

ProQR Therapeutics N.V.

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BUY (PRQR, \$4.30)

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Reports 1Q18 – QR-110 Clinical Trial Enrollment Update; Ophthalmic Pipeline Progressing As Planned: Reiterate BUY/\$10 TP
What Happened:

- This morning, ProQR announced 1Q18 results and provided clinical and operational updates, reporting a cash position of €38M, enough to fund operations through the end of 1Q19, as per our projections.
- ProQR reported that eight out of twelve patients have been enrolled in the Phase 1/2 trial of QR-110 for Leber congenital amaurosis type 10 (LCA 10), a congenital eye disease for which there are no approved treatments. Interim safety and efficacy results are expected in 2H18 and topline final data are expected in 2019.
- The company also announced it has entered into a partnership with Foundation Fighting Blindness (FFB) which will allow ProQR to receive up to \$7.5M in funding for pre-clinical and clinical development of QR-421a for Usher syndrome type 2A targeting mutations in exon 13. Data from the QR-421a clinical study for Usher syndrome will be released within the next twelve months.
- ProQR and Galapagos N.V. have entered a research collaboration in which ProQR's Axiomer technology will be applied to certain fibrosis targets. ProQR plans to use the Axiomer platform for additional licensing and other strategic relationships in select therapeutic areas.

Our Take:

- ProQR's drug candidates are unified by the fact that they are all RNA-based molecules that exert their effects on mutated mRNA such that a wild-type or at least functionally correct protein can be made from the corrected mRNA. ProQR's drugs have either been shown clinically, or are expected, to have a highly favorable safety profile. ProQR circumvents the historical problem of RNA-based drugs being difficult to usefully give systemically by focusing solely on diseases that can be addressed with local drug administration. This is exemplified by ProQR's focus on ophthalmic diseases that can be treated with injections directly into the eye, which delivers the drug to the target area and minimizes the chances for systemic toxicity.
- ProQR initially focused on CF with eluforsen (QR-010), but that market has become increasingly competitive such that eluforsen will proceed into Phase 2 once ProQR finds a partner for the program, which is expected later in 2018. A larger partner for eluforsen would give the drug a far better chance in a competitive marketplace, despite its apparent differentiating qualities. ProQR instead will focus on orphan ophthalmic markets where it can realistically own the market given the absence of competition in the disease markets it is targeting.
- We project 2H18 to deliver 2 important catalysts for ProQR, interim Phase 1b/2 data for QR-110 in LCA 10, and Phase 1 data for QR-313 in dystrophic epidermolysis bullosa (DEB). We also look forward to the CF program resuming Phase 2, likely in 2H18, as well as QR-421a entering the clinic in Usher syndrome type 2 in 2018.



Risks

- **Clinical risk.** ProQR's clinical stage products could fail to deliver statistically significant results in late-stage clinical trials, substantially reducing the value of ProQR's product candidates and therefore our target price.
- **Regulatory risk.** Even if successful in the clinic, ProQR's products could fail to be approved by domestic and/or foreign regulatory bodies, which would reduce ProQR's value and therefore our target price.
- **Financing risk.** ProQR could need capital to fund its operations, should its marketed or soon to be marketed products fail to deliver the anticipated revenue, and such financing may not occur or it could be substantially dilutive to existing investors.
- **Competitive risk.** For any approved ProQR products, they may not be well adopted in a competitive marketplace, which would adversely affect ProQR's value and therefore our target price.
- **High stock price volatility.** This issue is common among small-cap biotechnology companies with relatively low trading volumes.

Valuation/Target Price

We derive our target price of \$10 through a DCF analysis, assuming a 25% discount rate that is applied to all cash flows and the terminal value, which is based on a 5 multiple of our projected 2030 EBITDA of \$399 million.

ProQR Therapeutics N.V.																								
Income Statement																								
Fiscal Year ends December																								
(in €000, except per share items)																								
	2016A	1Q17	2Q17	3Q17	4Q17	2017A	1Q18A	2Q18E	3Q18E	4Q18E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	
eluforsen US royalty																21,000	55,692	82,709	112,806	146,257	169,254	209,468	238,076	252,551
eluforsen EU royalty																14,063	35,859	51,207	67,155	83,719	100,920	118,775	129,227	
QR-110 US sales																12,500	31,518	46,910	63,779	79,189	95,995	110,937	127,146	
QR-110 EU royalty																	2,000	4,896	7,075	9,339	11,257	13,249	14,865	
Other Income	1,828	393	256	326	511	1,486	499	250	250	250	1,249	1,249	1,249	1,249										
Total Revenue	1,828	393	256	326	511	1,486	499	250	250	250	1,249	1,249	1,249	1,249	21,000	82,255	152,087	215,819	284,265	341,500	417,640	481,037	523,790	
Cost of revenue						-										1,250	3,152	4,691	6,378	7,919	9,599	11,094	12,715	
R&D	31,923	8,030	7,552	7,226	8,345	31,153	7,685	8,069	8,473	8,896	33,123	39,748	47,698	54,852	60,337	66,371	69,690	73,174	74,638	75,384	76,138	76,899	77,668	
G&A	9,478	2,304	2,892	2,753	2,891	10,840	2,672	2,752	2,835	2,920	11,179	12,297	13,526	14,879	16,367	27,823	29,214	30,675	32,209	32,853	33,510	34,180	34,864	
Total Operating Expenses	41,401	10,334	10,444	9,979	11,236	41,993	10,357	10,821	11,307	11,816	44,302	52,044	61,224	69,731	76,704	95,444	102,056	108,540	113,225	116,156	119,248	122,173	125,247	
Operating income	(39,573)	(9,941)	(10,188)	(9,653)	(10,725)	(40,507)	(9,858)	(10,571)	(11,057)	(11,566)	(43,053)	(50,795)	(59,975)	(68,482)	(55,704)	(13,190)	50,031	107,279	171,040	225,344	298,393	358,864	398,543	
Finance income (expense)	470	(537)	(1,184)	(868)	(586)	(3,175)	(859)				(859)	(773)	(696)	(626)	(564)									
Net income (pretax)	(39,103)	(10,478)	(11,372)	(10,521)	(11,311)	(43,682)	(10,717)	(10,571)	(11,057)	(11,566)	(43,912)	(51,569)	(60,671)	(69,108)	(56,268)	(13,190)	50,031	107,279	171,040	225,344	298,393	358,864	398,543	
Income tax expense (benefit)						-															15,774	35,807	53,830	83,694
Net income	(39,103)	(10,478)	(11,372)	(10,521)	(11,311)	(43,682)	(10,717)	(10,571)	(11,057)	(11,566)	(43,912)	(51,569)	(60,671)	(69,108)	(56,268)	(13,190)	50,031	107,279	171,040	209,570	262,586	305,034	314,849	
EPS basic	(1.67)	(0.45)	(0.47)	(0.42)	(0.39)	(1.72)	(0.34)	(0.33)	(0.34)	(0.35)	(1.36)	(1.49)	(1.67)	(1.82)	(1.41)	(0.31)	1.14	2.32	3.52	4.11	4.90	5.42	5.33	
EPS diluted	(1.67)	(0.45)	(0.47)	(0.42)	(0.39)	(1.72)	(0.34)	(0.33)	(0.34)	(0.35)	(1.36)	(1.49)	(1.67)	(1.82)	(1.41)	(0.31)	1.06	2.18	3.32	3.88	4.64	5.15	5.07	
Basic shares outstanding	23,347	23,473	23,991	25,282	28,695	25,360	31,921	32,240	32,563	32,888	32,403	34,533	36,259	38,072	39,976	41,975	44,073	46,277	48,591	51,020	53,571	56,250	59,063	
Diluted shares outstanding	23,347	23,473	23,991	25,282	28,695	25,360	31,921	32,240	32,563	32,888	32,403	34,533	36,259	38,072	39,976	41,975	44,073	46,277	48,591	51,020	53,571	56,250	59,250	62,063

Source: Company reports, Opus National Capital Markets estimates

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NR: Not Rated

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Charts – PRQR



Source: Big Charts

PGNX	Date	Rating	Price Target
Initiation	March 6, 2018	BUY	\$10