

Ultragenyx

2Q Snapshot & Model Update

We are updating our RARE model based on the company's 2Q results (there was no conference call). Progress with the five clinical-stage pipeline programs is on track (evidenced by recent positive data updates for KRN23, rhGUS and SA-ER), and we are maintaining our OW rating. We continue to see RARE as well positioned with a broad and diverse portfolio of orphan disease assets and a highly regarded management team with a strong track record in the space. We look forward to several additional data readouts this year and into 2015, which we believe could offer meaningful de-risking and value creation events.

- **Upcoming events: several data readouts this year and into 2015 could help de-risk the story and be important investment catalysts.** Interim 12-wk data from the Phase 1/2 study of rhGUS in MPS 7 will be presented at SSIEM on Sept 3; data from the long-term Phase 1/2 trial of KRN23 will be presented at ASBMR on September 14; and preliminary Phase 2 extension study data for SA-ER in HIBM is expected at the International Congress of the WMS on October 11. In addition, a Phase 3 study of rhGUS in MPS 7 could be initiated this year. In 2015, RARE expects data from the Phase 2 studies of triheptanoin in both LC-FAOD and Glut1 DS, as well as interim data from the Phase 2 trial evaluating KRN23 in pediatric pts.
- **2Q results.** 2Q EPS was \$(0.45), inline vs. cons of \$(0.45) and JPMe of \$(0.45). 2Q OpEx of \$13.7M was inline with JPMe of \$13.5M. That said, RARE is not yet an earnings story.
- **Estimate changes; establishing a YE15 PT of \$66.** We have updated our model for 2Q results. We're also establishing a Dec 15 PT of \$66 (unchanged vs. our previous YE14 target).
- **Balance sheet update.** RARE ended 2Q with \$153M in cash (and no debt). In July, RARE completed a secondary offering of common stock (J.P. Morgan acted as joint book-running manager) generating ~\$60M in net proceeds. The company believes its current cash position should be sufficient to fund operations through 2016.

Ultragenyx Pharmaceutical (RARE;RARE US)

FYE Dec	2013A	2014E (Prev)	2014E (Curr)	2015E (Prev)	2015E (Curr)
EPS Reported (\$)					
Q1 (Mar)	-	(0.85)A	(0.85)A	-	-
Q2 (Jun)	-	(0.45)A	(0.45)A	-	-
Q3 (Sep)	-	(0.55)	(0.51)	-	-
Q4 (Dec)	-	(0.63)	(0.59)	-	-
FY	(14.87)	(2.41)	(2.32)	(2.28)	(2.21)

Source: Company data, Bloomberg, J.P. Morgan estimates.

See page 6 for analyst certification and important disclosures.

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Overweight

RARE, RARE US

Price: \$49.15

Price Target: \$66.00

Biotechnology

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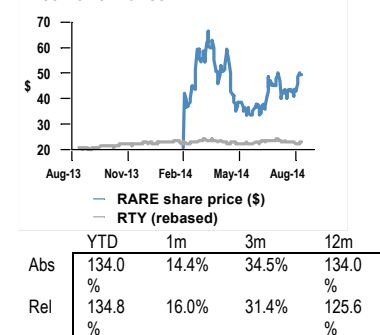
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Price Performance



Company Data

Price (\$)	49.15
Date Of Price	11 Aug 14
52-week Range (\$)	69.77-32.02
Market Cap (\$ mn)	1,477.25
Fiscal Year End	Dec
Shares O/S (mn)	30
Price Target (\$)	66.00
Price Target End Date	31-Dec-15

Pipeline

Figure 1: RARE Pipeline

Product Pipeline Highlights

Program	P/C	Ph 1	Ph 2	Ph 3	FDA	Mkt.	Partner	Comments
UX023 (KRN23) XLH							Kyowa Hakko Kirin	Anti-FGF23 mAb
UX003 (rhGUS) MPS 7								Enzyme replacement
UX007 (Triheptanoin) LC-FAOD								Substrate replacement
UX007G (Triheptanoin) Glut1 DS								Substrate replacement
UX001 (SA-ER) HIBM								Substrate replacement
UX004 (rhPPCA) galactosialidosis								Enzyme replacement

Source: Company reports and J.P. Morgan estimates.

Upcoming Events

Figure 2: RARE Upcoming Events

Anticipated Newsflow Highlights

Program	Event	Expected Timing	Significance
UX023 (KRN23)	Data from the long-term Phase 1/2 trial	ASBMR (Sept 14)	High
	Interim data from Phase 2 trial in pediatric XLH patients	2015	High
	Initiate adult XLH Phase 2b study	2015	Low
UX003 (rhGUS)	Report interim data from Phase 1/2 studies in MPS7	SSIEM (Sept 3)	High
	Potential initiation of pivotal Phase 3 study in MPS 7	2H14	Low
UX007 (Triheptanoin)	Interim data from Phase 2 trial in LC-FAOD	2015	High
UX007G (Triheptanoin)	Interim data from Phase 2 trial in Glut1	2015	High
UX001 (SA-ER)	Preliminary data from extension study	WMS (Oct 11)	Medium

Source: Company reports and J.P. Morgan estimates.

Figure 3: RARE Income Statement

Ultragenyx Pharmaceutical Income Statement

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	2011A	2012A	2013A	1Q14A	2Q14A	3Q14E	4Q14E	2014E	2015E	2016E	2017E	2018E
Net Revenue to Ultragenyx												
UX003 (rhGUS)									-	-	7.8	23.3
UX007 (FAOD)									-	-	3.3	54.9
UX007G (Glut-1)									-	-	-	46.8
UX023 / KRN23 (XLH) - Profit Share			-					-	-	-	-	15.8
UX001 (SA-ER)												
License & Milestone		-								-	(10.6)	(2.0)
Total Revenues	-	-	-	-	-	-	-	-	-	-	0.5	138.9
COGS											1.9	17.1
R&D	4.7	12.6	27.8	8.4	11.2	12.2	14.1	45.9	57.1	70.2	70.3	75.2
SG&A	1.8	3.3	4.5	2.0	2.4	4.4	5.0	13.7	16.7	24.0	41.3	41.7
Total Operating Expenses	6.6	16.0	32.3	10.3	13.7	16.6	19.1	59.6	73.8	94.2	113.5	133.9
Operating Income	(6.6)	(16.0)	(32.3)	(10.3)	(13.7)	(16.6)	(19.1)	(59.6)	(73.8)	(94.2)	(113.0)	4.9
Net interest & other income	(0.3)	(0.3)	(2.8)	(3.3)	0.1	-	-	(3.2)	-	-	-	-
Profit (Loss) before Income Tax	(6.8)	(16.3)	(35.1)	(13.6)	(13.6)	(16.6)	(19.1)	(62.8)	(73.8)	(94.2)	(113.0)	4.9
Income Tax benefit (expense)	-	-	-	-	-	-	-	-	-	-	-	(1.5)
Net Income	(6.8)	(16.3)	(35.1)	(13.6)	(13.6)	(16.6)	(19.1)	(62.8)	(73.8)	(94.2)	(113.0)	3.5
Accretion and dividends on convertible preferred stock	(0.6)	(4.0)	(15.2)	(4.8)				(4.8)				
Net Income attributable to common stockholders	(7.5)	(20.3)	(50.3)	(18.4)	(13.6)	(16.6)	(19.1)	(67.6)	(73.8)	(94.2)	(113.0)	3.5
Basic EPS	(4.62)	(2.04)	(14.87)	(0.85)	(0.45)	(0.51)	(0.59)	(2.32)	(2.21)	(2.74)	(2.92)	0.09
Diluted EPS	(4.62)	(2.04)	(14.87)	(0.85)	(0.45)	(0.51)	(0.59)	(2.32)	(2.21)	(2.74)	(2.92)	0.08
Basic Shares Outstanding	1.6	9.9	3.4	21.6	30.1	32.4	32.5	29.1	33.4	34.4	38.7	39.7
Margin Analysis:												
Gross margin		NM	NM	NM	NM	NM	NM	NM	NM	NM	-272%	88%
Operating margin		NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	3.55%
Net margin		NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	2.49%
Tax Rate		0%	30%	0%	0%	0%	0%	30%	30%	30%	30%	30%
Cost Analysis:												
COGS as % of tot. prod. sales		NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	12.28%
R&D as % of tot. revenue		NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	54.11%
SG&A as % of tot. revenue		NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	30.05%
Year-over-year growth:												
Total revenue			NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
R&D Expense			120.15%	47.49%	56.10%	80.49%	71.93%	64.94%	24.40%	22.95%	0.16%	6.88%
SG&A Expense			33.10%	83.35%	131.18%	335.42%	274.72%	207.99%	21.97%	43.49%	72.10%	1.09%
Total operating expenses			101.94%	53.25%	65.64%	113.31%	100.05%	84.66%	23.84%	27.60%	20.49%	18.02%
Operating income			NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Net income			NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
EPS			NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Basic Shares			-65.96%	NM	NM	774.31%	767.29%	760.98%	14.72%	2.99%	12.46%	2.58%
Diluted Shares			-65.96%	NM	NM	774.31%	767.29%	760.98%	14.72%	2.99%	12.46%	9.48%

Source: J.P. Morgan estimates, Company data.

Investment Thesis, Valuation and Risks

Ultragenyx (Overweight; Price Target: \$66.00)

Investment Thesis

We have an OW rating on RARE. We believe RARE is uniquely positioned with a broad and diverse pipeline of orphan disease assets and a highly regarded management team with a strong track record in the space. The company has five product candidates focused on orphan indications with a high unmet medical need, which provides both diversification and increased probability of ultimate success, in our view.

Valuation

Our probability weighted Dec-15 PT of \$66 is based on a blended average of our proprietary probability-adjusted sum-of-the-parts scenario analysis (50% weighting) and risk-adjusted NPV model (50% weighting).

RARE Valuation Summary

Ultragenyx Valuation Summary			
Discount rate	10.0%		
4Q15 Fully Diluted Shares (mm)	36.1		
Main value drivers	Prob of approval	Peak WW sales est (avg. scenario)	Avg peak yr
KRN23	65%	\$ 275	2024
rhGUS	75%	\$ 75	2022
Tripheptanoin (FAOD)	45%	\$ 300	2024
Tripheptanoin (Glut1 DS)	45%	\$ 825	2024
SA-ER	30%	\$ 125	2022
Valuation methodology	Value / share	Weighting	Adj. value/ share
Real options scenario analysis	\$ 68.74	50%	34.37
Risk adjusted NPV analysis	\$ 63.76	50%	31.88
Total		100%	\$ 66.25
Catalyst/liquidity discount			0%
YE15 Price Target			\$ 66

Source: J.P. Morgan estimates.

Risks to Rating and Price Target

RARE is susceptible to the standard risks that apply to the entire biotech industry, including development, regulatory, commercial, manufacturing, financing, and IP pitfalls. More specific risks to the downside include clinical setbacks for key candidates (incl KRN23, rhGUS, tripheptanoin, and SA-ER), regulatory hurdles, commercial setbacks, and personnel risk.

Ultragenyx: Summary of Financials

Income Statement - Annual	FY13A	FY14E	FY15E	FY16E	Income Statement - Quarterly	1Q14A	2Q14A	3Q14E	4Q14E
Revenues	0	0	0	0	Revenues	0A	0A	0	0
Cost of products sold	0	0	0	0	Cost of products sold	0A	0A	0	0
Gross profit	-	-	-	-	Gross profit	-	-	-	-
SG&A	(4)	(14)	(17)	(24)	SG&A	(2)A	(2)A	(4)	(5)
R&D	(28)	(46)	(57)	(70)	R&D	(8)A	(11)A	(12)	(14)
Operating income	(32)	(60)	(74)	(94)	Operating income	(10)A	(14)A	(17)	(19)
EBITDA	(32)	(60)	(74)	(94)	EBITDA	(10)A	(14)A	(17)	(19)
Net interest (income) / expense	0	0	0	0	Net interest (income) / expense	0A	0A	0	0
Other income / (expense)	(3)	(3)	0	0	Other income / (expense)	(3)A	0A	0	0
Income taxes	0	0	0	0	Income taxes	0A	0A	0	0
Net income - GAAP	(35)	(63)	(74)	(94)	Net income - GAAP	(14)A	(14)A	(17)	(19)
Net income - recurring	(50)	(68)	(74)	(94)	Net income - recurring	(18)A	(14)A	(17)	(19)
Diluted shares outstanding	3	29	33	34	Diluted shares outstanding	22A	30A	32	32
EPS - excluding non-recurring	(14.87)	(2.32)	(2.21)	(2.74)	EPS - excluding non-recurring	(0.85)A	(0.45)A	(0.51)	(0.59)
EPS - recurring	(14.87)	(2.32)	(2.21)	(2.74)	EPS - recurring	(0.85)A	(0.45)A	(0.51)	(0.59)
Balance Sheet and Cash Flow Data	FY13A	FY14E	FY15E	FY16E	Ratio Analysis	FY13A	FY14E	FY15E	FY16E
Cash and cash equivalents	53	174	253	161	Sales growth	-	-	-	-
Accounts receivable	0	0	0	0	EBIT growth	-	84.7%	23.8%	27.6%
Inventories	-	-	-	-	EPS growth - recurring	-	(84.4%)	(4.9%)	23.9%
Other current assets	0	0	0	0	Gross margin	-	-	-	-
Current assets	53	175	253	162	EBIT margin	-	-	-	-
PP&E	1	2	2	2	EBITDA margin	-	-	-	-
Total assets	55	177	256	165	Tax rate	0.0%	0.0%	0.0%	0.0%
Total debt	-	-	-	-	Net margin	-	-	-	-
Total liabilities	4	5	5	5	Net Debt / EBITDA	-	-	-	-
Shareholders' equity	51	172	251	159	Net Debt / Capital (book)	-	-	-	-
Net income (including charges)	(35)	(63)	(74)	(94)	Return on assets (ROA)	(70.0%)	(58.2%)	(34.1%)	(44.8%)
D&A	0	0	0	0	Return on equity (ROE)	(74.2%)	(60.5%)	(34.9%)	(46.0%)
Change in working capital	0	0	0	0	Enterprise value / sales	-	-	-	-
Other	1	1	2	2	Enterprise value / EBITDA	NM	NM	NM	NM
Cash flow from operations	(33)	(61)	(71)	(92)	Free cash flow yield	(20.0%)	(4.2%)	(4.4%)	(5.4%)
Capex	0	0	0	0					
Free cash flow	(33)	(61)	(71)	(92)					
Cash flow from investing activities	0	0	0	0					
Cash flow from financing activities	0	182	150	0					
Dividends	-	-	-	-					
Dividend yield	-	-	-	-					

Source: Company reports and J.P. Morgan estimates.

Note: \$ in millions (except per-share data). Fiscal year ends Dec

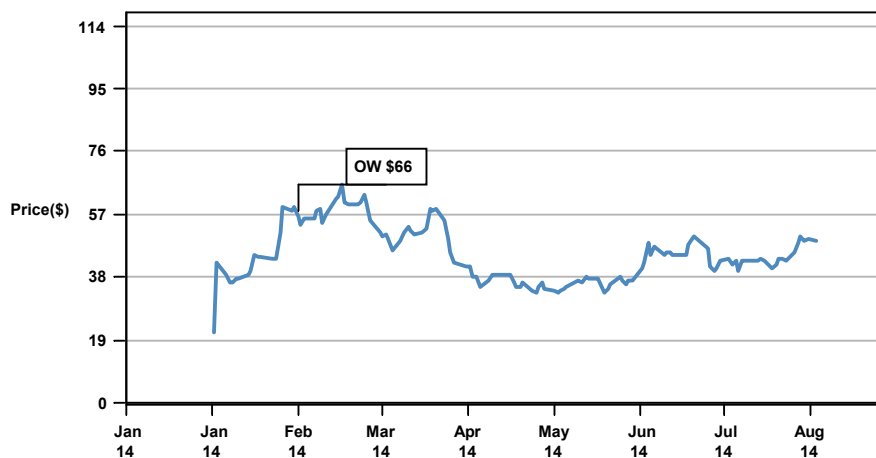
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Ultragenyx (RARE, RARE US) Price Chart



Date	Rating	Share Price (\$)	Price Target (\$)
25-Feb-14	OW	58.01	66.00

Source: Bloomberg and J.P. Morgan; price data adjusted for stock splits and dividends.
Initiated coverage Feb 25, 2014.

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IB clients*	75%	66%	54%

*Percentage of investment banking clients in each rating category.

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