ULTRAGENYX – Growing once growing twice, GONE to the smart investor.

Nathan Talbot:

Nathan.talbot1@ucdconnect.ie

Patrick Connaughton

Patrick.Connaughton@ucdconnect.ie

Overall:

In our assessment Ultragenyx value is 49% above the current stock price. The Company has been public since 2011 but has only brought drugs to market in 2017 and 2018 respectfully.

Their stock has been driven down by earnings call woes and slow cash conversion rates of 600 plus days.

Value driver:

- Two on the market drugs, with realised forecasted earnings of $506 Million by 2026, currently achieving $100 million. Both drugs have 100% market dominance and are entering the rapid growth stage of their product life cycle. Due to issues getting the drugs to market and hitting earnings estimates, the stock price has been negatively impacted.
- Four pipeline drugs at various stages of testing. Using a combination of DCF modelling and probability forecasting we estimate the pipeline is valued at $1 billion. This has not been fully priced into the stock as investors are worried about the ability of this company to bring drugs to market on schedule.

Market Lag

Risk aversion: This is another factor for large undervaluation, as investors will wait till further progress is made on R&D stage drugs before purchasing them, they also use an array of option strategy's to get exposure to potential growth, which is not reflected in the stock price. Open interest on options is 4-1 in favour of call options at the strike of 35$. This can be seen to be a sum 0 but the premiums are high compared to peers indicating high levels or demand.

Market sentiment: Investors are sceptical of pharmaceuticals since the fall of Theranos. Many fear manipulation and skewed accountancy, this has driven overall interest in this industry down.

Ultragenyx has a Beneish M score of -1.44 (greater than -2.44) signalling that the company could potentially be manipulating their financial statements. This is a slight cause for concern but nothing to impacting as this metric is close to the indication region.

Recommendation:

Avail of this growth stock by longing it. However, if risk-averse, keep an on the progression of the R&D stage drugs, or engage in various options strategies depending on your risk pallet.

Ticker: RARE

NAICS: 325412

Market Cap Current: $2.12B

Implied Equity Value: $3.16B

P/E: -5.08

EPS: -7.24

Dividend Yield: 0%

Share Price: $36.78

Target Price: $54.70

Undervaluation: 49%

Date: 15th November 2019

Recommendation: BUY

Contents

Company Profile

Business Description:

Ultragenyx Pharmaceutical Inc. is an early-stage biopharmaceutical company. The Company is focused on the identification, acquisition, development, and commercialization of products for the treatment of rare and ultra-rare genetic diseases. Its clinical-stage pipeline consists of two product categories: Biologics and small-molecule substrate replacement therapies. Founded in 2010 the Company currently employs 289 people. The Company was founded by current President and Chief Executive Officer, Dr Emil Kakkis.

Portfolio:

Figure 1 - Pipeline drugs (Ultragenyx 10-K)

Crysvita:

Patients with XLH have low serum phosphate levels due to excessive phosphate loss into the urine, which is directly caused by the effect on kidney function of excess FGF23 production in bone cells. Low phosphate levels lead to poor bone mineralization and a variety of clinical manifestations, including rickets leading to bowing and other skeletal deformities, short stature, and osteomalacia, which can lead to fractures. Ultragenyx is in a 50/50 partnership with KKE on this drug.

TIO results from typically benign tumours that produce excess levels of FGF23, which can lead to severe hypophosphatemia, osteomalacia, bone fractures, fatigue, bone and muscle pain, and muscle weakness.

Mepsevii:

MPS VII is caused by a deficiency of the lysosomal enzyme beta-glucuronidase, which is required for the breakdown of certain complex carbohydrates known as glycosaminoglycans. The inability to properly break down GAGs leads to their accumulation in many tissues, resulting in a serious multi-system disease. MPS VII is one of the rarest MPS disorders.

Pipeline:

UX007 for the treatment of Long Chain Fatty-Acid Oxidation Disorders, or LC-FAOD:

Patients with LC-FAOD have a deficiency that impairs the ability to produce energy from long-chain fatty acids, which can lead to depletion of glucose in the body, and severe liver, muscle, and heart disease, as well as death.

DTX301 for the treatment of ornithine transcarbamylase, (OTC):

OTC is part of the urea cycle, an enzymatic pathway in the liver that converts excess nitrogen, in the form of ammonia, to urea for excretion. OTC deficiency is the most common urea cycle disorder and leads to increased levels of ammonia. Patients with OTC deficiency suffer from acute hyperammonaemia episodes that can lead to hospitalization, adverse

cognitive and neurological effects, and death. We estimate that there are approximately 10,000 patients in the developed world with OTC deficiency.

DTX401 for the treatment of glycogen storage disease type Ia (GSDIa):

AAV8 gene therapy program for the treatment of patients with GSDIa. GSDIa is the most common genetically inherited glycogen storage disease. It is caused by a defective gene for the enzyme G6Pase-α, resulting in the inability to regulate blood sugar (glucose). Hypoglycemia in patients with GSDIa can be life-threatening, and the accumulation of the complex sugar glycogen in certain organs and tissues can impair the ability of these tissues to function normally.

DTX201 for the treatment of Hemophilia A:

DTX201 is a Factor VIII gene therapy program for the treatment of haemophilia A being developed in collaboration with Bayer Healthcare LLC. Hemophilia A is the most common form of haemophilia with approximately 144,000 patients in the developed world.

All these pipeline drugs have been detailed in the valuation section.

Risks:

- Company posted wider than expected losses over the last two quarters. Separately, the stock has also seen some volatility since phase 3 trials of the company's UX007 drug for patients with glucose transporter type-1 deficiency syndrome failed late last year. Worth noting that with orphan drug companies, that is common that if a drug isn't successful in trials for example DX007, it might immediately begin testing for a different disease. Other examples can be seen with Alexion – ALXN1840 was being tested to treat diseases ranging from Wilsons disease (Metabolic disorder) to WA/HA (Hemophilia)[1].
- The gene therapies for Ultragenyx are only in both phase 1 and 2, which would be the major risk factors in our evaluation. We have adjusted for their probabilities of success in our evaluation, yet with all probability testing and forecasting predicting the future is often more difficult then we can account for.
- Hemophilia A gene therapy market is highly competitive with competition from BioMarin, Spark and Pfizer. Many Biotech companies recognise the financial possibilities in this field which make it one of the most potentially lucrative markets in Biotech[2].

[1] Alexion 2019 10-k

[2] https://www.medpagetoday.com/recent-developments/hemophilia/78500

Revenue Streams

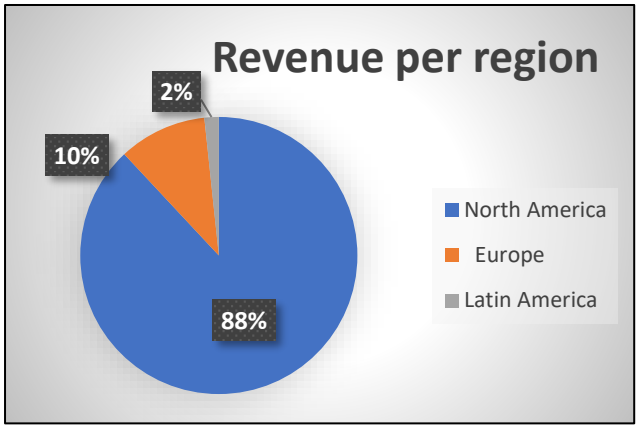


Figure 2 – Revenue by location – Author

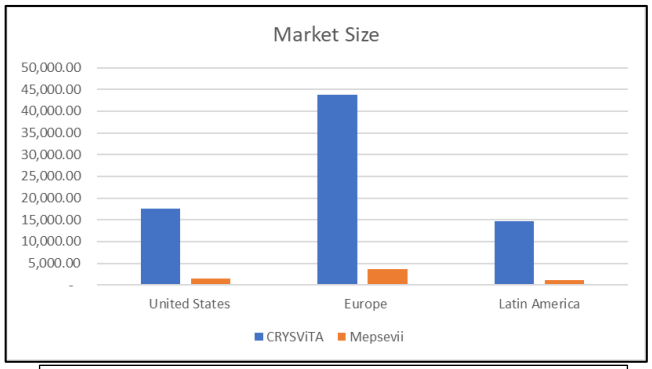


Figure 3 – the Market size of on-market drugs

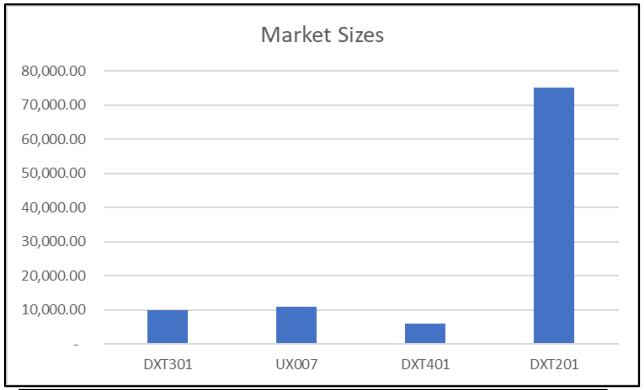


Figure 4 – the Market size of pipeline drugs

On the market drugs:

As can be seen in fig 2, Ultragenyx generates 88% of its revenue from North America. This showcases the opportunities for growth in Europe and Latin America, as shown in fig 3, the largest markets for the two respective drugs in Europe. Currently, only 10% of revenue is generated in this region. Ultragenyx aims to explore this market extensively in the coming years. We have included an initial saturation of the European market in our estimations of sales but have estimated growth mainly based of foundation growth in North America than a combined market growth and exploration growth in the EU and Latin America to come.

We expect Ultragenyx to experience no issues in their penetration into the EU or Latin America markets, due to competent marketing, shown by their ability to get 617 individual physicians to prescribe Crysvita and onboard roughly 1400 patients within two years. They are also currently selling into Colombia, Argentina and are negotiations with the Brazilian health minister over a trade deal.

The market sizes for the on the market drugs are extremely promising, with Crysvita the larger of the two. This drug is forecasted to be a blockbuster, and we estimate the total revenue for this drug is roughly $600 Million by 2026, with Ultragenyx realizing 50% of this revenue.

Internal Pipeline:

As shown in figure 4 and explained in the Business description, the pipeline of this Company is promising; they have 4 drugs at various stages of clinical trials, all with larger market sizes that Mepsevii. We have valued each drug based on various factors outlined in the valuation section of this report.

External Pipeline:

Ultragenyx has engaged in a private OTC option with Gene TX, which required them to foot up $20 Million in the capital, allowing them the option to further invest 25$ million and become the sole partner for a gene therapy for Angelman disease. This market is 22,000 people in the developed world. The average gene therapy price is in excess of $1million. We see this as great potential for the Company. But unfortunately, due to the privacy of the deal, it is not possible to value this play and include it in our valuation.

Statement Analysis:

Altman Z-Score			
PARAMETERS			
Income statement			
Net sales			51
Operating income		-	371
Balance sheet			
Current assets			522
Total assets			720
Current liabilities			75
Total liabilities			111
Retained earnings		-	1,030
Public companies			
Market value of equity			2,461
Private companies			
Book value of equity			
CALCUATIONS			Z
		Factor	Public Mfg
Working capital/Total assets	X1	0.62	1.2
Retained earning /Total assets	X2	-1.43	1.4
EBIT/Total assets	X3	-0.52	3.3
Market value of equity/Total liabilities	X4	22.24	0.6
Book value of equity/Total liabilities	X4A	0.00	
Net sales/Total assets	X5	0.07	1.0
		Z-Score	**10.45**
LEGEND			
Financially sound if greater than			2.99
Caution required if between			2.77 - 2.99
Likely to go bankrupt within 2 years if between			1.8 - 2.7
Likelihood of bankruptcy is high if below			1.88
Average for nonbankrupt companies			5.02
Average for bankrupt companies			-0.29

Figure 5 – Altman Z score – Author

Firstly, to analyze if this Company is in a sound financial state, we conducted a Z score analysis. This analysis signified that this Company is financially stable, with a Z score of 10.45 well over the 2.99 thresholds of financial stability.

We decided to look at some key metrics for evaluating early-stage companies:

Cash Conversion Ration:

The cash conversion cycle (CCC) is a metric that expresses the time (measured in days) it takes for a company to convert its investments in inventory and other resources into cash flows from sales.

Company	Cash Conversion Cycle
Abbvie	148.72
Alexion	594
Amgen	236.49
Bayer	176.21
Eli Lilly and Co	203.69
Ulltrageyx	610.00

Figure 6 – Altman Z score – Author

Ultragenyx is in early-stage product release with two drugs fresh onto the market, as discussed later in the analysis, they run a reimbursement programme that generates a high CCC compared to industry peers. This leaves them with lower realized sales than they should have with the level of patients currently using their drugs.

Capital structure:

Ultragenyx has no debt on its balance sheet, funded purely through equity investment. This is a positive sign as it leaves the Company with room to increase their leverage for further growth and expansion. They have a cash reserve of $122 Million and a current ratio of 6.99, showing the company's strong ability to meet its short-term liabilities.

We can conclude this Company is of sound financial state and provides a foundation for growth both organically and through leverage.

Business Life Cycle:

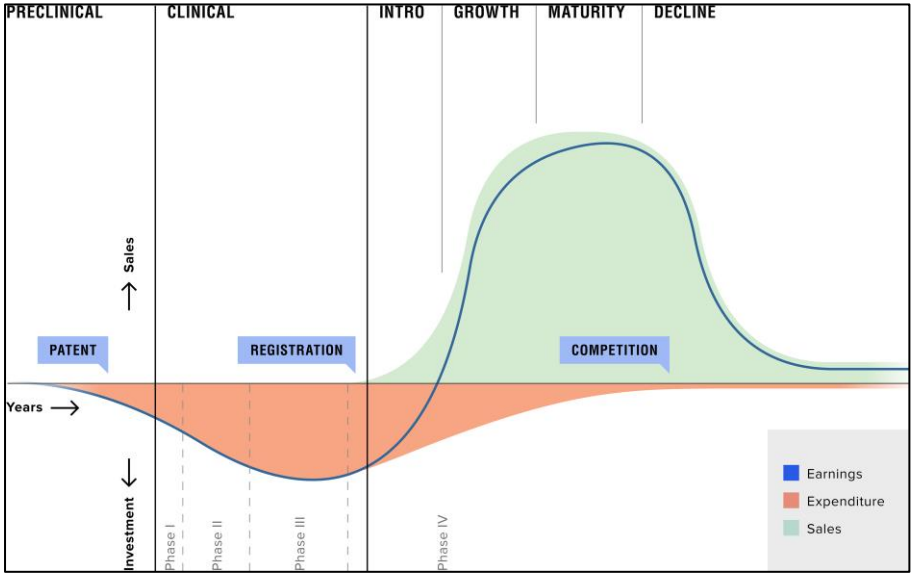


Figure 7 – Pharma business cycle [3] –

As can be seen in figure 7, pharma companies follow a business cycle of loss and rapid growth followed by maturity if failure to produce new drugs.

Ultragenyx currently is in the intro stage with 2 drugs fresh onto the market; they are forecasted to experience rapid growth. This growth is proven, and the forecast is in our sales forecast section.

We believe with an array of drugs in the pipeline Ultragenyx will be successful prior to their rapid growth stage and maintain stable growth through the steady introduction of gene therapies in their pipeline.

[3] https://journals.plos.org/plosone/article?id=10.1371/journal.pone.0182613

Forecasts

Sales:

We forecasted sales using an array of factors listed below:

Marketed Drug Revenue forecast	
Drug	Forecasting metrics
Crysvita	Baby Births Per Year(1) Inflation adjusted for presidential election(2) Population Growth(3)
Mepsevi	Baby Births Per Year(1) Inflation adjusted for presidential election(2)

Figure 8 – Sales forecast metric – Author

1) We took data from the world bank on the fertility rates in the territory regions for Ultragenyx and the corresponding population for women for reproduction ages.
   We then combined the diagnosis rate with the baby birth rate created to forecast a figure for the patients being diagnosed with the associated disease. This was taken as the patient or market growth rate per year.
2) We used the probabilities associated with the US presidential election and the impact it will have on the inflation rate of prices for Ultragenyx drugs into the future. Shown in figure 10, the inflation rate we arrived at was 4.7%.
3) Using the same method as (1) but changing from baby growth rates, to the population growth rate for the respective territories.
   For Crysvita, the market makeup is as shown in figure 9, as can be seen, the market is broken into 45% adult and 55% paediatric. Therefore, we had to introduce a separate growth rate for the adult proportion of sales, this growth rate comprised of the increase in the population aged 15-65 in the territories of Ultragenyx operations, combined with the diagnosis rate for XHL.

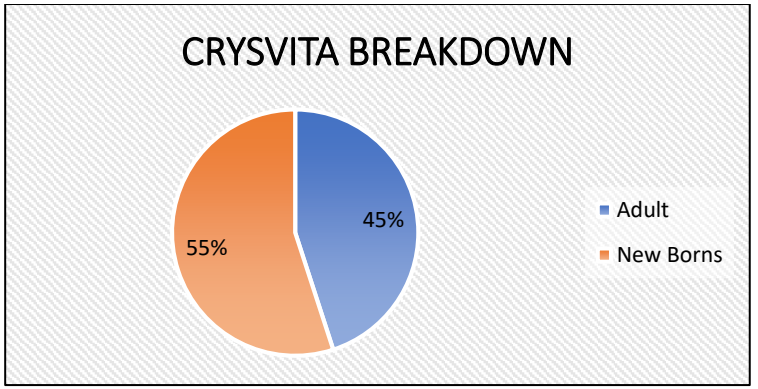


Figure 9 – Crysvita market segments– Author

US 2020 Election Effect on Prices			
Indursty Price Inflation	5%		
	Democrat	Republican	Independent
Bookmaker odds	8/3	6/5	60/1
Implied Probabilty	56.79%	41.70%	1.50%
Affect	-2%	2%	0%
Price Inflation	4.7%		

Figure 10 – price inflation-adjusted for the presidential election– Author

Ultragenyx sales forecasts:

	2017	2018	2019	2020	2021	2022	2023	2024	2025	2026
Crystvita	**0.01**	**18.87**	**90.62**	**118.90**	**149.45**	**182.64**	**218.56**	**257.37**	**299.31**	**344.60**
Pedeatrics (55%)	0.00	10.38	49.84	65.39	82.20	100.45	120.21	141.56	164.62	189.53
Adults (45%)	0.00	8.49	40.78	53.50	67.25	82.19	98.35	115.82	134.69	155.07
Reimbursement adjustment				(11.89)	(14.94)	(18.26)	(21.86)	(25.74)	(29.93)	(34.46)
Reimburesemnt recoup					2.38	2.99	7.31	8.74	10.29	11.97
Realised revenue	**0.01**	**18.87**	**90.62**	**107.01**	**136.88**	**167.36**	**204.01**	**240.38**	**279.68**	**322.11**
Mepsevii	**0.48**	**7.90**	**15.82**	**36.74**	**59.39**	**83.89**	**110.36**	**138.92**	**169.69**	**202.81**
Reimbursement adjustment				(10.70)	(13.69)	(16.74)	(20.40)	(24.04)	(27.97)	(32.21)
Reimburesemnt recoup					2.14	2.74	6.69	8.16	9.62	11.19
Realised reevenue	**0.48**	**7.90**	**15.82**	**26.04**	**47.84**	**69.89**	**96.65**	**123.04**	**151.34**	**181.79**
Collaberations (excluding CRYSTVITA)	**2.13**	**23.47**	**2.13**	**2.23**	**2.33**	**2.44**	**2.55**	**2.67**	**2.80**	**2.93**
Total	**2.61**	**50.24**	**108.57**	**135.27**	**187.06**	**239.69**	**303.22**	**366.09**	**433.82**	**506.83**
Growth		1823.43%	116.11%	24.59%	38.28%	28.14%	26.50%	20.74%	18.50%	16.83%
				2020	**2021**	**2022**	**2023**	**2024**	**2025**	**2026**
Adjustment down				10%	10%	10%	10%	10%	10%	10%
Adjustment Up*					20%	20%	20%	20%	20%	20%

Figure 11 – Sales forecast breakdown– Author

- Reimbursement adjustment. Since we forecasted the sales of drugs to rise in line with the patients coming onto it, we had to adjust for the reimbursement factor. This factor includes in our estimations the fact that Ultragenyx does not realize the full benefit of sales from patients coming onto the drugs immediately. Therefore, we had to discount sales down due to the factor that partial sales will be not realized in the year of administration. Then allow for recouping of this reimbursement factors in the following years. The reimbursement factor is shown in figure 11 above:
- Collaborations include royalties' receivables and payable as a net, this figure grows with inflation of drug prices, sales growth point 2. These collaborations include royalties' receivables and payable to many different organizations (for on the market and off the market), the majority being negligible for the immediate future. Any substantial royalties have been included in the valuation of pipeline drugs through additional WACC, as explained in that section.

*adjustment up is X% per year of the adjustment down of the previous year. This is to account for the slow re-imbursement from governments and insurance companies.

Valuation:

We split the Company into two valuations, On the market drugs and the Pipeline drugs. Then combined in order to get the full valuation of the Company.

Steps to value:

1) Sales forecast as shown previously
2) Selecting Proxy company as shown previously
3) WACC calculation
4) DCF of the on the market drugs
5) DCF of the Pipeline of Ultragenyx.
6) Combining the two

On the market valuation:

Model Type: DCF by Proxy

The model we used is a DCF Proxy model, where we use a company that has followed the projected path for Ultragenyx. Keep in mind we are comparing the position of both companies two years after first drug launch.

We picked a company that had a similar structure to Ultragenyx at this stage of its life cycle. We then worked out the evolution of this selected companies Free cash flow ratio from revenue and used it as a proxy in our DCF model.

This path includes the reliance on a limited number of drugs to generate sales. We analysed many companies and chose Alexion for several reasons:

Market position: Both Alexion and Ultragenyx are in the orphan drugs business and have 100% dominance over the selected industry they operate in. Competition for both companies is/was minimal at this stage of the business cycle.

Cash: At this stage of the life cycle, both companies had roughly $100-200 million in cash reserves on their balance sheet.

Portfolio of drugs: Both companies are reliant on the success of one drug but have complete market dominance in the respective fields. As Alexion did, Ultragenyx is gearing up to produce additional drugs through in house and external R&D.

Growth: At this stage, Alexion started to experience rapid growth with the drug Solaris, Ultragenyx is also experiencing this rapid growth, with a CAGR of 66% from 2017. The reason why the revenue stream is not growing into the billions rapidly is due to Ultragenyx sharing the revenue 50/50 with their partner KKE.

Alexion ticked all the boxes, and we chose it as our proxy company, as we believe Ultragenyx will follow a similar path post-marketing of its two drugs outlined previously.

	Current	Forecasted						
Years Since Launch	+2	+3	+4	+5	+6	+7	+8	+9
Alexion Revenue ($M)	386.8	541.0	783.4	1,134.1	1,551.3	2,234.0	2,604.0	3,084.1
Growth		39.85%	44.82%	44.76%	36.79%	44.00%	16.56%	18.44%
ULTRAGENYX Revenue ($M)	108.572	135.2722	187.0566	239.6936	303.2188	366.093	433.8177	506.8261
Growth		24.59%	38.28%	28.14%	26.50%	20.74%	18.50%	16.83%
Cash								
Alexion	176.22							
ULTRAGENYX	122.577							
Drugs on market								
Alexion	1							
ULTRAGENYX	2							
Debt								
Alexion	10.421							
Ultragenyx	0							

Figure 12 – Proxy comparison – Author

Internal Pipeline:

Ultragenyx has 4 new drugs in the pipeline. We valued each of these clinical-stage drugs using a DCF model driven by several assumptions. All assumptions are outlined in the respective sections of this report.

Weighted Average Cost Of Capital (WACC)

WACC Calculation	
Shares outstanding (1)	58
Share Price (2)	37
Market Cap	2,124
Debt (3)	-
Tax Rate (4)	20%
Cost of debt (5)	0.0%
Expected Market Return (6)	7.4%
Risk Free Rate (7)	1.56%
Levered Beta (8)	
Unlevered Beta (9)	1.38
WACC based on CAPM	
Cost of equity (10)	9.6%
WACC	9.6%

Figure 13 –WACC calculation – Author

Tax Rate: Is based on a Weighted average tax rate, calculated the current sales revenue from each region:

Weighted Average Tax Rate (WATR)			
Revenue by geographic location	2019	% of Revenue	Tax Rate
United States	21,700	84%	21%
Europe (Ireland)	2,790	11%	12.50%
Latin America	1,310	5%	15%
Total Revenues	25,800	1	
WATR	20%		

Figure 14 –WATR calculation – Author

Debt: Company has no debt

Risk-free rate: 3-month T bill

Expected market return: the 40-year average return of the Russell 3000 index

Beta: 1-year beta against the Russell 3000 index

Cost of equity: Calculated using CAPM

Current Marketed drugs valuation using Proxy (Alexion and market average)

	12/31/2019	2020	2021	2022	2023	2024	2025	2026	Terminal
Forecasted Revenue	43.37	135.27	187.06	239.69	303.22	366.09	433.82	506.83	
PROXY SALES TO FCF	-49.28%	2.44%	4.37%	13.06%	15.99%	24.07%	16.59%	30.00%	
FCF	- 21.37	3.30	8.18	31.29	48.49	88.13	71.97	152.05	3,459.46
Discount Factor	0.25	1.25	2.25	3.25	4.25	5.25	6.25	7.25	7.25
Present Value of cashflows	- 20.89	2.94	6.65	23.22	32.83	54.42	40.55	78.15	1,778.09
Enterprise Value	1,995.97								
Add back initial Cash	122.58								
Asset Value	2,118.55								

WACC	9.61%
Growth	5%

Figure 15 – DCF on market drugs with proxy Alexion– Author

Proxy: As mentioned previously we used the FCF from sales generated from Alexion at the same stage of the business cycle and followed its evolution for 6 years, then we switched out to the industry average, as we didn't want to assume that Ultragenyx would behave like Alexion terminally. Therefore, we used the industry average FCF to the sales rate of 30% to represent the convergence to industry standard.

Growth: This reflects our beliefs along with the Company's belief for the two drugs on the market to continue growing deep into the future. With a strong base of patients and a steady influx of patients coming onto the drug, the growth rate will be roughly 5% for the life of the product, this 5% also represents the belief that the Company will continue to bring successful drugs onto the market, and grow at a rate equivalent to industry inflation.

	WACC				
Long Term Growth	2,118.55	7.61%	8.61%	9.61%	10.61%	11.61%
	3%	2085	1774	1557	1398	1275
	4%	2589	2102	1788	1569	1408
	5%	3479	2610	2119	1802	1581
	6%	5470	3508	2632	2135	1816
	7%	13940	5519	3538	2654	2152

Figure 16 – sensitivity analysis of DCF on market drugs with proxy Alexion– Author

The above figure 16 is a sensitivity analysis to show how changes in the WACC and Long-term growth affect the valuation of the on the market drugs section the Company.

DXT301:

WACC	9.61%		Drug Value (USD)	377,939,317.22							
Premium for risk of project	2%		Stage 2 Probability	50%							
Total WACC	11.61%		Expected probability	188,969,658.61							
	2022	2023	2024	2025	2026	2027	2028	2029	2030	2031	2032
Market size (Per person)	10,000	10,027	10,052	10,077	9,998	9,823	9,563	9,229	8,829	8,374	7,883
Market growth (Per person)	427	427	427	425	425	428	431	430	428	430	434
Market decrease (per person)	400	401	402	504	600	688	765	831	883	921	946
Price in USD	400,000	400,000	400,000	400,000	400,000	400,000	400,000	400,000	400,000	400,000	400,000
Patients treated (per person)	400	401	402	504	600	688	765	831	883	921	946
Penetration rate %	4.00%	4.00%	4.00%	5%	6%	7%	8%	9%	10%	11%	12%
	2022	2023	2024	2025	2026	2027	2028	2029	2030	2031	2032
Revenue USD	160,000,000	160,424,412	160,832,873	201,534,277	239,948,245	275,034,210	306,019,606	332,261,837	353,146,441	368,441,482	378,385,413
FCF/Sales Rate %	30%	30%	30%	30%	30%	30%	30%	30%	30%	30%	30%
Free Cash Flow USD	48,000,000	48,127,324	48,249,862	60,460,283	71,984,474	82,510,263	91,805,882	99,678,551	105,943,932	110,532,445	113,515,624
Discount Factor years	2.25	3.25	4.25	5.25	6.25	7.25	8.25	9.25	10.25	11.25	12.25
Discounted FCF USD	37,485,769.62	33,674,004.88	30,246,630.25	33,956,972.44	36,222,255.01	37,198,248.10	37,081,977.46	36,072,144.36	34,349,798.67	32,108,184.31	29,543,332.11

Figure 17 – DCF of DXT301 – Author

- ***Price*** – For the price of DXT301, we researched into the prices of gene therapies across the industry and aligned with the gene therapy of rare diseases with similar population sizes and diseases[4].
- ***Market size*** – We researched investor slides and US National Library of Medicines[5].Market growth is based on population growth, as discussed in point 3 of the sales forecast. The decrease in market size is due to the nature of gene therapy, the drug is a one and done treatment (at least for 10-years), therefore patients who get treated are no longer in market population.
- ***Penetration*** – The Company expects to have a low penetration rate but to grow over time in line with current products
- ***Valuation Length*** – We have chosen a 10-year lifespan for the drugs, due to patent laws in the industry.
- ***Free cash flows generated from sales*** – We chose the industry average, this is market consensus, as we do not know the exact COGS, SG&A and R&D costs associated with this drug post-launch.
- ***WACC***: The discount rate for this project is adjusted versus the overall company discount rate to account for increased risk and costs in clinical trial stage drugs and the royalties owed to certain firms.
- ***Expected probabilities***: We utilized the expected probability of drugs in this field of research for drugs in stage 2 to make it to market. We used the probabilities to estimate an expected value adjusted for the probability of successful trials [6]
- ***Limitations*** –: The drug will be on the market post valuation period, but due to uncertainty post-patent expiration (price and market penetration), we cannot forecast any further with accuracy. Therefore, we can assume that the drug value is greater than $188 million.

[4] https://www.technologyreview.com/s/609197/tracking-the-cost-of-gene-therapy/)
[5] https://ghr.nlm.nih.gov/condition/ornithine-transcarbamylase-deficiency
[6] https://www.bio.org/sites/default/files/Clinical%20Development%20Success%20Rates%202006-2015%20-%20BIO,%20Biomedtracker,%20Amplion%202016.pdf

UX007

WACC	9.61%		Drug Value (USD)	307,171,105.55							
Premium for risk of project	2%		Stage 3 Probability	65%							
Total WACC	11.61%		Expected probability	199,661,218.60							
	2020	2021	2022	2023	2024	2025	2026	2027	2028	2029	2030
Market size (Per person)	11,000	11,427	11,853	12,280	12,705	13,129	13,558	13,989	14,419	14,847	15,277
Market growth (Per person)	427	427	427	425	425	428	431	430	428	430	434
Price in USD	300,000	300,000	300,000	300,000	300,000	300,000	300,000	300,000	300,000	300,000	300,000
Patients treated (per person)	110	143	178	215	254	525	813	1,119	1,442	1,782	2,139
Penetration rate %	1.00%	1.25%	1.50%	2%	2%	4%	6%	8%	10%	12%	14%
	2020	2021	2022	2023	2024	2025	2026	2027	2028	2029	2030
Revenue USD	33,000,000	42,849,472	53,339,020	64,469,249	76,228,935	157,553,999	244,035,859	335,735,509	432,563,990	534,480,293	641,639,792
FCF/Sales Rate %	30%	30%	30%	30%	30%	30%	30%	30%	30%	30%	30%
Free Cash Flow USD	9,900,000	12,854,841	16,001,706	19,340,775	22,868,680	47,266,200	73,210,758	100,720,653	129,769,197	160,344,088	192,491,938
Discount Factor years	1.25	2.25	3.25	4.25	5.25	6.25	7.25	8.25	9.25	10.25	11.25
Discounted FCF USD	8,629,438.24	10,039,033.89	11,196,166.46	12,124,247.45	12,843,988.03	23,784,133.58	33,005,735.65	40,682,807.02	46,961,489.25	51,987,754.46	55,916,311.52

Figure 18 – DCF of UX007 – Author

- ***Price*** – For the price, we went based on previously released drugs for autosomal recessive genetic disorders such as Cystic Fibrosis. There are currently four drugs of the market for long-chain fatty acids oxidation disorders (LC-FAOD). We used the most recently released cystic fibrosis drug 'Trikafta' by Vertex pharmaceuticals as my basis for my evaluation, whose price aligned with previously released CF drugs[7].
- ***Market size*** - The market size for patients with LC-FOAD currently is 11,000 (An average of the 8000-14000 patients on the investors slides (Source – Ultragenyx investor slides). Market growth is based on population growth, as discussed in point 3 of the sales forecast. This is not a one and done drug. Therefore patients who join are patients for life. The penetration rate is extremely low due to the drug being a biological rather than a gene therapy. These types of drugs take longer to gain traction.
- ***Penetration*** – the Company expects to have a low penetration rate but to grow over time in line with current products
- ***Valuation length*** – We have chosen a 10-year lifespan for the drugs, due to patent laws in the industry.
- ***Free cash flows generated from sales*** – We chose the industry average, this is market consensus, as we do not know the exact COGS, SG&A and R&D costs associated with this drug post-launch.
- ***WACC:*** The discount rate for this project is adjusted versus the overall company discount rate to account for increased risk and costs in clinical trial stage drugs.
- ***Expected probabilities:*** We utilized the expected probability of drugs in this field of research for drugs in stage 3 to make it to market. We used the probabilities to estimate an expected value adjusted for the probability of successful trials[8].
- ***Limitations*** –: The drug will be on the market post valuation period, but due to uncertainty post-patent expiration (price and market penetration), we cannot forecast any further with accuracy. Therefore, we can assume that the drug value is greater than $199 million.

[7] https://www.ajmc.com/newsroom/price-tag-for-newly-approved-crysvita-is-responsible-says-pbm-express-scripts

[8] https://www.bio.org/sites/default/files/Clinical%20Development%20Success%20Rates%202006-2015%20-%20BIO,%20Biomedtracker,%20Amplion%202016.pdf

DXT401

WACC	9.61%		Drug Value (USD)	314,605,100.03								
			Stage 2 Probability of									
Premium for risk of project	2%		success	50%								
Total WACC	11.61%		Expected probability	157,302,550.02								
	2022	2023	2024	2025	2026	2027	2028	2029	2030	2031	2032	
Market size (Per person)	6,000	5,410	4,825	4,256	3,712	3,202	2,731	2,304	1,921	1,585	1,293	
Market growth (Per person)	10	10	10	10	10	10	9	9	9	9	9	
Market decrease (per person)	600	595	579	553	520	480	437	392	346	301	259	
Price in USD	400,000	400,000	400,000	400,000	400,000	400,000	400,000	400,000	400,000	400,000	400,000	
Patients treated (per person)	600	595	579	553	520	480	437	392	346	301	259	
Penetration rate %	10.00%	11.00%	12.00%	13%	14%	15%	16%	17%	18%	19%	20%	
	2022	2023	2024	2025	2026	2027	2028	2029	2030	2031	2032	
Revenue USD	240,000,000	238,037,188	231,585,413	221,286,104	207,870,479	192,113,627	174,791,306	156,641,346	138,330,297	120,431,486	103,410,473	
FCF/Sales Rate %	30%	30%	30%	30%	30%	30%	30%	30%	30%	30%	30%	
Free Cash Flow USD	72,000,000	71,411,156	69,475,624	66,385,831	62,361,144	57,634,088	52,437,392	46,992,404	41,499,089	36,129,446	31,023,142	
Discount Factor years	**2.25**	**3.25**	**4.25**	**5.25**	**6.25**	**7.25**	**8.25**	**9.25**	**10.25**	**11.25**	**12.25**	
Discounted FCF USD	**56,228,654.42**	**49,965,371.87**	**43,552,528.95**	**37,285,003.18**	**31,379,839.76**	**25,983,278.11**	**21,180,366.10**	**17,005,832.76**	**13,455,092.00**	**10,495,116.70**	**8,074,016.20**	

Figure 19 – DCF of UX007 – Author

- ***Price*** – For the price of DXT401, we researched into the prices of gene therapies across the industry and aligned with the gene therapy of rare diseases with similar population sizes and diseases[9]
- ***Market size*** – We researched investor slides and US National Library of Medicines[10]. Market growth is based on population growth, as discussed in point 3 of the sales forecast. The decrease in market size is due to the nature of gene therapy, the drug is a one and done treatment (at least for 10 years), therefore patients who get treated are no longer in market population.
- ***Valuation Length*** **-** We have chosen a 10-year lifespan for the drugs, due to patent laws in the industry.
- ***Penetration*** – the Company expects to have a higher penetration rate due to the lower market size and interest from physicians on the market to prescribe.
- ***Free cash flows generated from sales*** – We chose the industry average, this is market consensus, as we do not know the exact COGS, SG&A and R&D costs associated with this drug post-launch.
- ***WACC:*** The discount rate for this project is adjusted versus the overall company discount rate to account for increased risk and costs in clinical trial stage drugs.
- ***Expected probabilities***: We utilized the expected probability of drugs in this field of research for drugs in stage 2 to make it to market. We used the probabilities to estimate an expected value adjusted for the probability of successful trials.[11]
- ***Limitations*** –: The drug will be on the market post valuation period, but due to uncertainty post-patent expiration (price and market penetration), we cannot forecast any further with accuracy. Therefore, we can assume that the drug value is greater than $157 million.

[9] https://www.technologyreview.com/s/609197/tracking-the-cost-of-gene-therapy/

[10] - https://ghr.nlm.nih.gov/condition/ornithine-transcarbamylase-deficiency

[11] https://www.bio.org/sites/default/files/Clinical%20Development%20Success%20Rates%202006-2015%20-%20BIO,%20Biomedtracker,%20Amplion%202016.pdf

DXT201

Wacc	9.61%		Drug Value (USD)	1,901,671,779.42								
			Stage 3 Probability of									
Premium for risk of project	2%		success	26%								
Total WACC	11.61%		Expected probability	494,434,662.65								
	2026	2027	2028	2029	2030	2031	2032	2033	2034	2035	2036	
Market size (Per person)	75,000	72,171	69,453	66,842	64,334	61,924	59,608	57,384	55,245	53,190	51,212	
Market growth (Per person)	171	169	167	165	163	162	159	157	155	149	147	
Market decrease (per person)	3,000	2,887	2,778	2,674	2,573	2,477	2,384	2,295	2,210	2,128	2,048	
Price in USD	1,000,000	1,000,000	1,000,000	1,000,000	1,000,000	1,000,000	1,000,000	1,000,000	1,000,000	1,000,000	1,000,000	
Patients treated (per person)	3,000	2,887	2,778	2,674	2,573	2,477	2,384	2,295	2,210	2,128	2,048	
Penetration rate %	4.00%	4.00%	4.00%	4%	4%	4%	4%	4%	4%	4%	4%	
	2026	2027	2028	2029	2030	2031	2032	2033	2034	2035	2036	
Revenue USD	3,000,000,000	2,886,824,642	2,778,110,372	2,673,679,863	2,573,351,894	2,476,957,506	2,384,339,518	2,295,340,349	2,209,811,975	2,127,616,020	2,048,472,862	
FCF/Sales Rate %	30%	30%	30%	30%	30%	30%	30%	30%	30%	30%	30%	
Free Cash Flow USD	900,000,000	866,047,393	833,433,112	802,103,959	772,005,568	743,087,252	715,301,855	688,602,105	662,943,593	638,284,806	614,541,858	
Discount Factor years	7.25	8.25	9.25	10.25	11.25	12.25	13.25	14.25	15.25	16.25	17.25	
Discounted FCF USD	405,748,594.89	349,811,463.79	301,606,706.40	260,063,119.39	224,257,204.72	193,394,289.70	166,790,400.52	143,855,976.71	124,083,491.65	107,035,982.28	92,330,380.66	

Figure 20 – DCF of UX007 – Author

- ***Price:*** This drug is a "one and done" treatment, where patients receive an injection of the gene therapy then they will experience decay of symptoms associated with haemophilia A. Our last report we evaluated a similar drug called 'Valrox' and priced it at $2 million per treatment. Due to its 'late-stage' onto the market and the increased technological capacities, we have priced the drug at $1 million.
- ***Market Size and Penetration:*** The market size for patients with HA in Ultragenyx's sales territory is currently 75,000. Market growth is based on 1 in 5000 live births being born with the disease. We believe that only limited patients will be able to access this drug throughout the lifespan of the patent due cost and willingness of Ultragenyx to treat and cure all the markets. The decrease in market size is due to the nature of gene therapy, the drug is a one and done treatment (at least for 10 years), therefore patients who get treated are no longer in market population.
- ***Valuation Length:*** We have chosen to value the product over a ten-year life span due to patents laws for the industry.
- ***Free cash flows generated from sales*** – We chose the industry average, this is market consensus, as we do not know the exact COGS, SG&A and R&D costs associated with this drug post-launch.
- WACC: The discount rate for this project is adjusted versus the overall company discount rate to account for increased risk and costs in clinical trial stage drugs.
- Expected probabilities: We utilized the expected probability of drugs in this field of research for drugs in stage 1 to make it to market. We used the probabilities to estimate an expected value adjusted for the probability of successful trials[12].
- ***Limitations:*** The drug will be on the market post valuation period, but due to uncertainty post-patent expiration (price and market penetration), we cannot forecast any further with accuracy. Therefore, we can assume that the drug value is greater than $494 million.

[12] https://www.bio.org/sites/default/files/Clinical%20Development%20Success%20Rates%202006-2015%20-%20BIO,%20Biomedtracker,%20Amplion%202016.pdf

Overall Valuation:

$ Millions	
On Market	2,118.55
DXT301	188.97
UX007	199.66
DTX401	157.30
DTX201	494.43
Implied equity value	3,158.91
Shares outstanding	57.75
Price per share	54.70
Current market	36.78
Difference	48.72%

Figure 21 – Implied equity value – Author

As can be seen, by the above graph, we valued the Company in two steps:

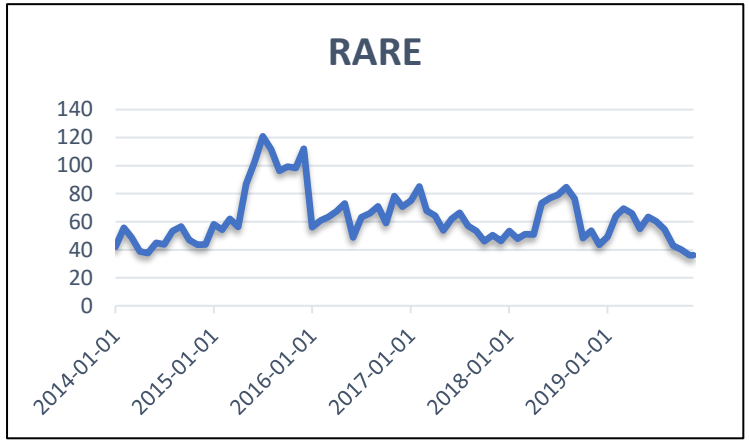
1. The current on the market drugs using an FCF proxy (Alexion), the driver of growth being the rapid expansion and sales of their two on the market drugs C and M
2. Valuation of the pipeline, using a combination of DCF modelling and probabilities to generate an expected NPV for each pipeline drug.

Overall the Company is forecasted to have an implied equity valuation of 3.15 Billion and a per-share price of $54.70.

Investment Thesis and Recommendation: Buy.

Based on a healthy forecast for sales and a valuation of the pipeline that highly considers the risks involved in this stock. We come to an undervaluation of 48.72%. Leading to a strong buy.

Explanations for the large undervaluation:



Represented in figure 22 Ultragenyx has been driven down in the last few weeks due to poor earnings performance in Q3 of 2019. The company CEO had an excuse that the poor earnings were attributable to a mismatch in delivery times for big order. They claim a big order was placed in the closing days of the quarter, but due to a delivery mix up, they could not attribute the order to this quarter.

Investors are also wary of the decision to give Gene TX $20 million to develop a gene therapy drug for haemophilia A, a lot of analysts and investors are finding it hard to value this position.

Over the past investors have been bullish on the stock due to the belief that the drugs they have are potential blockbusters, which we believe is the case. But the stock price was halved in 2016 due to failure to bring C onto the market, but the drug has since made it to market.

Figure 22 – Stock price evolution – Author

Reason for Bullish position:

- The market is extremely wrong on this stock due to the following reasons:
  - Failed to bring a drug to market in 2016
  - Poor earnings reports
  - Uncertainty about some of the R&D behaviours
- Why we believe the stock has potential:
  - No debt and adequate cash reserves give a platform for rapid growth
  - This Company has two drugs on the market, with one drug having a market size 76,000 people, with a maximum peak of 6$ Billion in Revenue for Ultragenyx, currently in year two and growing rapidly (double-digit growth in all Quarters of this year).
  - Ultragenyx has a very lucrative pipeline, which we value at roughly 1$ billion adjusted for high risks of clinical-stage drugs.
  - Innovation focused business, with an ‘entrepreneurial mindset’, unlikely to become an acquisition target due to this commitment to innovation investment in gene therapeutic companies such as GeneTx and Dimension [13]

[13] https://www.fiercebiotech.com/keyword/ultragenyx

Important Disclosure:

This report has been written by MBA students at Yale's School of Management in partial fulfilment of their course requirements. The reports are a student, not professional; they are intended to serve solely as examples of student work at Yale's School of Management. They are not intended as investment advice. They are based on publicly available information and may not be a complete analysis of all relevant data.

If you use these reports for any purpose, you do so at your own risk. YALE UNIVERSITY, YALE SCHOOL OF MANAGEMENT, AND YALE UNIVERSITY'S OFFICERS, FELLOWS, FACULTY, STAFF, AND STUDENTS MAKE NO REPRESENTATIONS OR WARRANTIES, EXPRESS OR IMPLIED, ABOUT THE ACCURACY OR SUITABILITY FOR ANY USE OF THESE REPORTS, AND EXPRESSLY DISCLAIM RESPONSIBILITY FOR ANY LOSS OR DAMAGE, DIRECT OR INDIRECT, CAUSED BY USE OF OR RELIANCE ON THESE REPORTS.