# 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

May 31, 2017

# **PROQR THERAPEUTICS N.V.**

Zernikedreef 9 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 May 31, 2017, ProQR Therapeutics N.V. issued a press release titled, "ProQR Receives Fast Track Designation from the FDA for QR-110 for Leber's Congenital Amaurosis Type 10." 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. 333-207245).

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.

Date: May 31, 2017

### PROQR THERAPEUTICS N.V.

By: /s/ Smital Shah

Smital Shah Chief Financial Officer

## INDEX TO EXHIBITS

#### Description

Number

99.1

Press release, dated May 31, 2017, titled "ProQR Receives Fast Track Designation from the FDA for QR-110 for Leber's Congenital Amaurosis Type 10



Exhibit 99.1

## ProQR Receives Fast Track Designation from the FDA for QR-110 for Leber's Congenital Amaurosis Type 10

#### **Key Updates**

- ProQR receives Fast Track designation by the U.S. Food and Drug Administration (FDA). Closer interaction with FDA could potentially accelerate the development of QR-110 in patients with Leber's Congenital Amaurosis Type 10 (LCA 10).
- LCA 10 is one of the most prevalent forms of gene-related blindness in children worldwide and currently there are no therapies commercially available or in clinical development.
- QR-110 is currently in clinical development with the planned Phase 1/2 open-label trial (PQ-110-001) that will assess the safety, tolerability, pharmacokinetics and efficacy of multiple administrations of QR-110 in one eye of each patient and will include approximately 6 adults and 6 children with LCA 10
- Top-line trial results are expected in 2018.

LEIDEN, the Netherlands, May 31, 2017 – ProQR Therapeutics N.V. (Nasdaq:PRQR) today announced that it received Fast Track designation from the U.S. Food and Drug Administration (FDA) for QR-110, the lead molecule in its ophthalmology pipeline. QR-110 is being developed for the treatment of patients with Leber's Congenital Amaurosis Type 10 (LCA 10), a rare genetic disease that causes individuals to lose sight, often in the first years of life. QR-110 is a novel investigational RNA oligonucleotide targeting LCA 10 due to the p.Cys998X mutation, which is one of the most prevalent forms of gene-related blindness in children and currently there are no disease modifying therapies commercially available or in clinical development.

Fast Track designation is granted by the FDA to drugs that are under development for serious conditions and have the potential to fulfill an unmet medical need. It was established with the intention to bring promising drugs to patients sooner by facilitating the development with more frequent FDA interactions and expediting the review process.

"QR-110 is a unique and elegant approach to addressing the underlying genetic defect that leads to blindness in individuals with LCA 10 due to the p.Cys998X mutation. We are very pleased with granting of the Fast Track designation by the FDA for this program as it highlights the need for innovative and efficacious medicines for this devastating disease for which there is currently nothing available," said Noreen R. Henig, Chief Medical Officer of ProQR. "We are also excited to be able to initiate our first trial for QR-110 as a multidose study and for that we will benefit from the Fast Track Designation. We believe development of QR-110 also opens the possibilities for RNA approaches to target other causes of genetic blindness. We are building our pipeline in ophthalmology and will use our rapid development approach to QR-110 as a model for how to bring RNA therapeutics to patients in need."

#### **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 cystic fibrosis, Leber's congenital amaurosis Type 10 and dystrophic epidermolysis bullosa. Based on our unique proprietary RNA repair platform technologies we are growing our pipeline with patients and loved ones in mind. \*Since 2012\*

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#### About QR-110

QR-110 is a first-in-class investigational RNA-based oligonucleotide designed to address the underlying cause of Leber's congenital amaurosis Type 10 due to the p.Cys998X 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. QR-110 is designed to restore wild-type CEP290 mRNA leading to the production of wild-type CEP290 protein by binding to the mutated location in the pre-mRNA causing normal splicing of the pre-mRNA. QR-110 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.

#### About Leber's Congenital Amaurosis Type 10

Leber's congenital amaurosis is the most common cause of blindness due to genetic disease in children and consists of a group of diseases of which LCA Type 10 (LCA 10) is one of the more severe forms. LCA 10 is caused by mutations in the CEP290 gene of which the p.Cys998X mutation is most common. LCA 10 leads to progressive loss of vision causing most patients to lose their sight in the first few years of life. To date, there are no treatments approved or products in clinical development that treat the underlying cause of the disease. Although prevalence rates vary, we believe approximately 2,000 people in the Western world have LCA 10 because of this mutation.

#### About the PQ-110-001 Study

PQ-110-001 is an open-label trial that will include approximately 6 children (age 6- 17 years) and 6 adults (<sup>3</sup> 18 years) that have LCA 10 due to one or two copies of the p.Cys998X mutation. During the trial, patients will receive four intravitreal injections of QR-110 into one eye; one every three months for one year. The QR-110 trial is expected to be conducted in three centers with significant expertise in genetic retinal disease in the US and Europe.

The primary endpoints will be safety and tolerability. Secondary efficacy endpoints will assess the pharmacokinetics and restoration/improvement of visual function and retinal structure through ophthalmic tests such as visual acuity, full field stimulus testing (FST), optical coherence tomography (OCT), pupillary light reflex (PLR), mobility course and fixation stability. Changes in quality of life in LCA subjects will also be evaluated. Top-line results from the trial are expected to be available in 2018.

#### 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. 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. These forward-looking statements include, but are not limited to, statements regarding QR-110 and the clinical development and the therapeutic potential thereof, statements regarding PQ-110-001, including trial design and expected timing of results, statements regarding Fast Track designation, and statements regarding our ongoing and planned discovery and development of product candidates and the timing thereof, including those in our innovation pipeline. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with our clinical development activities, including that positive results observed in our prior and ongoing studies may not be replicated in later trials or guarantee approval of any product candidate by regulatory authorities, that a Fast Track designation by the FDA may not actually lead to a faster development, regulatory review or approval

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process, manufacturing processes and facilities, regulatory oversight, product commercialization, intellectual property claims, and 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.

#### **ProQR Therapeutics N.V.:**

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