# UNITED STATES SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 6	<b>5-K</b>
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Report of Foreign Private Issuer Pursuant to Rule 13a-16 or 15d-16 of the Securities Exchange Act of 1934

**November 20, 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): □

Furnished as Exhibit 99.1 to this Report on Form 6-K are the unaudited financial statements of ProQR Therapeutics N.V. (the "Company") for the three and nine month period ended September 30, 2017 and furnished as Exhibit 99.2 to this Report on Form 6-K is a press release of ProQR Therapeutics N.V. dated November 20, 2017, announcing the Company's results for the three and nine month period ended September 30, 2017. The Company hereby incorporates by reference the information contained in this report 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: November 20, 2017

# PROQR THERAPEUTICS N.V.

By: /s/ Smital Shah

Smital Shah

Chief Financial Officer

# INDEX TO EXHIBITS

Number	Description
99.1	Unaudited financial statements of ProQR Therapeutics N.V. for the three and nine month period ended September 30, 2017.
99.2	Press Release of ProQR Therapeutics N.V. dated November 20, 2017, announcing the Company's results for the three and nine month period ended September 30, 2017.

# PROQR THERAPEUTICS N.V. Index to Unaudited Condensed Consolidated Financial Statements

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# PROQR THERAPEUTICS N.V. Unaudited Condensed Consolidated Statement of Financial Position

	September 30, 	December 31, 2016
	€ 1,000	€ 1,000
Assets		
Current assets		
Cash and cash equivalents	39,742	59,200
Prepayments and other receivables	2,283	2,420
Social securities and other taxes	777	395
Total current assets	42,802	62,015
Property, plant and equipment	2,740	3,438
Intangible assets	52	90
Total assets	45,594	65,543
Equity and liabilities		
Equity		
Equity attributable to owners of the Company	32,749	53,136
Non-controlling interests	(10)	
Total equity	32,739	53,136
Current liabilities		· <u> </u>
Trade payables	250	328
Social securities and other taxes	314	312
Pension premiums	34	13
Deferred income	657	
Other current liabilities	5,117	6,057
Total current liabilities	6,372	6,710
Borrowings	6,483	5,697
Total liabilities	12,855	12,407
Total equity and liabilities	45,594	65,543

The notes are an integral part of these condensed consolidated financial statements.

# PROQR THERAPEUTICS N.V.

# **Unaudited Condensed Consolidated Statement of Profit or Loss and OCI**

(€ in thousands, except share and per share data)

		onth period ptember 30,	Nine month period ended September 30,		
	2017	2016	2017	2016	
	€ 1,000	€ 1,000	<u>€ 1,000</u>	€ 1,000	
Other income	326	447	984	1,725	
Research and development costs	(7,226)	(8,319)	(22,808)	(23,823)	
General and administrative costs	(2,753)	(2,001)	(7,949)	(7,218)	
Total operating costs	(9,979)	(10,320)	(30,757)	(31,041)	
Operating result	(9,653)	(9,873)	(29,773)	(29,316)	
Finance income and expense	(868)	(254)	(2,589)	(968)	
Result before corporate income taxes	(10,521)	(10,127)	(32,362)	(30,284)	
Income taxes			(2)	_	
Result for the period	(10,521)	(10,127)	(32,364)	(30,284)	
Other comprehensive income	49	_	114	_	
Total comprehensive income (attributable to owners of the					
Company)	(10,472)	(10,127)	(32,250)	(30,284)	
Result attributable to					
Owners of the Company	(10,511)	(10,127)	(32,354)	(30,284)	
Non-controlling interests	(10)	_	(10)	_	
	(10,521)	(10,127)	(32,364)	(30,284)	
Share information					
Weighted average number of shares outstanding1	25,282,588	23,346,856	24,255,792	23,346,390	
Earnings per share attributable to owners of the Company					
(expressed in Euro per share)					
Basic loss per share <sup>1</sup>	(0.42)	(0.43)	(1.33)	(1.30)	
Diluted loss per share <sup>1</sup>	(0.42)	(0.43)	(1.33)	(1.30)	

The notes are an integral part of these condensed consolidated financial statements.

1. For this period presented in these financial statements, the potential exercise of share options is not included in the diluted earnings per share calculation as the Company was loss-making in all periods. Due to the anti-dilutive nature of the outstanding options, basic and diluted earnings per share are equal in this period.

# PROQR THERAPEUTICS N.V.

# **Unaudited Condensed Consolidated Statement of Changes in Equity**

		Attributable to owners of the Company							
	Number of shares	Share Capital	Share Premium	Equity Settled Employee Benefit Reserve	Translation Reserve	Accumulated Deficit	Total	Non- controlling interests	Total Equity
		€1,000	€ 1,000	€ 1,000	€ 1,000	€ 1,000	€ 1,000	€ 1,000	€ 1,000
Balance at January 1, 2016	23,345,965	934	123,595	1,899	1	(36,630)	89,799		89,799
Result for the period						(30,284)	(30,284)		(30,284)
Other comprehensive income					0		0		0
Recognition of share-based payments				1,917			1,917	_	1,917
Share options exercised	891	0	2				2		2
Balance at September 30, 2016	23,346,856	934	123,597	3,816	1	(66,914)	61,434		61,434
Balance at January 1, 2017	23,346,856	934	123,597	4,353	(15)	(75,733)	53,136	_	53,136
Result for the period				_		(32,354)	(32,354)	(10)	(32,364)
Other comprehensive income					114		114	_	114
Recognition of share-based payments				3,090			3,090		3,090
Shares issued in the period	2,115,612	85	8,677	_	_	_	8,762	0	8,762
Share options exercised	381	0	1				1		1
Balance at September 30, 2017	25,462,849	1,019	132,275	7,443	99	(108,087)	32,749	(10)	32,739

The notes are an integral part of these condensed consolidated financial statements

# PROQR THERAPEUTICS N.V. Unaudited Condensed Consolidated Statement of Cash Flows

	Three month period ended September 30,		Nine mon ended Sept	ember 30,
	2017	2016	2017	2016
	€ 1,000	€ 1,000	<u>€ 1,000</u>	<u>€ 1,000</u>
Cash flows from operating activities  Net result	(10,472)	(10,127)	(32,250)	(30,284)
Adjustments for:	(10,472)	(10,127)	(32,230)	(30,204)
— Depreciation	267	284	807	978
— Share-based compensation	890	628	3,090	1,917
— Financial income and expenses	868	254	2,589	968
Changes in working capital	829	(1,843)	(539)	(551)
Cash used in operations	(7,618)	(10,804)	(26,303)	(26,972)
Corporate income tax paid			(2)	
Interest received/(paid)	10	11	69	77
Net cash used in operating activities	(7,608)	(10,793)	(26,236)	(26,895)
Cash flow from investing activities				
Purchases of intangible assets				
Purchases of property, plant and equipment	(18)	(422)	(111)	(2,495)
Net cash used in investing activities	(18)	(422)	(111)	(2,495)
Cash flow from financing activities				
Proceeds from issuance of shares, net of transaction costs	5,539		8,762	
Proceeds from exercise of share options			1	2
Proceeds from borrowings	100		201	193
Proceeds from convertible loans	150	_	150	_
Redemption of financial lease				(15)
Net cash generated by financing activities	5,789		9,114	180
Net increase/(decrease) in cash and cash equivalents	(1,837)	(11,215)	(17,233)	(29,210)
Currency effect cash and cash equivalents	(742)	(175)	(2,225)	(734)
Cash and cash equivalents, at beginning of the period	42,321	76,311	59,200	94,865
Cash and cash equivalents at the end of the period	39,742	64,921	39,742	64,921

The notes are an integral part of these condensed consolidated financial statements.

#### PROOR THERAPEUTICS N.V.

### **Notes to Unaudited Condensed Consolidated Financial Statements**

#### 1. General information

ProQR Therapeutics N.V., or "ProQR" or the "Company", is a development stage company that primarily focuses on the development and commercialization of novel therapeutic medicines.

Since September 18, 2014, the Company's ordinary shares are listed on the NASDAQ Global Market under ticker symbol PRQR.

The Company was incorporated in the Netherlands, on February 21, 2012 and has been reorganized from a private company with limited liability to a public company with limited liability on September 23, 2014. The Company has its statutory seat in Leiden, the Netherlands. The address of its headquarters and registered office is Zernikedreef 9, 2333 CK Leiden, the Netherlands.

ProQR Therapeutics N.V. is the ultimate parent company of the following entities:

- ProQR Therapeutics Holding B.V. (100%);
- ProQR Therapeutics I B.V. (100%);
- ProQR Therapeutics II B.V. (100%);
- ProQR Therapeutics III B.V. (100%);
- ProQR Therapeutics IV B.V. (100%);
- ProQR Therapeutics VI B.V. (100%);
- ProQR Therapeutics VII B.V. (100%);
- ProQR Therapeutics VIII B.V. (100%);
- ProQR Therapeutics IX B.V. (100%);
- ProQR Therapeutics I Inc. (100%);
- Stichting Bewaarneming Aandelen ProQR (100%);
- Amylon Therapeutics B.V. (majority interest).

As used in these condensed consolidated financial statements, unless the context indicates otherwise, all references to "ProQR" or the "Company" refer to ProQR Therapeutics N.V. including its subsidiaries.

## 2. Significant Accounting Policies

These condensed consolidated financial statements have been prepared in accordance with International Financial Reporting Standards ("IFRS"), as issued by the International Accounting Standards Board ("IASB"), in particular IAS 34 - Interim Financial Reporting. Certain information and disclosures normally included in financial statements prepared in accordance with IFRS have been condensed or omitted. Accordingly, these condensed consolidated financial statements should be read in conjunction with the Company's annual financial statements for the year ended December 31, 2016. In the opinion of management, all adjustments, consisting of normal recurring nature, considered necessary for a fair presentation have been included in the condensed consolidated financial statements.

The Company's financial results have varied substantially, and are expected to continue to vary, from period to period. The Company believes that its ordinary activities are not linked to any particular seasonal factors.

The Company operates in one reportable segment, which comprises the discovery and development of innovative, RNA based therapeutics.

## 3. Adoption of new and revised International Financial Reporting Standards

The accounting policies adopted in the preparation of the condensed consolidated financial statements are consistent with those applied in the preparation of the Company's annual financial statements for the year ended December 31, 2016. New Standards and Interpretations, which became effective as of January 1, 2017, did not have a material impact on our condensed consolidated financial statements.

### 4. Critical Accounting Estimates and Judgments

In the application of the Company's accounting policies, management is required to make judgments, estimates and assumptions about the carrying amounts of assets and liabilities that are not readily apparent from other sources. The estimates and associated assumptions are based on historical experience and other factors that are considered to be relevant. Actual results may differ from these estimates.

The estimates and underlying assumptions are reviewed on an ongoing basis. Revisions to accounting estimates are recognized in the period in which the estimate is revised if the revision affects only that period or in the period of the revision and future periods if the revision affects both current and future periods.

#### (a) Share-based payments

Share options granted to employees and consultants are measured at the fair value of the equity instruments granted. Fair value is determined through the use of the Black-Scholes option-pricing model, which is considered the most appropriate model for this purpose by management.

Initially, the Company's ordinary shares were not publicly traded and consequently the Company needed to estimate the fair value of its share and the expected volatility of that value. Please refer to the Company's annual financial statements for the year ended December 31, 2016 for the assumptions used in those estimates. The value of the underlying shares was determined on the basis of the prior sale of company stock method. As such, the Company has benchmarked the value per share to external transactions of Company shares and external financing rounds.

For options granted from the moment of listing, the Company uses the closing price of the ordinary shares on the previous business day as exercise price of the options granted.

The result of the share option valuations and the related compensation expense is dependent on the model and input parameters used. Even though Management considers the fair values reasonable and defensible based on the methodologies applied and the information available, others might derive a different fair value for the Company's share options.

### (b) Corporate income taxes

The Company recognizes deferred tax assets arising from unused tax losses or tax credits only to the extent that the Company has sufficient taxable temporary differences or there is convincing evidence that sufficient taxable profit will be available against which the unused tax losses or unused tax credits can be utilized. Management's judgment is that such convincing evidence is currently not sufficiently available and a deferred tax asset is therefore only recognized to the extent that the Company has sufficient taxable temporary differences.

#### (c) Grant income

Grants (to be) received are reflected in the balance sheet as other receivables or deferred income. At each balance sheet date, for grants approved, the Company estimates the associated costs incurred, the level of service performed and the progress of the associated projects. Based on this analysis grant income is recognized.

# (d) Research and development expenditures

Research expenditures are currently not capitalized but are reflected in the income statement because the criteria for capitalization are not met. At each balance sheet date, the Company estimates the level of service performed by the vendors and the associated costs incurred for the services performed.

Although we do not expect the estimates to be materially different from amounts actually incurred, the understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and could result in reporting amounts that are too high or too low in any particular period.

The condensed consolidated financial statements do not include all disclosures for critical accounting estimates and judgments that are required in the annual consolidated financial statements and should be read in conjunction with the Company's annual financial statements for the year ended December 31, 2016.

#### 5. Cash and Cash Equivalents

At September 30, 2017, the Company's cash and equivalents were  $\in$  39,742,000 as compared to  $\in$  59,200,000 at December 31, 2016. A significant portion of the cash balance is denominated in US dollars. The cash balances are held at banks with investment grade credit ratings. The cash at banks is at full disposal of the Company.

#### 6. Current liabilities

At September 30, 2017 and December 31, 2016, the other current liabilities consisted principally of accruals for services provided by vendors not yet billed and other miscellaneous liabilities.

#### 7. Borrowings

	September 30, 2017	December 31, 2016	
	€ 1,000	€ 1,000	
Innovation credit	4,799	4,598	
Accrued interest on innovation credit	1,533	1,099	
Convertible notes	151		
Total borrowings	6,483	5,697	

Innovation credit ("Innovatiekrediet")

On June 1, 2012, ProQR was awarded an Innovation credit by the Dutch government, through its agency RVO (previously: "AgentschapNL") of the Ministry of Economic Affairs, for the Company's cystic fibrosis program. The credit was increased in the course of 2013 through 2017. The credit covers 35% of the costs incurred in respect of the program up to an initial maximum of € 5.0 million through March 31, 2018.

The credit is interest-bearing at a rate of 10% per annum. The credit, including accrued interest, is repayable in three instalments on November 30, 2018, November 30, 2019 and November 30, 2020, depending on the technical success of the program.

The assets which are co-financed with the granted innovation credit are subject to a right of pledge for the benefit of RVO.

### 8. Shareholders' equity

The authorized share capital of the Company amounting to  $\{0.04\}$  per share. All issued shares have been fully paid in cash.

In October 2015, we entered into an agreement for an at-the-market offering facility, or ATM facility, pursuant to which we may issue shares of our common stock from time to time under our shelf registration statement up to a maximum of \$ 60.0 million. In the third quarter 2017, 157,600 shares were issued pursuant to our ATM facility, resulting in proceeds of € 675,000, net of € 21,000 of offering expenses. As at September 30, 2017, we have issued 915,612 shares pursuant to our ATM facility, resulting in proceeds of € 3,898,000, net of € 120,000 of offering expenses.

#### Translation reserve

The translation reserve comprises all foreign currency differences arising from the translation of the financial statements of foreign operations.

#### Share options

The Company operates an equity-settled share-based compensation plan which was introduced in 2013. The supervisory board may grant options to employees, members of the supervisory board, members of the management board and consultants. The quarterly compensation expenses included in operating costs for this plan were € 890,000 (2016: € 628,000), of which € 468,000 (2016: € 384,000) was recorded in general and administrative costs and € 422,000 (2016: € 244,000) was recorded in research and development costs.

#### 9. Other income

	Three month	Three month period ended September 30,			
	2017		2016		
	€ 1,00	0 €	1,000		
Grant income	13-	4	402		
Rental income from property subleases	193	2	45		
	32	6	447		

In August 2014, the Company entered into an agreement with Cystic Fibrosis Foundation Therapeutics, Inc., or CFFT, a subsidiary of the Cystic Fibrosis Foundation, pursuant to which CFFT agreed to provide the Company with up to \$ 3 million to support the clinical development of QR-010.

Pursuant to the terms of the agreement, the Company was obligated to make a one-time milestone payment to CFFT of up to approximately \$ 80 million, payable in three equal annual installments following the first commercial sale of QR-010, as well as certain other milestones and royalties. In August 2017, the one-time milestone payment was amended to approximately \$ 16 million, payable in the same schedule. Further, an amendment was made to the effect that the approximately \$ 6 million payable in case of a change of control transaction may be set-off against the milestone payment mentioned in the preceding sentence. There were no other changes to the milestones or royalties.

In 2015, the European Commission (EC) through its Horizon 2020 program awarded ProQR and its academic partners a grant of € 6 million (ProQR: € 4.4 million) to support the clinical development of QR-010 in the period up till December 31, 2017. Horizon 2020 is one of the largest research and innovation programs in the European Union with nearly € 80 billion in available funding for qualified projects from 2014 to 2020.

Both grants are recognized in other income in the same period in which the related R&D costs are recognized.

#### 10. Research and development costs

Research and development costs amount to & 7,226,000 for the quarter ended September 30, 2017 compared to & 8,319,000 for the same period in 2016 and comprised of allocated employee costs including share-based payments, the costs of materials and laboratory consumables, outsourced activities, license and intellectual property costs and other allocated costs. The decrease in expenses was primarily due to the completion of the nasal potential difference (NPD) study for QR-010.

#### 11. General and administrative costs

General and administrative costs amount to € 2,753,000 for the quarter ended September 30, 2017 compared to € 2,001,000 for the quarter ended September 30, 2016.

#### 12. Income taxes

Due to the operating losses incurred since inception the Company has no tax provisions as of the balance sheet date. Furthermore, no significant temporary differences exist between accounting and tax results. Realization of deferred tax assets is dependent on future earnings, if any, the timing and amount of which are uncertain. Accordingly, the Company has not yet recognized any deferred tax asset related to operating losses.

#### 13. Events after balance sheet date

- In November, Chief Medical Officer, Noreen R. Henig, M.D., departed the Company to pursue a new opportunity and will serve as a special advisor to the Company going forward. David Rodman, M.D., Chief Development Strategy Officer, assumed leadership over clinical development. Dr. Rodman joined ProQR in March 2017 with extensive experience in rare disease drug development, translational medicine and RNA therapeutics, having previously served in leadership roles with Novartis Institute for Biomedical Research (NIBR), Vertex Pharmaceuticals, miRagen Therapeutics and Nivalis Therapeutics. The Company also announced the promotions of Peter Adamson to Senior Vice President Ophthalmology Franchise and Robert Friesen to Senior Vice President Science and Early Development.
- In November, the first patient was dosed in the Phase 1/2 open-label trial (PQ-110-001) assessing the safety, tolerability, pharmacokinetics and efficacy of QR-110 in patients with LCA 10, the most common cause of blindness due to genetic disease in children. The trial will enroll six children (age 6 17 years) and six adults (3 18 years) who have LCA 10 due to one or two copies of the p.Cys998X mutation in the CEP290 gene. During the trial, patients will receive four intravitreal injections of QR-110 into one eye; once every three months. 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 objectives of the trial will include safety, tolerability, pharmacokinetics and efficacy as measured by restoration or improvement of visual function and retinal structure through ophthalmic endpoints 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 the trial subjects will also be evaluated. QR-110 is the Company's lead program for genetic blindness and has received fast track designation by the FDA and been granted ODD in the US and EU. Interim safety and efficacy trial results from the majority of patients after six months of treatment are expected in 2018, with full 12-month treatment data from all patients expected in 2019.
- In November, the Company consummated an underwritten public offering and concurrent registered direct offering of ordinary shares at a price of \$3.25 per share. In addition, in the public offering, ProQR granted the underwriters a 30-day option to purchase up to 745,471 additional ordinary shares at the public offering price, less underwriting discounts and commissions. Gross proceeds from both offerings are expected to be approximately \$20 million, assuming no exercise of the underwriters' option to purchase additional shares in the public offering. Proceeds from these offerings along with existing cash on the balance sheet, are expected to fund operations into the second half of 2019.

ProQR Therapeutics N.V. Press Release November 20, 2017

#### FINAL - FOR RELEASE

ProQR'

ProQR Announces Results for the Third Quarter of 2017

#### **Key updates**

- Runway into the second half of 2019: cash of €39.7 million at end of Q3, increased by gross proceeds of approximately \$20 million from an issuance of ordinary shares in November 2017 that will fund operations through potential clinical data readouts in three different programs.
- Positive results from the Phase 1b safety and tolerability clinical trial of QR-010 in cystic fibrosis (CF) patients with the F508del mutation. QR-010 was observed to be safe and well-tolerated, and demonstrated encouraging efficacy responses in CF patients.
- First patient dosed in the Phase 1/2 safety & efficacy trial of QR-110 in children and adults with Leber's congenital amaurosis 10 in November. First clinical data readout expected in 2018.
- QR-313 for dystrophic epidermolysis bullosa completed the IND enabling studies, received ODD from FDA and pre-clinical data was presented at two scientific meetings. QR-313 is expected to enter the clinic in 2018, with interim data also expected in 2018.
- Spin-out of Amylon Therapeutics as a privately-held company focused on central nervous system therapeutics. ProQR continues to be a majority shareholder and is eligible to receive future milestones and royalties.
- Drs. Phil Zamore, Cy Stein, Scott Armstrong and Thaddeus (Ted) Dryja appointed to ProQR's Scientific Advisory Board (SAB).
- In vivo proof of concept data for novel Axiomer® RNA editing platform technology presented at the OTS meeting and a DIA industry event.
- QR-421 and QR-411 for Usher syndrome received orphan drug designation (ODD) from FDA and EMA.

LEIDEN, the Netherlands, November 20, 2017 - ProQR Therapeutics N.V. (Nasdaq: PRQR), today announced results for the third quarter of 2017.

"I am proud of the milestones our team achieved recently, which included the successful completion and positive data from our Phase 1b study of QR-010 in CF patients, dosing of the first LCA 10 patient in our Phase 1/2 trial of QR-110, announcing the *in vivo* PoC for our Axiomer technology and the completion of the pre-clinical IND-enabling studies for QR-313 to enable the potential start of our clinical trial in 2018. We look forward to the data-rich 12-18 months ahead of us, with important clinical safety & efficacy readouts in three different clinical programs," said Daniel A. de Boer, CEO of ProQR. "Beyond these, we have made significant progress in our pipeline of ophthalmology programs and our novel RNA-editing technology, Axiomer®. We are also thrilled with the spin out of Amylon as a privately-held company to a group of private and institutional investors, while maintaining our majority ownership in Amylon. This enables the program to attract external funding for these important central nervous system (CNS) programs that have the potential to make a meaningful difference for patients. Finally, I would like to take this opportunity to welcome Drs. Phil Zamore, Cy Stein, Scott Armstrong and Thaddeus (Ted) Dryja to the SAB, who are all well-regarded in their respective fields and bring with them a wealth of knowledge and experience that will help us to further build and advance our pipeline."

ProQR Therapeutics N.V. | Zernikedreef 9, 2333 CK Leiden, The Netherlands | +31 88 166 7000 | info@proqr.com | www.proqr.com

### **Corporate Highlights**

- In July, the Company completed a \$6.0 million registered direct offering of 1.2 million ordinary shares at an issue price of \$5.00 per share with institutional investors.
- In July, QR-411 for Usher syndrome received orphan drug designation (ODD) from the FDA and European Medicines Agency (EMA). QR-411 targets the underlying cause of Usher syndrome due to the c.7595-2144A>G mutation in the USH2A gene. In September, the QR-421 candidate received ODD from the FDA and EMA. QR-421 is designed to address Usher syndrome due to mutations in exon 13 of the USH2A gene. QR-411 and QR-421 are part of the Company's growing ophthalmology pipeline which also includes lead candidate, QR-110 for LCA 10 currently in clinical trials, and two additional pipeline programs, QRX-1011 for Stargardt's disease, and QRX-504 for Fuchs endothelial corneal dystrophy.
- In August, Dr. Phil Zamore, Dr. Cy Stein and Dr. Scott Armstrong were appointed to the Scientific Advisory Board (SAB), joining fellow members Dr. Art Levin and Dr. Annemieke Aartsma-Rus. The SAB will play a key strategic role as the Company develops its pipeline of RNA therapeutics and novel proprietary technology platforms for severe genetic rare diseases.
- In September, the Company completed the spin out of Amylon Therapeutics as a privately-held company focused on the development of therapies for central nervous system (CNS) disorders. ProQR granted an exclusive license to Amylon to develop therapeutics for CNS disorders, with an initial focus on a RNA-based treatment for a rare genetic disease that leads to strokes at mid-adulthood, called Hereditary Cerebral Hemorrhage with Amyloidosis of the Dutch type (HCHWA-D). ProQR retained majority ownership in Amylon and is entitled to future milestones and royalties from potential products developed under the license agreement.
- In September, QR-313 for dystrophic epidermolysis bullosa (DEB) received ODD from the FDA, representing the fifth program in the Company's pipeline to receive ODD in the U.S. DEB is a rare genetic disease that can lead to severe blistering of the skin resulting in high treatment burden and poor quality of life for people with DEB. QR-313 is designed for topical administration and targets the most common mutations within DEB, the mutations in exon 73 of the COL7A1 gene. QR-313 pre-clinical data was presented at the EB2017 Research Conference and ESDR Meeting, both in Salzburg, Austria. A first-in-human clinical trial of QR-313 is expected to be initiated in 2018, with interim data readout also expected in 2018 and full data in 2019.
- In September, ProQR presented *in vivo* proof of concept data for its novel and proprietary Axiomer® RNA editing technology platform at the Oligonucleotide Therapeutics Society (OTS) meeting in Bordeaux, France and at the Drug Information Association (DIA) industry event. The data demonstrated that in an *in vivo* research model of Hurler syndrome, treatment with the Axiomer® EONs resulted in editing of RNA and partial restoration of the enzymatic activity that is missing in this syndrome. Additionally, the increase in enzymatic activity correlated well with reduced levels of the enzyme's substrate, the accumulation of which results in the characteristics of the syndrome. The Axiomer® "Editing Oligo Nucleotides" (EONs) recruit an endogenously expressed RNA editing system called ADAR, which it can direct to change an Adenosine (A) to an Inosine (I) in the RNA an Inosine is translated as a Guanosine (G). The Axiomer® platform has the potential to yield a new class of medicines for genetic diseases caused by single nucleotide G-to-A mutations.
- In September, the Company announced positive preliminary top-line results from the Phase 1b randomized, double-blind, placebo-controlled, dose escalation study (Study PQ-010-001) to evaluate the safety, tolerability, pharmacokinetics and exploratory efficacy of QR-010 in adults with CF homozygous for the F508del mutation. A number of exploratory efficacy endpoints were assessed in the multiple dose groups. A total of 4 dose levels were studied: 6.25, 12.5, 25 and 50 mg of QR-010 in solution per dose administered via inhalation using the PARI eFlow® nebulizer. Patients eligible to participate were males and females of 18 years and over with a ppFEV<sub>1</sub> of <sup>3</sup>70% at time of inclusion, homozygous for the F508del mutation, and not taking CFTR modulator drugs. The study was designed to enroll 8 cohorts of 8 subjects (6 receiving QR-010, 2 receiving placebo). In cohorts 1-4, a single dose of QR-010 was administered, and in cohorts 5-8, twelve doses of QR-010 were administered over a 4-week period. Based on preliminary top-line results from the trial, QR-010 was observed to be safe and well-tolerated

across all dose levels and we believe that QR-010 may have therapeutic benefit for CF patients. Most patients in the trial reported having a reduction in CF symptoms after receiving QR-010 (as measured by an increase in the Cystic Fibrosis Questionnaire-Revised Respiratory Symptom Score, or CFQ-R RSS) compared to placebo. 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. Subjects that received placebo did not report this reduction in CF symptoms or improvement in lung function. As expected, no change was observed on sweat chloride and weight gain

#### **Subsequent Events**

- In October, Dr. Thaddeus (Ted) Dryja, a pioneer in the field of retinal genetic diseases, joined the Company's Scientific Advisory Board. Dr. Dryja is a member of the faculty at Massachusetts Eye and Ear and Harvard Medical School, and previously served as the Global Head of Ophthalmology Research at the Novartis Institutes for BioMedical Research. He is expected to be a key advisor as the Company advances its growing ophthalmology pipeline.
- In November, Chief Medical Officer, Noreen R. Henig, M.D., departed the Company to pursue a new opportunity and will serve as a special advisor to the Company going forward. David Rodman, M.D., Chief Development Strategy Officer, assumed leadership over clinical development. Dr. Rodman joined ProQR in March 2017 with extensive experience in rare disease drug development, translational medicine and RNA therapeutics, having previously served in leadership roles with Novartis Institute for Biomedical Research (NIBR), Vertex Pharmaceuticals, miRagen Therapeutics and Nivalis Therapeutics. The Company also announced the promotions of Peter Adamson to Senior Vice President Ophthalmology Franchise and Robert Friesen to Senior Vice President Science and Early Development.
- In November, J. Stuart Elborn, Clinical Chair in Respiratory Medicine at Imperial College, Consultant at Royal Brompton Hospital, and immediate past-president of the European Cystic Fibrosis Society, presented data from the Phase 1b safety, tolerability and exploratory efficacy study of QR-010 during the North American Cystic Fibrosis Conference (NACFC) in Indianapolis, Indiana. During the meeting, the Company also held an investor and analyst event to discuss the recent Phase 1b data, new opportunities to target stop-codon (or Class I) mutations in the CFTR gene using its Axiomer® technology platform, and provide an update on other candidates in the pipeline.
- In November, the Company presented its oligonucleotide therapeutics approach at the EuroTIDES meeting in Vienna, Austria.
- In November, the first patient was dosed in the Phase 1/2 open-label trial (PQ-110-001) assessing the safety, tolerability, pharmacokinetics and efficacy of QR-110 in patients with LCA 10, the most common cause of blindness due to genetic disease in children. The trial will enroll six children (age 6 17 years) and six adults (3 18 years) who have LCA 10 due to one or two copies of the p.Cys998X mutation in the *CEP290* gene. During the trial, patients will receive four intravitreal injections of QR-110 into one eye; once every three months. 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 objectives of the trial will include safety, tolerability, pharmacokinetics and efficacy as measured by restoration or improvement of visual function and retinal structure through ophthalmic endpoints 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 the trial subjects will also be evaluated. QR-110 is the Company's lead program for genetic blindness and has received fast track designation by the FDA and been granted ODD in the US and EU. Interim safety and efficacy trial results from the majority of patients after six months of treatment are expected in 2018, with full 12-month treatment data from all patients expected in 2019.
- On November 16, 2017, the Company consummated an underwritten public offering and concurrent registered direct offering of approximately 6 million ordinary shares at a price of \$3.25 per share. ProQR granted the underwriters a 30-day option to purchase up to 745,471 additional ordinary shares

at the public offering price, less underwriting discounts and commissions. Gross proceeds from both offerings were approximately \$20 million, assuming no exercise of the underwriters' option to purchase additional shares in the underwritten public offering. Investors in the offering included several new and existing shareholders, along with significant participation from the Management team and Supervisory Board in the underwritten public offering. Proceeds from these offerings along with existing cash on the balance sheet, are expected to fund operations into the second half of 2019.

#### **Financial Highlights**

At September 30, 2017, ProQR held cash and cash equivalents of €39.7 million, compared to €59.2 million at December 31, 2016. Net cash used in operating activities during the three month period ended September 30, 2017 was €7.6 million, compared to €10.8 million for the same period last year.

Research and development costs totaled €7.2 million for the quarter ended September 30, 2017 compared to €8.3 million for the same period last year and comprised of allocated employee costs including share-based payments, the costs of materials and laboratory consumables, outsourced activities, license and intellectual property costs, and other allocated costs. The decrease in expenses was primarily due to the completion of the nasal potential difference (NPD) study for QR-010.

General and administrative costs increased to €2.8 million for the quarter ended September 30, 2017 compared to €2.0 million for the quarter ended September 30, 2016, which were primarily due to increased investments in our support organization and intellectual property.

Net result for the three month period ended September 30, 2017 was a €10.5 million loss or €0.42 per share, compared to a €10.1 million loss or €0.43 per share for the same period last year. For further financial information for the period ending September 30, 2017, please refer to the financial statements appearing at the end of this release.

### **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 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\*

#### **About QR-010**

QR-010 is a first-in-class RNA-based oligonucleotide designed to address the underlying cause of the disease by targeting the mRNA in CF patients that have the F508del mutation. The technology was exclusively licensed from Massachusetts General Hospital. The F508del mutation results in the production of a misfolded CFTR protein that does not function normally. QR-010 is a single agent designed to bind to the defective CFTR mRNA and to restore CFTR function. QR-010 is designed to be self-administered via an optimized eFlow® Nebulizer (PARI Pharma GmbH). eFlow® is a small, handheld aerosol delivery device which nebulizes QR-010 into a mist inhaled directly into the lungs. QR-010 has been granted orphan drug designation in the United States and the European Union and fast-track status by the FDA. The QR-010 project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No 633545.

#### **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 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 normal (wild-type) CEP290 mRNA leading to the production of normal 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 and received fast-track designation by the FDA.

#### **About QR-313**

QR-313 is a first-in-class RNA-based oligonucleotide designed to address the underlying cause of dystrophic epidermolysis bullosa (DEB) due to mutations in exon 73 of the *COL7A1* gene. Mutations in this exon can cause loss of functional collagen type VII (C7) protein. Absence of C7 results in the loss of anchoring fibrils that normally link the dermal and epidermal layers of the skin together. QR-313 is designed to exclude exon 73 from the mRNA (exon skipping) and produce a functional C7 protein, thereby restoring functionality of the anchoring fibrils.

### **About QR-421**

QR-421 is a first-in-class investigational RNA-based oligonucleotide designed to address the underlying cause of Usher syndrome due to mutations in exon 13 of the USH2A gene. Mutations in this exon can cause loss of functional USH2A protein that causes the disease. QR-421 is designed to exclude exon 13 from the mRNA (exon skipping) and produce truncated but functional USH2A protein, thereby modifying the underlying disease.

### **About QR-411**

QR-411 is a first-in-class investigational RNA-based oligonucleotide designed to address the underlying cause of Usher syndrome due to the c.7595-2144A>G mutation in the USH2A gene. The mutation is a substitution of one nucleotide in the pre-mRNA that leads to aberrant splicing of the mRNA and non-functional or absence of USH2A protein. QR-411 is designed to restore wild-type USH2A mRNA leading to the production of wild-type USH2A protein by binding the mutated pre-mRNA causing normal splicing of the pre-mRNA. QR-411 has been granted orphan drug designation in the United States and the European Union.

### About Next-Generation Axiomer® Technology Platform

ProQR is pioneering a next-generation RNA technology called Axiomer<sup>®</sup>, which could potentially yield a new class of medicines for genetic diseases. Axiomer<sup>®</sup> EONS mediate single nucleotide changes to RNA in a highly specific and targeted way using molecular machinery that is present in human cells. The Axiomer<sup>®</sup> "Editing Oligo Nucleotides", or EONs, recruit an endogenously expressed RNA editing system called ADAR, which it can direct to change an Adenosine (A) to an Inosine (I) in the RNA – an Inosine is translated as a Guanosine (G).

### 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-010, QR-110, QR-411, QR-421, QR-313 and the clinical development and the therapeutic potential thereof, including our ongoing clinical

trial of QR-110, statements regarding our ongoing and planned discovery and development of product candidates and the timing thereof, including those in our innovation pipeline and the potential of our Axiomer® technology, statements regarding release of clinical data, and statements regarding our financial resources and cash runway. 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, 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.: Investor and Media Contact: Bonnie Ortega T: +1 858 245 3983 ir@proqr.com

# PROQR THERAPEUTICS N.V. Unaudited Condensed Consolidated Statement of Financial Position

	September 30, 2017	December 31, 2016
	€ 1,000	€ 1,000
Assets		
Current assets		
Cash and cash equivalents	39,742	59,200
Prepayments and other receivables	2,283	2,420
Social securities and other taxes	777	395
Total current assets	42,802	62,015
Property, plant and equipment	2,740	3,438
Intangible assets	52	90
Total assets	45,594	65,543
Equity and liabilities		
Equity		
Equity attributable to owners of the Company	32,749	53,136
Non-controlling interests	(10)	
Total equity	32,739	53,136
Current liabilities		
Trade payables	250	328
Social securities and other taxes	314	312
Pension premiums	34	13
Deferred income	657	_
Other current liabilities	5,117	6,057
Total current liabilities	6,372	6,710
Borrowings	6,483	5,697
Total liabilities	12,855	12,407
Total equity and liabilities	45,594	65,543

The notes are an integral part of these condensed consolidated financial statements.

## PROQR THERAPEUTICS N.V.

# **Unaudited Condensed Consolidated Statement of Profit or Loss and OCI**

(€ in thousands, except share and per share data)

	Three morended Sep	tember 30,	Nine month period ended September 30,			
	2017	2016	2017	2016		
	<u>€ 1,000</u>	<u>€ 1,000</u>	€ 1,000	€ 1,000		
Other income	326	447	984	1,725		
Research and development costs	(7,226)	(8,319)	(22,808)	(23,823)		
General and administrative costs	(2,753)	(2,001)	(7,949)	(7,218)		
Total operating costs	(9,979)	(10,320)	(30,757)	(31,041)		
Operating result	(9,653)	(9,873)	(29,773)	(29,316)		
Finance income and expense	(868)	(254)	(2,589)	(968)		
Result before corporate income taxes	(10,521)	(10,127)	(32,362)	(30,284)		
Income taxes	_		(2)	_		
Result for the period	(10,521)	(10,127)	(32,364)	(30,284)		
Other comprehensive income	49		114	_		
Total comprehensive income (attributable to owners of the Company)	(10,472)	(10,127)	(32,250)	(30,284)		
Result attributable to						
Owners of the Company	(10,511)	(10,127)	(32,354)	(30,284)		
Non-controlling interests	(10)		(10)	_		
	(10,521)	(10,127)	(32,364)	(30,284)		
Share information						
Weighted average number of shares outstanding <sup>1</sup>	25,282,588	23,346,856	24,255,792	23,346,390		
Earnings per share attributable to owners of the Company (expressed in Euro per share)						
Basic loss per share <sup>1</sup>	(0.42)	(0.43)	(1.33)	(1.30)		
-			_ <del></del>			
Diluted loss per share <sup>1</sup>	(0.42)	(0.43)	(1.33)	(1.30)		

The notes are an integral part of these condensed consolidated financial statements.

1. For this period presented in these financial statements, the potential exercise of share options is not included in the diluted earnings per share calculation as the Company was loss-making in all periods. Due to the anti-dilutive nature of the outstanding options, basic and diluted earnings per share are equal in this period.

PROQR THERAPEUTICS N.V. Unaudited Condensed Consolidated Statement of Changes in Equity

Attributable to owners of the Company									
	Number of shares	Share Capital	Share Premium	Equity Settled Employee Benefit Reserve	Translation Reserve	Accumulated Deficit	Total	Non-controlling interests	Total Equity
		€1,000	€ 1,000	€ 1,000	€ 1,000	€ 1,000	€ 1,000	€ 1,000	€ 1,000
Balance at January 1, 2016	23,345,965	934	123,595	1,899	1	(36,630)	89,799		89,799
Result for the period						(30,284)	(30,284)		(30,284)
Other comprehensive income			_	_	0		0		0
Recognition of share-based payments			_	1,917	_	_	1,917	_	1,917
Share options exercised	891	0	2		_	_	2	_	2
Balance at September 30, 2016	23,346,856	934	123,597	3,816	1	(66,914)	61,434		61,434
Balance at January 1, 2017	23,346,856	934	123,597	4,353	(15)	(75,733)	53,136	_	53,136
Result for the period			_			(32,354)	(32,354)	(10)	(32,364)
Other comprehensive income	_	_	_		114	_	114	_	114
Recognition of share-based payments				3,090			3,090		3,090
Shares issued in the period	2,115,612	85	8,677				8,762	0	8,762
Share options exercised	381	0	1				1		1
Balance at September 30, 2017	25,462,849	1,019	132,275	7,443	99	(108,087)	32,749	(10)	32,739

The notes are an integral part of these condensed consolidated financial statements.

# PROQR THERAPEUTICS N.V.

# **Unaudited Condensed Consolidated Statement of Cash Flows**

		Three month period ended September 30,		Nine month period ended September 30,	
	2017	2016	2017	2016	
	<u>€ 1,000</u>	<i>€</i> 1,000	€ 1,000	<i>€</i> 1,000	
Cash flows from operating activities					
Net result	(10,472)	(10,127)	(32,250)	(30,284)	
Adjustments for:					
— Depreciation	267	284	807	978	
— Share-based compensation	890	628	3,090	1,917	
— Financial income and expenses	868	254	2,589	968	
Changes in working capital	829	(1,843)	(539)	(551)	
Cash used in operations	(7,618)	(10,804)	(26,303)	(26,972)	
Corporate income tax paid	<u> </u>		(2)		
Interest received/(paid)	10	11	69	77	
Net cash used in operating activities	(7,608)	(10,793)	(26,236)	(26,895)	
Cash flow from investing activities					
Purchases of intangible assets					
Purchases of property, plant and equipment	(18)	(422)	(111)	(2,495)	
Net cash used in investing activities	(18)	(422)	(111)	(2,495)	
Cash flow from financing activities					
Proceeds from issuance of shares, net of transaction costs	5,539		8,762		
Proceeds from exercise of share options		_	1	2	
Proceeds from borrowings	100		201	193	
Proceeds from convertible loans	150		150	_	
Redemption of financial lease				(15)	
Net cash generated by financing activities	5,789		9,114	180	
Net increase/(decrease) in cash and cash equivalents	(1,837)	(11,215)	(17,233)	(29,210)	
Currency effect cash and cash equivalents	(742)	(175)	(2,225)	(734)	
Cash and cash equivalents, at beginning of the period	42,321	76,311	59,200	94,865	
Cash and cash equivalents at the end of the period	39,742	64,921	39,742	64,921	

The notes are an integral part of these condensed consolidated financial statements.