

## ProQR Therapeutics N.V. (PRQR)

Dramatic Gain of Vision for LCA10 Patients in First QR-110 Study

### MARKET OUTPERFORM

Price: \$7.95  
Price Target: \$25.00

### INVESTMENT HIGHLIGHTS

- **Proof-of-concept study in LCA10 hits on multiple efficacy measures enabling Phase 3 to start in 1H19; we reiterate our Market Outperform rating and raise our risk-adjusted, DCF-derived price target on ProQR Therapeutics to \$25 from \$20.**
- We are excited by the first look at data of QR-110 in patients with LCA10 – a rare retinal dystrophy leading to childhood blindness. In particular, we are impressed with the concordance of the data showing a rapid and sustained benefit on every metric of vision assessed, especially the change in visual acuity, which we viewed as a high hurdle (see our preview [here](#)).
- The logMAR scale is used to measure vision improvement (negative values) and vision loss (positive values) on the commonly used ETDRS letter chart where the clinically meaningful threshold is 0.3 logMAR (3 lines/15 letters). This is important as it is a commonly used regulatory metric for efficacy, and in this study, over 60% of patients responded with a mean response of 0.67 logMAR, well above the clinically meaningful threshold. Spark Therapeutics (ONCE, NC) used a novel endpoint for Luxturna approval utilizing a mobility test and the FDA determined two levels to be clinically relevant on this instrument. Here, QR-110 also showed positive data (Figure 1).
- Based on the data, the FDA has agreed to the Phase 2/3 ILLUMINATE study as a next step that should begin in 1H19, followed closely by a study in children under age six years, which could enable even greater therapeutic benefit. We think the quick expansion into the younger population underscores the clean safety profile of QR-110.
- We see LCA10 as a >\$700M opportunity, which may or may not be shared by the only other competitor we are aware of: Editas Medicines' (EDIT, CIT) gene editing approach.
- Following these data, we raise our probability of success (POS) for QR-110 to 60% from 20% and our POS for QR-421a in Usher's syndrome slightly to 20% from 10%, as we see some read-through from the optic cup model from this study. Based on the accelerated Phase 3 start, we now model a U.S. launch of QR-110 in 2021 (2022 previously). We also remove PRQR's cystic fibrosis program, eluforsen, from our model. QR-110 now accounts for ~\$13/share of our \$25 target (\$5/share of our previous \$20 target).
- Management is hosting a conference call this morning at 8:15 a.m. ET where we will look for additional color on the data. We are hosting a call with ophthalmic expert Dr. Mark Pennesi at 10:00 a.m. ET to discuss LCA10 and the QR-110 data. Please contact your JMP Securities sales representative for dial-in information.

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### MARKET DATA

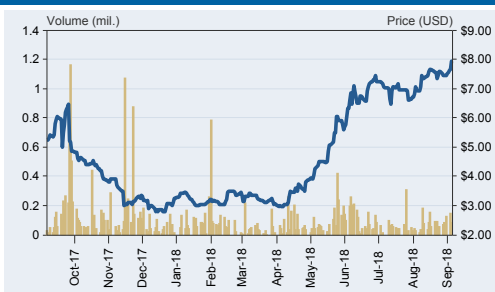
Price	\$7.95
52-Week Range:	\$2.75 - \$7.95
Shares Out. (M):	31.9
Market Cap (\$M):	\$253.6
Average Daily Vol. (000):	87.0
Cash (M):	\$39
LT Debt (M):	\$0

Source: Thomson Reuters and JMP Securities LLC

FY DEC		2017A	2018E	2019E
Revenue (\$M)	1Q	\$0.4	\$0.6A	--
	2Q	\$0.3	\$1.1A	--
	3Q	\$0.4	\$1.0	--
	4Q	\$0.6	\$1.0	--
	<b>FY</b>	<b>\$1.7</b>	<b>\$3.7</b>	<b>\$4.5</b>
EPS	1Q	(\$0.48)	(\$0.42)A	--
	2Q	(\$0.54)	(\$0.27)A	--
	3Q	(\$0.50)	(\$0.30)	--
	4Q	(\$0.47)	(\$0.32)	--
	<b>FY</b>	<b>(\$1.98)</b>	<b>(\$1.32)</b>	<b>(\$1.17)</b>

Source: Company reports and JMP Securities LLC

### STOCK PRICE PERFORMANCE



**FIGURE 1. QR-110 Visual Improvement**

	Clinically Meaningful Change	Mean Change at 3 Months	
		Treated Eye	Untreated Eye
Visual Acuity (LogMAR)*	$\geq -0.3$	-0.67	+0.02
Mobility Course (Level)	$\geq 2$	2.57	1.36

\*Negative value=improvement; positive value=worsening

Source: Company Reports

**FIGURE 2. Risk-Adjusted, Discounted Cash Flow Analysis, 2018E-2030E**

Discounted Cash Flow Valuation		2018	2019	2020	2021	2022	2023	2024	2025	2026	2027	2028	2029	2030
<b>Revenues ('000)</b>		<b>2,000</b>	<b>4,500</b>	-	<b>23,052</b>	<b>125,363</b>	<b>245,186</b>	<b>513,085</b>	<b>893,964</b>	<b>1,294,711</b>	<b>1,330,608</b>	<b>1,367,753</b>	<b>1,406,185</b>	<b>1,445,943</b>
	QR-110 - LCA	-	-	-	23,052	115,262	157,034	249,733	322,496	361,910	367,982	374,258	380,746	387,449
	QR-313 - DEB	-	-	-	-	10,101	23,486	45,166	74,752	89,787	92,627	95,566	98,605	101,749
	QR-421a - Usher	-	-	-	-	-	64,667	218,186	496,716	843,013	869,999	897,929	926,834	956,745
	Other Revenue	2,000	4,500	-	-	-	-	-	-	-	-	-	-	-
<b>Operating expenses ('000)</b>		<b>22,300</b>	<b>55,200</b>	<b>78,600</b>	<b>148,500</b>	<b>211,604</b>	<b>237,413</b>	<b>273,817</b>	<b>329,704</b>	<b>392,985</b>	<b>406,920</b>	<b>421,443</b>	<b>436,580</b>	<b>452,356</b>
	COGS	-	-	-	-	18,804	36,778	76,963	134,095	194,207	199,591	205,163	210,928	216,892
	R&D	15,500	38,400	57,600	72,000	80,000	60,000	30,000	15,000	10,000	10,000	10,000	10,000	10,000
	SG&A	6,800	16,800	21,000	76,500	112,800	140,635	166,854	180,610	188,778	197,329	206,281	215,652	225,465
<b>Operating Income ('000)</b>		<b>(20,300)</b>	<b>(50,700)</b>	<b>(78,600)</b>	<b>(125,448)</b>	<b>(86,241)</b>	<b>7,773</b>	<b>239,268</b>	<b>564,260</b>	<b>901,726</b>	<b>923,688</b>	<b>946,309</b>	<b>969,605</b>	<b>993,587</b>
<b>Weighted Risk</b>	<b>POS</b>	<b>100%</b>	<b>100%</b>	<b>100%</b>	<b>100%</b>	<b>56%</b>	<b>45%</b>	<b>39%</b>	<b>34%</b>	<b>31%</b>	<b>31%</b>	<b>31%</b>	<b>30%</b>	<b>30%</b>
	QR-110 - LCA	40%	40%	40%	40%	55%	38%	29%	22%	17%	17%	16%	16%	16%
	QR-313 - DEB	30%	30%	30%	30%	1%	1%	1%	1%	1%	1%	1%	1%	1%
	QR-421a - Usher	30%	30%	30%	30%	0%	5%	9%	11%	13%	13%	13%	13%	13%
<b>Tax</b>		<b>0%</b>	<b>0%</b>	<b>0%</b>	<b>0%</b>	<b>0%</b>	<b>0%</b>	<b>5%</b>	<b>10%</b>	<b>15%</b>	<b>20%</b>	<b>20%</b>	<b>20%</b>	<b>20%</b>
<b>Risk adjusted Net Income</b>		<b>(20,300)</b>	<b>(50,700)</b>	<b>(78,600)</b>	<b>(125,448)</b>	<b>(48,618)</b>	<b>3,509</b>	<b>88,715</b>	<b>172,724</b>	<b>236,336</b>	<b>226,961</b>	<b>231,625</b>	<b>236,428</b>	<b>241,373</b>
	QR-110 - LCA	(8,120)	(20,280)	(31,440)	(50,179)	(47,576)	2,987	66,381	109,920	128,550	122,615	124,291	126,017	127,794
	QR-313 - DEB	(6,090)	(15,210)	(23,580)	(37,634)	(1,042)	112	3,001	6,370	7,973	7,716	7,934	8,159	8,390
	QR-421a - Usher	(6,090)	(15,210)	(23,580)	(37,634)	-	410	19,332	56,434	99,813	96,630	99,400	102,253	105,189
<b>Year for discounting</b>		<b>0.0</b>	<b>0.3</b>	<b>1.3</b>	<b>2.3</b>	<b>3.3</b>	<b>4.3</b>	<b>5.3</b>	<b>6.3</b>	<b>7.3</b>	<b>8.3</b>	<b>9.3</b>	<b>10.3</b>	<b>11.3</b>
<b>NPV</b>		<b>\$(20,300)</b>	<b>\$(49,278)</b>	<b>\$(68,180)</b>	<b>\$(97,115)</b>	<b>\$(33,590)</b>	<b>\$ 2,164</b>	<b>\$ 48,818</b>	<b>\$ 84,826</b>	<b>\$ 103,584</b>	<b>\$ 88,778</b>	<b>\$ 80,859</b>	<b>\$ 73,659</b>	<b>\$ 67,113</b>
	QR-110 - LCA	\$(8,120)	\$(19,711)	\$(27,272)	\$(38,846)	\$(32,870)	\$ 1,842	\$ 36,529	\$ 53,983	\$ 56,343	\$ 47,962	\$ 43,389	\$ 39,261	\$ 35,533
	QR-313 - DEB	\$(6,090)	\$(14,783)	\$(20,454)	\$(29,134)	\$(720)	\$ 69	\$ 1,652	\$ 3,128	\$ 3,495	\$ 3,018	\$ 2,770	\$ 2,542	\$ 2,333
	QR-421a - Usher	\$(6,090)	\$(14,783)	\$(20,454)	\$(29,134)	\$ -	\$ 253	\$ 10,638	\$ 27,715	\$ 43,747	\$ 37,798	\$ 34,700	\$ 31,857	\$ 29,247
<b>NPV</b>		<b>\$ 825,363</b>												
<b>+ Current Cash &amp; Equivalents</b>		<b>\$ 38,497</b>												
<b>Value of the Company</b>		<b>\$ 863,860</b>												
- L-T Debt														
<b>Value of Equity</b>		<b>\$ 863,860</b>												
<b>Value per Share</b>		<b>25</b>												

Assumptions	
Terminal g =	0.00%
Discount rate	12%
Fully Diluted shares outstanding	35,254
<b>Target Price</b>	<b>\$ 25</b>

Contributions	
QR-110	13
QR-313	(1)
QR-421a	11
Cash	1
<b>Total</b>	<b>25</b>

Source: JMP Securities LLC

## Company Description

ProQR Therapeutics is a biopharmaceutical company engaged in building a platform for RNA-based therapeutics for the treatment of severe, rare genetic diseases such as Leber's congenital amaurosis 10 and dystrophic epidermolysis bullosa. ProQR utilizes its unique proprietary RNA repair technology to develop candidates to specifically target and repair the defective messenger RNA (mRNA), a product of a mutated gene, and to restore the expression and function of normal protein.

## Investment Risks

**Regulatory risk.** ProQR, like all other drug development companies, is reliant on the regulatory pace of evaluating new drugs and clinical plans and also on regulators' willingness to approve new drugs.

**Clinical development risk.** Drug development is a risky and capital-intensive endeavor. The vast majority of drugs that enter clinical development fail to reach the market.

**Funding risk.** Reliance on the capital markets poses a risk in terms of investor appetite for biotech stocks and the degree of dilution, depending upon the timing of a deal.

**Competitive risk.** There are other drugs in development for conditions that ProQR is targeting. These programs could provide advantages over ProQR's candidate, which could lead to a lower than anticipated market share.

**Patent risk.** Patent expiration can result in a negative impact to sales. Additionally, generic companies may file abbreviated new drug applications to challenge current products with patent protection.

**Sector risk.** Valuation of pharmaceutical stocks is subject to both investor assessments of the prospects of the underlying companies, and investor tolerance for risk and confidence in the prospects of pharmaceutical stocks as a group. Therefore, ProQR's stock price may fall, even as the company meets or exceeds investor expectations.

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JMP Securities currently makes a market in the securities of ProQR Therapeutics N.V. and Editas Medicine, Inc.

JMP Securities expects to receive OR intends to seek compensation for investment banking services from ProQR Therapeutics N.V. and Editas Medicine, Inc. in the next 3 months.

### JMP Securities Investment Opinion Definitions:

Market Outperform (MO): JMP Securities expects the stock price to outperform relevant market indices over the next 12 months.

Market Perform (MP): JMP Securities expects the stock price to perform in line with relevant market indices over the next 12 months.

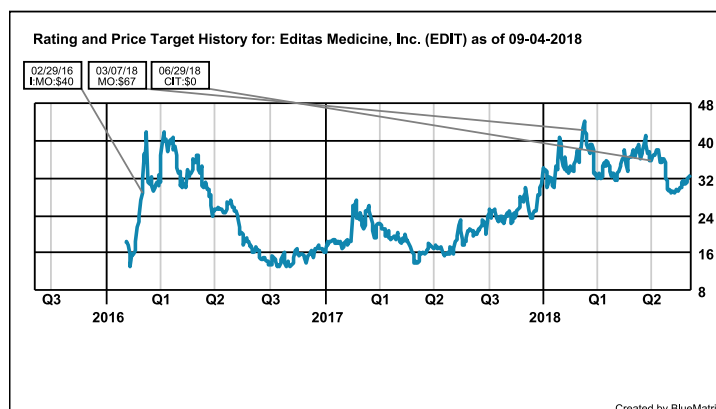
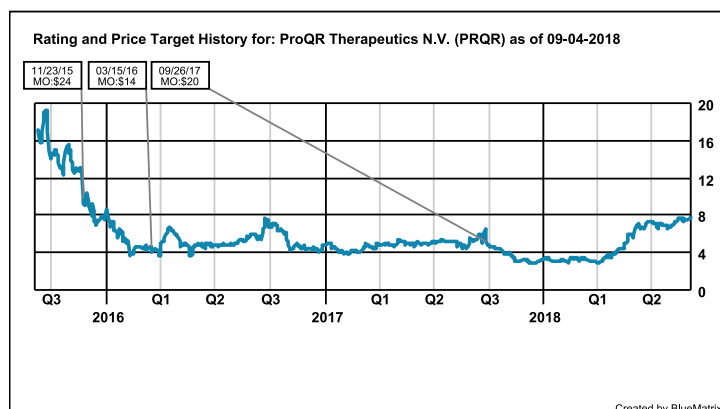
Market Underperform (MU): JMP Securities expects the stock price to underperform relevant market indices over the next 12 months.

### JMP Securities Research Ratings and Investment Banking Services: (as of September 5, 2018)

JMP Rating	Regulatory Equivalent	# Co's Under Coverage	% of Total	Regulatory Equivalent	# Co's Under Coverage	% of Total	# Co's Receiving IB Services in Past 12 Months	
							# Co's	% of Co's With This Rating
MARKET OUTPERFORM	Buy	268	60.77%	Buy	268	60.77%	74	27.61%
MARKET PERFORM	Hold	155	35.15%	Hold	155	35.15%	22	14.19%
MARKET UNDERPERFORM	Sell	6	1.36%	Sell	6	1.36%	0	0%
COVERAGE IN TRANSITION		9	2.04%		9	2.04%	2	22.22%
RATING SUSPENDED		0	0.00%		0	0.00%	0	0%
<b>TOTAL:</b>		<b>441</b>	<b>100%</b>		<b>441</b>	<b>100%</b>	<b>98</b>	<b>22.22%</b>

### Stock Price Chart of Rating and Target Price Changes:

Note: First annotation denotes initiation of coverage or 3 years, whichever is shorter. If no target price is listed, then the target price is N/A. In accordance with FINRA Rule 2241, the chart(s) below reflect(s) price range and any changes to the rating or price target as of the end of the most recent calendar quarter. The action reflected in this note is not annotated in the stock price chart. Source: JMP Securities.



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