
**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

FORM 6-K

**Report of Foreign Private Issuer
Pursuant to Rule 13a-16 or 15d-16 of
the Securities Exchange Act of 1934**

For the month of October 2019

Commission File Number: 001-36622

PROQR THERAPEUTICS N.V.

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2333 CK Leiden
The Netherlands**

Tel: +31 88 166 7000

(Address, Including ZIP Code, and Telephone Number,
Including Area Code, of Registrant's Principal Executive Offices)

Indicate by check mark whether the registrant files or will file annual reports under cover of Form 20-F or Form 40-F.

Form 20-F Form 40-F

Indicate by check mark if the registrant is submitting the Form 6-K in paper as permitted by Regulation S-T Rule 101(b)(1):

Indicate by check mark if the registrant is submitting the Form 6-K in paper as permitted by Regulation S-T Rule 101(b)(7):

On October 10, 2019, ProQR Therapeutics N.V. (the "Company") issued a press release titled, "ProQR Announces Positive Top-Line Results from the Phase 1/2 Study of Sepofarsen in LCA10 Patients." A copy of this press release is attached hereto as Exhibit 99.1 and is incorporated herein by reference. The Company hereby incorporates by reference the information contained herein into the Company's registration statement on Form F-3 (File No. 333-228251).

In addition, the Company presented top-line results from its Phase 1/2 clinical trial of Sepofarsen in LCA10 patients using a webcasted conference call. A copy of the presentation is attached hereto as Exhibit 99.2, which is intended to be furnished and shall not be deemed to be "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

PROQR THERAPEUTICS N.V.

Date: October 10, 2019

By: /s/ Smital Shah
Smital Shah
Chief Financial Officer

INDEX TO EXHIBITS

Number	Description
99.1	Press Release dated October 10, 2019.
99.2	Presentation for webcasted conference call.

ProQR Announces Positive Top-Line Results from the Phase 1/2 Study of Sepofarsen in LCA10 Patients

- Reported rapid, significant and durable improvements in vision at twelve months
- Concordant improvement in key secondary outcome measures
- The target registration dose of sepofarsen was well-tolerated with a favorable benefit/risk profile
- Strengthens confidence in the ongoing Phase 2/3 pivotal study, top-line data expected first half of 2021
- Management to host a conference call today at 8:00 a.m. ET

LEIDEN, Netherlands & CAMBRIDGE, Mass., Oct 10, 2019 — ProQR Therapeutics N.V. (Nasdaq:PRQR), a company dedicated to changing lives through the creation of transformative RNA medicines for severe genetic rare diseases, today announced positive top-line results from the PQ-110-001 study, a Phase 1/2 dose range finding, first-in-human trial of sepofarsen (QR-110) in patients with Leber's congenital amaurosis 10 (LCA10) due to the p.Cys998X mutation in the *CEP290* gene.

"We reported today that patients receiving sepofarsen had a clinically meaningful improvement in vision, and in some cases the patient's vision improved to a level that could be deemed life changing. This is very encouraging for the LCA10 community and the Inherited Retinal Disease community as a whole," said Stephen R. Russell, MD, Schrage Professor of Ophthalmology and Visual Sciences and Principal Investigator at the University of Iowa. "LCA10 is a severe inherited retinal disease that leads to blindness, and for which there is currently no treatment."

David Rodman, M.D., Executive Vice President of Research & Development of ProQR, said, "We are very pleased with the data reported from the Phase 1/2 study, in which LCA10 patients treated with sepofarsen experienced a rapid and durable improvement in vision. The top-line data from this study strengthen our confidence in the design of the ongoing Phase 2/3 trial, which could be the sole registration trial for the sepofarsen program. We appreciate the patients' and medical communities' ongoing support for the sepofarsen clinical studies, and we will continue to work with the regulators to advance this program as efficiently as possible."

Top-line results from the Phase 1/2 trial

Based on positive 3-month interim results from the Phase 1/2 trial (Nature Medicine 2018) the Phase 2/3 *Illuminate* trial was initiated earlier this year. The 12-month top-line results from the Phase 1/2 trial that will be presented today confirm durable activity of sepofarsen for up to one year in patients with LCA10. Furthermore, the results support the assumptions used in the design and powering of *Illuminate*, including:

- the target registration dose (80 µg with a 160 µg loading dose) was associated with a clinically meaningful and statistically significant improvement in vision and had a favorable benefit/risk profile,
 - a six-month dosing frequency, as used in *Illuminate*, was associated with durable improvements in vision,
-

- the response observed at 12 months in the target registration dose was equal to or greater than the response at the 3-month interim analysis,
- and subjects with better vision than light perception (BCVA > LogMAR 3.0) at baseline, the study population in *Illuminate*, were more likely to respond to treatment with seprofarsen.

Table: Change from baseline in treated eye

Endpoint	Direction of improvement	Pooled analysis, all dose groups (SEM)	n	Target registration dose group, 160/80 µg (SEM)	n
BCVA	Down ↓	-0.55 (0.26)	11	-0.93 (0.43)	6
in LogMAR		p<0.05		p<0.01	
FST red	Down ↓	-0.92 (0.18)	10	-0.66 (0.14)	6
in Log(cd/m(2))		p<0.01		p<0.01	
FST blue	Down ↓	-0.79 (0.23)	10	-0.63 (0.31)	6
in Log(cd/m(2))		p<0.02		p<0.01	
Mobility course	Up ↑	+2.5 (0.99)	10	+4.0 (1.27)	6
in Levels		p=0.1		p<0.01	

Analysis: Pooled analysis: Unpaired t-test, between group change from baseline versus untreated. Target registration dose group: MMRM with repeated measures, within group change from baseline versus baseline

Visual acuity

Statistically significant improvement from baseline in best corrected visual acuity (BCVA) was observed as assessed by the Early Treatment of Diabetic Retinopathy Study (ETDRS) eye chart and the Berkeley Rudimentary Vision Test (BRVT). In the pooled analysis with all patients after twelve months of treatment, the mean improvement (and standard error of mean, SEM) was -0.55 LogMAR (SEM 0.26). The mean change in the untreated contralateral eye was -0.11 LogMAR (SEM 0.07).

In the target registration dose group, the mean change from baseline at twelve months was -0.93 LogMAR (SEM 0.43) with four of six subjects showing an improvement of more than -0.3 LogMAR from baseline equivalent to 3 lines, or 15 letters, on ETDRS chart (generally considered clinically meaningful by US regulators). Five of six subjects showed an improvement greater than -0.2 LogMAR (generally considered clinically meaningful by EU regulators). The mean change in the untreated contralateral eye in this group was -0.22 LogMAR (SEM 0.11). In all five subjects in this group, in which a six-month dosing frequency was explored, the observed benefit in visual acuity was maintained during the twelve-month follow up.

Full field stimulus threshold test (FST)

Improvements in visual function were supported by a meaningful increase in the ability to detect flashes of red or blue light as determined by the FST test. In the pooled analysis after twelve months, mean improvement in red light sensitivity was -0.92 log Cd/m(2) (SEM 0.18) and improvement in blue light sensitivity was -0.79 log Cd/m(2) (SEM 0.23). The mean change in the untreated contralateral eye was -0.16 log Cd/m(2) (SEM 0.16) for red light and 0.02 log Cd/m(2) (SEM 0.11) for blue light.

In the target registration dose group, the mean change from baseline at twelve months in red light sensitivity was -0.66 log Cd/m(2) (SEM 0.14) and improvement in blue light sensitivity was -0.63 log Cd/m(2) (SEM 0.31).

Three of six subjects showed an improvement of greater than $-0.5 \log \text{ Cd/m}^2$ for blue light, which can be regarded as clinically meaningful. Five of six showed a clinically meaningful improvement for red light. The mean change in the untreated contralateral eye in this group was $0.05 \log \text{ Cd/m}^2$ (SEM 0.17) for red light and $-0.12 \log \text{ Cd/m}^2$ (SEM 0.16) for blue light.

Mobility course

Most patients demonstrated improvement in functional vision, as assessed using a series of mobility courses at increasing difficulty and multiple light intensities. In the pooled analysis the mean improvement for patients navigating the mobility course after twelve months of treatment was 2.5 levels (SEM 0.99). The mean change in the untreated contralateral eye was 1.75 (SEM 0.75). This increase was likely due to a training effect. An adjusted mobility course endpoint is being validated in parallel with the *Illuminate* trial.

In the target registration dose group, the mean change at twelve months of treatment was 4.0 levels (SEM 1.27) with five of six subjects improving by more than 2.0 levels, which can be regarded as clinically meaningful. The mean change in the untreated contralateral eye was 2.7 levels (SEM 1.11).

Safety

Subjects received up to four doses of seprofarsen (range 1-4), and all eleven subjects in the study completed twelve months of follow up. This represents data equivalent to over 4,000 treatment days. Sepofarsen was observed to be well-tolerated with manageable safety findings. In total, eight cases of lens opacities (cataract) were observed (three in the target registration dose cohort and five in high dose cohort). All six of the subjects that had lens replacement surgery regained their pre-cataract vision. Four cases (in three subjects) of retinal findings were observed in the now retired 320/160 μg dose group: Two incidences of mild cystoid macular edema were resolved with topical treatment and two incidences of subclinical retinal thinning stabilized within two months of last dose without additional treatment.

Conference call

Management will discuss the data during a conference call today at 8:00 a.m. ET. The live and archived webcast (primary connection method) can be accessed at ir.proqr.com/events-and-presentations. The dial-in details for the call are +1 631 510 7495 or +44 2071 928000 (international), conference ID: 3496655.

About the PQ-110-001 Phase 1/2 trial

PQ-110-001 was an open-label trial designed to enroll children (over six years of age) and adults who have LCA10 due to one or two copies of the p.Cys998X mutation in the *CEP290* gene. A total of twelve patients were screened, of which eleven subjects were dosed. All eleven patients were enrolled in either the 80 μg dose cohort (160 μg loading dose) or 160 μg dose cohort (320 μg loading dose) and received one to four intravitreal injections with seprofarsen in the treated eye at varying dosing intervals, with the other (contralateral) eye remaining untreated. Patients in the trial have completed the 12-month treatment and observation period and eligible patients are being given the option to participate in *Insight*, an open-label extension study including the possibility of receiving treatment in the second eye.

The trial was conducted at specialized centers with significant expertise in inherited 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.

The primary objectives of the PQ-110-001 trial were safety and tolerability. Secondary objectives included pharmacokinetics, as well as restoration/improvement of visual function and retinal structure through ophthalmic endpoints, such as visual acuity (BCVA), mobility course, full field stimulus testing (FST) and optical coherence tomography (OCT). Additional exploratory endpoints including ocular instability (OCI), pupillary light reflex (PLR) and changes in quality of life in the trial subjects are also being evaluated.

About the Phase 2/3 *Illuminate* trial

Illuminate, or PQ-110-003, is a randomized, prospective, double-masked, sham-controlled 24-month trial of seprofarsen that will initially enroll 30 adults and children (eight years of age and over) who have LCA10 due to one or two copies of the p.Cys998X mutation in the *CEP290* gene and a baseline best corrected visual acuity (BCVA) of 3.0 LogMAR or better. The trial is designed as the sole registration trial for the program.

Participants will be assigned equally to three parallel study arms (two active dose levels; 40 µg (with an 80 µg loading dose) and 80 µg (with a 160 µg loading dose); and a sham control arm) with ten participants in each arm. Participants will receive a dose of seprofarsen or a sham-procedure at the start of the trial, at three months and then every six months afterwards.

The primary endpoint will be mean change in BCVA from baseline in the active treated arms compared to the control arm. A mobility course endpoint will also be evaluated. Additional endpoints include full field stimulus testing, ocular instability and optical coherence tomography. Changes in quality of life in the trial participants will also be evaluated. The study incorporates adaptive sample size re-estimation based on pre-specified criteria overseen by an independent statistician. *Illuminate* is a global trial conducted at sites with significant expertise in genetic retinal diseases in North America and selected European countries.

About seprofarsen

Sepofarsen (QR-110) is a first-in-class investigational RNA-based oligonucleotide designed to address the underlying cause of Leber's congenital amaurosis 10 due to the p.Cys998X mutation (also known as the c.2991+1655A>G mutation) in the *CEP290* gene. The p.Cys998X mutation is a substitution of one nucleotide in the pre-mRNA that leads to aberrant splicing of the mRNA and non-functional CEP290 protein.

Sepofarsen is designed to bind to the mutated location in the pre-mRNA and enable normal splicing, resulting in restoration of normal (wild type) *CEP290* mRNA and subsequent production of functional CEP290 protein.

Sepofarsen is intended to be administered through intravitreal injections in the eye and has been granted orphan drug designation in the United States and the European Union and received fast-track designation from the FDA as well as access to the PRIME scheme by the EMA.

About Leber's congenital amaurosis 10

Leber's congenital amaurosis (LCA) is the most common cause of blindness due to genetic disease in children. It consists of a group of diseases of which LCA10 is the most frequent and one of the most severe forms. LCA10 is caused by mutations in the *CEP290* gene, of which the p.Cys998X mutation has the highest prevalence. LCA10 leads to early loss of vision causing most people to lose their sight in the first few years of life. To date, there are no treatments approved or other products in clinical development that treat the

underlying cause of the disease. Approximately 2,000 people in the Western world have LCA10 because of this mutation.

About ProQR

ProQR Therapeutics is dedicated to changing lives through the creation of transformative RNA medicines for the treatment of severe genetic rare diseases such as Leber's congenital amaurosis 10, Usher syndrome type 2 and autosomal dominant retinitis pigmentosa. Based on our unique proprietary RNA repair platform technologies we are growing our pipeline with patients and loved ones in mind.

Since 2012

FORWARD-LOOKING STATEMENTS

This press release contains forward-looking statements. All statements other than statements of historical fact are forward-looking statements, which are often indicated by terms such as "anticipate," "believe," "could," "estimate," "expect," "goal," "intend," "look forward to," "may," "plan," "potential," "predict," "project," "should," "will," "would" and similar expressions. Such statements include those relating to sepofarsen and the clinical development and therapeutic potential thereof, including our statements regarding release of clinical data, and the clinical development plan for sepofarsen including the potential sole registration trial for the sepofarsen program. Forward-looking statements are based on management's beliefs and assumptions and on information available to management only as of the date of this press release. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, the risks, uncertainties and other factors in our filings made with the Securities and Exchange Commission, including certain sections of our annual report filed on Form 20-F. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and we assume no obligation to update these forward-looking statements, even if new information becomes available in the future, except as required by law.

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TOP-LINE RESULTS: PHASE 1/2 TRIAL OF SEPOFARSEN IN LCA10 PATIENTS

Conference *call*

October 10, 2019



Forward looking statements

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Forward-looking statements are based on management's beliefs and assumptions and on information available to management only as of the date of this press release. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, the risks, uncertainties and other factors in our filings made with the Securities and Exchange Commission, including certain sections of our annual report filed on Form 20-F. These risks include, but are

not limited to, any one or more of our product candidates will not be successfully developed or commercialized, the risk of cessation or delay of any ongoing or planned clinical trials, we may not realize the intended benefits of our current and potential future strategic collaborations, we may not discover or develop any new product candidates, that prior results observed from preclinical or clinical trials, will not be replicated or will not continue in ongoing or future studies or trials, that we may not successfully submit applications for marketing approval for our product candidates on time or at all, that regulatory authorities may require additional clinical trials beyond those that we currently contemplate conducting, that we will be unable to obtain and maintain regulatory approval for our product candidates, the risk that the size and growth potential of the market for our product candidates will not materialize as expected, risks associated with our dependence on third-party suppliers and manufacturers, risks regarding the accuracy of our estimates of expenses and future revenue, risks relating to our capital requirements and needs for additional financing, and risks relating to our ability to obtain and maintain intellectual property protection for our product candidates. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and we assume no obligation to update these forward-looking statements, even if new information becomes available in the future, except as required by law.

Agenda

Welcome and introduction

by Smital Shah



Smital Shah
*Chief Business
& Financial Officer*

Top-line results of Phase 1/2 trial

by David Rodman, MD



David Rodman, MD
*Executive Vice President
Research & Development*

Inherited retinal disease pipeline

by Daniel de Boer



Daniel A. de Boer
Chief Executive Officer



Sepofarsen Phase 1/2 trial topline results

David Rodman, MD - Executive Vice President
Research & Development

Phase 1/2 data presentation agenda

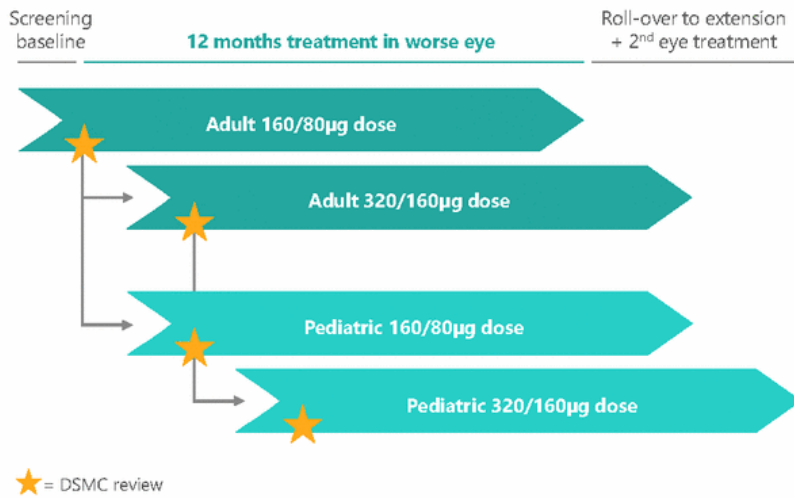
- Trial design and top-line summary
- Best corrected visual acuity
- Secondary outcome measures

Trial design and top-line summary

Protocol, baseline demographics, subject disposition and top-line efficacy data

Phase 1/2 – Trial design

Open label, multiple dose, dose escalation study, 11 LCA10 patients with 1 or 2 copies of p.Cys998X, age 8-44, 3 sites (2 US, 1 EU)



Objectives	
Base case	Safety/tolerability
	Mechanistic proof-of-concept (full-field stimulation)
Up side	Clinical proof-of-concept (best corrected visual acuity)
	Identify target dose
	Mobility course feasibility in LCA10
Explore	Additional secondary outcome measures

Phase 1/2 – Baseline Demographics

160µg/80µg cohort, n=6; 2 LP only, 3 BRVT, 1 ETDRS

320µg/160µg cohort, n=5; 3 LP only, 1 BRVT, 1 ETDRS

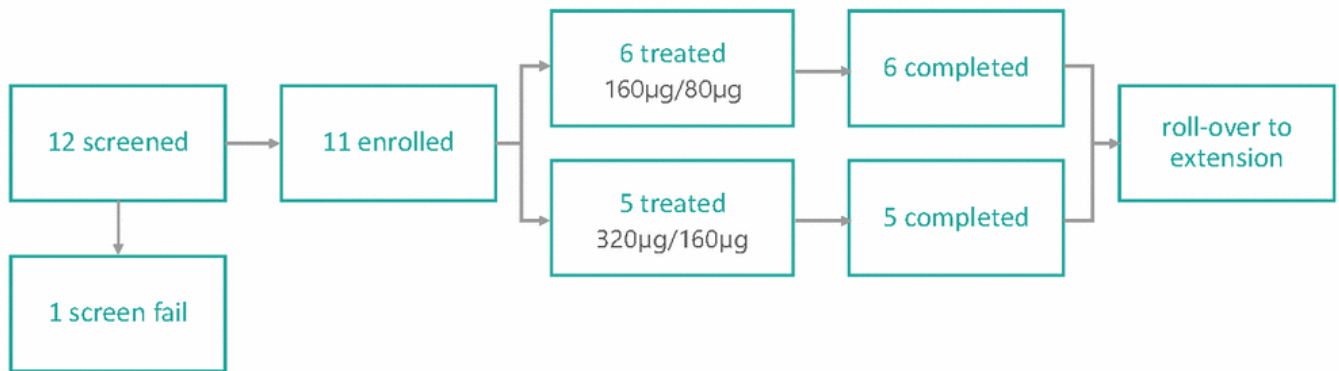
Gender	2 nd CEP290 Allele	Age/Group	Baseline BCVA [LogMAR]	Treated Eye	Dose [µg]
M	c.2503_2504delAC	19 / Adult	LP / LP	Right	160/80
M	c.4723A>T	41 / Adult	LP / LP	Right	160/80
M	c.5668G>T	44 / Adult	2.4 / 2.3	Left	160/80
F	c.4438-3delC	16 / Pediatric	2.5 / 2.5	Right	160/80
M	c.6277delG	8 / Pediatric	1.9 / 2.1	Left	160/80
F	c.2991+1655A>G	14 / Pediatric	0.6 / 0.6	Left	160/80
F	c.3167_3168insA	21 / Adult	LP / LP	Right	320/160
F	c.4723A>T	27 / Adult	1.1 / 0.7	Right	320/160
F	c.4393C>T	24 / Adult	LP / LP	Right	320/160
M	c.6277delG	10 / Pediatric	1.9 / 1.4	Right	320/160
F	c..547_550delTACC	15 / Pediatric	LP / LP	Right	320/160

BRVT = Berkley Rudimentary Vision Test (1.7-4.0 LogMAR) | ETDRS = Standard Eye Chart (0.0-1.6 LogMAR)

4.0 LogMAR = Light perception (LP) only | 3.0 LogMAR = Hand motion | 2.0 LogMAR = Finger counting | 1.0 LogMAR = 20/200 | 0.0 LogMAR 20/20

Disposition

>4000 subject treatment-days at two dose levels



Top-line efficacy results

Primary and key secondary outcome measures pooled analysis n=11

Objective	Assessment	Direction of improvement	Responder threshold	Mean change from baseline at month 12 (SEM)	
				Treated (TE)	Untreated (CE)
Mechanistic proof-of-concept	Full field stimulus (FST) red – log cd/m ² (n=10)	↓ = improved	-0.5	-0.92 (0.18) p<0.01 vs. CE	-0.16 (0.16)
	Full field stimulus (FST) blue – log cd/m ² (n=10)	↓ = improved	-0.5	-0.79 (0.23) p<0.02 vs. CE	0.02 (0.11)
Clinical proof-of-concept	Best corrected visual acuity (BCVA) – LogMAR (n=11)	↓ = improved	-0.3	-0.55 (0.26) p<0.05 vs. CE	-0.11 (0.07)
Secondary outcome*	Mobility course – composite score (n=10)	↑ = improved	2	2.5 (0.99) p=0.1 vs. CE	1.75 (0.75)

*Additional exploratory outcome measures, including OCI, PLR, OCT, PROs being analyzed

Example of mobility course responder (video)

Before and after treatment

Top-line safety summary

Positive benefit/risk in 160µg/80µg cohort with 50% incidence of lens opacity

Subclinical retinal findings in 320µg/160µg cohort

	Cataracts	Cystoid Macular Edema	Retinal thinning
SAE/AE	6 SAE (surgery)/2 AE	0 SAE / 2 AE	0 SAE / 2 AE
Dose-dependent incidence	Yes	Yes	Yes
Timing (160µg/80µg cohort)	8-12 months	No cases	No cases
Timing (320µg/160µg cohort)	3-9 months	3-4 months	3-10 months
Treatment-responsive	Yes	Yes	Stabilized

Phase 1/2 – Progress against Objectives

Base-case and upside objectives achieved

Regulatory agreement and global Ph2/3 adaptive pivotal trial underway

Objectives	Outcome
Safety/tolerability	✓ 160µg/80µg – 50% incidence of cataracts manageable with surgery
Mechanistic proof-of-concept (Full-field stimulation)	✓ Positive
Clinical proof-of-concept (BCVA)	✓ Positive
Identify target dose	✓ Positive - target dose for pivotal trial confirmation identified and agreed with FDA and EMA
Mobility course feasibility in LCA10	✓ Positive - formal validation study in progress

Best corrected visual acuity

Primary outcome measure: best corrected visual acuity (BCVA), month 12 vs. baseline

BCVA stratified by dose cohort

Primary outcome measure – mean change in BCVA

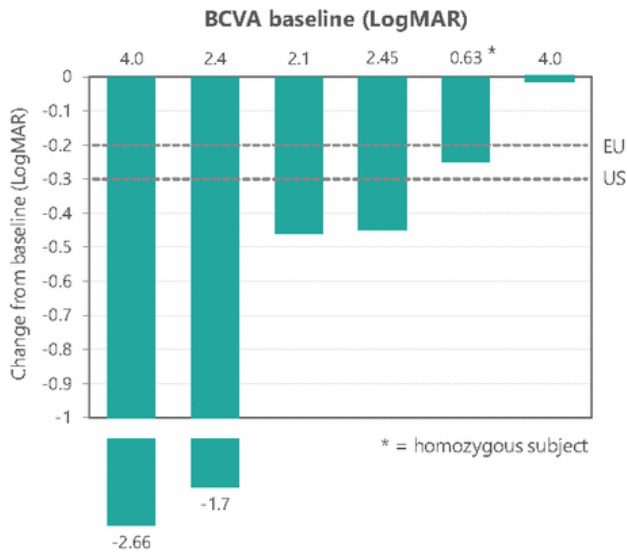
Benefit maintained from month 3 to month 12

Mean Δ BCVA LogMAR	Treated eye (SEM)		Contralateral eye (SEM)	
	month 3	month 12	month 3	month 12
Pooled analysis (n=11)	-0.50 (0.24)	-0.55 (0.26)	0.0 (0.04)	-0.11 (0.07)
160 μ g/80 μ g (n=6)	-0.81 (0.41)	-0.93 (0.43)	0.01 (0.08)	-0.22 (0.11)
320 μ g/160 μ g (n=5)	-0.13 (0.1)	-0.11 (0.07)	0.0 (0.0)	0.01 (0.04)

← Phase 2/3 trial target dose

160µg/80µg cohort

Consistent improvement with favorable benefit/risk



Responder Rate

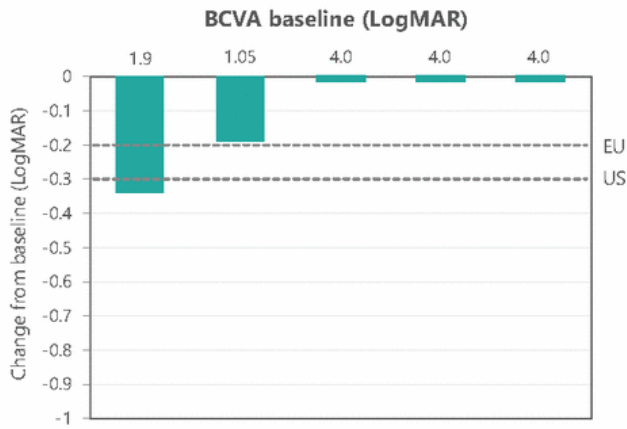
Responder (%)	Treated eye		Contralateral eye	
	US responder threshold	EU responder threshold	US responder threshold	EU responder threshold
160µg/80µg (n=6)	67%	83%	33%	33%

Safety Findings

	160µg/80µg (n=6)
Tolerability	No issues
Systemic safety	No issues
Lens opacity	3 findings
Cataract surgery outcome	2 surgeries. Complete recovery of pre-cataract benefit 2/2 subjects
Retinal findings	No issues

320µg/160µg cohort

Less improvement with dose-limiting safety findings



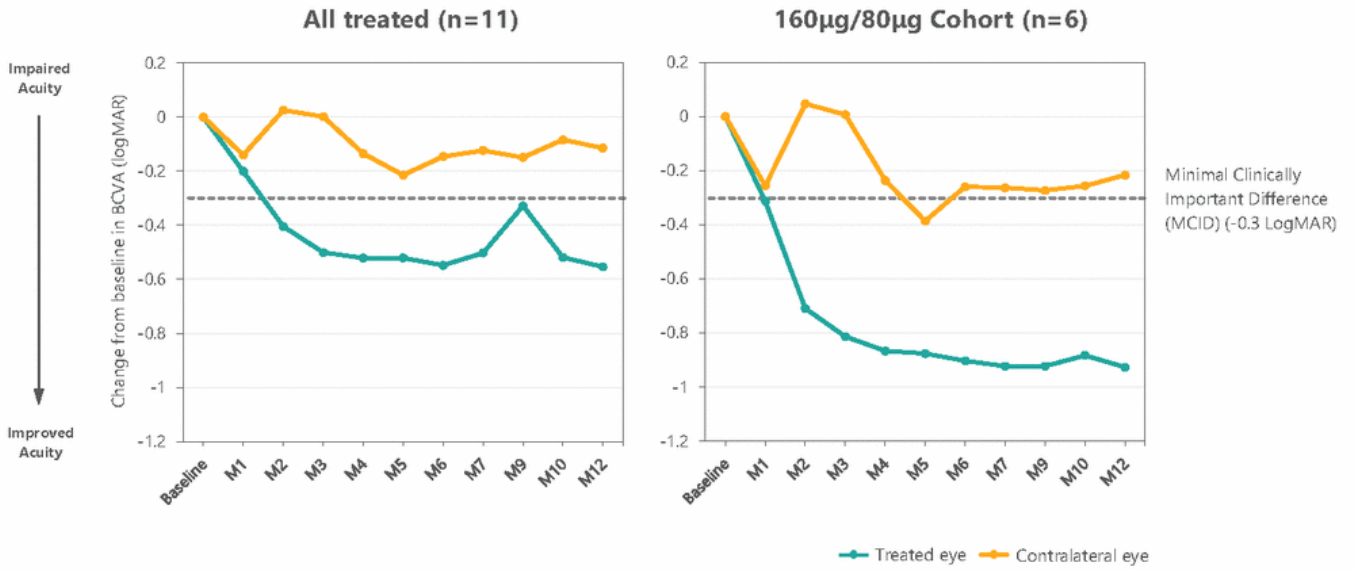
Responder (%)	Treated eye		Contralateral eye	
	US responder threshold	EU responder threshold	US responder threshold	EU responder threshold
320µg/160µg (n=5)	20%	20%	0%	0%

	320µg/160µg (n=5)
Tolerability	No issues
Systemic safety	No issues
Lens opacity	5 findings
Cataract surgery outcome	4 surgeries. Complete recovery of pre-cataract benefit 4/4 subjects
Retinal findings	4 findings in 3 individuals

CME treated topically with improvement. Retinal thinning stabilized 2-3 months

Sustained improvement in BCVA for at least 1 year

All responses (7/7) were maintained for a minimum of 6 months after a maintenance dose



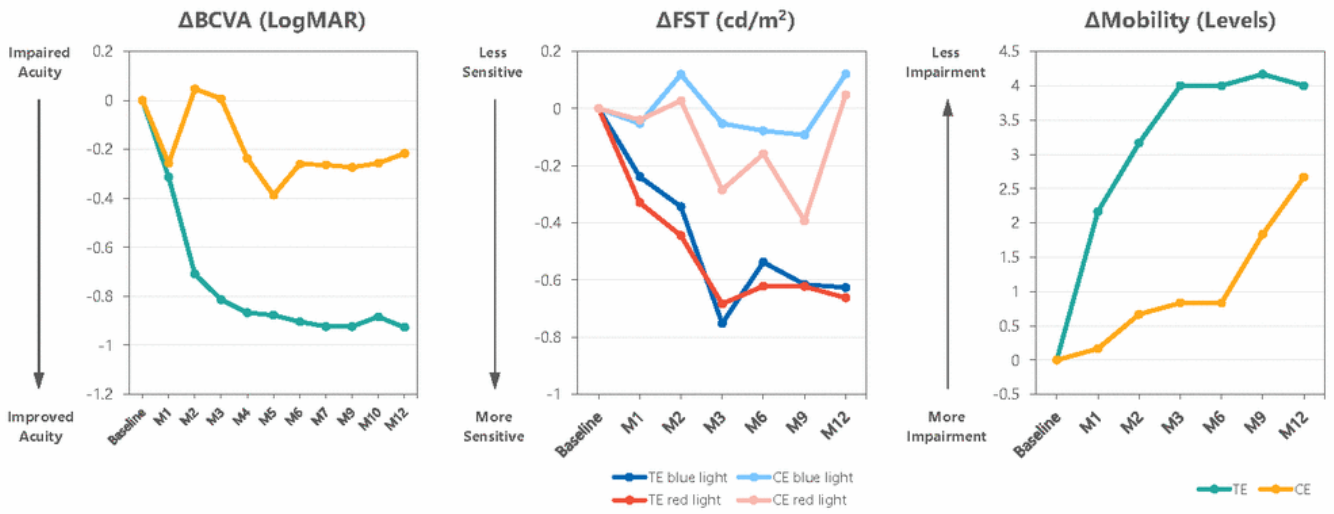
Secondary outcome measures

Mobility testing and full field stimulation

Key outcome measures change month 12

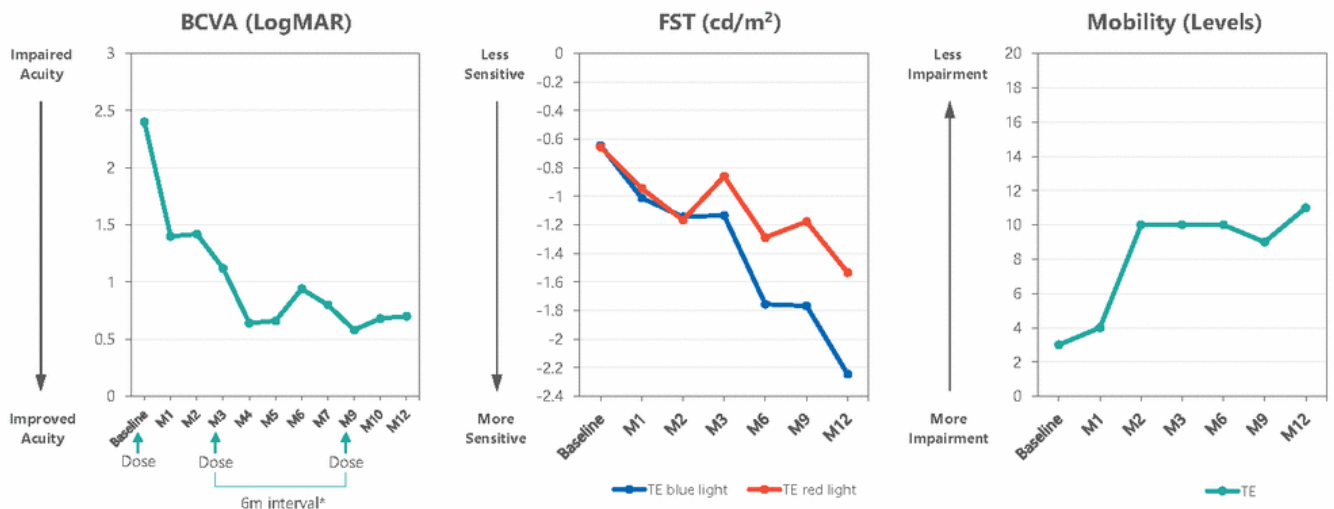
Target registration dose level: 160µg/80µg (n=6)

Every six-month dosing interval-maintained benefit



Example of a 160µg/80µg responder

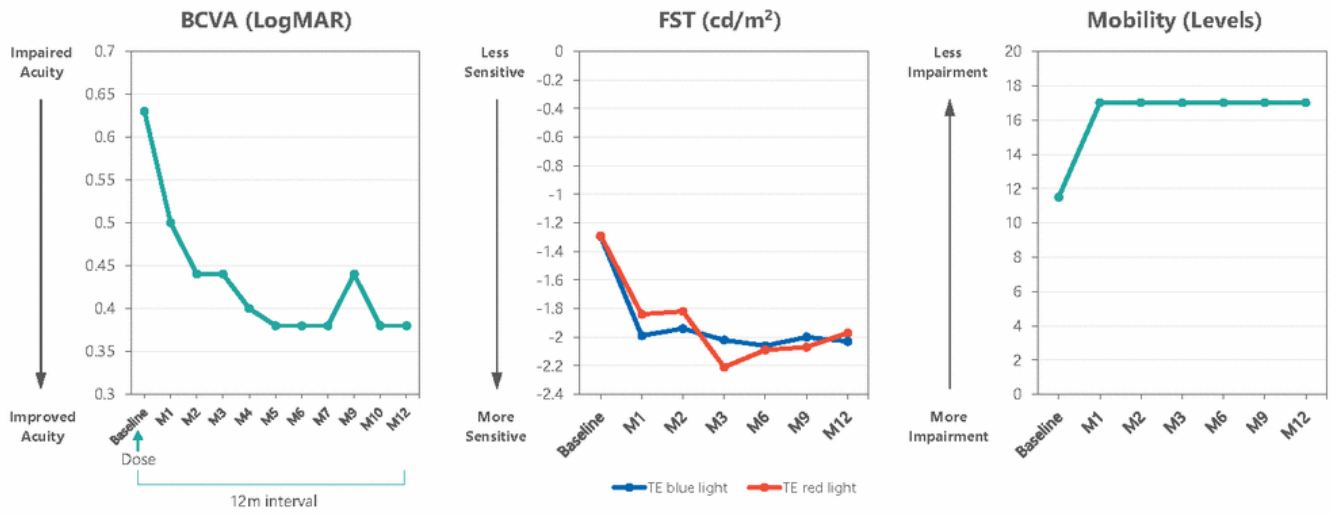
7/7 trial subjects with BCVA improvement sustained that benefit during ≥6-month dosing interval



*7/7 trial subjects with BCVA improvement sustained that benefit during ≥6-month dosing interval

Example of homozygous subject

13-letter improvement in BCVA with robust improvement in mobility and FST



Top-line efficacy data

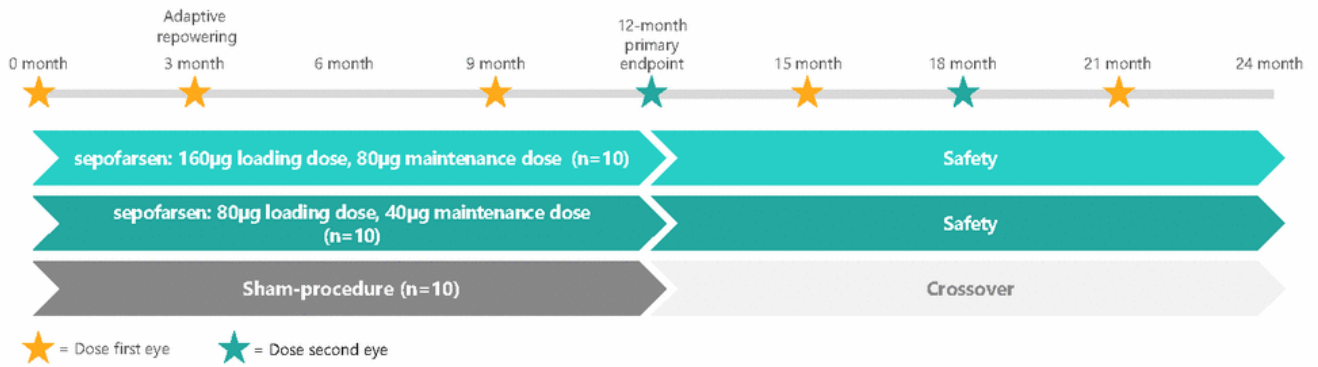
Target registration dose level: 160µg/80µg (n=6)

Group mean	Treated eye	Contralateral eye
BCVA (LogMAR)	-0.93 P<0.01 vs. baseline	-0.22 P=N.S. vs. baseline
FST Red (Log)	-0.66 P<0.01 vs. baseline	0.05 P=N.S. vs. baseline
FST Blue (Log)	-0.63 P<0.01 vs. baseline	0.12 P=N.S. vs. baseline
Mobility (levels)	+4.0 P<0.01 vs. baseline	+2.7 P<0.05 vs. baseline

Significance assessed by mixed effects repeated measures model

Pivotal phase 2/3 trial

Design agreed on with FDA and EMA



- Double-masked, randomized, controlled, 12-month, multiple dose study
- Could serve as the sole registration trial
- Sites in North America and select European countries
- Minimum 30 subjects ≥ 8 years old
- Adaptive repowering when 18 subjects reach month 3 (BCVA, active vs. sham, 80% power, alpha 0.05)
- Enrollment started in 1H 2019
- Primary (registration) endpoint:
 - Visual acuity (ETDRS, BRVT)
- Key secondary endpoints
 - Mobility course
 - Full field stimulus testing (FST)



Inherited retinal disease pipeline

Daniel A. de Boer - Chief Executive Officer

ProQR's VISION2023

A FULLY INTEGRATED INHERITED RETINAL DISEASE COMPANY BY 2023



2 COMMERCIAL PRODUCTS

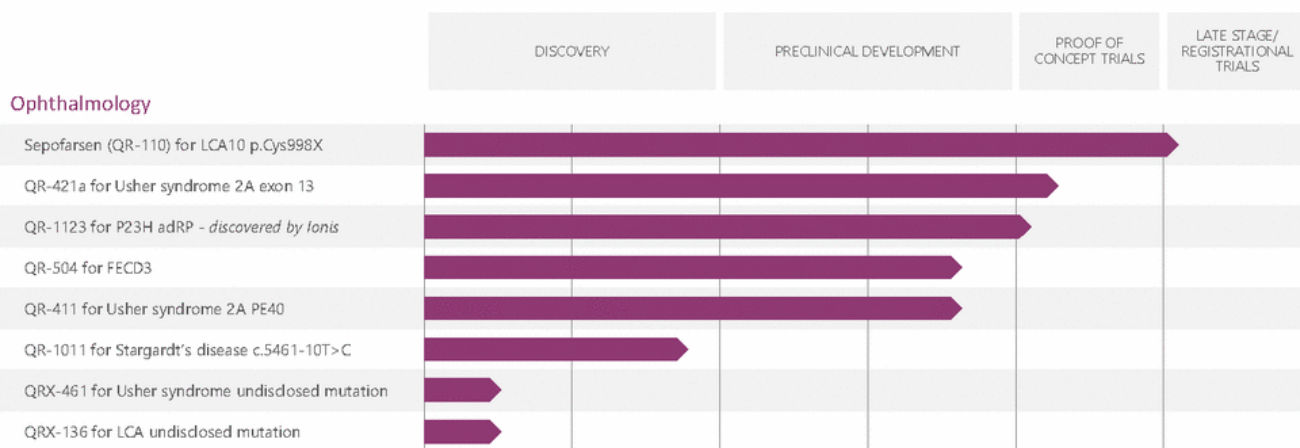


3 LATE STAGE PROGRAMS



7 EARLY STAGE PROGRAMS

Inherited retinal disease pipeline



ProQR's VISION2023

THE 3 PILLARS OF VISION 2023



Discovery engine

- Specialized integrated discovery engine
- Discovering 10 or more new IRD drugs per year
- Use human optic cups to pre-clinically validate clinical molecules



Development

- Utilize digital clinical trials
- Use innovative Bayesian adaptive designs to accelerate time to NDA / MAA



Commercial

- Establish IRD specialized commercial infrastructure in North America and Europe
- Focused on ~approx. 30 specialist centers that treat majority of IRD patients
- Leverage commercial infrastructure for multiple products to same call points

Summary

- **Sepofarsen for LCA10**
 - Phase 1/2 top line data confirms all assumptions for the Phase 2/3 Illuminate pivotal trial
 - Phase 2/3 on track to deliver data in H1 2021
- **QR-421a for Usher syndrome**
 - On track to deliver 12 patient interim data in Q1 2020
- **QR-1123 for adRP**
 - Recently opened IND and expect to dose a first patient in Q4
- Preparations for commercial launch of first product on track

Q&A



IT'S IN
OUR RNA