Ultragenyx Pharmaceutical, Inc. (RARE)

FDA & EMA Sign-Off on rhGUS Phase III; Possible New Indications for KRN23

Buy

Price Target: $82.00
Prior: $82.00

Price (Dec. 12, 2014)	$41.48
52-Wk Range	$66.18-$33.36
Market Cap ($M)	$1,311
ADTV	284,628
Shares Out (M)	31.6
Short Interest Ratio/% Of Float	8.2%
TR to Target	97.7%

Cash Per Share	$5.16
Total Debt	$0.0
Cash And Equivalents ($M)	$201.0

	2013A	2014E		2015E	
		Curr.	Prior	Curr.	Prior
EPS Adjusted					
1Q	(3.36)	(0.85)A	(0.85)	(0.68)	(0.68)
2Q	(3.23)	(0.45)A	(0.45)	(0.59)	(0.59)
3Q	(2.58)	(0.50)A	(0.50)	(0.62)	(0.62)
4Q	(4.98)	(0.52)	(0.52)	(0.65)	(0.65)
FY	(14.15)	(2.32)	(2.32)	(2.53)	(2.53)
P/E	NM	NM		NM	
Revenue ($M)					
FY	$0	$0	$0	$0	$0
Consensus EPS					
FY	($11.25)	($2.07)A	($2.07)	($2.27)	($2.27)
Consensus Rev					
FY	$0	$0	$0	$0	$0
FYE Dec					

What's Incremental
Today, RARE reported the FDA & EMA have agreed on the pivotal trial design for rhGUS in MPS 7. We view this development as encouraging as the FDA acknowledges that the heterogeneity and rarity of the disease necessitates evaluation on a per-patient basis. Additionally, we report that KRN23 is in a small Phase II proof of concept trial in 2 diseases (TIO & ENS) where a tumor/lesion may secrete FGF23 to cause symptoms of rickets.

First patient dosed & FDA/EMA agreement for rhGUS Phase III in MPS 7. The Phase III study will entail only U.S. sites but its design satisfies global regulatory requirements, and will enroll 12 patients in a blind-start across 4 dose-cohorts. The first cohort will begin rhGUS therapy immediately, while the other 3 will start on placebo and then cross over to drug at predefined time points in a blinded manner. RARE has chosen to move forward with the 4 mg/kg dose based on internal assessment of the full 36-week data from the previous Phase I/II (to be presented at WORLD 2015). The trial will last 48 weeks, with the minimum duration of dosing at 24 weeks on drug. While the EMA had already agreed that approval is possible with a single Phase III using the primary endpoint of a reduction in urinary GAG (sugars) with supporting trends in improvement in 6MWT, FEV1, and spleen/liver volume, the FDA advised against the declaration of a single primary endpoint to allow for greater flexibility in evaluation of efficacy on a per patient basis in light of the heterogeneous/rare nature of the disease. We expect data in early 2016.

KRN23 is being studied in tumor-induced osteomalacia (TIO) and epidermal nevus syndrome (ENS). Clinicaltrials.gov indicates that a Phase II trial is planned to study KRN23 in 2 disorders in which a tumor or a nevus (benign lesion of skin) is believed to secrete FGF23, thus causing hypophosphatemia and the symptoms of rickets (pain in the spine, pelvis, and legs, muscle weakness, etc.). Inclusion criteria include evidence of excessive FGF23 levels (>=2X the upper limit of normal) that are not amenable to curative surgery by removal of the tumor/lesion. In TIO, we estimate that ~50% of patients are inoperable due to small benign tumors that may be hard to locate or are in difficult locations such as the jaw that could require removal of healthy tissue in surgery. TIO is likely RARE's main focus over ENS due to more severe rickets symptoms. In our view, the rationale for this study is strong as KRN23 has demonstrated the ability to decease circulating levels of FGF23 which is believed to be the key cause

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SEE PAGE 3 FOR REQUIRED DISCLOSURE INFORMATION

of both TIO and ENS. Two anecdotal case studies reported elevated FGF23 in the blood of one TIO and one ENS patient. The trial will enroll 6 patients, with data expected in April 2016. In terms of prevalence, literature on TIO is limited, but the condition is likely a lot rarer than XLH the main indication for KRN23.

Company Description

Ultragenyx is developing therapies for ultra-rare diseases, with 4 drugs in development for 5 different indications. The company focuses on identification, acquisition, development, and commercialization of new products for the treatment of ultra-rare diseases with a focus on metabolic, genetic and under-served diseases.

Investment Thesis

RARE boasts a portfolio of mid- to late-stage clinical assets to tackle genetic ultra-orphan diseases. With 4 clinical stage therapies developed for 5 indications, we anticipate a slew of data catalysts in 2015, with potential product approvals in 2017/18. **Physician feedback suggests all programs have good clinical probabilities of success.**

Valuation and Risks

Valuation:

We arrive at our 12-month price target of $82 by means of a sum-of-the-parts discounted cash flow analysis, which ascribes $23.60/share to KRN23, $8.10/share to rhGUS, $42.90/share to triheptanoin, $2.00/share to SA-ER and $5.72/share in cash, with the following assumptions: we assign KRN23 a 75% probability of success, rhGUS an 85% probability of success, triheptanoin a 60% probability of success and SA-ER a 50% probability of success. We assign a discount rate of 10% to KRN23, triheptanoin, and SA-ER, and a 9% discount rate to rhGUS. We assume a 2% terminal growth rate for KRN23, triheptanoin, and SA-ER, and a 3% terminal growth rate for rhGUS

The primary investment risks for Ultragenyx include the following:

- Clinical development risk: There is some uncertainly as to whether earlier stage experiments (preclinical, Phase I, or historical case reports) will translate into efficacy in Phase III.
- Regulatory risk: Even upon successful clinical data, the FDA may not view the results as worthy of regulatory approval for commercial sale. In particular, we highlight the risk of the FDA not accepting certain biomarker endpoints for the accelerated approval pathway.
- Commercial risk: Each product may fail to achieve revenues in line with our peak estimates in the commercial market.
- Competitive risk: The emergence of competing therapies may reduce Ultragenyx's market share.
- Partnership risk: Ultragenyx relies on partnerships for some of its therapies, KRN23 in particular. KHK manufactures and will commercialize the product in the E.U., thus Ultragenyx is vulnerable to shortcomings of their partners.
- Management risk: Ultragenyx has assembled a team of all-stars from the rare disease world, especially the CEO, Emil Kakkis. If certain employee were to leave at inopportune times, it may damage the company's chances of success.
- Financing risk: As a development stage company, Ultragenyx is not profitable and may not turn profitable in the near future. Future financings may be required to develop drugs, which may dilute existing shareholders.

Analyst Certification

I, Salveen Richter , hereby certify that the views expressed in this research report accurately reflect my personal views about the subject company(ies) and its (their) securities. I also certify that I have not been, am not, and will not be receiving direct or indirect compensation in exchange for expressing the specific recommendation(s) in this report.

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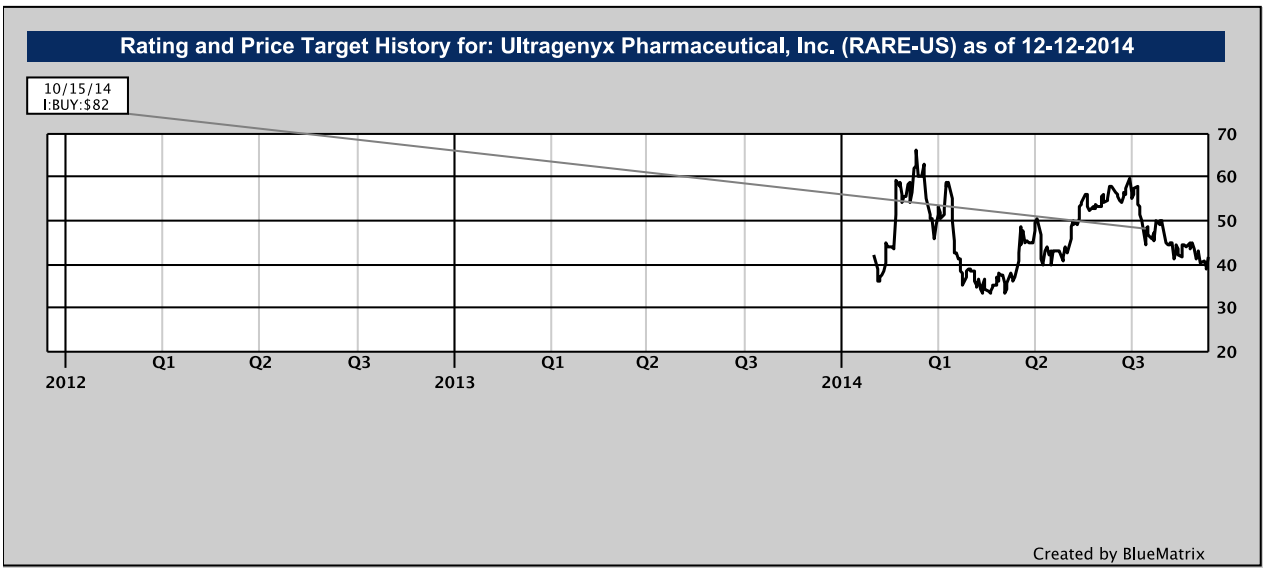

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3 designations based on total returns* within a 12-month period**

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- **Reduce** – total return ≤ negative 10% (5% for low Beta securities)
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*Total return (price appreciation + dividends)
**Price targets are within a 12-month period, unless otherwise noted
***Low Beta defined as securities with an average Beta of 0.8 or less, using Bloomberg's 5-year average Beta

Legend for Rating and Price Target History Charts:
D = drop coverage
I = initiate coverage
T = transfer coverage

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