

Ultragenyx Pharmaceutical (RARE : US\$57.92)

Ultragenyx Pharmaceutical continues uptrend, now jumps 29.4% and in top 5% of the market

Ultragenyx Pharmaceutical Inc, NASDAQ's 2nd largest healthcare-products company by market cap, jumped US\$13.17 (or 29.4%) in the quarter ended September 30 to close at US\$57.92. This gain means the stock is among the top 5% of NASDAQ-listed stocks in quarter ended September 30. The volume in the quarter was 1.6 times the average trading of 275,650 shares. Compared with the NASDAQ-100 Index which rose 150.2 points (or 3.8%), this represents a relative price increase of 25.6%. In the third quarter the market cap jumped US\$469.7 million.

Introduction

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

What makes us most bullish on the stock is the following:

- Its Moving Average Convergence Divergence indicators are rising, with its 12-day EMA higher than its 26-day EMA.
- In the last three months the number of rises outnumbered falls 27:22 or 1.2:1.
- It is beating 86% of the market the past 6 months.
- The price to 50-day EMAP ratio is 1.1, a bullish indicator. The 50-day EMAP has increased to US\$53.71.

Stock Summary

Sector	Healthcare-Products
Market Cap	US\$1.8 billion
Shares Outstanding	31,673,167
ISIN	US90400D1081
Relative Strength (3 mo)	95

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	57.9	1.8 billion

Analysis

Bearish Signals

- The price is at a premium of 5.3% to the 1-month volume weighted average price of US\$54.99. Given that this premium has been under 5.3% eighty-six times and over 5.3% sixty-eight times in the last year, the downside:upside probability is estimated at 86:68 or 1.3:1.
- In the NASDAQ Short Selling market of 2463 stocks, short selling as a % of issued shares ranked 595th and within the top quartile of stocks. This means it was higher than market average of 0.54%, a bearish indicator.
- The current short volume is 1.8 times its own historical average of 0.3%.

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
Volatility %	4.6	In Bottom Quartile
Price/MAP50	0.64	In Bottom Quartile
Price Change %	-3.1	In Bottom 8%

Note

Bullish Signals:

- In the last 20 trading sessions there has been a net rise of 8.44%; the stock has advanced eleven times and the biggest one day rise was 5.4% on Sep 10.
- In the NASDAQ market of 2,413 stocks traded today, the stock has a 6-month relative strength of 86 which means it is beating 86% of the market.
- The Moving Average Convergence Divergence (MACD) indicator of 12-day Exponential Moving Average (EMA) of 56.74 minus the 26-day EMA of 55.32 is positive suggesting a bullish signal. Both the 12-day EMA as well as the 26-day EMA are rising, another bullish signal.

Other Bullish Signals:

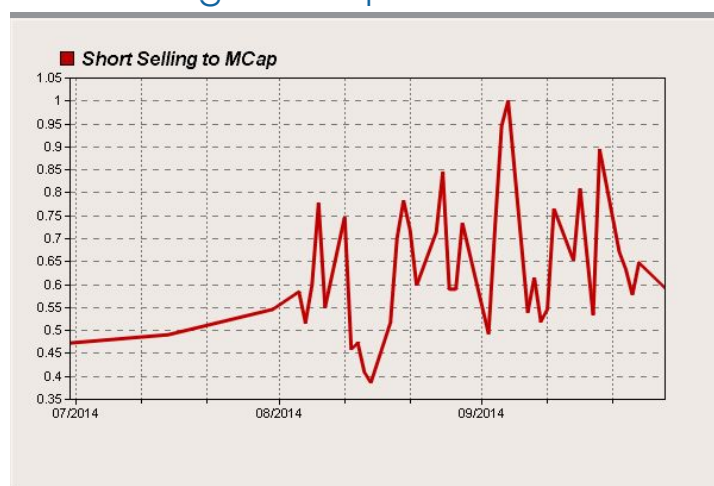
Also, the following criteria set by Benjamin Graham:

- "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
Relative Strength (6M)	86	In Top Quartile
Turnover in Quarter	US\$1.4 billion	In Top Quartile
MCap	US\$1.8 billion	In Top Quartile
%Prem To Vol Wtd Price	5.3	In Top Quartile

Short Selling to MCap

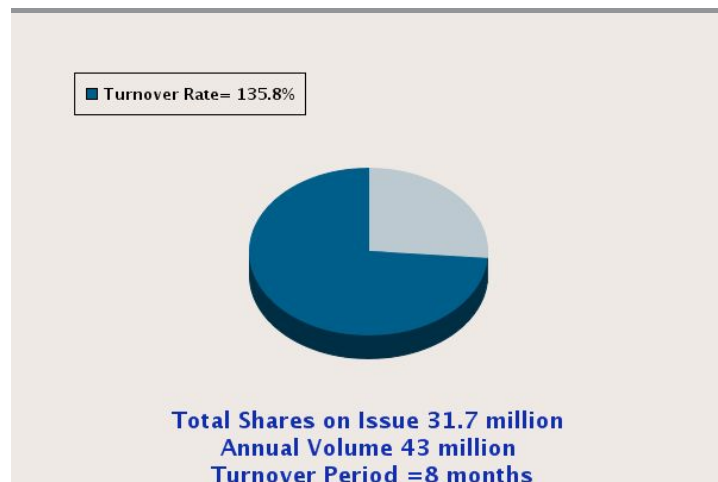


Insider Trades



Analysis (continued)

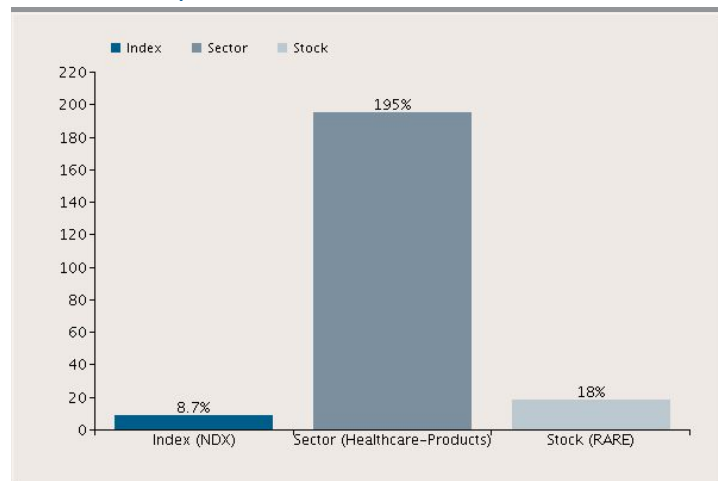
Turnover Rate & Turnover Period



Trailing 3 months

Turnover	US\$1.4 billion
Volume	28,128,018
Volume Index (1 is avg)	1.6

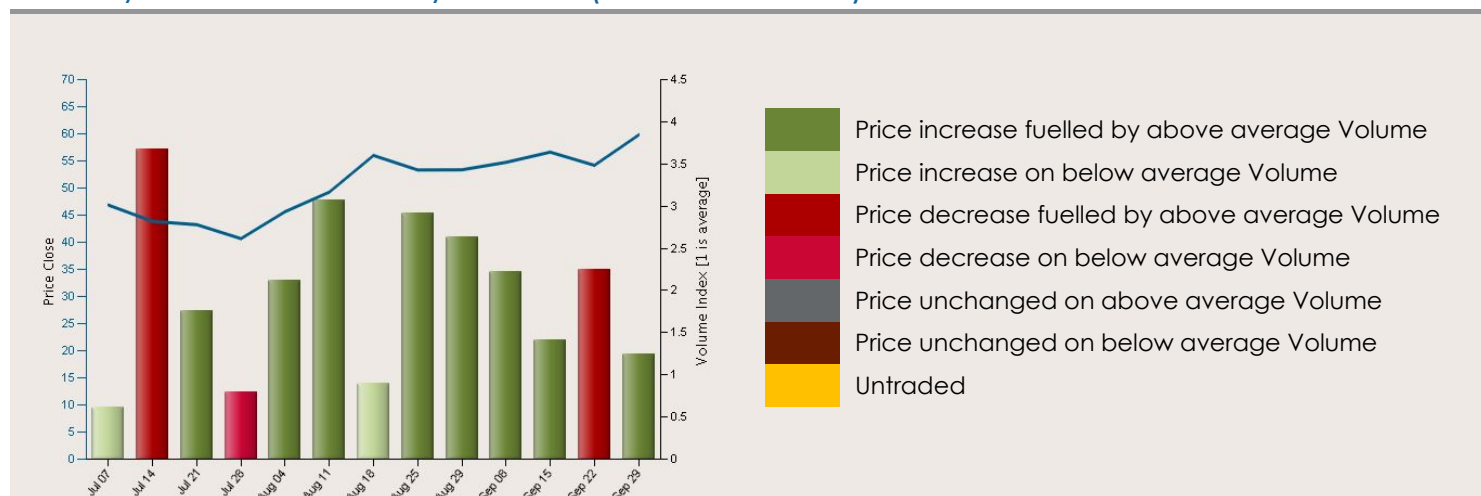
YTD Comparison Stock Sector Index



Price Volume Trend: Trailing Quarter



Weekly Price Volume Dynamics (Last 12 Weeks)



Recent Analyst Recommendations

Date	Brokerage	Action	Past Rating	Current Rating	Prev Target	Price Target
15-Jul-2014	Robert W. Baird	Initiated Coverage	-	Outperform	-	64

Reported Buying Selling by Insiders

Insider	Date	Transaction Type	No. of Shares	Price, US\$	Value, US\$
Sharp Shalini	Sep 18, 2014	Sold	4,000	56	224,000
Kassberg Thomas Richard	Sep 10, 2014	Sold	3,333	54.82	182,715
Sharp Shalini	Aug 18, 2014	Sold	4,000	53.97	215,880
Coulter James G	Jul 14, 2014	Sold	488,820	37.6	18,379,632

Company Overview

Regulatory Announcements

To view Recent Company Headlines, [click here](#)

September 24: Ultragenyx to Present at the Leerink Partners Rare Disease Roundtable

September 19: Ultragenyx Pharmaceutical insider sells; shares at an eight-day low

September 15: Ultragenyx Announces Positive Results From a Long-Term Phase 1/2 Study of KRN23 in Adult Patients With X-Linked Hypophosphatemia

September 12: Ultragenyx Pharmaceutical insider sells; shares at a seven-day high

September 04: Ultragenyx to Present at the Morgan Stanley Global Healthcare Conference

September 03: Ultragenyx Announces Positive Interim Data From Phase 1/2 Study of Recombinant Human Beta-Glucuronidase in Mucopolysaccharidosis 7

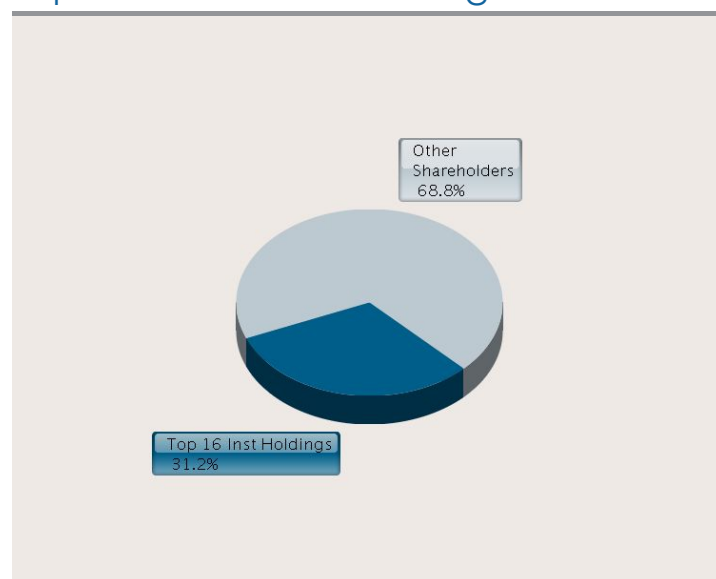
August 27: Ultragenyx to Present at the Baird Health Care Conference

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 16 Institutional Holdings



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

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 Agenus Inc., a biotechnology company, from August 2003 until May 2012. Prior to Agenus, 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 Agenus since May 2012. Ms. Sharp holds a B.A. and an M.B.A. from Harvard University.

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® 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 (Annual)

31 December, 2013

INCOME STATEMENT

Currency in Thousand of USD	2013-12-31	2012-12-31	Change %
Operating expenses:			
Research and development	27,829	12,641	Up 120.1
General and administrative	4,451	3,344	Up 33.1
Total operating expenses	32,280	15,985	Up 101.9
Loss from operations	-32,280	-15,985	Deterioration 101.9
Other income (expense), net:			
Interest income	216	1	Up 21,500.0
Interest expense			
Other expense, net	-3,006	-350	Deterioration 758.9
Total other income (expense), net	-2,790	-349	Deterioration 699.4
Net loss	-35,070	-16,334	Deterioration 114.7
Net loss attributable to common stockholders	-50,289	-19,561	Deterioration 157.1
Net loss per share attributable to common stockholders, basic and diluted	-1,487.0c	-1,420.0c	Deterioration 4.7
Shares used in computing net loss per share attributable to common stockholders, basic and diluted	3,382,489	1,377,207	Up 145.6

BALANCE SHEET

Currency in Thousand of USD	2013-12-31	2012-12-31	Change %
Assets			
Current assets:			
Cash and cash equivalents	7,427	86,190	Down 91.4
Short-term investments	45,950		
Prepaid expenses and other current assets	1,848	255	Up 624.7
Total current assets	55,225	86,445	Down 36.1
Property and equipment, net	1,325	1,362	Down 2.7
Restricted cash	451	476	Down 5.3
Other assets	2,648	33	Up 7,924.2
Total assets	59,649	88,316	Down 32.5
Liabilities, Convertible Preferred Stock and Stockholders' Deficit			
Current liabilities:			
Accounts payable	1,437	1,200	Up 19.8
Accrued liabilities	4,406	1,913	Up 130.3
Deferred rent-current portion	78	75	Up 4.0
Total current liabilities	5,921	3,188	Up 85.7
Convertible preferred stock warrant liability	3,419	518	Up 560.0
Other liabilities	200	270	Down 25.9
Total liabilities	9,540	3,976	Up 139.9

Financial Results (Annual) (continued)

31 December, 2013

Commitments and contingencies			
Series A redeemable convertible preferred stock, par value of 0,001 per share-35,377,556 shares authorized as of December 31, 2012 and 2013; 34,349,894 shares issued and outstanding as of December 31, 2012 and 2013; redemption value of 138,155 as of December 31, 2013	51,001	37,458	Up 36.2
Series B convertible preferred stock, par value of 0,001 per share-27,081,680 shares authorized, issued and outstanding as of December 31, 2012 and 2013; aggregate liquidation preference of 76,739 as of December 31, 2013	73,929	73,929	Steady
	124,930	111,387	Up 12.2
Stockholders' deficit:			
Common stock, par value of 0,001 per share-85,000,000 shares authorized; 3,458,928 and 3,766,289 shares issued and outstanding as of December 31, 2012 and 2013	4	3	Up 33.3
Additional paid-in capital		8	
Accumulated other comprehensive income	11		
Deficit accumulated during the development stage	-74,836	-27,058	Deterioration 176.6
Total stockholders' deficit	-74,821	-27,047	Deterioration 176.6
Total liabilities, convertible preferred stock and stockholders' deficit	59,649	88,316	Down 32.5

CASH FLOW

Currency in Thousand of USD	2013-12-31	2012-12-31	Change %
Operating activities:			
Net loss	-35,070	-16,334	Deterioration 114.7
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	444	313	Up 41.9
Noncash interest expense			
Amortization of premium on investment securities	1,413		
Stock-based compensation	657	891	Down 26.3
Revaluation of convertible preferred stock warrant liability	2,901	302	Up 860.6
Changes in operating assets and liabilities:			
Prepaid expenses and other current assets	-1,593	-47	Deterioration 3,289.4
Other assets	-2,615	8	Deterioration
Accounts payable	237	858	Down 72.4
Accrued expenses, deferred rent and other liabilities	2,426	1,505	Up 61.2
Net cash used in operating activities	-31,200	-12,504	Deterioration 149.5

Financial Results (Annual) (continued)

31 December, 2013

Investing activities:			
Purchase of property and equipment	-407	-1,091	Reduced 62.7
Purchase of investments	-63,953		
Proceeds from maturities of investments	16,601		
Decrease (increase) in restricted cash	25	-100	Recovery
Net cash used in investing activities	-47,734	-1,191	Deterioration 3,907.9
Financing activities:			
Net proceeds from issuance of convertible preferred stock		89,009	
Net proceeds from issuance of common stock	171	131	Up 30.5
Proceeds from issuance of promissory notes		100	
Net cash provided by financing activities	171	89,240	Down 99.8
Net increase (decrease) in cash and cash equivalents	-78,763	75,545	Deterioration
Cash and cash equivalents at beginning of period	86,190	10,645	Up 709.7
Cash and cash equivalents at end of period	7,427	86,190	Down 91.4

PERFORMANCE RATIOS

	2013-12-31	2012-12-31	Change %
Return on Assets (%)	-84.31	-22.15	Deterioration 280.6
Total Liabilities to Operating Cash Flow	-0.31	-0.32	Reduced 3.8
Total Liabilities/Total Assets	0.16	0.05	Up 255.3
Current Ratio	9.33	27.12	Down 65.6

PER SHARE FIGURES

	2013-12-31	2012-12-31	Change %
Cash	US\$2.20	US\$62.58	Down 96.5
Working Capital	US\$14.58	US\$60.45	Down 75.9
Shareholders' Funds	-2,212.01c	-1,963.90c	Deterioration 12.6
Total Liabilities	US\$2.82	US\$2.89	Down 2.3
Total Assets	US\$17.63	US\$64.13	Down 72.5
Net Tangible Assets	-2,212.01c	-1,963.90c	Deterioration 12.6
EPS Final	-1,487.0c	-1,420.0c	Deterioration 4.7

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