

## ProQR Therapeutics N.V. (PRQR)

In-Line Financials as PRQR Pushes Rare Disease Pipeline into the Clinic

### MARKET OUTPERFORM

Price: \$3.45  
Price Target: \$20.00

### INVESTMENT HIGHLIGHTS

- **ProQR Therapeutics reported in-line 4Q17 financial results as focus shifts to advancing the rare disease pipeline; we reiterate our Market Outperform rating and \$20 risk-adjusted, DCF-derived price target.**
- PRQR's 4Q17 opex of \$13.6M was just slightly higher than our \$12.8M estimate leading to a net loss of \$13.6M, or \$0.47 per share, vs. our estimate of a loss of \$12.8M or \$0.40 per share. Importantly, the company ended the year with ~\$58M in cash which should be sufficient to fund operations into 2H19 and past clinical readouts from three early-stage programs (Figure 1).
- The first data are expected this year with the six-month readout of a Phase 1/2 of QR-110 in Leber's congenital amaurosis type 10 (LCA10) where we will look for an improvement from baseline on full-field vision stimulation tests as signs of success. Given the recent success from another LCA program by Spark Therapeutics (ONCE, NC), we believe potential positive results from the 12-month readout in 2019 could set the path for registration.
- PRQR has also completed IND-enabling studies for the Phase 1/2 WINGS study of QR-313 for dystrophic epidermolysis bullosa (DEB), which is slated to begin this year with interim data later in 2018 and full results in 2019. The trial is focused on typical safety, tolerability and pharmacokinetics but potential wound healing would be a positive early sign of success.
- Around YE18, we expect QR-421a to start a clinical trial for Usher syndrome type 2A targeting mutations in exon 13, and we were recently pleased with non-dilutive funding (up to \$7.5M) from the Foundation Fighting Blindness which provides external validation for the program, in our view (see our note [here](#)).
- We continue to recommend PRQR shares and at current levels, we see a positive risk-reward profile given cash on hand that should get past potential clinical proof of concept for three rare disease programs with the possibility of quick-to-market development.

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### MARKET DATA

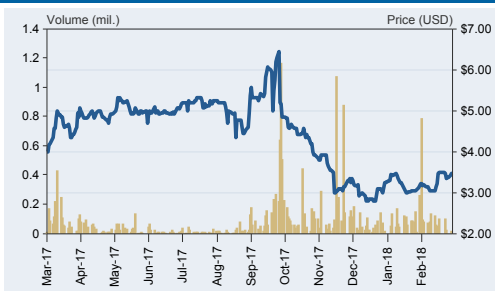
Price	\$3.45
52-Week Range:	\$2.75 - \$6.90
Shares Out. (M):	31.9
Market Cap (\$M):	\$110.1
Average Daily Vol. (000):	17.0
Cash (M):	\$58
LT Debt (M):	\$0

Source: Thomson Reuters and JMP Securities LLC

FY DEC	2017A	2018E	2019E
Revenue (\$M)	1Q \$0.4	\$0.0	--
	2Q \$0.3	\$1.0	--
	3Q \$0.4	\$1.0	--
	4Q \$0.6	\$1.0	--
	<b>FY \$1.7</b>	<b>\$3.0</b>	<b>\$4.5</b>
EPS	1Q (\$0.48)	(\$0.50)	--
	2Q (\$0.54)	(\$0.49)	--
	3Q (\$0.50)	(\$0.50)	--
	4Q (\$0.47)	(\$0.55)	--
	<b>FY (\$1.98)</b>	<b>(\$2.05)</b>	<b>(\$2.16)</b>
Previous FY	(\$1.91)	(\$1.78)	NE

Source: Company reports and JMP Securities LLC

### STOCK PRICE PERFORMANCE



**FIGURE 1. Upcoming Events**

Timing	Event
2018	6-month data from Phase 1/2 of QR-110 for LCA 10
2018	Start Phase 1/2 of QR-313 for DEB
2018	Interim data from Phase 1/2 of QR-313 for DEB
2018	Start Phase 2 of eluforsen (formerly QR-010) for CF subject to a partnership
YE18	Start clinical testing of QR-421a for Usher syndrome type 2a
2019	Data from clinical trial of QR-421a for Usher syndrome type 2a
2019	12-month data from Phase 1/2 of QR-110 for LCA 10
2019	Full data from Phase 1/2 of QR-313 for DEB

Source: Company Reports

## Company Description

ProQR Therapeutics is a biopharmaceutical company engaged in building a platform for RNA-based therapeutics for the treatment of severe genetic disorders, with an initial focus on QR-010, a drug candidate for cystic fibrosis (CF). ProQR utilizes its unique proprietary RNA repair technology to develop candidates to specifically target and repair the defective messenger RNA (mRNA), a product of a mutated gene, and to restore the expression and function of normal protein.

## Investment Risks

**Regulatory risk.** ProQR, like all other drug development companies, is reliant on the regulatory pace of evaluating new drugs and clinical plans and also on regulators' willingness to approve new drugs.

**Clinical development risk.** Drug development is a risky and capital-intensive endeavor. The vast majority of drugs that enter clinical development fail to reach the market.

**Funding risk.** Reliance on the capital markets poses a risk in terms of investor appetite for biotech stocks and the degree of dilution, depending upon the timing of a deal.

**Competitive risk.** There are other drugs in development for cystic fibrosis, by companies including Vertex, Galapagos/AbbVie, and Proteostasis. These programs could provide advantages over ProQR's candidate, which could lead to a lower than anticipated market share.

**Patent risk.** Patent expiration can result in a negative impact to sales. Additionally, generic companies may file abbreviated new drug applications to challenge current products with patent protection.

**Sector risk.** Valuation of pharmaceutical stocks is subject to both investor assessments of the prospects of the underlying companies, and investor tolerance for risk and confidence in the prospects of pharmaceutical stocks as a group. Therefore, ProQR's stock price may fall, even as the company meets or exceeds investor expectations.

**JMP FACTS AND DISCLOSURES**

**Analyst Certification:**

The research analyst(s) who prepared this report does/do hereby certify that the views presented in this report are in accordance with my/our personal views on the securities and issuers discussed in this report. As mandated by SEC Regulation AC no part of my/our compensation was, is or will be directly or indirectly related to the specific views or recommendations expressed herein. This certification is made under the obligations set forth in SEC Regulation AC. Any other person or entity may not use it for any other purpose. This certification is made based on my/our analysis on the date of this report's publication. I/We assume no obligation to update this certification to reflect any facts, circumstances or events that may subsequently come to my/our attention. Signed Liisa A. Bayko and Jonathan Wolleben

**JMP Securities Disclosures:**

JMP Securities currently makes a market in the security of ProQR Therapeutics N.V.

JMP Securities expects to receive OR intends to seek compensation for investment banking services from ProQR Therapeutics N.V. in the next 3 months.

**JMP Securities Investment Opinion Definitions:**

Market Outperform (MO): JMP Securities expects the stock price to outperform relevant market indices over the next 12 months.

Market Perform (MP): JMP Securities expects the stock price to perform in line with relevant market indices over the next 12 months.

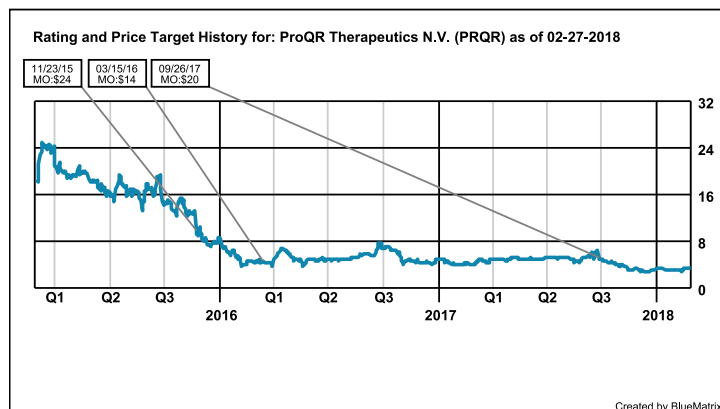
Market Underperform (MU): JMP Securities expects the stock price to underperform relevant market indices over the next 12 months.

**JMP Securities Research Ratings and Investment Banking Services:** (as of February 28, 2018)

JMP Rating	Regulatory Equivalent	# Co's Under Coverage	% of Total	Regulatory Equivalent	# Co's Under Coverage	% of Total	# Co's Receiving IB Services in Past 12 Months	% of Co's With This Rating
MARKET OUTPERFORM	Buy	274	61.85%	Buy	274	61.85%	80	29.20%
MARKET PERFORM	Hold	164	37.02%	Hold	164	37.02%	29	17.68%
MARKET UNDERPERFORM	Sell	5	1.13%	Sell	5	1.13%	0	0%
COVERAGE IN TRANSITION		0	0.00%		0	0.00%	0	0%
RATING SUSPENDED		0	0.00%		0	0.00%	0	0%
<b>TOTAL:</b>		<b>443</b>	<b>100%</b>		<b>443</b>	<b>100%</b>	<b>109</b>	<b>24.60%</b>

**Stock Price Chart of Rating and Target Price Changes:**

Note: First annotation denotes initiation of coverage or 3 years, whichever is shorter. If no target price is listed, then the target price is N/A. In accordance with FINRA Rule 2241, the chart(s) below reflect(s) price range and any changes to the rating or price target as of the end of the most recent calendar quarter. The action reflected in this note is not annotated in the stock price chart. Source: JMP Securities.



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