

Ultragenyx

4Q Snapshot & Model Update

We are updating our RARE model based on the company's 4Q results (there was no conference call). The company continues to make progress with its five clinical-stage pipeline programs, and we are maintaining our OW rating. In our view, RARE is 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 key data readouts this year, which we believe could offer meaningful de-risking and value creation events.

- **Upcoming events: several data readouts this year could provide de-risking events and investment catalysts.** Phase 1/2 data for KRN23 in XLH is expected in mid-2014, early urinary glycosaminoglycans data for rhGUS in the first 3 MPS 7 pts is expected at the Annual Clinical Genetics Meeting (March 25-29, Nashville), and interim Phase 1/2 data for rhGUS in MPS 7 is anticipated in 2H14. RARE will present data from the Phase 2 study of SA-ER in HIBM at AAN (April 26 - May 3, Philadelphia), and the Phase 2 extension study data for SA-ER in HIBM should readout by YE14. In addition, a number of trial initiations are expected this year, including a Phase 2 pediatric trial of KRN23 in XLH and a Phase 3 study of rhGUS in MPS 7. RARE expects data from the recently initiated Phase 2 studies of triheptanoin in both LC-FAOD and Glut1 DS in 2015.
- **4Q results.** 4Q EPS was \$(4.98), wider vs. cons of (\$0.35) and JPMe of (\$0.30) due to a share count difference (RARE did not use pro forma shares outstanding, which include shares issued in the IPO and shares converted from Series A and B preferred stock). 4Q OpEx of \$9.5M was higher vs. JPMe of \$7.6M. That said, RARE is not yet an earnings story, in our view.
- **Maintaining \$66 YE14 PT.** We have updated our model for 4Q results. Our 2014 estimates are unchanged and our YE14 PT remains at \$66.
- **Balance sheet update.** RARE ended 4Q with \$53.4M in cash (and no debt). Net proceeds of \$121.7M from the company's recent IPO, in addition to cash at year end, should provide Ultragenyx with sufficient capital to fund operations through 2015.

Overweight

RARE, RARE US

Price: \$51.41

Price Target: \$66.00

Biotechnology

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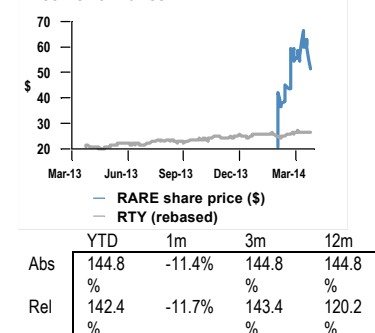
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Price Performance



Ultragenyx Pharmaceutical (RARE;RARE US)

FYE Dec	2013A	2014E (Prev)	2014E (Curr)	2015E (Prev)	2015E (Curr)
EPS Reported (\$)					
Q1 (Mar)	-	0.00	(0.35)	0.00	0.00
Q2 (Jun)	-	0.00	(0.44)	0.00	0.00
Q3 (Sep)	-	0.00	(0.53)	0.00	0.00
Q4 (Dec)	-	0.00	(0.61)	0.00	0.00
FY	(14.87)	(1.60)	(1.93)	(1.82)	(2.16)

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

Company Data

Price (\$)	51.41
Date Of Price	24 Mar 14
52-week Range (\$)	69.77-35.15
Market Cap (\$ mn)	192.51
Fiscal Year End	Dec
Shares O/S (mn)	4
Price Target (\$)	66.00
Price Target End Date	31-Dec-14

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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)	Initial data from Phase 1/2 studies in adult XLH patients	mid-2014	High
	Initiate Phase 2 trial in pediatric XLH patients	2014	Low
	Initiate adult XLH Phase 2b study	2015	Low
	Data from Phase 2 trial in pediatric XLH patients	2015	High
UX003 (rhGUS)	Report interim data from Phase 1/2 studies in MPS7	2H14	High
	Initiate 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)	Full 48-week Phase 2 data presented at AAN	April 30, 2014	High
	Data from extension study	YE14	Medium

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

Figure 3: RARE Income Statement

	2011A	2012A	2013A	1Q14E	2Q14E	3Q14E	4Q14E	2014E	2015E	2016E	2017E	2018E
Total Product Revenue												
UX003 (rhGUS)									-	-	7.8	24.0
UX007 (FAOD)									-	-	3.5	57.8
UX007G (Glut-1)									-	-	-	49.3
UX023 / KRN23 (XLH)									-	-	-	47.7
UX001 (SA-ER)												
Total Product Revenue									-	-	11.3	178.9
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	7.6	9.5	11.5	13.4	42.0	57.1	70.2	70.3	75.2
SG&A	1.8	3.3	4.5	3.0	3.6	4.4	5.0	15.9	16.7	24.0	41.3	41.8
Total Operating Expenses	6.6	16.0	32.3	10.6	13.1	15.9	18.4	57.9	73.8	94.2	113.5	134.0
Operating Income	(6.6)	(16.0)	(32.3)	(10.6)	(13.1)	(15.9)	(18.4)	(57.9)	(73.8)	(94.2)	(113.0)	4.9
Net interest & other income	(0.3)	(0.3)	(2.8)	-	-	-	-	-	-	-	-	-
Profit (Loss) before Income Tax	(6.8)	(16.3)	(35.1)	(10.6)	(13.1)	(15.9)	(18.4)	(57.9)	(73.8)	(94.2)	(113.0)	4.9
Income Tax benefit (expense)	-	-	-					-	-	-	-	(1.5)
Net Income	(6.8)	(16.3)	(35.1)	(10.6)	(13.1)	(15.9)	(18.4)	(57.9)	(73.8)	(94.2)	(113.0)	3.4
Accretion and dividends on convertible preferred stock	(0.6)	(4.0)	(15.2)									
Net Income attributable to common stockholders	(7.5)	(20.3)	(50.3)	(10.6)	(13.1)	(15.9)	(18.4)	(57.9)	(73.8)	(94.2)	(113.0)	3.4
Basic EPS	(4.62)	(2.04)	(14.87)	(0.35)	(0.44)	(0.53)	(0.61)	(1.93)	(2.16)	(2.68)	(2.86)	0.09
Diluted EPS	(4.62)	(2.04)	(14.87)	(0.35)	(0.44)	(0.53)	(0.61)	(1.93)	(2.16)	(2.68)	(2.86)	0.07

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-14 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%		
4Q14 Fully Diluted Shares (mm)	36.2		
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	\$ 69.15	50%	34.57
Risk adjusted NPV analysis	\$ 62.75	50%	31.37
Total		100%	\$ 65.95
Catalyst/liquidity discount			0%
YE14 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	1Q14E	2Q14E	3Q14E	4Q14E
Revenues	0	0	0	0	Revenues	0	0	0	0
Cost of products sold	0	0	0	0	Cost of products sold	0	0	0	0
Gross profit	-	-	-	-	Gross profit	-	-	-	-
SG&A	(4)	(16)	(17)	(24)	SG&A	(3)	(4)	(4)	(5)
R&D	(28)	(42)	(57)	(70)	R&D	(8)	(10)	(12)	(13)
Operating income	(32)	(58)	(74)	(94)	Operating income	(11)	(13)	(16)	(18)
EBITDA	(32)	(58)	(74)	(94)	EBITDA	(11)	(13)	(16)	(18)
Net interest (income) / expense	0	0	0	0	Net interest (income) / expense	0	0	0	0
Other income / (expense)	(3)	0	0	0	Other income / (expense)	0	0	0	0
Income taxes	0	0	0	0	Income taxes	0	0	0	0
Net income - GAAP	(35)	(58)	(74)	(94)	Net income - GAAP	(11)	(13)	(16)	(18)
Net income - recurring	(50)	(58)	(74)	(94)	Net income - recurring	(11)	(13)	(16)	(18)
Diluted shares outstanding	3	30	34	35	Diluted shares outstanding	30	30	30	30
EPS - excluding non-recurring	(14.87)	(1.93)	(2.16)	(2.68)	EPS - excluding non-recurring	(0.35)	(0.44)	(0.53)	(0.61)
EPS - recurring	(14.87)	(1.93)	(2.16)	(2.68)	EPS - recurring	(0.35)	(0.44)	(0.53)	(0.61)
Balance Sheet and Cash Flow Data	FY13A	FY14E	FY15E	FY16E	Ratio Analysis	FY13A	FY14E	FY15E	FY16E
Cash and cash equivalents	53	119	198	106	Sales growth	-	-	-	-
Accounts receivable	0	0	0	0	EBIT growth	-	79.4%	27.5%	27.6%
Inventories	-	-	-	-	EPS growth - recurring	-	(87.0%)	11.5%	24.0%
Other current assets	0	0	0	0	Gross margin	-	-	-	-
Current assets	53	119	198	107	EBIT margin	-	-	-	-
PP&E	1	2	2	2	EBITDA margin	-	-	-	-
Total assets	55	122	201	109	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	117	196	104	Net Debt / Capital (book)	-	-	-	-
Net income (including charges)	(35)	(58)	(74)	(94)	Return on assets (ROA)	(70.0%)	(65.4%)	(45.8%)	(60.8%)
D&A	0	0	0	0	Return on equity (ROE)	(74.2%)	(68.8%)	(47.2%)	(62.9%)
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)	(56)	(71)	(92)	Free cash flow yield	(19.1%)	(3.6%)	(4.1%)	(5.1%)
Capex	0	0	0	0					
Free cash flow	(33)	(56)	(71)	(92)					
Cash flow from investing activities	0	0	0	0					
Cash flow from financing activities	0	122	150	0					
Dividends	-	-	-	-					
Dividend yield	-	-	-	-					

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

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

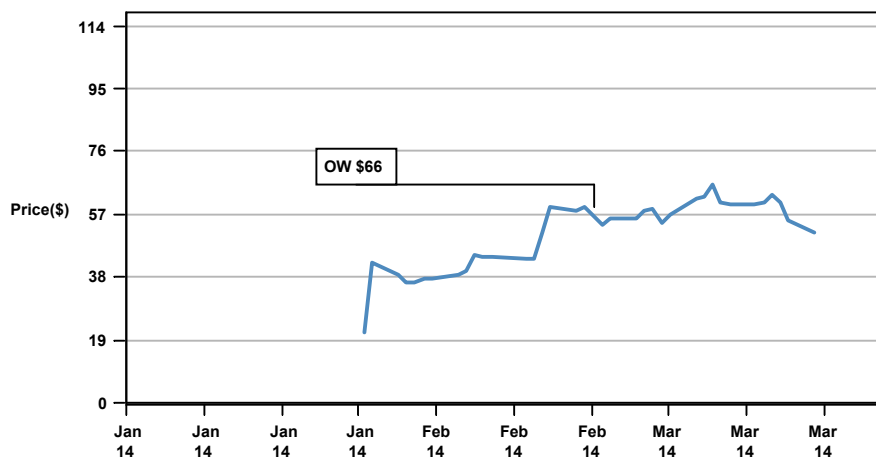
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Ultragenyx (RARE, RARE US) Price Chart



Date	Rating	Share Price (\$)	Price Target (\$)
25-Feb-14	OW	59.00	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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