

Reason for report:

FLASH NOTE

PROQR THERAPEUTICS N.V.

Galapagos Collaboration Adds Validation to Novel RNA Editing Platform

• **Bottom Line:** Today ProQR announced plans to leverage its fully-owned Axiomer Editing Oligonucleotides (EONs) technology in a collaboration with Galapagos [GLPG,NR], a clinical-stage biotechnology company with a long track record of clinical development, to identify biological targets and design therapies for fibrotic diseases. This arrangement is a first for ProQR, which adds value to both companies by combining proven leadership in clinical development with a novel RNA editing platform that may have broad applicability in the field of fibrosis. In addition, our conversation with management provided updates on ProQR's clinical pipeline.

• **Collaboration with GLPG positions ProQR favorably in competitive fibrosis pipeline.** Historically, ProQR's lead clinical program in cystic fibrosis (CF) had taken longer than expected to generate data ([LINK](#)). We see the announcement today as a positive for ProQR as it provides the opportunity to pair its novel and fully-owned EONs platform with a company which is focused on progressing clinical programs, as evidenced by GLPG's ongoing clinical suite, which contains three Ph.3 and eleven Ph.2 trials. Though the exact identities of the biological targets the companies plan to go after have not been disclosed, the overall aim of their collaboration is limited to diseases that are fibrotic in nature. Our discussion with ProQR's management clarified that CF is not a target indication, as GLPG's sole collaborator in CF is [ABBV,OP]. The attraction of ProQR's Axiomer EON platform comes from the ability to substitute adenosine within the RNA with inosine.

• **Financial details about the collaboration have not been released, but meaningful progress from their partnership could potentially set up future revenue streams for ProQR in the form of licensing fees and/or a novel therapeutic product(s).** Our takeaway is that contingent upon a successful collaboration, ProQR could benefit from an expanded pipeline, and we view the interest from GLPG as a good validation for ProQR's RNA editing technology.

• **Four major programs to look out for in 2018 and early 2019 from ProQR.** Investors will recall that ProQR has developed a diverse rare disease clinical portfolio due to the broad applicability of its RNA repair platform. 2018 is a big year for ProQR, as the company plans on reporting top-line data from its Ph.1b/2 Leber's congenital amaurosis 10 study in mid-late '18 and initiate/report Ph.1 data from its dystrophic epidermolysis bullosa (DEB) trial in '18-'19. In addition, the company stated plans to initiate its Usher syndrome program at YE18 with a full data readout in 1H19. As of now the company is awaiting a partnership to initiate its Ph.2 trial in CF, but talks with regulators have been fruitful with the acceptance of cystic fibrosis questionnaire-revised (CFQ-R) as a primary endpoint in the US, a parameter ProQR has shown efficacy that is on par with Vertex's (VRTX [OP]) Kalydeco. Successful advancement in all of these programs, along with the future program(s) with GLPG, should instill investor confidence in the company's ability to execute

Key Stats:

(NASDAQ: PRQR)

Sector:	Biotechnology
S&P 600 Health Care Index:	2,387.71
Price :	\$3.45
52 Week High:	\$6.90
52 Week Low:	\$2.75
Shares Outstanding (mil):	25.3
Market Capitalization (mil):	87.3

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and provide enthusiasm around its RNA repair technology as a viable approach to genetic diseases.

Disclosures Appendix

Analyst Certification

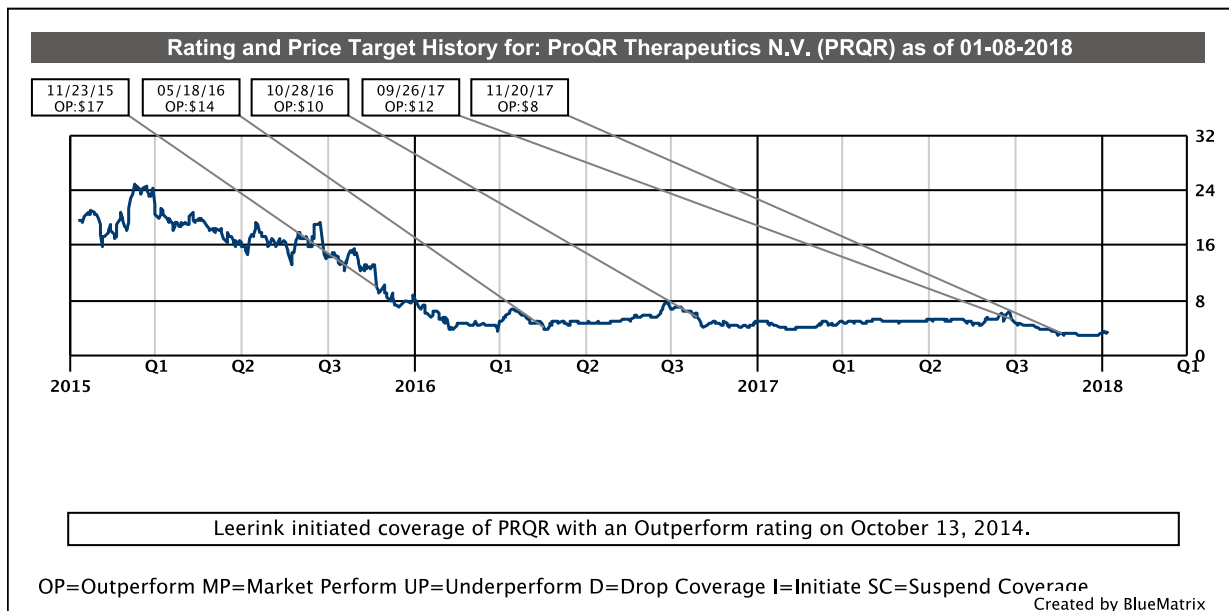
I, Joseph P. Schwartz, certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

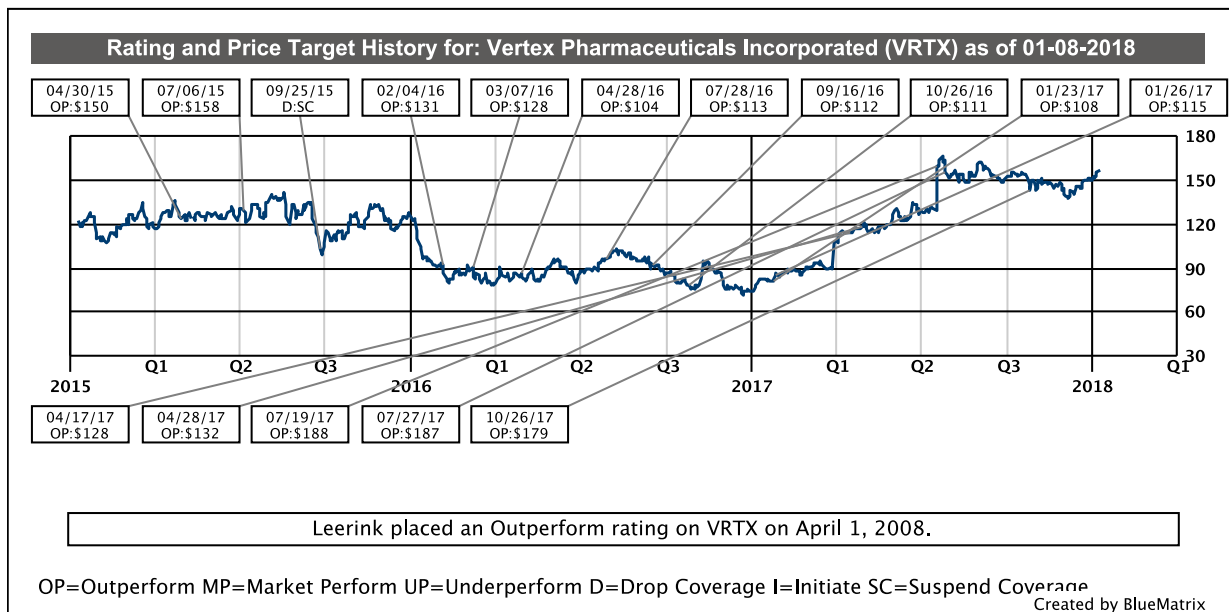
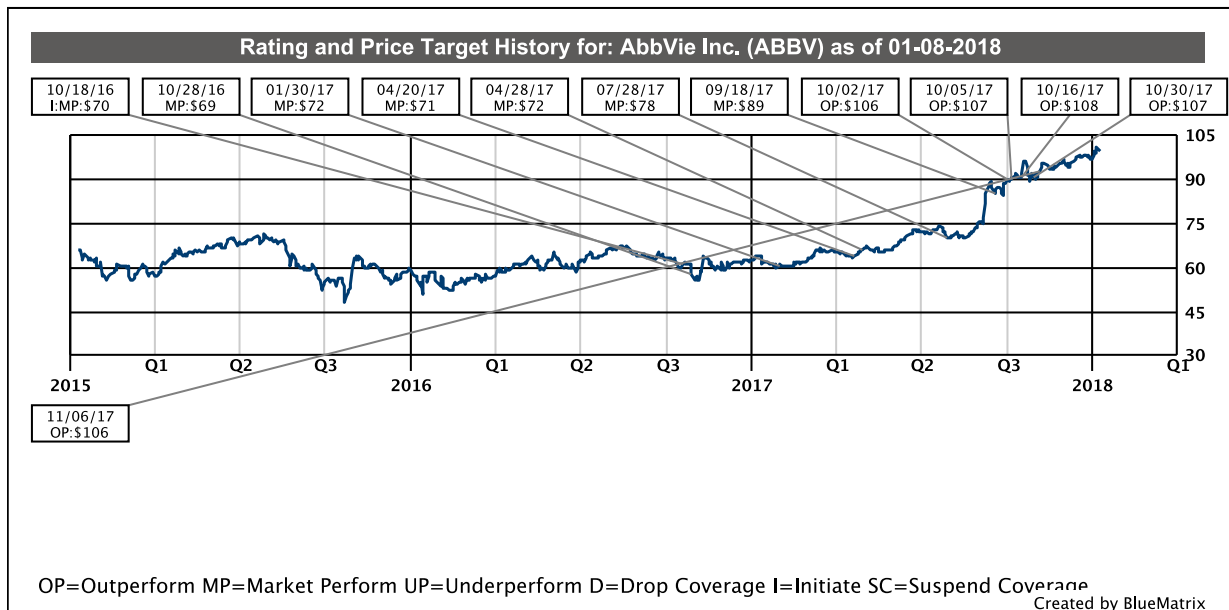
Valuation

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 09/30/17				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY [OP]	122	67.4	40	32.8
HOLD [MP]	59	32.6	4	6.8
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.

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., AbbVie Inc. and Vertex Pharmaceuticals Incorporated.

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

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

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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
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
	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