# 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

November 16, 2020

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)

metalling rate could, or regional or rate and connects,
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): $\Box$
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): $\Box$

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 periods ended September 30, 2020 and furnished as Exhibit 99.2 to this Report on Form 6-K is a press release of ProQR Therapeutics N.V. dated November 16, 2020, announcing the Company's results for the three- and nine-month periods ended September 30, 2020.

# 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: November 16, 2020 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 periods ended September 30, 2020.
99.2	Press Release of ProQR Therapeutics N.V. dated November 16, 2020, announcing the Company's results for the three- and nine-month periods ended September 30, 2020.

# Exhibit 99.1

# 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,
	2020	2019
	€ 1,000	€ 1,000
Assets		
Current assets		
Cash and cash equivalents	88,847	111,950
Prepayments and other receivables	2,759	1,866
Social securities and other taxes	552	850
Total current assets	92,158	114,666
Property, plant and equipment	17,875	2,440
Investments in associates	159	429
Total assets	110,192	117,535
Equity and liabilities		
Equity		
Equity attributable to owners of the Company	68,626	94,329
Non-controlling interests	(534)	(496
Total equity	68,092	93,833
Current liabilities		
Borrowings	949	343
Lease liabilities	1,308	508
Derivative financial instruments	1,092	
Trade payables	291	445
Current income tax liability		64
Social securities and other taxes	155	108
Pension premiums		2
Deferred income	863	711
Other current liabilities	6,159	8,812
Total current liabilities	10,817	10,993
Borrowings	16,577	12,709
Lease liabilities	14,706	
Total liabilities	42,100	23,702
Total equity and liabilities	110,192	117,535

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

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

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

		nonth period	Nine month period ended September 30,		
	2020	<u>2019</u>	2020	2019	
	€ 1,000	€ 1,000	€ 1,000	€ 1,000	
Other income	251	530	9,188	1,509	
Research and development costs	(8,304)	(11,074)	(29,716)	(32,560)	
General and administrative costs	(2,809)	(2,903)	(10,173)	(8,970)	
Total operating costs	(11,113)	(13,977)	(39,889)	(41,530)	
Operating result	(10,862)	(13,447)	(30,701)	(40,021)	
Finance income and expense	(1,863)	1,375	(2,024)	1,339	
Results related to financial liabilities measured at fair value through profit or loss	(305)		(305)		
Results related to associates	(84)	(119)	(270)	579	
Result before corporate income taxes	(13,114)	(12,191)	(33,300)	(38,103)	
Income taxes	(75)		(86)	(64)	
Result for the period	(13,189)	(12,191)	(33,386)	(38,167)	
Other comprehensive income	(255)	147	(134)	121	
Total comprehensive income (attributable to owners of the Company)	(13,444)	(12,044)	(33,520)	(38,046)	
Result attributable to					
Owners of the Company	(13,181)	(12,139)	(33,348)	(37,945)	
Non-controlling interests	(8)	(52)	(38)	(222)	
	(13,189)	(12,191)	(33,386)	(38,167)	
Share information		-		-	
Weighted average number of shares outstanding <sup>1</sup>	50,143,262	38,912,701	50,017,990	38,902,203	
Earnings per share attributable to the equity holders of the Company (expressed in Euro per share)					
Basic loss per share <sup>1</sup>	(0.26)	(0.31)	(0.67)	(0.98)	
Diluted loss per share <sup>1</sup>	(0.26)	(0.31)	(0.67)	(0.98)	

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	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, 2019	43,149,987	1,726	235,744	10,780		108	(155,443)	92,915	(230)	92,685
Result for the period							(37,945)	(37,945)	(222)	(38,167)
Other comprehensive income						121		121		121
Recognition of share-based payments	_	_	_	4,614	_	_	_	4,614	_	4,614
Issuance of ordinary shares										
Treasury shares transferred	(40,259)									
Share options lapsed				(33)			33			_
Share options exercised	40,259		166	(115)			115	166		166
Balance at September 30, 2019	43,149,987	1,726	235,910	15,246		229	(193,240)	59,871	(452)	59,419
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							(33,348)	(33,348)	(38)	(33,386)
Other comprehensive income						(134)		(134)		(134)
Recognition of share-based										
payments		2	283	6,218				6,503		6,503
Issuance of ordinary shares	100,902	2	270					272		272
Treasury shares transferred	(299,615)									
Recognition of equity component of convertible loan	_	_	_	_	280	_	_	280	_	280
Share options lapsed				(63)			63			
Share options exercised	299,615		724	(466)			466	724		724
Balance at September 30, 2020	54,076,740	2,163	288,491	22,240	280	17	(244,565)	68,626	(534)	68,092

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

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

	Three month period ended September 30,			nonth period
	2020	2019	2020	2019
	€ 1,000	€ 1,000	€ 1,000	€ 1,000
Cash flows from operating activities				
Net result	(13,189)	(12,191)	(33,386)	(38,167)
Adjustments for:				<u> </u>
— Depreciation	651	506	1,703	1,543
— Share-based compensation	1,676	1,226	6,348	4,614
— Other income			(8,423)	
— Financial income and expenses	1,863	(1,375)	2,024	(1,339)
— Results related to associates	84	119	270	(579)
<ul> <li>Results related to financial liabilities measured at fair value through profit or loss</li> </ul>	305		305	
— Net foreign exchange gain / (loss)	(255)	148	(134)	122
Changes in working capital	(246)	2,718	(3,354)	1,744
Cash used in operations	(9,111)	(8,849)	(34,647)	(32,062)
Corporate income tax paid	(157)	<del></del>	(168)	(64)
Interest received	27	90	118	176
Interest paid	(569)	(13)	(607)	(64)
Net cash used in operating activities	(9,810)	(8,772)	(35,304)	(32,014)
Cash flow from investing activities			<del></del> ,	
Purchases of property, plant and equipment	(264)	(32)	(806)	(341)
Net cash used in investing activities	(264)	(32)	(806)	(341)
Cash flow from financing activities	<del></del> -			
Proceeds from issuance of shares, net of transaction costs				
Proceeds from exercise of share options	12	2	724	166
Proceeds from borrowings			579	
Proceeds from convertible loans	13,477		13,542	690
Repayment of lease liability	(235)	(290)	(542)	(861)
Net cash generated by/(used in) financing activities	13,254	(288)	14,303	(5)
Net increase/(decrease) in cash and cash equivalents	3,180	(9,092)	(21,807)	(32,360)
Currency effect cash and cash equivalents	(1,474)	1,420	(1,296)	1,572
Cash and cash equivalents, at beginning of the period	87,141	82,464	111,950	105,580
Cash and cash equivalents at the end of the period	88,847	74,792	88,847	74,792

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

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#### **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. ProQR Therapeutics N.V. holds a 20% minority shareholding in Wings Therapeutics Inc.

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 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, 2019. 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.

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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, 2019.

New Standards and Interpretations, which became effective as of January 1, 2020, 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.

#### 5. Cash and Cash Equivalents

At September 30, 2020, the Company's cash and equivalents were € 88,847,000 as compared to € 111,950,000 at December 31, 2019. 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 September 30, 2020 and December 31, 2019, 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 € 15,923,000 at September 30, 2020 (December 31, 2019: € 606,000).

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#### 7. Current liabilities

At September 30, 2020 and December 31, 2019, 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

	September 30,	December 31,
	2020	2019
	€ 1,000	€ 1,000
Innovation credit	2,771	7,191
Accrued interest on innovation credit	237	3,124
Convertible notes	14,140	2,473
Accrued interest on convertible notes	378	264
Total borrowings	17,526	13,052
Current portion	(949)	(343)
	16,577	12,709

On June 1, 2012, ProQR was awarded an Innovation credit by the Dutch government, through its agency RVO of the Ministry of Economic Affairs, for the Company's cystic fibrosis program. Amounts were drawn under this facility in the course of the years 2013 through 2017. The credit covered 35% of the costs incurred in respect of the program up to € 5.0 million. The credit was interest-bearing at a rate of 10% per annum. In June 2020 ProQR received a final waiver of the full amount of the Innovation credit, including accumulated interest. Consequently, the carrying amount of € 8.4 million, including accumulated interest, was recognized in Other Income in the second quarter of 2020.

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 2021. The total credit of € 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 € 2.8 million had been received as at September 30, 2020. Accumulated interest amounted to € 0.2 million as at September 30, 2020. 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 tranch of \$ 10 million was drawn down at the end of the current reporting period and is recognized at amortized cost. 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 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 was drawn down at the end of the current reporting.

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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 September 30, 2020 and December 31, 2019, lease liabilities consisted of the Company's lease of office and laboratory facilities at Zernikedreef in Leiden, the Netherlands, where our headquarters and our laboratories are located. A new lease agreement was put in place on July 1, 2020 for a 10-year period, which may be extended for subsequent 5-year terms. The carrying amount of the right-of-use asset is disclosed under note 6 Property, plant & equipment.

#### 10. Shareholders' equity

The authorized share capital of the Company amounting to  $\in$  7,200,000 consists of 90,000,000 ordinary shares and 90,000,000 preference shares with a par value of  $\in$  0.04 per share. At September 30, 2020, 54,076,740 ordinary shares were issued and fully paid in cash, of which 3,930,536 were held by the Company as treasury shares (December 31, 2019: 4,230,151).

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.

In October 2019, the Company consummated an underwritten public offering of 10,454,545 ordinary shares at an issue price of \$ 5.50 per share. The gross proceeds from this offering amounted to  $\le$  51,597,000 while the transaction costs amounted to  $\le$  3,047,000, resulting in net proceeds of  $\le$  48,550,000.

In December 2019, the Company issued 371,306 shares in the aggregate amount of \$3.5 million, at \$9.43 (€8.51) per share to Ionis Pharmaceuticals, Inc. Under the terms of the agreement, the second installment of the upfront payment in

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ordinary shares to the Company's common stock was made to Ionis upon the dosing of the first patient in the phase 1/2 Aurora clinical trial for QR-1123.

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.

#### 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 September 30, 2020 were € 1,675,000 (2019: € 1,226,000), of which € 1,108,000 (2019: € 835,000) was recorded in general and administrative costs and € 567,000 (2019: € 391,000) was recorded in research and development costs.

#### 11. Other income

		Three month period		
	en	ended September 30,		
	2020	2019		
	€ 1,000	€ 1,000		
Grant income	212	518		
Other income	39	12		
	251	530		

In June 2020 ProQR received a final waiver of the full amount of the Innovation credit for the Company's cystic fibrosis program. Consequently, the carrying amount of €8.4 million, including accumulated interest, was recognized in Other Income in Q2 2020.

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.

In addition, funding was received for the Huntington's disease program.

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

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#### 12. Research and development costs

Research and development costs amount to € 8,304,000 for the three month period ended September 30, 2020 (2019: € 11,074,000) 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 € 2,809,000 for the three month period ended September 30, 2020 (2019: € 2,903,000).

#### 14. Results related to associates

The results related to associates for the three month period ended September 30, 2020 amount to € 84,000 (2019: € 119,000) and consist of our share of the net losses of Wings Therapeutics Inc.

#### 15. Income taxes

The current income tax liability amounts to € nil at September 30, 2020 (December 31, 2019: € 64,000). 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.

#### 16. Events after balance sheet date

No significant events have occurred after the balance sheet date.

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# ProQR Announces Third Quarter 2020 Operating and Financial Results

- *Illuminate* Phase 2/3 trial of sepofarsen expected to complete enrollment in Q1 2021; additional data from Phase 1/2 *InSight* extension study to be reported in H2 2021
- Enrollment completed for QR-421a dosing cohorts and data from next Phase 1/2 interim analysis expected in H1 2021
- Initial data from Phase 1/2 trial of QR-1123 expected in 2021
- QR-504a clinical study in Fuchs expected to start in H1 2021
- Cash runway into 2023

LEIDEN, Netherlands & CAMBRIDGE, Mass., November 16, 2020 -- ProQR Therapeutics N.V. (Nasdaq: PRQR) (the "Company"), a company dedicated to changing lives through the creation of transformative RNA therapies for inherited retinal diseases (IRDs), today reported its financial and operating results for the third quarter ended September 30, 2020 and provided a business update, including updated timeline guidance for the Company's clinical stage programs.

"We're pleased to report that our clinical trials are back on track after the COVID-19 disruption, with all ongoing trials either already completed or expected to complete enrollment in the near term. With this progress we are well positioned heading into 2021 and beyond to make meaningful advancements across our portfolio," said Daniel A. de Boer, Chief Executive Officer of ProQR. "We will enter 2021 with the *Illuminate* trial of sepofarsen expected to complete enrollment in the first quarter and we anticipate reporting data across our clinical pipeline during the year ahead, which represents the opportunity for multiple potential value inflection points. This will include the next interim analysis for QR-421a and initial data for QR-1123. We also plan to share updated data from the Phase 1/2 extension study of sepofarsen and to start dosing patients with QR-504a."

De Boer continued, "As we continue to build a robust IRD translational platform, we are pleased to have appointed Naveed Shams, MD, PhD, as our Chief Scientific Officer. Naveed is a proven leader with deep ophthalmology experience, including multiple product approvals globally. This expertise further strengthens our focus as an ophthalmology company and supports our commitment to bringing therapies to patients with inherited retinal diseases."

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# **Business Operations and Program Updates**

Sepofarsen, lead clinical candidate for Leber congenital amaurosis 10 (LCA10) in the Phase 2/3 *Illuminate* trial:

- In July, the Company presented positive data from the ongoing *InSight* extension study of sepofarsen for LCA10, in which patients from the completed Phase 1/2 study were offered treatment in their second eye. These data showed that in 4 out of 4 second eyes treated, the treatment response was consistent with the first eye treated including a significant and sustained benefit, building further confidence in the Phase 2/3 *Illuminate* trial.
  - In October and November, the following data was presented at Euretina and the American Academy
    of Ophthalmology (AAO) virtual annual meetings, and both presentations were selected as "Best
    Poster" at AAO:
  - Phase 1b/2 trial results of the intravitreal sepofarsen RNA therapy in LCA10 (encore presentations)
  - Full-field stimulus testing (FST) to assess sepofarsen patient response in LCA10
- The Company has activated sites in North America, Europe, and South America to enable rapid completion of the *Illuminate* trial enrollment. All of the remaining patients to be enrolled in the trial have been pre-screened and identified.
- Upcoming sepofarsen anticipated events:
  - The Company expects to complete enrollment in the Phase 2/3 pivotal *Illuminate* trial in Q1 2021, assuming sites are able to continue operations with respect to the COVID-19 public health emergency. The primary endpoint for *Illuminate* is mean change from baseline in BCVA at 12 months.
  - Start a pediatric trial of sepofarsen in patients under 8 years of age in 2021. The primary objectives of this study are safety and tolerability,
  - 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):

- Enrollment of the Phase 1/2 Stellar trial dose expansion (100 μg homozygous) and dose escalation (200 μg) cohorts is complete.
- The Company anticipates reporting data from the next planned interim analysis of the Phase 1/2 Stellar trial in H1 2021.

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

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- The Phase 1/2 Aurora trial is ongoing with 4 of the 5 planned single dose cohorts having completed enrollment.
- ProQR anticipates reporting initial data from the single dose cohorts of this program in 2021.

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

- All preparations for the start of a Proof of Mechanism trial of QR-504a in patients with FECD are completed and, pending the lifting of COVID-19 restrictions, the Company plans to start enrolling patients with FECD type 3 in H1 of 2021.
- Report initial data in H1 2022.

#### Business updates:

- In July, to support funding of the Company's pipeline, ProQR entered into a strategic convertible debt financing agreement with Pontifax Ventures where the Company will have access to up to \$30 million in three tranches of \$10 million with the first tranche drawn by the Company at closing. Subsequent to the closing with Pontifax, in August the loan facility was expanded by an additional €15 million (in three tranches of €5 million) with Kreos Capital, with €5 million drawn by the Company at closing. If fully drawn down, the capital from these facilities extends ProQR's runway into 2023.
- In October, Naveed Shams, MD, PhD, was appointed Chief Scientific Officer (CSO). As CSO, Dr.
  Shams provides strategic direction, oversight, and execution for ProQR's research and early
  development efforts. He joined ProQR from Santen, a global company focused on ophthalmology,
  where he most recently served as Senior Corporate Officer, Head of Global Research and Development
  and Chief Scientific Officer.

## **Financial Highlights**

At September 30, 2020, ProQR held cash and cash equivalents of €88.8 million, compared to €112.0 million at December 31, 2019. Net cash used in operating activities during the three-month period ended September 30, 2020 was €9.8 million, compared to €8.8 million for the same period last year.

Research and development costs were €8.3 million for the quarter ended September 30, 2020, compared to €11.1 million for the same period last year.

General and administrative costs were €2.8 million for the quarter ended September 30, 2020 compared to €2.9 million for the same period last year.

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Net loss for the three-month period ended September 30, 2020 was €13.2 million or €0.26 per share, compared to a €12.2 million loss or €0.31 per share for the same period last year.

For further financial information for the period ended September 30, 2020, 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*.

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### **About QR-421a**

QR-421a is being evaluated in the Phase 1/2 *Stellar* trial and 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 (RP) 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.

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### About Fuchs Endothelial Corneal Dystrophy (FECD)

Fuchs endothelial corneal dystrophy (FECD) is a common inherited condition characterized by the dysfunction and degeneration of the corneal endothelium, a single cell layer of cells on the inside of the cornea. FECD is a common disorder; it is estimated that FECD affects more than 4% of individuals over the age of 40 in the U.S., and similar prevalence is noted for other global regions. There are different types of this disease and we focus on age-related FECD Type 3 (FECD3). Some patients with age-related FECD develop advanced disease with corneal edema and corneal clouding. These symptoms can lead to complete vision loss and the need for surgery such as a corneal transplant.

# About QR-504a

We are developing QR-504a as an RNA therapy for the treatment of FECD3. We plan to advance the QR-504a program into a first-in-human clinical trial in late-stage disease patients in 2021. QR-504a is designed to target the intronic TNRs in the *TCF4* RNA. The aim is to reduce aggregation and the formation of RNA foci in order to normalize the RNA splicing patterns, and prevent or halt corneal degeneration in patients with FECD3.

### **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.

\*Since 2012\*

#### FORWARD-LOOKING STATEMENTS

This press release contains forward-looking statements. All statements other than statements of historical fact are forward-looking statements, which are often indicated by terms such as "anticipate," "believe," "could," "estimate," "expect," "goal," "intend," "look forward to", "may," "plan," "potential," "predict," "project," "should," "will," "would" and similar expressions. Such 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 our pipeline of programs targeting inherited retinal dystrophies, 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, our other

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programs and business operations, including timing of commencing clinical trials and enrollment of patients therein, 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, our loan facility with Pontifax 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 general business, financial and accounting risks and litigation. 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.

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# **Unaudited Condensed Consolidated Statement of Financial Position**

	September 30, 2020	December 31, 2019
	€ 1,000	€ 1,000
Assets		
Current assets		
Cash and cash equivalents	88,847	111,950
Prepayments and other receivables	2,759	1,866
Social securities and other taxes	552	850
Total current assets	92,158	114,666
Property, plant and equipment	17,875	2,440
Investments in associates	159	429
Total assets	110,192	117,535
Equity and liabilities	<del></del>	
Equity		
Equity attributable to owners of the Company	68,626	94,329
Non-controlling interests	(534)	(496
Total equity	68,092	93,833
Current liabilities		
Borrowings	949	343
Lease liabilities	1,308	508
Derivative financial instruments	1,092	_
Trade payables	291	445
Current income tax liability		64
Social securities and other taxes	155	108
Pension premiums	_	2
Deferred income	863	711
Other current liabilities	6,159	8,812
Total current liabilities	10,817	10,993
Borrowings	16,577	12,709
Lease liabilities	14,706	
Total liabilities	42,100	23,702
Total equity and liabilities	110,192	117,535

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

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

	Three	month period	Nine month period		
	ended S	eptember 30,	ended September 3		
	2020	2019	2020	2019	
	€ 1,000	€ 1,000	€ 1,000	€ 1,000	
Other income	251	530	9,188	1,509	
Research and development costs	(8,304)	(11,074)	(29,716)	(32,560)	
General and administrative costs	(2,809)	(2,903)	(10,173)	(8,970)	
Total operating costs	(11,113)	(13,977)	(39,889)	(41,530)	
Operating result	(10,862)	(13,447)	(30,701)	(40,021)	
Finance income and expense	(1,863)	1,375	(2,024)	1,339	
Results related to financial liabilities measured at fair value through profit or loss	(305)		(305)		
Results related to associates	(84)	(119)	(270)	579	
Result before corporate income taxes	(13,114)	(12,191)	(33,300)	(38,103)	
Income taxes	(75)		(86)	(64)	
Result for the period	(13,189)	(12,191)	(33,386)	(38,167)	
Other comprehensive income	(255)	147	(134)	121	
Total comprehensive income (attributable to owners of the Company)	(13,444)	(12,044)	(33,520)	(38,046)	
Result attributable to					
Owners of the Company	(13,181)	(12,139)	(33,348)	(37,945)	
Non-controlling interests	(8)	(52)	(38)	(222)	
	(13,189)	(12,191)	(33,386)	(38,167)	
Share information					
Weighted average number of shares outstanding <sup>1</sup>	50,143,262	38,912,701	50,017,990	38,902,203	
Earnings per share attributable to the equity holders of the Company (expressed in Euro per share)					
Basic loss per share <sup>1</sup>	(0.26)	(0.31)	(0.67)	(0.98)	
Diluted loss per share <sup>1</sup>	(0.26)	(0.31)	(0.67)	(0.98)	

<sup>1.</sup> 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.

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

			A	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, 2019	43,149,987	1,726	235,744	10,780		108	(155,443)	92,915	(230)	92,685
Result for the period							(37,945)	(37,945)	(222)	(38,167)
Other comprehensive income						121		121		121
Recognition of share-based payments				4,614	_			4,614		4,614
Issuance of ordinary shares					_					_
Treasury shares transferred	(40,259)				_					_
Share options lapsed				(33)			33			_
Share options exercised	40,259		166	(115)			115	166		166
Balance at September 30, 2019	43,149,987	1,726	235,910	15,246		229	(193,240)	59,871	(452)	59,419
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							(33,348)	(33,348)	(38)	(33,386)
Other comprehensive income					_	(134)		(134)		(134)
Recognition of share-based payments		2	283	6,218				6,503		6,503
Issuance of ordinary shares	100,902	2	270		_			272		272
Treasury shares transferred	(299,615)				_					_
Recognition of equity component of convertible loan					280			280		280
Share options lapsed				(63)			63			
Share options exercised	299,615		724	(466)			466	724		724
Balance at September 30, 2020	54,076,740	2,163	288,491	22,240	280	17	(244,565)	68,626	(534)	68,092

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

		onth period otember 30,	Nine month period ended September 30,		
	2020	2019	2020	2019	
	<u>2020</u> € 1,000	€ 1,000	€ 1,000	€ 1,000	
Cash flows from operating activities	01,000	01,000	01,000	0 1,000	
Net result	(13,189)	(12,191)	(33,386)	(38,167)	
Adjustments for:		<u> </u>	(==/==/	(/ - /	
— Depreciation	651	506	1,703	1,543	
— Share-based compensation	1,676	1,226	6,348	4,614	
— Other income			(8,423)		
— Financial income and expenses	1,863	(1,375)	2,024	(1,339)	
— Results related to associates	84	119	270	(579)	
— Results related to financial liabilities measured at fair value through profit or loss	305		305		
— Net foreign exchange gain / (loss)	(255)	148	(134)	122	
Changes in working capital	(246)	2,718	(3,354)	1,744	
Cash used in operations	(9,111)	(8,849)	(34,647)	(32,062)	
Corporate income tax paid	(157)		(168)	(64)	
Interest received	27	90	118	176	
Interest paid	(569)	(13)	(607)	(64)	
Net cash used in operating activities	(9,810)	(8,772)	(35,304)	(32,014)	
Cash flow from investing activities	<u> </u>				
Purchases of property, plant and equipment	(264)	(32)	(806)	(341)	
Net cash used in investing activities	(264)	(32)	(806)	(341)	
Cash flow from financing activities	<del></del> -	<del></del> -			
Proceeds from issuance of shares, net of transaction costs					
Proceeds from exercise of share options	12	2	724	166	
Proceeds from borrowings			579	_	
Proceeds from convertible loans	13,477		13,542	690	
Repayment of lease liability	(235)	(290)	(542)	(861)	
Net cash generated by/(used in) financing activities	13,254	(288)	14,303	(5)	
Net increase/(decrease) in cash and cash equivalents	3,180	(9,092)	(21,807)	(32,360)	
Currency effect cash and cash equivalents	(1,474)	1,420	(1,296)	1,572	
Cash and cash equivalents, at beginning of the period	87,141	82,464	111,950	105,580	
Cash and cash equivalents at the end of the period	88,847	74,792	88,847	74,792	

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