

Biotechnology

Ultragenyx

Equity Research

September 15, 2014

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

Price Target: NA

OUTPERFORM (1)

Eric Schmidt, Ph.D.

646.562.1345 eric.schmidt@cowen.com

Marc Frahm, Ph.D.

646.562.1394 marc.frahm@cowen.com

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 D_3 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.

Ultragenyx

September 15, 2014

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:

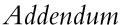
There are multiple risks that are inherent with an investment in the biotechnology sector. Beyond systemic risk, there is also clinical, regulatory, and commercial risk. Additionally, biotechnology companies require significant amounts of capital in order to develop their clinical programs. The capital-raising 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.

www.cowen.com

2



Stocks Mentioned In Important Disclosures

Ticker	Company Name
RARE	Ultragenyx

Analyst Certification

Each author of this research report hereby certifies that (i) the views expressed in the research report accurately reflect his or her personal views about any and all of the subject securities or issuers, and (ii) no part of his or her compensation was, is, or will be related, directly or indirectly, to the specific recommendations or views expressed in this report.

Important Disclosures

Cowen and Company, LLC and or its affiliates make a market in the stock of Ultragenyx securities.

Ultragenyx has been client(s) of Cowen and Company, LLC in the past 12 months.

Cowen and Company, LLC and/or its affiliates expect to receive, or intend to seek, compensation for investment banking services in the next 3 months from Ultragenyx.

Ultragenyx is or was in the past 12 months a client of Cowen and Company, LLC; during the past 12 months, Cowen and Company, LLC provided IB services.

Cowen and Company, LLC and/or its affiliates received in the past 12 months compensation for investment banking services from Ultragenyx.

Cowen and Company, LLC and/or its affiliates managed or co-managed a public offering of Ultragenyx within the past twelve months.

Cowen and Company, LLC compensates research analysts for activities and services intended to benefit the firm's investor clients. Individual compensation determinations for research analysts, including the author(s) of this report, are based on a variety of factors, including the overall profitability of the firm and the total revenue derived from all sources, including revenues from investment banking. Cowen and Company, LLC does not compensate research analysts based on specific investment banking transactions.

Disclaimer

This research is for our clients only. Our research is disseminated primarily electronically and, in some cases, in printed form. Research distributed electronically is available simultaneously to all Cowen and Company, LLC clients. All published research can be obtained on the Firm's client website, https://cowenlibrary.bluematrix.com/client/library.jsp.

Further information on any of the above securities may be obtained from our offices. This report is published solely for information purposes, and is not to be construed as an offer to sell or the solicitation of an offer to buy any security in any state where such an offer or solicitation would be illegal. Other than disclosures relating to Cowen and Company, LLC, the information herein is based on sources we believe to be reliable but is not guaranteed by us and does not purport to be a complete statement or summary of the available data. Any opinions expressed herein are statements of our judgment on this date and are subject to change without notice.

For important disclosures regarding the companies that are the subject of this research report, please contact Compliance Department, Cowen and Company, LLC, 599 Lexington Avenue, 20th Floor, New York, NY 10022. In addition, the same important disclosures, with the exception of the valuation methods and risks, are available on the Firm's disclosure website at https://cowen.bluematrix.com/sellside/Disclosures.action.

Price Targets: Cowen and Company, LLC assigns price targets on all covered companies unless noted otherwise. The price target for an issuer's stock represents the value that the analyst reasonably expects the stock to reach over a performance period of twelve months. The price targets in this report should be considered in the context of all prior published Cowen and Company, LLC research reports (including the disclosures in any such report or on the Firm's disclosure website), which may or may not include price targets, as well as developments relating to the issuer, its industry and the financial markets. For price target valuation methodology and risks associated with the achievement of any given price target, please see the analyst's research report publishing such targets.

Notice to UK Investors: This publication is produced by Cowen and Company, LLC which is regulated in the United States by FINRA. It is to be communicated only to persons of a kind described in Articles 19 and 49 of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005. It must not be further transmitted to any other person without our consent.

Copyright, User Agreement and other general information related to this report

© 2014 Cowen and Company, LLC. Member NYSE, FINRA and SIPC. All rights reserved. This research report is prepared for the exclusive use of Cowen clients and may not be reproduced, displayed, modified, distributed, transmitted or disclosed, in whole or in part, or in any form or manner, to others outside your organization without the express prior written consent of Cowen. Cowen research reports are distributed simultaneously to all clients eligible to receive such research reports. Any unauthorized use or disclosure is prohibited. Receipt and/or review of this research constitutes your agreement not to reproduce, display, modify, distribute, transmit, or disclose to others outside your organization the contents, opinions, conclusion, or information contained in this report (including any investment recommendations, estimates or price targets). All Cowen trademarks displayed in this report are owned by Cowen and may not be used without its prior written consent.

Cowen and Company, LLC. New York (646) 562-1000 Boston (617) 946-3700 San Francisco (415) 646-7200 Chicago (312) 577-2240 Cleveland (440) 331-3531 Atlanta (866) 544-7009 London (affiliate) 44-207-071-7500

COWEN AND COMPANY RATING DEFINITIONS

Cowen and Company Rating System effective May 25, 2013

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

www.cowen.com

Ultragenyx

Equity Research

September 15, 2014

Sell – The fundamentals/valuations of the subject company are deteriorating and the investment return is expected to be 5 to 15 percentage points lower than the general market return

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

Cowen And Company Rating Definitions

Distribution of Ratings/Investment Banking Services (IB) as of 06/30/14

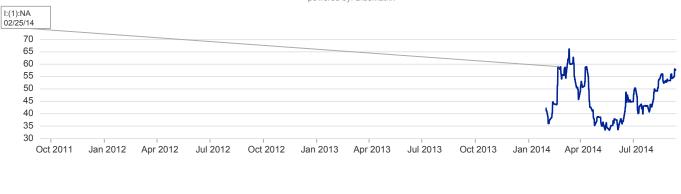
Rating	Count	Ratings Distribution	Count	IB Services/Past 12 Months
Buy (a)	417	58.57%	94	22.54%
Hold (b)	279	39.19%	7	2.51%
Sell (c)	16	2.25%	0	0.00%

(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

Equity Research

Ultragenyx

September 15, 2014

Points Of Contact

Reaching Cowen

Main U.S. Locations

New York

599 Lexington Avenue New York, NY 10022 646.562.1000 800.221.5616

Atlanta

3399 Peachtree Road NE Suite 417 Atlanta, GA 30326

866.544.7009

Boston

Two International Place Boston, MA 02110 617.946.3700 800.343.7068

Chicago

181 West Madison Street Suite 1925 Chicago, IL 60602 312.577.2240

Cleveland

20006 Detroit Road Suite 100 Rocky River, OH 44116 440.331.3531

San Francisco

555 California Street, 5th Floor San Francisco, CA 94104 415.646.7200 800.858.9316

International Locations

Cowen International

Limited

London

1 Snowden Street - 11th Floor London EC2A 2DQ **United Kingdom** 44.20.7071.7500

Cowen and Company (Asia)

Limited

Hong Kong

Suite 1401 Henley Building No. 5 Queens Road Central Central, Hong Kong 852 3752 2333







www.cowen.com