

May 17, 2017

OUTPERFORM

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Reason for report:

EARNINGS

PROQR THERAPEUTICS N.V.

1Q Recap: QR-110 Entering Ph.1/2; QR-010 MAD Update Mid-2017

• **Bottom Line:** In addition to the ongoing Ph.1 QR-010 study in cystic fibrosis (CF), clearance of the investigational new drug (IND) application for QR-110 (Leber's congenital amaurosis type 10/LCA 10) paves the way to evaluate PRQR's RNA repair mechanism in an ophthalmic indication. Near-term, investors can expect Ph.1b multiple ascending dose (MAD) data for QR-010 in mid-2017 and the initiation of a Ph.1/2 QR-110 study (with top-line data expected in 2018; [LINK](#)). We are updating our ProQR model following 1Q financial results reporting a net loss/EPS of €11M/(€0.45). Reiterate OP on PRQR with \$10 PT.

• **QR-010 MAD data on track for mid-2017 ([LINK](#)); issuance of key patents protects utility in CF.** Recall, ProQR presented intriguing nasal potential difference (NPD) data at the North American CF Conference (NACFC; [LINK](#)) that suggested potential efficacy in homozygous delF508 pts. In mid-2017, investors can gain additional insight on QR-010's product profile as the MAD study evaluates exploratory endpoints like forced expiratory volume in 1 second (FEV1), CFQ-R, weight gain, and sweat chloride. Our first glimpse on these clinically relevant metrics, albeit insufficiently powered to detect stat. sig., may help draw correlations to previously reported NPD data.

• **QR-110 in LCA10 clears IND with top-line results in 2018.** Mgmt. will soon initiate an open-label trial enrolling 12 pts. (2 pediatric, ages 6-17 + 2 adult pts./arm; with 1 or 2 copies of the p.Cys998X mutation) across 3 treatment arms. Each pt. will have generate both treated and control (untreated, contralateral eye) data. The trial will be 1 year in duration and will test 4 intravitreal administrations (1x/quarter) of QR-110. ProQR is evaluating numerous efficacy endpoints in this trial that are objective (e.g., visual acuity/VA, full field stimulus testing/FST, optical coherence tomography/OCT, and pupillary light reflex/PLR) and a more subjective modified mobility course - similar to those that ONCE [OP] used in voretigene neparvovec trials, in addition to safety and pharmacokinetic/PK data. ProQR expects to conduct the trial at 3 clinical sites in both the US and EU.

• **Model update for 1Q financial results.** We are adjusting our model for increased OpEx spend (assuming QR-010 and QR-110 clinical studies) and taking into account cash runway projection into 2H18.

Key Stats: (NASDAQ: PRQR)

Sector: Biotechnology
S&P 600 Health Care Index: 1,989.80
Price : \$4.95
Price Target: \$10.00
Methodology: DCF with 12% discount rate, 2% terminal value growth rate

52 Week High: \$8.70
52 Week Low: \$3.55
Shares Outstanding (mil): 23.5
Market Capitalization (mil): \$116.3
Cash Per Share: \$2.17
Net Debt to Total Capital: 0%
Dividend (ann): \$0.00
Dividend Yield: 0.0%

Completion: May 17, 2017, 9:26AM EDT.

Distribution: May 17, 2017, 9:26AM EDT.

Cash Per Share: Net cash per diluted share



Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2016A	€0.7	€0.6	€0.4	€0.1	€1.8	(€0.44)	(€0.43)	(€0.43)	(€0.38)	(€1.68)	NM
2017E - New	€0.4A	€0.3	€0.3	€0.3	€1.1	(€0.45)A	(€0.46)	(€0.48)	(€0.51)	(€1.90)	NM
2017E - Old	€0.3	€0.3	€0.3	€0.3	€1.0	(€0.46)	(€0.48)	(€0.32)	(€0.34)	(€1.53)	NM
2018E - New	--	--	--	--	€1.0	--	--	--	--	(€1.33)	NM
2018E - Old	--	--	--	--	€1.0	--	--	--	--	(€1.77)	NM

Source: Company Information and Leerink Partners LLC Research
IFRS; Revenues in millions.

INVESTMENT THESIS

We rate PRQR Outperform. PRQR is developing novel RNA therapeutics for severe genetic disorders. The company's lead candidate QR-010 has generated highly encouraging preclinical data in a number of validated pre-clinical assays used to evaluate drugs for cystic fibrosis, in our view. QR-010 is an inhaled antisense oligonucleotide (AON) designed to treat patients with $\Delta F508$ mutation, the most common mutation underlying cystic fibrosis (CF). The QR-010 RNA repair technology was established in published experiments first employed in cell cultures at Massachusetts General Hospital (MGH), and PRQR has been able to replicate and augment the molecule with an optimized antisense construct that has composition-of-matter patent protection until 2027 (not including up to 5 years of potential extension).

We look to multiple catalysts over the next 12-18 months for potential de-risking of QR-010 and appreciation in PRQR shares. We see an attractive risk/reward for PRQR shares at the stock's current valuation based on the robust pre-clinical data suggesting that QR-010 can restore CFTR function to near-wild-type levels and large amount of shareholder value created by VRTX, whose CFTR modulators have generated relatively modest FEV1 (forced expiratory volume in 1 second) improvements in homozygous $\Delta F508$ CF patients.

Our \$10 price target in 12 months is based on our assumptions of QR-010 pricing of \$175k/patient/year together with 50% peak market penetration of $\Delta F508$ CF homozygotes and heterozygotes, probability-weighted at 20% and 0%, respectively. PRQR has also identified around 50 other target indications for its pipeline, including a pre-clinical antisense compound QR-110 for Leber's Congenital Amaurosis (LCA), dystrophic epidermolysis bullosa (DEB), Usher syndrome, Fuchs endothelial corneal dystrophy, Alzheimer's disease, and other CFTR targets covering an additional 10%+ of CF patients, which we do not currently include in our valuation.

VALUATION

We derive a \$10 price target for PRQR shares in 12 months based on a 12% discount rate and a 2% terminal growth rate, which we believe are appropriate given: (1) the early stage of PRQR, and (2) the fact that our revenue estimates are already risk-adjusted via probabilities of success. We assume 20% and 0% probabilities of success for QR-010 in F508del homozygous and heterozygous cystic fibrosis patients, respectively. We model ~€530MM in peak risk-adjusted WW revenues in 2024E.

RISKS TO VALUATION

Risks include disappointing clinical data, regulatory and clinical setbacks, the potential for dilutive financing and commercial shortfalls. Since PRQR has only one product in clinical testing, any of the aforementioned setbacks could impact the stock significantly.

PRQR P&L (€MM) - IFRS	2012	2013	2014	2015	1Q16	2Q16	3Q16	4Q16	2016	1Q17	2Q17E	3Q17E	4Q17E	2017E	2018E
Revenue & Other Income	0.0	0.1	0.3	3.2	0.7	0.6	0.4	0.1	1.8	0.4	0.3	0.3	0.3	1.1	1.0
COGS	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
R&D	(0.3)	(2.6)	(10.3)	(23.4)	(6.9)	(8.6)	(8.3)	(8.1)	(31.9)	(8.0)	(8.4)	(9.0)	(9.7)	(35.1)	(43.9)
SG&A	(0.2)	(0.8)	(6.5)	(6.8)	(2.6)	(2.6)	(2.0)	(2.3)	(9.5)	(2.3)	(2.8)	(3.0)	(3.3)	(11.4)	(14.8)
Operating Expenses	(0.4)	(3.4)	(16.8)	(30.2)	(9.5)	(11.2)	(10.3)	(10.4)	(41.4)	(10.3)	(11.2)	(12.0)	(13.0)	(46.5)	(58.7)
Operating Income	(0.4)	(3.2)	(16.5)	(27.0)	(8.8)	(10.6)	(9.9)	(10.3)	(39.6)	(9.9)	(11.0)	(11.8)	(12.7)	(45.4)	(57.7)
Finance Income (Expense)	0.0	(0.0)	4.3	6.2	(1.4)	0.7	(0.3)	1.4	0.5	(0.5)	-	-	-	(0.5)	-
Other Income (Expense)	-	-	-	-	0.0	-	-	-	0.0	0.0	-	-	-	0.0	-
EBT	(0.4)	(3.3)	(12.1)	(20.8)	(10.2)	(10.0)	(10.1)	(8.8)	(39.1)	(10.5)	(11.0)	(11.8)	(12.7)	(45.9)	(57.7)
Tax Expense (Benefit)	-	-	-	-	-	-	-	-	-	(0.0)	-	-	-	(0.0)	-
Net Loss	(0.4)	(3.3)	(12.1)	(20.8)	(10.2)	(10.0)	(10.1)	(8.8)	(39.1)	(10.5)	(11.0)	(11.8)	(12.7)	(45.9)	(57.7)
Diluted EPS (Euros)	(0.17)	(0.59)	(1.10)	(0.89)	(0.44)	(0.43)	(0.43)	(0.38)	(1.68)	(0.45)	(0.46)	(0.48)	(0.51)	(1.90)	(1.33)
Weighted avg. shares outstanding	2.5	5.5	11.0	23.6	23.3	23.3	23.3	23.3	23.3	23.5	24.0	24.5	25.0	24.2	43.5

Source: SEC Filings and Leerink Partners Research

PRQR BS & CFS (€MM) - IFRS	2012	2013	2014	2015	1Q16	2Q16	3Q16	4Q16	2016	1Q17	2Q17E	3Q17E	4Q17E	2017E	2018E
Net Cash	0.2	0.7	109.9	90.0	80.3	71.0	61.3	53.5	53.5	46.3	35.8	24.5	12.3	12.3	106.6
Cash & Equivalents	0.2	4.1	112.7	94.9	85.5	76.3	64.9	59.2	59.2	52.1	41.6	28.4	16.2	16.2	108.5
Debt	-	3.5	2.8	4.8	5.1	5.3	3.6	5.7	5.7	5.8	5.8	3.9	3.9	3.9	1.9
Change in Cash	0.2	3.9	107.1	(17.8)	(9.4)	(9.2)	(11.4)	(5.7)	(35.7)	(7.1)	(10.5)	(13.2)	(12.2)	(43.0)	92.3
Cash Flow From Operations	(0.3)	(2.3)	(9.2)	(17.4)	(7.8)	(8.3)	(10.8)	(7.3)	(34.2)	(8.8)	(10.0)	(10.8)	(11.7)	(41.2)	(45.7)
Net Income	(0.4)	(3.3)	(12.1)	(20.8)	(10.2)	(10.0)	(10.1)	(8.8)	(39.1)	(10.5)	(11.0)	(11.8)	(12.7)	(45.9)	(57.7)
SOE	-	0.0	0.7	1.2	0.6	0.7	0.6	0.5	2.5	0.9	0.8	0.8	0.8	3.3	3.5
D&A	-	0.0	0.1	0.5	0.3	0.4	0.3	0.3	1.2	0.3	0.2	0.2	0.2	0.9	8.5
Other	0.1	0.9	2.1	1.7	1.5	0.6	(1.6)	0.7	1.2	0.5	-	-	-	0.5	-
Cash Flow From Investing	(0.0)	(0.1)	(0.7)	(1.3)	(0.5)	(1.6)	(0.4)	(0.0)	(2.5)	(0.0)	(0.5)	(0.5)	(0.5)	(1.5)	(10.0)
CapEx	-	(0.1)	(0.7)	(1.3)	(0.5)	(1.6)	(0.4)	(0.0)	(2.5)	(0.0)	(0.5)	(0.5)	(0.5)	(1.5)	(10.0)
Other	(0.0)	-	-	(0.0)	-	-	-	-	-	-	-	-	-	-	-
Cash Flow From Financing	0.6	6.3	117.1	1.7	0.2	(0.0)	-	0.2	0.4	2.2	-	(1.9)	-	0.2	148.1
Equity Issuance (Buyback)	0.5	3.0	118.1	0.0	-	-	-	-	-	2.2	-	(1.9)	-	2.2	150.0
Debt Issuance (Retirement)	0.1	3.3	(1.0)	1.3	-	-	-	0.2	0.2	-	-	(1.9)	-	(1.9)	(1.9)
Other	-	-	(0.0)	0.5	0.2	(0.0)	-	-	0.2	0.0	-	-	-	0.0	-
FX/Other	-	-	-	(0.8)	(1.3)	0.8	(0.2)	1.5	0.7	(0.4)	-	-	-	(0.4)	-

Source: SEC Filings and Leerink Partners Research

*Quarterly Financials Not Available for 2013; **Quarterly Financials Not Available for 1H14

PRQR DCF Analysis	2014	2015	2016	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	TV
CFO (€MM)	(9.2)	(17.4)	(34.2)	(41.2)	(45.7)	(75.2)	(85.1)	(100.1)	46.7	172.0	249.4	238.4	161.8	110.0	75.3	58.8	
CFI (€MM)	(0.7)	(1.3)	(2.5)	(1.5)	(10.0)	(15.0)	(20.0)	(20.0)	(20.0)	(20.0)	(20.0)	(20.0)	(20.0)	(20.0)	(20.0)	(20.0)	
Net Borrowing (€MM)	(1.0)	1.3	0.2	(1.9)	(1.9)	(1.9)	-	-	-	-	-	-	-	-	-	-	
Free Cash Flow to Equity (€MM)	(11.0)	(17.5)	(36.6)	(44.7)	(57.7)	(92.2)	(105.1)	(120.1)	26.7	152.0	229.4	218.4	141.8	90.0	55.3	38.8	380.4
Discount Periods	0	0	0	0	0.75	1.75	2.75	3.75	4.75	5.75	6.75	7.75	8.75	9.75	10.75	11.75	
Discounted FCFE (€MM)	-	-	-	(44.7)	(52.8)	(75.1)	(76.2)	(77.4)	15.3	77.6	104.2	88.3	51.0	28.8	15.7	9.8	96.3

Sum NPV FCFE	160.8
Net Cash 1Q17	46.3
FX: EUR/USD	1.10
Implied Mkt. Cap (\$MM)	\$ 227.3
PRQR Price Target	\$ 10

Discount Rate	12%
Terminal Growth Rate	2%
Diluted Shares Outstanding 1Q17	23.5

Source: Leerink Partners Research

Product	Event	Timing
QR-110	Initiate Phase Ib/II (LCA)	1H17
QR-010	Enrollment update	ECFS (Jun 7-10)
QR-010	Phase Ib MAD Data (CF)	Mid-2017
QR-010	Initiate Phase II (CF)	2018
QR-110	Phase Ib/II (LCA) Topline data	2018
QR-313	Initiate Phase I (DEB)	2018
QR-010	Phase II Data (CF)	2019
QR-010	Initiate Phase III (CF)	Mid-2019
QR-010	Complete Enrollment for CF	YE19
QR-010	Phase III Data (CF)	2H20
QR-010	NDA filing	1H21
QR-010	PDUFA Date	2H21

Source: SEC Filings and Leerink Partners Research

Disclosures Appendix

Analyst Certification

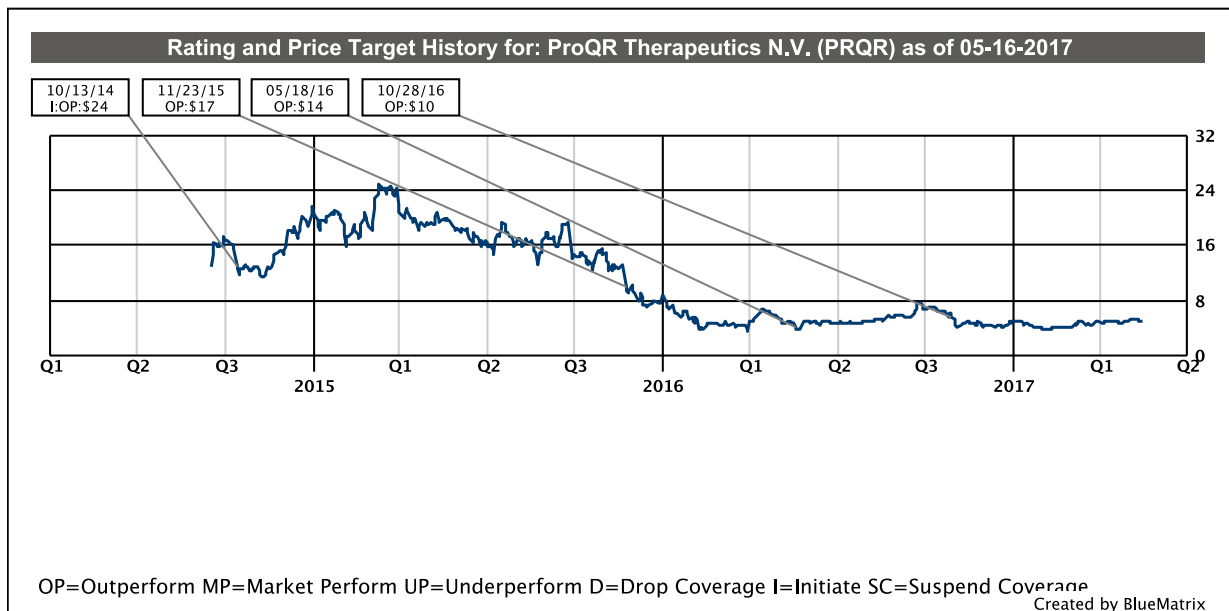
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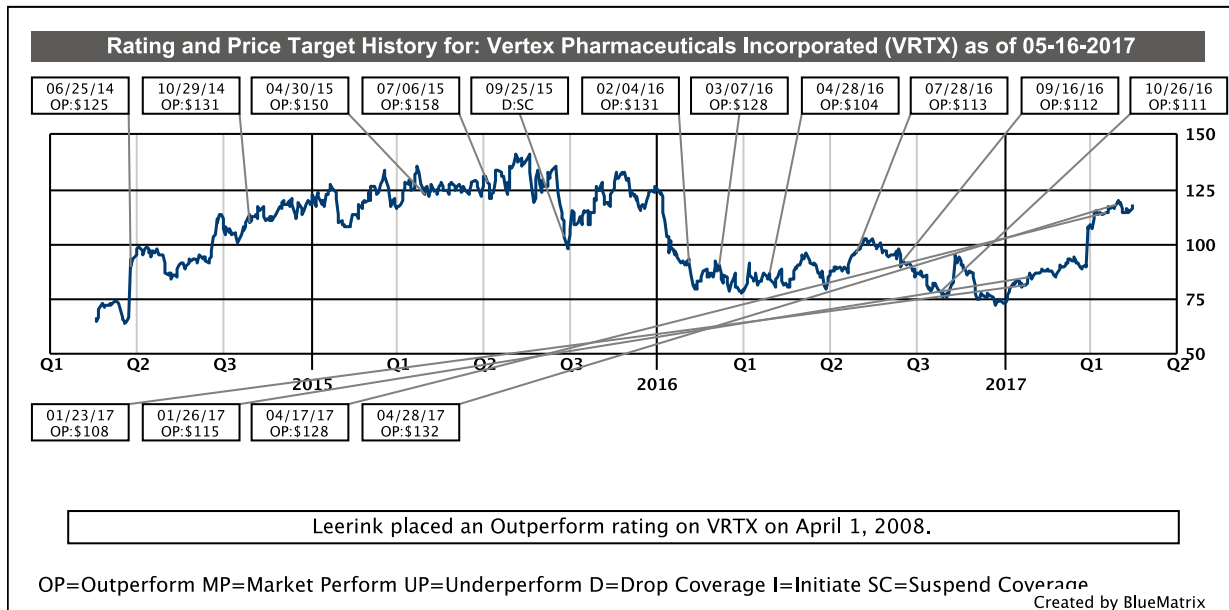
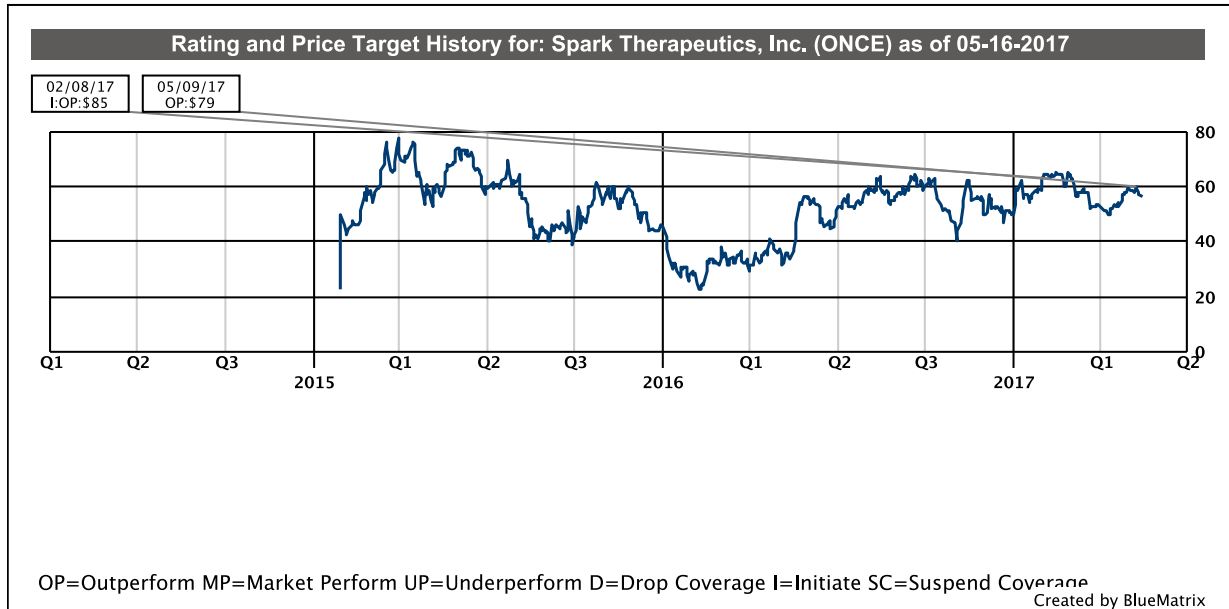
Valuation

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Risks to Valuation

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Distribution of Ratings/Investment Banking Services (IB) as of 03/31/17				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY [OP]	120	62.2	29	24.2
HOLD [MP]	73	37.8	4	5.5
SELL [UP]	0	0.0	0	0.0

Explanation of Ratings

Outperform (Buy): We expect this stock to outperform its benchmark over the next 12 months.

Market Perform (Hold/Neutral): We expect this stock to perform in line with its benchmark over the next 12 months.

Underperform (Sell): We expect this stock to underperform its benchmark over the next 12 months.

The degree of outperformance or underperformance required to warrant an Outperform or an Underperform rating should be commensurate with the risk profile of the company.

For the purposes of these definitions the relevant benchmark will be the S&P 600[®] Health Care Index for issuers with a market capitalization of less than \$2 billion and the S&P 500[®] Health Care Index for issuers with a market capitalization over \$2 billion.

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MEDACorp is a network of healthcare professionals, attorneys, physicians, key opinion leaders and other specialists accessed by Leerink and it provides information used by its analysts in preparing research.

Leerink Partners LLC makes a market in ProQR Therapeutics N.V., Spark Therapeutics, Inc. and Vertex Pharmaceuticals Incorporated.

Leerink placed an Outperform rating on VRTX on April 1, 2008.

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