Ultragenyx Pharmaceutical, Inc. (RARE)

Takeaways from Management Meeting

Buy

Price Target: $82.00
Prior: $82.00

Price (Jan. 14, 2015)	$53.99
52-Wk Range	$66.18-$33.36
Market Cap ($M)	$1,706
ADTV	246,643
Shares Out (M)	31.6
Short Interest Ratio/% Of Float	8.6%
TR to Target	51.9%

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
We met with RARE management and discussed the outlook for the company in 2015 as all 6 clinical programs are advancing nicely. We believe RARE is a name to own in 2015, given 1) Phase I 36-week data for rhGUS in MPS 7 at the WORLD meeting (pivotal dose of 4 mg/kg), 2) 16-week safety and serum phosphorous levels from the pediatric Phase II study of KRN23 in X-linked hypophosphatemia (XLH) in H1/15 and 40-week data for KRN23 by YE15/ early 16, 3) readout of interim data in two Phase II studies of triheptanoin, in Glut1 DS and LC-FAOD in H2/15 and 4) KRN23 TIO interim Phase II data in late-15.

SA-ER could have a faster than expected path to market in the E.U. RARE intends to file an MAA in the E.U. in 2H/15 for sialic acid extended-release (SA-ER) dosed at 6g/day, for the rare disease hereditary inclusion body myopathy (HIBM). Per management, submission will include encouraging data on upper extremity (UE) muscle strength from a Phase II study, natural history and long term extension data and the label will likely be restricted to benefit on UE muscle strength. RARE's strategy is to secure conditional approval in the E.U., while a confirmatory Phase III study of SA-ER (6g/day) in HIBM is expected to start mid-2015 (global, randomized, double-blind, placebo-controlled). This study is already in place to support U.S. approval. With the earlier E.U. submission timelines, SA-ER could get conditional approval in 2H/16 vs our estimate of ~2018.

RARE provided color on the Phase II study of KRN23 in tumor-induced osteomalacia (TIO). This bone softening disease is the result of benign tumors that produce excess FGF23. Thus, TIO patients experience hypophosphatemia, fractures, bone and muscle pain. The study will enroll 6 adult TIO patients in H1/15, to be given different doses of the FGF23 antibody KRN23. Efficacy will be established using radiographic assessments and measures of: 1) muscle strength, 2) walking ability, and 3) patient reported measures of pain, disability, and quality of life. The trial will also assess changes in biomarkers such as serum phosphorus, and includes 2 parts: 1) a 16-week individual dose finding portion to achieve target phosphorus levels and 2) a 28 week period of monthly subQ dosing. Interim safety and efficacy data are expected by YE15. RARE estimates a 5-10K U.S. prevalence for TIO, smaller compared to the lead indication of XLH.

The Phase II study of KRN23 is fully enrolled, interim serum phosphorous data expected in H1/15. Management now anticipates interim 16-week serum phosophorous data from the ongoing Phase II study of KRN23 in pediatric XLH in H1/15. Full data are expected by YE15/early 2016. A Phase IIb is being designed with input from the FDA and the EMA.

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Triheptanoin could surprise in Huntington's disease (HD). RARE is planning a study of triheptanoin in HD after it licensed the IP from INSERM for this disease. Medical literature includes a pilot study of 10 patients treated with dietary triheptanoin for one month, Results were suggestive of improvement in disease scores via impact on energy metabolism. A randomized, double-blind, placebo-controlled IST is slated to evaluate triheptanoin in ~100 HD patients. Should the data demonstrate benefit, the study could be used for product registration in this indication.

Several catalysts could support RARE share growth in 2015. We look towards Phase I/II 36-week data for rhGUS (at the pivotal dose of 4 mg/kg) in MPS 7 at the WORLD Symposium (Feb 9-13, Orlando). 16-week phosphate and safety data from the Phase II pediatric study of KRN23 in XLH are expected in H1/15, while full 40-week data could readout YE15/early 2016. The Phase II studies of triheptanoin (in FAODD and Glut1 DS) are expected in H2/15. Lastly, the Phase II study of KRN23 in TIO will report out in late-15.

Ultragenyx Pharmaceutical

(NASDAQ: RARE)

Consolidated Income Statement

($thousands, except per share data)

	FY 2013A	Mar Q1 2014A	Jun Q2 2014A	Sep Q3 2014A	Dec Q4 2014E	FY 2014E	Mar Q1 2015E	Jun Q2 2015E	Sep Q3 2015E	Dec Q4 2015E	FY 2015E	FY 2016E	FY 2017E	FY 2018E	FY 2019E	FY 2020E
Revenue																
KRN23 revenue booked by RARE	-	-	-	-	-	-	-	-	-	-	-	-	-	-	2,796	31,835
rhGUS revenue	-	-	-	-	-	-	-	-	-	-	-	-	617	13,962	32,172	47,022
Triheptanoin revenue	-	-	-	-	-	-	-	-	-	-	-	-	-	617	22,713	54,412
SA-ER revenue	-	-	-	-	-	-	-	-	-	-	-	-	-	953	5,245	13,248
Total product revenue	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ 617**	**$ 15,532**	**$ 62,926**	**$ 146,517**
KRN23 U.S./Canada profit share/royalty	-	-	-	-	-	-	-	-	-	-	-	-	-	172	8,102	22,761
KRN23 E.U. royalty	-	-	-	-	-	-	-	-	-	-	-	-	-	-	883	6,020
SA-ER ex-U.S. royalty	-	-	-	-	-	-	-	-	-	-	-	-	-	25	124	298
Total profit share / royalty	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ 197**	**$ 9,109**	**$ 29,080**
Total Revenue	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ 617**	**$ 15,729**	**$ 72,034**	**$ 175,597**
COGS	-					-					-	-	100	10,702	3,439	21,308
Gross profit	-	-	-	-	-	-	-	-	-	-	-	-	517	5,026	68,595	154,289
Operating expense																
R&D (GAAP)	27,829	8,353	11,239	12,854	14,445	46,891	15,045	16,543	17,512	18,552	67,652	86,232	95,923	102,332	109,223	116,232
SG&A (GAAP)	4,451	1,986	2,422	2,981	3,111	10,500	3,211	3,454	3,654	4,002	14,321	17,388	22,555	27,017	29,765	32,888
Stock-based compensation	657	795	946	3,393	3,511	8,645	3,555	3,650	3,777	4,001	14,983	16,503	18,009	19,554	21,007	23,005
Total operating expense (GAAP)	32,280	10,339	13,661	15,835	17,556	57,391	18,256	19,997	21,166	22,554	81,973	103,620	118,478	129,349	138,988	149,120
Operating income (loss)	**(32,280)**	**(10,339)**	**(13,661)**	**(15,835)**	**(17,556)**	**(57,391)**	**(18,256)**	**(19,997)**	**(21,166)**	**(22,554)**	**(81,973)**	**(103,620)**	**(117,961)**	**(124,323)**	**(70,393)**	**5,169**
Interest income	216	93	149	171	187	600	174	159	143	198	673	879	932	1,384	1,229	1,716
Interest expense	-	-	-	-	-	-	-		-	-	-	-	-	-	-	-
Other income (expense), net	(3,006)	(3,384)	(73)	(185)	-	(3,642)	-	-	-	-	-	-	-	-	-	-
Total other (expense) income, net	(2,790)	(3,291)	76	(14)	187	(3,042)	174	159	143	198	673	879	932	1,384	1,229	1,716
Net gain (loss) before taxes	**(35,070)**	**(13,630)**	**(13,585)**	**(15,849)**	**(17,369)**	**(60,433)**	**(18,082)**	**(19,838)**	**(21,023)**	**(22,356)**	**(81,300)**	**(102,741)**	**(117,028)**	**(122,939)**	**(69,164)**	**6,885**
Income Tax Provision	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Net income (loss) attributable to common stockholders	**$ (42,338)**	**$ (18,438)**	**$ (13,585)**	**$ (15,849)**	**$ (17,369)**	**$ (65,241)**	**$ (22,890)**	**$ (19,838)**	**$ (21,023)**	**$ (22,356)**	**$ (86,108)**	**$ (102,741)**	**$ (117,028)**	**$ (122,939)**	**$ (69,164)**	**$ 6,885**
EPS (basic and diluted)	**$ (14.16)**	**$ (0.85)**	**$ (0.45)**	**$ (0.50)**	**$ (0.52)**	**$ (2.32)**	**$ (0.68)**	**$ (0.59)**	**$ (0.62)**	**$ (0.65)**	**$ (2.53)**	**$ (2.83)**	**$ (3.02)**	**$ (3.14)**	**$ (1.75)**	**$ 0.17**
Weighted shares outstanding diluted	3,763	21,582	30,056	31,631	33,562	29,208	33,730	33,899	34,068	34,239	33,984	36,341	38,704	39,092	39,482	40,666
Margin Analysis:																
Cost of goods sold	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	16%	69%	5%	15%
KRN23	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	0%	12%	20%	25%
rhGUS	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	0%	1%	2%	14%
Triheptanoin	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	0%	4%	8%	10%
SA-ER	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	0%	4%	8%	10%
Gross margin	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	31%	95%	85%
R&D (GAAP)	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	651%	152%	66%
SG&A (GAAP)	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	172%	41%	19%
Total operating expense	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	822%	193%	85%
Operating margin	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A
Income tax provision	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%
Net margin (GAAP)	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A
Y/Y change:																
Total revenue	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	2447%	358%	144%
KRN23 revenue	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	1039%
rhGUS revenue	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	2161%	130%	46%
Triheptanoin revenue	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	3584%	140%
SA-ER revenue	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	450%	153%
Total profit share / royalty	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	4534%	219%
KRN23 U.S. / Canada profit share / royalty	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	4622%	181%
KRN23 E.U. royalty	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	582%
SA-ER ex-U.S. royalty	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	N/A	395%	141%
R&D (GAAP)	120%	N/A	N/A	N/A	N/A	68%	N/A	N/A	N/A	N/A	44%	27%	11%	7%	7%	6%
SG&A (GAAP)	33%	N/A	N/A	N/A	N/A	136%	N/A	N/A	N/A	N/A	36%	21%	30%	20%	10%	10%
Total operating expense	102%	N/A	N/A	N/A	N/A	78%	N/A	N/A	N/A	N/A	43%	26%	14%	9%	7%	7%
Operating income	102%	N/A	N/A	N/A	N/A	78%	N/A	N/A	N/A	N/A	43%	26%	14%	5%	-43%	-107%
Net income (loss)	116%	N/A	N/A	N/A	N/A	54%	N/A	N/A	N/A	N/A	32%	19%	14%	5%	-44%	-110%
GAAP EPS (diluted)	-10%	N/A	N/A	N/A	N/A	-84%	N/A	N/A	N/A	N/A	9%	12%	7%	4%	-44%	-110%
Shares outstanding - GAAP	173%	N/A	N/A	N/A	N/A	676%	N/A	N/A	N/A	N/A	16%	7%	7%	1%	1%	3%

Sources: Company reports, STRH Research

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.

Companies Mentioned in This Note

Ultragenyx Pharmaceutical, Inc. (RARE, $53.99, Buy)

Analyst Certification

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

- **Buy** – total return ≥ 15% (10% for low-Beta securities)***
- **Reduce** – total return ≤ negative 10% (5% for low Beta securities)
- **Neutral** – total return is within the bounds above
- **NR** – NOT RATED, STRH does not provide equity research coverage
- **CS** – Coverage Suspended

*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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Coverage Universe			Investment Banking Clients Past 12 Months		
Rating	**Count**	**Percent**	**Rating**	**Count**	**Percent**
Buy	285	52.58%	Buy	96	33.68%
Neutral	250	46.13%	Neutral	39	15.60%
Sell/Reduce	7	1.29%	Sell/Reduce	0	0.00%

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