

Ultragenyx

1Q Snapshot & Model Update

We are updating our RARE model based on the company's 1Q results (there was no conference call). Progress with the five clinical-stage pipeline programs is on track (evidenced by recent positive data updates for 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 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 help de-risk the story and be important investment catalysts.** Phase 1/2 data for KRN23 in XLH is expected in 2014, and interim Phase 1/2 data for rhGUS in MPS 7 is anticipated in 2H14. 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.
- **1Q results.** 1Q EPS was \$(0.85), wider vs. cons of (\$0.38) and JPMe of (\$0.35) due to dividends and other charges related to outstanding preferred stock, which was converted into common shares upon the IPO. 1Q OpEx of \$10.3M was ~in line with JPMe of \$10.6M. That said, RARE is not yet an earnings story.
- **Estimate changes; maintaining \$66 YE14 PT.** We have updated our model for 1Q results; changes to our 2014 estimates were minor. Our YE14 PT remains at \$66.
- **Balance sheet update.** RARE ended 1Q with \$165M in cash (and no debt), which should be sufficient capital to fund operations into 2016.

Overweight

RARE, RARE US

Price: \$34.04

Price Target: \$66.00

Biotechnology

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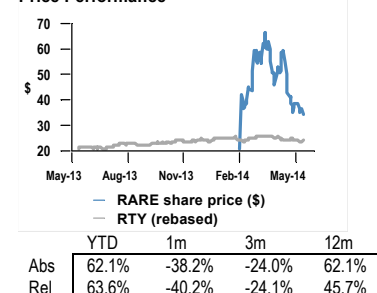
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Price Performance



Ultragenyx Pharmaceutical (RARE;RARE US)

| FYE Dec | 2013A | 2014E (Prev) | 2014E (Curr) | 2015E (Prev) | 2015E (Curr) |
|-----------------------|---------|-----------------|-----------------|-----------------|-----------------|
| EPS Reported (\$) | | | | | |
| Q1 (Mar) | - | (0.35) | (0.85)A | 0.00 | - |
| Q2 (Jun) | - | (0.44) | (0.45) | 0.00 | - |
| Q3 (Sep) | - | (0.53) | (0.55) | 0.00 | - |
| Q4 (Dec) | - | (0.61) | (0.63) | 0.00 | - |
| FY | (14.87) | (1.93) | (2.41) | (2.16) | (2.28) |
| Bloomberg EPS FY (\$) | -3.04 | - | -1.82 | - | -2.10 |

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

Company Data

| | |
|-----------------------|-------------|
| Price (\$) | 34.04 |
| Date Of Price | 12 May 14 |
| 52-week Range (\$) | 69.77-33.70 |
| Market Cap (\$ mn) | 734.67 |
| Fiscal Year End | Dec |
| Shares O/S (mn) | 22 |
| Price Target (\$) | 66.00 |
| Price Target End Date | 31-Dec-14 |

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) | Initial data from Phase 1/2 studies in adult XLH patients | 2014 | High |
| | Initiate Phase 2 trial in pediatric XLH patients | 2H14 | 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 |
| | 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) | Data from extension study | YE14 | 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 | 2Q14E | 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 | 10.2 | 12.2 | 14.1 | 44.9 | 57.1 | 70.2 | 70.3 | 75.2 |
| SG&A | 1.8 | 3.3 | 4.5 | 2.0 | 3.3 | 4.4 | 5.0 | 14.6 | 16.7 | 24.0 | 41.3 | 41.7 |
| Total Operating Expenses | 6.6 | 16.0 | 32.3 | 10.3 | 13.5 | 16.6 | 19.1 | 59.5 | 73.8 | 94.2 | 113.5 | 133.9 |
| Operating Income | (6.6) | (16.0) | (32.3) | (10.3) | (13.5) | (16.6) | (19.1) | (59.5) | (73.8) | (94.2) | (113.0) | 4.9 |
| Net interest & other income | (0.3) | (0.3) | (2.8) | (3.3) | - | - | - | (3.3) | - | - | - | - |
| Profit (Loss) before Income Tax | (6.8) | (16.3) | (35.1) | (13.6) | (13.5) | (16.6) | (19.1) | (62.7) | (73.8) | (94.2) | (113.0) | 4.9 |
| Income Tax benefit (expense) | - | - | - | | | | | - | - | - | - | (1.5) |
| Net Income | (6.8) | (16.3) | (35.1) | (13.6) | (13.5) | (16.6) | (19.1) | (62.7) | (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) | (13.5) | (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) | (0.55) | (0.63) | (2.41) | (2.28) | (2.82) | (3.00) | 0.09 |
| Diluted EPS | (4.62) | (2.04) | (14.87) | (0.85) | (0.45) | (0.55) | (0.63) | (2.41) | (2.28) | (2.82) | (3.00) | 0.08 |
| Basic Shares Outstanding | 1.6 | 9.9 | 3.4 | 21.6 | 30.1 | 30.2 | 30.3 | 28.1 | 32.4 | 33.4 | 37.7 | 38.7 |
| Shares added in the period | - | 8.3 | - | | 0.1 | 0.1 | 0.1 | 0.3 | 4.3 | 1.0 | 4.3 | 1.0 |
| Diluted Shares Outstanding | 1.6 | 9.9 | 3.4 | 21.6 | 30.1 | 30.2 | 30.3 | 28.1 | 32.4 | 33.4 | 37.7 | 41.3 |
| 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 | 3.715185 | 0.122797 |
| R&D as % of tot. revenue | | NM | NM | NM | NM | NM | NM | NM | NM | NM | 13820.23% | 54.11% |
| SG&A as % of tot. revenue | | NM | NM | NM | NM | NM | NM | NM | NM | NM | 8114.75% | 30.05% |
| Year-over-year growth: | | | | | | | | | | | | |
| Total revenue | | | NM | NM | NM | NM | NM | NM | NM | NM | NM | 27195.12% |
| R&D Expense | | 120.15% | 47.49% | 41.74% | 80.49% | 71.93% | 61.22% | 27.26% | 22.95% | 0.16% | 6.88% | |
| SG&A Expense | | 33.10% | 83.35% | 214.98% | 335.42% | 274.72% | 227.71% | 14.62% | 43.49% | 72.10% | 1.09% | |
| Total operating expenses | | 101.94% | 53.25% | 63.74% | 113.31% | 100.05% | 84.18% | 24.16% | 27.60% | 20.49% | 18.02% | |
| Operating income | | NM | NM | NM | NM | NM | NM | NM | NM | NM | NM | NM |
| Net income | | NM | NM | NM | NM | NM | NM | NM | NM | NM | NM | NM |
| EPS | | NM | NM | NM | NM | NM | NM | NM | NM | NM | NM | NM |
| Basic Shares | | -65.96% | NM | NM | 716.89% | 710.51% | 730.24% | 15.26% | 3.09% | 12.84% | 2.66% | |
| Diluted Shares | | -65.96% | NM | NM | 716.89% | 710.51% | 730.24% | 15.26% | 3.09% | 12.84% | 9.74% | |

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 | 1Q14A | 2Q14E | 3Q14E | 4Q14E |
|-------------------------------------|---------|--------|--------|--------|---------------------------------|---------|---------|---------|---------|
| Revenues | 0 | 0 | 0 | 0 | Revenues | 0A | 0 | 0 | 0 |
| Cost of products sold | 0 | 0 | 0 | 0 | Cost of products sold | 0A | 0 | 0 | 0 |
| Gross profit | - | - | - | - | Gross profit | - | - | - | - |
| SG&A | (4) | (15) | (17) | (24) | SG&A | (2)A | (3) | (4) | (5) |
| R&D | (28) | (45) | (57) | (70) | R&D | (8)A | (10) | (12) | (14) |
| Operating income | (32) | (59) | (74) | (94) | Operating income | (10)A | (14) | (17) | (19) |
| EBITDA | (32) | (59) | (74) | (94) | EBITDA | (10)A | (14) | (17) | (19) |
| Net interest (income) / expense | 0 | 0 | 0 | 0 | Net interest (income) / expense | 0A | 0 | 0 | 0 |
| Other income / (expense) | (3) | (3) | 0 | 0 | Other income / (expense) | (3)A | 0 | 0 | 0 |
| Income taxes | 0 | 0 | 0 | 0 | Income taxes | 0A | 0 | 0 | 0 |
| Net income - GAAP | (35) | (63) | (74) | (94) | Net income - GAAP | (14)A | (14) | (17) | (19) |
| Net income - recurring | (50) | (68) | (74) | (94) | Net income - recurring | (18)A | (14) | (17) | (19) |
| Diluted shares outstanding | 3 | 28 | 32 | 33 | Diluted shares outstanding | 22A | 30 | 30 | 30 |
| EPS - excluding non-recurring | (14.87) | (2.41) | (2.28) | (2.82) | EPS - excluding non-recurring | (0.85)A | (0.45) | (0.55) | (0.63) |
| EPS - recurring | (14.87) | (2.41) | (2.28) | (2.82) | EPS - recurring | (0.85)A | (0.45) | (0.55) | (0.63) |
| Balance Sheet and Cash Flow Data | FY13A | FY14E | FY15E | FY16E | Ratio Analysis | FY13A | FY14E | FY15E | FY16E |
| Cash and cash equivalents | 53 | 114 | 193 | 101 | Sales growth | - | - | - | - |
| Accounts receivable | 0 | 0 | 0 | 0 | EBIT growth | - | 84.2% | 24.2% | 27.6% |
| Inventories | - | - | - | - | EPS growth - recurring | - | (83.8%) | (5.2%) | 23.8% |
| Other current assets | 0 | 0 | 0 | 0 | Gross margin | - | - | - | - |
| Current assets | 53 | 115 | 193 | 102 | EBIT margin | - | - | - | - |
| PP&E | 1 | 2 | 2 | 2 | EBITDA margin | - | - | - | - |
| Total assets | 55 | 117 | 196 | 104 | 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 | 112 | 191 | 99 | Net Debt / Capital (book) | - | - | - | - |
| Net income (including charges) | (35) | (63) | (74) | (94) | Return on assets (ROA) | (70.0%) | (78.4%) | (47.2%) | (62.8%) |
| D&A | 0 | 0 | 0 | 0 | Return on equity (ROE) | (74.2%) | (82.7%) | (48.7%) | (65.0%) |
| 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 | (28.9%) | (6.3%) | (6.5%) | (8.1%) |
| 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 | 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

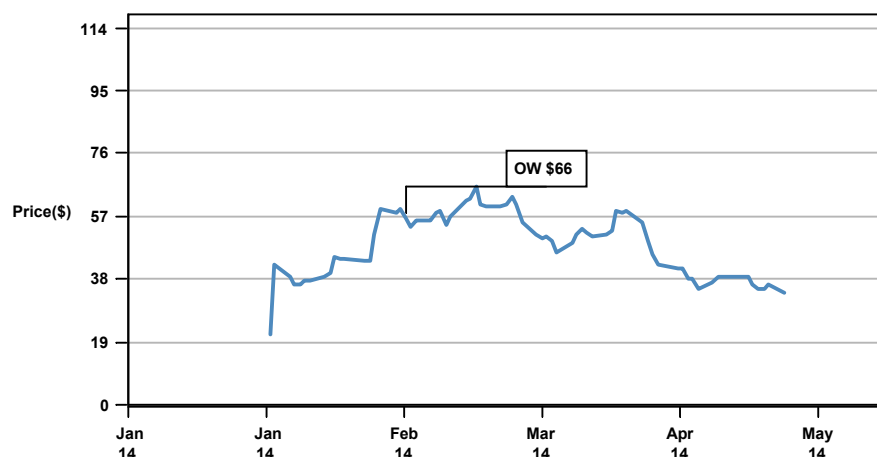
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Ultragenyx (RARE, RARE US) Price Chart



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