Study to Evaluate the Feasibility and Variability of Select Vision Assessments in Subjects with a Leber's Congenital Amaurosis (LCA) Type Phenotype

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PQ-110-004, is designed to evaluate if a mobility course using multiple light levels simulating real world conditions, can detect changes in vision in subjects with a phenotype representative of LCA Type 10 and therefore serves as an assessment tool...

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Eye disorders congenital
Study type	Interventional

Summary

ID

NL-OMON48307

Source
ToetsingOnline

Brief title
PQ-110-004

Condition

- Eye disorders congenital
- Congenital eye disorders (excl glaucoma)

Synonym
Leber's Congenital Amaurosis Type Phenotype, Leber's disease

Research involving
Human

Sponsors and support

Primary sponsor: ProQR Therapeutics
Source(s) of monetary or material Support: ProQR Therapeutics

Intervention

Keyword: LCA10, Leber's Congenital Amaurosis Type Phenotype, Mobility Course

Outcome measures

Primary outcome

The primary objectives of this study are to evaluate:

* The feasibility of the mobility course as an assessment tool to evaluate

efficacy

endpoints in clinical studies in subjects with a phenotype representative of LCA

Type 10

* The feasibility of performing the following assessments in subjects with a

phenotype representative of LCA Type 10:

o Best corrected visual acuity (BCVA)including low luminance visual

acuity [LLVA])

o Mobility course

o Full field stimulus testing (FST)

o Spectral Domain Optical Coherence Tomography (SD-OCT)

o Fundus Autofluorescence (FAF)

o Patient Reported Outcome (PRO) of Patient Global Impressions of

Severity (PGI-S)

o PRO of Patient Global Impressions of Change (PGI-C)

* The inter-visit variability of the following assessments in subjects with a

phenotype representative of LCA Type 10:

o BCVA (including LLVA)

o FST

o Mobility course

o PGI-S

o PGI-C

Secondary outcome

Not applicable

Study description

Background summary

Leber*s congenital amaurosis (LCA) is a severe inherited retinal degenerative disease resulting in blindness, often in early childhood. In subjects with LCA due to the p.Cys998X mutation in Centrosomal Protein of 290 kDa (CEP290), visual symptoms are usually detectable before 1 year of age and further deterioration over time has also been reported (den Hollander 2008, Yzer 2012). Patients show severe vision disturbances from an early age and slow progressive loss of remaining vision (Cideciyan 2007, Cideciyan 2011). There are currently no approved therapies for the treatment of LCA due to the p.Cys998X mutation in CEP290 (subsequently referred to as the CEP290 p.Cys998X mutation) and a large unmet medical need exists.

Study objective

PQ-110-004, is designed to evaluate if a mobility course using multiple light levels simulating real world conditions, can detect changes in vision in subjects with a phenotype representative of LCA Type 10 and therefore serves as an assessment tool to evaluate efficacy endpoints in clinical studies.

Study design

Study PQ-110-004 will evaluate the feasibility and inter-visit variability of selected visual assessments in subjects with a phenotype representative of LCA

Type 10 for use as efficacy endpoints in clinical studies. No study drug or placebo will be administered during this study; however, participating subjects with LCA due to the CEP290 p.Cys998X mutation may be eligible to enroll in the phase 2/3 clinical study PQ-110-003.

Participating sites should pre identify subjects from their database known to have a clinical diagnosis: 1) LCA Type 10, 2) any other LCA subtype or 3) any subtype of inherited retinal disease, and who have a phenotype representative of LCA Type 10.

Each site should plan to enroll all eligible subjects expected to be suitable for participation in a phase 2/3 clinical study PQ-110-003.

Subjects will attend a Screening Visit to determine if they meet the eligibility criteria. Subjects meeting all eligibility criteria will be scheduled to undergo a series of assessments during 2 consecutive days within 1 month of screening as outlined in the Schedule of Events. All assessments will be performed in both eyes.

Intervention

Study subjects have to complete a mobility course with multiple light levels. In addition, they need to undergo the following tests:
- visual acuity test including LLVA
- ophthalmic examination
- FST

Study burden and risks

The study duration is about 2 months and consists of 1 screening visit (may be completed over multiple days) and 2 consecutive days (day 1 and day 2) with tests

The tests being done in this study:
- visual acuity test
- ophthalmic examination
- mobility course
- FST

Risks: see section E9

Contacts

Public

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Scientific
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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Adults (18-64 years)
Children (2-11 years)
Elderly (65 years and older)

Inclusion criteria

1. Persistence of detectable outer nuclear layer (ONL) in the area of the macula in the opinion of the Investigator, as determined by OCT
2. Clear ocular media and adequate pupillary dilation to permit good quality retinal imaging, as assessed by the Investigator
3. An adult (* 18 years) willing and able to provide informed consent for participation OR a minor (6 to < 18 years) with a parent or legal guardian willing and able to provide written permission for the subject*s participation prior to performing any study related procedures and minor subjects able to provide age appropriate assent for study participation
4. An adult willing to comply with the protocol, follow study instructions, attend study visits as required and willing and able to complete all study assessments, in the opinion of the Investigator OR a minor able to complete all study assessments and comply with the protocol and has a parent or

caregiver willing and able to follow study instructions and attend study visits with the subject as required, in the opinion of the Investigator
5. Adequate verbal communication as to allow assessment via mobility course, in the opinion of the Investigator
6. Clinical diagnosis of: 1) LCA Type 10, 2) any other LCA subtype or 3) any subtype of inherited retinal disease, which has a phenotype representative of LCA Type 10, as assessed with concurrence of the Medical Monitor.

Exclusion criteria

1. Any ocular and/or general disease or condition that could compromise subject*s safety or interfere with assessment of efficacy and safety, as determined by the Investigator
2. Any parent/guardian or subject who, in the opinion of the Investigator, may not be compliant with the study procedures or schedule.

Study design

Design

Study type: Interventional

Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Diagnostic

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	23-07-2019
Enrollment:	15
Type:	Actual

Ethics review

Approved WMO

Date:	18-04-2019
Application type:	First submission

Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	18-07-2019
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	20-08-2019
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	19-12-2019
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	27-05-2021
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
CCMO	NL68527.000.18

Study results

Date completed: 02-12-2020

Actual enrolment: 7

Summary results

Trial is onging in other countries