

# ProQR Therapeutics N.V. (PRQR)

## 1Q17 Financial Results

### MARKET DATA

Price	\$5.05
52-Week Range:	\$3.55 - \$8.70
Shares Out. (M):	23.3
Market Cap (\$M):	\$117.7
Average Daily Vol. (000):	31.0
Cash (M):	\$56
LT Debt (M):	\$0

Source: Thomson Reuters and JMP Securities LLC

**MARKET OUTPERFORM** | Price: \$5.05 | Target Price: \$14.00

### INVESTMENT HIGHLIGHTS

**Financial results for 1Q17 in line as we look forward to first patient data for lead program QR-010 in CF in mid-2017; we reiterate our Market Outperform rating and \$14 risk-adjusted, DCF-derived price target on ProQR Therapeutics.** PRQR ended 1Q17 with ~\$56M in cash, which we believe should be sufficient to fund operations into 2018. Importantly, data from the ongoing Phase 1b cystic fibrosis (CF) study for lead asset QR-010 remain on track in mid-2017. While the purpose of this one-month study is primarily safety and tolerability, and is not powered to detect statistically robust clinical metrics like lung function, we hope to see some trends on FEV1 as a signal of a potential treatment effect. However, clinical improvements are not a gating factor for PRQR to move forward into Phase 2; the company believes it has demonstrated efficacy in the NPD study that reported out in the fall (see our Nov. 2016 note [here](#)). Beyond CF, late last month, PRQR announced it is starting the open-label Phase 1/2 PQ-110-001 trial of QR-110 in patients with Leber's congenital amaurosis Type 10 (LCA10, the most severe form of LCA, which is the most common cause of childhood genetic blindness), and we expect data in 2018. We look for more details on the program and PRQR's early-stage pipeline at an R&D day on June 15. We continue to recommend PRQR shares as we await CF and more long term, data from LCA, which we view as attractive upside to our valuation as we do not yet model any future revenues from the program in our DCF analysis.

### FY DEC 2016A 2017E 2018E

	2016A	2017E	2018E
Revenue (\$M) 1Q	\$0.8	\$0.4A	--
2Q	\$0.6	\$0.0	--
3Q	\$0.5	\$0.0	--
4Q	\$0.1	\$0.0	--
<b>FY</b>	<b>\$2.0</b>	<b>\$0.4</b>	<b>\$0.0</b>
EPS 1Q	(\$0.50)	(\$0.48)A	--
2Q	(\$0.45)	(\$0.50)	--
3Q	(\$0.49)	(\$0.55)	--
4Q	(\$0.40)	(\$0.61)	--
<b>FY</b>	<b>(\$1.77)</b>	<b>(\$2.14)</b>	<b>(\$2.16)</b>
Previous FY	NC	(\$2.04)	NE

Source: Company reports and JMP Securities LLC

### STOCK PRICE PERFORMANCE



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## 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.

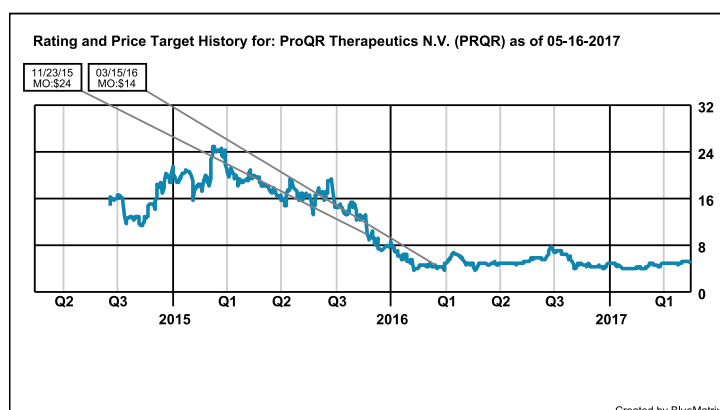
Market Underperform (MU): JMP Securities expects the stock price to underperform relevant market indices over the next 12 months.

### JMP Securities Research Ratings and Investment Banking Services: (as of May 17, 2017)

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	254	55.58%	Buy	254	55.58%	58	22.83%
MARKET PERFORM	Hold	189	41.36%	Hold	189	41.36%	21	11.11%
MARKET UNDERPERFORM	Sell	7	1.53%	Sell	7	1.53%	0	0%
COVERAGE IN TRANSITION		1	0.22%		1	0.22%	0	0%
RATING SUSPENDED		0	0.00%		0	0.00%	0	0%
<b>TOTAL:</b>		<b>457</b>	<b>100%</b>		<b>457</b>	<b>100%</b>	<b>83</b>	<b>18.16%</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 NASD Rule 2711, 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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