

Equity Research

September 15, 2014

Price: \$57.72 (09/12/2014)

Price Target: NA

OUTPERFORM (1)

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Key Data

Symbol	NASDAQ: RARE
Market Cap (MM)	\$1,830.0

Company Quick Take

KRN23 Continues To Look Good In Phase I/II Extension

The Cowen Insight

12-month extension data were released from Ultragenyx's Phase I/II trial of KRN23 in XLH. Patients largely continued to experience normalized serum phosphorus without the development of anti-KRN23 antibodies or significant hypercalcemia. We believe KRN23 has \$1-2B worldwide sales potential in XLH and remain at Outperform.

The News: Ultragenyx presented longer-term data from the Phase I/II extension trial of KRN23 (anti-FGF23 antibody) in X-linked hypophosphatemia (XLH) at ASBMR. 22 Adult patients previously treated for 4-months as part of the Phase I/II trial (data reported in June 2014) were treated with monthly injections of KRN23 for an additional 12-months. The previously observed increases in serum phosphorus, urinary phosphorus reabsorption, 1,25 dihydroxy Vitamin D₃, and bone remodeling were sustained during the extension study. Specifically, between 52.6% and 85.7% of patients achieved normal serum phosphorus levels (2.5-4.5 mg/dL) following each dose. The most-common adverse events included injection site reactions, arthralgia, diarrhea, restless legs syndrome, headache, and decreased neutrophil count. Three SAEs were reported, but none were considered treatment related. One patient discontinued treatment due to nephrolithiasis (kidney stone) and one discontinued due to restless legs syndrome. Importantly, no significant changes in PTH or serum calcium were observed. Finally, no anti-KRN23 antibodies were detected.

Our Take: The data on KRN23 continues to support the hypothesis that inhibiting FGF23 can normalize serum phosphorus levels in XLH patients. Interestingly, KRN23's ability to increase 1,25 dihydroxy Vitamin D₃ does not appear to be placing patients at significant risk of hypercalcemia as XLH patients have intact calcium homeostasis machinery. In addition, the theoretical concern of anti-KRN23 antibodies continues to be de-risked. Therefore, our conviction that the KRN23 program will be successful is incrementally higher.

Next Steps: Ultragenyx and its partner KHK initiated a Phase IIb trial in pediatric XLH patients in June 2014. Data from this trial are expected in 2015. If successful, management expects to run a Phase III pediatric trial and Phase IIb adult trial concurrently. These could support launches in both indications in 2018. Outside of KRN23, Ultragenyx will present data from the Phase II 12g dose escalation of SA-ER in HIBM on October 11, 2014 at WMS.

Our Thesis: Ultragenyx is financed through potential value creating milestones across multiple programs. We expect the stock to outperform as milestones in 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

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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

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

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 09/12/2014

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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