September 3, 2014

OUTPERFORM

Reason for report:

EARNINGS

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LEERINK

UNIQURE, N.V.

2Q14 Recap; Glybera US Study Launch Delayed; Other Programs On Track

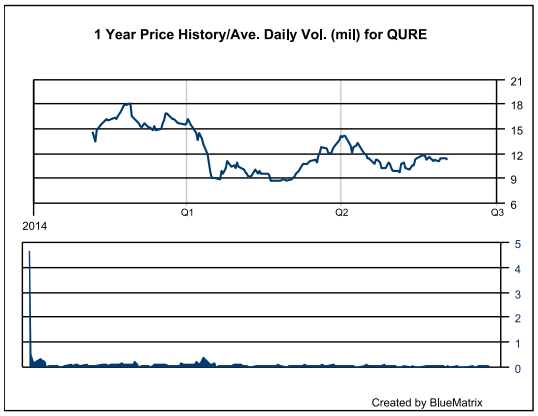
• **Bottom Line:** QURE reported 2Q14 financial results yesterday and provided a pipeline update. The company reported a slight delay with regard to the regulatory process for Glybera (LPLD) in the US. More time than initially expected is needed to align EU post-approval trial requirements with US pivotal trial design. We are now projecting a potential US Glybera launch in early 2019 vs. mid-2018 previously. Other pipeline programs remain on track. QURE ended 2Q14 with €72.1M in cash and equivalents. We continue to believe QURE shares are poised to appreciate primarily driven by progress of the Glybera launch in Europe, clinical progress toward approval in the U.S., and by advances of pipeline candidates targeting significantly larger indications than does Glybera, such as Hemophilia-A and -B, and potentially congestive heart failure, among others. We reiterate our OP rating and $28 price target.

• **Six months delay in Glybera US regulatory progress expected.** This delay is in part the result of longer-than-expected EMA approval timelines due to required changes in the trial protocol in support of the US filing strategy, resulting in a planned Phase 4 trial start now in mid-2015, and in part the result of limited product supply due to a Glybera-specific batch release assay being out of specification. Recall, the FDA previously indicated in a pre-IND meeting in 2H13 that the company can use the data from the required EU post-approval trial and patient registry for US approval, but with changes to an endpoint measure definition. QURE is currently amending the trial protocols so that they could also serve as a single clinical program with a design that addresses the FDA's requirements for US approval requiring a change to EMA post-approval requirements. Another FDA meeting is planned for November 2014 as well. Trials are now expected to initiate in mid-2015, and mgmt has suggested that it would take ~1 year to enroll and another 2 years for follow-up. We are now projecting a potential US Glybera launch in early 2019 (vs. mid-2018 previously). With regard to manufacturing, mgmt noted that the company expects to be able to meet 2015 EU commercial demand with current stock and from future production runs commencing later in 2014, assuming higher manufacturing standards are successfully implemented. The European launch of Glybera is still expected in 4Q14/1Q15.

• **Other pipeline programs remain on track.** Initiation of the Phase 1/2 trial for hemophilia B is expected in 2H14. Collaborator-sponsored programs for acute intermittent porphyria and Sanfilippo B are on track with data expected in 2H14 and 2015, respectively.

• **QURE ended 2Q14 with €72.1M in cash and equivalents.** We update our model to reflect 2Q14 results. QURE reported 2Q14 revenue of €1M, vs. our estimate of €1.2M; a net loss of ~€9M, vs. our net loss estimate of €8M; and diluted EPS of (€0.51), vs. our estimate of (€0.46).

Key Stats:	**(NASDAQ:QURE)**
S&P 600 Health Care Index:	**1,332.17**
Price:	**$11.18**
Price Target:	$28.00
Methodology:	DCF analysis with 12% discount rate
52 Week High:	$18.75
52 Week Low:	$8.29
Shares Outstanding (mil):	17.6
Market Capitalization (mil):	$196.8
Book Value/Share:	$4.13
Cash Per Share:	$5.38
Dividend (ann):	$0.00
Dividend Yield:	0.0%



Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2013A	--	--	--	--	€2.9	--	--	--	--	(€2.48)	NM
2014E - New	**€1.2A**	**€1.0A**	**€1.2**	**€1.2**	**€4.7**	**(€0.52)A**	**(€0.51)A**	**(€0.53)**	**(€0.52)**	**(€2.08)**	**NM**
2014E - Old	€1.2A	€1.2	€1.2	€1.2	€4.8	(€0.52)A	(€0.46)	(€0.46)	(€0.45)	(€1.88)	NM
2015E	--	--	--	--	€7.9	--	--	--	--	(€1.90)	NM

Source: Company Information and Leerink Partners LLC Research
Revenues in million EUROs

INVESTMENT THESIS

We rate QURE Outperform with a $28/share price target. QURE is a leader in the field of gene therapy and has developed the first and currently the only gene therapy product to receive regulatory approval in the Western World. Gene therapy offers the prospect of long-term and potentially curative benefit to patients. QURE's Glybera has been EMA-approved for treatment of patients with lipoprotein lipase deficiency (LPLD), a ultra-rare metabolic disease. Glybera is partnered with Chiesi Farmaceutici in Europe, and QURE retains full commercial rights in the United States. QURE is developing a pipeline of additional gene therapies through multiple collaborations that are designed to accelerate the development and commercialization of these programs. QURE's gene therapies are delivered through an engineered non-replicating version of the adeno-associated virus (AAV), and therapies are being developed using an innovative, modular technology platform, which consists of a suite of components that may be applied to multiple gene therapies and includes QURE's proprietary cost effective commercial scale manufacturing process. We believe QURE shares are poised to appreciate primarily driven by progress of the Glybera launch in Europe, clinical progress toward approval in the U.S., and by advances of pipeline candidates targeting significantly larger indications than does Glybera, such as Hemophilia-A and -B, congestive heart failure, Sanfilippo B, Acute Intermittent Porphyria, and Parkinson's Disease, among others. QURE has multiple potential meaningful catalysts over the next two years.

VALUATION

We estimate a $28 fair value for QURE shares in one year, based on a discounted multiple of sales analysis. We attribute $5/share to Glybera EU royalties, $4/share to the Glybera US franchise and assume a 70% probability of success, $14 to AMO-060 in Hemophilia B, assuming a 30% probability of success, and the rest to net cash. We use a 12% WACC as the discount rate, similar to other small-cap biotech stocks in our coverage since our revenue assumptions are already probability-of-success weighted. We model 2025E US Glybera sales of €46M, 2025E Glybera EU royalties of €22M, 2025E US AMF-060 sales of €233M, and 2025E EU AMT-060 royalties of €82M (all before probability adjustment).

RISKS TO VALUATION

An investment in QURE is fundamentally a high-risk, high-reward investment, in our opinion. For risks associated with LPLD in EU, a poorer-than-expected launch, reimbursement, and inability to identify patients despite approval could harm the stock. In the US, Glybera has additional clinical, regulatory risks. On the hemophilia front, competition from Longer Acting Factors (e.g., BIIB), RNAi (e.g., ALNY) and other Gene Therapy companies could deem QURE’s program as risky. Clinical and regulatory risks remain with QURE’s early hemophilia program. Systemic impact from other Gene Therapy failure such as a new case of leukemia with competitor initiative could pose significant risk. Finally, financing risks also remain and we don't expect the company to be cash-flow positive for several years.

QURE P&L (in million EUROs, except EPS)	2012A	1Q13A	2Q13A	3Q13A	4Q13A	2013A	1Q14A	2Q14A	3Q14E	4Q14E	2014E	2015E	2016E	2017E
License revenues	-	-	-	0.2	0.2	0.4	0.2	0.2	0.2	0.2	0.9	0.9	0.9	0.9
Collaboration revenues	-	-	0.8	1.8		2.5	1.0	0.8	1.0	1.0	3.8	4.0	4.0	4.0
Milestones	-	-	-	-	-	-	-	-	-	-	-	-	5.0	10.0
Product sales	-	-	-			-	-	-	-	-	-	-	-	-
Royalty revenue	-	-	-			-	-	-	-	-	-	3.0	7.5	15.0
Total revenue	**-**	**-**	**0.8**	**2.1**		**2.9**	**1.2**	**1.0**	**1.2**	**1.2**	**4.7**	**7.9**	**17.4**	**29.9**
COGS	-	-	(0.8)	(0.8)		(0.8)	-	-	-	-	-	1.5	1.9	3.8
Other income	0.6	0.2	0.2	0.7		0.6	0.2	0.2	0.2	0.2	0.9	-	-	-
R&D expense	(10.2)	(3.6)	(2.9)	(9.9)		(13.2)	(6.2)	(8.0)	(8.0)	(8.0)	(30.2)	(32.0)	(35.0)	(40.0)
SG&A expense	(4.6)	(1.7)	(2.4)	(7.6)		(11.6)	(2.3)	(2.5)	(2.5)	(2.5)	(9.9)	(10.0)	(10.2)	(15.2)
Other gains/losses, net	(0.0)	0.0	0.0	(0.3)		(0.5)	(0.5)	0.5	-	-	0.0	-	-	-
Total operating expenses	(14.2)	(5.1)	(5.9)	(17.9)		(25.5)	(8.8)	(9.8)	(10.3)	(10.3)	(39.3)	(40.5)	(43.3)	(51.5)
Operating income (loss)	**(14.2)**	**(5.1)**	**(5.1)**	**(15.8)**		**(22.5)**	**(7.6)**	**(8.8)**	**(9.1)**	**(9.1)**	**(34.6)**	**(32.6)**	**(25.9)**	**(21.6)**
Finance income	0.0	0.0	-	0.0		0.1	0.0	0.0	-	-	0.1	-	-	-
Finance expense	(0.5)	(0.1)	(2.7)	(4.7)		(4.4)	(0.3)	(0.3)	(0.2)	(0.2)	(1.0)	(1.1)	(0.9)	(0.0)
Finance income/expense, net	(0.5)	(0.1)	(2.7)	(4.6)		(4.3)	(0.2)	(0.2)	(0.2)	(0.2)	(0.9)	(1.1)	(0.9)	(0.0)
EBT	**(14.7)**	**(5.2)**	**(7.8)**	**(20.4)**		**(26.8)**	**(7.8)**	**(9.0)**	**(9.3)**	**(9.3)**	**(35.5)**	**(33.7)**	**(26.9)**	**(21.6)**
Income Tax expense	-	-	-	-		-	-	-	-	-	-	-	-	-
Net income (loss)	**(14.7)**	**(5.2)**	**(7.8)**	**(20.4)**		**(26.8)**	**(7.8)**	**(9.0)**	**(9.3)**	**(9.3)**	**(35.5)**	**(33.7)**	**(26.9)**	**(21.6)**
Other comprehensive income		-	-	0.0		0.0	0.0	(0.0)	-	-	(0.0)	-	-	-
Total comprehensive income (loss)	(14.7)	(5.2)	(7.8)	(20.4)		(26.8)	(7.8)	(9.0)	(9.3)	(9.3)	(35.5)	(33.7)	(26.9)	(21.6)
Common shares outstanding (basic)	8.6	9.8	9.7	10.6		12.2	15.1	17.6	17.6	17.8	17.0	17.8	21.0	21.0
Common shares outstanding (diluted)	8.6	9.8	9.7	10.6		12.2	15.1	17.6	17.6	17.8	17.0	17.8	21.0	21.0
EPS (basic)	(1.70)	(0.53)	(0.80)	(1.95)		(2.48)	(0.52)	(0.51)	(0.53)	(0.52)	(2.08)	(1.90)	(1.28)	(1.03)
EPS (diluted))	**(1.70)**	**(0.53)**	**(0.80)**	**(1.95)**		**(2.48)**	**(0.52)**	**(0.51)**	**(0.53)**	**(0.52)**	**(2.08)**	**(1.90)**	**(1.28)**	**(1.03)**

QURE BS & CFS (in million EUROs, except EPS)	2012A	1Q13A	2Q13A	3Q13A	4Q13A	2013A	1Q14A	2Q14A	3Q14E	4Q14E	2014E	2015E	2016E	2017E
Cash & equivalents	0.3	8.5		31.4	23.8	23.8	77.5	72.1	59.7	48.3	48.3	17.5	70.2	44.7
Debt	1.5			8.5	7.9	7.9	7.5	15.2	15.0	14.4	14.4	11.3	4.4	-
Change in Cash	(0.8)		9.3	21.8		23.5	53.7	(5.5)	(12.3)	(11.4)	24.5	(30.8)	52.7	(25.5)
Cash from operations	**(11.3)**		**(8.8)**	**10.5**		**(4.1)**	**(5.1)**	**(4.1)**	**(7.8)**	**(7.8)**	**(24.8)**	**(27.7)**	**(20.4)**	**(13.6)**
Net income (loss)	(14.7)		(7.8)	(20.4)		(26.8)	(7.8)	(9.0)	(9.3)	(9.3)	(35.5)	(33.7)	(26.9)	(21.6)
Share based comp	1.8		0.9	0.5		2.0	2.3	2.3	1.6	1.6	7.8	6.3	6.8	8.3
D&A	0.5		0.3	0.1		0.5	0.1	0.2	0.1	0.1	0.6	0.6	0.6	0.6
Other (Change in WC)	1.1		(2.2)	30.3		20.1	0.3	2.4	(0.2)	(0.2)	2.3	(0.9)	(0.9)	(0.9)
Cash from investing	**(0.8)**		**(1.5)**	**(2.6)**		**(6.0)**	**(3.1)**	**(8.6)**	**(6.0)**	**(3.0)**	**(20.7)**	**-**	**-**	**(7.5)**
CapEx	(0.4)		(0.3)	(0.2)		(1.3)	(2.0)	(7.8)	(3.0)	(3.0)	(15.8)	-	-	(7.5)
Acquisitions	-		-	-		-	-	-	(3.0)	-	(3.0)	-	-	-
Other	(0.4)		(1.2)	(2.4)		(4.6)	(1.1)	(0.8)	-	-	(1.9)	-	-	-
Cash from financing	**11.3**		**19.7**	**14.0**		**33.6**	**61.9**	**7.2**	**1.5**	**(0.6)**	**70.0**	**(3.1)**	**73.1**	**(4.4)**
Equity issue (buyback)	9.8		-	14.3		14.3	62.0	-	1.5	-	63.5	-	80.0	-
Debt issue (principal payment)	1.5		19.5	-		19.5	-	7.2	-	(0.6)	6.6	(3.1)	(6.9)	(4.4)
Other	-		0.2	(0.3)		(0.1)	(0.0)	(0.0)	-	-	(0.1)	-	-	-

Source: SEC Filings and Leerink Partners Estimates

Proprietary pipeline						
Agent	**Partner**	**Vector - Gene**	**Indication**	**Status**	**n=**	**Next Events**
Glybera - EU	Chiesi (EU)	AAV1-LPL	Lipopritein lipase deficiency	Approved in EU		Chiesi launch in 4Q14/1Q15
Glybera - US	Proprietary	AAV1-LPL	Lipopritein lipase deficiency	Phase IV		Initiate mid-15
						File BLA in 2018
						US approval in 2019
AMT-060	Chiesi (EU)	AAV5-Factor IX	Hemophilia B	Phase I/II	13-16	Initiate Phase I/II in 2H14
						Phase I/II interim data in mid-2015
	St. Jude	AAV8-Factor IX	Hemophilia B	IST Phase I/II	8	Interim data published
	n/a	AAV5-Factor VIII	Hemophilia A	Preclinical		Initiate Phase I/II in 1H16
	n/a	AAV9-S100A1	Congestive Heart Failure	Preclinical		Initiate Phase I/II in 1H16
Collaborator-sponsored programs						
Agent	**Partner**	**Vector - Gene**	**Indication**	**Status**	**n=**	**Next Events**
AMT-021	Digna Biotech	AAV5-PBGD	Acute Intermittent Porphyria	Phase I	8	Data in 2H14
AMT-110	Institut Pasteur	AAV5-NaGLU	Sanfilippo B	Phase I/II	4	Data in 2015
AAV2/GDNF	UCSF/NIH	AAV2-GDNF	Parkinson's Disease	Phase I	24	Data in 2015
			Huntington's Diseases miRNA	Preclinical		

Source: Leerink Partners estimates, clinical trials.gov; company updates

Disclosures Appendix

Analyst Certification

I, Michael Schmidt, Ph.D., 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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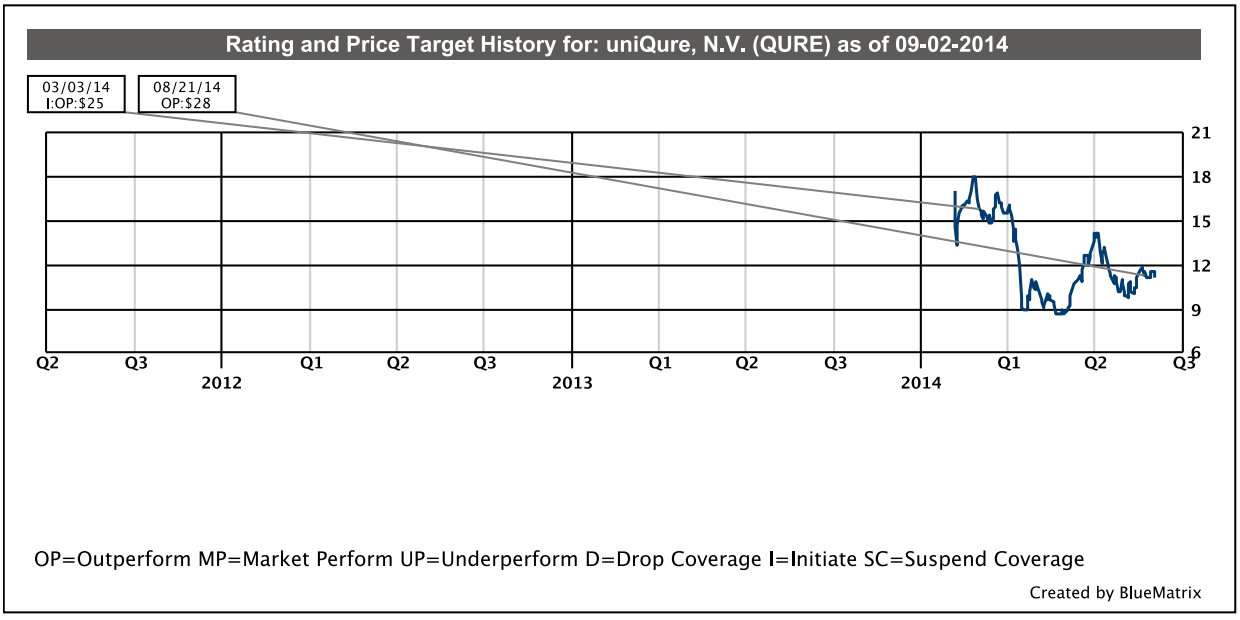
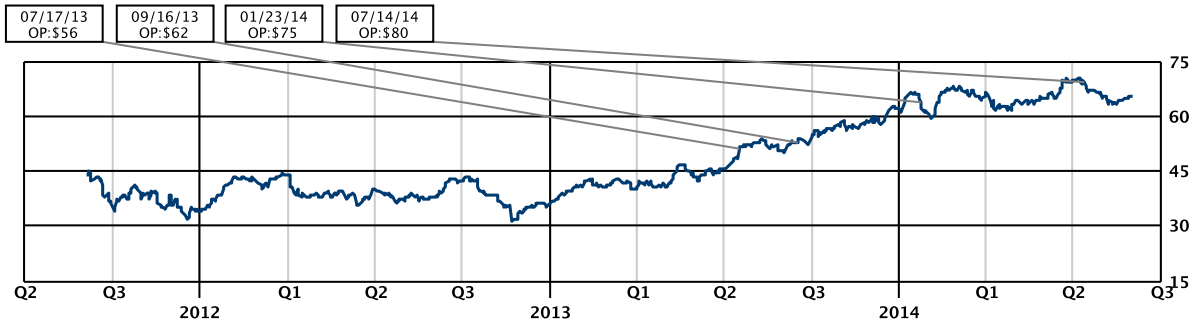


Rating and Price Target History for: St. Jude Medical, Inc. (STJ) as of 09-02-2014



Leerink Swann initiated coverage of STJ with an Outperform rating on June 23, 2008. On June 11, 2013, Leerink Swann began a transition to specific price targets for the stocks under its coverage, replacing valuation ranges.

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 06/30/14				
			IB Serv./Past 12 Mos.	
Rating	Count	Percent	Count	Percent
BUY [OP]	138	69.00	50	36.20
HOLD [MP]	62	31.00	2	3.20
SELL [UP]	0	0.00	0	0.00

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 uniQure, N.V. .

Leerink Partners LLC makes a market in uniQure, N.V., Alnylam Pharmaceuticals, Inc. and Biogen IDEC, Inc.

Leerink Partners LLC is willing to sell to, or buy from, clients the common stock of St. Jude Medical, Inc. on a principal basis.

Leerink Partners LLC has acted as the manager for a public offering of uniQure, N.V. in the past 12 months.

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