# 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 6, 2021

Commission File Number: 001-36622

# **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-month period ended March 31, 2021 and furnished as Exhibit 99.2 to this Report on Form 6-K is a press release of ProQR Therapeutics N.V. dated May 6, 2021, announcing the Company's results for the three-month period ended March 31, 2021.

## 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: May 6, 2021

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-month period ended March 31, 2021.							
99.2	Press Release of ProQR Therapeutics N.V. dated May 6, 2021, announcing the Company's results for the three-month period ended March 31, 2021.							

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

	PAGE
Unaudited Condensed Consolidated Statement of Financial Position at March 31, 2021 and March 31, 2020	1
Unaudited Condensed Consolidated Statement of Profit or Loss and Other Comprehensive Income for the Three- Month Periods ended March 31, 2021 and 2020	2
Unaudited Condensed Consolidated Statement of Changes in Equity for the Three-Month Periods Ended March 31, 2021 and 2020	3
Unaudited Condensed Consolidated Statement of Cash Flows for the Three-Month Periods ended March 31, 2021 and 2020	4
Notes to Unaudited Condensed Consolidated Financial Statements	5

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

	March 31,	December 31,
	2021	2020
	€ 1,000	€ 1,000
Assets		
Current assets		
Cash and cash equivalents	67,878	75,838
Prepayments and other receivables	4,534	3,762
Social securities and other taxes	454	421
Total current assets	72,866	80,021
Property, plant and equipment	18,122	18,601
Investments in associates		10,001
Investments in financial assets	621	
	021	
Total assets	91,609	98,729
Equity and liabilities		
Equity		-
Equity attributable to owners of the Company	49,736	57,091
Non-controlling interests	(556)	(545)
Total equity	49,180	56,546
·······	43,100	50,540
Current liabilities		
Borrowings	1,337	1,135
Lease liabilities	1,021	1,260
Derivative financial instruments	1,604	839
Trade payables	275	221
Current income tax liability		
Social securities and other taxes	296	22
Pension premiums		6
Deferred income	570	700
Other current liabilities	5,391	6,118
Total current liabilities	10,494	10,301
	10,404	10,001
Borrowings	16,506	16,189
Lease liabilities	15,429	15,693
Total liabilities	42,429	42,183
Total equity and liabilities		00 =00
בסומי בקוווני מות וומטווותכא	91,609	98,729

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)

		month period
		ed March 31,
	2021	2020
Other income	€ 1,000	€ 1,000
Other income	141	263
Research and development costs	(8,905)	(12,825)
General and administrative costs	(3,339)	(3,918)
Total operating costs	(12,244)	(16,743)
Operating result	(12,103)	(16,480)
Finance income and expense	(293)	536
Results related to associates	(107)	(134)
Gain on recognition of financial asset	621	
Results related to financial liabilities measured at fair value through profit or loss	(729)	
Result before corporate income taxes	(12,611)	(16,078)
Income taxes	(7)	
Result for the period	(12,618)	(16,078)
Other comprehensive income (foreign exchange differences on translation of foreign operation)	396	256
Total comprehensive income	(12,222)	(15,822)
Result attributable to		
Owners of the Company	(12,607)	(16,055)
Non-controlling interests	(11)	(23)
	(12,618)	(16,078)
Total comprehensive income attributable to		
Owners of the Company	(12,211)	(15,799)
Non-controlling interests	(11)	(23)
	(12,222)	(15,822)
Share information		
Weighted average number of shares outstanding <sup>1</sup>	50,811,135	49,906,033
Earnings per share attributable to the equity holders of the Company (expressed in Euro per share)		
Basic loss per share <sup>1</sup>	(0.25)	(0.32)
Diluted loss per share <sup>1</sup>	(0.25)	(0.32)

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 o						
	Number	Share	Share	Equity settled	Option	Translation	Accumulated	Total	Non-	Total
	of shares	Capital	Premium	Employee		Reserve	Deficit		controlling	Equity
				Benefit	convertible				interests	
		6.4.000	6.4.000	Reserve	loan	6.1.000	6.1.000	6.1.000	6.4.000	6 4 000
		€ 1,000	€ 1,000	€ 1,000	€ 1,000	€ 1,000	€ 1,000	€ 1,000	€ 1,000	€ 1,000
Balance at January 1, 2020	53,975,838	2,159	287,214	16,551		151	(211,746)	94,329	(496)	93,833
Result for the period							(16,055)	(16,055)	(23)	(16,078)
Other comprehensive income						256		256		256
Recognition of share-based payments				2,870				2,870		2,870
Issuance of ordinary shares										
Treasury shares transferred	(220,958)									
Share options lapsed				(2)			2			
Share options exercised	220,958		469	(304)			304	469		469
Balance at March 31, 2020	53,975,838	2,159	287,683	19,115		407	(227,495)	81,869	(519)	81,350
Balance at January 1, 2021	54,131,553	2,165	288,757	23,825	280	(189)	(257,747)	57,091	(545)	56,546
Result for the period							(12,607)	(12,607)	(11)	(12,618)
Other comprehensive income						396		396		396
Recognition of share-based payments	112,657	5	382	1,248				1,635		1,635
Issuance of ordinary shares	585,398	23	2,629	_				2,652		2,652
Treasury shares transferred	(180,126)									
Recognition of equity component of				-						
convertible loan	_		_	_	_	_	_	_	_	_
Share options lapsed				(89)			89			
Share options exercised	180,126		569	(388)			388	569		569
Balance at March 31, 2021	54,829,608	2,193	292,337	24,596	280	207	(269,877)	49,736	(556)	49,180

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

Unaudited Condensed Consolidated Financial Statements

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

	Three month per ended March		
	2021	2020	
	€ 1,000	€ 1,000	
Cash flows from operating activities	01,000	01,000	
Net result	(12,618)	(16,078)	
Adjustments for:	(12,010)	(10,070)	
- Depreciation	631	522	
- Share-based compensation	1,248	2,870	
— Financial income and expenses	293	(536)	
Results related to associates	107	134	
— Gain on recognition of financial asset	(621)	- 104	
— Results related to financial liabilities measured at fair value through profit or loss	729		
Net foreign exchange gain / (loss)	396	256	
- Income tax expenses		250	
- income tax expenses	/		
Changes in working capital	(1,348)	(2,200)	
Cash used in operations	(11,176)	(15,032)	
		( - / /	
Corporate income tax paid	(7)		
Interest received		29	
Interest paid	(578)	(4)	
Net cash used in operating activities	(11,761)	(15,007)	
Cash flow from investing activities			
Purchases of property, plant and equipment	(32)	(198)	
Net cash used in investing activities	(32)	(198)	
Cash flow from financing activities			
Proceeds from issuance of shares, net of transaction costs	2,652	_	
Proceeds from exercise of share options	569	469	
Proceeds from borrowings		290	
Proceeds from convertible loans		_	
Repayment of lease liability	(236)	(202)	
Net cash generated by financing activities	2,985	557	
Net decrease in cash and cash equivalents	(8,808)	(14,648)	
Currency effect cash and cash equivalents	848	761	
Cash and cash equivalents, at beginning of the period	75,838	111,950	
Cash and cash equivalents at the end of the period	67,878	98,063	

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

## PROQR 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 domiciled in the Netherlands 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 was 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%);
- Amylon Therapeutics B.V. (80%);
- Amylon Therapeutics Inc. (80%);

ProQR Therapeutics N.V. is also statutory director of Stichting Bewaarneming Aandelen ProQR ("ESOP Foundation") and has full control over this entity.

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 and the ESOP Foundation.

#### 2. Significant Accounting Policies

These condensed consolidated financial statements have been prepared in accordance with the recognition and measurement criteria of IFRS. Certain disclosures required by IAS 34 *Interim Financial Statements* 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, 2020. In the opinion of management, all events and transactions that are significant to an understanding of the changes in financial position and performance of the Company since the end of the last annual reporting period are disclosed in these 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, 2020.

New Standards and Interpretations, which became effective as of January 1, 2021, 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.

#### 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.

#### Convertible debt

The terms of our convertible debt agreements are evaluated to determine whether the convertible debt instruments contain both liability and equity components, in which case the instrument is a compound financial instrument. Convertible debt agreements are also evaluated to determine whether they contain embedded derivatives, in which case the instrument is a hybrid financial instrument. Judgement is required to determine the classification of such financial instruments based on the terms and conditions of the convertible debt agreements, the currencies in which the debt instruments are denominated and the Company's functional currency.

Estimation methods are used to determine the fair values of the liability and equity components of compound financial instruments and to determine the fair value of embedded derivatives included in hybrid financial instruments. The determination of the effective interest used for the host contracts of hybrid financial instruments and the liability components of compound financial instruments is dependent on the outcome of such estimations. Evaluating the reasonableness of these estimations and the assumptions and inputs used in the valuation methods requires a significant amount of judgement and is therefore subject to an inherent risk of error.

#### 5. Cash and Cash Equivalents

At March 31, 2021, the Company's cash and equivalents were € 67,878,000 as compared to € 75,838,000 at December 31, 2020. The cash balances are held at banks with investment grade credit ratings. The cash at banks is at full disposal of the Company.

#### 6. Property, plant and equipment

At March 31, 2021 and December 31, 2020, property plant and equipment consisted of buildings and leasehold improvements, laboratory equipment and other assets. Buildings and leasehold improvements include a right-of-use asset relating to the lease of our Leiden office and laboratory space, with a carrying amount of € 16,375,000 at March 31, 2021 (December 31, 2020: € 16,775,000).

#### 7. Current liabilities

At March 31, 2021 and December 31, 2020, the other current liabilities consisted principally of accruals for services provided by vendors not yet billed, payroll related accruals and other miscellaneous liabilities.

#### 8. Borrowings

	March 31,	December 31,
	2021	2020
	€ 1,000	€ 1,000
Innovation credit	2,771	2,770
Accrued interest on innovation credit	375	307
Convertible notes	14,234	13,812
Accrued interest on convertible notes	463	435
Total borrowings	17,843	17,324
Current portion	(1,337)	(1,135)
	16,506	16,189

On December 10, 2018 ProQR was awarded an Innovation credit for the sepofarsen program for LCA 10. Amounts will be drawn under this facility from 2018 through 2022. The total credit of  $\notin$  4.7 million will be used to conduct the Phase 2/3 clinical study for sepofarsen and to finance efforts to obtain regulatory and ethical market approval (NDA/MAA). The credit, including accrued interest of 10% per annum, is repayable depending on ProQR obtaining market approval for sepofarsen. An amount of  $\notin$  2.8 million had been received as at March 31, 2021. Accumulated interest amounted to  $\notin$  0.4 million as at March 31, 2021. The assets that are co-financed with the granted innovation credit are subject to a right of pledge for the benefit of RVO.

#### Convertible loans

On July 14, 2020, the Company entered into a convertible debt financing agreement with Pontifax Medison Debt Financing. Under the agreement, the Company will have access to up to \$ 30 million in convertible debt financing in three tranches of \$ 10 million each that will mature over a 54-month period and have an interest-only period of 24 months. One tranche of \$ 10 million had been drawn down as at March 31, 2021.

A second close of the convertible debt financing agreement was completed on August 6, 2020 with Kreos Capital. Under the second agreement, the Company will have access to up to € 15 million in convertible debt financing in three tranches

#### Unaudited Condensed Consolidated Financial Statements

of € 5 million each that will mature over a 54-month period and have an interest-only period of 24 months. One tranche of € 5 million had been drawn down as at March 31, 2021.

Pontifax and/or Kreos may elect to convert the outstanding loans into ProQR ordinary shares at any time prior to repayment at a fixed conversion price. ProQR also has the ability to convert the loans into its ordinary shares, at the same conversion price, if the Company's stock price reaches a pre-determined threshold. In connection with the loan agreement, the Company issued to Pontifax and Kreos warrants to purchase up to an aggregate of 302,676 shares of its common stock at a fixed exercise price.

Pontifax' conversion option and warrants are accounted for as embedded derivatives and are recognized separately from the host contract as financial liabilities at fair value through profit or loss. The host contract is recognized at amortized cost.

The Kreos loan is accounted for as a compound financial instrument. The liability component is recognized at amortized cost. The equity component is initially recognized at fair value as option premium on convertible loan and will not be subsequently remeasured. Kreos' warrants are accounted for as embedded derivatives and are recognized as financial liabilities at fair value through profit or loss.

Convertible loans were issued to Amylon Therapeutics B.V. and are interest-bearing at an average rate of 8% per annum. They are convertible into a variable number of ordinary shares within 36 months at the option of the holder or the Company in case financing criteria are met. Any unconverted loans become payable on demand after 24 – 36 months in equal quarterly terms.

#### 9. Lease liabilities

At March 31, 2021 and December 31, 2020, lease liabilities primarily consisted of the Company's lease of office and laboratory facilities at Zernikedreef in Leiden, the Netherlands.

The lease agreement for our Leiden headquarters, where our main offices and laboratories are located, was put in place on July 1, 2020 and the current lease term is 11 years. The lease agreement may be further extended for subsequent 5-year terms. The carrying amount of the right-of-use asset is disclosed in note 6 Property, plant & equipment.

#### 10. Shareholders' equity

The authorized share capital of the Company amounting to  $\notin$  7,200,000 consists of 90,000,000 ordinary shares and 90,000,000 preference shares with a par value of  $\notin$  0.04 per share. At March 31, 2021, 54,829,608 ordinary shares were issued and fully paid in cash, of which 3,746,617 were held by the Company as treasury shares (December 31, 2020: 3,926,743).

On November 7, 2018, the Company filed a shelf registration statement, which permitted the offering, issuance and sale by the Company of up to a maximum aggregate offering price of \$ 300,000,000 of its ordinary shares, warrants and/or units.

On March 31, 2020, the Company entered into a sales agreement, which permitted the offering, issuance and sale by the Company of up to a maximum aggregate offering price of \$ 75,000,000 of its ordinary shares that may be issued and sold in one or more at-the-market offerings with Citigroup Global Markets, Inc. and Cantor Fitzgerald & Co. In 2020, no shares were issued pursuant to this ATM facility.

## PAGE 9 Unaudited Condensed Consolidated Financial Statements

In January 2021, the Company issued 585,398 ordinary shares under our sales agreement for at-the-market offerings with Citigroup Global Markets Inc. and Cantor Fitzgerald & Co. The gross proceeds from this sale amounted to € 2,767,000, with transaction costs amounting to € 114,000, resulting in net proceeds of € 2,653,000.

#### 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. Options may be granted to employees, members of the Supervisory Board, members of the Management Board and consultants. The compensation expenses included in operating costs for this plan in the three-month period ended March 31, 2021 were  $\in$  1,248,000 (three-month period ended March 31, 2020:  $\notin$  2,870,000), of which  $\notin$  804,000 (three-month period ended March 31, 2020:  $\notin$  1,121,000) was recorded in general and administrative costs and  $\notin$  444,000 (three-month period ended March 31, 2020:  $\notin$  1,749,000) was recorded in research and development costs.

#### 11. Other income

		rree month period ended March 31,
	2021	2020
	€ 1,000	€ 1,000
Grant income	141	229
Other income		34
	141	263

On February 9, 2018, the Company entered into a partnership agreement with Foundation Fighting Blindness (FFB), under which FFB has agreed to provide funding of \$7.5 million for the pre-clinical and clinical development of QR-421a for Usher syndrome type 2A targeting mutations in exon 13.

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

#### 12. Research and development costs

Research and development costs amount to € 8,905,000 for the three-month period ended March 31, 2021 (three-month period ended March 31, 2020: € 12,825,000) and are 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.

#### 13. General and administrative costs

General and administrative costs amount to € 3,339,000 for the three-month period ended March 31, 2021 (three-month period ended March 31, 2020: € 3,918,000).

#### 14. Results related to associates

In January 2021, our associate company Wings Therapeutics Inc. merged into Phoenicis Therapeutics Inc. Consequently, Wings Therapeutics Inc. ceased to exist and the related investment was derecognized. ProQR does not have significant

influence in Phoenicis Therapeutics Inc. Our interest in Phoenicis is recognized as a financial asset, as disclosed in note 15.

The results related to associates for the three-month period ended March 31, 2021 amount to a loss on derecognition of Wings Therapeutics Inc. of  $\notin$  107,000. The results related to associates for the three-month period ended March 31, 2020 amount to a loss of  $\notin$  134,000 and consist of our share of the net losses of Wings Therapeutics Inc.

#### 15. Gain on recognition of financial asset

In January 2021, Wings Therapeutics Inc. merged into Phoenicis Therapeutics Inc. by means of a non-cash transaction. ProQR holds a 3.9% interest in Phoenicis Therapeutics Inc.

The gain on recognition of financial asset for the three-month period ended March 31, 2021 of € 621,000 relates to the gain realized on our investment in the equity instruments of Phoenicis Therapeutics Inc. The Company elected to recognize subsequent changes in the fair value of our investment in Phoenicis in Other Comprehensive Income.

#### 16. Income taxes

The current income tax liability amounts to  $\in$  nil at March 31, 2021 (December 31, 2020:  $\in$  nil). 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.

#### 17. Events after balance sheet date

In April 2021, the Company consummated an underwritten public offering of 15,923,077 ordinary shares at an issue price of \$ 6.50 per share. The gross proceeds from this offering amounted to \$ 103,500,000 while the transaction costs amounted to \$ 6,410,000, resulting in net proceeds of \$ 97,090,000.

In May 2021, the Company entered into an exclusive worldwide license and discovery collaboration for an undisclosed target with Yarrow Biotechnology, an RTW Investments, LP incubated company. Under the terms of the agreement, ProQR is eligible to receive up to \$115 million of upfront and milestone payments, plus single digit percentage royalties on the net sales of any resulting products during the royalty term. ProQR will also have the right to receive an undisclosed percentage of equity in the form of shares of common stock of Yarrow. ProQR will be responsible for certain preclinical activities with reimbursement for the research costs by Yarrow, while Yarrow will be responsible for continuing development of the program and commercialization activities.

# ProQR Announces First Quarter 2021 Operating and Financial Results

- Phase 2/3 pivotal Illuminate trial of sepofarsen for LCA10 enrollment complete with top-line data anticipated in H1 2022
- Positive data reported from the Phase 1/2 clinical trial of QR-421a for the treatment of Usher syndrome and non-syndromic retinitis pigmentosa, with plans to advance the program into two pivotal Phase 2/3 trials before year end
- Exclusive worldwide license and discovery collaboration for non-ophthalmic target with Yarrow Biotechnology, an RTW Investments, LP incubated company
- Strengthened balance sheet with \$103.5 million gross proceeds from public offering

LEIDEN, Netherlands & CAMBRIDGE, Mass., May 6, 2021 -- ProQR Therapeutics N.V. (Nasdaq: PRQR) (the "Company"), a company dedicated to changing lives through the creation of transformative RNA therapies for genetic eye diseases, today reported its financial and operating results for the first quarter ended March 31, 2021 and provided a business update.

"During the first quarter we made significant progress, including completing enrollment in the pivotal trial of sepofarsen, reporting positive clinical data for QR-421a, and strengthening our financial position with a public offering," said Daniel A. de Boer, Founder and CEO of ProQR. "Based on the findings from the Phase 1/2 Stellar trial of QR-421a, we are preparing to initiate two pivotal Phase 2/3 trials for this program before year end. We are on track to share the first clinical data for QR-1123 for adRP in the second half of the year, as well as an update from the InSight Phase 1/2 extension study of sepofarsen for LCA10. Our fourth ophthalmology pipeline program, QR-504a for Fuchs endothelial corneal dystrophy, is expected to advance to clinical testing in the second quarter."

De Boer continued, "The collaboration with RTW and Yarrow Biotechnology is an important endorsement of the broad applicability of our platform, and we are pleased to partner with them, aiming to bring a therapy to patients in an area of unmet need for a non-ophthalmic disease. We strengthened our balance sheet this quarter, allowing us to further the clinical development of our four pipeline programs for genetic eye diseases, to progress our earlier-stage pipeline candidates, and to advance our Axiomer® and Trident<sup>TM</sup> RNA-editing technology platforms."

## **Business Operations and Program Updates**

Sepofarsen for Leber congenital amaurosis 10 (LCA10):

- In April, the first patient was dosed in the Phase 2/3 Brighten trial of sepofarsen in LCA10. The primary objective of the Brighten study is to evaluate safety and tolerability of sepofarsen in patients under 8 years of age.
- In April, Nature Medicine published a case study highlighting a patient who is homozygous for the c.2991+1655A>G mutation in CEP290 and was part of a larger cohort in the Phase 1/2 clinical trial of sepofarsen. Improvements were noted in concordant measures of visual function and retinal structure, including visual acuity, light sensitivity, and visual fields.
- In April, an oral **presentation** was given at the annual meeting of the American Association for Pediatric Ophthalmology and Strabismus (AAPOS) reviewing the Phase 1/2 data for sepofarsen. An oral **presentation** will be given at the Association for Research in Vision and Ophthalmology (ARVO) on May 6, which is an encore presentation of the InSight second treated eye data.
- Upcoming anticipated sepofarsen events:
  - Report top-line results from the pivotal Phase 2/3 Illuminate trial in H1 2022. The Illuminate trial completed enrollment in January following randomization of 36 patients aged 8 years or older to receive either sepofarsen at the target registration dose, a low dose, or sham treatment. The primary endpoint for Illuminate is mean change from baseline in best-corrected visual acuity (BCVA) at Month 12.
  - Report updated data from the next interim analysis of the Phase 1/2 InSight extension study in H2 2021.

**QR-421a** for Usher syndrome and non-syndromic retinitis pigmentosa (nsRP):

- In March, the Company reported results from a planned analysis of the Phase 1/2 Stellar trial demonstrating concordant benefit on multiple measures of vision, including visual acuity, visual fields, and the objective measure of optical coherence tomography (OCT) retinal imaging, after a single dose of QR-421a. QR-421a was observed to be well tolerated with no serious adverse events reported.
- Based on the findings from Stellar, the Company plans to advance QR-421a into two pivotal Phase 2/3 trials by year end, pending finalization of the study designs with Regulatory authorities. Each trial could potentially serve as the sole registration trial.
  - The "Sirius" trial is a Phase 2/3 study that will focus on advanced patients with baseline BCVA ≤20/40. The primary endpoint in this trial will be BCVA at 18 months, with potential for an earlier interim analysis.

- The "Celeste" trial is a Phase 2/3 study that will focus on early-moderate patients. The primary endpoint in this trial will be based on static perimetry at 18 months, with potential for an earlier interim analysis.
- In April, preclinical data for QR-421a were published in *Molecular Therapy*.

**QR-1123** for autosomal dominant retinitis pigmentosa (adRP):

• ProQR anticipates reporting initial data from the single dose cohorts (n=8) of the Phase 1/2 Aurora trial in 2021.

QR-504a for Fuchs Endothelial Corneal Dystrophy (FECD):

- The Fuchs Focus study is expected to start enrolling patients in Q2 2021. This study will evaluate safety, tolerability, and molecular biomarker(s), i.e., target engagement, in the corneal endothelium following a single intravitreal injection of QR-504a in approximately 10 patients with FECD who are scheduled for corneal transplant with concurrent lens replacement.
- ProQR anticipates reporting initial data from this trial in 2022.

Business updates:

- The Company closed an underwritten public offering of 13,846,154 ordinary shares on April 5, 2021 at a price of \$6.50 per share. The closing included the full exercise of underwriters' option to purchase 2,076,923 additional shares. Gross proceeds totaled approximately \$103.5 million.
- In May, Yarrow Biotechnology, Inc., an RTW Investments, LP incubated company, licensed exclusive rights to ProQR's antisense oligonucleotide technology to develop and commercialize potential therapies for an undisclosed non-ophthalmic target. Under the terms of the agreement, ProQR is eligible to receive up to \$115 million of upfront and milestone payments, plus single digit percentage royalties on the net sales of any resulting products during the royalty term. ProQR will also have the right to receive an undisclosed percentage of equity in the form of shares of common stock of Yarrow.

## **Financial Highlights**

At March 31, 2021, ProQR held cash and cash equivalents of €67.9 million, compared to €75.8 million at December 31, 2020. Net cash used in operating activities during the three-month period ended March 31, 2021 was €11.8 million, compared to €15.0 million for the same period last year.

Research and development (R&D) costs were &8.9 million for the quarter ended March 31, 2021 compared to &12.8 million for the same period last year.

General and administrative costs were €3.3 million for the quarter ended March 31, 2021 compared to €3.9 million for the same period last year.

Net loss for the three-month period ended March 31, 2021 was &12.6 million, or &0.25 per diluted share, compared to &16.1 million, or &0.32 per diluted share, for the same period last year. For further financial information for the period ending March 31, 2021, please refer to the financial statements appearing at the end of this release.

## About Leber Congenital Amaurosis 10 (LCA10)

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

## **About Sepofarsen**

Sepofarsen (QR-110) is being evaluated in the pivotal Phase 2/3 Illuminate trial and is a first-in-class investigational RNA therapy designed to address the underlying cause of Leber 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 leads to aberrant splicing of the mRNA and non-functional CEP290 protein. Sepofarsen is designed to 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 and rare pediatric disease designation from the FDA as well as access to the PRIME scheme by the EMA.

## About Usher Syndrome Type 2 and Non-Syndromic Retinitis Pigmentosa

Usher syndrome is the leading cause of combined deafness and blindness. People with Usher syndrome type 2 are usually born with hearing loss and start to have progressive vision loss during adulthood. The vision loss can also occur without hearing loss in a disease called non-syndromic retinitis pigmentosa. Usher syndrome type 2 and non-syndromic retinitis pigmentosa can be caused by mutations in the *USH2A* gene. To date, there are no pharmaceutical

treatments approved or in clinical development that treat the vision loss associated with mutations in *USH2A*.

## About QR-421a

QR-421a is a first-in-class investigational RNA therapy designed to address the underlying cause of vision loss in Usher syndrome type 2a and non-syndromic retinitis pigmentosa due to mutations in exon 13 of the USH2A gene. QR-421a is designed to restore functional usherin protein by using an exon skipping approach with the aim to stop or reverse vision loss in patients. QR-421a is intended to be administered through intravitreal injections in the eye and has been granted orphan drug designation in the US and the European Union and received fast-track and rare pediatric disease designations from the FDA.

## About Autosomal Dominant Retinitis Pigmentosa (adRP)

Autosomal dominant retinitis pigmentosa, or adRP, is a severe and rare genetic disease that causes progressive problems in night vision during childhood, leading to visual field loss and frequently resulting in blindness in mid adulthood. In the United States, the most prevalent mutation associated with adRP is the P23H point mutation (also known as the c.68C>A mutation) in the *rhodopsin (RHO)* gene and affects approximately 2,500 people. This mutation causes misfolding of the rhodopsin protein that becomes toxic to the photoreceptor cells and at the same time diminishes the function of the wild type allele. Over time this results in cell death and progressive vision loss. There are currently no therapies approved or in clinical development for P23H adRP. A natural history study in patients with P23H adRP has been conducted.

## About QR-1123

QR-1123 is being evaluated in the Phase 1/2 Aurora trial and is a first-in-class investigational RNA therapy designed to treat adRP due to the P23H mutation in the RHO gene. QR-1123 was discovered and developed by Ionis Pharmaceuticals using Ionis' proprietary antisense technology. The therapy aims to inhibit the formation of the mutated toxic version of the rhodopsin protein by specifically binding the mutated RHO mRNA. Binding of QR-1123 causes allele specific knockdown of the mutant mRNA by a mechanism called RNase H mediated cleavage without affecting the normal RHO mRNA. QR-1123 is intended to be administered through intravitreal injections in the eye. QR-1123 was in-licensed from Ionis Pharmaceuticals in 2018. QR-1123 has been granted Orphan Drug designation in the United States and received Fast Track designation from the FDA.

# About Fuchs Endothelial Corneal Dystrophy (FECD)

Fuchs endothelial corneal dystrophy (FECD) type 3 is a common genetic disease that leads to progressive degeneration of the corneal endothelium resulting in corneal edema, scarring and vision loss. Blisters on the cornea are a major cause of pain in patients with advanced FECD. Currently there are no treatment options available to stop or slow down FECD and disease management is aimed to reduce symptoms. The only effective therapy for late-stage FECD is corneal transplantation. The availability of donors, risk of rejection, and the inherent risk of such surgeries are some of the limitations of this option. FECD is a common disorder affecting more than 4% of people over the age of 40 in the United States, with similar numbers reported for other parts of the World. Trinucleotide repeat (TNR) expansion mutations in the TCF4 gene are a common cause of FECD. In people of European descent, around 75% of FECD patients have TNR expansions in TCF4.

## About QR-504a

QR-504a is a first-in-class investigational RNA therapy designed to address the underlying cause of Fuchs endothelial corneal dystrophy (FECD) due to trinucleotide repeat (TNR) expansion mutations in the TCF4 gene. The TNR expansions cause the TCF4 RNA to aggregate in the corneal endothelial cells forming the characteristic nuclear RNA foci and eventually resulting in FECD. QR-504a is designed to target the TNRs in the TCF4 RNA. The aim is to reduce aggregation and the formation of RNA foci to prevent or stop corneal degeneration in patients with FECD. QR-504a is intended to be administered through intravitreal injections in the eye.

## **About Axiomer and Trident**

ProQR is pioneering a next-generation RNA technology called Axiomer®, which could potentially yield a new class of medicines for genetic diseases. Axiomer "Editing Oligonucleotides", or 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® EONs are designed to recruit an endogenously expressed RNA editing system called ADAR, which can direct the change of an Adenosine (A) to an Inosine (I) in the RNA – an Inosine is translated as a Guanosine (G).

Our TRIDENT<sup>™</sup> RNA pseudouridylation platform enables the suppression of nonsense mutations and premature stop codons (PTC) that cause human genetic diseases. Since all premature stop codons contain uridine, pseudouridylation of that uridine converts those nonsense codons into sense codons. TRIDENT technology harnesses endogenously expressed pseudouridylation machinery to guide RNAs to inhibit nonsense mRNA-mediated decay (NMD) in a sequence-specific manner and promote PTC readthrough. The TRIDENT technology has the potential to be applied in approximately 11% of all genetic mutations.

## **About ProQR**

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

## Learn more about ProQR at <u>www.proqr.com</u>.

## 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 forward-looking statements include, but are not limited to, statements regarding sepofarsen (QR-110) and the clinical development and the therapeutic potential thereof, statements regarding QR-421a and the clinical development and the therapeutic potential thereof, statements regarding QR-1123 and the clinical development and therapeutic potential thereof, statements regarding the QR-504a and the clinical development and therapeutic potential thereof, statements regarding our pipeline of programs targeting inherited retinal dystrophies, including timing of commencing clinical trials and enrollment of patients therein, our other programs and business operations (including Axiomer and Trident), the expected impact of the COVID-19 on our business operations, including our research and development plans and timelines and the supply chain for our clinical and development programs, statements regarding the collaboration with RTW and Yarrow and the intended benefits thereof, including milestone and royalty payments from commercial product sales, if any, from the products covered by the collaboration and the issuance of equity in Yarrow to ProQR and our financial position and cash runway. 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 and uncertainties include, among others, the cost, timing and results of preclinical studies and clinical trials and other development activities by us and our collaborative partners whose operations and activities may be slowed or halted by the COVID-19 pandemic; the likelihood of our clinical programs being executed on timelines provided and reliance on our contract research organizations and predictability of timely enrollment of subjects and patients to advance our clinical trials and maintain their own operations; our reliance on contract manufacturers to supply materials for research and

development and the risk of supply interruption from a contract manufacturer; the potential for future data to alter initial and preliminary results of early-stage clinical trials; the unpredictability of the duration and results of the regulatory review of applications or clearances that are necessary to initiate and continue to advance and progress our clinical programs; the ability to secure, maintain and realize the intended benefits of collaborations with partners; the possible impairment of, inability to obtain, and costs to obtain intellectual property rights; possible safety or efficacy concerns that could emerge as new data are generated in research and development; our ability to maintain and service our loan facility with Pontifax and Kreos; general business, operational, financial and accounting risks; and risks related to litigation and disputes with third parties. 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.

## **Cautionary Note on Future Updates**

The statements contained in this press release reflect our current views with respect to future events, which may change significantly as the global consequences of the COVID-19 pandemic rapidly develop. Accordingly, we do not undertake and specifically disclaim any obligation to update any forward-looking statements.

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

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# **Financial Tables**

# PROQR THERAPEUTICS N.V.

## Unaudited Condensed Consolidated Statement of Financial Position

	March 31,	December 31,
	2021	2020
	€ 1,000	€ 1,000
Assets		
Current assets		
Cash and cash equivalents	67,878	75,838
Prepayments and other receivables	4,534	3,762
Social securities and other taxes	454	421
Total current assets	72,866	80,021
Property, plant and equipment	18,122	18,601
Investments in associates		107
Investments in financial assets	621	
Total assets	91,609	98,729
Equity and liabilities	·	
Equity		
Equity attributable to owners of the Company	49,736	57,091
Non-controlling interests	(556)	(545
Total equity	49,180	56,546
Current liabilities		
Borrowings	1,337	1,135
Lease liabilities	1,021	1,260
Derivative financial instruments	1,604	839
Trade payables	275	221
Current income tax liability		
Social securities and other taxes	296	22
Pension premiums		6
Deferred income	570	700
Other current liabilities	5,391	6,118
Total current liabilities	10,494	10,301
Borrowings	16,506	16,189
Lease liabilities	15,429	15,693
Total liabilities	42,429	42,183
Total equity and liabilities	91,609	98,729

## PROQR THERAPEUTICS N.V.

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

( $\in$  in thousands, except share and per share data)

		month period ed March 31,
	2021	2020
	€ 1,000	€ 1,000
Other income	141	263
Research and development costs	(8,905)	(12,825)
General and administrative costs	(3,339)	(3,918)
Total operating costs	(12,244)	(16,743)
Operating result	(12,103)	(16,480)
Finance income and expense	(293)	536
Results related to associates	(107)	(134)
Gain on recognition of financial asset	621	_
Results related to financial liabilities measured at fair value through profit or loss	(729)	
Result before corporate income taxes	(12,611)	(16,078)
Income taxes	(7)	
Result for the period	(12,618)	(16,078)
Other comprehensive income (foreign exchange differences on translation of foreign operation)	396	256
Total comprehensive income	(12,222)	(15,822)
Result attributable to		
Owners of the Company	(12,607)	(16,055)
Non-controlling interests	(11)	(23)
	(12,618)	(16,078)
Total comprehensive income attributable to		
Owners of the Company	(12,211)	(15,799)
Non-controlling interests	(11)	(23)
	(12,222)	(15,822)
Share information		
Weighted average number of shares outstanding <sup>1</sup>	50,811,135	49,906,033
Earnings per share attributable to the equity holders of the Company (expressed in Euro per share)		
Basic loss per share <sup>1</sup>	(0.25)	(0.32)
Diluted loss per share <sup>1</sup>	(0.25)	(0.32)

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

			А	ttributable to ow	ners of the Co	mpany				
	Number of shares	Share Capital	Share Premium	Equity settled Employee Benefit Reserve	Option premium on convertible loan	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	€ 1,000
Balance at January 1, 2020	53,975,838	2,159	287,214	16,551		151	(211,746)	94,329	(496)	93,833
Result for the period							(16,055)	(16,055)	(23)	(16,078)
Other comprehensive income						256		256		256
Recognition of share-based payments				2,870				2,870		2,870
Issuance of ordinary shares										
Treasury shares transferred	(220,958)									_
Share options lapsed				(2)			2			
Share options exercised	220,958		469	(304)			304	469		469
Balance at March 31, 2020	53,975,838	2,159	287,683	19,115		407	(227,495)	81,869	(519)	81,350
Balance at January 1, 2021	54,131,553	2,165	288,757	23,825	280	(189)	(257,747)	57,091	(545)	56,546
Result for the period							(12,607)	(12,607)	(11)	(12,618)
Other comprehensive income						396		396		396
Recognition of share-based payments	112,657	5	382	1,248	_	_	_	1,635	_	1,635
Issuance of ordinary shares	585,398	23	2,629			—		2,652		2,652
Treasury shares transferred	(180,126)				_		_			_
Recognition of equity component of convertible loan	_	_	_	_		_	_	_	_	_
Share options lapsed				(89)			89			
Share options exercised	180,126		569	(388)			388	569		569
Balance at March 31, 2021	54,829,608	2,193	292,337	24,596	280	207	(269,877)	49,736	(556)	49,180

# PROQR THERAPEUTICS N.V.

## Unaudited Condensed Consolidated Statement of Cash Flows

		month period ed March 31,
	2021	2020
	€ 1,000	€ 1,000
Cash flows from operating activities		,
Net result	(12,618)	(16,078)
Adjustments for:	<u>_</u>	
— Depreciation	631	522
- Share-based compensation	1,248	2,870
— Financial income and expenses	293	(536)
- Results related to associates	107	134
— Gain on recognition of financial asset	(621)	_
<ul> <li>— Results related to financial liabilities measured at fair value through profit or loss</li> </ul>	729	
— Net foreign exchange gain / (loss)	396	256
— Income tax expenses	7	_
Changes in working capital	(1,348)	(2,200)
Cash used in operations	(11,176)	(15,032)
X	(11,17,0)	(10,002)
Corporate income tax paid	(7)	_
Interest received		29
Interest paid	(578)	(4)
Net cash used in operating activities	(11,761)	(15,007)
Cash flow from investing activities		
Purchases of property, plant and equipment	(32)	(198)
Net cash used in investing activities	(32)	(198)
Cash flow from financing activities		
Proceeds from issuance of shares, net of transaction costs	2,652	_
Proceeds from exercise of share options	569	469
Proceeds from borrowings		290
Proceeds from convertible loans		
Repayment of lease liability	(236)	(202)
Net cash generated by financing activities	2,985	557
Net decrease in cash and cash equivalents	(8,808)	(14,648)
	(0,000)	(,. 10)
Currency effect cash and cash equivalents	848	761
Cash and cash equivalents, at beginning of the period	75,838	111,950