

Equity Research

June 24, 2014

**Price: \$44.77** (06/23/2014)

**Price Target: NA**

**OUTPERFORM (1)**

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**Key Data**

Symbol **NASDAQ: RARE**

Market Cap (MM) **\$1,345.8**

Company Quick Take

## *More Favorable Data On KRN23*

### **The Cowen Insight**

Ultragenyx presented Phase I/II data from KRN23's trial in adult hypophosphatemia patients at the 2014 ENDO meeting. Data were consistent with that disclosed in abstract form, and indicated that KRN23 reduced serum phosphate without meaningful change in calcium or PTH. We view KRN23 as a low risk candidate with significant >\$1B commercial potential. RARE remains a top pick.

### **SA-ER Produces Encouraging Data**

**The News:** Earlier today, Ultragenyx presented full data from a Phase I/II multi-dose trial on KRN23 at the ENDO meeting in Chicago. KRN23, an mAb directed at FGF23 is being developed for patients with X-linked hypophosphatemia (XLH) under a collaboration with Kirin. Recall that a prior single-dose trial showed the drug capable of maintaining serum phosphate and vitamin D, without significant changes in other blood parameters like calcium and PTH. In this multi-dose trial, 28 adult patients were treated with escalating doses of KRN23 (0.05, 0.1, 0.3, and 0.6 mg/kg) every 28 days for 4 months. Both serum phosphate and reabsorption of phosphate from the urine (TmP/GFR) increased from the baseline at all time points ( $p < 0.05$ ), after the first dose trough. Mean serum phosphorus peaked after the fourth dose to  $3.03 \pm 0.42$  mg/dL representing approximately 60% increase from the mean baseline levels of  $1.89 \pm 0.33$  mg/dL. Serum  $1,25(\text{OH})_2$  Vitamin D also increased from baseline except for the first and second-dose troughs ( $p < 0.05$ ). Quality of life measures also presented improvements. Importantly, no patients experienced serum phosphate above 4.5 mg/dL. Parathyroid hormone, serum calcium and urinary calcium excretion did not exhibit any significant changes. The most common adverse events were nasopharyngitis and arthralgia. There were no serious adverse events and development of anti-KRN23 antibodies was not observed. However, one patient did discontinue KRN23 due to injection site pain.

**Our Take:** These data support the hypothesis that by inhibiting FGF23, the key regulator of potassium excretion, serum phosphate levels in XLH patients can be normalized. The data further de-risk theoretical concerns for KRN23: the development of anti-KRN23 antibodies and the potential for hypercalcemia. We view KRN23 as a "magic bullet" type of approach for XLH and believe this low risk candidate could achieve WW sales of ~\$1B. Ultragenyx is entitled to receive approximately 30% of KRN23's value on a worldwide basis.

**What Is Next For Ultragenyx?** Initiation of pediatric trials on KRN23 (H2:14); Phase I/II data on rhGUS for MPS7 (H2:14); FDA discussions on the design of a pivotal trial for rhGUS (H2:14); Phase II extension data on SA-ER for HIBM (H2:14).

**Our Thesis On RARE:** 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*

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### **Valuation Methodology**

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#### **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**

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#### **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**

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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 Dahlgren 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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**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

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### 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.

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### Ultragenyx Rating History as of 06/23/2014

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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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