

Morgan Stanley & Co. LLC

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Stock Rating  
**Overweight**

Industry View  
**In-Line**

## Ultragenyx Pharmaceutical Inc

### Quick Take on SA-ER

**SA-ER 12g/day data shows no clinically meaningful advantage over 6g/day dosing:** Ultragenyx presented the results from a PhII extension study of SA-ER (sialic acid extended-release) in HIBM (hereditary inclusion body myopathy) pts at the WMS (World Muscle Society) today. The results included 49 out of 59 pts who had 24 weeks of data at the 12g/day dose. Pts receiving the higher dose appeared to experience a higher rate of mild to moderate GI AEs, however no drug-related SAEs were reported. Mgt. plans to move ahead with the program and discuss a PhIII program with regulators.

**Upside case for SA-ER now off the table:** As a reminder, we had previously highlighted that if SA-ER were able to demonstrate a benefit in lower limb function with the 12mg data we would consider adding it to our model. Today's data clearly demonstrated no add'l benefit with the higher dose and thus that status quo remains.

**Milestones ahead for RARE:** Key upcoming milestones for the company include **(1)** KRN23 pediatric and adult studies with radiographic markers in 2015, a sig. milestone for the KRN23 program, in our view. **(2)** On triheptanoin, interim PhII data in severely affected LC-FAOD pts is expected in 2015. **(3)** Interim data from the PhII study of triheptanoin in Glut-1 DS are also expected in 2015. **(4)** Finally, Ultragenyx plans to initiate a pivotal PhIII study of rhGUS in MPS 7 by YE14.

**We continue not to model SA-ER:** While some had hoped that (and we highlighted the possibility that) the 12mg dose could achieve lower limb function it clearly did not which is disappointing. This add'l data does not change our view that the benefits of SA-ER are modest and therefore do not model.

### Key Ratios and Statistics

Reuters: RARE.O Bloomberg: RARE US

Biotechnology / United States of America

Shr price, close (Oct 10, 2014)	\$49.90
Mkt cap, curr (mm)	\$1,572
52-Week Range	\$69.77-32.02

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(as of September 30, 2014)

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Stock Rating Category	Coverage Universe		Investment Banking Clients (IBC)		
	Count	% of Total	Count	% of Total IBC	% of Rating Category
<b>Overweight/Buy</b>	<b>1113</b>	<b>35%</b>	<b>353</b>	<b>40%</b>	<b>32%</b>
<b>Equal-weight/Hold</b>	<b>1390</b>	<b>44%</b>	<b>410</b>	<b>47%</b>	<b>29%</b>
<b>Not-Rated/Hold</b>	<b>109</b>	<b>3%</b>	<b>21</b>	<b>2%</b>	<b>19%</b>
<b>Underweight/Sell</b>	<b>575</b>	<b>18%</b>	<b>96</b>	<b>11%</b>	<b>17%</b>
<b>Total</b>	<b>3,187</b>		<b>880</b>		

Data include common stock and ADRs currently assigned ratings. Investment Banking Clients are companies from whom Morgan Stanley received investment banking compensation in the last 12 months.

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Overweight (O). The stock's total return is expected to exceed the average total return of the analyst's industry (or industry team's) coverage universe, on a risk-adjusted basis, over the next 12-18 months.

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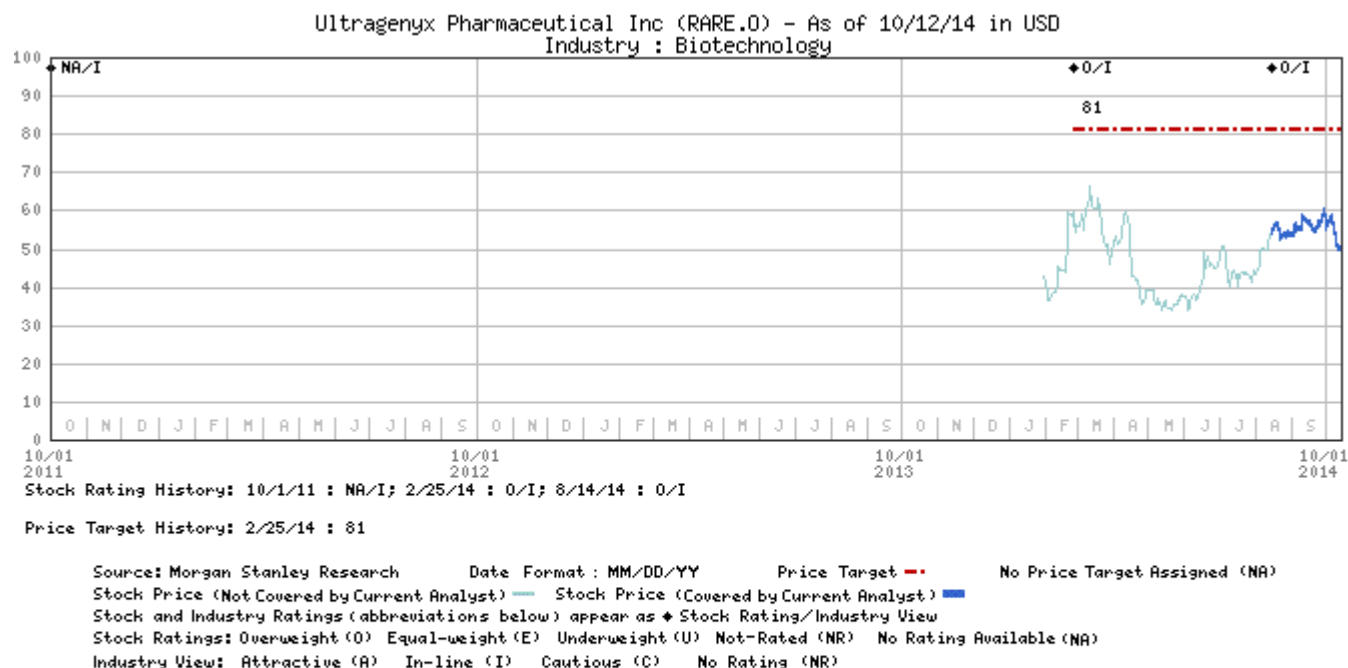
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## Stock Price, Price Target and Rating History (See Rating Definitions)

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## Industry Coverage:Biotechnology

Company (Ticker)	Rating (as of)	Price* (10/10/2014)
<b>Matthew Harrison</b>		
Alexion Pharmaceuticals (ALXN.O)	O (08/14/2014)	\$167.8
Amgen Inc. (AMGN.O)	O (03/26/2014)	\$137.39
Biogen Idec Inc. (BIIB.O)	O (03/26/2014)	\$315.14
Celgene Corp (CELG.O)	E (03/26/2014)	\$90.92
Chimerix Inc (CMRX.O)	O (08/14/2014)	\$31.06
Cubist Pharmaceuticals Inc. (CBST.O)	O (08/14/2014)	\$63.19
Gilead Sciences Inc. (GILD.O)	E (03/26/2014)	\$103.73
ImmunoGen Inc. (IMGN.O)	U (06/25/2014)	\$8.93
Infinity Pharmaceuticals Inc (INFI.O)	O (06/25/2014)	\$12.51
Ironwood Pharmaceuticals, Inc. (IRWD.O)	E (08/14/2014)	\$11.97
Ophthotech Corp (OPHT.O)	O (08/14/2014)	\$37.55
Pharmacycics Inc. (PCYC.O)	E (03/26/2014)	\$105.97
Portola Pharmaceuticals Inc (PTLA.O)	O (08/14/2014)	\$25.63
Regeneron Pharmaceuticals Inc. (REGN.O)	E (03/26/2014)	\$343.51
Theravance Inc (THR.X.O)	U (08/14/2014)	\$16.24
Ultragenyx Pharmaceutical Inc (RARE.O)	O (08/14/2014)	\$49.9
Vertex Pharmaceuticals (VRTX.O)	E (08/14/2014)	\$104.73

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\* Historical prices are not split adjusted.