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May 15, 2014

Stock Rating
Overweight

Industry View
In-Line

Ultragenyx Pharmaceutical Inc

1Q Update In-Line; A Few HIBM Notes from AAN Data

RARE reported an uneventful 1Q earnings. We saw incremental data in HIBM that yielded a few learnings in advance of higher dose data .

Ultragenyx reported a mostly uneventful 1Q. The focus for us and the Street remains on pipeline progress.

KRN23 (UX023 for X-linked hypophosphatemia): This drug completed Ph 1/2 in adults, with the first set of data expected in mid-2014 at the ENDO meeting. We expect the data release to mainly focus on laboratory changes over time with repeat dosing. The company, as planned, will be entering a pediatric Ph 2 study in 2H14.

Triheptanoin (UX007 for GLUT-1-DS and FAOD): This drug is currently being tested in two enrolling Ph 2 studies – 1 in GLUT-1-DS pts and 1 in FAOD pts. Data are expected from both trials in 2015.

rhGUS (UX003 for MPS 7): The first three treated pts have reported out, with good urinary GAG changes and incremental liver size improvements at 3 mo with no infusion rxns. We expect more 3 mo data 2H14.

ER-Sialic Acid (UX001 for HIBM): To date, the drug (3 and 6 gm/d) has been tested for 48 wks in 47 HIBM pts. The company is now testing 12 gm/d, with muscle strength data expected in 2H14.

At the recent AAN mtg, the company presented the full 48 wk UX001 data at 3 and 6 gm. One interesting point in the data is that while the company has decided to eval a 12 gm dose, the 6 gm dose did show signs of solid upper extremity (UE) benefit. UE strength was essentially maintained in the 6 gm arm through 48 wks vs. the 3 gm arm where strength declined by ~3.5kg (~8.5%). This benefit held up even when the majority of placebo pts rotated into that arm @ 24 wks. Given that a) the disease is one of progressive muscle wasting with atrophic features, and b) the pts at baseline had almost 2x the % of expected strength in upper vs. lower extremities (LE), we are not surprised to see more of a benefit there. This benefit was even more clear in pts with more preserved LE function, suggestive of a milder overall pt. We will look for data at higher dose(s) and potentially in earlier stage pts as well to best determine the value of this asset (we currently ascribe none).

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Key Ratios and Statistics

Reuters: RARE.O Bloomberg: RARE US

Biotechnology / United States of America

Price target	\$81.00
Shr price, close (May 14, 2014)	\$35.26
Mkt cap, curr (mm)	\$1,061
52-Week Range	\$69.77-33.02

Fiscal Year ending	12/13	12/14e	12/15e	12/16e
ModelWare EPS (\$)	(10.37)	(1.83)	(2.13)	(2.79)
Prior ModelWare EPS (\$)	-	(1.66)	(2.15)	(2.82)
P/E	NM	NM	NM	NM
Consensus EPS (\$)	(1.52)	(1.82)	(2.13)	(2.60)
Div yld (%)	-	0.0	0.0	0.0

Unless otherwise noted, all metrics are based on Morgan Stanley ModelWare framework (please see explanation later in this note).

\$ = Consensus data is provided by Thomson Reuters Estimates.

e = Morgan Stanley Research estimates

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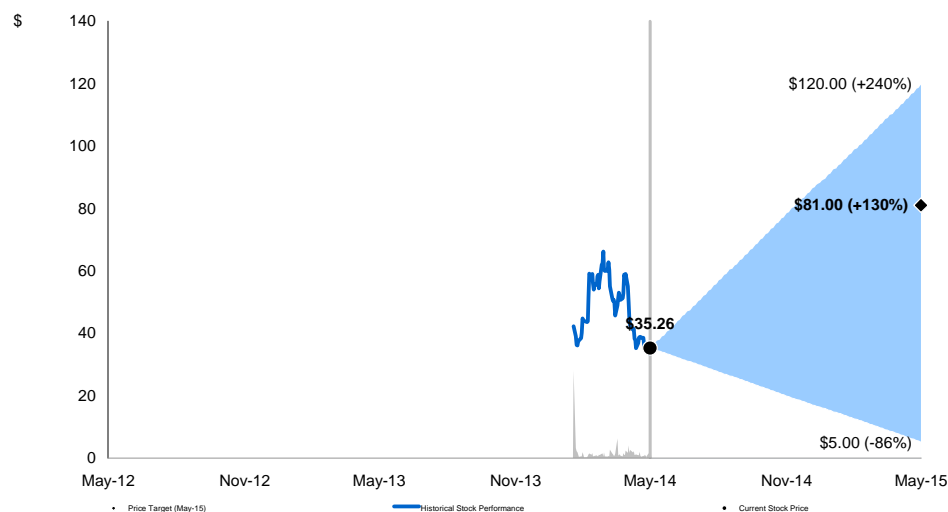
For analyst certification and other important disclosures, refer to the Disclosure Section, located at the end of this report.

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Ultragenyx Pharmaceutical Inc

Risk-Reward Snapshot: Ultragenyx (RARE, OW, PT \$81)

Triheptanoin, KRN23, and rhGUS Drive Risk-Reward



Source: Morgan Stanley Research estimates, Thomson Reuters

Price Target \$81	We derive our PT from a discounted cash flow analysis that uses a WACC of 15% and a 0% terminal growth rate. The main revenue drivers in our model are the launches of triheptanoin, KRN23, and rhGUS.	
Bull Case \$120	DCF	Triheptanoin, KRN23, rhGUS, and sialic acid extended release are all approved globally with better market penetration than in our base case. We assume: <ol style="list-style-type: none"> 1) ~\$1.1bn WW peak (2025) sales for triheptanoin in Glut-1 DS, 2) ~\$720mn WW peak (2025) sales for triheptanoin in LC-FAOD, 3) ~\$1.9bn WW peak (2025) sales for KRN23 in XLH, 4) ~\$80mn WW peak (2025) sales for rhGUS in MPS 7, and 5) ~\$160mn WW peak (2025) sales for sialic acid ER in HIBM.
Base Case \$81	DCF	Triheptanoin, KRN23, and rhGUS are all approved globally with good commercial penetration. Sialic-acid extended release does not make it to market. We assume: <ol style="list-style-type: none"> 1) ~\$840mn WW peak (2025) sales for triheptanoin in Glut-1 DS, 2) ~\$560mn WW peak (2025) sales for triheptanoin in LC-FAOD, 3) ~\$1.5bn WW peak (2025) sales for KRN23 in XLH, and 4) ~\$70mn WW peak (2025) sales for rhGUS in MPS 7.
Bear Case \$5	Cash Based Value	None of the drugs in development makes it to market. <p>We view this scenario as low probability given proof-of-concept data for multiple drugs in relevant diseases. We expect the stock would trade at or modestly below cash in this scenario as the remaining pipeline, while interesting, is much earlier and hard to place concrete values on.</p>

Investment Thesis

- We are OW Ultragenyx as we believe the company has multiple compelling orphan drugs in development.
 - Triheptanoin (UX007), a synthetic medium chain triglyceride, for Glut-1 DS (glucose transported type-1 deficiency syndrome) and LC-FAODs (long chained fatty acid oxidation disorders) has shown solid anecdotal efficacy and a decent safety profile.
 - KRN23 (UX0023) for XLH (X-linked hypophosphatemia) had improvements in serum phosphate and active vitamin D in a Ph 1 trial, and we see a clear commercial opportunity particularly in the more severe pediatric patients.
 - rhGUS (UX003), an enzyme replacement therapy for MPS 7, is early with data in one patient, but is likely to be an additional revenue driver for the company.
 - Sialic acid – extended release (UX001) for HIBM (hereditary inclusion body myopathy) is upside to our model. Efficacy data have looked modest so far, but data at higher doses may be more interesting.
- ### Risks to our price target
- 1) Ultragenyx's drugs may fail in clinical testing due to an efficacy or safety issue,
 - 2) Ultragenyx's drugs may not have good commercial uptake due to alternative standard of care options,
 - 3) target markets may be smaller than expected.

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Ultragenyx Pharmaceutical Inc

Ultragenyx Valuation

Exhibit 1

DCF Drives Valuation

	2013	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E	2033E	2034E	2035E
Free Cash Flow	(\$31.61)	(\$53.32)	(\$58.03)	(\$76.22)	(\$105.62)	(\$73.72)	\$116.89	\$391.85	\$528.05	\$721.91	\$858	\$955	\$1,034	\$827	\$662	\$675	\$679	\$680	\$680	\$680	\$544	\$435	\$348
YoY Growth	132.5%	68.7%	8.8%	31.4%	38.6%	-30.2%	-258.6%	235.2%	34.8%	36.7%	18.9%	11.3%	8.3%	-20.0%	-20.0%	2.1%	0.5%	0.1%	0.0%	0.0%	-20.0%	-20.0%	-20.0%
Net Cash Proxy for Dilution		(\$6)	(\$6)	(\$7)	(\$8)	(\$9)	(\$10)	(\$11)	(\$9)	(\$10)	(\$11)	(\$12)	(\$13)	(\$13)	(\$13)	(\$13)	(\$13)	(\$13)	(\$13)	(\$13)	(\$13)	(\$13)	(\$13)
Free Cash Flow for DCF	(\$32)	(\$59)	(\$64)	(\$84)	(\$114)	(\$83)	\$107	\$381	\$519	\$712	\$847	\$943	\$1,021	\$814	\$648	\$662	\$666	\$667	\$667	\$667	\$531	\$422	\$335
Present Value of Free Cash Flow		(\$59)	(\$58)	(\$65)	(\$78)	(\$49)	\$55	\$171	\$202	\$241	\$249	\$241	\$227	\$158	\$109	\$111	\$112	\$112	\$112	\$112	\$89	\$71	\$56

Source: Company data, Morgan Stanley Research estimates

Exhibit 2

DCF Valuation Suggests Significant Upside

Valuation Date	2014.25
Discount Rate	15%
Terminal Growth Rate	0%
Terminal Value Year	2035

Sum of Discounted FCF	\$2,136
Discounted Terminal Value	\$123
Net Cash	\$165
Equity Value	\$2,424
Equity Value/Sh (Basic)	\$81
Shares Outstanding (Basic)	30.1

Source: Company Data, Morgan Stanley Research estimates

\$81 PT includes triheptanoin for Glut-1 DS and LC-FAOD, KRN23 in XLH, and rhGUS in MPS 7.

We derive our PT from a discounted cash flow (DCF) analysis that uses a WACC of 15% and a terminal growth rate of 0% post 2030. We incorporate the cash cost of stock options.

Valuation Methodology: We use a DCF to value Ultragenyx as well as most other companies under coverage. We believe a DCF best captures the longer term nature of drug development and commercialization. We do not feel that a multiples analysis accomplishes the same goal, as it only evaluates a company during a snapshot in time.

Discount Rate: We typically use a discount rate of 15% for development stage companies. We see this rate as reasonable for Ultragenyx as their drugs have clinical and regulatory risk. **We currently use a 12.5% discount rate for companies that have proven proof of concept in a formal, company-run Ph 2 trial for a key value driving drug. We believe that ongoing trials for KRN23 and triheptanoin, if successful, could ultimately yield this important, formalized proof of concept leading to a valuation**

step-up based on declining risk. We use a 10% discount rate for commercial stage companies.

Terminal Growth Rate: Our modeled cash flows extend to 2025. Beyond this, we use a 20% decline in 2026 and 2027 due to triheptanoin patent expiry. We decline cash flows by 20% annually from 2033-2035 due to the ~2030 composition of matter patent expiry for KRN23 (the final drug in our model). Beyond 2035, we use a terminal growth rate of 0%.

Revenue: The revenue drivers in our model are sales of 1) triheptanoin for Glut-1 DS and LC-FAODs, 2) KRN23 in XLH, and 3) rhGUS in MPS 7.

Economics: Triheptanoin – Ultragenyx licensed WW rights to triheptanoin from Baylor and pays them a mid-single digit royalty. **KRN23** – Ultragenyx shares rights to KRN23 with Kyowa Hakko Kirin. In the US, there is a profit split that switches to a mid-high 20% royalty after 5 years. In the EU, Ultragenyx receives a 10% royalty. Ultragenyx has full rights to KRN23 in the Latin America and pays a 10% royalty to Kyowa Hakko Kirin. **rhGUS** – Ultragenyx licensed WW rights from St. Louis University and pays a low single digit royalty.

COGS: We model COGS of 15% at drug launches improving to 13% over time.

Operating Expenses:

R&D: We model R&D increasing through 2025 as Ultragenyx continues developing their pipeline.

SG&A: We model SG&A increasing significantly in 2017+ as Ultragenyx builds out a WW sales force.

Financings: We model a ~\$90mn raise in 2016 and a ~\$200mn raise in 2017.

Key Risks To Our Price Target Include: 1) Ultragenyx's drugs may fail in clinical testing due to an efficacy or safety issue, 2) Ultragenyx's drugs may not have good commercial uptake due to alternative standard of care options, 3) target markets may be smaller than expected.

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Ultragenyx Pharmaceutical Inc

Exhibit 3

Ultragenyx Catalyst Calendar

Drug	Type	Event	Expected Timing
KRN23	Clinical Data	Ph 1/2 multidose data in XLH adult patients (ENDO - June)	2Q14
KRN23	Product Advancement	Initiate Ph 2 study in XLH pediatric patients	2H14
rhGUS (UX003)	Clinical Data	Ph 1/2 interim 12 week data	2H14
rhGUS (UX003)	Product Advancement	Ph 3 initiation (gated by Ph 1/2 interim data)	2H14
Sialic Acid - ER	Clinical Data	Ph 2 extension trial at higher doses data	2H14
Triheptanoin (UX007)	Clinical Data	Ph 2 in Glut-1 DS	2015
Triheptanoin (UX007)	Clinical Data	Ph 2 data in LC-FAOD	2H15

Source: Company Data, Morgan Stanley Research

Exhibit 4

Ultragenyx Variance Table

	4Q13 Actual	1Q14 MS Est	1Q14 Actual	% Var. from est	Q/Q % chg
Total Revenues	\$0	\$0	\$0		
R&D	\$8	\$8	\$8	-4.4%	-5.9%
SG&A	\$1	\$2	\$2	-5.2%	54.1%
Total Operating Expenses	\$9	\$10	\$10	-4.6%	2.0%
Operating Income (Loss)	(\$9)	(\$10)	-\$10	-4.6%	2.0%
Net Income (Loss)	(\$11)	(\$11)	-\$18	65.4%	62.9%

Source: Company Data, Morgan Stanley Research

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Ultragenyx Pharmaceutical Inc

Exhibit 5

Ultragenyx Changes to Model

	2012	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
WW Triheptanoin Sales (\$mn)														
Current	0	0	0	0	0	0	59	226	486	742	982	1,162	1,286	1,393
Prior	0	0	0	0	0	0	59	226	486	742	982	1,162	1,286	1,393
WW KRN23 Sales (\$mn)														
Current	0	0	0	0	0	0	87	400	781	1,037	1,216	1,334	1,398	1,453
Prior	0	0	0	0	0	0	87	400	781	1,037	1,216	1,334	1,398	1,453
WW rhGUS Sales (\$mn)														
Current	0	0	0	0	0	2	17	41	57	65	69	70	72	74
Prior	0	0	0	0	0	2	17	41	57	65	69	70	72	74
WW Total Revenue (\$mn)														
Current	0	0	0	0	0	2	77	350	766	1,146	1,464	1,683	1,840	1,969
Prior	0	0	0	0	0	2	77	350	766	1,146	1,464	1,683	1,840	1,969
COGS (\$mn)														
Current	0.0	0.0	0.0	0.0	0.0	0.1	10.9	59.3	137.2	193.5	249.5	291.1	319.3	341.7
Prior	0.0	0.0	0.0	0.0	0.0	0.1	10.9	59.3	137.2	193.5	249.5	291.1	319.3	341.7
SG&A (\$mn)														
Current	2.6	4.1	9.1	13.6	27.6	55.1	76.4	92.6	109.5	125.8	128.8	131.9	135.2	138.5
Prior	2.6	4.1	9.2	13.8	27.7	55.3	76.7	92.9	109.8	126.1	129.2	132.3	135.5	138.9
R&D (\$mn)														
Current	12.5	27.5	34.6	45.0	51.8	54.4	57.1	60.0	63.0	66.1	67.4	68.8	70.2	71.6
Prior	12.5	27.5	35.0	45.5	52.3	54.9	57.7	60.6	63.6	66.8	68.1	69.5	70.9	72.3
Interest, Other Income, net														
Current	(0.3)	(2.8)	(3.1)	0.2	0.2	0.3	0.3	0.4	0.9	1.9	3.3	5.1	7.1	9.4
Prior	(0.3)	(2.8)	(0.8)	0.2	0.2	0.3	0.3	0.4	0.9	2.0	3.3	5.1	7.1	9.4
Net Income, non-GAAP, ex-ESO (\$mn)														
Current	(15)	(34)	(47)	(58)	(79)	(107)	(67)	139	427	557	746	874	965	1,041
Prior	(15)	(34)	(45)	(59)	(80)	(108)	(68)	138	427	556	745	873	964	1,040
Diluted EPS with option expense														
Current	(\$3.78)	(\$10.37)	(\$2.00)	(\$2.13)	(\$2.79)	(\$3.46)	(\$2.19)	\$3.58	\$11.60	\$15.22	\$20.38	\$23.81	\$26.22	\$28.20
Prior	(\$3.78)	(\$10.37)	(\$1.66)	(\$2.15)	(\$2.82)	(\$3.48)	(\$2.21)	\$3.56	\$11.60	\$15.20	\$20.36	\$23.79	\$26.20	\$28.18
Diluted Shares Outstanding														
Current	4.3	3.4	28.0	30.3	31.2	33.6	35.2	35.7	35.8	36.0	36.1	36.2	36.4	36.5
Prior	4.3	3.4	27.9	30.3	31.2	33.6	35.2	35.7	35.8	36.0	36.1	36.2	36.4	36.5

Source: Company Data, Morgan Stanley Research

Exhibit 6

Ultragenyx Quarterly Income Statement

(\$ in millions)	1Q14E	2Q14E	3Q14E	4Q14E	2014E
Revenues	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00
Cost of Sales	0.0	0.0	0.0	0.0	0
% total product sales	-	-	-	-	-
R&D	7.6	8.5	9.0	9.5	34.65
% of revenue	-	-	-	-	-
SG&A	1.9	2.2	2.4	2.6	9.10
% of revenue	-	-	-	-	-
Total Operating Expenses	\$9.544	\$10.7	\$11.4	\$12.1	\$43.7
Operating Income (Loss)	(\$9.544)	(\$10.7)	(\$11.4)	(\$12.1)	(\$43.7)
Operating Margin	-	-	-	-	-
Interest income	0.09	0.1	0.1	0.1	0.24
Interest expense and other	-3.38	0.0	0.0	0.0	-3.38
Pretax Income (Loss)	(\$12.84)	(\$10.65)	(\$11.35)	(\$12.05)	(\$46.89)
Provision for Income Taxes	0	0	0	0	0
Effective tax rate	-	-	-	-	-
Net Income (Loss)	(\$12.84)	(\$10.65)	(\$11.35)	(\$12.05)	(\$46.89)
Options Expense	0.80	1.00	1.20	1.40	4.38
% of operating expense	8.3%	9.3%	10.5%	11.5%	10.0%
Tax benefit from options	-	-	-	-	-
Net Income (incl. options expense)	(\$13.63)	(\$11.65)	(\$12.55)	(\$13.45)	(\$51.27)
EPS, diluted (incl. options expense)	(\$0.63)	(\$0.39)	(\$0.42)	(\$0.44)	(\$1.83)
Basic Shares Outstanding	21.58	30.09	30.18	30.27	28.03
Diluted Shares Outstanding	21.58	30.09	30.18	30.27	28.03
Restructuring Charges	0	0	0	0	0
GAAP Net Income (incl. options)	(\$18.44)	(\$11.65)	(\$12.55)	(\$13.45)	(\$56.07)
GAAP EPS (dil, incl. options)	(\$0.85)	(\$0.39)	(\$0.42)	(\$0.44)	(\$2.00)

Source: Company Data, Morgan Stanley Research

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Ultragenyx Pharmaceutical Inc

Exhibit 7

Ultragenyx Annual Income Statement

(\$ in millions)	2011	2012	2013	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
Triheptanoin (UX007)															
US sales			\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$25.2	\$98.2	\$193.7	\$281.6	\$354.8	\$408.8	\$452.6	\$497.0
EU sales			\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$33.5	\$127.8	\$247.2	\$362.3	\$473.4	\$544.9	\$584.0	\$622.1
ROW sales			\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$44.8	\$97.7	\$153.8	\$207.8	\$249.3	\$274.0
Total Triheptanoin (UX007) Sales			\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$58.7	\$226.0	\$485.7	\$741.6	\$982.0	\$1,161.5	\$1,286.0	\$1,393.1
rhGUS (UX003)															
US sales			\$0.0	\$0.0	\$0.0	\$0.0	\$0.7	\$5.7	\$13.9	\$19.8	\$22.9	\$24.5	\$25.4	\$26.3	\$27.3
ROW sales			\$0.0	\$0.0	\$0.0	\$0.0	\$1.4	\$11.2	\$26.8	\$37.4	\$42.4	\$44.4	\$45.1	\$45.8	\$46.5
Total rhGUS (UX003) Sales			\$0.0	\$0.0	\$0.0	\$0.0	\$2.1	\$16.9	\$40.7	\$57.2	\$65.3	\$68.9	\$70.5	\$72.1	\$73.8
Sialic Acid-ER (UX001)															
US sales			\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0
ROW sales			\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0
Total Sialic Acid-ER (UX001) Sales			\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0
KRN23 (UX0023)															
US Sales			\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$35.5	\$156.0	\$285.7	\$363.9	\$425.7	\$467.2	\$491.5	\$516.2
EU Sales					\$0.0	\$0.0	\$0.0	\$51.1	\$220.3	\$395.4	\$493.8	\$566.3	\$609.3	\$628.5	\$647.1
ROW Sales					\$0.0	\$0.0	\$0.0	\$23.2	\$100.1	\$224.4	\$279.7	\$324.4	\$357.4	\$378.3	\$389.4
Total KRN23 Sales					\$0.0	\$0.0	\$0.0	\$86.6	\$399.5	\$781.3	\$1,037.4	\$1,216.4	\$1,333.9	\$1,398.3	\$1,452.8
US Revenue to Ultragenyx					\$0.0	\$0.0	\$0.0	-\$3.7	\$38.4	\$83.8	\$110.4	\$132.3	\$133.2	\$140.4	\$147.9
EU Revenue to Ultragenyx					\$0.0	\$0.0	\$0.0	\$5.1	\$22.0	\$39.5	\$49.4	\$56.6	\$60.9	\$62.8	\$64.7
ROW Revenue to Ultragenyx					\$0.0	\$0.0	\$0.0	\$0.0	\$23.2	\$100.1	\$179.7	\$224.4	\$257.4	\$278.3	\$289.4
Total KRN23 Revenue to Ultragenyx					\$0.0	\$0.0	\$0.0	\$1.4	\$83.7	\$223.5	\$339.5	\$413.3	\$451.5	\$481.6	\$502.0
Total Revenue	\$0	\$0	\$0	\$0	\$0	\$0	\$2	\$77	\$350	\$766	\$1,146	\$1,464	\$1,683	\$1,840	\$1,969
Cost of Sales				\$0	\$0	\$0	\$0	\$11	\$59	\$137	\$193	\$250	\$291	\$319	\$342
% total sales							0%	10%	15%	13%	13%	13%	13%	13%	13%
% total sales							3%	14%	17%	18%	17%	17%	17%	17%	17%
R&D	\$5	\$13	\$28	\$35	\$45	\$52	\$54	\$57	\$60	\$63	\$66	\$67	\$69	\$70	\$72
YoY growth		165%	120%	26%	30%	15%	5%	5%	5%	5%	5%	2%	2%	2%	2%
% of revenue							2556%	74%	17%	8%	6%	5%	4%	4%	4%
SG&A	\$2	\$3	\$4	\$9	\$14	\$28	\$55	\$76	\$93	\$109	\$126	\$129	\$132	\$135	\$139
YoY growth		40%	58%	123%	50%	102%	100%	39%	21%	18%	15%	2%	2%	2%	2%
% of revenue							2587%	99%	26%	14%	11%	9%	8%	7%	7%
Total Operating Expenses	\$6.6	\$15.1	\$32	\$44	\$59	\$79	\$110	\$144	\$212	\$310	\$385	\$446	\$492	\$525	\$552
Operating Income (Loss)	-\$7	-\$15	-\$32	-\$44	-\$59	-\$79	-\$107	-\$67	\$139	\$457	\$761	\$1,018	\$1,192	\$1,315	\$1,417
Operating Margin							-5046%	-87%	40%	60%	66%	70%	71%	71%	72%
Interest income	\$0.0	\$0.0	\$0.2	\$0.2	\$0.2	\$0.2	\$0.3	\$0.3	\$0.4	\$0.9	\$1.9	\$3.3	\$5.1	\$7.1	\$9.4
Interest expense and other	-\$0.3	-\$0.3	-\$2.9	-\$3	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Pretax Income (Loss)	(\$7)	(\$15)	(\$34)	(\$47)	(\$58)	(\$79)	(\$107)	(\$67)	\$139	\$458	\$763	\$1,022	\$1,197	\$1,322	\$1,426
Provision for Income Taxes	\$0.00	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$31	\$206	\$276	\$323	\$357	\$385
Effective Tax Rate			0%	0%	0%	0%	0.0%	0%	0%	7%	27%	27%	27%	27%	27%
Net Income (Loss)	(\$6.8)	(\$15.4)	(\$34.4)	(\$46.9)	(\$58.5)	(\$79.2)	(\$107.1)	(\$67.1)	\$138.9	\$427.1	\$556.9	\$745.9	\$873.7	\$965.1	\$1,041.3
EPS, basic	(\$1.35)	(\$3.58)	(\$10.17)	(\$1.67)	(\$1.93)	(\$2.54)	(\$3.19)	(\$1.90)	\$3.93	\$12.04	\$15.65	\$20.89	\$24.37	\$26.83	\$28.84
EPS, diluted	(\$1.35)	(\$3.58)	(\$10.17)	(\$1.67)	(\$1.93)	(\$2.54)	(\$3.19)	(\$1.90)	\$3.89	\$11.92	\$15.48	\$20.66	\$24.12	\$26.55	\$28.54
Options Expense		\$0.9	\$0.7	\$4.4	\$6	\$8	\$9	\$10	\$11	\$12	\$13	\$14	\$15	\$16	\$17
% of operating expense		5.9%	2.1%	10.0%	10.2%	10.1%	8.2%	6.9%	5.2%	3.9%	3.4%	3.1%	3.1%	3.0%	3.1%
Tax Benefit from Options	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$1	\$4	\$4	\$4	\$4	\$5
GAAP Net Income (incl. options expense)	(\$6.8)	(\$16.334)	(\$35.070)	(\$56.1)	(\$64.5)	(\$87.2)	(\$116.1)	(\$77.1)	\$127.9	\$415.9	\$547.4	\$735.7	\$862.7	\$953.4	\$1,028.9
GAAP EPS, diluted (incl. ESOs)	(\$1.35)	(\$3.78)	(\$10.37)	(\$2.00)	(\$2.13)	(\$2.79)	(\$3.46)	(\$2.19)	\$3.58	\$11.60	\$15.22	\$20.38	\$23.81	\$26.22	\$28.20
Basic Shares Outstanding	5.07	4.32	3.38	28.03	30.33	31.20	33.57	35.20	35.33	35.46	35.59	35.71	35.84	35.97	36.10
Diluted Shares Outstanding	5.07	4.32	3.38	28.03	30.33	31.20	33.57	35.20	35.71	35.84	35.97	36.10	36.23	36.36	36.49

Source: Company Data, Morgan Stanley Research

May 15, 2014

Ultragenyx Pharmaceutical Inc

Exhibit 8

Ultragenyx Balance Sheet

(\$ in millions)	2011	2012	2013	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
Assets															
Cash and cash equivalents	\$10.6	\$86.19	\$7	\$76	\$18	\$32	\$127	\$53	\$170	\$562	\$1,090	\$1,812	\$2,670	\$3,625	\$4,660
Marketable securities	\$0.0	\$0	\$46	\$46	\$46	\$46	\$46	\$46	\$46	\$46	\$46	\$46	\$46	\$46	\$46
Receivables due from related party	\$0.1	\$0	\$0	\$0	\$0	\$0	\$0	\$4	\$14	\$31	\$46	\$59	\$67	\$74	\$79
Prepaid expenses and other current assets	\$0.2	\$0	\$2	\$3	\$3	\$2	\$2	\$3	\$4	\$6	\$8	\$9	\$10	\$10	\$11
Total current assets	\$11.0	\$86	\$55	\$124	\$67	\$80	\$175	\$106	\$234	\$645	\$1,189	\$1,925	\$2,793	\$3,755	\$4,795
Property, plant and equipment, net	\$0.76	\$1.4	\$1	\$1	\$1	\$2	\$2	\$3	\$4	\$6	\$8	\$11	\$13	\$16	\$18
Restricted cash	\$0.4	\$0.5	\$0.5	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Other assets	\$0	\$0	\$3	\$1	\$1	\$2	\$2	\$3	\$4	\$6	\$8	\$9	\$10	\$10	\$11
Inventory	\$0	\$0.0	\$0	\$0	\$0	\$0	\$0	\$4	\$18	\$38	\$57	\$73	\$84	\$92	\$98
Total assets	\$12.129	\$88.3	\$59.6	\$127.0	\$70.3	\$84.2	\$179.8	\$115.8	\$260.5	\$695.7	\$1,263.4	\$2,018.9	\$2,901.2	\$3,874.1	\$4,923.1
Liabilities and stockholders' equity															
Accounts payable	\$0.3	\$1	\$1	\$2	\$3	\$4	\$5	\$7	\$11	\$15	\$19	\$22	\$25	\$26	\$28
Accrued liabilities	\$0.7	\$2	\$4	\$4	\$5	\$6	\$8	\$9	\$11	\$12	\$15	\$18	\$20	\$21	\$22
Deferred revenue - current portion	\$0.0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Total current liabilities	\$1.00	\$3.2	\$6	\$7	\$8	\$10	\$13	\$16	\$21	\$28	\$35	\$40	\$44	\$47	\$50
Convertible preferred stock warrant liability	\$0.22	\$1	\$3	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Other liabilities	\$0.27	\$0	\$0	\$0	\$0	\$1	\$1	\$1	\$1	\$2	\$2	\$3	\$3	\$3	\$3
Total liabilities	\$1.49	\$4.0	\$10	\$7	\$9	\$11	\$14	\$17	\$23	\$30	\$37	\$43	\$47	\$51	\$53
Series A redeemable convert preferred stock	\$19	\$37	\$51	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Series B redeemable convert preferred stock	\$0	\$74	\$74	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Common stock	\$0.01	\$0.01	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00
APIC	\$0	\$0	\$0	\$251	\$257	\$356	\$565	\$575	\$586	\$598	\$611	\$625	\$640	\$656	\$674
Accumulated deficit	(\$8)	(\$27)	(\$75)	(\$131)	(\$195)	(\$283)	(\$399)	(\$476)	(\$348)	\$68	\$616	\$1,351	\$2,214	\$3,167	\$4,196
Accumulated other comprehensive income	\$0.00	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Total stockholders' equity	-\$8	-\$27	-\$75	\$120	\$62	\$73	\$166	\$99	\$238	\$666	\$1,226	\$1,976	\$2,854	\$3,824	\$4,870
Total liabilities and stockholder's equity	-\$6.48	-\$23	-\$65	\$127	\$70	\$84	\$180	\$116	\$261	\$696	\$1,263	\$2,019	\$2,901	\$3,874	\$4,923

Source: Company Data, Morgan Stanley Research

Exhibit 9

Ultragenyx Cash Flow Statement

(\$ in millions)	2011	2012	2013	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
CASH FLOWS FROM OPERATING ACTIVITIES															
Net loss	(\$7)	(\$16.334)	(\$35.070)	(\$56)	(\$64)	(\$87)	(\$116)	(\$77)	\$128	\$416	\$547	\$736	\$863	\$953	\$1,029
Depreciation and amortization	\$0	\$0.3	\$0.4	\$0.4	\$0.5	\$0.5	\$0.6	\$0.7	\$0.9	\$1.2	\$1.5	\$2.0	\$2.4	\$2.9	\$3.5
Non-cash interest expense	\$0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0
Amortization of premium on investment securities	\$0	\$0	\$1.4	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Employee stock-based compensation	\$0	\$0.9	\$0.7	\$4.4	\$6.0	\$8.0	\$9.0	\$10.0	\$11.0	\$11.2	\$9.5	\$10.2	\$11.0	\$11.7	\$12.4
Revaluation of convertible preferred stock warrant liability	\$0	\$0.3	\$2.9	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0
Tax benefit from stock based compensation	\$0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.8	\$3.4	\$3.7	\$4.1	\$4.5	\$4.9
Changes in operating assets and liabilities:															
Receivable - related party	(\$0)	\$0	\$0	\$0	\$0	\$0	(\$0)	(\$4)	(\$10)	(\$17)	(\$15)	(\$13)	(\$9)	(\$6)	(\$5)
Prepaid expenses and other current assets	(\$0)	(\$0)	(\$2)	(\$1)	(\$1)	\$1	(\$0)	(\$1)	(\$1)	(\$2)	(\$2)	(\$1)	(\$1)	(\$1)	(\$1)
Other assets	(\$0)	\$0	(\$3)	\$2	(\$0)	(\$0)	(\$1)	(\$1)	(\$1)	(\$2)	(\$2)	(\$1)	(\$1)	(\$1)	(\$1)
Inventory	\$0	\$0	\$0	\$0	\$0	\$0	(\$0)	(\$4)	(\$14)	(\$21)	(\$19)	(\$16)	(\$11)	(\$8)	(\$6)
Accounts payable	\$0	\$1	\$0	\$1	\$1	\$1	\$2	\$2	\$3	\$5	\$4	\$3	\$2	\$2	\$1
Accrued expenses and other liabilities	\$0	\$2	\$2	(\$3)	\$1	\$1	\$2	\$1	\$2	\$2	\$4	\$3	\$2	\$2	\$1
Net cash used in operating activities	(\$5.6)	(\$12.5)	(\$31.200)	(\$52.9)	(\$57.4)	(\$75.4)	(\$104.5)	(\$72.3)	\$119.0	\$395.0	\$531.9	\$726.4	\$863.0	\$960.4	\$1,039.6
CASH FLOWS FROM INVESTING ACTIVITIES															
Purchases of property, plant and equipment	(\$0.55)	(\$1.1)	(\$0.4)	(\$0.4)	(\$0.6)	(\$0.8)	(\$1.1)	(\$1.4)	(\$2.1)	(\$3.1)	(\$3.9)	(\$4.5)	(\$4.9)	(\$5.2)	(\$5.5)
Increase in restricted cash	(\$0)	(\$0)	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Purchase of marketable securities	\$0	\$0	(\$64)	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Sales and maturities of marketable securities	\$0	\$0	\$17	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Net cash used in investing activities	(\$0.9)	(\$1.2)	(\$47.7)	(\$0.4)	(\$0.6)	(\$0.8)	(\$1.1)	(\$1.4)	(\$2.1)	(\$3.1)	(\$3.9)	(\$4.5)	(\$4.9)	(\$5.2)	(\$5.5)
CASH FLOWS FROM FINANCING ACTIVITIES															
Net proceeds from issuance of convertible preferred stock	\$14.9	\$89	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Net proceeds from issuance of common stock	\$0.0	\$0	\$0	\$122	\$0	\$91	\$200	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Proceeds from issuance of promissory notes	\$2.5	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Proceeds from exercise of options	\$0.0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Net cash provided by financing activities	\$17.3	\$89.2	\$0.2	\$121.7	\$0.0	\$90.8	\$199.7	\$0.1	\$0.1	\$0.1	\$0.1	\$0.1	\$0.1	\$0.1	\$0.1
Effect of exchange rate on changes in cash															
Increase in cash and cash equivalents	\$10.58	\$75.54	(\$78.76)	\$68.42	(\$57.98)	\$14.59	\$94.10	(\$73.65)	\$116.97	\$391.94	\$528.14	\$722.02	\$858.23	\$955.23	\$1,034.23
Cash and equivalents at beginning of year	\$0.1	\$10.6	\$86.190	\$7	\$76	\$18	\$32	\$127	\$53	\$170	\$562	\$1,090	\$1,812	\$2,670	\$3,625
Cash and equivalents at end of year	\$10.645	\$86.2	\$7.426	\$76	\$18	\$32	\$127	\$53	\$170	\$562	\$1,090	\$1,812	\$2,670	\$3,625	\$4,660

Source: Company Data, Morgan Stanley Research

May 15, 2014

Ultragenyx Pharmaceutical Inc



Morgan Stanley ModelWare is a proprietary analytic framework that helps clients uncover value, adjusting for distortions and ambiguities created by local accounting regulations. For example, ModelWare EPS adjusts for one-time events, capitalizes operating leases (where their use is significant), and converts inventory from LIFO costing to a FIFO basis. ModelWare also emphasizes the separation of operating performance of a company from its financing for a more complete view of how a company generates earnings.

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(as of April 30, 2014)

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Stock Rating Category	Coverage Universe		Investment Banking Clients (IBC)		
	Count	% of Total	Count	% of Total IBC	% of Rating Category
Overweight/Buy	1045	35%	355	38%	34%
Equal-weight/Hold	1301	43%	455	48%	35%
Not-Rated/Hold	110	4%	22	2%	20%
Underweight/Sell	543	18%	109	12%	20%
Total	2,999		941		

Data include common stock and ADRs currently assigned ratings. Investment Banking Clients are companies from whom Morgan Stanley received investment banking compensation in the last 12 months.

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Overweight (O). The stock's total return is expected to exceed the average total return of the analyst's industry (or industry team's) coverage universe, on a risk-adjusted basis, over the next 12-18 months.

Equal-weight (E). The stock's total return is expected to be in line with the average total return of the analyst's industry (or industry team's) coverage universe, on a risk-adjusted basis, over the next 12-18 months.

Not-Rated (NR). Currently the analyst does not have adequate conviction about the stock's total return relative to the average total return of the analyst's industry (or industry team's) coverage universe, on a risk-adjusted basis, over the next 12-18 months.

Underweight (U). The stock's total return is expected to be below the average total return of the analyst's industry (or industry team's) coverage universe, on a risk-adjusted basis, over the next 12-18 months.

Unless otherwise specified, the time frame for price targets included in Morgan Stanley Research is 12 to 18 months.

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Attractive (A): The analyst expects the performance of his or her industry coverage universe over the next 12-18 months to be attractive vs. the relevant broad market benchmark, as indicated below.

In-Line (I): The analyst expects the performance of his or her industry coverage universe over the next 12-18 months to be in line with the relevant broad market benchmark, as indicated below.

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Benchmarks for each region are as follows: North America - S&P 500; Latin America - relevant MSCI country index or MSCI Latin America Index; Europe - MSCI Europe; Japan - TOPIX; Asia - relevant MSCI country index or MSCI sub-regional index or MSCI AC Asia Pacific ex Japan Index.

Stock Price, Price Target and Rating History (See Rating Definitions)

May 15, 2014

Ultrasgenyx Pharmaceutical Inc



Source: Morgan Stanley Research Date Format : MM/DD/YY Price Target -- No Price Target Assigned (NA)
 Stock Price (Not Covered by Current Analyst) — Stock Price (Covered by Current Analyst) —
 Stock and Industry Ratings (abbreviations below) appear as ♦ Stock Rating/Industry View
 Stock Ratings: Overweight (O) Equal-weight (E) Underweight (U) Not-Rated (NR) No Rating Available (NA)
 Industry View: Attractive (A) In-line (I) Cautious (C) No Rating (NR)

Effective January 13, 2014, the stocks covered by Morgan Stanley Asia Pacific will be rated relative to the analyst's industry (or industry team's) coverage.

Effective January 13, 2014, the industry view benchmarks for Morgan Stanley Asia Pacific are as follows: relevant MSCI country index or MSCI sub-regional index or MSCI AC Asia Pacific ex Japan Index.

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May 15, 2014

Ultragenyx Pharmaceutical Inc

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* Historical prices are not split adjusted.

Industry Coverage: Biotechnology

Company (Ticker)	Rating (as of)	Price* (05/14/2014)
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David Friedman, M.D.

AMAG Pharmaceuticals, Inc. (AMAG.O)	E (11/21/2011)	\$18.01
Akebia Therapeutics Inc (AKBA.O)	O (04/14/2014)	\$22.8
Alexion Pharmaceuticals (ALXN.O)	O (09/07/2010)	\$159.72
Alnylam Pharmaceuticals (ALNY.O)	E (01/14/2014)	\$56.7
Auxilium Pharmaceuticals (AUXL.O)	U (03/06/2014)	\$19.58
Chimerix Inc (CMRX.O)	O (05/06/2013)	\$16.65
Cubist Pharmaceuticals Inc. (CBST.O)	O (11/13/2013)	\$69.33
GW Pharmaceuticals PLC (GWPH.O)	O (04/22/2014)	\$71.49
Idenix Pharmaceuticals, Inc. (IDIX.O)	E (03/18/2011)	\$5.76
Incyte Corporation (INCY.O)	U (01/23/2013)	\$55.9
InterMune (ITMN.O)	E (09/07/2010)	\$35.41
Ironwood Pharmaceuticals, Inc. (IRWD.O)	E (04/24/2013)	\$13.72
Lexicon Pharmaceuticals, Inc. (LXRX.O)	U (06/11/2013)	\$1.38
NPS Pharmaceuticals (NPSP.O)	O (10/03/2012)	\$26.53
Neurocrine Biosciences Inc (NBIX.O)	E (01/08/2014)	\$13.86
Ophthotech Corp (OPHT.O)	O- (10/21/2013)	\$31.41
Portola Pharmaceuticals Inc (PTLA.O)	O (06/17/2013)	\$22.29
Relypsa, Inc. (RLYP.O)	O (12/10/2013)	\$21.99
Synageva Biopharma Corp (GEVA.O)	O (04/20/2012)	\$86.14
Theravance Inc (THRX.O)	U (07/22/2013)	\$28.86
Ultragenyx Pharmaceutical Inc (RARE.O)	O (02/25/2014)	\$35.26
Versartis, Inc. (VSAR.O)	O (04/15/2014)	\$28.49
Vertex Pharmaceuticals (VRTX.O)	E (05/08/2012)	\$67.69
XenoPort Inc (XNPT.O)	U (06/11/2013)	\$3.54

Matthew Harrison

Amgen Inc. (AMGN.O)	O (03/26/2014)	\$112.07
Biogen Idec Inc. (BIIB.O)	O (03/26/2014)	\$296.5
Celgene Corp (CELG.O)	E (03/26/2014)	\$151.75
Gilead Sciences Inc. (GILD.O)	E (03/26/2014)	\$80.97
Pharmacyclics Inc. (PCYC.O)	E (03/26/2014)	\$103.37
Regeneron Pharmaceuticals Inc. (REGN.O)	E (03/26/2014)	\$287.38