COWEN
AND COMPANY

Biotechnology

Ultragenyx

Equity Research

Earnings Update

May 12, 2014

Price: $36.55 (05/9/2014)

Price Target: NA

OUTPERFORM (1)

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Key Data

Symbol	NASDAQ: RARE
52-Week Range:	$69.77 - 34.12
Market Cap (MM):	$1,097.8
Net Debt (MM):	$(165.4)
Cash/Share:	$24.92
Dil. Shares Out (MM):	21.6
Enterprise Value (MM):	$1,152.2
ROIC:	NA
ROE (LTM):	NA
BV/Share:	$(15.35)
Dividend:	NA

FY (Dec)	2013A	2014E	2015E
Earnings Per Share			
Q1	$(2.87)	$(0.85)A	-
Prior Q1	-	*$(0.40)*	-
Q2	$(3.44)	$(0.44)	-
Prior Q2	-	-	-
Q3	$(3.39)	$(0.45)	-
Prior Q3	-	-	-
Q4	$(4.98)	$(0.50)	-
Prior Q4	-	-	-
Year	$(14.87)	$(1.98)	$(1.90)
Prior Year	-	*$(1.80)*	-
P/E	NM	NM	NM
Revenue (MM)			
Q1	$0.0	$0.0A	-
Q2	$0.0	$0.0	-
Q3	$0.0	$0.0	-
Q4	$0.0	$0.0	-
Year	$0.0	$0.0	$0.0

Q1 Financials, Pipeline On Track

The Cowen Insight

Ultragenyx released Q1 financials and ended the quarter with $165.4MM, sufficient for operations into 2016. The company's 5 clinical programs continue to show promise and are advancing towards important data readouts in H2:14 and 2015. We expect multiple programs to ultimately succeed and RARE remains a top pick.

Financial Update:

Ultragenyx reported Q1:14 financials. OpEx was $10.3MM compared to our $11.5MME. However, charges relating to the preferred stock conversions in conjunction with its February IPO resulted in a net loss attributable to stockholders of $18.4MM versus our $12MME. Ultragenyx ended the quarter with $165.4MM in cash. Management projects that this is sufficient to fund operations into 2016.

Pipeline Update:

During Q1, Ultragenyx reported encouraging data demonstrating improvement in uGAG excretion and liver size from the initial MPS7 patients treated with rhGUS in a Phase I/II trial. 12-week data from the entire cohort is expected in H2:14. 48-week data from the Phase II trial of SA-ER in HIBM was also presented during the quarter. SA-ER continued to generate improvements in upper extremity strength, although other endpoints saw limited impact. Data from a further sialic acid dose escalation is expected in H2:14. Ultragenyx's partner KHK has generated positive single-dose Phase I data for KRN23 in XLH patients. Data from the Phase I/II repeat-dose study is expected to be presented in 2014, and support a Phase II study in pediatric XLH patients which is anticipated to begin in H2:14. Finally, Ultragenyx continues to enroll patients in PII studies for LC-FAOD and Glut1 DS. Data in each indication is expected in 2015.

Our Thesis:

Ultragenyx is focused on developing novel therapeutics for rare, serious metabolic disorders. Management has built a multi-faceted pipeline via business development that contains 4 PII clinical candidates for 5 indications. Ultragenyx is financed through potential value creating milestones in all 5 programs. We expect the stock to outperform as milestones in many of these programs are achieved.

At A Glance

Our Investment Thesis

KRN23's animal model and early human (Phase I) data are compelling, and we view it as a relatively low-risk candidate with >$1B in worldwide potential. Triheptanoin's metabolic profile should prove superior to existing dietary alternatives, and early clinical data support this view. FAOD and Glut1 DS could each represent $400MM + markets for triheptanoin. SA-ER, and extended release form of sialic acid, is in a randomized Phase II trial for HIBM, a rare muscle disease. Initial data from the study have produced intriguing signs of activity. Ultragenyx is financed into 2016 and through potential value creating milestones on all of its programs. We expect the stock to outperform as milestones on these and other programs are achieved.

Forthcoming Catalysts

- Phase I/II repeat dosing data for KRN23 in adult XLH patients
- Phase I/II data for rhGUS in MPS7
- Data from Phase II trial extension of SA-ER in HIBM in late 2014

Base Case Assumptions

- KRN23 is launched in 2018; achieves sales of over $1B in XLH
- Triheptanoin is launched for Glut1 DS in 2018 and LC-FAOD in 2019; achieves nearly $1B in sales by 2027
- rhGUS is launched in 2017 for MPS7; achieves $100MM in sales by 2027

Upside Scenario

- SA-ER demonstrates clear efficacy in HIBM; achieves commercial success
- Triheptanoin demonstrates a cognitive benefit for Glut1 DS patients
- Increased screening expands the diagnosed ex-U.S. LC-FAOD patient population

Downside Scenario

- Repeat dosing of KRN23 generates anti-KRN23 antibodies
- Triheptanoin fails to demonstrate a benefit in Glut1 DS patients
- Orphan drug pricing comes under pressure

Price Performance

Source: Bloomberg

Company Description

Ultragenyx is focused on developing novel therapeutics for rare, serious metabolic disorders. Ultragenyx has built a multi-faceted pipeline via business development that contains four clinical candidates for five indications. KRN23 is a monoclonal antibody specific for FGF23 being developed for X-linked hypophosphatemia (XLH) under a collaboration with Kirin. KRN23 is currently in Phase I/II trials expected to readout in 2014. Triheptanoin is a wholly owned 7-chain carbon alternative source of energy in Phase II trials for long-chain fatty acid oxidation disorders (LC-FAOD) and glucose transporter 1 deficiency (Glut1 DS). Data for both indications is anticipated for 2015. rhGUS is an enzyme replacement therapy in Phase I for the lysosomal storage disease MPS7. Data is expected in H2:14, and could propel rhGUS straight into Phase III. Finally, sialic acid extended release (SA-ER) is in Phase II trials for HIBM which is proposed to be driven by a lack of sialic acid.

Analyst Top Picks

	Ticker	Price (05/9/2014)	Price Target	Rating
Sunesis Pharmaceuticals	SNSS	$5.01	$NA	Outperform
Relypsa, Inc	RLYP	$19.60	$NA	Outperform
Ultragenyx	RARE	$36.55	$NA	Outperform

Investment Thesis

Ultragenyx is focused on developing novel therapeutics for rare, serious metabolic disorders. Ultragenyx has built a multi-faceted pipeline via business development that contains four clinical candidates for five indications. KRN23 is a monoclonal antibody specific for FGF23 being developed for X-linked hypophosphatemia (XLH) under a collaboration with Kirin. KRN23 is currently in Phase I/II trials expected to readout in 2014. KRN23's animal model and early human (Phase I) data are compelling, and we view it as a relatively low-risk candidate with >$1B in worldwide potential. Triheptanoin is a wholly owned 7-chain carbon alternative source of energy in Phase II trials for long-chain fatty acid oxidation disorders (LC-FAOD) and glucose transporter 1 deficiency (Glut1 DS). Data for both indications is anticipated for 2015. Triheptanoin's metabolic profile should prove superior to existing dietary alternatives, and early clinical data support this view. FAOD and Glut1 DS could each represent $400MM+ markets for triheptanoin. rhGUS is an enzyme replacement therapy in Phase I for the lysosomal storage disease MPS7. Data is expected in H2:14, and could propel rhGUS straight into a Phase III trial. Finally, sialic acid extended release (SA-ER) is an extended release form of sialic acid. SA-ER is in a randomized Phase II trial for HIBM, a rare muscle disease. Initial data from a randomized Phase II study have produced intriguing signs of activity. Ultragenyx is financed into 2016 and through potential value creating milestones on all of its programs. We expect the stock to outperform as milestones on these and other programs are achieved.

Ultragenyx - Upcoming Milestones/Events

Indication/Milestone	Timing
12-week Phase I/II data for UX003 (rhGUS) in MPS7	H2:14
Initiate KRN23 Phase II trial in pediatric XLH	H2:14
Discussions with the FDA regarding uGAG as a Phase III endpoint for MPS7	H2:14
Data from the Phase II extension study of UX001 (SA-ER) in HIBM	H2:14
Phase I/II repeat dosing data for KRN23 in adult XLH	2014
24-week data from UX007 (triheptanoin) trial in LC-FAOD	2015
8-week data from UX007 (triheptanoin) trial in Glut1 DS	2015
Phase II data for KRN23 in pediatric XLH	2015
Phase III data for UX003 (rhGUS) in MPS7	2015

Source: Cowen and Company

Ultragenyx Quarterly P&L

	Q1:13A	Q2:13A	Q3:13A	Q4:13A	2013A	Q1:14a	Q2:14E	Q3:14E	Q4:14E	2014E
KRN23 Revenue										
Triheptanoin Revenue										
rhGUS Revenue										
Collaborative/Grant/Other Revenue										
Total Revenue	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**
Y/Y growth										
COGS	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
GMs										
R&D	5.7	7.2	6.8	8.2	27.8	8.4	10.5	11.0	12.0	41.9
SG&A	1.1	1.0	1.0	1.3	4.5	2.0	3.0	3.0	3.5	11.5
Total Expenses	**6.7**	**8.2**	**7.8**	**9.5**	**32.3**	**10.3**	**13.5**	**14.0**	**15.5**	**53.3**
Operating Income/Loss	**(6.7)**	**(8.2)**	**(7.8)**	**(9.5)**	**(32.3)**	**(10.3)**	**(13.5)**	**(14.0)**	**(15.5)**	**(53.3)**
Interest Income/Expense	0.0	0.1	0.1	0.0	0.2	0.0	0.0	0.0	0.0	0.0
Other Income/Expense	(0.0)	(0.4)	(0.7)	(1.8)	(2.9)	(3.3)	(0.5)	(0.5)	(0.5)	(4.8)
Pre-tax Income/Loss	(6.7)	(8.6)	(8.4)	(11.3)	(35.1)	(13.6)	(14.0)	(14.5)	(16.0)	(58.1)
Tax rate (%)	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%
Provision for income taxes	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Net Income (Loss)	**(6.7)**	**(8.6)**	**(8.4)**	**(11.3)**	**(35.1)**	**(13.6)**	**(14.0)**	**(14.5)**	**(16.0)**	**(58.1)**
Accretion and dividends on convertible preferred stock	(2.6)	(2.6)	(2.6)	(7.3)	(15.2)	(4.8)	0.0	0.0	0.0	(4.8)
Net Income (Loss) attributable to common stockholders	**(9.4)**	**(11.2)**	**(11.1)**	**(18.7)**	**(50.3)**	**(18.4)**	**(14.0)**	**(14.5)**	**(16.0)**	**(62.9)**
GAAP EPS	**($2.87)**	**($3.44)**	**($3.39)**	**($4.98)**	**($14.87)**	**($0.85)**	**($0.44)**	**($0.45)**	**($0.50)**	**($1.98)**
Diluted Shares	3.3	3.3	3.3	3.7	3.4	21.6	31.8	31.9	32.1	29.3

Source: Cowen and Company

Ultragenyx Annual P&L

	2013A	2014E	2015E	2016E	2017E	2018E
KRN23 Revenue	0.0	0.0	0.0	0.0	0.0	0.0
Triheptanoin Revenue	0.0	0.0	0.0	0.0	0.0	20.0
rhGUS Revenue	0.0	0.0	0.0	0.0	11.0	29.0
Collaborative/Grant/Other Revenue	0.0	0.0	0.0	0.0	0.0	0.0
Total Revenue	**0.0**	**0.0**	**0.0**	**0.0**	**11.0**	**49.0**
COGS	0.0	0.0	0.0	0.0	2.0	7.6
GMs					*0.8*	*0.8*
R&D	27.8	41.9	50.0	65.0	70.0	73.0
Total Expenses	**32.3**	**53.3**	**65.5**	**83.0**	**112.0**	**140.6**
	0.0	0.0	0.0	0.0	0.0	0.0
Operating Income/Loss	**(32.3)**	**(53.3)**	**(65.5)**	**(83.0)**	**(101.0)**	**(91.6)**
Interest Income/Expense	0.2	0.0	0.0	0.0	0.0	0.0
Other Income/Expense	(2.9)	(4.8)	(1.0)	(0.3)	(0.3)	(0.3)
Pre-tax Income/Loss	(35.1)	(58.1)	(66.5)	(83.3)	(101.3)	(91.9)
Provision for income taxes	0.0	0.0	0.0	0.0	0.0	0.0
Net Income (Loss)	**(35.1)**	**(58.1)**	**(66.5)**	**(83.3)**	**(101.3)**	**(91.9)**
Accretion and dividends on convertible preferred stock	(15.2)	(4.8)	0.0	0.0	0.0	0.0
Net Income (Loss) attributable to common stockholders	**(50.3)**	**(62.9)**	**(66.5)**	**(83.3)**	**(101.3)**	**(91.9)**
GAAP EPS	**($14.87)**	**($1.98)**	**($1.90)**	**($2.25)**	**($2.60)**	**($2.30)**
Diluted Shares	3.4	29.3	35.0	37.0	39.0	40.0

Source: Cowen and Company

Valuation Methodology And Risks

Valuation Methodology

Biotechnology:
In calculating our 12-month target price, we employ one or more valuation methodologies, which include a discounted earnings analysis, discounted cash flow analysis, net present value analysis and/or a comparable company analysis. These analyses may or may not require the use of objective measures such as price-to-earnings or price-to-sales multiples as well as subjective measures such as discount rates.

We make investment recommendations on early stage (pre-commercial) biotechnology companies based upon an assessment of their technology, the probability of pipeline success, and the potential market opportunity in the event of success. However, because these companies lack traditional financial metrics, we do not believe there are any good methodologies for assigning a specific target price to such stocks.

Investment Risks

Biotechnology:
There are multiple risks that are inherent with an investment in the biotechnology sector. Beyond systemic risk, there is also clinical, regulatory, and commercial risk. Additionally, biotechnology companies require significant amounts of capital in order to develop their clinical programs. The capital-raising environment is always changing and there is risk that necessary capital to complete development may not be readily available.

Risks To The Price Target

Investing in development stage biotechnology companies is risky, and many things could prevent Ultragenyx from achieving the success we model.

Addendum

Stocks Mentioned In Important Disclosures

Ticker	Company Name
RLYP	Relypsa, Inc
SNSS	Sunesis Pharmaceuticals
RARE	Ultragenyx

Analyst Certification

Each author of this research report hereby certifies that (i) the views expressed in the research report accurately reflect his or her personal views about any and all of the subject securities or issuers, and (ii) no part of his or her compensation was, is, or will be related, directly or indirectly, to the specific recommendations or views expressed in this report.

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Cowen and Company, LLC and/or its affiliates received in the past 12 months compensation for investment banking services from Ultragenyx, Relypsa, Inc and Sunesis Pharmaceuticals.

Cowen and Company, LLC and/or its affiliates managed or co-managed a public offering of Ultragenyx, Relypsa, Inc and Sunesis Pharmaceuticals within the past twelve months.

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Cowen and Company Rating System effective May 25, 2013

Outperform (1): The stock is expected to achieve a total positive return of at least 15% over the next 12 months

Market Perform (2): The stock is expected to have a total return that falls between the parameters of an Outperform and Underperform over the next 12 months

Underperform (3): Stock is expected to achieve a total negative return of at least 10% over the next 12 months

Assumption: The expected total return calculation includes anticipated dividend yield

Cowen and Company Rating System until May 25, 2013

Outperform (1): Stock expected to outperform the S&P 500

Neutral (2): Stock expected to perform in line with the S&P 500

Underperform (3): Stock expected to underperform the S&P 500

Assumptions: Time horizon is 12 months; S&P 500 is flat over forecast period

Cowen Securities, formerly known as Dahlman Rose & Company, Rating System until May 25, 2013

Buy – The fundamentals/valuations of the subject company are improving and the investment return is expected to be 5 to 15 percentage points higher than the general market return

Sell – The fundamentals/valuations of the subject company are deteriorating and the investment return is expected to be 5 to 15 percentage points lower than the general market return

Hold – The fundamentals/valuations of the subject company are neither improving nor deteriorating and the investment return is expected to be in line with the general market return

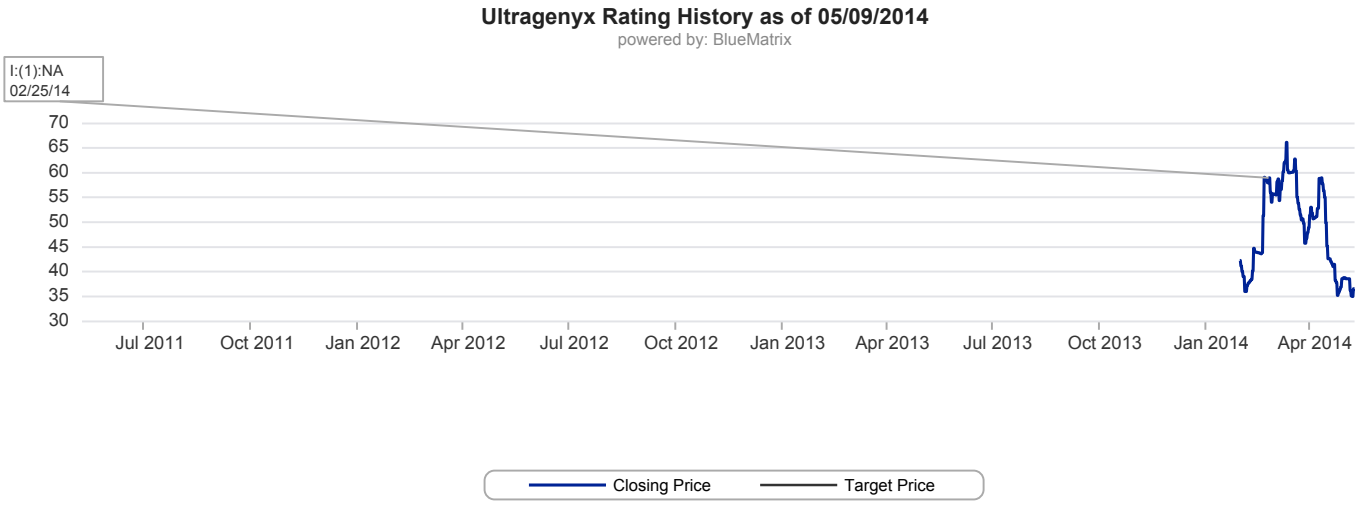
Cowen And Company Rating Definitions

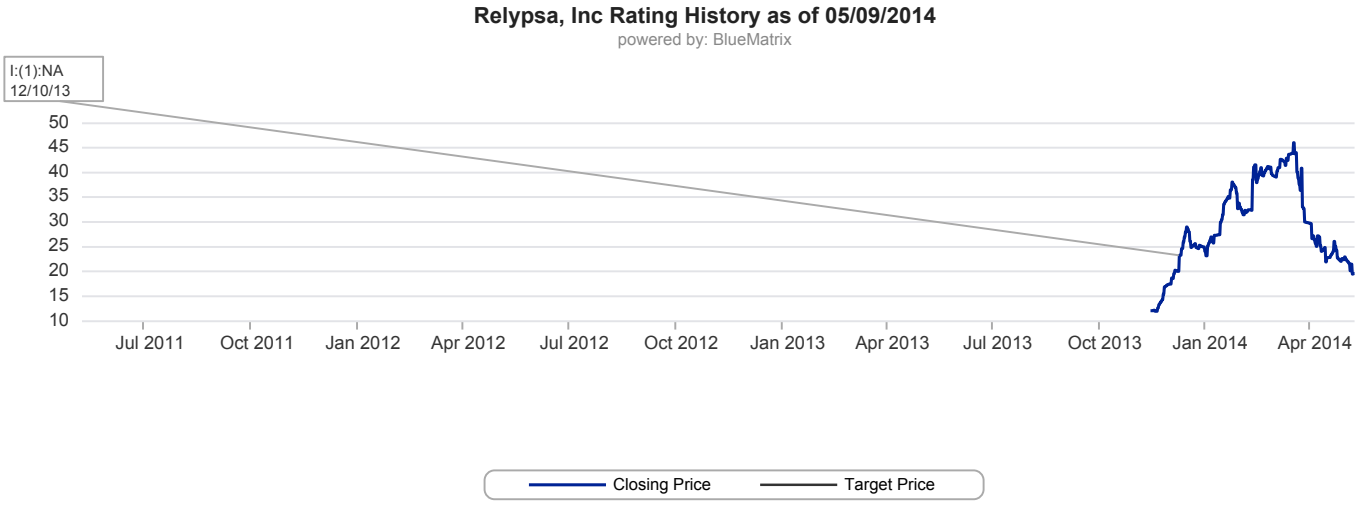
Distribution of Ratings/Investment Banking Services (IB) as of 03/31/14

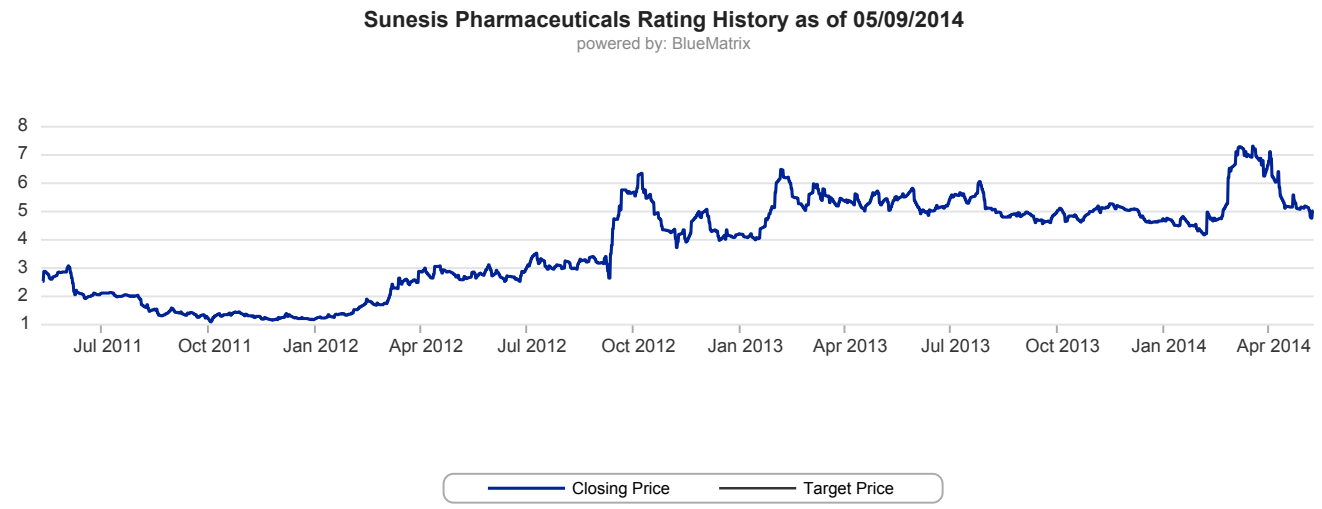
Rating	Count	Ratings Distribution	Count	IB Services/Past 12 Months
Buy (a)	407	57.08%	85	20.88%
Hold (b)	288	40.39%	8	2.78%
Sell (c)	18	2.52%	1	5.56%

(a) Corresponds to "Outperform" rated stocks as defined in Cowen and Company, LLC's rating definitions. (b) Corresponds to "Market Perform" as defined in Cowen and Company, LLC's ratings definitions. (c) Corresponds to "Underperform" as defined in Cowen and Company, LLC's ratings definitions.

Note: "Buy", "Hold" and "Sell" are not terms that Cowen and Company, LLC uses in its ratings system and should not be construed as investment options. Rather, these ratings terms are used illustratively to comply with FINRA and NYSE regulations.







Rating Change - 2/21/2006 - Outperform Rating

Legend for Price Chart:

I = Initation | 1 = Outperform | 2 = Market Perform | 3 = Underperform | UR = Price Target Under Review | T = Terminated Coverage | $xx = Price Target | NA = Not Available | S=Suspended

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