

# Ultragenyx Pharmaceuticals

## Focus on upcoming AS update in mid-1H; Reiterate Buy

Reiterate Rating: BUY | PO: 85.00 USD | Price: 46.26 USD

### Total revenues expected to grow ~20% y/y in 2024

RARE reported 4Q results and reiterated FY24 guidance of \$500-530mn in total rev, \$375-400mn in Crysvita rev and \$75-80mn in Dojolvi rev. Recall, the company pre-announced 4Q results (see our [Jan 8 note](#)). Mgmt highlighted Crysvita and Dojolvi will continue to be the main drivers citing further penetration in current commercial markets. Given the dynamic nature in which revenues are realized from different territories, RARE expects continued quarterly variability but notes confidence in growing overall demand. We continue to be encouraged by the company's commercial performance and strong 2024 outlook given guidance to ~20% increase in revenue. We highlight our near-term focus for the company continues to be on several upcoming pipeline catalysts (AS, OI and gene therapy franchise) that if positive could offer future avenues of growth. We reiterate our Buy with \$85 PO and highlight RARE as one of our top picks of 2024.

### High focus on upcoming Angelman update in mid-1H

The company guides to providing a ph 1/2 update for GTX-102 in AS in mid-1H. The update will include safety and efficacy data for 20+ expansion cohort pts (minimum of Day 170 data). Mgmt guides to a presentation similar to their recent [Analyst Day update](#) including key AS domain evaluations with direct comparisons to NH data when available. Additionally, mgmt expects the expansion data will be sufficient to initiate an end-of-phase 2 meeting with regulators in mid-24 with hopes of initiating a ph 3 trial later in 2024. RARE continues to seek regulatory alignment on a ph 3 trial design but notes they expect to evaluate a similar patient poppy as the ph 1/2 study and enroll ~100-120 pts with a traditional randomized study design. As such, we note high focus on the update and subsequent regulatory feedback given additional delays could derail RARE's first-to-market path. We currently assign a 30% LoS to the program and model risk-adj. peak sales of \$615mn in 2033 (launch in 2026), which contributes \$15/sh to our PO.

### Multiple pipeline catalysts expected in 2024

Other key takeaways include: 1) ph 3 OI trial Cosmic and Orbit are expected complete enrollment in 1Q and 1H, respectively. Mgmt also expects to report out additional longer-term ph 2 safety and efficacy data in 2H, 2) ph 3 data for DTX401 in GSD1a will readout in 1H, 3) interim Stage 1 data for UX701 in WD is now expected in mid-24 (prev. 1H), 4) ph 3 trial evaluating DTX301 in OTC deficiency is expected to complete enrollment in 1H, and 5) RARE continues to engage with regulators to seek an accelerated review path for UX111 in MPS IIIA. In our DCF-based model, we update for 4Q results, cash and share count. We also slightly increase our 2024 Evkeeza and Mepsevii revenue estimates in-line with recent trend. We tweak opex in-line with recent trend and mgmt commentary guiding to lower spend in 2024.

Estimates (Dec) (US\$)	2022A	2023A	2024E	2025E	2026E
EPS	(10.12)	(8.25)	(8.12)	(6.82)	(2.08)
EPS Change (YoY)	-51.0%	18.5%	1.6%	16.0%	69.5%
Consensus EPS (Bloomberg)			(6.27)	(4.55)	(1.87)
DPS	0	0	0	0	0
Valuation (Dec)					
Free Cash Flow Yield*	-14.6%	-13.1%	-12.9%	-10.1%	0.4%

\* For full definitions of *IQmethod*<sup>SM</sup> measures, see page 5.

16 February 2024

#### Equity

##### Key Changes

(US\$)	Previous	Current
2024E Rev (m)	493.5	499.7
2025E Rev (m)	635.4	666.8
2026E Rev (m)	NA	1,054.6
2024E EPS	-8.95	-8.12
2025E EPS	-8.00	-6.82
2026E EPS	NA	-2.08

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#### Stock Data

Price	46.26 USD
Price Objective	85.00 USD
Date Established	8-Jan-2024
Investment Opinion	C-1-9
52-Week Range	31.52 USD - 54.98 USD
Mkt Val (mn) / Shares Out (mn)	3,402 USD / 73.5
Free Float	94.0%
Average Daily Value (mn)	37.13 USD
BofA Ticker / Exchange	RARE / NAS
Bloomberg / Reuters	RARE US / RARE.OQ
ROE (2024E)	-927.2%
Net Dbt to Eqty (Dec-2023A)	-176.4%
ESGMeter <sup>TM</sup>	Medium

ESGMeter<sup>TM</sup> is not indicative of a company's future stock price performance and is not an investment recommendation or rating. ESGMeter is independent of BofA Global Research's equity investment rating, volatility risk rating, income rating, and price objective for that company. For full details, refer to ["BofA ESGMeter Methodology"](#).

#### See page 3 for abbreviations

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# iQprofile<sup>SM</sup> Ultragenyx Pharmaceuticals

## iQmethod<sup>SM</sup> – Bus Performance\*

(US\$ Millions)	2022A	2023A	2024E	2025E	2026E
Return on Capital Employed	-44.1%	-41.1%	-41.7%	-32.4%	-6.5%
Return on Equity	-111.0%	-258.3%	-927.2%	NM	-116.0%
Operating Margin	-178.6%	-131.1%	-113.2%	-71.3%	-11.4%
Free Cash Flow	(497)	(444)	(439)	(345)	14

## iQmethod<sup>SM</sup> – Quality of Earnings\*

(US\$ Millions)	2022A	2023A	2024E	2025E	2026E
Cash Realization Ratio	NM	NM	NM	NM	NM
Asset Replacement Ratio	6.4x	2.5x	2.6x	2.8x	2.9x
Tax Rate	NM	0.3%	NM	NM	NM
Net Debt-to-Equity Ratio	-34.4%	-176.4%	-2,947.0%	NM	-427.9%
Interest Cover	-15.1x	-8.6x	-8.6x	-7.2x	-1.8x

## Income Statement Data (Dec)

(US\$ Millions)	2022A	2023A	2024E	2025E	2026E
Sales	363	434	500	667	1,055
% Change	3.4%	19.5%	15.1%	33.4%	58.2%
Gross Profit	335	389	377	513	851
% Change	-0.1%	16.1%	-3.1%	36.2%	65.8%
EBITDA	(631)	(551)	(548)	(457)	(102)
% Change	-71.2%	12.6%	0.6%	16.5%	77.7%
Net Interest & Other Income	(53)	(39)	(39)	(39)	(39)
<b>Net Income (Adjusted)</b>	<b>(708)</b>	<b>(607)</b>	<b>(605)</b>	<b>(515)</b>	<b>(159)</b>
<b>% Change</b>	<b>-55.8%</b>	<b>14.2%</b>	<b>0.2%</b>	<b>14.9%</b>	<b>69.1%</b>

## Free Cash Flow Data (Dec)

(US\$ Millions)	2022A	2023A	2024E	2025E	2026E
Net Income from Cont Operations (GAAP)	(707)	(607)	(605)	(515)	(159)
Depreciation & Amortization	18	18	18	18	18
Change in Working Capital	60	50	49	48	47
Deferred Taxation Charge	NA	NA	NA	NA	NA
Other Adjustments, Net	249	140	146	154	161
Capital Expenditure	(116)	(45)	(47)	(50)	(54)
<b>Free Cash Flow</b>	<b>-497</b>	<b>-444</b>	<b>-439</b>	<b>-345</b>	<b>14</b>
<b>% Change</b>	<b>-20.6%</b>	<b>10.6%</b>	<b>1.1%</b>	<b>21.4%</b>	<b>NM</b>
Share / Issue Repurchase	11	0	0	0	0
Cost of Dividends Paid	0	0	0	0	0
Change in Debt	0	0	0	0	0

## Balance Sheet Data (Dec)

(US\$ Millions)	2022A	2023A	2024E	2025E	2026E
Cash & Equivalents	133	219	403	760	1,256
Trade Receivables	40	42	45	47	49
Other Current Assets	711	646	483	244	225
Property, Plant & Equipment	265	292	321	353	388
Other Non-Current Assets	396	247	247	247	247
<b>Total Assets</b>	<b>1,545</b>	<b>1,446</b>	<b>1,499</b>	<b>1,651</b>	<b>2,166</b>
Short-Term Debt	12	12	12	12	12
Other Current Liabilities	249	250	252	255	257
Long-Term Debt	0	0	0	0	0
Other Non-Current Liabilities	932	1,067	1,222	1,401	1,606
<b>Total Liabilities</b>	<b>1,193</b>	<b>1,329</b>	<b>1,486</b>	<b>1,667</b>	<b>1,875</b>
<b>Total Equity</b>	<b>352</b>	<b>117</b>	<b>13</b>	<b>(16)</b>	<b>291</b>
<b>Total Equity &amp; Liabilities</b>	<b>1,545</b>	<b>1,446</b>	<b>1,499</b>	<b>1,651</b>	<b>2,166</b>

\* For full definitions of iQmethod<sup>SM</sup> measures, see page 5.

## Company Sector

Biotechnology

## Company Description

Ultragenyx is a biotechnology company focused on development and commercialization of therapeutics for rare diseases. The company licenses assets that could treat small, underserved populations and have clear biology. Its strategy rests on timely and cost-efficient commercialization. RARE's portfolio includes three commercial assets and multiple clinical drug candidates.

## Investment Rationale

We rate RARE shares Buy. Ultragenyx has three approved therapies on the market including Crysvita, Mepsevii and Dojolvi. We believe those assets will continue to provide a solid floor valuation, while the clinical-stage pipeline appears undervalued by the market. We will look for continued visibility and additional validation from its pipeline portfolio.

## Stock Data

Average Daily Volume 802,696

## Quarterly Earnings Estimates

	2023	2024
Q1	-2.33A	-2.02E
Q2	-2.25A	-1.74E
Q3	-2.23A	-1.84E
Q4	-1.52A	-2.56E

## Abbreviations

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AS: Angelman syndrome

GSD1a: glycogen storage disease 1a

LoS: likelihood-of-success

Mgmt: management

MPS IIIA: mucopolysaccharidosis type IIIA; Sanfilippo syndrome

NH: natural history

OI: osteogenesis imperfecta

Opex: operating expenses

OTC: ornithine transcarbamylase

Ph: phase

Pts: patients

WD: Wilson disease

## Price objective basis & risk

### Ultragenyx Pharmaceuticals (RARE)

Our DCF-derived PO of \$85 for RARE consists of \$9/share for Dojolvi in LC-FAOD, \$19/share for Crysvita in XLH and TIO, \$0.5/share for Mepsevii in MPS7, \$15/share for Angelman, \$42/share for gene therapy assets and the remainder for cash and RARE's pipeline. We use a 9% WACC for approved products, 11-12% for clinical-stage products, such as gene therapy programs.

Upside risks to our price objective are: 1) better than expected uptake for its approved products, 2) positive data for clinical stage assets, and 3) accelerated approval for its drug candidates.

Downside risks are: 1) low penetration into rare disease populations, 2) negative data for clinical stage assets, 3) unexpected safety risks associated with clinical stage drug candidates, 4) unexpected generic competition.

## Analyst Certification

I, Tazeen Ahmad, hereby certify that the views expressed in this research report accurately reflect my personal views about the subject securities and issuers. I also certify that no part of my compensation was, is, or will be, directly or indirectly, related to the specific recommendations or view expressed in this research report.

### US - Biotechnology Coverage Cluster

Investment rating	Company	BofA Ticker	Bloomberg symbol	Analyst
<b>BUY</b>				
	4D Molecular Therapeutics, Inc.	FDMT	FDMT US	Tazeen Ahmad
	Alnylam Pharmaceuticals	ALNY	ALNY US	Tazeen Ahmad
	Amicus Therapeutics	FOLD	FOLD US	Tazeen Ahmad
	Annexon Biosciences	ANNX	ANNX US	Tazeen Ahmad
	Apellis Pharmaceuticals	APLS	APLS US	Tazeen Ahmad
	Argenx SE	ARGX	ARGX US	Tazeen Ahmad
	Arvinas	ARVN	ARVN US	Tazeen Ahmad
	Ascendis Pharma	ASND	ASND US	Tazeen Ahmad
	Biocryst Pharmaceuticals Inc	BCRX	BCRX US	Tazeen Ahmad
	BioNTech	BNTX	BNTX US	Tazeen Ahmad
	Denali Therapeutics	DNLI	DNLI US	Tazeen Ahmad
	Inozyme Pharma, Inc.	INZY	INZY US	Tazeen Ahmad
	Merus	MRUS	MRUS US	Tazeen Ahmad
	Neurocrine Biosciences	NBIX	NBIX US	Tazeen Ahmad
	Ocular Therapeutix	OCUL	OCUL US	Tazeen Ahmad
	PepGen Inc	PEPG	PEPG US	Tazeen Ahmad
	Rhythm Pharmaceuticals	RYTM	RYTM US	Tazeen Ahmad
	Sarepta Therapeutics	SRPT	SRPT US	Tazeen Ahmad
	Ultragenyx Pharmaceuticals	RARE	RARE US	Tazeen Ahmad
<b>NEUTRAL</b>				
	Acadia Pharmaceuticals	ACAD	ACAD US	Tazeen Ahmad
	Incyte Corporation	INCY	INCY US	Tazeen Ahmad
	Prothena Corporation	PRTA	PRTA US	Tazeen Ahmad
	SAGE Therapeutics	SAGE	SAGE US	Tazeen Ahmad
<b>UNDERPERFORM</b>				
	Achilles Therapeutics	ACHL	ACHL US	Tazeen Ahmad
	Fate Therapeutics	FATE	FATE US	Tazeen Ahmad
	Fulcrum Therapeutics	FULC	FULC US	Tazeen Ahmad
	Pharvaris	PHVS	PHVS US	Tazeen Ahmad
	PTC Therapeutics	PTCT	PTCT US	Tazeen Ahmad

## iQmethod<sup>SM</sup> Measures Definitions

### Business Performance

Return On Capital Employed

Return On Equity

Operating Margin

Earnings Growth

Free Cash Flow

### Quality of Earnings

Cash Realization Ratio

Asset Replacement Ratio

Tax Rate

Net Debt-To-Equity Ratio

Interest Cover

### Valuation Toolkit

Price / Earnings Ratio

Price / Book Value

Dividend Yield

Free Cash Flow Yield

Enterprise Value / Sales

EV / EBITDA

### Numerator

$\text{NOPAT} = (\text{EBIT} + \text{Interest Income}) \times (1 - \text{Tax Rate}) + \text{Goodwill Amortization}$

Net Income

Operating Profit

Expected 5 Year CAGR From Latest Actual

Cash Flow From Operations – Total Capex

### Numerator

Cash Flow From Operations

Capex

Tax Charge

Net Debt = Total Debt – Cash & Equivalents

EBIT

### Numerator

Current Share Price

Current Share Price

Annualised Declared Cash Dividend

Cash Flow From Operations – Total Capex

$\text{EV} = \text{Current Share Price} \times \text{Current Shares} + \text{Minority Equity} + \text{Net Debt} +$

Other LT Liabilities

Enterprise Value

### Denominator

$\text{Total Assets} - \text{Current Liabilities} + \text{ST Debt} + \text{Accumulated Goodwill}$

Amortization

Shareholders' Equity

Sales

N/A

N/A

### Denominator

Net Income

Depreciation

Pre-Tax Income

Total Equity

Interest Expense

### Denominator

Diluted Earnings Per Share (Basis As Specified)

Shareholders' Equity / Current Basic Shares

Current Share Price

$\text{Market Cap} = \text{Current Share Price} \times \text{Current Basic Shares}$

Sales

Basic EBIT + Depreciation + Amortization

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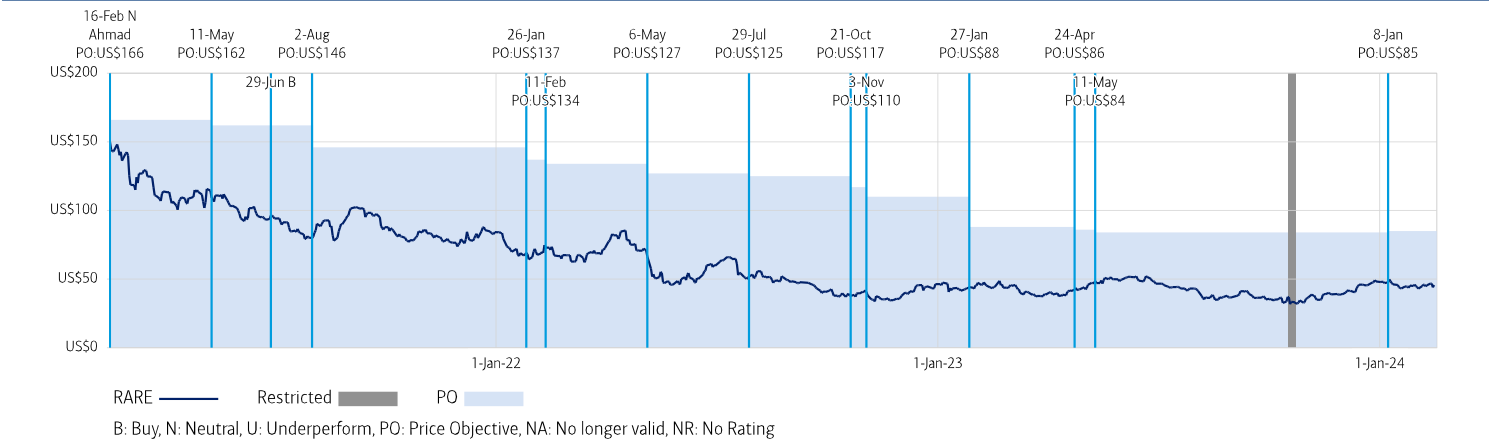
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Ultragenyx Pharm (RARE) Price Chart



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Equity Investment Rating Distribution: Health Care Group (as of 31 Dec 2023)

Coverage Universe	Count	Percent	Inv. Banking Relationships <sup>R1</sup>	Count	Percent
Buy	234	60.94%	Buy	115	49.15%
Hold	80	20.83%	Hold	36	45.00%
Sell	70	18.23%	Sell	29	41.43%

Equity Investment Rating Distribution: Global Group (as of 31 Dec 2023)

Coverage Universe	Count	Percent	Inv. Banking Relationships <sup>R1</sup>	Count	Percent
Buy	1895	53.62%	Buy	1083	57.15%
Hold	832	23.54%	Hold	454	54.57%
Sell	807	22.84%	Sell	383	47.46%

<sup>R1</sup> Issuers that were investment banking clients of BofA Securities or one of its affiliates within the past 12 months. For purposes of this Investment Rating Distribution, the coverage universe includes only stocks. A stock rated Neutral is included as a Hold, and a stock rated Underperform is included as a Sell.

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Investment rating	Total return expectation (within 12-month period of date of initial rating)	Ratings dispersion guidelines for coverage cluster <sup>R2</sup>
Buy	≥ 10%	≤ 70%
Neutral	≥ 0%	≤ 30%
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