

## ProQR Therapeutics N.V. (PRQR)

Expert Comments on NPD Results

### MARKET DATA

Price	\$4.95
52-Week Range:	\$3.48 - \$8.70
Shares Out. (M):	23.3
Market Cap (\$M):	\$115.3
Average Daily Vol. (000):	27.0
Cash (M):	\$73
LT Debt (M):	\$0

Source: Thomson Reuters and JMP Securities LLC

**MARKET OUTPERFORM** | Price: \$4.95 | Target Price: \$14.00

### INVESTMENT HIGHLIGHTS

**Dr. Rowe projects 5-8% FEV1 improvement for QR-010 based on NPD results; we reiterate our Market Outperform rating and \$14 risk-adjusted, DCF-derived price target on ProQR Therapeutics.** We hosted a call with nasal potential difference (NPD) expert and central reader of PRQR's NPD study, Dr. Steven Rowe, who projected that if the amount of CFTR activity observed in the study translated perfectly to the lungs, QR-010 could lead to a 5.0-8.0% improvement in absolute perfect predicted FEV1, which would be an improvement upon the current standard of care, Orkambi (2.6-3.0% in homozygous F508del mutations). That said, he recognized that delivery to the lung could be a challenge, resulting in a lower FEV change than his optimistic projection. Recall, QR-010 led to a -4.1 mV change in patients homozygous for the F508del mutation in its NPD study vs. -4.0 to -5.6 mV change for Kalydeco in patients with a G551D mutation, but Dr. Rowe mentioned the Kalydeco data may be discounted 5-10% because the older data did not fully implement the now standardized procedures used in ProQR's study (Orkambi was not tested in a NPD study). On our call, Dr. Rowe also highlighted key points that were first mentioned at a dinner we hosted for investors in New York (see our [November 18 note](#)), including: 1) the NPD study followed a well standardized procedure; 2) the results were above the noise of the assay and not driven by outliers; and 3) he actually expected the results in the cohort of patients heterozygous for the F508del mutation (+2.2 mV change) and he believes that data adds confidence to homozygous results. Net-net, we think the NPD results are solid and lay a good foundation for the ongoing MAD study (we expect data in mid-2017); therefore, we feel the shares are undervalued at current levels before assigning any value to the rest of the pipeline, which could begin to deliver data in the 2018-2019 timeframe. A transcript of the call can be obtained from your JMP Securities sales representative.

### FY DEC 2015A 2016E 2017E

Revenue (\$M)	1Q	\$0.4	\$0.8A	\$0.0
	2Q	\$0.8	\$0.6A	\$0.0
	3Q	\$1.3	\$0.5A	\$0.0
	4Q	\$1.0	\$0.0	\$0.0
	<b>FY</b>	<b>\$3.6</b>	<b>\$1.9</b>	<b>\$0.0</b>
EPS	1Q	\$0.01	(\$0.50)A	(\$0.63)
	2Q	(\$0.41)	(\$0.45)A	(\$0.74)
	3Q	(\$0.30)	(\$0.49)A	(\$0.53)
	4Q	(\$0.29)	(\$0.58)	(\$0.55)
	<b>FY</b>	<b>(\$0.98)</b>	<b>(\$1.94)</b>	<b>(\$2.40)</b>
Previous FY	NC	(\$1.92)	(\$1.86)	

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 upon 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.

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**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.

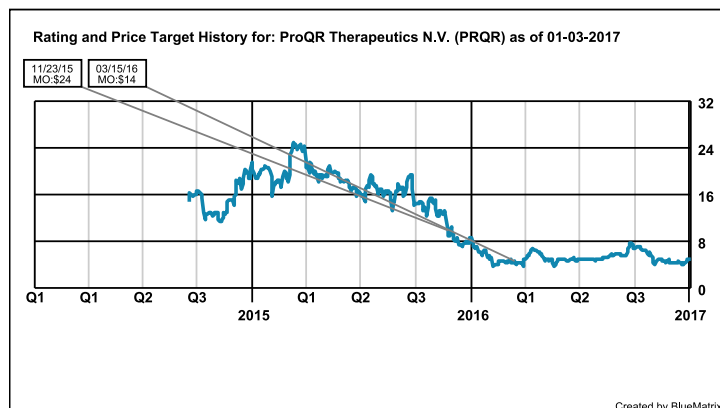
Market Underperform (MU): JMP Securities expects the stock price to underperform relevant market indices over the next 12 months.

**JMP Securities Research Ratings and Investment Banking Services:** (as of January 4, 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	261	57.49%	Buy	261	57.49%	49	18.77%
MARKET PERFORM	Hold	171	37.67%	Hold	171	37.67%	14	8.19%
MARKET UNDERPERFORM	Sell	8	1.76%	Sell	8	1.76%	0	0%
COVERAGE IN TRANSITION		12	2.64%		12	2.64%	1	8.33%
RATING SUSPENDED		1	0.22%		1	0.22%	0	0%
<b>TOTAL:</b>		<b>454</b>	<b>100%</b>		<b>454</b>	<b>100%</b>	<b>65</b>	<b>14.32%</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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