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OUTPERFORM

Reason for report:

FLASH NOTE

Joseph P. Schwartz
(617) 918-4575
Joseph.Schwartz@Leerink.com

Dae Gon Ha, Ph.D.
(617) 918-4093
Daegon.Ha@Leerink.com

LEERINK

PROQR THERAPEUTICS N.V.

PRQR - Key Takeaways from LEERINK'S Global Healthcare Conference today

• Bottom Line: At our conference today we spoke with CEO Daniel de Boer about key topics for the company in 2018. In particular, we touched upon several clinical programs including QR-010 in cystic fibrosis (CF), QR-110 in Leber's congenital amaurosis type 10 (LCA10), and QR-421a in Usher syndrome. We also briefly discussed their collaboration with Galapagos [GLPG, NR] involving the use of their fully-owned Axiomer Editing Oligonucleotides (EONs) technology to discover novel candidates in fibrotic diseases.

• On the tailwind of positive Ph.1b data, PRQR seeks collaborator(s) for future development of QR-010 in CF. Recall, last September the company released results from their placebo-controlled Ph.1b multiple ascending dose (MAD) study evaluating QR-010 in homozygous F508del CFTR mutated cystic fibrosis (CF) with patients demonstrating favorable tolerability and safety across all doses with improvements observed in exploratory endpoints, most notably, in patient symptoms according to the CFQ-R outcome (12.5mg dose, p=0.0072) (LINK). Moving forward, the company is seeking a collaborator(s) prior to advancing QR-010 into Ph.2. PRQR's vision is to position the product as a monotherapy at this time, but given the numerous products in the mkt. and/or under development, '010 may need to be combined with other agents. Interactions with regulators in US and EU have resulted in a clear path to a 12-week Ph.2 trial with FDA accepting the CFQ-R as a registrational endpoint.

• PRQR's Ph.1b/2 program for QR-110 in LCA 10, a rare genetic disorder resulting in blindness, continues on-track with interim 6 month data readout in 2H18 and 12 month data in 2H19. The main objective of their Ph.1b/2 trial is to evaluate the safety/tolerability, and efficacy of QR-110. The company plans on evaluating efficacy via functional ophthalmic endpoints such as visual acuity, full field stimulus testing (FST), pupillary light reflex (PLR), and mobility course and fixation stability. Mgmt. went on to state that QR-110 is differentiated from competing products due to its RNA, rather than DNA, editing mechanism of action. Mgmt. stated enrollment is on track with initial 6-month data in 2H18.

• QR-421a is first-in-class oligonucleotide therapy aimed at potentially preserving vision in Usher syndrome type 2A patients. Recently, PRQR procured $7.5M in funding from the Foundation Fighting Blindness for preclinical and clinical advancement of QR-421a (LINK), which mgmt. stated could fund approx. half of the Ph. 1/2 program. Mgmt. plans on initiating the initial trial by YE18 with preliminary data in 1H19. QR-421a will be administered intravitreally, similar to QR-110, and leverages exon-skipping mechanism to address the underlying mutations causing the disease. Another candidate in Usher syndrome, QR-411 (splice correction mechanism), is earlier in development and will advance as QR-421a data validates the approach in this disease.

• GLPG collaboration, which is centered on PRQR's fully owned Axiomer Editing Oligonucleotides (EONs) technology, looks to

Key Stats:	**(NASDAQ: PRQR)**
Sector:	**Biotechnology**
S&P 600 Health Care Index:	**2,472.44**
Price :	**$3.15**
52 Week High:	$6.90
52 Week Low:	$2.75
Shares Outstanding (mil):	25.3
Market Capitalization (mil):	79.7

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target fibrotic diseases. Recall, earlier this year PRQR entered into a collaboration with GLPG, which leverages PRQR's EONs technology to identify biological targets and design novel therapies for fibrotic diseases (LINK). The EONs platform allows the substitution of adenosine for inosine within the RNA, which is applicable to 20,000 disease causing mutations. At this time, neither the identification of biological targets nor the financial terms of the agreement have been disclosed so we look forward to future updates.

• **Mgmt. believes their cash position is sufficient into the back half of 2019 and are funded for near-term clinical readouts.**

Disclosures Appendix

Analyst Certification

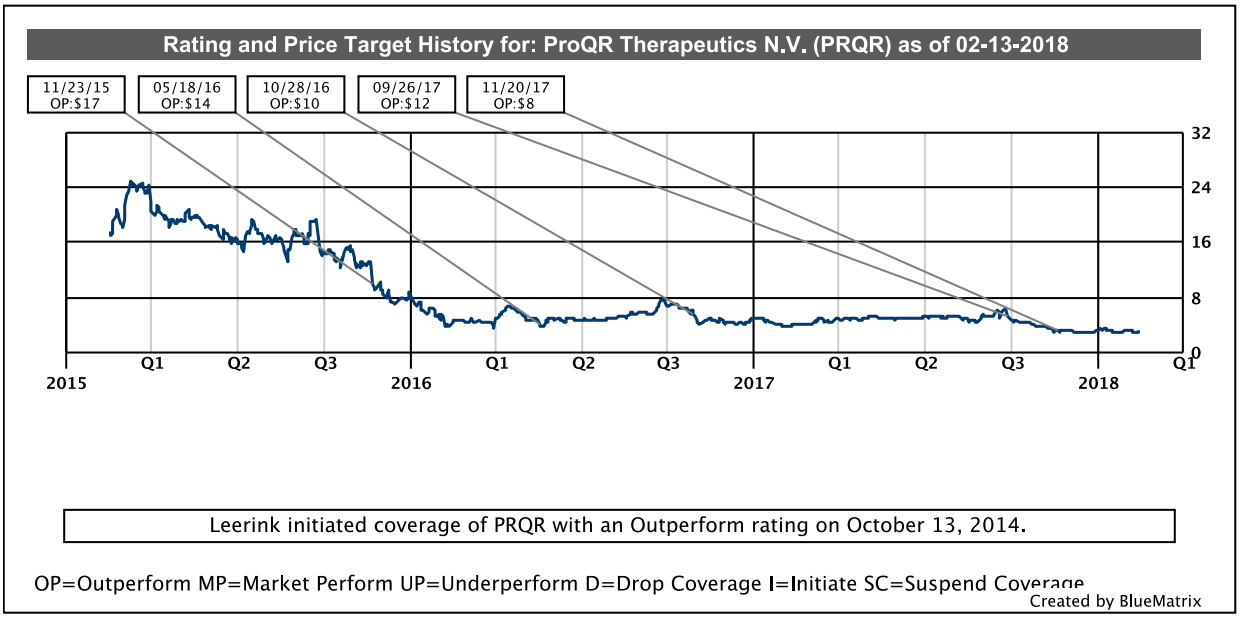
I, Joseph P. Schwartz, certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

Valuation

We derive a $8 price target for PRQR shares in 12 months based on a DCF with 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 30% 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 12/31/17				
			IB Serv./Past 12 Mos.	
Rating	Count	Percent	Count	Percent
BUY [OP]	130	69.9	48	36.9
HOLD [MP]	56	30.1	2	3.6
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.

Important Disclosures

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In the past 12 months, the Firm has received compensation for providing investment banking services to ProQR Therapeutics N.V. .

Leerink Partners LLC makes a market in ProQR Therapeutics N.V.

Leerink initiated coverage of PRQR with an Outperform rating on October 13, 2014.

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LEERINK PARTNERS LLC EQUITY RESEARCH

Director of Equity Research	**John L. Sullivan, CFA**	(617) 918-4875	john.sullivan@leerink.com
Associate Director of Research	**James Kelly**	(212) 277-6096	jim.kelly@leerink.com
Vice President	Jean Roberts, Ph.D.	(212) 277-6093	jean.roberts@leerink.com
Director of Therapeutic Research	**Geoffrey C. Porges, MBBS**	(212) 277-6092	geoffrey.porges@leerink.com
Major Pharmaceuticals	**Seamus Fernandez**	(617) 918-4011	seamus.fernandez@leerink.com
	Richard Goss	(617) 918-4059	richard.goss@leerink.com
	Etzer Darout, Ph.D.	(617) 918-4020	etzer.darout@leerink.com
Large Cap Biotechnology	**Geoffrey C. Porges, MBBS**	(212) 277-6092	geoffrey.porges@leerink.com
	Bradley Canino, CPA	(212) 277-6158	bradley.canino@leerink.com
Mid- and Small-Cap Biotechnology	**Joseph P. Schwartz**	(617) 918-4575	joseph.schwartz@leerink.com
	Michael Schmidt, Ph.D.	(617) 918-4588	michael.schmidt@leerink.com
	Paul Matteis	(617) 918-4585	paul.matties@leerink.com
	Benjamin Burnett, Ph.D.	(212) 277-6071	benjamin.burnett@leerink.com
	Jonathan Chang, Ph.D., CFA	(617) 918-4015	jonathan.chang@leerink.com
	Dae Gon Ha, Ph.D.	(617) 918-4093	daegon.ha@leerink.com
	Varun Kumar, Ph.D.	(617) 918-4518	varun.kumar@leerink.com
	Jeffrey Lin, Ph.D.	(617) 918-4838	jeffrey.lin@leerink.com
Specialty Pharmaceuticals & Generics	**Ami Fadia**	(212) 277-6047	ami.fadia@leerink.com
Life Science Tools & Diagnostics	**Puneet Souda**	(212) 277-6091	puneet.souda@leerink.com
	Kai Wang, CFA	(212) 277-6066	kai.wang@leerink.com
Medical Devices, Cardiology	**Danielle Antalffy**	(212) 277-6044	danielle.antalffy@leerink.com
	Rebecca Wang	(212) 277-6087	rebecca.wang@leerink.com
Medical Devices, Orthopedics	**Richard Newitter**	(212) 277-6088	richard.newitter@leerink.com
	Jaime L. Morgan	(212) 277-6073	jaime.morgan@leerink.com
Healthcare Services, Managed Care & Facilities	**Ana Gupte, Ph.D.**	(212) 277-6040	ana.gupte@leerink.com
	John Sourbeer	(212) 277-6139	john.sourbeer@leerink.com
Healthcare Technology & Distribution, Digital Health	**David Larsen, CFA**	(617) 918-4502	david.larsen@leerink.com
	Jonathan McGraw Bentley	(617) 918-4887	jonathan.bentley@leerink.com
	Matt Dellelo, CFA	(617) 918-4812	matt.dellelo@leerink.com
Sr. Editor/Supervisory Analyst	Mary Ellen Eagan, CFA	(617) 918-4837	maryellen.eagan@leerink.com
Supervisory Analysts	Randy Brougher		randy.brougher@leerink.com
	Robert Egan		bob.egan@leerink.com
	Amy N. Sonne		amy.sonne@leerink.com
Editorial Associate	Emily Singletary	(212) 277-6115	emily.singletary@leerink.com

BOSTON
One Federal St., 37th Fl.
Boston, MA 02110
(800) 808-7525

NEW YORK
299 Park Ave., 21st Fl.
New York, NY 10171
(800) 778-1653

CHARLOTTE
227 West Trade St., Ste. 2050
Charlotte, NC 28202
(704) 969-8944

SAN FRANCISCO
255 California St., 12th Fl.
San Francisco, CA 94111
(415) 905-7200