Equity Research

Quick Take: Company Update

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Price: $54.06 (01/9/2015)

Price Target: NA

OUTPERFORM (1)

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Key Data

Symbol	NASDAQ: RARE
Market Cap (MM)	$1,724.4

An Early EMA Filing For SA-ER

The Cowen Insight

Ultragenyx will file an MAA with the EMA seeking conditional approval for SA-ER for patients with HIBM in H2:15. This news is surprising as investors had (1) viewed the Ph. II data on SA-ER somewhat skeptically, and (2) had anticipated Ph. III data would be needed to file. Conditional EU approval in this ~$400MM WW indication would substantially accelerate RARE's path to revenue and profitability.

The News: Based upon Scientific Advice recently received from the EMA's CHMP, Ultragenyx now intends to file an MAA for Syalic Acid Extended Release (SA-ER or UX001) for the treatment of Hereditary Inclusion Body Myopathy (HIBM). Any approval would be conditional, and dependent upon Ultragenyx confirming SA-ER's ability to stabilize upper extremity muscle strength in a Phase III trial. Ultragenyx anticipates filing the MAA in the second half of 2015.

Our Take: Recall that in October, Ultragenyx presented updated Phase II data from its Phase II trial on SA-ER showing a preservation on upper extremity body strength for the 6mg dose at 48 weeks. These data were greeted somewhat skeptically by investors given a limited dose response curve and lack of efficacy on lower extremity endpoints like the six minute walk test. While some may still take a "wait and see" view toward the early MAA filing, it is apparent that the EMA views the Phase II data on SA-ER as significantly more compelling than does the Street. We think there is now a meaningful chance of EMA approval as early as 2016. With the ex-U.S. market anticipated to be larger than the U.S. market, and Ultragenyx owning 100% of WW rights, early EMA approval could substantially accelerate timelines to profitability. We do not include estimates for SA-ER in our model.

Background On HIBM: Hereditary Inclusion Body Myopathy is a rare myopathy believed to occur as a result of mutations that deprive cells of sialic acid required for protein sialization and function. Ultragenyx had previously guided to an incidence of 1,200-2,000 patients in the developed world. However, a recent genetic publication suggests the incidence could be 3X larger. In addition, management has already generated a database of 1,200 identified HIBM patients. Ultragenyx is developing an extended release formulation of sialic acid (SA-ER) for these patients. A Phase II trial of 3, 6, and 12g (6g of SA-ER+6g of immediate release SA) generated positive data in patients with a baseline 6MWT >20m. This trial has demonstrated significant preservation of upper extremity muscle strength in the 6g group relative to either placebo (+5.5%; p=0.040) at 24 weeks or 3g (+8.5%; p=0.0033) of SA-ER at 48 weeks. Importantly, the upper extremity effect using 6g was strongest among patients with a baseline 6MWT of >200m (+9.6%; p=0.00055). Strength preservation was not observed in lower extremities and a 12g trial extension did not further improve efficacy across the population. Nonetheless, management has reported that some patients "clearly" benefited from higher doses and will be kept on the 12g dose during extension trials. In mid-2015, management intends to initiate a global Phase III trial using the 6g dose in ~80 HIBM patients with 6MWT tests >200m. Patients will be

treated for one year. The FDA has accepted Ultragenyx's primary endpoint of upper extremity muscle strength instead of the traditional muscle disorder endpoint of 6MWT. Assuming 2,000 treatable patients and a price of $200K/year, SA-ER might address a worldwide market worth $400MM. Ultragenyx owns full rights.

What is Next For Ultragenyx: Full 36 week data from the Phase I/II trial of rhGUS in MPS7 is scheduled to be presented at the Lysosomal Disease Network's Annual World Symposium in February 2015. During H1:15, management expects to begin an XLH natural history study along with a Phase IIb KRN23 adult XLH trial. Interim 24-week data from KRN23's PII trial in pediatric XLH patients is anticipated for mid-2015. Based upon these results the pediatric trial could be upsized and combined with the natural history trial to support approval. Phase II data from the initial 24-week treatment period of triheptanoin's trial in LC-FAOD is also expected to be released in mid-2015.

Our Thesis On RARE: Ultragenyx is focused on creating value in orphan disorders by advancing a pipeline that features two Phase III and three Phase II programs. Limited H2:14 newsflow has been an overhang on shares, but 2015 promises to be a data rich year. RARE remains a top smid cap pick.

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

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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

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

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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

Distribution of Ratings/Investment Banking Services (IB) as of 12/31/14

Rating	Count	Ratings Distribution	Count	IB Services/Past 12 Months
Buy (a)	461	60.50%	109	23.64%
Hold (b)	288	37.80%	14	4.86%
Sell (c)	13	1.71%	0	0.00%

(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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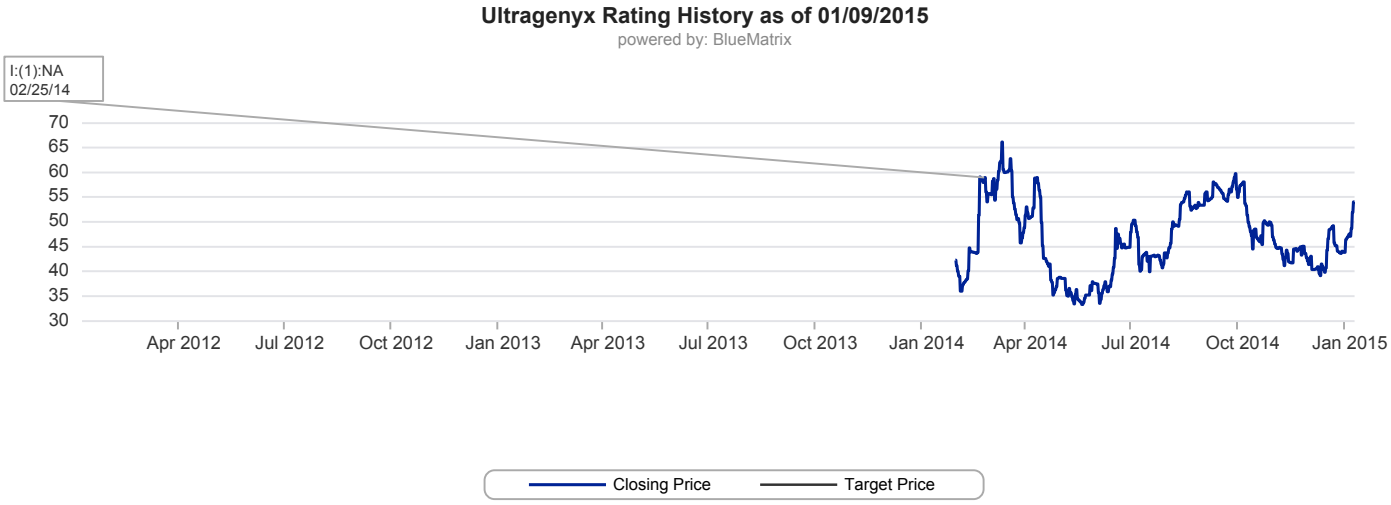

Legend for Price Chart:

I = Initiation | 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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