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First Take

ProQR Therapeutics N.V. (PRQR)

June 20, 2017

Price: $4.90; Market Cap (M): $115; *6/19/2017 Close*

Rating: Buy; Price Target: $40.00

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Takeaways From the R&D Day

We see multipronged value going forward after last week's R&D Day. We believe that in the last three years since its IPO, ProQr has executed successfully on a transformation from a one-oligo CF company to an RNA-platform pipeline with multiple orphan/pediatric indications on deck. In doing so, the company has elected to make RNA technology its core competency, and is now doubling down by leveraging this core competency to hit multiple orphan targets. Meanwhile, as we look at the development and clinical plan, we applaud the array of indications that appears designed to minimize risk in formulation and delivery, and shorten development/regulatory time. According to management, the current goal is to have three clinical programs with data by end-2018. In our view, this looks achievable, with Phase 1b CF data on tap for this Sept, the IND for the Phase 1 LCA study now open, and a study in EB planned for 2018. Of note, we highlight that the rising programs in ophtho and derm may allow for a short trial and regulatory timetable, potentially even beating the CF program on a race to commercial value, all else equal. Overall, we are encouraged by the company's track of execution, and when considering three expected orphan clinical programs in 2018 in light of the current $115 market cap ($58M cash), we remain bullish on the company's value proposition.

With CF data on track for September, LCA program leads ophthalmology pipeline into the clinic. As ProQR has now transformed from a CF-only company into a diversified RNA-platform, we believe that what will truly kick off this transformation will be the imminent first patient dosing of QR-110 in Leber's congenital amaurosis type 10 (LCA 10). The program now has Fast Track designation and IND clearance in hand. Thanks to administration via intravitreal injection (IVT) and to the specificity of the RNA approach, QR-110 is expected to have: (1) longer retinal PK (months) vs. vitreal PK (days), allowing for infrequent IVT dosing; (2) efficient entry into cells; (3) low systemic exposure; and most importantly (4) the benefit of the optic cup organoid model to significantly de-risk clinical studies. The induced pluripotent stem cells (iPSCs) technology applied to retinal cells can provide an appropriate cellular model with genetic mutations to investigate disease mechanism and evaluate candidate therapies. Designed to recapitulate the genomic context of the disease state, success in the patient-derived iPSC optic cups should be viewed as a superior POC compared to traditional preclinical models. While the Phase 1/2 is expected to assess safety, tolerability, PK of different doses of QR-110 in one eye of each patient (6 adults and 6 children), we expect preliminary efficacy signals to arise in exploratory endpoints (i.e., mobility testing, visual acuity, FST, OCT, PRO, ERG). Most importantly, we believe any efficacy signal coming out of the Leber's clinical program should perpetuate the validating potential of the optic cups, and thus lift the value-propositions of the follow-on ophtho programs (Usher's syndrome, Stargardt's disease, FECD).

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New Axiomer technology POC expected in 2017. At last week's R&D day, the company introduced its latest direction within the RNA space: editing oligonucleotides (EONs). The technology offers the ability to alter stop codons (by changing a target adenosine to an inosine, which is then read as a guanosine for all practical purposes), and can thus restore an open reading frame. As a preclinical demo, at the R&D day the company shared some POC data from a Hurler model (MPS-I). Theoretically, the same principle could be applied to alpha-1 antitrypsin deficiency, Parkinson's, Factor V Leiden deficiency, CF, etc. Since the company referenced these same indications, we assume that they may have already started pursuing them at a POC level. In our view, this platform offers a timely alternative to the wave of DNA editing programs, with fewer potential technical and clinical challenges. Specifically, while EONs may not offer permanent editing (which we would expect of DNA editing technologies), their reversibility could be an advantage when it comes to safety or off-target editing. Also, EONs would not require viral delivery and extra protein expression, and would not introduce genomic breaks that could carry more cellular complications. The company expects to complete POC *in vitro* and *in vivo* in 2017.

Valuation and risks to achievement of target price. Our price target of $40/share is based on a DCF/NPV analysis (discount rate 12.5%, growth rate 2%). Risks to our investment thesis and target price include: (1) failure of QR-010 in clinical studies; (2) failure of QR-010 to secure regulatory approval; (3) failure of QR-010 to achieve peak commercial revenue estimates in our model due to market size, penetration rates, and pricing; and (4) other pipeline failures.

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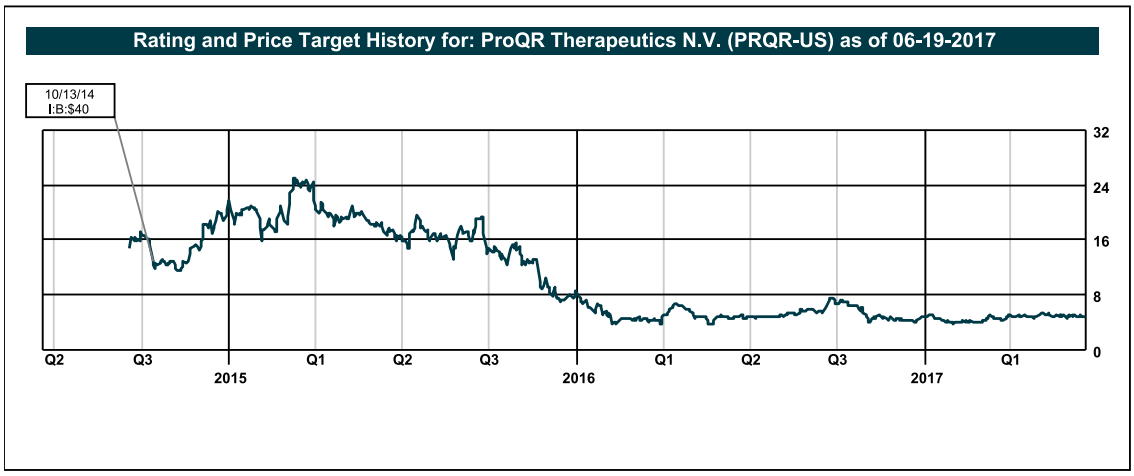

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			IB Service/Past 12 Months	
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Sell	0	0.00%	0	0.00%
Under Review	1	0.41%	1	100.00%
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