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Stock Rating
Overweight

Industry View
In-Line

Initiation

Ultragenyx Pharmaceutical Inc

Solid, Growing Orphan Drug Pipeline; Initiate OW, PT $81

Ultragenyx is focused on identifying promising early/mid stage assets in the orphan disease space.

Ultragenyx is an orphan disease focused co. that has in-licensed a series of interesting early/mid stage drugs. The current in-licensing model mitigates some of the high spend and clinical risk in drug discovery, setting the co. up ideally to have solid profitability. We currently view KRN23 and triheptanoin as key value drivers within the current pipeline, and expect business dev't to expand the pipeline with time. Importantly, while our risk/reward framework implies both sig. upside and downside are possible vs. the current stock price and our PT, we view the bear case (no drug success from the current pipeline) as very unlikely. This low probability skews the risk/reward sig. upwards.

Triheptanoin (UX007): This medium chain oil is being developed for GLUT-1 DS and LC-FAODs. In both cases, we believe the drug provides a key energy source for pts that have genetic mutations which leave them with insufficient energy production. While this set of disorders is somewhat heterogeneous, we believe the early data and scientific rationale for triheptanoin suggest a key role for the drug in these pts' diet. Formal Ph 2 studies will solidify key issues such as dosing and the best EPs for pivotal studies. We view the indications as having a combined $1+bn WW peak sales potential.

KRN23 (UX023): This Ph 1/2 drug, partnered with KHK, is in dev't for XLH and may represent a $1+bn WW peak sales opp'y. In Ph 1, the drug showed key, positive changes to phosphate and Vit. D – both of which are inappropriately low in XLH pts. We await Ph 2 data in adults in mid-14 to assess functional EP changes that could anchor a pivotal trial, and believe the biochem changes in Ph 1 should translate into clinical benefit.

MPS-7 (UX003): This drug is earlier stage, with 1 pt treated (successfully) that we know of. However, drug dev't for MPS diseases with a sig. non-CNS component, incl. MPS-7, has typically been successful. Solid animal data for this drug helps corroborate our positive view. A quick path to mkt should be possible given the ultra-rare pt numbers (~200 WW), but the mkt potential and valuation is likely limited for these same reasons.

ER Sialic acid (UX001): This drug is in Ph 2 for HIBM. Given the modest benefit in Ph 2 to date, we reserve this drug for our bull case (~$160mn peak WW). Higher dose data should better clarify this drug's potential.

Key Ratios and Statistics

Reuters: RARE.O Bloomberg: RARE US
Biotechnology / United States of America

Price target	**$81.00**
Shr price, close (Feb 21, 2014)	$59.10
Mkt cap, curr (mm)	$1,769
52-Week Range	$62.48-35.15

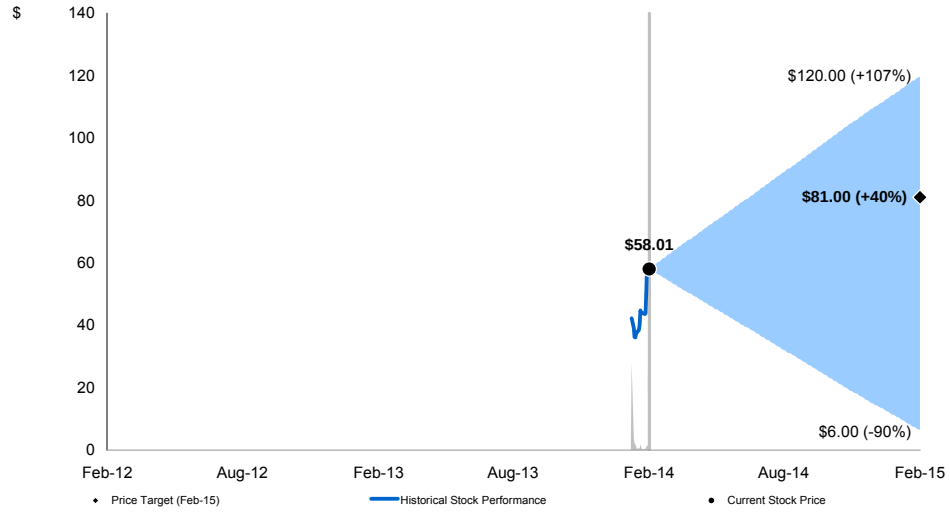
Fiscal Year ending	**12/13**	**12/14e**	**12/15e**	**12/16e**
ModelWare EPS ($)#	**(7.90)**	**(1.91)**	**(2.52)**	**(3.09)**
P/E	**NM**	**NM**	**NM**	**NM**
Consensus EPS ($)§	**-**	**-**	**-**	**-**
Div yld (%)	-	0.0	0.0	0.0

Unless otherwise noted, all metrics are based on Morgan Stanley ModelWare framework (please see explanation later in this note).
= Our pension accounting has changed in ModelWare, which will affect ModelWare EPS figures for some stocks under coverage. Visit www.ms.com/mw.pdf for details
§ = Consensus data is provided by Thomson Reuters Estimates.
e = Morgan Stanley Research estimates

Glut-1 DS (glucose transporter type-1 deficiency syndrome)	Brain energy metabolism disorder.
LC-FAODs (long-chained fatty acid oxidation disorders)	Disease group that leads to an inability to convert some types of fat (long chains fatty acids) into energy.
XLH (X-linked hypophosphatemia)	Disease resulting in low serum phosphate levels and poor bone mineralization.
MPS 7 (mucopolysaccharidosis 7)	Mucopolysaccharidosis with severe organ dysfunction.
HIBM (Hereditary Inclusion Body Myopathy)	Neuromuscular disease characterized by severe muscle weakness.
Galactosialidosis	Lysosomal storage disease leading to an accumulation of oligosaccharides.
EP	Endpoint
PT	Price target

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:** 1) ~$1.2bn WW peak (2025) sales for triheptanoin in Glut-1 DS, 2) ~$800mn WW peak (2025) sales for triheptanoin in LC-FAOD, 3) ~$1.8bn 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:** 1) ~$900mn WW peak (2025) sales for triheptanoin in Glut-1 DS, 2) ~$600mn WW peak (2025) sales for triheptanoin in LC-FAOD, 3) ~$1.4bn WW peak (2025) sales for KRN23 in XLH, and 4) ~$70mn WW peak (2025) sales for rhGUS in MPS 7.
Bear Case $6	**Cash Based Value**	**None of the drugs in development makes it to market.** 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.

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.

Investment Case

Summary & Conclusions

We are initiating coverage of Ultragenyx (RARE) with an Overweight rating and an $81 price target.
Ultragenyx is a biotechnology company focused on the development of drugs for ultra-orphan diseases. We view this as an area of drug development that often affords shortened development paths, focused commercial infrastructures, and significant drug pricing power given the typically few options and devastating outcomes for pts. In addition, by focusing to date on an in-licensing based business model, the company should have an increased likelihood of reaching solid profitability as they do not need to support a large research discovery engine.

Importantly, when one evaluates our risk/reward assessment for this stock (p. 2), we note that it appears as if there is a chance of both significant upside and downside vs. the current stock price and our price target. **However, we note that given the accumulated evidence to date, we view the bear case (current pipeline generates no approvable drugs) as very unlikely. Therefore, we see a clearly upward based skew to the risk/reward for this stock.**

We see triheptanoin (for Glut-1 DS and LC-FAOD) and KRN23 (for XLH) as the key value drivers for our valuation. We also model rhGUS for MPS 7, but overall view this as a smaller opportunity based on a lower pt prevalence.

In this report we discuss the following:
1) Ultragenyx's business model
2) Triheptanoin (UX007) for Glut-1 DS and LC-FAOD,
3) KRN23 (UX023) for XLH,
4) rhGUS (UX003) for MPS 7,
5) Sialic Acid-Extended Release (UX001) for HIBM, and
6) Other pipeline drugs.

1) Ultragenyx's Business Model

Ultragenyx's business model to date has focused on in-licensing products for ultra-orphan sized diseases. We typically think about populations <10K in the US or EU as fitting this group. We see several advantages to this business model and the current team that is executing on it.

First, the assets have tended to be partially de-risked, as early proof-of-concept via metabolic studies, case cohorts, and/or anecdotal pt data has often been available to the company prior to in-licensing a program.

Second, as a result of both in-licensing already identified drugs/targets and thus not having to perform all the pre-clinical work, **the company should have a lower core R&D spend** as compared to other companies. We believe this could ultimately trim >$100mn annually from their peak R&D budget, although it does place a burden on finding new assets.

Third, the company's drug risk is already spread out among multiple products. We do not tend to subscribe to the "multiple shots on goal" thesis in the absence of a) clear clinical activity, b) a reasonable development path, and c) a logical commercial market. However, given that Ultragenyx has been able to find assets that tend to fit most/all of these buckets, we like the diversification and "risk sharing" that comes with multiple potential value drivers.

Finally, management has already proven their experience in orphan drug development. In our view, early patient identification efforts are crucial as they can support a) swifter trial enrollment, b) initial commercial success, and c) a model for more sustained pt identification and diagnosis. To this end, the company's focus on identifying patients - in some cases already identifying 50+% of the prevalent pts as predicted in the literature (Ex. 1) - is an area where we think Ultragenyx is quite advanced. Ultimately, we see early pt identification as emblematic of a more broad integration with the rare disease community, where small physician networks can manage large parts of the prevalent pt pool. Finally, we note that in cases where a large portion of the prevalent pool is already identified, it is possible that the prevalence is underestimated.

Exhibit 1
Ultragenyx Has Focused on Early Patient ID

Disease	Prevalence	# of Patients Identified	% of Prevalence
Glut1 DS	~3,000-7,000 US	>80 patients US; ~200 patients WW	~2% of US
LC-FAOD	~2,000-3,500 US	>600 patients US; ~1,300 patients WW	~22% of US
XLH	~3,000 pediatric and ~9,000 adult patients US	n/a	n/a
MPS 7	~200 patients WW	~15 patients US; ~90 patients WW	~45% of WW
HIBM	~300-400 patients US; ~1,200-2,000 patients WW	>300 patients US; >800 patients WW	~86% of US; 50% of WW

Source: Company Data, Morgan Stanley Research

There are some risks to the business model as we see it, which we discuss below. In the end, we believe these risks are more than offset by the above described benefits.

First, the company could have trouble finding assets over time which have attractive qualities including a rationale for or proven proof-of-concept, an ability to effectively treat diseases with a high unmet need, and an ability to be commercialized profitably. Given the speed with which the company identified and in-licensed the first set of assets, we expect that there are likely other opportunities out there or that will emerge over the coming years. In addition, the company's relationships within the rare disease community and explicit focus on the rare disease space should help in-licensing efforts.

Second, the company is already managing multiple clinical programs. Compared to other small companies which devote focus to only one program, Ultragenyx is solely or partly managing four clinical programs. In addition, many of the drugs, if successful, are likely to launch commercially around the same time (~2017-2018). As the company expands with time, we expect their ability to manage multiple programs will increase as well. More importantly, the focused development programs typical of orphan drugs help mitigate some of the risk from managing large clinical trial efforts. That being said, we do expect the recent in-licensing pace to slow down for now in order to allow for solid Ph 2 work on current assets.

Finally, the company's focus exclusively on orphan drugs puts a "burden on the drugs" to deliver meaningful clinical benefit. While we like to think that all drugs destined for approval would deliver clear pt value, we note the expected pricing required to make the orphan drug business model successful almost demands a strong drug benefit. In this case, a lack of a full, expensive discovery engine, coupled with a focused business model should maximize profitability, potentially allowing the company to make smaller drugs successful where other companies cannot. We view this as a competitive advantage in business development activities.

Exhibit 2

Drug Summary Chart for Ultragenyx

Drug	Indication	Stage	Drug Form/Delivery	Partner	IP
KRN23 (UX023)	XLH (X-linked hypophosphatemia)	Ph 1/2 in adults ongoing; Ph 2 in peds to initiate 2H14	Subcutaneous Antibody	KHK (Kyowa Hakko Kirin) regional rights	Patents expire 2022-2029
rhGUS (UX003)	MPS 7 (mucopolysaccharidosis 7)	Ph 1/2 interim data 1H14	IV ERT (enzyme replacement therapy)	WW rights licensed from St. Louis University	In-progress
Tripheptanoin UX007	LC-FAOD (long-chained fatty acid oxidation disorder) Glut 1 DS (glucose transporter type-1 deficiency syndrome)	Ph 2 data 2015 Initiate Ph 2 1H14; data 2015	Oral liquid	WW rights licensed from Baylor Research Institute	Patents expire 2020-2024; pending patents 2020-2034
Sialic Acid - ER UX001	HIBM (Hereditary Inclusion Body Myopathy)	Ph 2 extension study at higher doses data 2H14	Oral	WW rights excl. Japan and some of Asia from Nobelpharma	Patents expire 2028-2033
rhPPCA UX004	Galactosialidosis	Pre-clinical	ERT (enzyme replacement therapy)	Licensed from St. Jude Children's Research Hospital	

Source: Company Data, Morgan Stanley Research

Below we briefly highlight the drugs in development, with a more fulsome discussion of each drug in subsequent sections.

2) Triheptanoin (UX007) for Glut-1 DS and LC-FAODs

Triheptanoin, a synthetic medium chain fatty acid, is in Ph 2 development for two severe diseases: Glut-1 DS and LC-FAODs. Both diseases are a result of the body's inability to properly manage energy production, either systemically or in specific organs. While still early, case cohort and anecdotal data look promising and consistently solid. Ph 2 data for both indications in 2015 should help confirm efficacy under a more controlled setting. **We currently model peak WW sales of ~$600mn in LC-FAODs and ~$900mn in GLUT-1 DS.**

3) KRN23 (UX023) for XLH

KRN23 is an antibody that binds to FGF23, a protein that is elevated in patients with X-linked hypophosphatemia (XLH). These pts have excess urinary phosphate and poor vitamin D control, leading to poor bone mineralization and in some cases (typically pediatric) rickets, bowed legs, and fractures. A Ph 1 single dose trial showed increases in serum phosphate and active vitamin D levels, which we believe will ultimately translate into bone benefits with time. This drug is partnered with Kyowa Hakka Kirin (KHK), with regional economics described inside. **We currently model ~$1.4bn peak WW sales.**

4) rhGUS (UX003) for MPS 7

While the MPS 7 market is small, we see this as a solid add-on indication to Ultragenyx's other pipeline drugs. MPS 7 is a very rare mucopolysaccharidosises (MPS) with similar pathology to other MPS's. rhGUS is an enzyme replacement therapy, and data in one patient treated under an emergency IND program look promising. We expect success similar to other MPS drugs which target MPS subtypes that have a large peripheral component. **We currently model ~$70mn in peak WW sales.**

5) Extended Release Sialic Acid (UX001) for HIBM

We reserve sialic acid sales for our bull case. The drug showed some activity in Ph 2, but results were not consistent or strong enough to justify orphan drug pricing. The company is hoping that higher doses in a Ph 2 extension trial may help show more meaningful clinical results. **In our bull case, we currently model peak WW sales of ~$160mn.**

6) Other Pipeline Drugs

A preclinical enzyme replacement therapy, rhPPCA (recombinant human protective protein cathepsin-A) or UX004, is in development for galactosialidosis. Ultragenyx licensed this drug from St. Jude Children's Research Hospital, and intends to begin clinical studies in 2015 or 2016. While too early for us to value, we view this drug as interesting.

Galactosialidosis is a lysosomal storage disease leading to an accumulation of oligosaccharides with severe skeletal and organ dysfunction that eventually leads to death. In the infantile form, hydrops fetalis may develop.

Exhibit 3

Catalyst Calendar

Drug	Type	Event	Expected Timing
rhGUS (UX003)	Clinical Data	Ph 1/2 interim (12 week) data	1H14
Triheptanoin (UX007)	Product Advancement	Ph 2 in Glut-1 DS initiation	1H14
KRN23	Clinical Data	Ph 1/2 multidose data in XLH adult patients	Mid-14
KRN23	Product Advancement	Initiate Ph 2 study in XLH pediatric patients	2H14
rhGUS (UX003)	Clinical Data	Ph 1/2 dose titration 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 data in LC-FAOD	2H15

Source: Company Data, Morgan Stanley Research

Valuation

Exhibit 4
DCF Drives Valuation

	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E	2033E
Free Cash Flow	($33.35)	($51.72)	($69.22)	($86.88)	($114.22)	($88.74)	$137.61	$450.34	$634.89	$844.92	$971	$1,058	$1,128	$903	$722	$722	$722	$722	$578	$462	$370
YoY Growth	*145.3%*	*55.1%*	*33.9%*	*25.5%*	*31.5%*	*-22.3%*	*-255.1%*	*227.3%*	*41.0%*	*33.1%*	*15.0%*	*8.9%*	*6.7%*	*-20.0%*	*-20.0%*	*0.0%*	*0.0%*	*0.0%*	*-20.0%*	*-20.0%*	*-20.0%*
Net Cash Proxy for Dilution		($3.75)	($4.33)	($4.96)	($5.62)	($6.30)	($7.00)	($6.78)	($6.18)	($6.80)	($7.48)	($8.23)	($9.05)	($9.05)	($9.05)	($9.05)	($9.05)	($9.05)	($9.05)	($9.05)	($9.05)
Free Cash Flow for DCF	($33.35)	($55.47)	($73.56)	($91.83)	($119.83)	($95.04)	$130.61	$443.57	$628.70	$838.12	$964	$1,049	$1,119	$894	$713	$713.06	$713.06	$713.06	$568.64	$453.10	$360.67
Present Value of Free Cash Flow		($55.47)	($63.96)	($69.44)	($78.79)	($54.34)	$64.93	$191.77	$236.35	$273.98	$274.02	$259.42	$240.57	$167.02	$115.89	$115.89	$115.89	$115.89	$92.42	$73.64	$58.62

Source: Company data, Morgan Stanley Research estimates

Exhibit 5
DCF Valuation Suggests Significant Upside

Valuation Date	**2014.0**
Discount Rate	**15%**
Terminal Growth Rate	0%
Terminal Value Year	2033
Sum of Discounted FCF	$2,074
Discounted Terminal Value	$169
Net Cash	$190
Equity Value	**$2,433**
Equity Value/Sh (Basic)	**$81**
Shares Outstanding (Basic)	29.9

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 2030-2033 due to the ~2030 composition of matter patent expiry for KRN23 (the final drug in our model). Beyond 2033, 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.

Triheptanoin (UX007) for Glut-1 DS and LC-FAODs

We see significant potential for triheptanoin in both Glut-1 DS and LC-FAODs. Both of these diseases involve an inability of the body to convert certain types of food into energy. For Glut-1 DS (Ex. 6), the body cannot appropriately take up glucose in the brain. In LC-FAODs (Ex. 7), the body cannot metabolize long chain fats throughout the body. **While specific drugs and diets are currently available and used in both diseases, they are difficult to adhere to chronically and yield insufficient benefits. Thus, we see a clear unmet need for a more effective therapy, and believe the early evidence for triheptanoin suggests it could fill this role.**

Exhibit 6

Glut-1 DS Disease Summary

Glut-1 DS (Glucose Transporter Type-1 Deficiency Syndrome)	
Disease	Brain energy metabolism disorder
Cause	Mutation in Glut1, which transports glucose (main energy supply for brain) across cell membranes
Prevalence	~3,000-7,000 patients in the US
Patients Identified by RARE	>80 patients in the US and ~200 individual patients WW
Symptoms	Seizures; development, movement, and behavior disorders
Current Treatments	Ketogenic diet (high fat, low protein, low carb diet); antiepileptic drugs
Diagnosis	Clinical symptoms, spinal fluid glucose level (measured via spinal tap), genetic tests

Source: Company Data, Morgan Stanley Research

Exhibit 7

LC-FAODs Disease Summary

LC-FAOD (Long-Chained Fatty Acid Oxidation Disorders)	
Disease	Inability to convert some types of fat (long chains fatty acids) into energy. Includes CPT-I and II, CACT, VLCAD, LCHAD, TFP, SCAD (different disorder types).
Cause	Due to autosomal recessive defect in genes that encode fatty acid metabolism enzyme
Prevalence	~2,000-3,500 patients in the US
Patients Identified by RARE	>600 patients in the US and ~1,300 patients WW
Symptoms	Muscle problems (pain, rupture, fatigue, breakdown); hypoglycemic events due to depletion of glucose; early death (sudden infant death syndrome), hospitalization
Current Treatments	Diet of high carbohydrate, low fat foods; avoidance of fasting (frequent eating); MCT (Medium chain triglyceride) oil
Diagnosis	US: Diagnosed through newborn screening tests; Ex-US: May be diagnosed through symptoms

Source: Company Data, Morgan Stanley Research

Triheptanoin is a synthetic odd-chained triglyceride composed of three seven-carbon fatty acids. The drug is an oil and is taken orally in liquid form.

Below we address in more detail:
1) Case cohort and anecdotal **efficacy data** which we believe is suggestive of drug benefit,
2) **Safety data**, which is early but relatively clean so far,
3) The **path forward** in both indications, and
4) The ~$1.5+bn peak WW **market opportunity** in Glut-1DS and LC-FAODs combined.

1) Solid Efficacy Data

Triheptanoin has been studied in ~130 patients, including 65 patients with LC-FAODs (2 academic pt series) and 2+ Glut-1 DS patients, for up to 13 years. While these data are anecdotal and/or retrospective, we are able to gain conviction in triheptanoin's benefit given the drug's clear rationale and mechanism for treating GLUT-1DS/LC-FAODs and the apparent consistency of response to date. Ph 2 trial data should provide key supportive data, if positive.

Mechanism of Action Supports Drug Efficacy

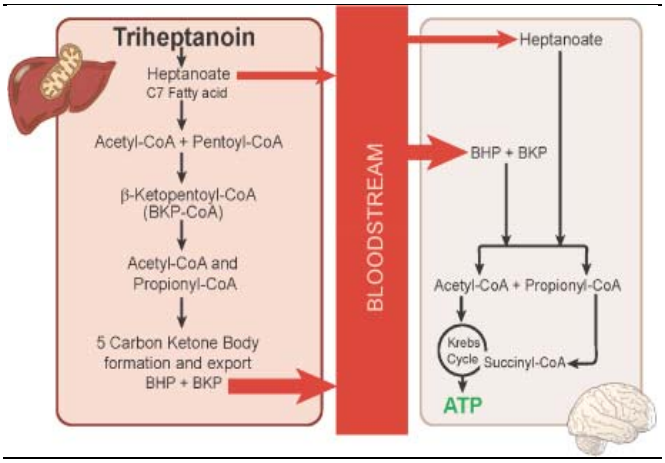
The body in general works with three sources of energy: carbohydrates, fats, and proteins. Carbohydrates are the body's first and preferred energy source, with the core mediator of this pathway being glucose. Fat is typically reserved for use under stress, exercise, or fasting conditions when the body's glucose stores are lower. In a normal person at rest, odd chain fats (and possibly even chain ones as well) are thought to be broken down and then used to make glucose. Proteins can be broken down and used for energy. While the body is capable of easily using dietary protein for energy production, it "tries" to avoid breaking down endogenous proteins (e.g. muscle) and does so often only in cases of severe stress or malnutrition.

The brain specifically has a preferred energy source in glucose, which is formed from carbohydrates. An alternative source that the brain can use is ketones, which are formed when fat is metabolized.

Glut-1 DS: In Glut-1 DS patients, a defect in Glut1, which transports glucose across cell membranes primarily in the brain (and also red blood cells), leads to a lack of glucose and therefore low energy in the brain. One result that is thought to come from a brain that is stressed due to insufficient energy production is seizure. While a ketogenic diet (e.g. a diet that is meant to maximize ketone production, discussed below) has long been tried to aid in seizure control, it is often insufficient due to difficult compliance.

Triheptanoin has two mechanisms that should allow for a more efficient increase in brain energy (Ex. 8). First, a primary metabolite of triheptanoin, heptanoate, can cross the blood/brain barrier and be used to form glucose – the preferred energy source. Second, ketones, which can easily be made from treiheptanoin, can cross into the brain and be used as the alternative energy form to glucose. We believe this two-pronged benefit should augment efficacy.

Exhibit 8
Triheptanoin Mechanism for Glut-1DS

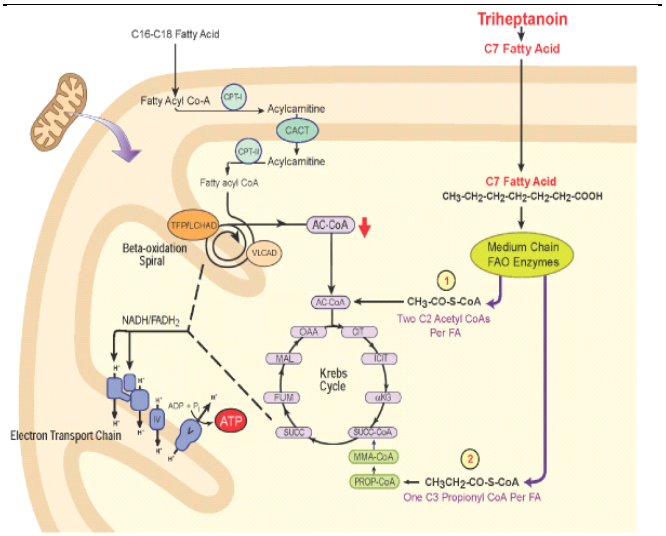


Source: Company Data, Morgan Stanley Research

LC-FAOD: In healthy people, the Krebs Cycle, a series of chemical reactions in the mitochondria (the energy producing part of a cell), produces energy from carbohydrates, fats, and proteins (Ex. 9). However, in patients with LC-FAODs, the body is not able to appropriately break down long chain fats. This problem ultimately causes the Krebs cycle to not function properly, leading to insufficient energy production that is exacerbated in times of physical stress (e.g. illness).

Triheptanoin acts as an alternative medium chain fat that can provide two key benefits that not all other medium chain fats can provide. First, it can be broken down and serve as an input to the Kreb's Cycle, leading to energy production. However, just as importantly, the drug appears to have the ability to restore the actual machinery of the Kreb's Cycle (i.e. it is anaplerotic). This last point is key to allowing the body to maximize energy production sustainably.

Exhibit 9
Triheptanoin Mechanism for LC-FAOD



Source: Company Data, Morgan Stanley Research

Efficacy Data Early but Solid
Data for triheptanoin is mostly in LC-FAODs patients, but there are anecdotal accounts of two Glut-1 DS pts treated with triheptanoin.

Glut-1 DS Data: Our physician diligence suggests that efficacy results in LC-FAOD are likely to translate into Glut-1 DS given the clear drug mechanism. Triheptanoin is being studied in multiple Glut-1 DS investigator initiated trials. While results have yet to be reported, there are anecdotal accounts of seizure and development benefit in two patients out of University of Texas. Both had improved seizure control and improvements in motor, cognitive, and language development.

LC-FAODs Data: ~65 LC-FAODs patients have received triheptanoin for up to 13 years, with results aggregated in multiple patient series. While these data are not controlled, we believe that the demonstrated benefits suggest strong clinical activity for this drug in what is otherwise a devastating disease.

A retrospective medical record analysis of major medical events in 20-24 patients in a triheptanoin compassionate use program at the University of Pittsburgh showed solid efficacy (Ex. 10). Triheptanoin significantly lowered mean total hospital days/year and hypoglycemia events/year. There were also trends in lower total hospitalizations/year, although we think this may prove to be a tougher hurdle given the conservative behavior that surrounds such serious diseases.

Exhibit 10
U Pitt Compassionate Use Study for LC-FAOD Pts

Description	Pre-treatment	Post-treatment	% decrease	n	p-value
Mean total hospitalizations/year[1]	1.94	1.26	36%	16	0.1126
Mean total hospital days/year[1],[2]	17.55	5.4	69%	15	0.0242
Mean infant total hospitalizations/year[3]	13.01	1.37	89%	4	0.0892
Mean hypoglycemia events/year[1],[4]	0.92	0.04	96%	9	0.0091
Mean hypoglycemia total hospital days/year[1],[2],[4]	8.42	0.18	98%	9	0.0257
Mean rhabdomyolysis events/year[1],[5]	1.05	0.68	35%	11	0.4604
Mean rhabdomyolysis total hospital days/year[1],[5]	5.94	2.16	64%	9	0.1224
Mean peak creatine kinase (units) for rhabdomyolysis events[1],[5]	85,855	25,797	68%	7	0.1279

Source: Company Data, Morgan Stanley Research

A study from Baylor University studied the effect of switching patients from the current standard of care therapy (MCT oil and/or low-fat, high carbohydrate diet) to triheptanoin. The trial showed cardiac, muscle, liver, energy, and hypoglycemia improvements (Ex. 11) in patients after starting triheptanoin.

Exhibit 11
Baylor SoC to Triheptanoin Switch Study

Symptoms	# of Symptomatic Patients (n=48) Before Triheptanoin (MCT oil and/or low fat, high carb diet)	After Triheptanoin
Cardiac	10	1
Rhabdomyolysis	36	15
Weakness/fatigue	44	10
Hypoglycemia	24	1
Hepatomegaly	26	2
Retinopathy	3	3

Source: Company Data, Morgan Stanley Research

2) Safety Data

In the 65 LC-FAODs patients treated with triheptanoin, the safety profile has been mostly mild. GI side effects, particularly cramping, diarrhea, and loose stools, have affected some patients. These seem less severe than the GI problems we have heard associated with MCT oil. We do not expect major issues to arise with larger pt exposures.

There were three serious adverse events possibly related to triheptanoin, all of which we believe are typical for LC-FAODs patients and consistent with disease sequelae. These patients were treated with low-dose triheptanoin. Two patients had muscle cell rupture and elevated creatine kinase (associated with infections) and one patient had myoglobinura, which is associated with muscle destruction.

3) Path Forward

Glut-1 DS: Ultragenyx plans to initiate a Ph 2 trial in early 2014, with data expected in early 2015, in ~50 Glut-1 DS pediatric patients aged 2-17 years old that are either not on or not fully compliant with a ketogenic diet (Ex. 12). The trial will be placebo (safflower oil) controlled for both a 2 week titration period and an 8 week treatment period, after which patients will have the option of enrolling in the extension trial. We believe that triheptanoin may ultimately be dosed at a target of ~35% of the total caloric intake. Patients will be able to remain on up to 3 antiepileptics during the trial, ideally at stable doses.

While there was some initial concern that enrollment could be difficult if criteria were restricted to pts not on any type of ketogenic diet (given it is part of the standard of care), the looser criteria of pts who are either not on or not compliant/successful with the diet should allow easier recruitment. We will keep an eye on timelines here.

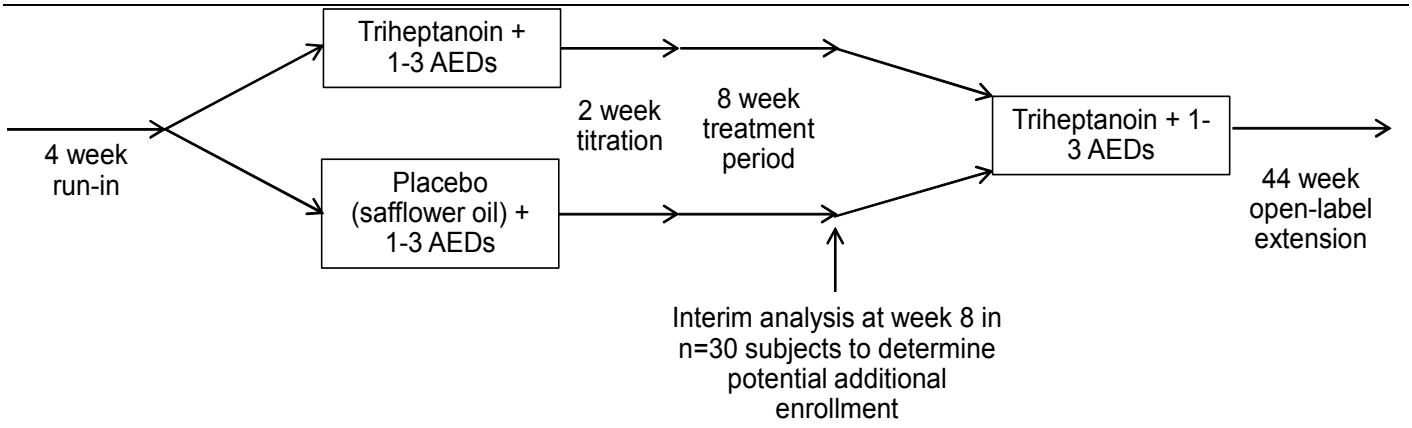
The primary endpoint for this trial will be a reduction in frequency of generalized or partial onset seizures. Our diligence suggests that doctors would ideally like to see a trend showing improvements in development, movement, and/or behavior symptoms as well as a seizure improvement. While the diet, even with good compliance, typically does not lead to other behavioral improvements, we note that poor dietary compliance suggests that even good seizure control would be an improvement in many pts.

We expect that a Ph 3 trial will likely focus on a seizure related endpoint, but commercial success would be augmented by triheptanoin's ability to improve other endpoints as well.

LC-FAODs: A Ph 2 open-label study in ~30 LC-FAODS patients aged 6 months to 35 years is enrolling with interim data likely in 2015 (Ex. 13). Triheptanoin will be dosed up to 25-35% total caloric intake at least 4x/day. Endpoints highlighted by Ultragenyx include cycle ergometer performance, 12 meter walk test, muscle strength, creatinine kinase levels, hypoglycemia, liver size, and cardiac disease. These are all endpoints that will focus on the metabolic potential of the body.

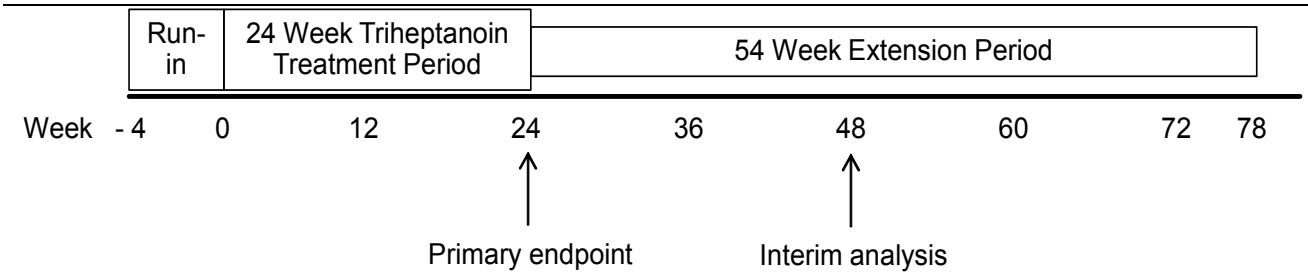
Ph 3 initiation may start after the Ph 2 24 week data. Given the range of disease symptoms existing and being tested, the company intends to use the Ph 2 trial to determine an appropriate clinical endpoint for Ph 3. The focus will be on endpoints such as skeletal myopathy, liver disease, and cardiac disease. A key will be determining which endpoints can be consistently measured with significance in a small trial during an ideally short, defined time period.

Exhibit 12
Trial Design for Glut-1 DS Ph 2



Source: Company Data, Morgan Stanley Research

Exhibit 13
Trial Design for LC-FAOD Ph 2



Source: Company Data, Morgan Stanley Research

4) Market Opportunity

We see ~$940mn peak (2025) sales for Glut-1 DS and ~$600mn (2025) peak sales for LC-FAODs.
Below, we address:
a) Glut-1DS and LC-FAODs disease severity,
b) Competition and differentiation vs. current treatments, and
c) Economics and market opportunity.

a) Glut-1 DS and LC-FAODs Are Very Severe Diseases
Glut-1 DS: In Glut-1 DS patients, the lack of glucose in the brain leads to early onset seizures as well as cognitive, movement, and behavior disorders. The epilepsy component is heterogeneous in terms of type and frequency of seizures. Movement disorders may include body jerks, walking difficulties, fidgety movements, twitching, etc.

Patients are often diagnosed via clinical symptoms. Both spinal taps measuring (low) glucose levels in the spinal fluid and genetic tests may confirm the disease, though physicians may not be able to find the relevant mutation in some cases.

LC-FAODs: LC-FAODs patients develop various issues relating to the inability to process fats. Symptoms often are worse between meals or during periods of high energy consumption/glucose requirements, such as illness or exercise. Severe muscle problems such as pain, fatigue, and muscle breakdown (rhabdomyolysis) are common. Cardiac disease may develop as the heart uses long-chain fatty acids for >1/2 of its energy and is less tolerant of low energy states than skeletal muscle. Hypoglycemia, weakness, and fatigue may occur as well when glucose is depleted. LC-FAODs are also a suspected cause of sudden infant death syndrome (SIDS). Though some LC-FAODs patients never become symptomatic, others may be frequently hospitalized due to the above symptoms.

In the US, patients are often diagnosed through newborn screening tests, although not all states test for all subtypes. Ex-US, where newborn screening for LC-FAODS is less common, patients may be diagnosed by the above symptoms.

b) Competition and Current Treatment Options
Glut-1 DS: Patients are typically treated with a ketogenic diet (high fat, low protein, low carbohydrate) to stimulate the

production of ketones. While a ketogenic diet, if strictly adhered to, may help with seizures, it often has little effect on other symptoms of Glut-1 DS. Over time, many patients find it hard to stick with the diet despite the serious consequences. The diet can also lead to elevated cholesterol and triglyceride levels, which may result in other negative health effects.

Patients are also often treated on 1+ antiepileptic drugs, but many patients tend to be resistant. There are no anti-epileptic drugs approved to treat Glut-1 DS specifically.

LC-FAODs: A typical LC-FAODs diet attempts to provide energy substrates for the body with substances other than long-chain fatty acids. The diet is high in carbohydrates and low in fats. Patients are also advised to avoid fasting and to eat frequently in order to maintain a steady supply of glucose.

MCT (medium chain triglyceride), which is composed of medium even chain fatty acids, is typically used in the diet. Similar to triheptanoin, this oil can bypass long-chain fatty acid blocks to provide energy. However, it is not wholly effective at preventing disease complications and cannot be used by the body to replenish the energy producing machinery. GI tolerability is poor, but can be somewhat improved when the oil is mixed with food. As this oil is not approved as a drug substance, the ~$5-20k of annual oil cost is out of pocket for some patients if insurance does not reimburse it. We understand compliance to be poor over time.

The drug Bezafibrate, a fibrate, has some use ex-US. We do not view this drug as a sig. competitor as a 3 month double blind trial did not improve fatty acid oxidation in patients with carnitine palmitoyltransferase and very long-chain acyl-CoA-dehydrogenase deficiency.

B. Braun Medical has also applied for and received orphan drug designation for triheptanoin in the EU, but we are not aware of any ongoing development activity.

c) Economics

Ultragenyx licensed triheptanoin from Baylor Research Institute and pays a mid-single digit royalty on net sales to Baylor.

Our $360mn of peak (2025) US sales for Glut-1 DS assumes:
1) a 2018 launch,
2) ~5k Glut-1 patients,
3) ~55% penetration into the market, and
4) a $112.5k annual net price.

Our $580mn of peak (2025) ex-US sales for Glut-1 DS assumes:
1) a 2018-2019 launch,
2) ~12-13k Glut-1 patients,
3) ~40-50% penetration into the market, and
4) a $100k annual net price.

Our $260mn of peak (2025) US sales for LC-FAODs assumes:
1) a 2018 launch,
2) 3.5k LC-FAODs patients,
3) ~55% penetration into the market, and
4) a $112.5k annual net price.

Our $340mn of peak (2025) ex-US sales for LC-FAODs assumes:
1) a 2018-2020 launch,
2) ~7k LC-FAODs patients,
3) ~40-50% penetration into the market, and
4) a $100k annual net price.

Exhibit 14

Glut 1 DS Market Model

US ($ in millions)	2013	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
GLUT1 patients	5,000	5,050	5,101	5,152	5,203	5,255	5,308	5,361	5,414	5,468	5,523	5,578	5,634
YoY growth		1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%
Beginning yr pts						0	263	1,046	1,798	2,249	2,684	2,825	2,963
007 new additions						263	796	804	541	547	276	279	282
% of GLUT1 pts						5%	15%	15%	10%	10%	5%	5%	5%
Deaths/Discon						0	13	52	90	112	134	141	148
% of chronic 007 treated						5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%
End of Yr Pts						263	1,046	1,798	2,249	2,684	2,825	2,963	3,097
End of Yr share (% GLUT1 pts)						*5%*	*20%*	*34%*	*42%*	*49%*	*51%*	*53%*	*55%*
Cost per month						$9,375	$9,563	$9,754	$9,949	$10,148	$10,351	$10,558	$10,769
Price Increase YoY							2%	2%	2%	2%	2%	2%	2%
New pt duration of therapy (mo)						6	6	6	6	6	6	6	6
Existing pt duration of therapy (mo)						11	11	11	11	11	11	11	11
New pt revenue						15	46	47	32	33	17	18	18
Existing pt revenue						0	27	109	192	245	298	320	342
Total US 007 GLUT1 Sales						**$15**	**$73**	**$156**	**$224**	**$278**	**$315**	**$338**	**$360**

EU ($ in millions)	2013	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
GLUT1 patients	8,000	8,080	8,161	8,242	8,325	8,408	8,492	8,577	8,663	8,749	8,837	8,925	9,015
YoY growth		1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%
Beginning yr pts						0	378	1,506	2,588	3,239	3,864	4,069	4,267
007 new additions						378	1,146	1,158	780	787	398	402	406
% of GLUT1 pts						5%	14%	14%	9%	9%	5%	5%	5%
Deaths/Discon						0	19	75	129	162	193	203	213
% of chronic 007 treated						5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%
End of Yr Pts						378	1,506	2,588	3,239	3,864	4,069	4,267	4,459
End of Yr share (% GLUT1 pts)						*5%*	*18%*	*30%*	*37%*	*44%*	*46%*	*48%*	*49%*
Cost per month						$8,438	$8,438	$8,438	$8,438	$8,438	$8,438	$8,438	$8,438
Price Increase YoY							0%	0%	0%	0%	0%	0%	0%
New pt duration of therapy (mo)						6	6	6	6	6	6	6	6
Existing pt duration of therapy (mo)						11	11	11	11	11	11	11	11
New pt revenue						19	58	59	39	40	20	20	21
Existing pt revenue						0	34	136	234	293	350	368	386
Total EU 007 GLUT1 Sales						**$19**	**$92**	**$195**	**$274**	**$333**	**$370**	**$389**	**$407**

ROW ($ in millions)	2013	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
GLUT1 patients	4,000	4,040	4,080	4,121	4,162	4,204	4,246	4,289	4,331	4,375	4,418	4,463	4,507
YoY growth		1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%
Beginning yr pts							0	172	684	1,176	1,472	1,756	1,849
007 new additions							172	521	526	354	358	181	183
% of GLUT1 pts							4%	12%	12%	8%	8%	4%	4%
Deaths/Discon							0	9	34	59	74	88	92
% of chronic 007 treated							5%	5%	5%	5%	5%	5%	5%
End of Yr Pts							172	684	1,176	1,472	1,756	1,849	1,939
End of Yr share (% GLUT1 pts)							*4%*	*16%*	*27%*	*34%*	*40%*	*41%*	*43%*
Cost per month							$0	$8,438	$8,438	$8,438	$8,438	$8,438	$8,438
Price Increase YoY							0%	0%	0%	0%	0%	0%	0%
New pt duration of therapy (mo)							6	6	6	6	6	6	6
Existing pt duration of therapy (mo)							11	11	11	11	11	11	11
New pt revenue							0	26	27	18	18	9	9
Existing pt revenue							0	16	62	106	133	159	167
Total ROW 007 GLUT1 Sales							**$0**	**$42**	**$89**	**$124**	**$151**	**$168**	**$177**

Total WW GLUT1 Sales						**$34**	**$165**	**$393**	**$586**	**$735**	**$836**	**$894**	**$944**

Source: Company Data, Morgan Stanley Research

Exhibit 15

LC-FAODs Market Model

US ($ in millions)	2013	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
FAOD Prevalent Pts	3,500	3,535	3,570	3,606	3,642	3,715	3,789	3,865	3,942	4,021	4,102	4,184	4,267
YoY Growth		1.0%	1.0%	1.0%	1.0%	2.0%	2.0%	2.0%	2.0%	2.0%	2.0%	2.0%	2.0%
FAOD Pts on 007													
Beginning yr pts						0	186	745	1,287	1,617	1,938	2,047	2,153
007 new additions						186	568	580	394	402	205	209	213
% of severe FAOD pts						5%	15%	15%	10%	10%	5%	5%	5%
Deaths/Discon						0	9	37	64	81	97	102	108
% of beginning yr pts						5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%
End of Yr Pts						186	745	1,287	1,617	1,938	2,047	2,153	2,259
Share of prevalent pts (EOY)						*5%*	*20%*	*33%*	*41%*	*48%*	*50%*	*51%*	*53%*
Cost per month						$9,375	$9,563	$9,754	$9,949	$10,148	$10,351	$10,558	$10,769
Price Increase YoY							2%	2%	2%	2%	2%	2%	2%
New pt duration of therapy (mo)						6	6	6	6	6	6	6	6
Existing pt duration of therapy (mo)						11	11	11	11	11	11	11	11
New pt revenue						10	33	34	24	24	13	13	14
Existing pt revenue						0	19	78	137	176	215	232	249
Total US 007 FAOD Sales						**$10**	**$52**	**$112**	**$161**	**$200**	**$228**	**$245**	**$263**

EU ($ in millions)	2013	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
FAOD Prevalent Pts	4,550	4,596	4,641	4,688	4,735	4,829	4,926	5,025	5,125	5,228	5,332	5,439	5,548
YoY Growth		1.0%	1.0%	1.0%	1.0%	2.0%	2.0%	2.0%	2.0%	2.0%	2.0%	2.0%	2.0%
FAOD Pts on 007													
Beginning yr pts						0	217	871	1,506	1,892	2,268	2,395	2,520
007 new additions						217	665	678	461	470	240	245	250
% of FAOD pts						5%	14%	14%	9%	9%	5%	5%	5%
Deaths/Discon						0	11	44	75	95	113	120	126
% of beginning yr pts						5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%
End of Yr Pts						217	871	1,506	1,892	2,268	2,395	2,520	2,643
Share of prevalent pts (EOY)						*5%*	*18%*	*30%*	*37%*	*43%*	*45%*	*46%*	*48%*
Cost per month						$8,438	$8,438	$8,438	$8,438	$8,438	$8,438	$8,438	$8,438
Price Increase YoY							0%	0%	0%	0%	0%	0%	0%
New pt duration of therapy (mo)						6	6	6	6	6	6	6	6
Existing pt duration of therapy (mo)						11	11	11	11	11	11	11	11
New pt revenue						11	34	34	23	24	12	12	13
Existing pt revenue						0	20	79	136	171	205	217	228
Total EU 007 FAOD Sales						**$11**	**$53**	**$113**	**$160**	**$195**	**$217**	**$229**	**$241**

ROW ($ in millions)	2013	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
FAOD Prevalent Pts	2,275	2,298	2,321	2,344	2,367	2,391	2,415	2,463	2,513	2,563	2,614	2,666	2,720
YoY Growth		1.0%	1.0%	1.0%	1.0%	1.0%	1.0%	2.0%	2.0%	2.0%	2.0%	2.0%	2.0%
FAOD Pts on 007													
Beginning yr pts								0	100	400	691	869	1,041
007 new additions								100	305	311	212	216	110
% of FAOD pts								4%	12%	12%	8%	8%	4%
Deaths/Discon								0	5	20	35	43	52
% of beginning yr pts								5%	5%	5%	5%	5%	5%
End of Yr Pts								100	400	691	869	1,041	1,099
Share of prevalent pts (EOY)								*4%*	*16%*	*27%*	*33%*	*39%*	*40%*
Cost per month								$8,438	$8,438	$8,438	$8,438	$8,438	$8,438
Price Increase YoY									0%	0%	0%	0%	0%
New pt duration of therapy (mo)								6	6	6	6	6	6
Existing pt duration of therapy (mo)								11	11	11	11	11	11
New pt revenue								5	15	16	11	11	6
Existing pt revenue								0	9	36	63	79	94
Total ROW 007 FAOD Sales								**$5**	**$24**	**$52**	**$73**	**$90**	**$100**
Total WW FAOD Sales						**$21**	**$105**	**$230**	**$345**	**$448**	**$519**	**$564**	**$603**

Source: Company Data, Morgan Stanley Research

KRN23 (UX023) for X-Linked Hypophosphatemia

We see significant commercial potential for KRN23, a subcutaneous monthly or every other week recombinant monoclonal antibody that binds to FGF23. FGF23 levels are elevated in patients with X-linked hypophosphatemia, a disorder of inappropriate phosphate and vitamin D control that yields severe bone manifestations (Ex. 16). This drug is partnered with Kyowa Hakka Kirin (KHK) in a commercial relationship described below.

Exhibit 16
XLH Disease Summary

XLH (X-Linked Hypophosphatemia)	
Disease	Low serum phosphate levels result in poor bone mineralization
Cause	Loss of function of PHEX gene (phosphate regulating gene) → elevated circulating FGF23 (fibroblast growth factor) → excessive renal phosphate wasting → low serum phosphate and active Vit D levels
Prevalence	3,000 pediatric and 9,000 adult patients in the US
Symptoms	Rickets, bowed legs, short stature, fractures, pain, muscle weakness
Current Treatments	Oral phosphate replacement and active vitamin D therapy (calcitriol)
Diagnosis	Clinical assessment, urine and blood tests

Source: Company Data, Morgan Stanley Research

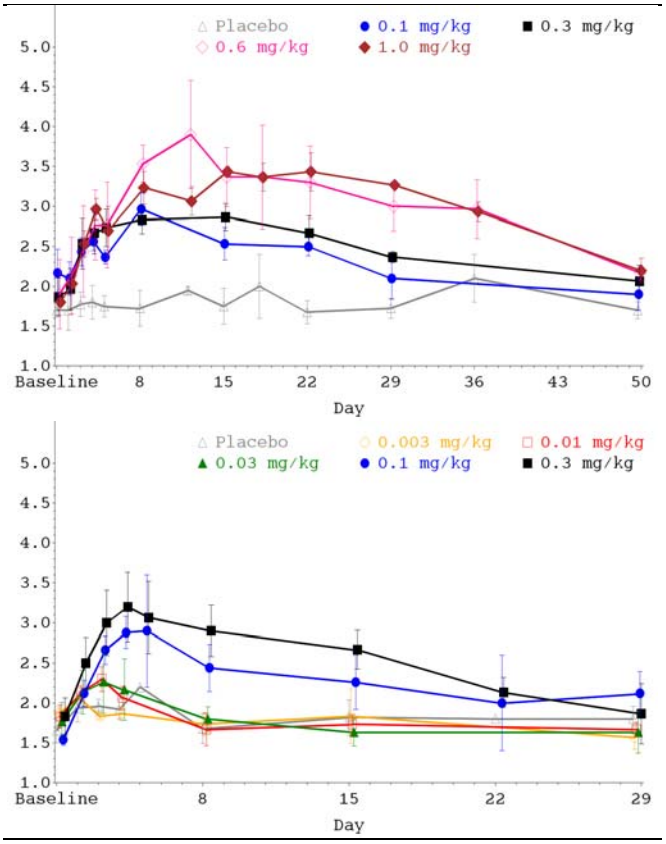
Below we address in more detail:
1) Ph 1 single dose **efficacy data** in XLH patients showing improvements on key biochemical endpoints,
2) **Safety data**, which is early but lacks any major issues,
3) The **next steps** for children and adult XLH patients, and
4) The ~$1.4bn **commercial opportunity** for adult and pediatric XLH patients.

1) Efficacy Data

A Ph 1 single dose trial in 38 adult XLH patients was presented at the ASBMR conference in 2013. Patients received various doses of either IV (0.003-0.3mg/kg) or subcutaneous (0.1-1 mg/kg) KRN23 vs. either IV or subcutaneous placebo in a 3:1 drug:placebo randomization.

KRN23 appeared to meaningfully improve serum phosphate levels (Ex. 17) and Vit D levels (Ex. 18). Though doses were not clearly separate in these charts for the two highest doses (0.6 mg/kg and 1 mg/kg), it appears to us that in general there is a dose response for the higher doses vs. the lowest doses. The lack of clear separation at the two highest doses may be due to small patient numbers or a peaking of the efficacy.

Exhibit 17
Serum Phosphate (mg/dL) Levels Increased Following A Single KRN23 Dose
Top is subcutaneous doses; bottom is IV doses

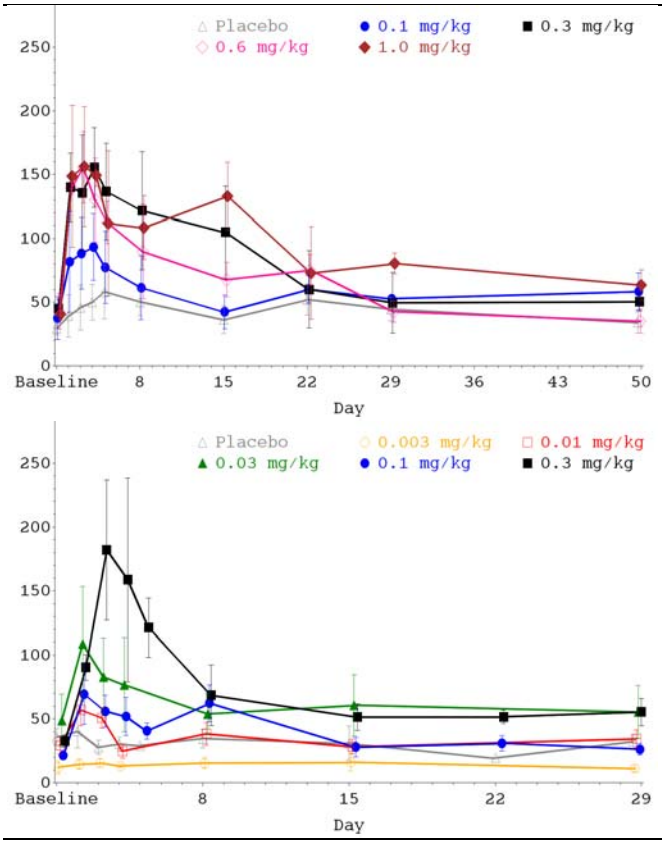


Source: Company Data, Morgan Stanley Research

KRN23 also increased active vitamin D levels in XLH patients. While the benefits in general showed a good dose response, there was some time point/dose variability. We believe this is most likely due to small numbers of pts and a heterogeneous metric over time after only a single dose of the drug. Importantly, the upward trend in vitamin D is what was expected and observed. We see repeat dosing and more pts as likely drivers to smooth out these curves.

Importantly, we believe both of these biochemical changes a) are what was expected of the drug, b) help solidify the link between FGF23 and the disease manifestations, and c) are likely to lead to a rebuilding of bone given the fundamental role adequate phosphate and vitamin D play in bone growth/stability.

Exhibit 18
Active Vitamin D (pg/mL) Improved Following A Single KRN23 Dose
Top is subcutaneous doses; bottom is IV doses



Source: Company Data, Morgan Stanley Research

2) Safety Data

Safety data have looked mostly clean to us, though data has been single dose only and in small patient numbers. In the single dose trial, two patients had elevated serum amylase levels and two different patients had back pain. While the serum amylase safety data bears watching given concerns around pancreatitis, we are comforted by the fact that a) one patient had elevated serum amylase at baseline and the other only had a mild elevation in serum amylase, and b**) the pts with back pain were not the same pts that had elevated serum amylase.** There were no hypersensitivity/infusion reactions. We will be watching for signals such as "off target" impacts of FGF23 inhibition (we do not expect any) and anti-drug antibodies with repeat dosing.

3) Path Forward

Ultragenyx management reviewed multi-dose Ph 1/2 data in adult pts prior to partnering with KHK, but these data have yet to be disclosed. We expect to see these data in 2014 at a medical meeting, and hope they can provide additional insight into dosing frequency, efficacy, and safety.

Ultragenyx intends to initiate a Ph 2 pediatric study in 2H14 with data expected in 2H15. An adult Ph 2b study is likely to initiate in parallel with the Ph 3 pediatric study in 2015.

One key investor question is what primary endpoint will be used to anchor the Ph 3 clinical program. We expect that endpoints measuring bone growth/structure/mineralization over time, such as radiographs, will be useful on top of clinical symptom data.

4) Market Opportunity

We see $1.4bn peak (2025) sales of KRN23 for XLH. Below, we address:
a) XLH disease severity for the children and adult markets,
b) Current standard of care, and
c) Economics with Kyowa Hakko Kirin and the XLH market potential.

a) XLH Disease Severity

The disease phenotype in children may vary, but it is often severe with rickets, bowed legs, short stature, fractures, pain, and muscle weakness. Adults may be less severe than children, with some mild patients only experiencing minor aches and pains. We believe that there is an addressable market of severe adults where KRN23 would be useful pending solid bone benefits.

b) Standard of Care

There is no approved treatment for the underlying cause of XLH. Patients are treated with oral phosphate replacement and active vitamin D therapy (calcitriol). Children are treated until growth is complete while adults are typically only treated if they need symptomatic relief to decrease pain.

We do not view this current standard of care as ideal for two reasons. First, the side effects of chronic, sometimes high dose, active Vitamin D/oral phosphate replacement include increased risk of hyperparathyroidism and nephrocalcinosis (calcification in the kidney). Both of these problems have their own serious sequelae. Second, and related to the problems of aggressive repletion of phosphate and vitamin D, the current goal of treatment is to improve clinical endpoints decently well. This gentler, but clinically sometimes suboptimal, approach is designed to minimize harmful Vit D/oral phosphate side effects. **We believe a therapy that could more physiologically normalize phosphate levels would be advantageous over a therapy with incomplete disease control.**

c) Economics and Market Potential

Ultragenyx has a development and commercial partnership with Kyowa Hakko Kirin (KHK). In the US, this agreement takes the form of a profit split that switches to a mid-high 20%

royalty after 5 years. In the EU, Ultragenyx has a 10% royalty. In Latin America, Ultragenyx retains full rights and pays KHK a low single digit royalty on net sales.

Our $520mn of peak (2025) US KRN23 sales for XLH assumes:
1) a 2018 launch,
2) ~3k children and ~9k adult XLH patients,
3) ~65% penetration into the market, and
4) a $180k annual net price at launch.

Our $580mn of peak (2025) EU KRN23 sales for XLH assumes:
1) a 2018 launch,
2) ~5k children and ~14k adult XLH patients,
3) ~60% penetration into the market, and
4) a ~$160k annual net price at launch.

Our $260mn of peak (2025) ROW KRN23 sales for XLH assumes:
1) a 2019 launch,
2) ~2k children and ~7k adult XLH patients,
3) ~55% penetration into the market, and
4) a ~$160k annual net price at launch.

Exhibit 19

XLH Market Model

US ($ in millions)	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
XLH patients	12,000	12,120	12,241	12,364	12,487	12,612	12,738	12,866	12,994	13,124	13,255	13,388	13,522
XLH patients - children	3,000	3,030	3,060	3,091	3,122	3,153	3,185	3,216	3,249	3,281	3,314	3,347	3,380
YoY growth		1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%
XLH patients - adults	9,000	9,090	9,181	9,273	9,365	9,459	9,554	9,649	9,746	9,843	9,942	10,041	10,141
YoY growth		1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%
Addressable XLH patients	3,750	3,788	3,825	3,864	3,902	3,941	3,981	4,021	4,061	4,101	4,142	4,184	4,226
Addressable XLH patients - children	2,400	2,424	2,448	2,473	2,497	2,522	2,548	2,573	2,599	2,625	2,651	2,678	2,704
YoY growth	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%
Addressable XLH patients - adults	1,350	1,364	1,377	1,391	1,405	1,419	1,433	1,447	1,462	1,476	1,491	1,506	1,521
YoY growth	15%	15%	15%	15%	15%	15%	15%	15%	15%	15%	15%	15%	15%
Beginning yr pts						0	394	1,370	1,904	2,215	2,514	2,596	2,675
KRN23 new additions						394	995	603	406	410	207	209	211
KRN23 new additions - children						252	637	386	260	262	133	134	135
% of addressable XLH patients - children						10%	25%	15%	10%	10%	5%	5%	5%
KRN23 new additions - adults						142	358	217	146	148	75	75	76
% of addressable XLH patients - adults						10.0%	25.0%	15.0%	10.0%	10.0%	5.0%	5.0%	5.0%
Deaths/Discon						0	20	68	95	111	126	130	134
% of chronic 003 treated						5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%
End of Yr Pts						394	1,370	1,904	2,215	2,514	2,596	2,675	2,753
End of Yr share (% addressable XLH patients)						10%	34%	47%	55%	61%	63%	64%	65%
Cost per month						$15,000	$15,300	$15,606	$15,918	$16,236	$16,561	$16,892	$17,230
Price Increase YoY							2%	2%	2%	2%	2%	2%	2%
New pt duration of therapy (mo)						6	6	6	6	6	6	6	6
Existing pt duration of therapy (mo)						11	11	11	11	11	11	11	11
New pt revenue						35	91	56	39	40	21	21	22
Existing pt revenue						0	65	229	325	386	447	470	494
Total US KRN23 Sales						**$35**	**$156**	**$286**	**$364**	**$426**	**$467**	**$491**	**$516**
Commercial Supply Cost													
COGS + markup						7	31	57	73	85	93	98	103
% of sales						20%	20%	20%	20%	20%	20%	20%	20%
Total SG&A for US KRN23						33	36	40	40	41	42	43	44
KHK Sales Force Cost						16	16	17	17	18	18	19	20
# reps						50	50	50	50	50	50	50	50
Cost/rep						0.32	0.33	0.34	0.35	0.36	0.37	0.38	0.39
Ultragenyx Sales Force Cost						7	10	13	13	13	14	14	15
% of sales force attributable to KRN23						30%	30%	30%	30%	30%	30%	30%	30%
Marketing Cost						10	10	10	10	10	10	10	10
Profit Split													
KRN23 Profit						-5	89	189	251	299	331	350	369
KRN23 Profit to Ultragenyx during Profit Share						-2	44	95	125	150	166	175	184
Royalty Agreement													
KRN23 Royalty to Ultragenyx during Royalty Agreement						9	42	79	102	121	133	140	148
Royalty Rate						26%	27%	28%	28%	28%	29%	29%	29%
KRN23 Profit Share/Royalty to Ultragenyx						**-$2**	**$44**	**$95**	**$125**	**$150**	**$133**	**$140**	**$148**
% of US sales						-7%	28%	33%	34%	35%	29%	29%	29%

EU ($ in millions)	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
XLH patients	19,200	19,392	19,586	19,782	19,980	20,179	20,381	20,585	20,791	20,999	21,209	21,421	21,635
XLH patients - children	4,800	4,848	4,896	4,945	4,995	5,045	5,095	5,146	5,198	5,250	5,302	5,355	5,409
YoY growth		1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%
XLH patients - adults	14,400	14,544	14,689	14,836	14,985	15,135	15,286	15,439	15,593	15,749	15,907	16,066	16,226
YoY growth		1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%
Addressable XLH patients	6,000	6,060	6,121	6,182	6,244	6,306	6,369	6,433	6,497	6,562	6,628	6,694	6,761
Addressable XLH patients - children	3,840	3,878	3,917	3,956	3,996	4,036	4,076	4,117	4,158	4,200	4,242	4,284	4,327
YoY growth	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%
Addressable XLH patients - adults	2,160	2,182	2,203	2,225	2,248	2,270	2,293	2,316	2,339	2,362	2,386	2,410	2,434
YoY growth	15%	15%	15%	15%	15%	15%	15%	15%	15%	15%	15%	15%	15%
Beginning yr pts						0	568	1,972	2,742	3,190	3,621	3,738	3,852
KRN23 new additions						568	1433	868	585	591	298	301	304
KRN23 new additions - children						363	917	556	374	378	191	193	195
% of addressable XLH patients - children						9%	23%	14%	9%	9%	5%	5%	5%
KRN23 new additions - adults						204	516	313	211	213	107	108	110
% of addressable XLH patients - adults						9%	23%	14%	9%	9%	5%	5%	5%
Deaths/Discon						0	28	99	137	159	181	187	193
% of chronic 003 treated						5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%
End of Yr Pts						568	1,972	2,742	3,190	3,621	3,738	3,852	3,964
End of Yr share (% addressable XLH patients)						9%	31%	43%	49%	55%	56%	58%	59%
Cost per month						$13,500	$13,500	$13,500	$13,500	$13,500	$13,500	$13,500	$13,500
Price Increase YoY							0%	0%	0%	0%	0%	0%	0%
New pt duration of therapy (mo)						6	6	6	6	6	6	6	6
Existing pt duration of therapy (mo)						11	11	11	11	11	11	11	11
New pt revenue						46	116	70	47	48	24	24	25
Existing pt revenue						0	82	286	397	462	524	541	558
Total EU KRN23 Sales						**$46**	**$198**	**$356**	**$444**	**$510**	**$548**	**$566**	**$582**
Total EU KRN23 Revenue to Ultragenyx						**$5**	**$20**	**$36**	**$44**	**$51**	**$55**	**$57**	**$58**
Royalty Rate						10%	10%	10%	10%	10%	10%	10%	10%

ROW ($ in millions)	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
XLH patients	9,600	9,696	9,793	9,891	9,990	10,090	10,191	10,292	10,395	10,499	10,604	10,710	10,818
XLH patients - children	2,400	2,424	2,448	2,473	2,497	2,522	2,548	2,573	2,599	2,625	2,651	2,678	2,704
YoY growth		1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%
XLH patients - adults	7,200	7,272	7,345	7,418	7,492	7,567	7,643	7,719	7,797	7,875	7,953	8,033	8,113
YoY growth		1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%
Addressable XLH patients	3,000	3,030	3,060	3,091	3,122	3,153	3,185	3,216	3,249	3,281	3,314	3,347	3,380
Addressable XLH patients - children	1,920	1,939	1,959	1,978	1,998	2,018	2,038	2,058	2,079	2,100	2,121	2,142	2,164
YoY growth	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%
Addressable XLH patients - adults	1,080	1,091	1,102	1,113	1,124	1,135	1,146	1,158	1,169	1,181	1,193	1,205	1,217
YoY growth	15%	15%	15%	15%	15%	15%	15%	15%	15%	15%	15%	15%	15%
Beginning yr pts							0	258	896	1,246	1,450	1,646	1,714
KRN23 new additions							258	651	395	266	268	151	152
KRN23 new additions - children							165	417	253	170	172	96	97
% of addressable XLH patients - children							8%	20%	12%	8%	8%	5%	5%
KRN23 new additions - adults							93	234	142	96	97	54	55
% of addressable XLH patients - adults							8.1%	20.3%	12.2%	8.1%	8.1%	4.5%	4.5%
Deaths/Discon							0	13	45	62	72	82	86
% of chronic 003 treated							5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%
End of Yr Pts							258	896	1,246	1,450	1,646	1,714	1,780
End of Yr share (% addressable XLH patients)							8%	28%	38%	44%	50%	51%	53%
Cost per month							$13,500	$13,500	$13,500	$13,500	$13,500	$13,500	$13,500
Price Increase YoY								0%	0%	0%	0%	0%	0%
New pt duration of therapy (mo)							6	6	6	6	6	6	6
Existing pt duration of therapy (mo)							11	11	11	11	11	11	11
New pt revenue							21	53	32	22	22	12	12
Existing pt revenue							0	37	130	180	210	238	248
Total ROW KRN23 Sales							**$21**	**$90**	**$162**	**$202**	**$232**	**$250**	**$260**
COGS + markup							4	18	32	40	46	50	52
% of sales							20%	20%	20%	20%	20%	20%	20%
Total WW Sales						**$81**	**$375**	**$732**	**$970**	**$1,137**	**$1,247**	**$1,308**	**$1,359**
Total WW Revenue to Ultragenyx						**$2**	**$85**	**$220**	**$332**	**$403**	**$420**	**$447**	**$467**

Source: Company Data, Morgan Stanley Research

rhGUS (UX003) for MPS 7

We view rhGUS for MPS 7 as a smaller commercial opportunity vs. triheptanoin and KRN23, but one where Ultragenyx should ultimately have success. rhGUS is an IV enzyme replacement therapy of beta-glucuronidase (GUS). MPS 7 is one of the rarer mucopolysaccharidosises with severe symptoms, similar to some other MPS's with meaningful systemic symptoms (Ex. 20).

Exhibit 20

MPS 7 Disease Summary

MPS (Mucopolysaccharidosis) 7 / Sly Syndrome	
Disease	Mucopolysaccharidosis with severe organ dysfunction
Cause	Deficiency of lysosomal enzyme beta-glucuronidase (GUS) → poor breakdown of GAG (glycosaminoglycans) → GAG accumulation in tissues → multi-system organ dysfunction
Prevalence	Potentially ~200 patients WW
Patients Identified by RARE	~90 patients so far (15 in the US)
Symptoms	Lung/respiratory complications, enlargement of liver and spleen, heart valve disease, restricted mobility, coarsened facial features, short stature, abnormal skeletal features (dysostosis multiplex), hydrops fetalis in some patients
Current Treatments	No approved therapies; bone marrow/stem cell transplants have been considered
Diagnosis	Clinical presentation; uGAG urine test; enzyme activity measurement in blood or skin cells

Source: Company Data, Morgan Stanley Research

Below we address in more detail:
1) **Data** from a single MPS 7 patient and mice,
2) The **path forward** for approval in the US and EU, and
3) The $70+mn **commercial opportunity** for MPS 7 patients.

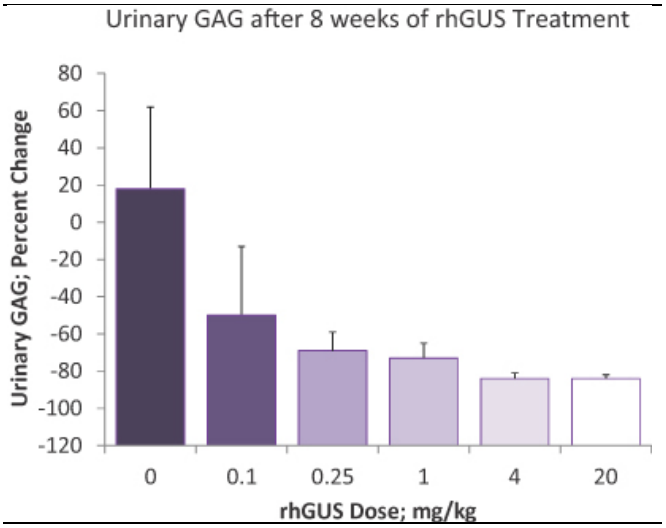
1) Data

Positive anecdotal data were presented recently at the LDN WORLD meeting (Lysosomal Disease Network) for a single MPS patient receiving drug under an emergency IND. After being treated for 14 weeks, the patient showed an improvement of pulmonary function and no infusion associated reactions.

Other data for rhGUS are pre-clinical. The drug has been studied in mice, with data showing dose-dependent urinary GAG reductions after 8 weeks (Ex. 21).

Exhibit 21

rhGUS Lowered Urinary GAG Levels in MPS 7 Mice



Source: Company Data, Morgan Stanley Research

Though we have minimal data, we are confident in rhGUS success as 1) rhGUS is an enzyme replacement therapy, 2) enzyme replacement therapies for other MPS's with similar systemic manifestations have worked, and 3) the animal data demonstrates the type of biochemical benefit that will be sought in humans.

2) Path Forward

rhGUS is currently in a Ph 1/2 trial in 5 MPS 7 patients aged 5-30 years. Management expects interim 12 week data 1H14. An extension trial with possible dose titration will continue in these patients and read-out in 2H14.

Ph 3 initiation (n=12 MPS 7 patients) will be gated by the Ph 1/2 12 week data and could begin as early as 2H14. The primary endpoint will be urinary GAG levels and secondary endpoints may include 6 minute walk test, pulmonary function, height and weight growth velocity, and other functional metrics. Per management's discussions with regulators, the EMEA is OK with urinary GAG as a primary endpoint as long as pts also show some directional benefit on clinical endpoints. The FDA would like to see additional correlation of GAG levels with other clinical endpoints, which we think could happen in Ph 1/2.

While the number of patients in the clinical program is small, we are confident that this will not be a hurdle for approval. There were <50 patients serving as the basis for approval for Aldurazyme for MPS I and Naglazyme for MPS IV. As MPS 7 has the smallest prevalence of the MPS's, 17 patients from the Ph 1/2 and 3 programs should be sufficient.

Further clinical development for this drug is likely. Ultragenyx intends to study rhGUS in MPS 7 patients <5 years old, including patients with hydrops fetalis. We view the hydrops fetalis opportunity as very interesting given that ~20 patients/year, or potentially half of the disease incidence of MPS 7, present at birth with this condition which is often fatal. If rhGUS shows efficacy here, we see the disease's prevalence increasing stepwise over time, making this disease more commercially valuable as pt lifespans increase on the drug.

3) Market Opportunity

We see ~$75mn peak (2025) rhGUS sales for MPS 7. Below, we address:
a) Disease severity and,
b) Economics and market opportunity.

a) Disease Severity

MPS 7 patients exhibit similar symptoms to other MPS's. Some patients present at birth with hydrops fetalis (abnormal amount of fluid buildup). More broadly for MPS 7 pts, major symptoms include lung/respiratory problems, enlargement of the liver and spleen, heart valve disease, restricted mobility, coarsened facial features, short stature, and abnormal skeletal features (dysostosis multiplex). Patients typically die in their teens to thirties.

There are no approved treatments for MPS 7, though bone marrow/stem cell transplants have been considered (and are used for similar diseases).

b) Economics and Market Opportunity

Ultragenyx licensed WW rights to rhGUS from St. Louis University and pays them a low-single digit royalty.

Our $30mn of peak (2025) US rhGUS sales for MPS 7 assumes:
1) a 2017 launch,
2) ~70 patients,
3) ~80% penetration into the market, and
4) a ~$400k annual net price.

Our $50mn of peak (2025) ex-US rhGUS sales for MPS 7 assumes:
1) a 2017 launch,
2) ~130 patients,
3) ~80% penetration into the market, and
4) a ~$400k annual price.

Exhibit 22

MPS 7 Market Model

US ($ in millions)	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
MPS 7 Sly Syndrome patients	67	67	68	69	70	71	73	74	76	77	79	80	82
YoY growth		1%	1%	1%	2%	2%	2%	2%	2%	2%	2%	2%	2%
Beginning yr pts					0	4	25	45	54	59	60	61	62
003 new additions					4	21	22	11	8	4	4	4	4
% of MPS 7 Sly pts					5%	30%	30%	15%	10%	5%	5%	5%	5%
Deaths/Discon					0.0	0.2	1.2	2.3	2.7	3.0	3.0	3.0	3.1
% of chronic 003 treated					5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%
End of Yr Pts					4	25	45	54	59	60	61	62	63
End of Yr share (% MPS 7 Sly pts)					5%	35%	62%	73%	78%	78%	77%	77%	77%
Cost per month					$33,750	$34,425	$35,114	$35,816	$36,532	$37,263	$38,008	$38,768	$39,544
Price Increase YoY					2%	2%	2%	2%	2%	2%	2%	2%	2%
New pt duration of therapy (mo)					6	6	6	6	6	6	6	6	6
Existing pt duration of therapy (mo)					11	11	11	11	11	11	11	11	11
New pt revenue					1	4	5	2	2	1	1	1	1
Existing pt revenue					0	1	9	17	21	24	24	25	26
Total US 003 MPS 7 Sly Sales					**$1**	**$6**	**$14**	**$20**	**$23**	**$25**	**$25**	**$26**	**$27**

ROW ($ in millions)	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
MPS 7 Sly Syndrome patients	133	135	136	137	140	143	146	149	152	155	158	161	164
YoY growth		1%	1%	1%	2%	2%	2%	2%	2%	2%	2%	2%	2%
Beginning yr pts					0	7	50	91	109	118	120	122	124
003 new additions					7	43	44	22	15	8	8	8	8
% of MPS 7 Sly pts					5%	30%	30%	15%	10%	5%	5%	5%	5%
Deaths/Discon					0.0	0.4	2.5	4.5	5.4	5.9	6.0	6.1	6.2
% of chronic 003 treated					5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%
End of Yr Pts					7	50	91	109	118	120	122	124	126
End of Yr share (% MPS 7 Sly pts)					5%	35%	62%	73%	78%	78%	77%	77%	77%
Cost per month					$33,750	$33,750	$33,750	$33,750	$33,750	$33,750	$33,750	$33,750	$33,750
Price Increase YoY						0%	0%	0%	0%	0%	0%	0%	0%
New pt duration of therapy (mo)					6	6	6	6	6	6	6	6	6
Existing pt duration of therapy (mo)					11	11	11	11	11	11	11	11	11
New pt revenue					1	9	9	5	3	2	2	2	2
Existing pt revenue					0	3	18	33	39	43	43	44	45
Total ROW 003 MPS 7 Sly Sales					**$1**	**$11**	**$27**	**$37**	**$42**	**$44**	**$45**	**$46**	**$47**
Total WW 003 MPS 7 Sly Sales					**$2**	**$17**	**$41**	**$57**	**$65**	**$69**	**$70**	**$72**	**$74**

Source: Company Data, Morgan Stanley Research

Extended Release (ER) Sialic Acid (UX001) for Hereditary Inclusion Body Myopathy

We do not model ER sialic acid in our base case given modest data so far, but see an interesting commercial opportunity if the higher dose data show robust benefit. ER sialic acid is an extended release oral formulation of sialic acid. It is intended to replace sialic acid in patients with HIBM, a severe neuromuscular disease, who are deficient in this metabolite (Ex. 23).

Exhibit 23
HIBM Disease Summary

HIBM (Hereditary Inclusion Body Myopathy / GNE Myopathy)	
Disease	Neuromuscular disease characterized by severe muscle weakness
Cause	Autosomal recessive defect in gene that encodes sialic acid biosynthesis → sialic acid deficiency
Prevalence	300-400 patients in the US; 1,200-2,000 patients WW
Patients Identified by RARE	>300 patients in the US and >800 patients WW
Symptoms	Severe muscle weakness
Current Treatments	No approved therapy
Diagnosis	Clinical presentation, muscle biopsy, genetic confirmation

Source: Company Data, Morgan Stanley Research

Below we focus on:
1) Ph 2 **efficacy data** which may be suggestive of some drug activity,
2) The **path forward** including testing at higher doses, and
3) The ~$160mn **commercial potential** if successful (we reserve sales for our bull case only).

1) Efficacy Data

A Ph 2 trial of 46 HIBM patients given either ER sialic acid at doses of 3 or 6 grams/day or placebo (placebo patients randomized to drug at 24 weeks) showed modest improvements in some endpoints at 48 weeks, but overall did not show consistent benefits. The upper extremity muscle strength composite showed a modest increase at 48 weeks at the 6 grams/day dose, particularly in patients with less severe ambulation at baseline. Ultragenyx intends to present the 48 week data at a medical conference in 2014.

Company comments suggest that the drug was well tolerated, with mostly GI tolerability issues and no SAEs in the trial. We look forward to updates when the full data set is released.

2) Path Forward

Management is hoping that increasing the ER sialic acid dose will allow for more consistent and statistically significant clinical improvements. An ongoing Ph 2 extension study is testing higher doses of ER sialic acid. Data from this study will be available in 2H14 and is likely to gate development.

Ultragenyx is also looking at pre-clinical prodrugs of sialic acid with potentially better penetration into muscle tissue. This may be a good strategy for Ultragenyx to pursue if higher doses of ER sialic acid do not produce the intended benefit.

3) Market Opportunity

We believe that, if successful (currently part of our bull case only), sialic acid may achieve $160mn peak (2025) WW sales for HIBM. Below, we address:
a) Disease severity,
b) Competition, and
c) Economics and market opportunity.

a) Disease Severity

HIBM is a slowly progressive disease with severe muscle weakness that often spares the quadriceps (thigh) muscle. Disease onset is in the late teens to early 20s, and patients are often wheelchair bound within ~20 years after onset.

Diagnosis is often made via clinical presentation and muscle biopsy. The disease can also be genetically confirmed.

b) Competition

There are no approved treatments for the underlying disease. Current therapy focuses on supportive care.

New Zealand Pharma has a compound, DEX-M74 or ManNAc, in development for HIBM as well. The drug is a metabolite in the sialic acid pathway that may have different distribution and uptake properties. A Ph 1 study conducted by the NIH has been completed, but results have yet to be released, to the best of our knowledge.

c) Economics and Market Opportunity

Ultragenyx licensed WW rights (excluding Japan and certain other Asian territories) from Nobelpharma. Ultragenyx pays a high single digit royalty to Nobelpharma on net sales in Ultragenyx territories while Nobelpharma pays Ultragenyx a mid-single digit royalty on net sales in their territories (ex-Japan).

If data are compelling (bull case only), we see ~$160mn of peak (2025) WW sales.

Our $40mn of peak (2025) US sales (bull case only) assumes:
1) a 2017 launch,
2) ~350 patients,
3) ~60% penetration into the market, and
4) a $180k annual price.

Our $120mn of peak (2025) ex-US sales (bull case only) assumes:
1) a 2017 launch,
2) ~1.25k patients,
3) ~50% penetration into the market, and
4) a $180k annual price.

Exhibit 24

HIBM Market Model (Bull Case Only)

US ($ in millions)	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
HIBM patients	350	354	357	361	364	368	372	375	379	383	387	390	394
YoY growth		1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%
Beginning yr pts					0	0	37	109	160	190	200	209	218
001 new additions					0	37	74	56	38	19	19	20	20
% of HIBM pts					0%	10%	20%	15%	10%	5%	5%	5%	5%
Deaths/Discon					0.0	0.0	1.8	5.5	8.0	9.5	10.0	10.4	10.9
% of beginning yr 001 treated					5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%
End of Yr Pts					0	37	109	160	190	200	209	218	227
End of Yr share (% HIBM pts)					0%	10%	29%	43%	50%	52%	54%	56%	58%
Cost per month					$15,000	$15,300	$15,606	$15,918	$16,236	$16,561	$16,892	$17,230	$17,575
Price Increase YoY					0%	2%	2%	2%	2%	2%	2%	2%	2%
New pt duration of therapy (mo)					6	6	6	6	6	6	6	6	6
Existing pt duration of therapy (mo)					11	11	11	11	11	11	11	11	11
New pt revenue					0	3	7	5	4	2	2	2	2
Existing pt revenue					0	0	6	19	28	34	36	39	41
Total US 001 HIBM Sales					**$0**	**$3**	**$13**	**$24**	**$32**	**$36**	**$38**	**$41**	**$43**

ROW ($ in millions)	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
HIBM patients	1,250	1,263	1,275	1,288	1,301	1,314	1,327	1,340	1,354	1,367	1,381	1,395	1,409
YoY growth		1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%
Beginning yr pts					0	0	118	351	515	611	642	672	701
001 new additions					0	118	239	181	122	62	62	63	63
% of HIBM pts					0%	9%	18%	14%	9%	5%	5%	5%	5%
Deaths/Discon					0.0	0.0	5.9	17.6	25.7	30.5	32.1	33.6	35.0
% of beginning yr 001 treated					5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%	5.0%
End of Yr Pts					0	118	351	515	611	642	672	701	729
End of Yr share (% HIBM pts)					0%	9%	26%	38%	45%	47%	49%	50%	52%
Cost per month					$15,000	$15,000	$15,000	$15,000	$15,000	$15,000	$15,000	$15,000	$15,000
Price Increase YoY						0%	0%	0%	0%	0%	0%	0%	0%
New pt duration of therapy (mo)					6	6	6	6	6	6	6	6	6
Existing pt duration of therapy (mo)					11	11	11	11	11	11	11	11	11
New pt revenue					0	11	21	16	11	6	6	6	6
Existing pt revenue					0	0	19	56	83	98	103	108	113
Total ROW 001 HIBM Sales					**$0**	**$11**	**$41**	**$73**	**$94**	**$104**	**$109**	**$114**	**$118**
Total WW 001 HIBM Sales					**$0**	**$14**	**$54**	**$97**	**$125**	**$139**	**$147**	**$154**	**$162**

Source: Company Data, Morgan Stanley Research

Exhibit 25

Annual Income Statement

($ in millions)	2011	2012	2013E	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	$124.3	$268.3	$385.0	$478.6	$543.0	$582.6	$622.9
EU sales			$0.0	$0.0	$0.0	$0.0	$0.0	$30.2	$145.6	$308.1	$433.4	$528.0	$587.2	$617.6	$647.3
ROW sales			$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$47.0	$113.1	$176.4	$224.6	$257.6	$276.4
Total Triheptanoin (UX007) Sales			**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$55.4**	**$269.9**	**$623.4**	**$931.4**	**$1,182.9**	**$1,354.8**	**$1,457.8**	**$1,546.6**
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	$0.0	$0.0	$46.0	$198.3	$355.9	$444.4	$509.7	$548.4	$565.6	$582.4
ROW Sales			$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$20.9	$90.1	$161.8	$202.0	$231.6	$250.5	$260.5
Total KRN23 Sales			**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$81.4**	**$375.2**	**$731.7**	**$970.0**	**$1,137.3**	**$1,247.2**	**$1,307.6**	**$1,359.1**
US Revenue to Ultragenyx			$0.0	$0.0	$0.0	$0.0	$0.0	-$2.3	$44.3	$94.5	$125.3	$149.6	$133.2	$140.4	$147.9
EU Revenue to Ultragenyx			$0.0	$0.0	$0.0	$0.0	$0.0	$4.6	$19.8	$35.6	$44.4	$51.0	$54.8	$56.6	$58.2
ROW Revenue to Ultragenyx			$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$20.9	$90.1	$161.8	$202.0	$231.6	$250.5	$260.5
Total KRN23 Revenue to Ultragenyx			**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$2.2**	**$85.0**	**$220.2**	**$331.5**	**$402.5**	**$419.6**	**$447.5**	**$466.6**
Total Revenue	$0	$0	$0	$0	$0	$0	$2	$75	$396	$901	$1,328	$1,654	$1,845	$1,977	$2,087
Cost of Sales				$0	$0	$0	$0	$14	$66	$155	$209	$263	$300	$322	$340
% total sales				*10%*	*10%*	*10%*	*15%*	*15%*	*15%*	*15%*	*13%*	*13%*	*13%*	*13%*	*13%*
R&D	$5	$13	$27	$41	$52	$54	$57	$60	$63	$66	$69	$71	$72	$73	$75
YoY growth		*168%*	*111%*	*55%*	*25%*	*5%*	*5%*	*5%*	*5%*	*5%*	*5%*	*2%*	*2%*	*2%*	*2%*
% of revenue							*2673%*	*80%*	*16%*	*7%*	*5%*	*4%*	*4%*	*4%*	*4%*
SG&A	$2	$3	$6	$12	$18	$33	$62	$84	$101	$119	$127	$130	$134	$137	$141
YoY growth		*81%*	*83%*	*100%*	*50%*	*82%*	*85%*	*36%*	*20%*	*18%*	*7%*	*3%*	*3%*	*3%*	*3%*
% of revenue							*2908%*	*113%*	*26%*	*13%*	*10%*	*8%*	*7%*	*7%*	*7%*
Total Operating Expenses	$6.6	$16.0	$33	$54	$70	$88	$119	$158	$230	$340	$405	$464	$505	$533	$556
Operating Income (Loss)	-$7	-$16	-$33	-$54	-$70	-$88	-$117	-$83	$166	$561	$923	$1,191	$1,339	$1,445	$1,531
Operating Margin							*-5498%*	*-112%*	*42%*	*62%*	*69%*	*72%*	*73%*	*73%*	*73%*
Interest income	$0.0	$0.0	$0.2	$0.3	$0.3	$0.2	$0.3	$0.3	$0.4	$1.3	$3.0	$5.3	$8.1	$11.2	$14.6
Interest expense and other	-$0.3	-$0.4	-$1.7	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0
Pretax Income (Loss)	($7)	($16)	($34)	($53)	($70)	($87)	($117)	($83)	$166	$562	$926	$1,196	$1,348	$1,456	$1,546
Provision for Income Taxes	$0.00	$0	$0	$0	$0	$0	$0	$0	$0	$68	$250	$323	$364	$393	$417
Effective Tax Rate			0%	0%	0%	0%	0.0%	0%	0%	12%	27%	27%	27%	27%	27%
Net Income (Loss)	($6.8)	($16.3)	($34.2)	($53.2)	($69.7)	($87.5)	($116.7)	($83.1)	$166.3	$494.8	$675.9	$873.1	$983.7	$1,062.7	$1,128.3
EPS, basic	($1.35)	($3.78)	($7.90)	($1.78)	($2.32)	($2.83)	($3.51)	($2.39)	$4.77	$14.15	$19.28	$24.84	$27.92	$30.08	$31.86
EPS, diluted	($1.35)	($3.78)	($7.90)	($1.78)	($2.32)	($2.83)	($3.51)	($2.39)	$4.71	$14.01	$19.13	$24.72	$27.78	$29.94	$31.70
Options Expense				$4	$6	$8	$9	$10	$11	$12	$13	$14	$15	$16	$17
% of operating expense			*0.0%*	*7.5%*	*8.6%*	*9.1%*	*7.6%*	*6.3%*	*4.8%*	*3.5%*	*3.2%*	*3.0%*	*3.0%*	*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)	($16.3)	($34.2)	($57.2)	($75.7)	($95.5)	($125.7)	($93.1)	$155.3	$484.3	$666.4	$862.9	$972.8	$1,051.0	$1,115.9
GAAP EPS, diluted (incl. ESOs)	($1.35)	($3.78)	($7.90)	($1.91)	($2.52)	($3.09)	($3.78)	($2.68)	$4.40	$13.71	$18.87	$24.43	$27.47	$29.61	$31.35
Basic Shares Outstanding	5.07	4.32	4.33	29.96	30.04	30.87	33.21	34.80	34.88	34.97	35.06	35.15	35.24	35.33	35.41
Diluted Shares Outstanding	5.07	4.32	4.33	29.96	30.04	30.87	33.21	34.80	35.32	35.32	35.32	35.32	35.41	35.50	35.59

Source: Company Data, Morgan Stanley Research

Exhibit 26

Balance Sheet

($ in millions)	2011	2012	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
Assets															
Cash and cash equivalents	$10.6	$86	$53	$128	$58	$62	$148	$59	$197	$648	$1,283	$2,128	$3,099	$4,157	$5,286
Marketable securities	$0.0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0
Receivables due from related party	$0.1	$0	$0	$0	$0	$0	$0	$4	$16	$36	$53	$66	$74	$79	$83
Prepaid expenses and other current assets	$0.2	$0	$0	$0	$0	$0	$0	$1	$8	$18	$27	$33	$37	$40	$42
Total current assets	**$11.0**	**$86**	**$53**	**$128**	**$58**	**$62**	**$148**	**$65**	**$221**	**$702**	**$1,362**	**$2,227**	**$3,210**	**$4,276**	**$5,411**
Property, plant and equipment, net	$0.76	$1.4	$3	$5	$8	$11	$14	$17	$21	$27	$31	$33	$33	$31	$25
Restricted cash	$0.4	$0.5	$0.5	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0
Other assets	$0	$0	$0	$0	$0	$0	$0	$1	$4	$9	$13	$17	$18	$20	$21
Inventory	$0	$0.0	$0	$0	$0	$0	$0	$4	$20	$45	$66	$83	$92	$99	$104
Total assets	**$12.129**	**$88.3**	**$56.3**	**$133.4**	**$66.8**	**$73.5**	**$162.4**	**$86.7**	**$266.5**	**$782.9**	**$1,473.4**	**$2,360.0**	**$3,354.5**	**$4,425.6**	**$5,561.7**
Liabilities and stockholders' equity															
Accounts payable	$0.3	$1	$2	$3	$3	$4	$6	$8	$11	$17	$20	$23	$25	$27	$28
Accrued liabilities	$0.7	$2	$4	$6	$8	$10	$14	$19	$27	$41	$49	$55	$60	$64	$67
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**	**$9**	**$12**	**$15**	**$20**	**$27**	**$39**	**$58**	**$69**	**$79**	**$86**	**$90**	**$94**
Convertible preferred stock warrant liability	$0.22	$1	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0
Other liabilities	$0.27	$0	$1	$1	$1	$1	$2	$3	$4	$6	$7	$8	$9	$9	$9
Total liabilities	**$1.49**	**$4.0**	**$6**	**$10**	**$13**	**$16**	**$22**	**$29**	**$43**	**$63**	**$76**	**$86**	**$94**	**$99**	**$104**
Series A redeemable convert preferred stock	$19	$37	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0
Series B redeemable convert preferred stock	$0	$74	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0
Common stock	$0.01	$0.01	$0.01	$0.01	$0.01	$0.01	$0.01	$0.01	$0.01	$0.01	$0.01	$0.01	$0.01	$0.01	$0.01
APIC	$0	$0	$111	$242	$248	$347	$556	$566	$577	$588	$600	$613	$627	$642	$658
Accumulated deficit	($8)	($27)	($61)	($118)	($194)	($290)	($415)	($508)	($353)	$131	$797	$1,660	$2,633	$3,684	$4,800
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**	**$50**	**$123**	**$54**	**$57**	**$140**	**$57**	**$224**	**$719**	**$1,398**	**$2,274**	**$3,260**	**$4,326**	**$5,458**
Total liabilities and stockholder's equity	**-$6.48**	**-$23**	**$56**	**$133**	**$67**	**$73**	**$162**	**$87**	**$266**	**$783**	**$1,473**	**$2,360**	**$3,354**	**$4,426**	**$5,562**

Source: Company Data, Morgan Stanley Research

Exhibit 27

Cash Flow Statement

($ in millions)	2011	2012	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
CASH FLOWS FROM OPERATING ACTIVITIES															
Net loss	($7)	($16)	($34)	($57)	($76)	($95)	($126)	($93)	$155	$484	$666	$863	$973	$1,051	$1,116
Depreciation and amortization	$0	$0.3	$0.5	$0.8	$1.1	$1.5	$2.0	$2.6	$3.3	$4.3	$5.2	$6.1	$6.7	$7.1	$7.3
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
Employee stock-based compensation	$0	$0.9	$0.0	$4.0	$6.0	$8.0	$9.0	$10.0	$11.0	$10.6	$9.5	$10.2	$11.0	$11.7	$12.4
Revaluation of convertible preferred stock warrant liability	$0	$0.3	$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
Tax benefit from stock based compensation	$0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.9	$2.3	$2.5	$2.8	$3.0	$3.3
Changes in operating assets and liabilities:															
Receiveable - related party	($0)	$0	$0	$0	$0	$0	($0)	($4)	($12)	($20)	($17)	($13)	($8)	($5)	($4)
Prepaid expenses and other current assets	($0)	($0)	$0	$0	$0	$0	($0)	($1)	($6)	($10)	($9)	($7)	($4)	($3)	($2)
Other assets	($0)	$0	$0	$0	$0	$0	($0)	($1)	($3)	($5)	($4)	($3)	($2)	($1)	($1)
Inventory	$0	$0	$0	$0	$0	$0	($0)	($4)	($16)	($25)	($21)	($16)	($10)	($7)	($5)
Accounts payable	$0	$1	$0	$1	$1	$1	$2	$2	$4	$6	$3	$3	$2	$1	$1
Accrued expenses and other liabilities	$0	$2	$2	$3	$2	$2	$4	$5	$10	$15	$9	$8	$6	$4	$3
Net cash used in operating activities	**($5.8)**	**($12.5)**	**($31.3)**	**($48.6)**	**($65.5)**	**($82.6)**	**($109.1)**	**($82.7)**	**$145.2**	**$459.9**	**$644.3**	**$853.4**	**$978.2**	**$1,062.1**	**$1,130.1**
CASH FLOWS FROM INVESTING ACTIVITIES															
Purchases of property, plant and equipment	($0.55)	($1.1)	($2.1)	($3.1)	($3.7)	($4.2)	($5.2)	($6.0)	($7.6)	($9.6)	($9.4)	($8.5)	($6.7)	($4.4)	($1.8)
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	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0
Sales and maturities of marketable securities	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0
Net cash used in investing activities	**($0.9)**	**($1.2)**	**($2.1)**	**($3.1)**	**($3.7)**	**($4.2)**	**($5.2)**	**($6.0)**	**($7.6)**	**($9.6)**	**($9.4)**	**($8.5)**	**($6.7)**	**($4.4)**	**($1.8)**
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	$126	$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**	**$0.0**	**$126.5**	**$0.1**	**$90.8**	**$199.7**	**$0.1**	**$0.1**	**$0.1**	**$0.1**	**$0.2**	**$0.2**	**$0.2**	**$0.2**

Source: Company Data, Morgan Stanley Research

Company Description

Ultragenyx is a biopharmaceutical company focused on orphan drug development. Their programs include triheptanoin for long-chained fatty acid oxidation disorders (LC-FAOD) and glucose transporter type-1 deficiency syndrome (Glut-1 DS), KRN23 for X-linked hypophosphatemia (XLH), rhGUS for MPS 7, sialic acid-extended release for hereditary inclusion body myopathy (HIBM), and rhPPCA for galactosialidosis.

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(as of January 31, 2014)

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Underweight/Sell	**543**	**18%**	**96**	**12%**	**18%**
Total	**2,973**		**825**		

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Industry Coverage:Biotechnology

Company (Ticker)	Rating (as of)	Price* (02/21/2014)
David Friedman, M.D.		
Ultragenyx Pharmaceutical Inc (RARE.O)	O (02/25/2014)	$59.1
AMAG Pharmaceuticals, Inc. (AMAG.O)	E (11/21/2011)	$21.35
Alexion Pharmaceuticals (ALXN.O)	O (09/07/2010)	$181.52
Alnylam Pharmaceuticals (ALNY.O)	E (01/14/2014)	$90.64
Auxilium Pharmaceuticals (AUXL.O)	E (05/03/2013)	$28.76
Chimerix Inc (CMRX.O)	O (05/06/2013)	$19.8
Cubist Pharmaceuticals Inc. (CBST.O)	O (11/13/2013)	$78.19
Idenix Pharmaceuticals, Inc. (IDIX.O)	E (03/18/2011)	$7
Incyte Corporation (INCY.O)	U (01/23/2013)	$64.98
InterMune (ITMN.O)	E (09/07/2010)	$13.77
Ironwood Pharmaceuticals, Inc. (IRWD.O)	E (04/24/2013)	$15.01
Lexicon Pharmaceuticals, Inc. (LXRX.O)	U (06/11/2013)	$1.92
NPS Pharmaceuticals (NPSP.O)	O (10/03/2012)	$38.57
Neurocrine Biosciences Inc (NBIX.O)	E (01/08/2014)	$18.29
Ophthotech Corp (OPHT.O)	O- (10/21/2013)	$35.64
Portola Pharmaceuticals Inc (PTLA.O)	O (06/17/2013)	$26.85
Relypsa, Inc. (RLYP.O)	O (12/10/2013)	$40.01
Synageva Biopharma Corp (GEVA.O)	O (04/20/2012)	$109.92
Tesaro Inc. (TSRO.O)	E (02/04/2014)	$32.28
Theravance Inc (THRX.O)	U (07/22/2013)	$39.29
Vertex Pharmaceuticals (VRTX.O)	E (05/08/2012)	$84.81
XenoPort Inc (XNPT.O)	U (06/11/2013)	$6.02

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