JMP

ProQR Therapeutics N.V. (PRQR)

Usher Syndrome Funding

MARKET OUTPERFORM
Price: $2.90
Price Target: $20.00

INVESTMENT HIGHLIGHTS

- **Foundation provides non-dilutive funding of up to $7.5M for preclinical Usher syndrome program; we reiterate our Market Outperform rating and $20 risk-adjusted, DCF-derived price target on ProQR Therapeutics.**
- PRQR will receive up to $7.5M in milestone-based co-funding from Foundation Fighting Blindness to develop QR-421a, an RNA oligonucleotide currently in preclinical development for Usher syndrome type 2A targeting mutations in exon 13 of the causative USH2A gene. QR-421a is intended to produce a shortened but functional usherin protein in the retina to stop disease progression and hopefully gain peripheral vision. We look for additional details about the first clinical trial of QR-421a this year and expect to have the first clinical data later in 2019.
- We like the non-dilutive funding from this deal, but we also like the external validation of the program. Further, PRQR will gain access to data from the Foundation which could help inform QR-421a development. For example, last year the Foundation launched the RUSH2A natural history study in 120 people with USH2A mutations at 20 sites around the world monitoring changes in vision and retinal structure to track disease progression. The goal is to better understand how USH2A mutations affect disease severity and the progressive vision loss and that information could benefit PRQR in future study designs.
- QR-421a is the second most advanced program in PRQR's ophthalmology pipeline. QR-110, the most advanced, is in a Phase 1/2 study for Leber's congenital amaurosis 10 (LCA 10) and we look to interim safety and efficacy data this year from the majority of patients after six months of treatment and full 12-month data from all 12 patients are expected in 2019. PRQR also has additional ophthalmology preclinical assets lined up, including QRX-411 for Usher syndrome type 2A due to the PE-40 mutation, QRX-1011 for Stargardt's disease and QRX-504 for Fuchs endothelial corneal dystrophy. We think ophthalmic indications make sense for PRQR's technology for a variety of reasons including: 1) a wide-array of RNA defects can be altered; 2) efficient entry into the cells; and 3) low systemic exposure.
- We continue to recommend shares of PRQR as the company makes headway on the early-stage rare disease portfolio leveraging the unique RNA editing platforms while delivering first-in-patient data from the ophthalmology and dermatology programs this year.

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MARKET DATA

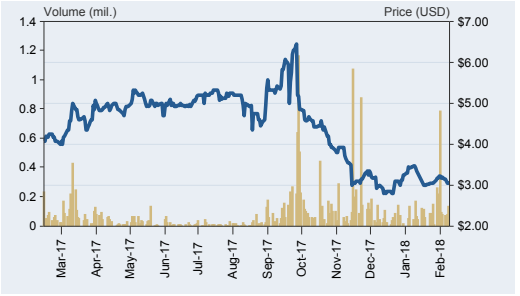
Price	$2.90
52-Week Range:	$2.75 - $6.90
Shares Out. (M):	31.9
Market Cap ($M):	92.5
Average Daily Vol. (000):	124.0
Cash (M):	$67
LT Debt (M):	$0

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	$0.4A	--
	4Q	$0.1	$0.0	--
	FY	**$2.0**	**$1.1**	**$0.0**
EPS	1Q	($0.50)	($0.48)A	--
	2Q	($0.45)	($0.54)A	--
	3Q	($0.49)	($0.50)A	--
	4Q	($0.40)	($0.40)	--
	FY	**($1.77)**	**($1.91)**	**($1.78)**

Source: Company reports and JMP Securities LLC

STOCK PRICE PERFORMANCE



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:

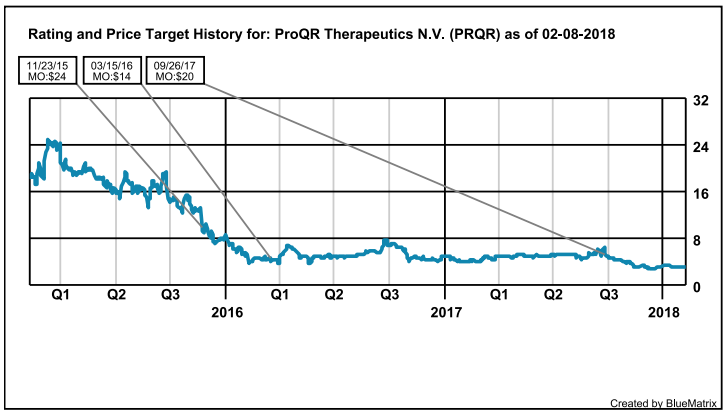
Market Outperform (MO): JMP Securities expects the stock price to outperform relevant market indices over the next 12 months.
Market Perform (MP): JMP Securities expects the stock price to perform in line with relevant market indices over the next 12 months.
Market Underperform (MU): JMP Securities expects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

JMP Securities Research Ratings and Investment Banking Services: (as of February 12, 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	279	*61.18%*	Buy	279	*61.18%*	76	*27.24%*
MARKET PERFORM	Hold	170	*37.28%*	Hold	170	*37.28%*	29	*17.06%*
MARKET UNDERPERFORM	Sell	7	*1.54%*	Sell	7	*1.54%*	0	*0%*
COVERAGE IN TRANSITION		0	*0.00%*		0	*0.00%*	0	*0%*
RATING SUSPENDED		0	*0.00%*		0	*0.00%*	0	*0%*
TOTAL:		456	*100%*		456	*100%*	105	*23.03%*

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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