

Ultragenyx Pharmaceutical (RARE : US\$58.21)

Ultragenyx Pharmaceutical jumps 25% in 2015, outperforming Healthcare-Products sector

Ultragenyx Pharmaceutical Inc, NASDAQ's 3rd largest healthcare-products company by market cap, has now jumped US\$11.81 (or 25.5%) year to date in 2015 to close at US\$58.21. This gain makes the stock the highest rising one in the Healthcare-Products sector which is up 5.3% in 2015. The volume YTD has been 1 times average trading of 268,755 shares. Compared with the NASDAQ-100 Index which has risen 1.1% YTD, this represents a relative price increase of 24.4%. In 2015 the market cap has jumped US\$374.1 million.

Introduction

We are currently bearish on this stock in the long-term based on fundamental metrics, but are neutral in the short-term based on technical indicators

There are some reasons why we remain neutral on the stock for the short-term:

- Its Moving Average Convergence Divergence indicators are rising, with its 12-day EMA higher than its 26-day EMA.
- Amongst stocks that are shorted, it was in the top quartile as a percentage of stock shorted, suggesting that there are many confident bears of the stock.
- The price to 200-day MAP ratio is 1.03, a bullish indicator.
- The stock is overbought according to the Williams % R indicator of -6.1.

Stock Summary

Sector	Healthcare-Products
Market Cap	US\$1.8 billion
Shares Outstanding	31,673,167
ISIN	US90400D1081
Relative Strength (3 mo)	87

Financial Summary

Year ended	Dec 2013	Dec 2012	Dec 2011
Pretax (USD)	-	-	(6.85M)
Net (USD)	(50.289M)	(19.561M)	(7.47M)
EPS (USD)	(14.87)	(14.2)	(0.26)

MCap: A Historical Perspective

	Share Price (USD)	Market Cap (US\$)
Last	58.2	1.8 billion

Analysis

Bullish Signals

- Rises to Falls: In the last three months the number of rises outnumbered falls 32:30 or 1.1:1.
- In the last 16 trading sessions there has been a net rise of 28.87%; the stock has advanced twelve times and the biggest one day rise was 11.5% on Jan 26.
- In the NASDAQ market of 2,412 stocks traded today, the stock has a 6-month relative strength of 88 which means it is beating 88% of the market.
- A price rise combined with a high relative strength is a bullish signal.
- The Moving Average Convergence Divergence (MACD) indicator of 12-day Exponential Moving Average (EMA) of 52.4 minus the 26-day EMA of 49.96 is positive suggesting a bullish signal. Both the 12-day EMA as well as the 26-day EMA are rising, another bullish signal.
- The price to 200-day MAP ratio is 1.03, a bullish indicator. In the past 200 days this ratio has exceeded 1.03, five times suggesting further upside. The 50-day MAP of US\$84.20 is higher than the 200-day MAP of US\$56.63, a bullish indicator. The 200-day MAP has increased to US\$56.63. An increase is another bullish indicator.
- The stock traded at a six-month high of US\$58.98 today.

Other Bullish Signals:

Also, the following criteria set by Benjamin Graham:

- "Current ratio of two or more"; current assets are 9.3 times current liabilities.

Bullish Indicators and Rank in Market:

Description	Value	Rank In Market
Price Change %	11.5	In Top 2%
%Prem To Vol Wtd Price	18.2	In Top 5%
Relative Strength (6M)	88	In Top Quartile
Turnover in Quarter	US\$722.4 million	In Top Quartile
MCap	US\$1.8 billion	In Top Quartile

Bearish Signals

- The price is at a premium of 18.2% to the 1-month volume weighted average price of US\$49.25. Given that this premium has been under 18.2% two hundred nineteen times and over 18.2% fifteen times in the last year, the downside:upside probability is estimated at 219:15 or 14.6:1.
- The Price/MAP of 1.03 for RARE is lower than the Price/MAP for the NASDAQ-100 Index of 1.0.

- The stock is overbought according to the Williams % R indicator of -6.1.
- In the NASDAQ Short Selling market of 2419 stocks, short selling as a % of issued shares ranked 542nd and within the top quartile of stocks. This means it was higher than market average of 0.48%, a bearish indicator.
- The current short volume is 1.4 times its own historical average of 0.4%.

Other Bearish Signals:

- Return on Assets has seen consecutive falls and seems to be on a slippery slope in recent years.

Annual Trend:

[Year ended, all figures in %]:

Year	ROA
Dec 13	-84.3
Dec 12	-22.1

Bearish Indicators and Rank in Market:

Description	Value	Rank In Market
Price/MAP50	0.69	In Bottom Quartile
Volatility %	16.0	In Bottom 3%

Short Selling to MCap

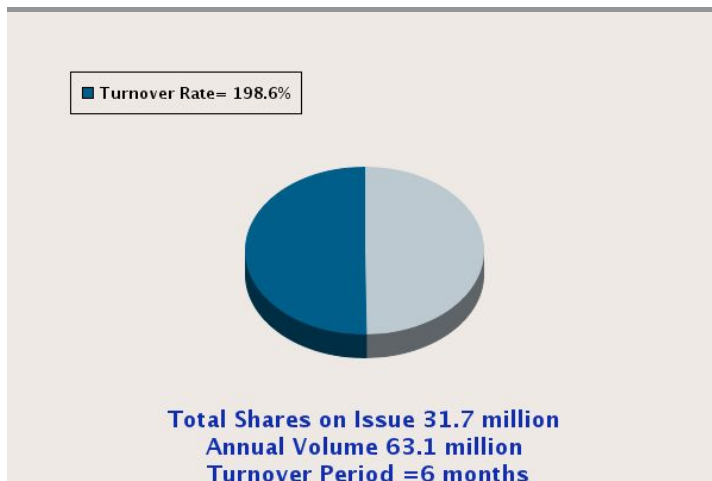


Analysis (continued)

Insider Trades



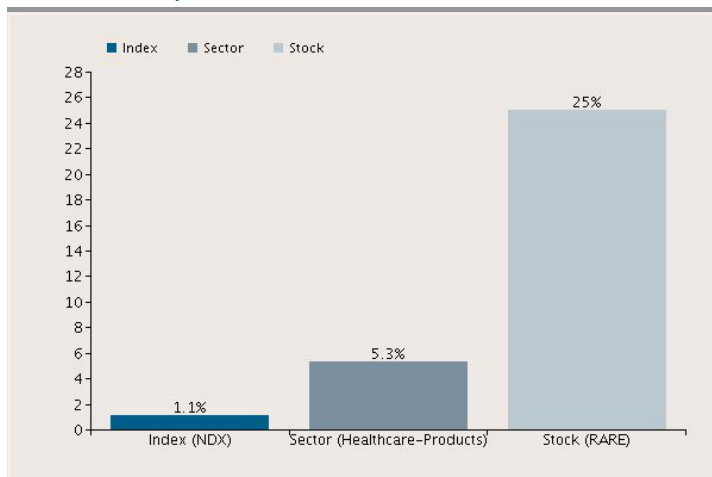
Turnover Rate & Turnover Period



Trailing 3 months

Turnover	US\$722.4 million
Volume	15,429,645
Volume Index (1 is avg)	0.9

YTD Comparison Stock Sector Index



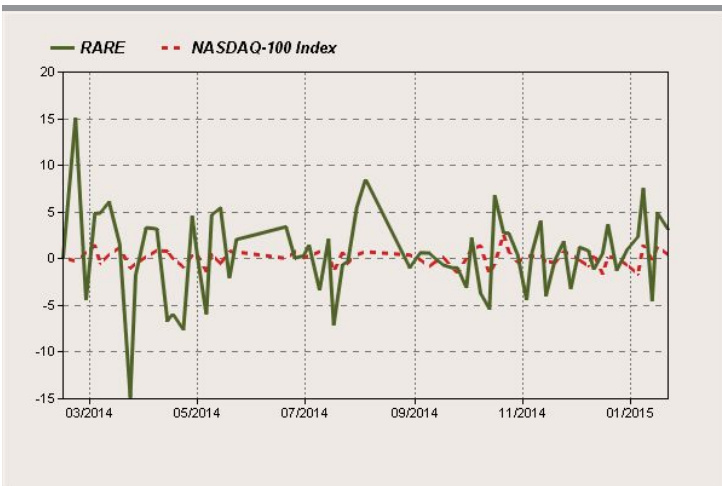
Price Volume Trend: Trailing Quarter



Weekly Price Volume Dynamics (Last 12 Weeks)



Stock v Index (Trailing Year)



Price/Moving Average Price [P/MAP]



Recent Analyst Recommendations

Date	Brokerage	Action	Past Rating	Current Rating	Prev Target	Price Target
13-Jan-2015	JPMorgan Chase & Co.	Reiterated Rating	-	Overweight	-	-
08-Jan-2015	Canaccord Genuity	Initiated Coverage	-	Buy	-	-

Reported Selling by Insiders

Insider	Date	Transaction Type	No. of Shares	Price, US\$	Value, US\$
Sharp Shalini	Jan 20, 2015	Sold	4,000	51.24	204,957
Kassberg Thomas Richard	Jan 14, 2015	Sold	3,333	51.51	171,683
Sharp Shalini	Dec 18, 2014	Sold	4,000	44.82	179,290
Kassberg Thomas Richard	Dec 10, 2014	Sold	3,333	40.22	134,053
Aliski William	Dec 04, 2014	Sold	6,000	42.15	252,900
Sharp Shalini	Nov 20, 2014	Sold	4,000	44.07	176,280
Kassberg Thomas Richard	Nov 12, 2014	Sold	3,333	41.85	139,486

Company Overview

Activities

Ultragenyx Pharmaceutical Inc. provides biotechnology services. The Company develops therapeutics and sialic acid for treating metabolic, body myopathy, glucuronidase, and rare genetic diseases. Ultragenyx Pharmaceutical conducts its business in the United States.

Further details can be found at
www.ultragenyx.com.

Economic Sector	Healthcare
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Regulatory Announcements

January 12: Ultragenyx Announces Intent to File for Conditional Approval in Europe for Sialic Acid Extended-Release Tablets in Hereditary Inclusion Body Myopathy

[News Story] NOVATO, Calif., Jan. 12, 2015 Ultragenyx Pharmaceutical Inc. (Nasdaq:RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, today announced its intent to file a Marketing Authorization Application (MAA) seeking conditional approval from the European Medicines Agency (EMA) for the use of six grams per day of sialic acid extended-release (SA-ER; UX001) tablets in the treatment of hereditary inclusion body myopathy (HIBM; also known as GNE myopathy).

[For more details click here.](#)

January 07: Ultragenyx Announces License of Intellectual Property Related to the Treatment of Huntington's Disease With Triheptanoin

[News Story] NOVATO, Calif., Jan. 7, 2015 -- Ultragenyx Pharmaceutical Inc. (Nasdaq:RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, today announced a license agreement with Inserm Transfert SA and Institut du Cerveau et de la Moelle Epiniere (ICM) for intellectual property related to the treatment of Huntington's disease with triheptanoin (UX007).

"Huntington's is a severe and lethal rare genetic disease," commented Emil D.

[For more details click here.](#)

January 06: Ultragenyx Initiates New Development Program Studying KRN23 for the Treatment of Tumor-Induced Osteomalacia

[News Story] NOVATO, Calif., Jan. 6, 2015 Ultragenyx Pharmaceutical Inc. (Nasdaq:RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, announced the initiation of a new development program for the human monoclonal anti-FGF23 antibody KRN23 (UX023) in tumor-induced osteomalacia (TIO).

[For more details click here.](#)

Conferences/Exhibitions

January 05: Ultragenyx to Present at the J.P. Morgan Healthcare Conference

[News Story] Novato, CA, Jan. 5, 2015 Ultragenyx Pharmaceutical Inc. (NASDAQ:RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, today announced that Emil D. Kakkis, M.D., Ph.D., the company's Chief Executive Officer, will be presenting at the J.P.

[For more details click here.](#)

December 15 2014: Ultragenyx Announces Initiation of Phase 3 Study of Recombinant Human Beta-Glucuronidase in Mucopolysaccharidosis Type 7

[Company Release] NOVATO, Calif., Dec. 15, 2014 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (Nasdaq:RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, today announced the dosing of the first patient in the pivotal Phase 3 study of recombinant human beta-glucuronidase (rhGUS, UX003), an investigational therapy for the treatment of Mucopolysaccharidosis 7 (MPS 7, Sly syndrome).

"We are pleased to have reached alignment with both the FDA and EMA on our single pivotal study design for this devastating disease," commented Sunil Agarwal, M.D., Chief Medical Officer of Ultragenyx.

Conferences/Exhibitions

December 03 2014: Ultragenyx to Present at the Oppenheimer Healthcare Conference

[News Story] Novato, CA, Dec. 3, 2014 Ultragenyx Pharmaceutical Inc. (NASDAQ:RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, today announced that Tom Kassberg, the company's Chief Business Officer, will be presenting at the Oppenheimer 25th Annual Healthcare Conference on December 10, 2014 at 9:10am ET in New York.

The live and archived webcast of the company presentation will be accessible from the company's website at <http://ir.ultragenyx.com/events.cfm>.

[For more details click here.](#)

Financial Results

November 10 2014: Ultragenyx Reports Third Quarter 2014 Financial Results and Corporate Update

[News Story]

<http://www.nasdaq.com/press-release/ultragenyx-reports-third-quarter-2014-financial-results-and-corporate-update-20141110-00672>

Source: Nasdaq Stock Exchange

[For more details click here.](#)

Company Overview (continued)

Issued Capital

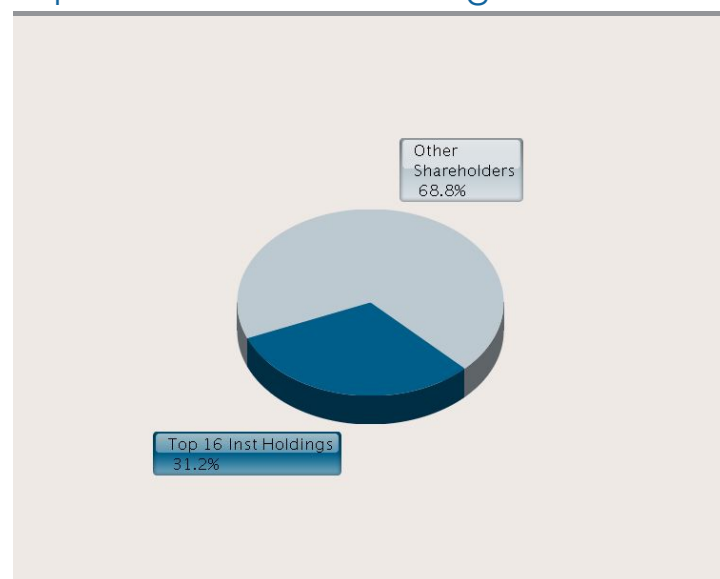
Based on 31,673,167 issued equity shares the market capitalisation is US\$1.8 billion.

The top sixteen shareholders hold 31.2% of the issued capital leaving a float of 68.8%.

Top 10 Shareholders

Name of Shareholder	No. of Shares	% Held
Fmr Llc	3,600,488	11.37
Capital Research Global Investors	2,063,176	6.51
Columbia Wanger Asset Management Llc	1,795,482	5.67
Wellington Management Co Llp	632,419	2
Capital World Investors	361,987	1.14
Alliancebernstein L.P.	247,097	0.78
Vanguard Group Inc	213,887	0.68
Rs Investment Management Co Llc	179,100	0.57
Blackrock Fund Advisors	164,642	0.52
Federated Investors Inc /Pa/	141,300	0.45
Total	9,399,578	29.68

Top 16 Institutional Holdings



Board and Management

Emil D. Kakkis
CEO & President & Director

Eran Nadav
Chairman

Shalini Sharp
CFO

Ms. Shalini Sharp is Chief Financial Officer and Senior Vice President of the company. She has served as our Chief Financial Officer and Senior Vice President since May 2012. Prior to Ultragenyx, Ms. Sharp served in various executive capacities, and ultimately as Chief Financial Officer, of Agenesis Inc., a biotechnology company, from August 2003 until May 2012. Prior to Agenesis, Ms. Sharp held strategic planning and corporate finance roles and ultimately served as chief of staff to the chairman of the board at Elan Pharmaceuticals, a biotechnology company, from August 1998 to August 1999 and September 2001 to August 2003. Prior to Elan, Ms. Sharp was a management consultant at McKinsey & Company and an investment banker at Goldman Sachs, specializing in pharmaceuticals and medical devices. Ms. Sharp has also served as a board member of Agenesis since May 2012. Ms. Sharp holds a B.A. and an M.B.A. from Harvard University.

William Aliski
Director

Mr. Aliski joined Ultragenyx as a Board member in January of 2011. He is an experienced orphan disease and biotech executive, having worked on the commercialization of a number of orphan products in the US as well as international markets. His commercial experience includes Chief Commercial Officer for Foldrx, an early stage company purchased by Pfizer, General Manager for Biomarin Europe and executive level positions at TKT 5S/Shire and Genzyme Corp. In addition he has developed pricing and reimbursement models and patient support programs for a number of enzyme replacement therapies including Ceredase/Cerezyme for Gaucher Disease and Replagal for Fabry Disease. Mr Aliski is currently a commercial consultant for early stage orphan disease companies and also serves on the board of Edimer Pharmaceuticals. He earned an undergraduate degree in economics from Boston College and a MPA from the Kennedy School of Government at Harvard University.

Board and Management (continued)

Thomas Kassberg **Senior Vice President &** **Chief Business Officer**

Mr. Kassberg joined Ultragenyx in November 2011. He is responsible for leading the company's business development and corporate strategic planning activities, as well as the human resources and legal affairs functions. Mr. Kassberg brings more than twenty years of experience in the pharmaceutical and biotechnology industries. Prior to joining Ultragenyx, Mr. Kassberg worked as an independent consultant in Corporate Development and Business Strategy and consulted with a number of companies, including Corium International, Inc. and Rib-X Pharmaceuticals, Inc. Before becoming a consultant, Mr. Kassberg worked at Proteolix, Inc., where he served as Executive Vice President of Corporate Development, overseeing business development, corporate strategic planning and legal affairs. Prior to Proteolix, Mr. Kassberg served as Senior Vice President of Corporate Development and Commercial Operations at InterMune, Inc. At InterMune, he oversaw the commercial team responsible for in-line sales of Actimmune(Registered) and pre-launch planning for late-stage products. Mr. Kassberg was also a co-founder of Plexxikon, where he served as Vice President, Business and Corporate Development. In this role, he helped to establish the company's initial operations in addition to leading the company's corporate development activities. Prior to Plexxikon, Mr. Kassberg served as the Senior Director of Business Development and Corporate Licensing at SUGEN until the company's acquisition by Pharmacia. Mr. Kassberg began his career at Bristol-Myers Squibb where he held a variety of positions in strategic planning, managed care sales, and financial and product analysis. He holds an M.B.A. from Northwestern University and a B.A. in Economics and Management from Gustavus Adolphus College.

Financial Results (Quarterly)

30 September, 2014

Quarterly Report: Ultragenyx Pharmaceutical (NASDAQ:RARE) \$US15.8m loss

Quarter ended Sep 30, 2014

Highlights

- Loss of \$US15.8m

Unfavourable Changes:

- EPS deterioration 16.5% to -50.11 U.S. cents from the year-earlier period.

Newly listed on January 31, 2014 Quarterly Report

Quarterly	Sep 30, 2014	Jun 30, 2014	Sep 30, 2013	Change %	Change %
Description	\$US ('000)	\$US ('000)	\$US ('000)	Q-on-Q [sequential comparison]	Y-on-Y [Quarter vs previous corresponding quarter]
Pre Tax Profit/(Loss)	(15,849.0)	(13,585.0)	(8,430.0)	Deterioration 16.7	Deterioration 88.0
Profit/(Loss) after Tax	(15,849.0)	(13,585.0)	(12,590.0)	Deterioration 16.7	Deterioration 25.9
Cash and cash equivalents	53,387.0	17,452.0	6,050.0	Up 205.9	Up 782.4
Net Assets	199,326.0	153,083.0	61,150.0	Up 30.2	Up 226.0
Per Share figures	-	-	-	-	-
EPS	(50.1 U.S. cents)	(45.2 U.S. cents)	(43.0 U.S. cents)	Deterioration 10.9	Deterioration 16.5

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