

ProQR Therapeutics N.V. (PRQR)

Visibility on QR-010 in Cystic Fibrosis Just Around the Corner

MARKET DATA

Price	\$5.75
52-Week Range:	\$3.65 - \$8.70
Shares Out. (M):	25.1
Market Cap (\$M):	\$144.3
Average Daily Vol. (000):	177.0
Cash (M):	\$48
LT Debt (M):	\$0

Source: Thomson Reuters and JMP Securities LLC

MARKET OUTPERFORM | Price: \$5.75 | Target Price: \$14.00

INVESTMENT HIGHLIGHTS

Looking for safety and possible efficacy signal in CF data on Monday; we reiterate our Market Outperform rating and \$14 risk-adjusted, DCF derived price target on ProQR Therapeutics. Monday is a key inflection point for PRQR as its lead asset QR-010, an inhaled RNA-based, CFTR modulator, will report safety and efficacy data from a small, 28-day, Phase 1b study in cystic fibrosis (CF) patients with F508del homozygous mutations. We look for good safety/tolerability (the earlier single-ascending dose study laid a nice foundation), and we believe some trends in lung function improvement would be a nice to have. In our view, reasons to be optimistic for efficacy trends include evidence of in vivo, on-target activity in the NPD study (see our note from 10/27/16) and strong animal data, offset by an unknown mechanism of action, emerging understanding of the PK/PD as this is the first multi-dose study, a relatively healthy study population with baseline FEV >70%, and unknown ability to cross the mucous layer in CF patients (in vitro experience in non-CF mucous showed good migration). We caution the direct comparison to oral CFTR correctors for these reasons (see Figure 1), but present the data strictly as a reminder of what has been seen in this class in the F508del homozygous population. As for other exploratory efficacy endpoints, we think the study is too short to expect weight gain and we do not expect improvements in sweat chloride due to the lack of uptake of QR-010 in sweat glands. If all goes well, a larger Phase 2 study in CF will start in 2018.

Beyond CF, we see significant potential upside from the company's broad, RNA-based, early-stage pipeline targeting a number of rare diseases in ophthalmology and dermatology as well as the new Axiomer RNA-editing technology platform (Figure 2). With QR-110 for Leber's congenital amaurosis Type 10 (LCA 10) and QR-313 for dystrophic epidermolysis bullosa (DEB) both entering the clinic soon, we think these programs are currently under the radar and under-appreciated by the Street. We continue to recommend shares of PRQR ahead of Phase 1b CF data on Monday and we see substantial potential upside long term from the remainder of the company's expanding pipeline.

FY DEC 2016A 2017E 2018E

		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

STOCK PRICE PERFORMANCE



FIGURE 1. Benchmark Efficacy Data in F508del Homozygotes

F508 del homozygotes	Mean absolute change in ppFEV1			
	Baseline	Day 28	Week 12	Week 24
Orkambi	~61%	+2.5%	+3.0-3.3%	+2.5-3.0%
Teza/iva combo	~60%	+4.4%	+3%	+3.4%
VX-440 triple combo	40-90%	+9.5%	-	-
VX-152 triple combo	40-90%	+7.3%	-	-
QR-101	>70%	-	-	-
F508 del homozygotes	Placebo-adjusted mean absolute change in ppFEV1			
F508 del homozygotes	Baseline	Day 28	Week 12	Week 24
Orkambi	~61%	+2.4%	+3.2-3.5%	+2.8-3.3%
Teza/iva combo	~60%	+4.8%	+2.0%	+4.0%
VX-440 triple combo	40-90%	+12.0%	-	-
VX-152 triple combo	40-90%	+8.7%	-	-
QR-101	>70%	-	-	-

Source: Company Reports

FIGURE 2. Key Programs in the Pipeline

<p>Cystic Fibrosis</p>	<p>Inherited blindness</p>	<p>Debilitating skin disorders</p>	<p>Highlights Innovation pipeline</p>
<p>QR-010 for F508del cystic fibrosis</p> <ul style="list-style-type: none"> ✓ Positive clinical data in NPD biomarker study • Phase 1b study top line data expected in September 2017 	<p>QR-110 for LCA10 Pipeline</p> <ul style="list-style-type: none"> • QRX-411 for Usher syndrome • QRX-421 for Usher syndrome • QRX-504 for FECD • QRX-1011 for Stargardt's Disease 	<p>QR-313 for DEB Pipeline</p> <ul style="list-style-type: none"> • QRX-323 for DEB • QRX-333 for DEB • QRX-343 for DEB 	<p>Axiomer®</p> <ul style="list-style-type: none"> • Novel RNA editing platform technology • Direct ADAR to make specific edits in RNA • >20,000 G>A mutations

Source: Company R&D Day 2017

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.

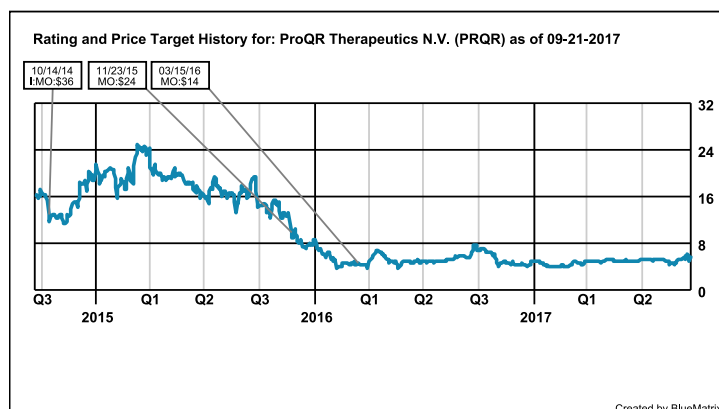
Market Underperform (MU): JMP Securities expects the stock price to underperform relevant market indices over the next 12 months.

JMP Securities Research Ratings and Investment Banking Services: (as of September 22, 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	279	60.00%	Buy	279	60.00%	76	27.24%
MARKET PERFORM	Hold	176	37.85%	Hold	176	37.85%	28	15.91%
MARKET UNDERPERFORM	Sell	8	1.72%	Sell	8	1.72%	0	0%
COVERAGE IN TRANSITION		0	0.00%		0	0.00%	0	0%
RATING SUSPENDED		0	0.00%		0	0.00%	0	0%
TOTAL:		465	100%		465	100%	105	22.58%

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.



JMP Disclaimer:

JMP Securities LLC (the “Firm”) compensates research analysts, like other Firm employees, based on the Firm’s profitability, which includes revenues from the Firm’s institutional sales, trading, and investment banking departments as well as on the quality of the services and activities performed that are intended to benefit the Firm’s institutional clients. These data have been prepared by JMP Securities LLC for informational purposes only and are based on information available to the public from sources that we believe to be reliable, but we do not guarantee their accuracy or completeness. Any opinions and projections expressed herein reflect our judgment at this date and are subject to change without notice. These data are neither intended nor should be considered as an offer to sell or a solicitation or a basis for any contract for the purchase of any security or other financial product. JMP Securities LLC, its affiliates, JMP Group LLC, Harvest Capital Strategies LLC, and their respective partners, directors, officers, and associates may have a long or short position in, may act as a market maker for, or may purchase or sell a position in the securities mentioned herein. JMP Securities LLC or its affiliates may be performing, have performed, or seek to perform investment banking, advisory, or other services and may have acted as manager or co-manager for a public offering of securities for any company mentioned herein. The reader should assume that JMP Securities LLC will solicit business from the company covered in this report. Members of our Sales and Trading Department provide oral and/or written market opinions and trading strategies to our clients that reflect their personal opinions about stocks that are the subject of the firm's research reports. Our research analysts discuss trading strategies with clients that sometimes reflect short-term expectations for the price of the securities that are the subject of research reports. These trading strategies are distinct from the analysts' fundamental rating for the stock, which is based upon the analysts' view compared to other stocks under coverage for the relevant time period. © Copyright 2017. All rights reserved by JMP Securities LLC. JMP Securities LLC is a member of FINRA, NASDAQ, and SIPC.

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

RESEARCH PROFESSIONALS

FINANCIAL SERVICES

Alternative Asset Managers

Devin Ryan (212) 906-3578
 Brian McKenna (212) 906-3545

Commercial & Specialty Finance

Christopher York (415) 835-8965
 Thomas Wenk (415) 835-8962

Consumer Finance

David M. Scharf (415) 835-8942
 Jeff Zhang, CFA (415) 835-8948

Financial Processing & Outsourcing

David M. Scharf (415) 835-8942
 Jeff Zhang, CFA (415) 835-8948

Insurance

Matthew J. Carletti (312) 768-1784
 Karol Chmiel (312) 768-1786

Investment Banks & Brokers

Devin Ryan (212) 906-3578
 Brian McKenna (212) 906-3545

Mortgage Operating Companies

REITs: Agency, Hybrid, & Commercial Mortgage

Steven C. DeLaney (212) 906-3517
 Trevor Cranston, CFA (415) 869-4431
 Mikhail Goberman (212) 906-3543
 Benjamin Zucker (212) 906-3529

Regional Banks

Emlen Harmon (212) 906-3547
 Chris Muller (212) 906-3559

HEALTHCARE

Biotechnology

Liisa A. Bayko (312) 768-1785
 Amy Wang (312) 768-1796
 Jonathan Wolleben (312) 768-1788
 Jason N. Butler, PhD (212) 906-3505
 Roy Buchanan (212) 906-3509
 Michael G. King, Jr. (212) 906-3520
 Konstantine Aprilakis, MD (212) 906-3503
 Michael Englander (212) 906-3540

Healthcare Services & Facilities

Peter L. Martin, CFA (415) 835-8904
 Brian Riley (415) 835-8908

Medical Devices & Supplies

David Turkaly (212) 906-3563
 John Gillings (212) 906-3564

Specialty Pharmaceuticals

Donald Ellis (212) 906-3507
 Nazibur Rahman (212) 906-3519

REAL ESTATE

Housing & Land Development

Peter L. Martin, CFA (415) 835-8904
 Aaron Hecht (415) 835-3963
 Doug Hansen (415) 835-8934

Lodging & Leisure/REITs: Hotels

Whitney Stevenson (212) 906-3538

Property Services

Mitch Germain (212) 906-3546
 Corey DeVito (212) 906-3525

REITs: Healthcare, Residential, & Specialty

Peter L. Martin, CFA (415) 835-8904
 Aaron Hecht (415) 835-3963
 Brian Riley (415) 835-8908

REITs: Diversified, Industrial, Office, & Retail

Mitch Germain (212) 906-3546
 Corey DeVito (212) 906-3525

Residential Services

Peter L. Martin, CFA (415) 835-8904
 Aaron Hecht (415) 835-3963

TECHNOLOGY

Energy Technology & Services

Industrial Technology

Joseph Osha (415) 835-8998
 McCrea Dunton (415) 835-8996

Internet Security, Communications Infrastructure, & Storage

Erik Suppiger (415) 835-3918
 Michael Berg (415) 835-3914

Internet & Digital Media

Ronald V. Josey III (212) 906-3528
 Andrew Boone, CFA (415) 835-3957
 Shweta Khajuria (415) 835-8916

Software

Patrick Walravens (415) 835-8943
 Mathew Spencer (415) 835-8930
 Greg McDowell (415) 835-3934
 Rishi Jaluria (415) 835-3961

ADDITIONAL CONTACTS

Thomas R. Wright
 Director of Equities
 (212) 906-3599

Charles Sweeney
 Director of Sales & Trading
 (212) 906-3573

600 Montgomery Street, Suite 1100
 San Francisco, CA 94111
www.jmpsecurities.com