Equity Research

Company Quick Take

April 30, 2014

Price: $38.78 (04/30/2014)

Price Target: NA

OUTPERFORM (1)

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Key Data

Symbol	NASDAQ: RARE
Market Cap (MM)	$1,164.8

SA-ER 48-Week Data Promising, But Not Conclusive

The Cowen Insight

Ultragenyx presented 48-week data from a Phase 2 study of SA-ER in HIBM at the AAN meeting. SA-ER continues to show promise in improving upper extremity strength, though the data come with a few caveats and the drug had limited impact on other endpoints. A dose escalation and further study extension is currently being pursued with data expected in H2:14. RARE remains a top pick.

SA-ER Produces Encouraging Data

The News: This evening, Ultragenyx presented detailed 48-week data from its Phase 2 study of extended release sialic acid (SA-ER) in hereditary inclusion body myopathy (HIBM). HIBM is a progressive muscle myopathy believed to result from dysfunctional sialic acid production. Patients (n=47) were randomized to receive placebo, 3g, or 6g of SA-ER for an initial 24 week period. Placebo patients were then randomized into the 3g or 6g groups and all patients were treated for a further 24 weeks. The 6g SA-ER group showed a 5.5% improvement in upper extremity muscle strength compared to placebo (p=0.04) at 24 weeks. Following the crossover, the 6g group continued to diverge from the 3g group, resulting in an 8.5% (p=0.0033) relative improvement. Within the prospectively identified subset of patients that walked >200m at baseline, 6g of SA-ER performed even better and generated a 9.6% relative improvement at 48 weeks vs. the 3g group (p=0.0006). The functional sit-to-stand test and weighted arm lift test produced trends towards improvement at 48 weeks. Conversely, 6g of SA-ER did not generate meaningful improvements in lower extremity muscle strength tests at both 24 and 48 weeks or in the walking tests. One possible explanation for this is that patients had more substantial baseline dysfunction in the lower extremities (25% of normal strength), so there was less muscle reserve for the drug to impact. A Patient Reported Outcome scale (GNEM-FAS) trended towards improvement with positive contributions from patient reported mobility and upper extremity components, but not self-care. Management remains unsure whether the 6g dose saturates SA's potential effect on HIBM progression. Consequently, all patients are now on 6g of SA-ER plus 6g of immediate release SA. An additional SA naïve cohort is also being enrolled for treatment with 12g of total SA. Data from 12g of total SA is expected in H2 2014.

Our Take: With greater time of follow up SA-ER has generated continued improvement in upper extremity physical function. Ultragenyx believes the FDA is amenable to approving drugs for HIBM on the basis of muscle strength assuming such improvements are clinically relevant. We think an 8% improvement in muscle strength coupled with the trends in GNEM-FAS support the clinical relevance of SA-ER's effect. On the other hand, the data come with the caveats that (1) the patient numbers are small, (2) the 48-week data could be confounded by placebo cross-over, (3) lower extremities do not appear to be experiencing a similar benefit, and (4) biopsy data is inconclusive. We are hopeful that increasing the dose of SA to 12g

while monitoring patients for longer periods of time will provide additional evidence in support of SA-ER's potential.

Our Thesis: Ultragenyx is financed through potential value creating milestones in all of 5 of its Phase 2 orphan disease programs. We expect the stock to outperform as milestones on many of these programs are achieved.

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
RARE	Ultragenyx

Analyst Certification

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

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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

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Cowen And Company Rating Definitions

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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.

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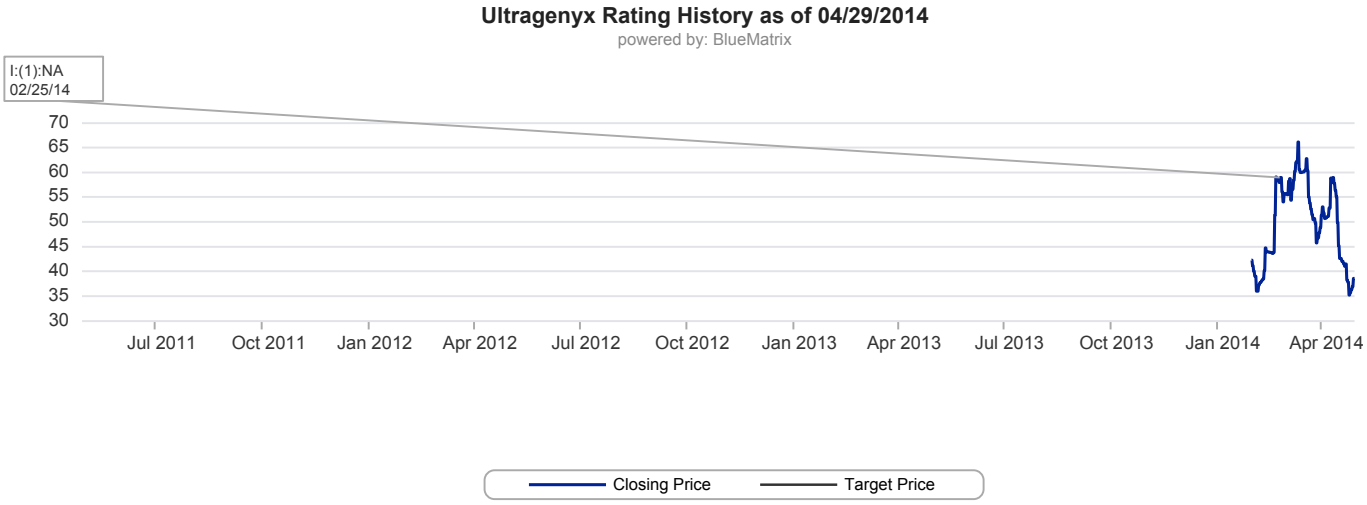

Legend for Price Chart:

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

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