GUGGENHEIM

October 13, 2022

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RARE

Ultragenyx Pharmaceutical Inc. Sector: Biotechnology

Rating Change

\$38.47
\$50.00
NA

BUY

Revenue (\$M)				
(FY DEC)	1Q	2Q	3Q	4Q	FY
2022	80.0	89.0	84.7E	87.0E	365.1E
EV/Sales					4.2x
2023	_	_	_	_	401.0E
Prior	_	_	_	_	410.7E
EV/Sales					3.8x
2024	_	_	_	_	482.0E
Prior	_	_	_	_	499.3E
EV/Sales					3.2x

EV/Sales					3.8x
2024	_	_	_	_	482.0E
Prior	_	_	_	_	499.3E
EV/Sales					3.2x
EPS (\$)					
(FY DEC)	1Q	2Q	3Q	4Q	FY
2022	(2.19)	(2.26)	(2.78)E	(1.74)E	(8.56)E
Prior	_	_	_	_	(8.16)E
P/E					NM
2023	_	_	_	_	(7.61)E
Prior	_	_	_	_	(6.98)E
P/E					NM
2024	_	_	_	_	(7.01)E
Prior	_	_	_	_	(6.14)E
P/E					NM

Cash (M)	\$1,100
Market Data	
52-Week Range	\$36.86 - \$89.56
Shares Out (M)	70.0
Market Cap (M)	\$2,694
ADV (3 mo; 000)	756
Enterprise Value (M)	\$1,524

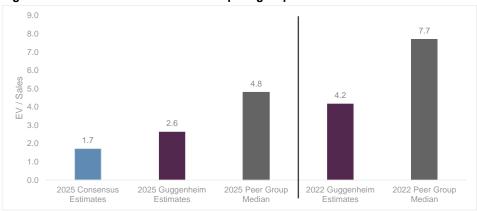
RARE: Upgrading to BUY as Near-Term Pipeline Risk Appears Adequately Discounted; PT \$50

Key Message: Ultragenyx trades at 2.6× our 2025E EV/S vs. a peer group median of 4.8×, a meaningful discount to its midcap commercial biotech peers. While sentiment on GTX-102 is at a nadir, reflected in the 78% draw down in the stock since its peak on 12/24/20 (vs. 22% decline in NBX over the same period), the program may not be a write-off should cumulative drug exposure sustain efficacy. Importantly, for every 10% increase in GTX-102 POS, we gain an incremental \$7 on our DCF analysis. With its commercial franchise and risk-adjusted pipeline providing a floor for the stock close to the current trading level, we are upgrading the shares to a BUY (from NEUTRAL) and introducing a \$50 price target.

Ultragenyx trades at a discount to commercial biotech peers

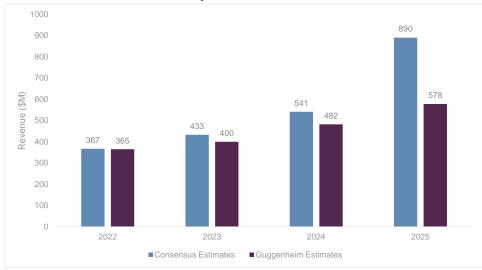
RARE trades at a discount to commercial biotech peers: (1) 4.2× 2022 EV/sales multiple vs. peer group median multiple of 7.7×; and (2) 2.6× 2025 EV/Sales at a discount to the 4.8× peer group median multiple. While a discount is somewhat warranted following the underwhelming July 15, 2022, GTX-102 update, the Angelman program could still have legs. The commercial franchise, while not poised for gangbusters growth coupled with remainder of the pipeline, offers a floor for the stock close to current level. Note, multiples are derived from Guggenheim estimates, which are discounted vs. consensus, Figures 2 and 3, and reflect realistic commercial launch timelines, Figure 5.

Figure 1 - RARE trades at a discount to peer group medians



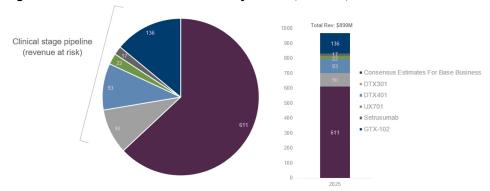
Source: Guggenheim Securities, LLC, FactSet and VisibleAlpha

Figure 2 - Our 2025 estimates are lower than consensus and reflect likely commercialization timelines for key assets



Source: Guggenheim Securities, LLC, FactSet and VisibleAlpha

Figure 3 - 2025 revenue at risk is driven by DTX301, DTX401, and GTX-102



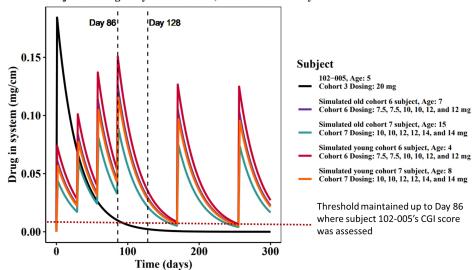
Source: Guggenheim Securities, LLC, FactSet and VisibleAlpha

Revisiting drug exposure projections—GTX-102 outlook has room for upside if the long-term exposure thesis plays out

Currently, our model leverages a 15% POS for the GTX-102. Our prior PK/PD modeling work (LINK) serves as a basis for this position. However, we recognize that our PK/PD efforts are inadequate for projecting the potential long-term therapeutic benefit that may be derived from sustained GTX-102 exposure beyond Day 128. Our GTX-102 exposures models do, however, suggest that the Cohort 6 (7.5, 7.5, 10, 10, 12 and 12 mg) and Cohort 7 (10, 10, 12, 12, 14, and 14 mg) dosing could sustain drug exposures at a level that previously resulted in strong 2-point improvement in non-subjective CGI domains (sleep, fine- and gross-motor function) for subject 102-005 (Figure 4). Taken together, this information suggests the Angelman POS will be accretive if the long-term drug exposure benefit comes to fruition.

Figure 4 - Our PK/PD modeling does not capture the benefit of long-term drug exposure and the Cohort 6 and 7 doses can sustain drug levels that have previously resulted in strong CGI signals

Projected Drug in System vs. Time, Half-Life = 20 Days



Source: Simulation conducted by Guggenheim Securities, LLC.

Valuation

We value Ultragenyx based on a blended average of risk-adjusted 2025E EV/S and risk-adjusted 10-year DCF analysis. We associate a 100% POS for approved products Crysvita, Mepsevii, and Dojolvi. We model 2022 revenues of \$365M, and 2023 revenues of ~\$400M (prior \$411M). Our 2022 EPS estimates decrease to \$(8.56) per share from \$(8.16) post GeneTx acquisition announcement. We assume an 12.0% discount rate (prior 8%), based on 5-year beta 1.50, risk-premium 5.3% (prior 4.63%), risk-free rate aligned with 10-year U.S. treasury bond rates, a terminal growth rate of 1.0%, and share count estimated from YE24. Beyond the approved medicines, we assign a: (1) 50% POS for DTX301 (prior 70%); (2) 70% POS for DTX401; (3) 60% POS for Setrusumab in pediatric OI; (4) 15% POS (prior 10%) for GTX102; (5) 10% POS for UX701; (6) 10% POS for UX053. Our valuation does not include the out-licensed gene therapy DTX201. We upgrade to BUY and introduce a \$50 price target.

Figure 5 - Probability of success, mid-decade pipeline expectations, and adjusted 2030 sales

Program	Probability of Success	Guggenheim vs Consensus 2025 Sales (\$MM)	Guggenheim Probability Adjusted 2030 Sales (\$MM)
GTX-102	15%	0 vs 136	247
Setrusumab	60%	0 vs 17	216
DTX-301	50%	0 vs 90	165
DTX-401	70%	15 vs 93	380
UX701	10%	0 vs 22	124
UX053	10%	0 vs 0	82

Source: Guggenheim Securities, LLC and VisibleAlpha

Investment Risks

RARE is susceptible to the same risks that apply to the entire biotech industry, including development, regulatory, commercial, manufacturing, financing, and IP. Risks more specific to RARE include:

Clinical risk. Downside risks: Several key pipeline products are still in clinical development and may have delays with clinical trial initiation or enrollment, unexpected safety issues, or efficacy that is lower than expectations.

Regulatory risk. Downside risks: If clinical trials are ultimately successful, it is possible that RARE's medicines will not be approved by global regulatory agencies. Given the novelty of gene therapy, regulatory agencies may require additional or longer-term data, which could delay a potential approval.

IP risk. Downside risks: RARE depends on licenses from third parties for its intellectual property. RARE's partners may not be able to defend its patents and could result in increased generic competition. The gene therapy landscape is rapidly evolving, and there is no guarantee that RARE will have access to any new developments or technologies that emerge in the space.

Competitor risk. Downside risks: It is possible that the risk/benefit of RARE's therapies may not be competitive. There are gene therapy/gene editing companies developing potential competitive medicines that could turn out to have better safety or efficacy than RARE or move faster to the market than RARE.

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GUGGENHEIM
D. Chattopadhyay
Ultragenyx (RARE) Income Statement

FY end Dec. 31	2019A	2020A	2021A	1Q22	2Q22A	3Q22E	4Q22E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E
Income statement																
\$ in thousands, except per share data)																
Product revenues	\$ 20,221	\$ 38,720	\$ 77,017	\$ 26,684	\$ 30,832	\$ 30,700	\$ 33,000	\$ 121,216	\$ 179,825	\$ 255,295	\$ 312,392	\$ 451,063	\$ 696,335	\$ 1,076,334	\$ 1,393,085	\$ 1,488,835
Crysvita in LatAm	4,286	10,350	21,422	9,394	12,402	11,000	11,000	43,796	52,696	72,898	83,267	83,826	84,413	85,004	85,599	86,198
Mepsevii	12,634	15,342	16,035	4,861	4,933	4,700	5,000	19,494	27,000	29,250	31,500	33,750	34,200	36,338	38,475	40,613
Dojolvi (UX007)	3,301	13,028	39,560	12,429	13,497	15,000	17,000	57,926	91,000	131,825	151,712	160,412	192,304	193,650	163,805	72,657
Evkeeza (Ex-US)	0	0	0	0	0	0	0	0	9,129	21,323	30,491	45,783	61,105	76,457	76,534	76,610
GTX-102 in Angelman	0	0	0	0	0	0	0	0	0	0	0	49,695	102,372	184,526	239,428	246,611
Setrusumab in OI	0	0	0	0	0	0	0	0	0	0	0	16,641	52,200	109,599	169,490	216,340
DTX-301 in OTC	0	0	0	0	0	0	0	0	0	0	0	0	25,682	73,574	122,916	164,706
DTX-401 in GSD	0	0	0	0	0	0	0	0	0	0	15,422	60,956	120,098	220,091	331,995	379,519
UX701 in Wilson's			0	0	0	0	0	0	0	0	0	0	16,712	69,892	113,760	123,964
UX053 in GSD III			0	0	0	0	0	0	0	0	0	0	7,250	27,203	51,083	81,617
Collaboration and License Revenue	83,493	219,315	270,517	53,251	58,511	54,000	54,000	219,762	184,931	171,756	186,936	204,533	205,930	211,036	203,753	262,552
Crysvita collaboration revenue in profit-share terr	74,869	128,597	171,198	45,164	51,609	51,000	51,000	198,773	167,931	171,756	184,936	199,533	200,930	202,336	203,753	205,179
Crysvita royalty revenue in European territory	8,120	1,498	14,323	4,838	5,423	3,000	3,000	16,261	17,000	0	0	0	0	0	0	57,373
Daiichi Sankyo Tech Trnsfr (\$183M)	0	89,220	84,996	3,249	1,479	0	0	4,728	0	0	0	0	0	0	0	(
Bayer DTX201 AAV.FVIII Milestones (\$232M)	504	0	0	0	0	0	0	0	0	0	2.000	5.000	5,000	8.700	0	
Bayer DTX201 AAV.FVIII Royalties		· ·	0	· ·	·	ŭ	· ·	ŭ	· ·	· ·	2,000	0,000	0,000	0,100	ŭ	·
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Non-cash Collaboration Royalty Revenue	0	12,995	3,872	0	0	0	0	24,087	35,953	55,189	78,780	79,332	79,887	80,446	81,010	24,203
Total Revenues	103,714	271,030	351,406	79,935	89,343	84,700	87,000	365,065	400,710	482,240	578,109	734,928	982,152	1,367,817	1,677,847	1,775,590
Factset consensus revenues as of 10/11/22	103,714	271,030	351,406	99,395	86,975	81,647	83,389	367,000	433,489	541,814	839,900	1,057,345	1,100,696	1,952,073	1,765,770	1,827,123
COGS as % of Product Revenue	45%	16%	15%	23%	14%	14%	15%	15%	15%	15%	15%	15%	15%	15%	15%	15%
Cost of goods sold	9,008	6,129	16,008	6,100	8,270	4,000	4,100	18,182	26,974	38,294	46,859	67,660	104,450	161,450	208,963	223,325
-																
Gross profit	94,706	264,901	335,398	73,835	81,073	80,700	82,900	346,883	373,736	443,945	531,250	667,269	877,702	1,206,367	1,468,884	1,552,265
Research and development	357,355	412,084	497,153	143,155	154,529	133,000	140,000	570,684	599,218	617,195	635,711	654,782	674,425	694,658	715,498	736,963
General and administrative	161,524	182,933	219,982	67,312	68,137	68,818	69,507	273,774	287,463	296,086	304,969	314,118	323,542	333,248	343,245	353,543
Total operating expenses	518,879	595,017	717,135	210,467	222,666	201,818	209,507	844,458	886,681	913,281	940,680	968,900	997,967	1,027,906	1,058,743	1,090,506
Operating gains (losses)	(424,173)	(330,116)	(381,737)	(136,632)	(141,593)	(121,118)	(126,607)	(497,575)	(512,945)	(469,336)	(409,429)	(301,631)	(120,265)	178,460	410,141	461,760
Interest Income	13,238	7,038	639	0	0	0	0	0	0	0	0	0	0	0	0	C
Change in fair value of equity securities	13,413	170,403	(42,063)	(9,329)	(10,184)	0	0	(19,513)	(19,513)	(19,513)	(19,513)	(19,513)	(19,513)	(19,513)	(19,513)	(19,513
Non-cash interest expense on liabilty related to sale	(1,135)	(33,291)	(29,422)	(6,584)	(6,052)	(4,000)	(4,000)	(20,636)	(28,812)	(28,812)	(28,812)	(28,812)	(28,812)	(28,812)	(28,812)	(1,401
Other income (expense)	(787)	607	(398)	783	(31)	(75,000)	(7)	(74,255)	(390)	(390)	(390)	(390)	(390)	(390)	(390)	(390
Total other income (expense)	24,729	144,757	(71,244)	(15,130)	(16,267)	(79,000)	(4,007)	(114,404)	(48,715)	(48,715)	(48,715)	(48,715)	(48,715)	(48,715)	(48,715)	(21,304
Gain (loss) before income taxes	(399,444)	(185,359)	(452,981)	(151,762)	(157,860)		(130,614)	(611,979)	(561,660)	(518,051)	(458,144)		(168,980)	,	361,426	440,456
Provision for income taxes	(3,283)	(1,207)	(1,044)	(558)	(302)	(200)	(200)	(1,260)	0	0	0	0	0	0	0	0
Net Income (loss)	(402,727)	(186,566)	(454,025)	(152,320)	(158,162)	(200,318)	(130,814)	(613,239)	(561,660)	(518,051)	(458,144)	(350,346)	(168,980)	129,745	361,426	440.456
Factset consensus Net Income as of 10/12/2022	(402,727)	(186,566)	(454,025)	(136, 141)	(122,428)		(122,458)	(606,997)	(515,308)	(422,206)	(146,200)	105,089	234,877	363,299	588,144	652,590
Earnings (loss) per share- basic	(7.12)	(3.07)	(6.70)	(2.19)	(2.26)	(2.78)	(1.74)	(8.56)	(7.61)	(7.01)	(5.60)	(4.27)	(2.06)	1.58	4.38	5.33
Factset consensus EPS as of 10/12/2022	(7.12)	(3.07)	(6.70)	(2.03)	(1.81)	* *	(1.79)	(8.50)	(7.08)	(5.86)	(1.97)	1.34	-(200)	2	7.30	0.0
Shares outstanding - basic	56,577	60,846	67,789	69,517	69,925	72,000	75,000	71,611	73,759	73,906	81,856	82,020	82,184	82,349	82,513	82,678
onares edistanting - basic	30,377	00,040	07,709	03,317	09,920	12,000	73,000	71,011	13,138	13,300	01,000	02,020	02,104	02,349	02,313	02,070
Earnings (loss) per share- diluted	(5.62)	(2.46)	(6.70)	(2.19)	(2.26)	(2.78)	(1.74)	(8.56)	(7.61)	(7.01)	(5.60)	(4.27)	(2.06)	1.58	4.38	5.33

Source: Guggenheim Securities LLC estimates, Company Reports, and FactSet.

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SELL	5	1.46%	0	0.00%

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