like to thank everyone who helped us reach this important milestone. In particular, we would like to acknowledge the important contributions of the people living with CF that participated and the CF experts who helped us design and conduct this clinical trial. This achievement was the result of a global community effort – 70 participants at 23 hospitals in 10 countries across North America and Europe.

ProQR remains committed to making a significant and positive impact on the lives of those affected by CF. We are making plans for the next QR-010 clinical trials and look forward to continued collaboration and support from the CF community. As the QR-010 development program advances we will keep the community updated on our progress.

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