June 16, 2017

OUTPERFORM

Reason for report:

COMPANY UPDATE

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LEERINK

PROQR THERAPEUTICS N.V.

Second Annual R&D Day Updates on Clinical Trials and Pipeline

• Bottom Line: PRQR held their second annual R&D day yesterday in NYC for investors and analysts. Mgmt provided updates on their lead clinical trial for Cystic fibrosis (CF), described their vision for treatment of inherited ophthalmic disorders (IHD), and commented on their pipeline of diverse programs that leverage their proprietary RNA repair technology. The company plans to continue to grow its portfolio due to the broad applicability of their RNA repair platform. Although PRQR's therapeutic approach has not yet been fully validated in the clinic, we believe their pre-clinical work is impressive. We are not making any changes to our model based on the information provided at the R&D day. Reiterate Outperform and $10 PT.

• Pt. enrollment for final cohort in randomized, double blind, placebo controlled Ph.1b dose escalation study in homozygous F508del CFTR gene mutated CF pts. treated with QR-010 is near completion and results are anticipated to be reported before YE2017. Mgmt is optimistic they will meet their primary safety and tolerability endpoints, although robust changes in FEV1 are not anticipated due to the mild severity of the CF pts. in the study. In addition, data from the sweat chloride read-out may not be informative since QR-010 is an oligomer, which is not expected to distribute sufficiently to sweat glands.

• QR-110 has been cleared for investigational new drug (IND) status, received CTA open (BE), and FDA designated fast track status. Next step for PRQR will be to announce enrollment completion for their open-label, single arm, dose escalation safety and tolerability Ph.1 study in pts. with LCA10. QR-110's MOA to correct the pCys998x CEP20 mut has positioned PRQR, in the long-term, to treat a larger pt. population than ONCE's (OP) potential IRD therapy, voretigene neparvovec. We anticipate results from this Ph.1 trial in 2018. Candidates for Usher's syndrome, QRX-421 and QRX-411, are ready for IND-enabling studies. QRX-421 was highlighted due to its efficacy to restore retinal function in a zebra fish model of Usher's disease. QRX-504 for treatment of Fuchs endothelial corneal dystrophy (FEDC) is also set for IND-enabling studies. Further down the ocular pipeline is QRX-1011 for Stargardt's disease.

• PRQR projects first clinical data reports for QR-313 in treatment of dystrophic epidermolysis bullosa (DEB) will be in 2018. However, this timeline may be optimistic as they still need to complete formulation work, non-clinical safety studies, IND/CTA filing and enrollment of a Phase 1/ PoC trial before generation of clinical data. QR-313 has the potential to be a major player in the market due to 1) disease modifying aspects, and 2) ease of topical administration.

• Proprietary RNA editing platform expansion with development of Axiomer®. The value in this platform is the potential use in over 20,000 disease-causing mutations & overcoming potential risks of CRISPR.

Key Stats:	**(NASDAQ: PRQR)**
Sector:	**Biotechnology**
S&P 600 Health Care Index:	**2,016.28**
Price :	**$4.95**
Price Target:	$10.00
Methodology:	DCF with 12% discount rate, 2% terminal value growth rate
52 Week High:	$8.70
52 Week Low:	$3.65
Shares Outstanding (mil):	23.5
Market Capitalization (mil):	$116.3
Cash Per Share:	$2.17
Net Debt to Total Capital:	0%
Dividend (ann):	$0.00
Dividend Yield:	0.0%

Completion: June 16, 2017, 8:22AM EDT.
Distribution: June 16, 2017, 8:22AM EDT.
Cash Per Share: Net cash per diluted share

Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2016A	€0.7	€0.6	€0.4	€0.1	€1.8	(€0.44)	(€0.43)	(€0.43)	(€0.38)	(€1.68)	NM
2017E	€0.4A	€0.3	€0.3	€0.3	€1.1	(€0.45)A	(€0.46)	(€0.48)	(€0.51)	(€1.90)	NM
2018E	--	--	--	--	€1.0	--	--	--	--	(€1.33)	NM

Source: Company Information and Leerink Partners LLC Research
IFRS; Revenues in millions.

INVESTMENT THESIS

We rate PRQR Outperform. PRQR is developing novel RNA therapeutics for severe genetic disorders. The company's lead candidate QR-010 has generated highly encouraging preclinical data in a number of validated pre-clinical assays used to evaluate drugs for cystic fibrosis, in our view. QR-010 is an inhaled antisense oligonucleotide (AON) designed to treat patients with ΔF508 mutation, the most common mutation underlying cystic fibrosis (CF). The QR-010 RNA repair technology was established in published experiments first employed in cell cultures at Massachusetts General Hospital (MGH), and PRQR has been able to replicate and augment the molecule with an optimized antisense construct that has composition-of-matter patent protection until 2027 (not including up to 5 years of potential extension).

We look to multiple catalysts over the next 12-18 months for potential de-risking of QR-010 and appreciation in PRQR shares. We see an attractive risk/reward for PRQR shares at the stock's current valuation based on the robust pre-clinical data suggesting that QR-010 can restore CFTR function to near-wild-type levels and large amount of shareholder value created by VRTX, whose CFTR modulators have generated relatively modest FEV1 (forced expiratory volume in 1 second) improvements in homozygous ΔF508 CF patients.

Our $10 price target in 12 months is based on our assumptions of QR-010 pricing of $175k/patient/year together with 50% peak market penetration of ΔF508 CF homozygotes and heterozygotes, probability-weighted at 20% and 0%, respectively. PRQR has also identified around 50 other target indications for its pipeline, including a pre-clinical antisense compound QR-110 for Leber's Congenital Amaurosis (LCA), dystrophic epidermolysis bullosa (DEB), Usher syndrome, Fuchs endothelial corneal dystrophy, Alzheimer's disease, and other CFTR targets covering an additional 10%+ of CF patients, which we do not currently include in our valuation.

VALUATION

We derive a $10 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 20% 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.

PRQR P&L (€MM) - IFRS	2012	2013	2014	2015	1Q16	2Q16	3Q16	4Q16	2016	1Q17	2Q17E	3Q17E	4Q17E	2017E	2018E
Revenue & Other Income	**0.0**	**0.1**	**0.3**	**3.2**	**0.7**	**0.6**	**0.4**	**0.1**	**1.8**	**0.4**	**0.3**	**0.3**	**0.3**	**1.1**	**1.0**
COGS	-	-	-	-	-	-	-	-	-	-				-	-
R&D	(0.3)	(2.6)	(10.3)	(23.4)	(6.9)	(8.6)	(8.3)	(8.1)	(31.9)	(8.0)	(8.4)	(9.0)	(9.7)	(35.1)	(43.9)
SG&A	(0.2)	(0.8)	(6.5)	(6.8)	(2.6)	(2.6)	(2.0)	(2.3)	(9.5)	(2.3)	(2.8)	(3.0)	(3.3)	(11.4)	(14.8)
Operating Expenses	(0.4)	(3.4)	(16.8)	(30.2)	(9.5)	(11.2)	(10.3)	(10.4)	(41.4)	(10.3)	(11.2)	(12.0)	(13.0)	(46.5)	(58.7)
Operating Income	(0.4)	(3.2)	(16.5)	(27.0)	(8.8)	(10.6)	(9.9)	(10.3)	(39.6)	(9.9)	(11.0)	(11.8)	(12.7)	(45.4)	(57.7)
Finance Income (Expense)	0.0	(0.0)	4.3	6.2	(1.4)	0.7	(0.3)	1.4	0.5	(0.5)				(0.5)	-
Other Income (Expense)	-	-	-	-	0.0	-	-	-	0.0	0.0				0.0	-
EBT	(0.4)	(3.3)	(12.1)	(20.8)	(10.2)	(10.0)	(10.1)	(8.8)	(39.1)	(10.5)	(11.0)	(11.8)	(12.7)	(45.9)	(57.7)
Tax Expense (Benefit)	-	-	-	-	-	-	-	-	-	(0.0)	-	-	-	(0.0)	
Net Loss	(0.4)	(3.3)	(12.1)	(20.8)	(10.2)	(10.0)	(10.1)	(8.8)	(39.1)	(10.5)	(11.0)	(11.8)	(12.7)	(45.9)	(57.7)
Diluted EPS (Euros)	**(0.17)**	**(0.59)**	**(1.10)**	**(0.89)**	**(0.44)**	**(0.43)**	**(0.43)**	**(0.38)**	**(1.68)**	**(0.45)**	**(0.46)**	**(0.48)**	**(0.51)**	**(1.90)**	**(1.33)**
Weighted avg. shares outstanding	2.5	5.5	11.0	23.6	23.3	23.3	23.3	23.3	23.3	23.5	24.0	24.5	25.0	24.2	43.5

Source: SEC Filings and Leerink Partners Research

PRQR BS & CFS (€MM) - IFRS	2012	2013	2014	2015	1Q16	2Q16	3Q16	4Q16	2016	1Q17	2Q17E	3Q17E	4Q17E	2017E	2018E
Net Cash	**0.2**	**0.7**	**109.9**	**90.0**	**80.3**	**71.0**	**61.3**	**53.5**	**53.5**	**46.3**	**35.8**	**24.5**	**12.3**	**12.3**	**106.6**
Cash & Equivalents	0.2	4.1	112.7	94.9	85.5	76.3	64.9	59.2	59.2	52.1	41.6	28.4	16.2	16.2	108.5
Debt	-	3.5	2.8	4.8	5.1	5.3	3.6	5.7	5.7	5.8	5.8	3.9	3.9	3.9	1.9
Change in Cash	**0.2**	**3.9**	**107.1**	**(17.8)**	**(9.4)**	**(9.2)**	**(11.4)**	**(5.7)**	**(35.7)**	**(7.1)**	**(10.5)**	**(13.2)**	**(12.2)**	**(43.0)**	**92.3**
Cash Flow From Operations	**(0.3)**	**(2.3)**	**(9.2)**	**(17.4)**	**(7.8)**	**(8.3)**	**(10.8)**	**(7.3)**	**(34.2)**	**(8.8)**	**(10.0)**	**(10.8)**	**(11.7)**	**(41.2)**	**(45.7)**
Net Income	(0.4)	(3.3)	(12.1)	(20.8)	(10.2)	(10.0)	(10.1)	(8.8)	(39.1)	(10.5)	(11.0)	(11.8)	(12.7)	(45.9)	(57.7)
SOE	-	0.0	0.7	1.2	0.6	0.7	0.6	0.5	2.5	0.9	0.8	0.8	0.8	3.3	3.5
D&A	-	0.0	0.1	0.5	0.3	0.4	0.3	0.3	1.2	0.3	0.2	0.2	0.2	0.9	8.5
Other	0.1	0.9	2.1	1.7	1.5	0.6	(1.6)	0.7	1.2	0.5				0.5	-
Cash Flow From Investing	**(0.0)**	**(0.1)**	**(0.7)**	**(1.3)**	**(0.5)**	**(1.6)**	**(0.4)**	**(0.0)**	**(2.5)**	**(0.0)**	**(0.5)**	**(0.5)**	**(0.5)**	**(1.5)**	**(10.0)**
CapEx	-	(0.1)	(0.7)	(1.3)	(0.5)	(1.6)	(0.4)	(0.0)	(2.5)	(0.0)	(0.5)	(0.5)	(0.5)	(1.5)	(10.0)
Other	(0.0)	-	-	(0.0)	-	-	-	-	-	-				-	-
Cash Flow From Financing	**0.6**	**6.3**	**117.1**	**1.7**	**0.2**	**(0.0)**	**-**	**0.2**	**0.4**	**2.2**	**-**	**(1.9)**	**-**	**0.2**	**148.1**
Equity Issuance (Buyback)	0.5	3.0	118.1	0.0	-	-	-	-	-	2.2				2.2	150.0
Debt Issuance (Retirement)	0.1	3.3	(1.0)	1.3	-	-	-	0.2	0.2	-		(1.9)		(1.9)	(1.9)
Other	-	-	(0.0)	0.5	0.2	(0.0)	-	-	0.2	0.0				0.0	-
FX/Other	-	-	-	(0.8)	(1.3)	0.8	(0.2)	1.5	0.7	(0.4)				(0.4)	-

Source: SEC Filings and Leerink Partners Research

*Quarterly Financials Not Available for 2013; **Quarterly Financials Not Available for 1H14

PRQR DCF Analysis	2014	2015	2016	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	TV
CFO (€MM)	(9.2)	(17.4)	(34.2)	(41.2)	(45.7)	(75.2)	(85.1)	(100.1)	46.7	172.0	249.4	238.4	161.8	110.0	75.3	58.8	
CFI (€MM)	(0.7)	(1.3)	(2.5)	(1.5)	(10.0)	(15.0)	(20.0)	(20.0)	(20.0)	(20.0)	(20.0)	(20.0)	(20.0)	(20.0)	(20.0)	(20.0)	
Net Borrowing (€MM)	(1.0)	1.3	0.2	(1.9)	(1.9)	(1.9)	-	-	-	-	-	-	-	-	-	-	
Free Cash Flow to Equity (€MM)	(11.0)	(17.5)	(36.6)	(44.7)	(57.7)	(92.2)	(105.1)	(120.1)	26.7	152.0	229.4	218.4	141.8	90.0	55.3	38.8	380.4
Discount Periods	0	0	0	0	0.75	1.75	2.75	3.75	4.75	5.75	6.75	7.75	8.75	9.75	10.75	11.75	
Discounted FCFE (€MM)	-	-	-	(44.7)	(52.8)	(75.1)	(76.2)	(77.4)	15.3	77.6	104.2	88.3	51.0	28.8	15.7	9.8	96.3

Sum NPV FCFE	160.8
Net Cash 1Q17	46.3
FX: EUR/USD	1.10
Implied Mkt. Cap ($MM)	$ 227.3
PRQR Price Target	**$ 10**

Discount Rate	12%
Terminal Growth Rate	2%
Diluted Shares Outstanding 1Q17	23.5

Source: Leerink Partners Research

Product	Event	Timing
QR-110	Initiate Phase Ib/II (LCA)	1H17
QR-010	Enrollment update	ECFS (Jun 7-10)
QR-010	Phase Ib MAD Data (CF)	Mid-2017
QR-010	Initiate Phase II (CF)	2018
QR-110	Phase Ib/II (LCA) Topline data	2018
QR-313	Initiate Phase I (DEB)	2018
QR-010	Phase II Data (CF)	2019
QR-010	Initiate Phase III (CF)	Mid-2019
QR-010	Complete Enrollment for CF	YE19
QR-010	Phase III Data (CF)	2H20
QR-010	NDA filing	1H21
QR-010	PDUFA Date	2H21

Source: SEC Filings and Leerink Partners Research

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

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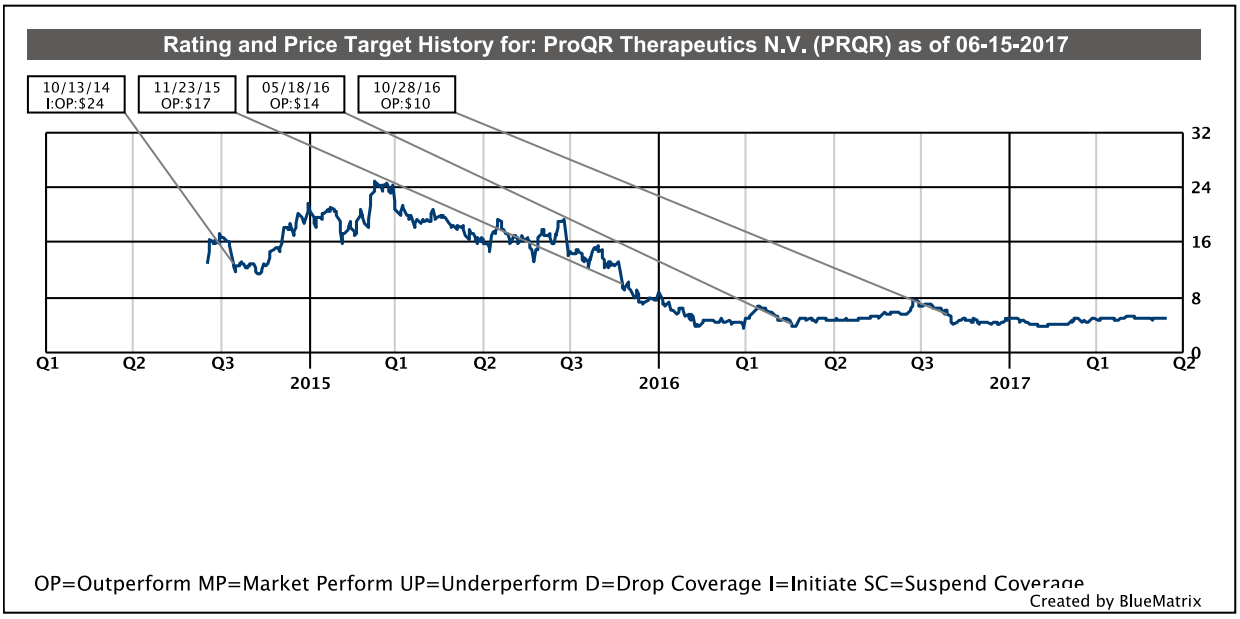

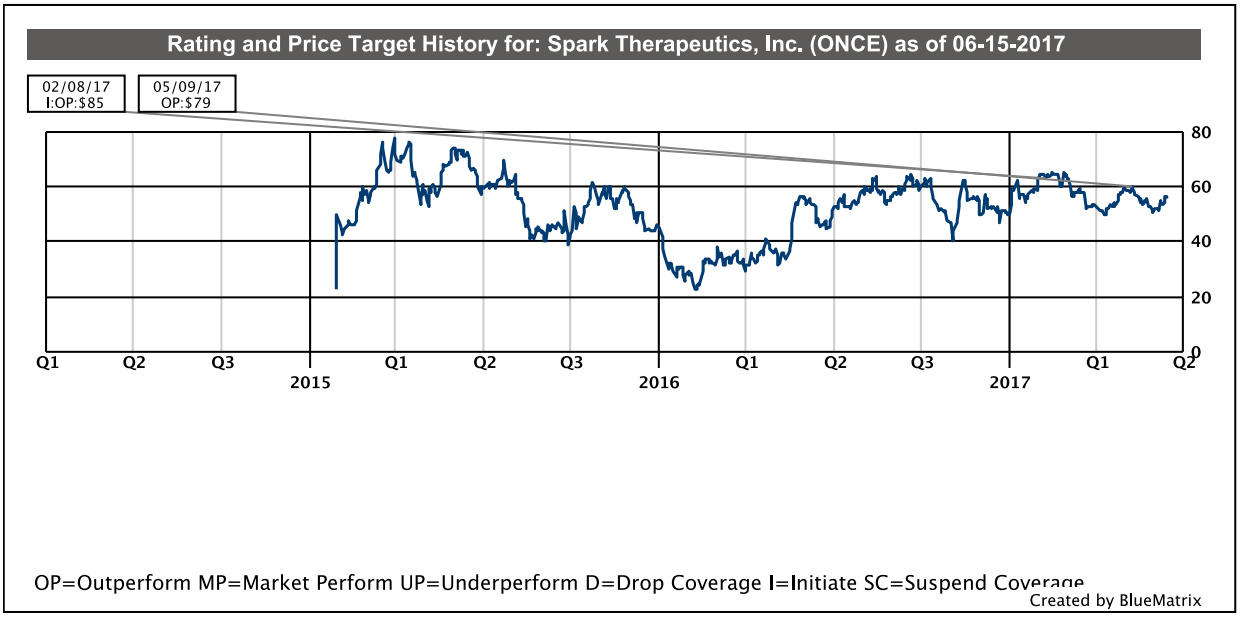
Rating and Price Target History for: Spark Therapeutics, Inc. (ONCE) as of 06-15-2017
02/08/17
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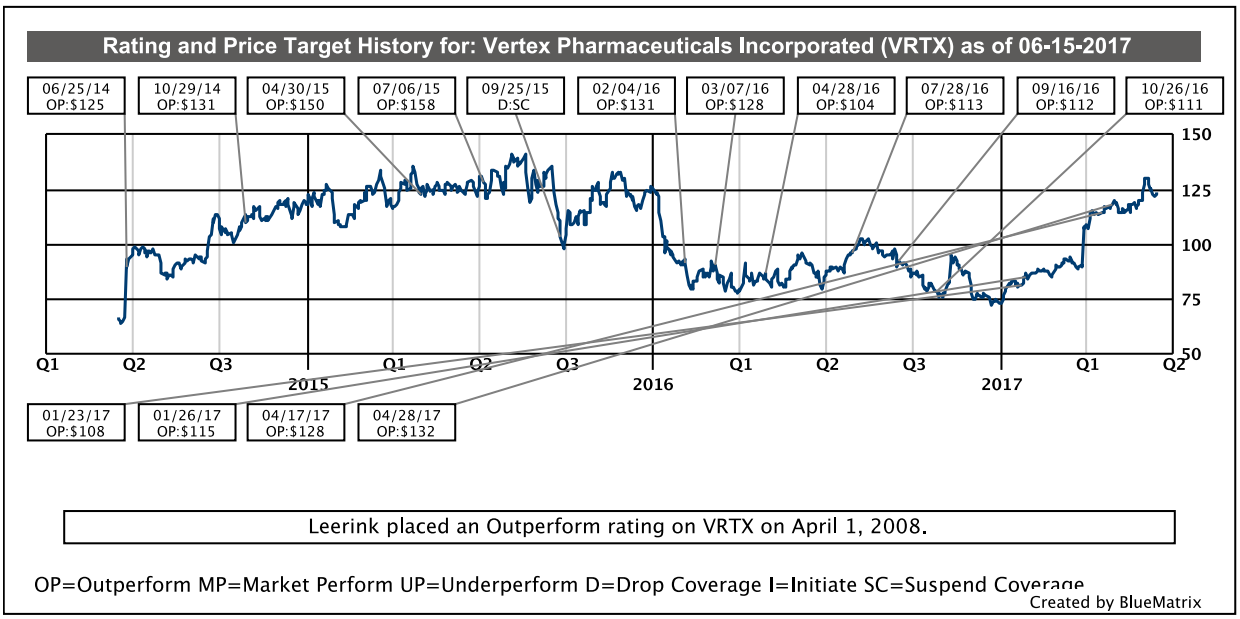
05/09/17
OP:$79
80
60
40
20
0
Q1
Q2
Q3
2015
Q1
Q2
Q3
2016
Q1
Q2
Q3
2017
Q1
Q2
OP=Outperform MP=Market Perform UP=Underperform D=Drop Coverage I=Initiate SC=Suspend Coverage
Created by BlueMatrix


Rating and Price Target History for: Vertex Pharmaceuticals Incorporated (VRTX) as of 06-15-2017
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10/29/14
OP:$131
04/30/15
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07/06/15
OP:$158
09/25/15
D:SC
02/04/16
OP:$131
03/07/16
OP:$128
04/28/16
OP:$104
07/28/16
OP:$113
09/16/16
OP:$112
10/26/16
OP:$111
150
125
100
75
50
Q1
Q2
Q3
2015
Q1
Q2
Q3
2016
Q1
Q2
Q3
2017
Q1
Q2
01/23/17
OP:$108
01/26/17
OP:$115
04/17/17
OP:$128
04/28/17
OP:$132
Leerink placed an Outperform rating on VRTX on April 1, 2008.
OP=Outperform MP=Market Perform UP=Underperform D=Drop Coverage I=Initiate SC=Suspend Coverage
Created by BlueMatrix

Distribution of Ratings/Investment Banking Services (IB) as of 03/31/17				
			IB Serv./Past 12 Mos.	
Rating	Count	Percent	Count	Percent
BUY [OP]	120	62.2	29	24.2
HOLD [MP]	73	37.8	4	5.5
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

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Leerink Partners LLC makes a market in ProQR Therapeutics N.V., Spark Therapeutics, Inc. and Vertex Pharmaceuticals Incorporated.

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

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