

# **ProQR Therapeutics N.V.** (PRQR)

CF Strategy Detailed at Investor Event Last Night

MARKET OUTPERFORM

Price: \$3.90 Price Target: \$20.00

#### Liisa A. Bayko

Ibayko@jmpsecurities.com (312) 768-1785

#### Jonathan Wolleben

jwolleben@jmpsecurities.com (312) 768-1788

# MARKET DATA Price \$3.90 52-Week Range: \$3.65 - \$6.90 Shares Out. (M): 25.1 Market Cap (\$M): \$97.9 Average Daily Vol. (000): 292.0 Cash (M): \$48 LT Debt (M): \$0

Source: Thomson Reuters and JMP Securities LLC

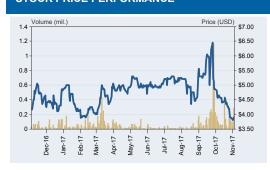
FY DEC		2016A	2017E	2018E
Revenue (\$M)	1Q	\$0.8	\$0.4A	
, ,	2Q	\$0.6	\$0.3A	
	3Q	\$0.5	\$0.0	
	4Q	\$0.1	\$0.0	
	FY	\$2.0	\$0.7	\$0.0
EPS	1Q	(\$0.50)	(\$0.48)A	
	2Q	(\$0.45)	(\$0.54)A	
	3Q	(\$0.49)	(\$0.51)	
	4Q	(\$0.40)	(\$0.54)	
	FY	(\$1.77)	(\$2.07)	(\$1.93)

Source: Company reports and JMP Securities LLC

# **INVESTMENT HIGHLIGHTS**

- ProQR Therapeutics lays out development strategy in CF; we reiterate our Market Outperform rating and \$20 risk-adjusted, DCF-derived price target.
   We attended PRQR's investor event yesterday at the NACFC annual meeting where its Phase 1b QR-010 data were discussed in detail (our takeaways remain primarily consistent with our top-line analysis <a href="here">here</a>) and the development strategy was discussed in detail for the first time.
- Proposed Phase 2 design. PRQR is attending an end-of-Phase 1 meeting with the FDA shortly to discuss its plans moving forward with QR-010 and management discussed its proposed outline of a Phase 2 program slated to begin in 2H18 pending regulatory and partnership discussions. The goal at this point is to move forward with a partner and therefore these plans are preliminary concepts. Key considerations are that PRQR does not want to change many variables from its Phase 1b study, but will potentially look at weekly dosing (vs. 3x a week in Phase 1b) and a primary endpoint of lung clearance index (LCI) in relatively healthy patients with FEV>70% with abnormal LCI. The choice of the population has two benefits: 1) consistency with Phase 1b, and 2) positioning for patients on CFTR modulators having better FEV.
- MOA coming along. QR-010's exact mechanism of action is still under investigation a common criticism of the program but the company is working diligently to learn more and confident it has enough information that will be sufficient for regulators while it may be more descriptive than mechanistic at this point in time. We think progress on this front will just provide investors/possible partners more peace of mind, while we continue to be impressed with the data that have unfolded.
- CF pipeline. Management also discussed in some detail its CF pipeline (Figure 1) going after the 5% of CF patients with Class 1 mutations that do not make any CFTR protein and thus cannot be treated with modulators. PRQR is working on developing leads for five different programs and the company was actually highlighted in the NACFC Day 1 Plenary session for going after this high unmet need population with a novel mechanism. In this patient population, PRQR would be willing to combine with a modulator as there is no downside in the context of no available therapies.
- Thoughts on the stock. Net-net, we like PRQR's strategy moving forward and over the past year have gained more confidence in QR-010 based on its ability to restore CFTR function as evidenced in NPD studies and improve symptoms in the more recent Phase 1b study. We continue to recommend owning shares of PRQR as we think the stock is significantly undervalued relative to the opportunity at hand in CF, not to mention the rest of the emerging rare disease pipeline (Figure 2) which could begin to bear fruit in 2018.

#### STOCK PRICE PERFORMANCE







Source: Company Presentation

### FIGURE 2.PRQR Full Development Pipeline



Source: Company Presentation

November 3, 2017 2



# **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.

November 3, 2017



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

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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.

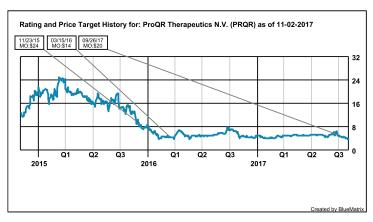
Market Underperform (MU): JMP Securities expects the stock price to underperform relevant market indices over the next 12 months.

JMP Securities Research Ratings and Investment Banking Services: (as of November 3, 2017)

		# Co's	%		# Co's	%	Services in	% of Co's
	Regulatory	Under	of	Regulatory	Under	of	Past 12	With This
JMP Rating	Equivalent	Coverage	Total	Equivalent	Coverage	Total	Months	Rating
MARKET OUTPERFORM	Buy	272	60.58%	Buy	272	60.58%	72	26.47%
MARKET PERFORM	Hold	168	37.42%	Hold	168	37.42%	30	17.86%
MARKET UNDERPERFORM	Sell	7	1.56%	Sell	7	1.56%	0	0%
COVERAGE IN TRANSITION		0	0.00%		0	0.00%	0	0%
RATING SUSPENDED		0	0.00%		0	0.00%	0	0%
TOTAL:		449	100%		449	100%	102	22.72%

#### **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.



November 3, 2017 4



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November 3, 2017 5



Jeffrey H. Spurr Director of Research (415) 835-3903

# **RESEARCH PROFESSIONALS**

#### **FINANCIAL SERVICES**

Alternative Asset Managers	(040) 000 0570	Medical Devices & Supplies	(040) 000 0500		
Devin Ryan	(212) 906-3578	David Turkaly	(212) 906-3563		
Brian McKenna	(212) 906-3545	John Gillings	(212) 906-3564		
Commercial & Specialty Finance		Specialty Pharmaceuticals			
Christopher York	(415) 835-8965	Donald Ellis	(212) 906-3507		
Thomas Wenk	(415) 835-8962	Nazibur Rahman	(212) 906-3519		
	( -,		` ,		
Consumer Finance		REAL ESTATE			
David M. Scharf	(415) 835-8942				
Jeff Zhang, CFA	(415) 835-8948	Housing & Land Development	(445) 005 0004		
		Peter L. Martin, CFA	(415) 835-8904		
Financial Processing & Outsourcing	(445) 005 0040	Aaron Hecht	(415) 835-3963		
David M. Scharf	(415) 835-8942	Doug Hansen	(415) 835-8934		
Jeff Zhang, CFA	(415) 835-8948	Droporty Convince			
Inc., man and		Property Services Mitch Germain	(212) 006 2546		
Insurance	(242) 700 4704	Corey DeVito	(212) 906-3546 (212) 906-3525		
Matthew J. Carletti	(312) 768-1784	Coley Devilo	(212) 900-3323		
Karol Chmiel	(312) 768-1786	REITs: Healthcare, Residential, & Specialty			
Investment Banks & Brokers		Peter L. Martin, CFA	(415) 835-8904		
Devin Ryan	(212) 906-3578	Aaron Hecht	(415) 835-3963		
Brian McKenna	(212) 906-3545	Brian Riley	(415) 835-8908		
Bhan Wortenna	(212) 300 0040	Brian Falloy	(110) 000 0000		
Mortgage Operating Companies		REITs: Diversified, Industrial, Office, & Re	tail		
REITs: Agency, Hybrid, & Commercial Mo	ortgage	Mitch Germain	(212) 906-3546		
Steven C. DeLaney	(212) 906-3517	Corey DeVito	(212) 906-3525		
Trevor Cranston, CFA	(415) 869-4431	•	, ,		
Mikhail Goberman	(212) 906-3543	Residential Services			
Benjamin Zucker	(212) 906-3529	Peter L. Martin, CFA	(415) 835-8904		
		Aaron Hecht	(415) 835-3963		
Regional Banks		TECHNOLOGY			
Emlen Harmon	(212) 906-3547	TECHNOLOGY			
Chris Muller	(212) 906-3559	Energy Technology & Complete			
HEALTHOADE		Energy Technology & Services Industrial Technology			
HEALTHCARE		Joseph Osha	(415) 835-8998		
Biotechnology		оозерн Озна	(413) 033-0330		
Liisa A. Bayko	(312) 768-1785	Internet Security, Communications Infras	tructure. & Storage		
Amy Wang	(312) 768-1796	Erik Suppiger	(415) 835-3918		
Jonathan Wolleben	(312) 768-1788	Michael Berg	(415) 835-3914		
Jason N. Butler, PhD	(212) 906-3505		( )		
Roy Buchanan	(212) 906-3509	Internet & Digital Media			
Michael G. King, Jr.	(212) 906-3520	Ronald V. Josey III	(212) 906-3528		
Konstantine Aprilakis, MD	(212) 906-3503	Andrew Boone, CFA	(415) 835-3957		
Michael Englander	(212) 906-3540	Shweta Khajuria	(415) 835-8916		
Simon Gruber	(212) 906-3538	·			
	, , , , , , , , , , , , , , , , , , , ,	Software			
Healthcare Services & Facilities		Patrick Walravens	(415) 835-8943		
Peter L. Martin, CFA	(415) 835-8904	Mathew Spencer	(415) 835-8930		
Brian Riley	(415) 835-8908	Greg McDowell	(415) 835-3934		
-	•	Rishi Jaluria	(415) 835-3961		

# **ADDITIONAL CONTACTS**