

Reason for report:
FLASH NOTE

PROQR THERAPEUTICS N.V.

A Sight for Sore Eyes/Funding "Usher's" in New Approach for Rare Ocular Disease

- **Bottom Line:** Today, PRQR issued a press release highlighting a collaboration with the Foundation Fighting Blindness to develop PRQR's QR-421a candidate for Usher syndrome type 2A, a genetic condition resulting in progressive vision loss. Under the terms of the agreement, the Foundation Fighting Blindness will provide milestone-based co-funding of up to \$7.5M paid to PRQR for preclinical and clinical advancement of QR-421a. This opportunity represents the second collaborative effort reported by the company in just over a month's time ([LINK](#)), which we find encouraging for PRQR as they continue to generate momentum around their novel RNA-based therapeutic approach. Currently, there are no therapies for Usher syndrome type 2A, representing a potential first-to-market opportunity and their second clinical program for rare ocular disease ([LINK](#)). The company expects QR-421a to advance toward the clinic in 2018, with a clinical readout in 2019.

- **QR-421a is first-in-class oligonucleotide capable of correcting the mutated USH2A protein and potentially preserving vision in Usher syndrome type 2A patients.** Briefly, Ushers syndrome is a rare genetic disease and a leading cause of combined deafness and blindness, with Ushers syndrome type 2 (USH2) representing the most common form of the disease. A mutation in exon 13 of the USH2A gene results in production of dysfunctional usherin protein, which manifests as progressive vision loss known as retinitis pigmentosa (RP). QR-421a is an in-licensed exon skipping technology from Radboud University Medical Center in the Netherlands, whose mechanism of action is aimed at treating the underlying cause of RP by skipping exon 13 from the USH2A mRNA, thereby allowing production of shorted, but functional Usherin protein in the retina. Thus far, the preclinical data have demonstrated that retinal exon 13 skipping resulted in restoration of the mRNA profile and USH2A protein, as well as production of functional usherin protein in zebrafish. Meanwhile, as evidenced by PRQR's lead rare ocular disease program, QR-110 for Leber's congenital amaurosis 10 (LCA10), currently in Ph. 1/2, the company is experienced in successfully designing IND enabling studies that meet regulatory requirements necessary for future clinical development.

- **Potentially, ~12,000 patients can be targeted in the Western world with QR-421a, a market 6x the size of their \$500M LCA10 program.** Overall, QR-421a is just 1 of 3 candidates the company has in the works for Usher syndrome, which comprises 3 of the 5 disclosed products in their ophthalmology franchise. Currently, we estimate the company's lead ocular program in LCA10, targeting ~2,000 pts., to represent a ~\$500M market ([LINK](#)). However, based on the unmet clinical need, the premium pricing for ultra orphan drugs, and a target population of ~12,000 pts., there could potentially be the same opportunity in the Usher's syndrome market. At this time we do not model the QR-421a program due to its preclinical status.

Key Stats: (NASDAQ: PRQR)

Sector:	Biotechnology
S&P 600 Health Care Index:	2,442.69
Price :	\$2.90
52 Week High:	\$6.90
52 Week Low:	\$2.75
Shares Outstanding (mil):	25.3
Market Capitalization (mil):	73.4

Completion: February 12, 2018, 9:44AM EDT.
Distribution: February 12, 2018, 9:44AM EDT.

Disclosures Appendix

Analyst Certification

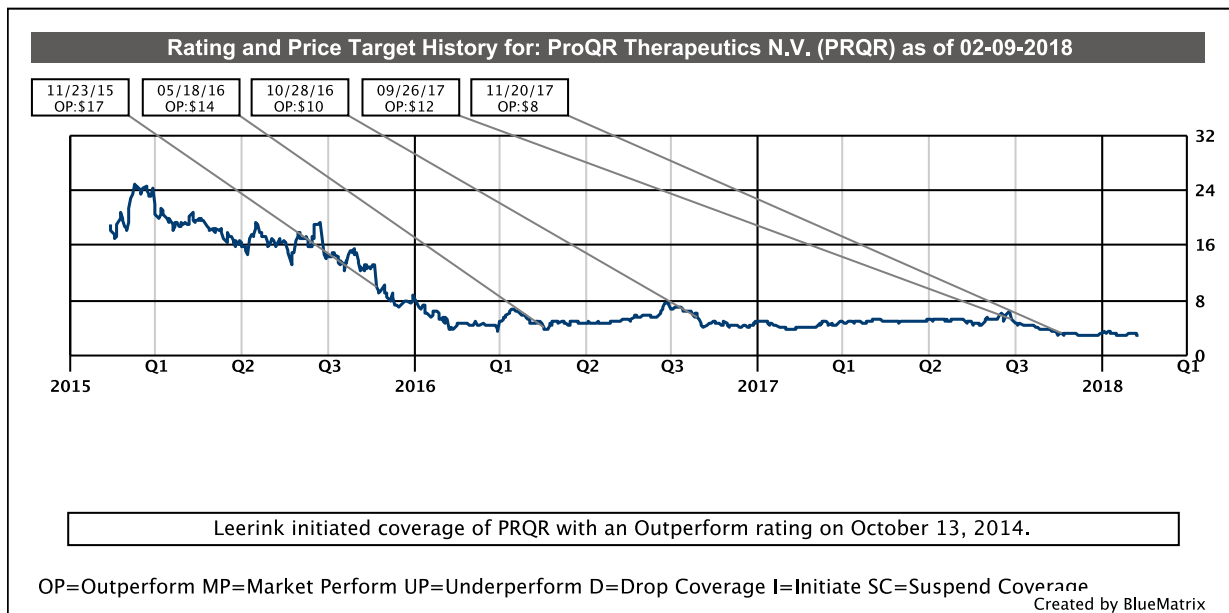
I, Joseph P. Schwartz, certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

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				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			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

This information (including, but not limited to, prices, quotes and statistics) has been obtained from sources that we believe reliable, but we do not represent that it is accurate or complete and it should not be relied upon as such. All information is subject to change without notice. This is provided for information purposes only and should not be regarded as an offer to sell or as a solicitation of an offer to buy any product to which this information relates. The Firm, its officers, directors, employees, proprietary accounts and affiliates may have a position, long or short, in the securities referred to in this report, and/or other related securities, and from time to time may increase or decrease the position or express a view that is contrary to that contained in this report. The Firm's salespeople, traders and other professionals may provide oral or written market commentary or trading strategies that are contrary to opinions expressed in this report. The Firm's proprietary accounts may make investment decisions that are inconsistent with the opinions expressed in this report. The past performance of securities does not guarantee or predict future performance. Transaction strategies described herein may not be suitable for all investors. Additional information is available upon request by contacting the Editorial Department at One Federal Street, 37th Floor, Boston, MA 02110.

Like all Firm employees, analysts receive compensation that is impacted by, among other factors, overall firm profitability, which includes revenues from, among other business units, Institutional Equities, and Investment Banking. Analysts, however, are not compensated for a specific investment banking services transaction or contributions to the Firm's investment banking activities.

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.

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.

Member FINRA/SIPC. ©2018 Leerink Partners LLC. All rights reserved. This document may not be reproduced or circulated without our written authority.

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