Ultragenyx Pharmaceutical, Inc. (RARE)

Marketing Meetings Unearth Pipeline Color

Buy

Price Target: $82.00
Prior: $82.00

Price (Nov. 5, 2014)	$44.59
52-Wk Range	$66.18-$33.36
Market Cap ($M)	$1,342
ADTV	424,605
Shares Out (M)	30.1
Short Interest Ratio/% Of Float	7.8%
TR to Target	83.9%

Cash Per Share	$7.08
Total Debt	$0.0
Cash And Equivalents ($M)	$153.0

	2013A	2014E		2015E	
		Curr.	Prior	Curr.	Prior
EPS Adjusted					
1Q	(3.36)	(0.85)A	(0.85)	(0.82)	(0.82)
2Q	(3.23)	(0.45)A	(0.45)	(0.62)	(0.62)
3Q	(2.58)	(0.47)	(0.47)	(0.65)	(0.65)
4Q	(4.98)	(0.55)	(0.55)	(0.69)	(0.69)
FY	(14.15)	(2.23)	(2.23)	(2.77)	(2.77)
P/E	NM	NM		NM	
Revenue ($M)					
FY	$0	$0	$0	$0	$0
Consensus EPS					
FY	($11.25)	($2.02)A	($2.02)	($2.16)	($2.16)
Consensus Rev					
FY	$0	$0	$0	$0	$0
FYE Dec					

What's Incremental

We recently marketed with RARE's CEO, Emil Kakkis. Our key takeaways relate to clarity on regulatory endpoints for rhGUS in MPS VII and SA-ER in HIBM, and a diagnostic test initiative for Glut1DS. We note data catalysts from 4 clinical programs in 2015.

Management confident in path to FDA approval for rhGUS in MPS VII. The EMA has agreed to a Phase III primary endpoint of reduction in urinary sugar (uGAG) levels, while also likely looking to a positive trend in a secondary endpoint. However, the FDA will not accept uGAG and is in discussions with RARE on an individualized clinical response (ICR) score that will measure a patient's disease severity - which CEO believes will satisfy the FDA.

For SA-ER in HIBM, management expects FDA to accept upper body strength as the Phase III endpoint. RARE is proposing a Phase III (initiate in mid-15 at 6mg dose) primary endpoint of upper extremity composite measure (studied in the Phase II). Management explained that the loss of autonomy that results from declining upper body function is devastating as patients are unable to brush their own teeth, work on a computer, etc. In addition, pharmacoeconomically, loss of lower body strength (patient is wheelchair-bound but autonomous), is less costly than loss of upper-body strength (requires 24/7 care). RARE plans to use patient-reported outcomes (secondary endpoint) to support this argument.

RARE is rolling out a diagnostic program for Glut1DS. For the triheptanoin (Phase II) program, RARE intends to provide free access to a diagnostic sequencing test, which will identify the mutated Glut1 gene (inner cheek swab) in refractory epilepsy patients, to neurologists. We note the two other diagnosis methods are laborious and expensive (Dr. DeVivo's (Columbia U.) red blood cell assay testing for Glut1 function), or highly invasive (lumbar puncture to measure sugar concentration in cerebrospinal fluid). The sequencing service has been contracted out to a third party. We expect this diagnosis effort to aid in further patient identification.

Undisclosed second preclinical asset; no problem finding additional pipeline products. RARE has another preclinical pipeline asset (in addition to rhPPCA) that has yet to be disclosed. While the CEO is aware of multiple low-cost assets from academic centers, RARE's current focus is on developing the bustling existing pipeline. We believe RARE's business development relationships combined with regulatory expertise in ultra-rare disease should bode well for a continued diversified pipeline.

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SEE PAGE 2 FOR REQUIRED DISCLOSURE INFORMATION

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, $44.59, Buy)

Analyst Certification

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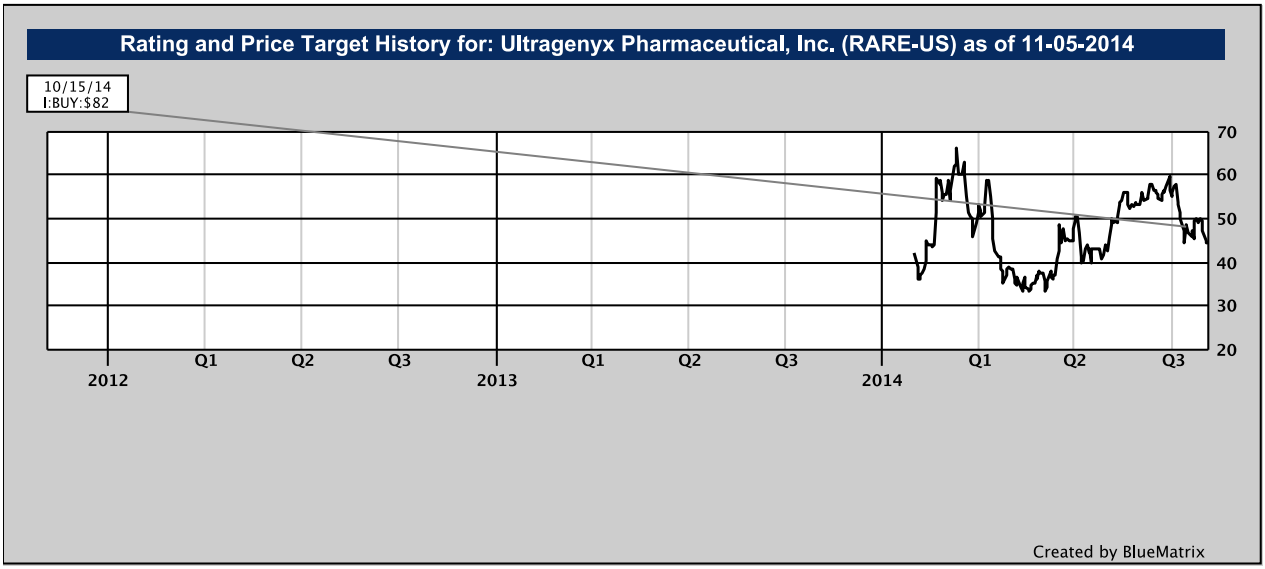

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- **Reduce** – total return ≤ negative 10% (5% for low Beta securities)
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*Total return (price appreciation + dividends)
**Price targets are within a 12-month period, unless otherwise noted
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Legend for Rating and Price Target History Charts:
D = drop coverage
I = initiate coverage
T = transfer coverage

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