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OUTPERFORM

Reason for report:

FLASH NOTE

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LEERINK

PROQR THERAPEUTICS N.V.

PRQR – Key Takeaways from LEERINK's Global Healthcare Conference

• **Bottom Line: At our annual Global Healthcare Conference, we hosted Smital Shah, CFO, from ProQR Therapeutics (PRQR).** Topics for discussion included the upcoming Ph.1b data for QR-010 in cystic fibrosis (CF) pts., which were preceded by the single-ascending dose (SAD) data at the North American Cystic Fibrosis Conference (NACFC) in 3Q16 (LINK, LINK). Just behind this lead drug candidate, ProQR is also advancing QR-110 targeting Leber's congenital amaurosis type 10 (LCA10), which is set to begin in 1H17. And an earlier stage candidate – QR-313 – in dystrophic epidermolysis bullosa (EB) continues to advance through the pipeline. Reiterate OP on PRQR.

• **By mid-2017, ProQR will report data from its Ph.1b multiple-ascending dose (MAD) cohort measuring both pulmonary and extra-pulmonary exploratory endpoints including forced expiratory volume in 1 second (FEV1), sweat chloride, CFQ-R, and weight gain, among others.** Mgmt. reminded investors that the intention of Ph.1b study is to evaluate safety and tolerability and identify an appropriate dose for subsequent Ph.2 and Ph.3 studies rather than demonstrating efficacy in a stat. sig. manner. And while pts. enrolled in Ph.1b study possessed a milder CF phenotype (i.e., ~70% FEV1 vs. 40-90% FEV1 range typically associated with drug trials), preclinical data suggest QR-010 is efficacious irrespective of disease severity. In the upcoming Ph.2 study, mgmt. plans on enrolling pts. with a more severe disease, which should result in a more pronounced benefit derived from QR-010. Lastly, whereas enrollment issues caused some setbacks in Ph.1b, mgmt. believes that the operational and logistical improvements should yield a smoother enrollment in future trials.

• **Other developments to keep an eye out for include QR-110 in LCA10 and QR-313 in dystrophic EB.** LCA10 is an ocular disease that QR-110 seeks to address by targeting and repairing an underlying gene – *CEP290*. ProQR intends on starting its first clinical trial in1H17 and mgmt. is putting final touches to a trial design that aims to recruit 12 pts. (6 adult and 6 pediatric) and testing 3 different doses administered 4x/yr. QR-313 is at an earlier stage and is being developed as a hydrogel for topical administration in dystrophic EB pts. As a first pass, QR-313 will target exon 73 within the dystrophic EB, but any data that are supportive of this mechanism can engender candidates targeting other mutations underlying EB.

• **Some notable catalysts looming on the horizon include** Ph.1b multiple-ascending dose (MAD) data for QR-010 in cystic fibrosis (CF) pts. by mid-2017 with Ph.2 possibly initiating in 2018 pending positive Ph.1b results. As for QR-110, initiation of a Ph.1 study is expected in Leber's congenital amaurosis type 10 (LCA10) in 1H17 with top-line data in 2018. Lastly, QR-313 study in dystrophic EB is slated for 2018.

Key Stats:	**(NASDAQ :PRQR)**
Sector:	**Biotechnology**
S&P 600 Health Care Index:	**1,867.94**
Price :	**$4.15**
52 Week High:	$8.70
52 Week Low:	$3.55
Shares Outstanding (mil):	23.3
Market Capitalization (mil):	$96.7

Completion: February 15, 2017, 9:51PM EDT.
Distribution: February 15, 2017, 9:51PM EDT.
Price: intra day price

Disclosures Appendix

Analyst Certification

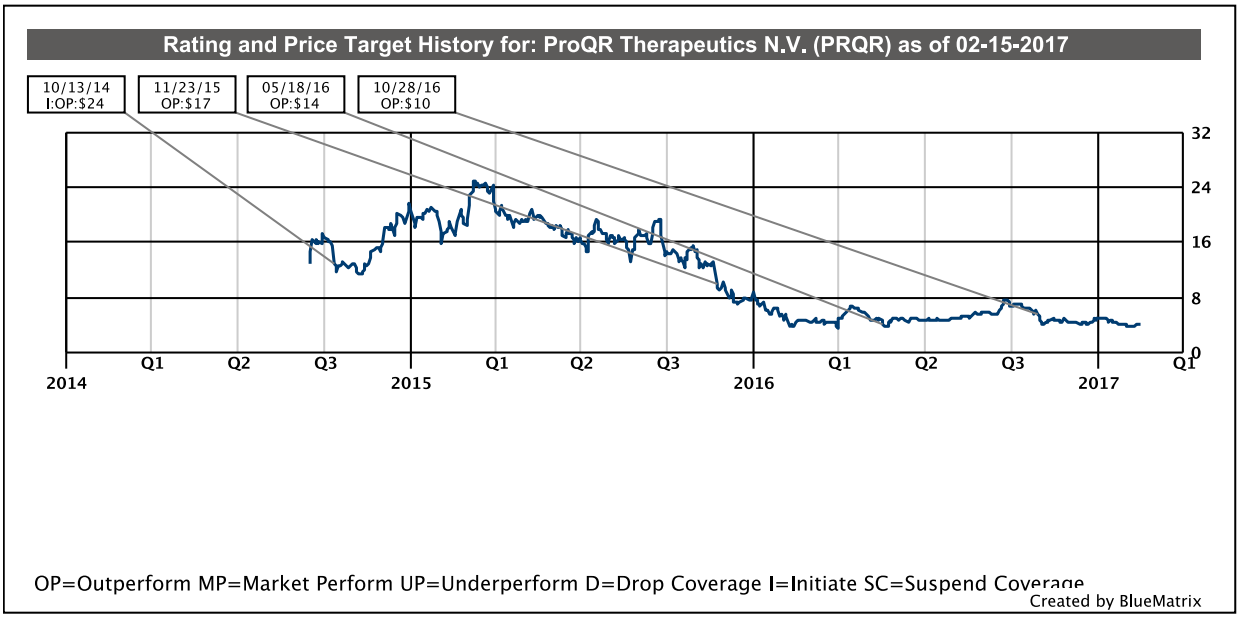
I, Joseph P. Schwartz, certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

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.



Distribution of Ratings/Investment Banking Services (IB) as of 12/31/16				
			IB Serv./Past 12 Mos.	
Rating	Count	Percent	Count	Percent
BUY [OP]	126	64.9	29	22.8
HOLD [MP]	68	35.1	3	4.5
SELL [UP]	0	0.00	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.

Important Disclosures

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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.

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