

## Ultragenyx

### 3Q Snapshot & Model Update

We are updating our RARE model based on the company's 3Q results (there was no conference call). Progress with the five clinical-stage pipeline programs continues (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 data readouts expected throughout 2015 that we believe could offer meaningful de-risking and value creation events.

- **Upcoming events: several data readouts in 2015 could help further de-risk the story and be important investment catalysts.** A pivotal Phase 3 study of rhGUS in MPS 7 is expected to begin by year-end 2014. In 2015, RARE expects interim 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 (reached the target enrollment of 30 pts in Nov, ahead of schedule). In addition, RARE plans to initiate a Phase 3 trial evaluating SA-ER in HIBM patients by mid-2015 (recently completed the EOP2 meeting in which the FDA agreed on a primary endpoint of a composite of upper extremity muscle strength and a secondary endpoint of a patient-reported outcome such as the GNE myopathy-functional activity scale).
- **3Q results.** 3Q EPS was \$(0.50), roughly in-line vs. cons/JPMe of \$(0.51). 3Q OpEx of \$15.8M was also ~in-line with JPMe of \$16.6M. That said, RARE is not yet an earnings story, in our view.
- **Estimate changes; maintaining our YE15 PT of \$66.** We have updated our model for 3Q results. Our YE15 price target of \$66 is unchanged.
- **Balance sheet update.** RARE ended 3Q with ~\$201M 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.

## Overweight

RARE, RARE US

Price: \$44.71

Price Target: \$66.00

### Biotechnology

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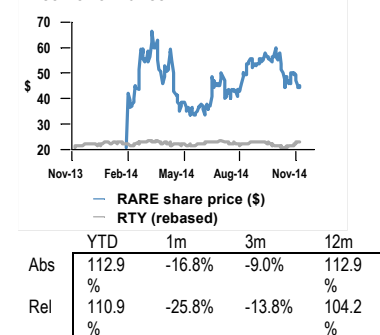
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### Price Performance



### Ultragenyx Pharmaceutical (RARE;RARE US)

FYE Dec	2013A	2014E (Prev)	2014E (Curr)	2015E (Prev)	2015E (Curr)
EPS Reported (\$)					
Q1 (Mar)	-	(0.85)A	(0.85)A	-	-
Q2 (Jun)	-	(0.45)A	(0.45)A	-	-
Q3 (Sep)	-	(0.51)	(0.50)A	-	-
Q4 (Dec)	-	(0.59)	(0.57)	-	-
FY	(14.87)	(2.32)	(2.29)	(2.21)	(2.24)

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

### Company Data

Price (\$)	44.71
Date Of Price	07 Nov 14
52-week Range (\$)	69.77-32.02
Market Cap (\$ mn)	1,417.49
Fiscal Year End	Dec
Shares O/S (mn)	32
Price Target (\$)	66.00
Price Target End Date	31-Dec-15

### See page 6 for analyst certification and important disclosures.

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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)	Interim data from Phase 2 trial in pediatric XLH patients	2015	High
	Initiate adult XLH Phase 2b study	2015	Low
UX003 (rhGUS)	Initiate pivotal Phase 3 study in MPS 7	YE14	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)	Initiate Phase 3 trial in HIBM patients	mid-2015	Low

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	3Q14A	4Q14E	2014E	2015E	2016E	2017E	2018E
<b>Total Product Revenue</b>												
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)									-	-	-	-
<b>Total Product Revenue</b>									-	-	11.3	178.9
<b>Net Revenue to Ultragenyx</b>												
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)
<b>Total Revenues</b>	-	-	-	-	-	-	-	-	-	-	0.5	138.9
COGS											1.9	17.1
R&D	4.7	12.6	27.8	8.4	11.2	12.9	14.1	46.5	58.0	71.2	71.3	76.2
SG&A	1.8	3.3	4.5	2.0	2.4	3.0	4.1	11.4	16.1	23.4	40.7	41.1
<b>Total Operating Expenses</b>	6.6	16.0	32.3	10.3	13.7	15.8	18.1	57.9	74.2	94.6	113.9	134.4
<b>Operating Income</b>	(6.6)	(16.0)	(32.3)	(10.3)	(13.7)	(15.8)	(18.1)	(57.9)	(74.2)	(94.6)	(113.4)	4.5
Net interest & other income	(0.3)	(0.3)	(2.8)	(3.3)	0.1	(0.0)	-	(3.2)	-	-	-	-
<b>Profit (Loss) before Income Tax</b>	(6.8)	(16.3)	(35.1)	(13.6)	(13.6)	(15.8)	(18.1)	(61.2)	(74.2)	(94.6)	(113.4)	4.5
Income Tax benefit (expense)	-	-	-					-	-	-	-	(1.4)
<b>Net Income</b>	(6.8)	(16.3)	(35.1)	(13.6)	(13.6)	(15.8)	(18.1)	(61.2)	(74.2)	(94.6)	(113.4)	3.2
Accretion and dividends on convertible preferred stock	(0.6)	(4.0)	(15.2)	(4.8)				(4.8)				
<b>Net Income attributable to common stockholders</b>	(7.5)	(20.3)	(50.3)	(18.4)	(13.6)	(15.8)	(18.1)	(66.0)	(74.2)	(94.6)	(113.4)	3.2
<b>Basic EPS</b>	(4.62)	(2.04)	(14.87)	(0.85)	(0.45)	(0.50)	(0.57)	(2.29)	(2.24)	(2.78)	(2.96)	0.08
<b>Diluted EPS</b>	(4.62)	(2.04)	(14.87)	(0.85)	(0.45)	(0.50)	(0.57)	(2.29)	(2.24)	(2.78)	(2.96)	0.07
Basic Shares Outstanding	1.6	9.9	3.4	21.6	30.1	31.6	31.7	28.8	33.0	34.0	38.3	39.3
<b>Margin Analysis:</b>												
Gross margin		NM	NM	NM	NM	NM	NM	NM	NM	NM	-2.72%	88%
Operating margin		NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	3.25%
Net margin		NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	2.28%
Tax Rate		0%	30%	0%	0%	0%	0%	30%	30%	30%	30%	30%
<b>Cost Analysis:</b>												
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	54.89%
SG&A as % of tot. revenue		NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	29.58%
<b>Year-over-year growth:</b>												
Total revenue			NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
R&D Expense			120.15%	47.49%	56.10%	90.09%	71.28%	67.08%	24.80%	22.66%	0.22%	6.85%
SG&A Expense			33.10%	83.35%	131.18%	198.39%	206.59%	157.01%	41.03%	44.92%	73.88%	1.04%
Total operating expenses			101.94%	53.25%	65.64%	104.03%	90.05%	79.48%	28.01%	27.50%	20.43%	17.98%
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	754.21%	747.41%	749.97%	14.91%	3.03%	12.59%	2.61%
Diluted Shares			-65.96%	NM	NM	754.21%	747.41%	749.97%	14.91%	3.03%	12.59%	10.22%

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)	36.0		
<b>Main value drivers</b>	<b>Prob of approval</b>	<b>Peak WW sales est (avg. scenario)</b>	<b>Avg peak yr</b>
KRN23	65%	\$ 275	2024
rhGUS	75%	\$ 75	2022
Tripheptanoin (FAOD)	45%	\$ 300	2024
Tripheptanoin (Glut1 DS)	45%	\$ 775	2024
SA-ER	30%	\$ 125	2022
<b>Valuation methodology</b>	<b>Value / share</b>	<b>Weighting</b>	<b>Adj. value/ share</b>
Real options scenario analysis	\$ 67.83	50%	33.91
Risk adjusted NPV analysis	\$ 63.46	50%	31.73
Total		100%	\$ 65.65
Catalyst/liquidity discount			0%
<b>YE15 Price Target</b>			<b>\$ 66</b>

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	3Q14A	4Q14E
Revenues	0	0	0	0	Revenues	0A	0A	0A	0
Cost of products sold	0	0	0	0	Cost of products sold	0A	0A	0A	0
Gross profit	-	-	-	-	Gross profit	-	-	-	-
SG&A	(4)	(11)	(16)	(23)	SG&A	(2)A	(2)A	(3)A	(4)
R&D	(28)	(46)	(58)	(71)	R&D	(8)A	(11)A	(13)A	(14)
Operating income	(32)	(58)	(74)	(95)	Operating income	(10)A	(14)A	(16)A	(18)
EBITDA	(32)	(58)	(74)	(95)	EBITDA	(10)A	(14)A	(16)A	(18)
Net interest (income) / expense	0	0	0	0	Net interest (income) / expense	0A	0A	0A	0
Other income / (expense)	(3)	(3)	0	0	Other income / (expense)	(3)A	0A	(0)A	0
Income taxes	0	0	0	0	Income taxes	0A	0A	0A	0
Net income - GAAP	(35)	(61)	(74)	(95)	Net income - GAAP	(14)A	(14)A	(16)A	(18)
Net income - recurring	(50)	(66)	(74)	(95)	Net income - recurring	(18)A	(14)A	(16)A	(18)
Diluted shares outstanding	3	29	33	34	Diluted shares outstanding	22A	30A	32A	32
EPS - excluding non-recurring	(14.87)	(2.29)	(2.24)	(2.78)	EPS - excluding non-recurring	(0.85)A	(0.45)A	(0.50)A	(0.57)
EPS - recurring	(14.87)	(2.29)	(2.24)	(2.78)	EPS - recurring	(0.85)A	(0.45)A	(0.50)A	(0.57)
Balance Sheet and Cash Flow Data	FY13A	FY14E	FY15E	FY16E	Ratio Analysis	FY13A	FY14E	FY15E	FY16E
Cash and cash equivalents	53	176	254	162	Sales growth	-	-	-	-
Accounts receivable	0	0	0	0	EBIT growth	-	79.5%	28.0%	27.5%
Inventories	-	-	-	-	EPS growth - recurring	-	(84.6%)	(2.2%)	23.8%
Other current assets	0	0	0	0	Gross margin	-	-	-	-
Current assets	53	176	255	163	EBIT margin	-	-	-	-
PP&E	1	2	2	2	EBITDA margin	-	-	-	-
Total assets	55	179	257	166	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	174	252	160	Net Debt / Capital (book)	-	-	-	-
Net income (including charges)	(35)	(61)	(74)	(95)	Return on assets (ROA)	(70.0%)	(56.4%)	(34.0%)	(44.7%)
D&A	0	0	0	0	Return on equity (ROE)	(74.2%)	(58.6%)	(34.8%)	(45.9%)
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)	(59)	(72)	(92)	Free cash flow yield	(22.0%)	(4.6%)	(4.9%)	(6.0%)
Capex	0	0	0	0					
Free cash flow	(33)	(59)	(72)	(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



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

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