

Equity Research

September 3, 2014

Price: \$53.41 (09/2/2014)

Price Target: NA

OUTPERFORM (1)

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Key Data

Symbol	NASDAQ: RARE
Market Cap (MM)	\$1,693.3

Company Quick Take

Continued Improvement In MPS7 Patients On rhGUS

The Cowen Insight

Ultragenyx released updated interim data from the Phase 1/2 trial of rhGUS in MPS7. The data shows incrementally increased efficacy compared to March's preliminary data and continued safety consistent with an approvable product. Ultragenyx remains well financed through additional value creating milestones across multiple programs, and we expect the stock to outperform as these are achieved.

The News: Mucopolysaccharidosis Type VII (MPS7) is one of 11 genetic MPS disorders, and afflicts ~200 patients worldwide. MPS7 is caused by a deficiency in beta-glucuronidase which leads to the accumulation of mucopolysaccharides/glycosaminoglycans (GAGs). Today, Ultragenyx released additional interim data from its Phase 1/2 trial with recombinant human beta-glucuronidase (rhGUS) in MPS7. In this study, patients were treated every other week with 2mg/kg of rhGUS for an initial 12 week period, followed by a series of three 8-week dose exploration periods (1, 4, 2mg/kg). Three patients have been treated for the initial 12 week period and a separate eIND patient has completed 24 weeks of treatment. At 12 weeks, urinary GAG (uGAG) excretion declined 40-50% from baselines, while the eIND patient exhibited a 50-70% decline through 24 weeks of treatment. In addition, both study patients with enlarged livers at baseline have experienced liver shrinkage. Similarly, the eIND patient's enlarged liver and spleen have been reduced in size and the patient is demonstrating improved pulmonary function. No serious adverse events, infusion-associated reactions, or significant increases in the immune response to rhGUS have been observed following a total of 38 infusions across all patients. The most common AEs have been infections and GI disorders. Management intends to initiate a pivotal Phase 3 trial by year-end 2014.

Our Take: Enzyme replacement therapies are well established in other MPS disorders (e.g. Aldurazyme and Elaprase). At the March 2014 update, three study patients demonstrated 30-50% uGAG improvement at 2, 6, and 12 weeks respectively. While today's data release includes no additional patients treated, it demonstrates that patients continue to perform well on therapy with longer followup. Therefore, we continue to believe rhGUS will ultimately be successful in its Phase 3 trial which management intends to initiate by year-end 2014. We model a commercial launch beginning in 2017. Given the ultra-rare nature of MPS7 we view rhGUS as an ~\$100MM opportunity inclusive of premium estimated pricing of \$500k/yr.

Upcoming Milestones: Today's data release is the first of three data updates from Ultragenyx over the next six weeks. Longer term dosing (12-dose) data will be presented from KR23's Phase 1/2 trial in XLH at ASBMR on September 14. This data will represent an additional 8 doses compared to June's ICE/ENDO presentation. On October 11, data from the 12g dosage escalation within the Phase 2 SA-ER trial in HIBM will be presented at WMS. Ultragenyx has previously reported improvements in upper extremity muscle strength using a 6g dose.

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

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

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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 06/30/14

Rating	Count	Ratings Distribution	Count	IB Services/Past 12 Months
Buy (a)	417	58.57%	94	22.54%
Hold (b)	279	39.19%	7	2.51%
Sell (c)	16	2.25%	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.

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.

Ultragenyx Rating History as of 09/02/2014

powered by: BlueMatrix



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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