

January 8, 2015

# Ultragenyx Pharmaceutical Inc

## Entering Huntington's Disease and TIO

Industry View	Stock Rating
In-Line	Overweight

Ultragenyx announced a license agreement with Inserm Transfert SA and Institut du Cerveau et de la Moelle Epiniere (ICM) for IP related to triheptanoin (UX007) for the treatment of Huntington's disease (HD). The news comes a day after the company initiated a new PhII program (n=6) exploring KRN23 in patients with tumor-induced osteomalacia (TIO).

**TIO is a small indication, but KRN23 bone data by YE15 will be worth watching:** TIO is caused by benign tumors producing excess levels of FGF23 affecting an estimated 500-1,000 patients in the US; approximately half of the cases are inoperable. When operable patients respond extremely well to the surgery, though, ~5-10% of tumors reoccur. Ultragenyx expects interim safety, efficacy and some bone data from the KRN23 PhII study in TIO by the end of 2015. To date, there has been no bone data reported therefore bone data at YE will provide important insight into both the TIO and XLH programs.

**The level of evidence from the UX007 pilot study in HD suggests a potential signal, but larger data sets are needed to confirm:** The pilot, open-label study examined levels of brain energy metabolism markers - phosphocreatine (PCr) and inorganic phosphate (Pi) - before, during, and after a visual stimulus. 10 pts with early HD were treated with UX007 for 1 month then had 31P brain magnetic resonance spectroscopy (MRS). After 1 month the MRS profile improved in pts treated with UX007 (vs. 13 control pts, p=0.005) (see [Neurology paper](#)). Given this initial signal, the company will support the development of UX007 in HD in a second investigator sponsored study through an IP agreement covering the treatment of HD with UX007. While the US prevalence of HD is estimated at ~30,000 these studies are focused on the treatment of early stage HD.

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Ultragenyx Pharmaceutical Inc ( RARE.O , RARE US )	
Biotechnology / United States of America	
<b>Stock Rating</b>	<b>Overweight</b>
<b>Industry View</b>	<b>In-Line</b>
Shr price, close (Jan 6, 2015)	\$47.10
Mkt cap, curr (mm)	\$1,509
52-Week Range	\$69.77-32.02

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(as of December 31, 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>1156</b>	<b>35%</b>	<b>328</b>	<b>42%</b>	<b>28%</b>
<b>Equal-weight/Hold</b>	<b>1439</b>	<b>44%</b>	<b>354</b>	<b>45%</b>	<b>25%</b>
<b>Not-Rated/Hold</b>	<b>107</b>	<b>3%</b>	<b>17</b>	<b>2%</b>	<b>16%</b>
<b>Underweight/Sell</b>	<b>589</b>	<b>18%</b>	<b>84</b>	<b>11%</b>	<b>14%</b>
<b>TOTAL</b>	<b>3,291</b>		<b>783</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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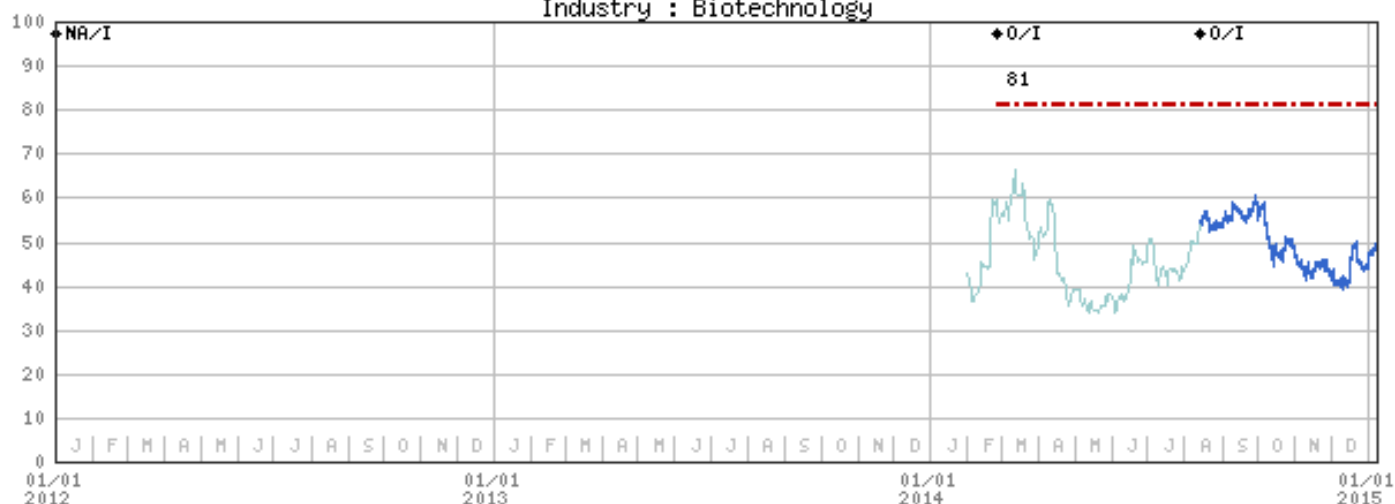
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### Stock Price, Price Target and Rating History (See Rating Definitions)

Ultragenyx Pharmaceutical Inc (RARE.O) - As of 1/7/15 in USD  
Industry : Biotechnology



Stock Rating History: 1/1/12 : NA/I; 2/25/14 : 0/I; 8/14/14 : 0/I

Price Target History: 2/25/14 : 81

Source: Morgan Stanley Research Date Format : MM/DD/YY Price Target -- No Price Target Assigned (NA)

Stock Price (Not Covered by Current Analyst) — Stock Price (Covered by Current Analyst) —

Stock and Industry Ratings (abbreviations below) appear as ♦ Stock Rating/Industry View

Stock Ratings: Overweight (O) Equal-weight (E) Underweight (U) Not-Rated (NR) No Rating Available (NA)

Industry View: Attractive (A) In-line (I) Cautious (C) No Rating (NR)

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INDUSTRY COVERAGE: **Biotechnology**

COMPANY (TICKER)	RATING (AS OF)	PRICE* (01/07/2015)
<b>Matthew Harrison</b>		
Alexion Pharmaceuticals (ALXN.O)	E (01/05/2015)	\$187.93
Amgen Inc. (AMGN.O)	E (01/05/2015)	\$158.24
Biogen Idec Inc. (BIIB.O)	O (03/26/2014)	\$353.24
Celgene Corp (CEL.G.O)	E (03/26/2014)	\$114.55
Chimerix Inc (CMRX.O)	O (08/14/2014)	\$39.31
Cubist Pharmaceuticals Inc. (CBST.O)	++	\$100.79
Gilead Sciences Inc. (GILD.O)	O (12/29/2014)	\$99.48
ImmunoGen Inc. (IMGN.O)	U (06/25/2014)	\$6.33
Infinity Pharmaceuticals Inc (INFI.O)	E (01/05/2015)	\$16.33
Ironwood Pharmaceuticals, Inc. (IRWD.O)	E (08/14/2014)	\$15.42
Ophthotech Corp (OPHT.O)	O (08/14/2014)	\$48.58
Pharmacyclics Inc. (PCYC.O)	E (03/26/2014)	\$123.47
Portola Pharmaceuticals Inc (PTLA.O)	O (08/14/2014)	\$27.65
Regeneron Pharmaceuticals Inc. (REGN.O)	O (01/05/2015)	\$407.72
Theravance Inc (THR.O)	U (08/14/2014)	\$13.15
Ultragenyx Pharmaceutical Inc (RARE.O)	O (08/14/2014)	\$48.65
Vertex Pharmaceuticals (VRTX.O)	E (08/14/2014)	\$120.66

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\* Historical prices are not split adjusted.

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