Ultragenyx

A RARE Opportunity in the Orphan Space…Initiating at Overweight

Initiation
Overweight

RARE, RARE US
Price: $58.01

Price Target: $66.00

We are initiating coverage of RARE with an OW rating and YE14 target of $66. With a broad and diverse pipeline of orphan disease assets and a highly regarded management team with a strong track record in the space, we believe Ultragenyx is poised for success. This apparently isn't going unnoticed, with the shares up 176% from its IPO. RARE is led by CEO, Dr. Emil Kakkis (former CMO of BMRN), who has a demonstrable reputation and, more importantly, strong relationships with rare disease researchers/KOLs globally. The company has five product candidates focused on orphan indications with a high unmet medical need, which provides both diversification and increased probability of ultimate success, in our view.

- **Portfolio of rare disease compounds provides a diversified opportunity for exposure to the orphan disease space.** RARE expects to have four compounds for five unique indications in Phase 2 development by early 2014. Although no single product stands out as the *key* asset, in our view, we believe the pipeline in total is well positioned with the potential to ultimately generate >$1B in total net revenue. In addition, we believe RARE's strategy of in-licensing transformative therapies with existing data and clear mechanisms of action should continue to fuel a deep and compelling new product pipeline with attractive economics.

- **RARE's management team has a solid track record in developing and commercializing rare disease therapies.** Dr. Emil Kakkis, founder and CEO, was instrumental in building BioMarin into a multi-product commercial stage company, bringing several orphan drugs through development to commercialization. RARE also has a deep and impressive bench that enhances the company's clinical, regulatory, and commercial capabilities.

- **Updates from several programs in 2014 offer potential value creation events.** Phase 1/2 data for KRN23 in XLH and rhGUS in MPS 7 are expected in mid-2014, and Phase 2 extension study data for SA-ER in HIBM should come by YE14. Phase 2 triheptanoin data in LC-FAOD and Glut1 DS are expected in 2015.

- **Initiating at OW with a probability-adjusted price target of $66.** Our target is based on an average of rNPV and SOTP scenario models and reflects a 65% probability of success for KRN23 (~$250M in peak economics to RARE), 75% probability for rhGUS (~$70M peak), 45% probability for triheptanoin in both FAOD and Glut1 DS (~$300M and $850M peak sales, respectively), and 30% probability for SA-ER (~$125M peak). We also believe upside to RARE's valuation is supported by a comparable company analysis.

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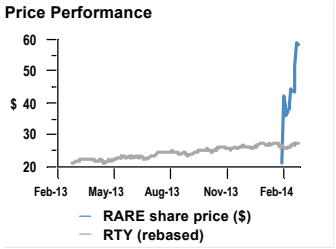
Price Performance



	YTD	1m	3m	12m
Abs	176.2 %	176.2 %	176.2 %	176.2 %
Rel	174.1 %	173.5 %	171.8 %	145.1 %

Ultragenyx Pharmaceutical (RARE;RARE US)

FYE Dec	2013E	2014E	2015E
EPS Reported ($)			
Q1 (Mar)	-	0.00	0.00
Q2 (Jun)	-	0.00	0.00
Q3 (Sep)	-	0.00	0.00
Q4 (Dec)	-	0.00	0.00
FY	(1.22)	(1.60)	(1.82)

Source: Company data, Bloomberg, J.P. Morgan estimates.

Company Data	
Price ($)	58.01
Date Of Price	24-Feb-14
52-week Range ($)	62.48-35.15
Market Cap ($ mn)	1,715.44
Fiscal Year End	Dec
Shares O/S (mn)	30
Price Target ($)	66.00
Price Target End Date	31-Dec-14

See page 49 for analyst certification and important disclosures.

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Investment Thesis

Ultragenyx Pharmaceutical (RARE)

Overweight

Ultragenyx is an emerging orphan disease company with a deep and broad clinical stage pipeline

The orphan disease space has been an area of intense investor focus over the past few years, which is unlikely to change anytime soon, in our view. Ultragenyx, with a complete dedication to rare and ultra-rare diseases, stands to benefit from this trend. Orphan disease products are often associated with high unmet medical need, accelerated development timelines, regulatory and commercial incentives, limited competition, premium pricing, and favorable reimbursement. While only founded in 2010, RARE has already amassed (via in-licensing) a broad pipeline of 5 compounds focused on 6 different debilitating conditions. RARE expects 4 of these programs (targeting 5 indications) to be in Phase 2 development by early 2014.

RARE's management team is highly regarded in both the medical and investment communities

RARE was founded by Dr. Emil Kakkis, the former Chief Medical Officer of BioMarin, who was instrumental in the approval of many of that company's core products; he brings with him valuable relationships within the industry, with advocacy groups and regulators. Beyond Dr. Kakkis, Ultragenyx employs a seasoned group that has gained impressive experience at a number of other orphan-focused companies. In addition to their deep knowledge of metabolic and orphan diseases, the team also has strong connections to key academic leaders/researchers, which has enabled RARE to in-license multiple compounds over a short time period for modest upfront and near-term milestone payments. We also believe management's reputation within the investment community is noteworthy and appears to have quickly built positive sentiment for RARE's story.

The diversification of Ultragenyx's portfolio not only provides broad exposure to the orphan disease space but also lessens reliance on the success of any one product

While many small orphan companies are focused on the development of 1 or maybe 2 products, Ultragenyx expects 4 products for 5 distinct indications to be in Phase 2 trials in 2014. Its portfolio is also diversified from a cost of goods/manufacturing perspective, with both biologic and small molecule product candidates. Additionally, we believe Ultragenyx's focus on diseases with clear biology further de-risks the pipeline candidates, as a clear understanding of the disease and therapeutics aimed at treating the underlying pathology has proven a successful strategy in the past (e.g., ERTs in the various MPS indications).

Of the five clinical-stage pipeline programs, we believe KRN23 and rhGUS have the highest probability of success

KRN23 (UX023) is an anti-FGF23 monoclonal antibody for the treatment of XLH that has the potential, in our view, to become a >$800M product (pre economic split). RARE formed a collaboration with Kyowa Hakko Kirin (KHK, not covered) in August 2013 to jointly develop and commercialize KRN23 for XLH. We look forward to data from Phase 1/2 studies in mid-2014 and anticipate promising results (*RARE reviewed data prior to signing its collaboration agreement with KHK*). In addition to KRN23, we view rhGUS for MPS 7 as an important contributor to RARE's pipeline, albeit for different reasons. We expect rhGUS to be RARE's first

product to market (2017 launch), and although peak sales will likely be modest (~$70M peak estimate), we think rhGUS will be important in establishing infrastructure and a commercial footprint that can be leveraged for other products.

Several data readouts this year could provide de-risking events and investment catalysts

The first clinical catalysts are expected around mid-2014, with Phase 1/2 data for KRN23 in XLH and rhGUS in MPS 7. The Phase 2 extension study data for SA-ER in HIBM should readout by YE14. In addition, there should be a number of trial initiations this year, including a Phase 2 pediatric trial of KRN23 in XLH and a Phase 3 study of rhGUS in MPS 7. RARE expects data from the recently initiated Phase 2 studies of triheptanoin in both LC-FAOD and Glut1 DS in 2015.

RARE's lean structure and relatively light expenses could provide a rapid path to profitability once commercial

RARE doesn't have in-house early-stage drug discovery programs, but rather relies on in-licensing preclinical and clinical stage assets to build its pipeline. RARE fully outsources its clinical trials and manufacturing, eliminating the need to build a sizeable infrastructure to support these functions. In addition, RARE's focus on compressed clinical development timelines could prove to be not only time-efficient, but cost-efficient as well. Once commercial, the company will likely have a relatively small sales and marketing footprint – another perk of the orphan business model – that could enable a relatively quick path to profitability.

Balance sheet check: Ultragenyx is relatively well positioned

As of the end of 3Q13, Ultragenyx had a cash balance of $64M and no debt. Combining this solid cash position with ~$126M in net proceeds from the recent IPO offering (for which J.P. Morgan acted as joint book runner) should provide Ultragenyx with sufficient capital to fund operations through 2015.

We are initiating coverage of RARE with an OW rating and probability-adjusted YE14 target of $66

We believe RARE's strong management team, orphan disease focus, and diversified pipeline offer a compelling story. Additionally, RARE's strategy of growth through business development rather than in-house discovery and focus on orphan indications with small, relatively shorter trials could lead to profitability within a few years of first commercial launch. This is an important differentiating factor, in our view. Our target is based on a blended average of our risk-adjusted NPV model and our proprietary sum-of-the-parts scenario analysis. We assume a 65% probability of success for KRN23 (~$250M in peak economics to RARE), 75% probability for rhGUS (~$70M peak sales), 45% probability for triheptanoin in both FAOD and Glut1 DS (~$300M and $850M peak sales, respectively), and 30% probability for SA-ER ($125M peak). In our opinion, upside to RARE's prevailing valuation is further supported by a comp analysis of other orphan-focused companies.

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Risks to Rating and Price Target

Ultragenyx is susceptible to the standard risks that apply to the entire biotech industry, including development, regulatory, commercial, manufacturing, financing, and IP pitfalls. Risks more specific to Ultragenyx are outlined below:

Clinical risk

Although Ultragenyx has a diverse pipeline, each product has limited data to date. There is the risk that pre-clinical data has little read-through to clinical safety/efficacy. There is also the risk that serious adverse events could emerge with any one of RARE's products. We note that the lack of a common thread between the products (disparate products not derived from similar platforms/technologies) helps to contain the risk given that a safety issue with one product doesn't have read-through to the pipeline.

Development/regulatory risk

Ultragenyx is developing products for orphan diseases, where clinical trials and the regulatory path are not standardized. Additionally, Ultragenyx intends to implement some novel/non-traditional trial designs for some products that have yet to be run by or agreed to by the FDA. There is a risk that the FDA will not sign off on these trials, and timelines to launch could be extended if longer and/or larger trials must be run.

Commercial risk

The rate of uptake by physicians may be slower than expected for Ultragenyx's products, if approved. The globally scattered nature of the relatively small target patient populations could pose unexpected difficulty for Ultragenyx, potentially limiting market uptake and sales. Although orphan disease products command premium pricing in today's market, this could change over the next several years if payors become more cost-conscious.

Personnel risk

In our opinion, CEO Emil Kakkis is critical to the initial success and longer-term outlook for Ultragenyx. If he were to leave the company for any reason, we suspect shares could come under significant pressure.

Company Description

Ultragenyx is a developmental stage biotechnology company committed to bringing life-enhancing therapeutics for patients with rare and ultra-rare genetic diseases to market. The company focuses on metabolic and rare diseases that may affect small numbers of patients, but for which the medical need is high and there are no effective treatments. Ultragenyx' therapeutic portfolio is focused on a variety of orphan diseases and consists of three biologics and two small molecules. The company was founded in 2010 by Emil Kakkis, the former Chief Medical Officer at BioMarin, and is located in Novato, California.

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Upcoming Events

Several key data readouts in 2014 could be meaningful de-risking events for three different products.

- **The most notable near-term catalyst, in our view, is Phase 1/2 data for KRN23 in adult XLH patients in mid-2014.** After positive Phase 1 dose escalation (single and repeat dose) data were released in Oct. 2013, Ultragenyx plans to report Phase 1/2 (extension study) repeat dose data in ~23 patients who have been treated with SubQ KRN23.

- **RARE also expects to announce Phase 1/2 data for rhGUS in MPS 7 in mid-2014.** Ultragenyx initiated a Phase 1/2 trial in late 2013. The open-label trial will enroll 5 MPS7 patients, and interim data from a 12-week primary analysis phase is expected in mid-2014.

- **Data from SA-ER's extension study in HIBM is expected by the end of 2014.** An open-label extension study is ongoing, evaluating SA-ER in ~56 adult HIBM patients. The extension study data presented will evaluate a higher dose of SA-ER that was used in the Phase 2 portion of the trial.

- In 2015, Phase 2 interim data for triheptanoin in both FAOD and Glut1 DS is expected, as well as data from the Phase 2 trial for KRN23 in pediatric XLH patients.

Figure 1: RARE Upcoming Events

Program	Event	Expected Timing	Significance
UX023 (KRN23)	Data from Phase 1/2 studies in adult XLH patients	mid-2014	High
	Initiate Phase 2 trial in pediatic XLH patients	2014	Low
	Initiate adult XLH Phase 2b study	2015	Low
	Data from Phase 2 trial in pediatic XLH patients	2015	High
UX003 (rhGUS)	Report data from Phase 1/2 studies in MPS7	mid-2014	Medium
	Initiate pivotal Phase 3 study in MPS 7	2014	Low
UX007 (Triheptanoin)	Interim data from Phase 2 trial in LC-FAOD	2015	High
UX007G (Triheptanoin)	Interim data from Phase 2 trial in Glut1	2015	High
UX001 (SA-ER)	Initiate higher dosage testing in Phase 2 extension study	Early 2014	Low
	Data from extension study	YE14	Medium

Source: Company reports.

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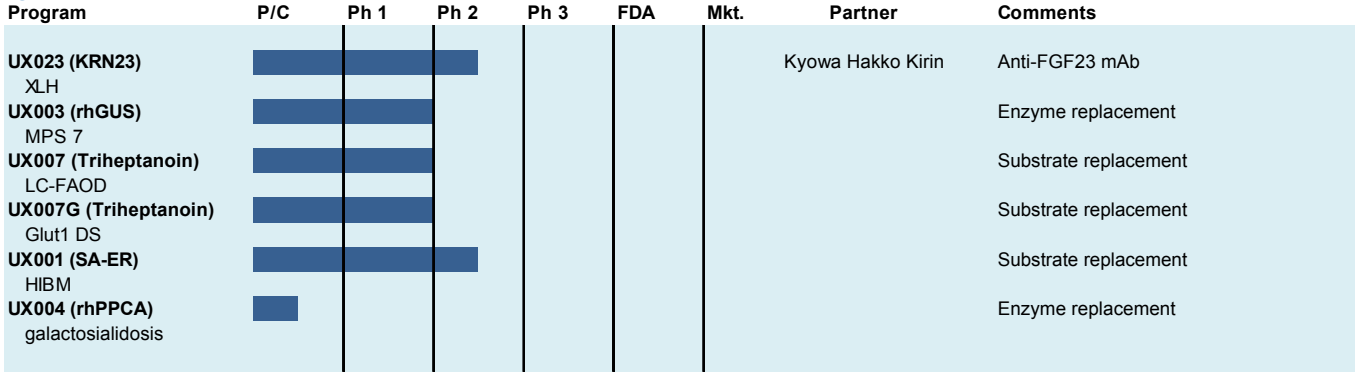
Pipeline Overview

Figure 2: RARE's Pipeline



Source: Company reports.

Ultragenyx currently has 5 products for 6 indications in its product pipeline. For a small company, its plate is pretty full. However, with an underlying strategy based on its business development capabilities, we suspect that this pipeline has the potential to look very different a few years down the road. In other words, future value drivers could change quickly based on the company's BD activities. The current clinical products in Ultragenyx's pipeline include:

- **KRN23 (UX023).** KRN23 is a monoclonal antibody designed to bind and reduce the activity of FGF23 to increase abnormally low phosphate levels in patients with XLH. Data from a single dose Phase 1 study was encouraging, in our view, with signs of increasing serum phosphate levels and reduced urinary excretion of phosphate. KRN23 also appears to be well tolerated. Phase 1/2 studies in adult XLH patients are underway, with data anticipated in mid-2014. RARE plans on initiating a Phase 2 pediatric study this year. Pediatrics could present a large market opportunity, as pediatric patients with XLH have the highest morbidity and thus potential to benefit. KRN23 for XLH is the most recently licensed program and is partnered with Kyowa Hakko Kirin Co (KHK) in the US, EU, and Latin America. KRN23 is the largest value driver, in our view, with roughly $1B in peak sales potential.

- **rhGUS (UX003).** rhGUS is an enzyme replacement therapy for the treatment of MPS 7, a multi-system disease in which patients suffer from severe cellular and organ dysfunction. rhGUS is currently being evaluated in an open-label Phase 1/2 trial in 4-5 MPS 7 patients between the ages of 5 and 30. Interim data for this Phase 1/2 study is expected in mid-2014, and a pivotal trial is set to begin later this year, if results are supportive. We expect rhGUS for MPS 7 to be the first product approved, and although it's a small market opportunity, in our view (we project peak sales of ~$70M), it could lay the groundwork for a global commercial footprint. This established infrastructure could be leveraged as the other products come to market.

- **Triheptanoin (UX007).** Triheptanoin is a substrate replacement therapy being evaluated in both LC-FAOD and Glut1 DS, significant metabolism disorders in which patients have energy deficiency in their muscles and brain, respectively. Ultragenyx recently initiated two Phase 2 studies, with interim data for both indications expected in 2015. We project a wide sales range potential (~$300M to >$1bn) for triheptanoin, depending on whether the LC-FAOD and/or Glut1 DS indications gain approval.

- **SA-ER (UX001).** SA-ER is an extended release, oral substrate replacement therapy of sialic acid for the treatment of HIBM, a disease characterized by severe muscular myopathy. We're taking a "wait-and-see" approach to SA-ER, as the initial Phase 2 data did not show much activity at the dose levels evaluated to date. However, Ultragenyx is currently conducting an extension study with a higher dose, and we look forward to data from this study in late 2014 for signs of efficacy while maintaining the good tolerability observed in the initial stages of the Phase 2 trial. We view SA-ER as a free call option at this point.

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KRN23 for XLH

KRN23 is an antibody that binds to FGF23, blocking its activity and increasing levels of phosphate in the blood.

Product Overview

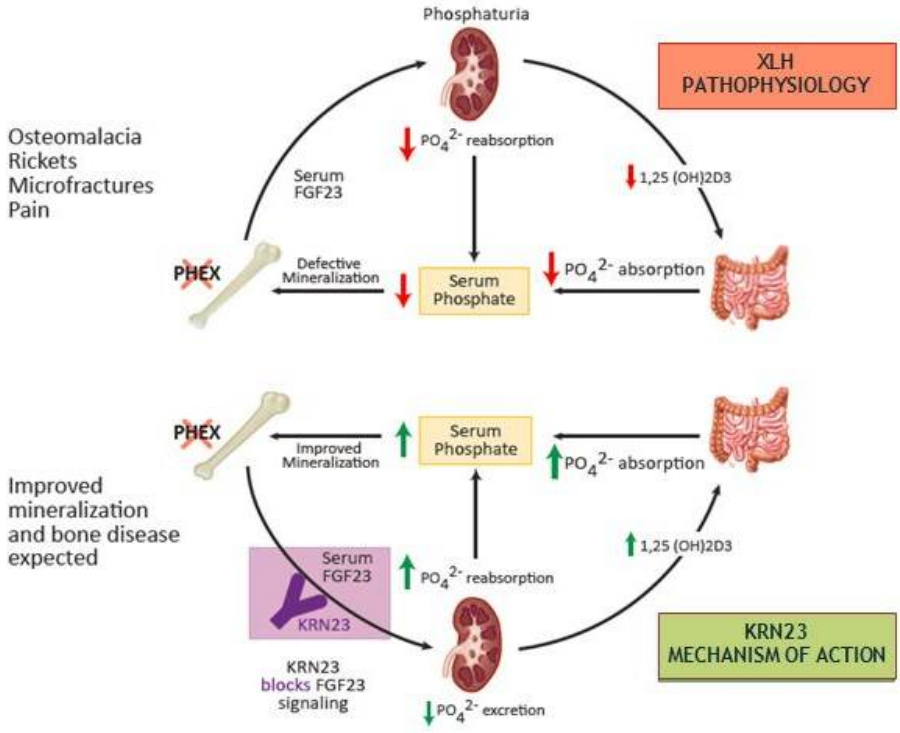
UX023 (KRN23) is a fully humanized monoclonal antibody that binds to and reduces the activity of fibroblast growth factor 23 (FGF23), a hormone that plays a key role in phosphate homeostasis. High levels of FGF23 cause over-excretion of phosphate in the urine, which leads to abnormally low levels of phosphorus in the blood (hypophosphatemia). KRN23 is administered via SubQ injection, and works by binding to FGF23 and interfering with its signaling mechanism, which prevents phosphate wasting and increases the amount of serum phosphorus. KRN23 is being developed in collaboration with Kyowa Hakko Kirin Co., Ltd. (KHK).

Patients with XLH have abnormally low levels of serum phosphate, or hyophosphatemia, which is caused by excess FGF23.

Disease Background

X-Linked Hypophosphatemia (XLH) is an inherited metabolic disease caused by mutations of the metalloendopeptidase PHEX (**P**hosphate regulating gene with **H**omology to **E**ndopeptidases located on the **X** chromosome). Loss of function of this peptidase is thought to result in more uncleaved full length FGF23 in the blood. Excess FGF23 impacts phosphate excretion; patients with XLH have low levels of serum phosphate due to over-excretion of phosphate in the urine. FGF23, a bone derived hormone, increases phosphate excretion in two ways: 1) reduces expression of a sodium phosphate co-transporter in the kidney, which reduces renal reabsorption, and 2) reduces intestinal absorption by reducing active vitamin D production.

Figure 1: Pathophysiology of XLH and Mechanism of Action of KRN23



Source: Company reports.

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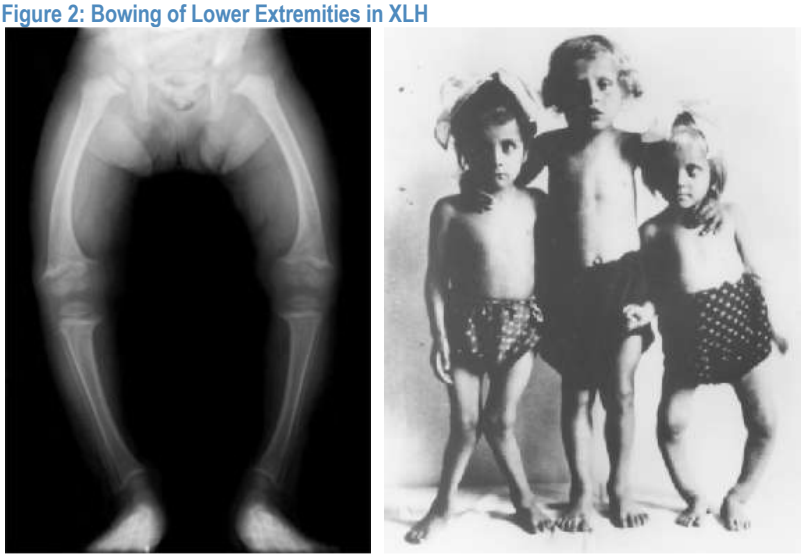
Chronic hypophosphatemia leads to a variety of symptoms, including skeletal effects and muscle weakness.

Low serum phosphate leads to a variety of complications, including poor bone mineralization, skeletal deformity, bone pain, motor impairment, short stature, muscle weakness, and lower than normal bone density.

Figure 2: Bowing of Lower Extremities in XLH



Source: Griffiths et al. 2002.

Clinical presentation of XLH ranges from isolated hypohosphatemia to severe lower extremity bowing. XLH typically presents in the first two years of life, when lower extremity bowing manifests as a result of weight bearing, or in less severe cases adults are diagnosed with previously unevaluated short stature. In adult patients, calcification of the tendons, ligaments and joint capsules may also be the presenting symptom. In XLH, poor bone mineralization also renders patients more prone to spontaneous dental abscesses, and sensorineural hearing loss has been reported. Diagnosis is typically based on clinical findings, radiographic findings, biochemical testing, and family history.

There is currently no approved drug to treat XLH. Pediatric patients take high does of oral phosphate and vitamin D with the hopes of improving bone pain and correcting bone deformations.

There is currently no approved therapy for the treatment of the underlying cause of XLH, and instead pharmacologic treatment focuses on improving pain and correcting bone deformation. The current standard of care includes oral phosphate replacement and vitamin D therapy, which is typically administered at high doses 3-5 times daily. In children, treatment typically begins at diagnosis and is continued until the growth of the long bones is complete. In adults, the benefit of these treatments has not been well studied, and treatment is generally reserved for those with symptoms.

Data Review

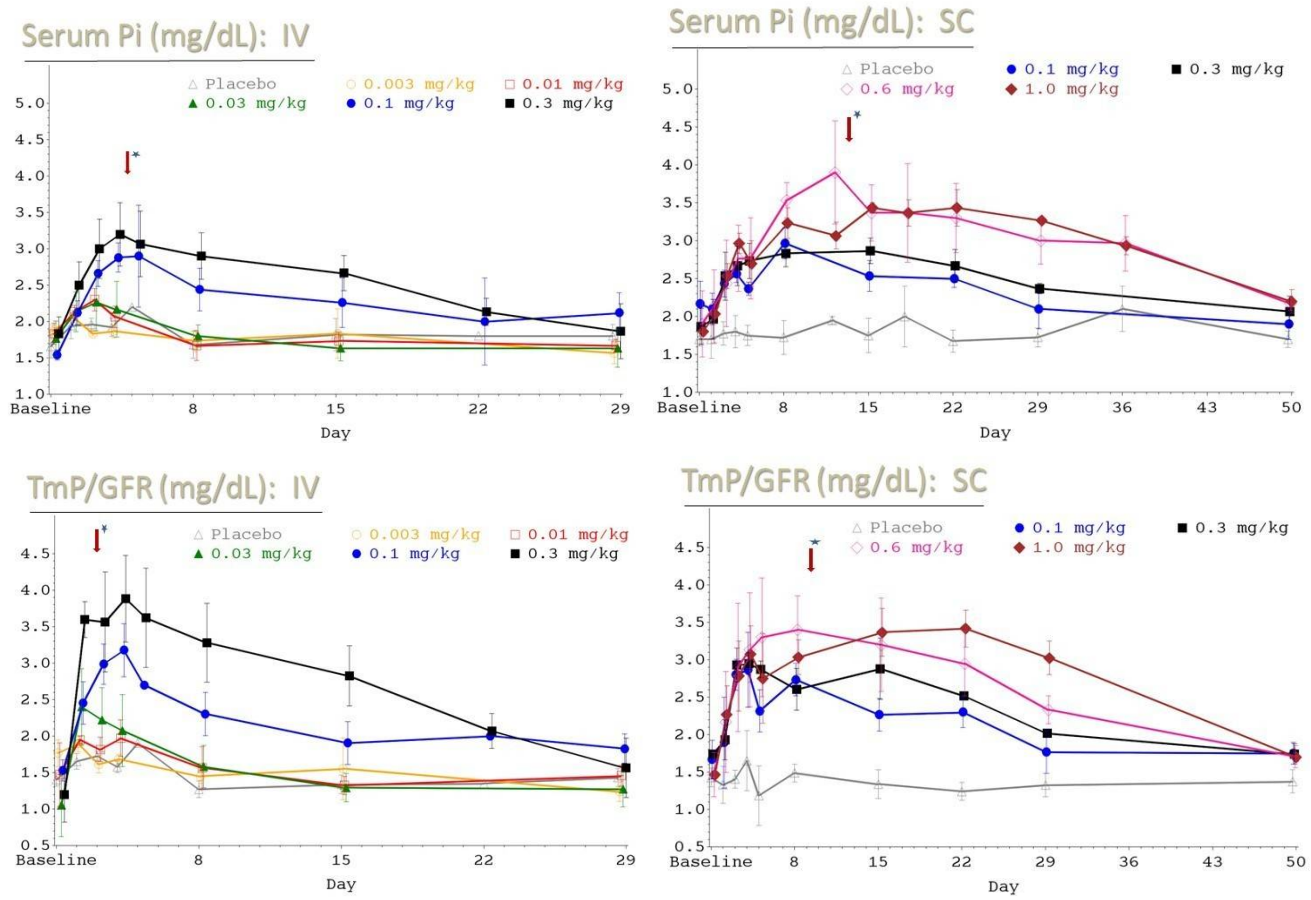
Ultragenyx licensed UX023 from KHK in September 2013, prior to which KHK had conducted a Phase 1 Study, a Phase 1/2 study and an extension study in adults with XLH. Data from the Phase 1 trial was presented in October 2013 at ASBMR, and data from the Phase 1/2 trial and extension is expected in 2014.

In a Phase 1 trial, KRN23 was shown to increase serum phosphorus in a dose dependent fashion, while being relatively safe and well tolerated.

Phase 1. The double blind, placebo controlled single-dose trial enrolled 38 patients with baseline FGF ≥30pg/mL. Patients were randomized to receive a single subcutaneous injection of 0.1, 0.3, 0.6, or 1.0 mg/kg (n=12), a single intravenous injection of 0.003, 0.01, 0.03, 0.1, or 0.3 mg/kg (n=17) or placebo (n=9). Single

doses of KRN23 were shown to increase serum phosphate, tubular reabsorption of phosphorus (TmP/GFR) and vitamin D as compared to placebo in the higher dose groups. In the SubQ dosing arm, increases were statistically significant at the 0.3, 0.6, and 1.0 mg/kg dose levels ($p<0.05$ for serum phosphorus, $p<0.001$ for vitamin D).

Figure 3: Phase 1 Study Results: Effect on Serum Phosphorus and the Tubular Reabsorption of Phosphorus (TmP/GFR)



Source: Carpenter et al. 2013; ASBMR Abstract # 1048.

The peak serum P concentration was reached sooner with IV dosing vs. SubQ (0.5-4 days vs. 8-15 days). The duration of effect was dose related and longer with SubQ dosing (persisted for 29 days) vs. IV. Importantly, no meaningful changes in serum calcium, serum parathyroid hormone, or urinary calcium excretion were observed. The rates of AEs in the active treatment arms were greater than placebo (82% vs. 40% IV; 83% vs. 50% SubQ), and the incidence and types of AEs did not appear to be dose related. Drug related AEs occurred in 6 patients (4 IV, 2 SubQ). The most common treatment emergent AEs were nausea (24%) and headache (18%) in the IV arm and elevated serum amylase and back pain (17% each) in the SubQ arm. There were no SAEs, no changes in biochemistries, ECGs or renal sonograms. Of note, no patients developed antibodies against KRN23 and no hypersensitivity or infusion related reactions were observed.

Figure 4: Phase 1 Study Results: Safety Assessments

	IV Cohorts		SC Cohorts	
Adverse Events	KRN23 (N=17)	Placebo (N=5)	KRN23 (N=12)	Placebo (N=4)
Number (n ([%]) of Patients with Any AE	14 (82.4)	2 (40)	10 (83.3)	2 (50)
Number (n ([%]) of Patients with AEs Related to Study Drug	4 (23.5)	0	2 (16.7)	0
Number (n ([%]) of Patients with AEs ≥Grade 3 in Severity	3 (17.6)	0	1 (8.3)	0
Number (n ([%]) of Patients with Drug-Related AEs ≥ Grade 3 in Severity	0	0	0	0

- Number of patients with AEs related to KRN23 was smaller in SC cohorts than in IV cohorts

Source: Carpenter et al. 2013; ASBMR Abstract # 1048.

Development Status

Data from the Phase 1/2 trial is expected to be released in 2014. Ultragenyx is beginning clinical development for pediatric XLH and plans to initiate a Phase 2 study in pediatric patients in 2014. The company also plans to continue development for the drug in adult patients, and intends to run a Phase 2b trial in adults in parallel with the expected Phase 3 pediatric trial.

Market Opportunity

RARE estimates that there are 3,000 pediatric and 9,000 adult XLH patients in the US.

Ultragenyx estimates that there are ~3,000 XLH pediatric patients and ~9,000 XLH adult patients in the US. There is a spectrum of severity in the pediatric population, but the target population is larger in children, with males more severely affected than females. Children suffer from more serious disease effects vs. adults due to higher metabolic rates in their bones. Because XLH is less debilitating in adults and progresses more slowly, many adults do not seek treatment. Ultragenyx estimates that 10-20% of the adult population may be eligible for treatment if their bone disease is severe.

Revenue Build

We assume launch in 2018, a WW prevalence of 24,000, a 70% peak penetration in pediatric patients and a 10% peak penetration in adult patients, giving peak revenue estimates of $800M.

In our base case, we estimate ~$800 million in worldwide peak sales for KRN23 in XLH (RARE retains about a third of the total value of the product). We assume a worldwide prevalence of 24,000 patients, which grows slightly year over year. Of this patient population, we assume 70% of the pediatric patients and 10% of the adult patients in the US are penetrated, as many adult patients may not seek treatment. In the rest of the world, we assume a peak penetration of 20%, which is an approximate weighted average of the pediatric/adult population assuming the same penetration rates as the US. Regarding price, we assume a base case price of $100K per patient, which translates into peak sales of ~$800 million. With a more conservative pricing assumption of $50K per patient, we achieve peak sales of ~$450 million, and with a more aggressive pricing assumption of $150K per patient, we achieve peak sales of ~$1.2 billion. We assume KRN23 launches in 2018.

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Figure 10: Our KRN23 Revenue Build for XLH

		2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E
XLH Prevalence worldwide									
	growth								
XLH Prevalence worldwide	*2%*	25,978	26,498	27,028	27,568	28,120	28,682	29,256	29,841
US XLH - Pediatrics									
US Pediatric Population	*2%*	3,247	3,312	3,378	3,446	3,515	3,585	3,657	3,730
Penetration			*5%*	*20%*	*35%*	*45%*	*55%*	*63%*	*70%*
# patients treated		-	149	676	1,206	1,582	1,972	2,286	2,611
Gross Price per patient ('000s)	*1.50%*								
Low pricing			50	51	52	52	53	54	55
Base pricing			100	102	103	105	106	108	109
High pricing			150	152	155	157	159	162	164
Net Price per patient ('000s)	*7%*								
Low pricing		-	47	47	48	49	49	50	51
Base pricing		-	93	94	96	97	99	100	102
High pricing		-	140	142	144	146	148	150	153
Total US Pediatric Sales									
Low pricing assumption		-	7	32	58	77	97	114	133
Base pricing assumption		-	14	64	116	154	195	229	266
High pricing assumption		-	21	96	173	231	292	343	398
Growth				*360%*	*81%*	*33%*	*27%*	*18%*	*16%*
US XLH - Adults									
US Adult Population	*2%*	9,742	9,937	10,135	10,338	10,545	10,756	10,971	11,190
Penetration			*1%*	*4%*	*6%*	*8%*	*10%*	*10%*	*10%*
# patients treated		-	99	405	620	844	1,076	1,097	1,119
Gross Price per patient ('000s)	*1.50%*								
Low pricing			50	51	52	52	53	54	55
Base pricing			100	102	103	105	106	108	109
High pricing			150	152	155	157	159	162	164
Net Price per patient ('000s)	*7%*								
Low pricing		-	47	47	48	49	49	50	51
Base pricing		-	93	94	96	97	99	100	102
High pricing		-	140	142	144	146	148	150	153
Total US Adult Sales									
Low pricing assumption		-	5	19	30	41	53	55	57
Base pricing assumption		-	9	38	59	82	106	110	114
High pricing assumption		-	14	57	89	123	159	165	171
Growth				*314%*	*55%*	*38%*	*29%*	*4%*	*4%*
Total US Sales									
Low pricing assumption		-	12	51	87	118	150	169	190
Base pricing assumption		-	23	102	175	236	301	339	379
High pricing assumption		-	35	153	262	354	451	508	569
Royalty from KHK			*50%*	*50%*	*50%*	*50%*	*50%*	*25%*	*28%*
US Revenue to Ultragenyx									
Low pricing assumption		**-**	**6**	**26**	**44**	**59**	**75**	**42**	**52**
Base pricing assumption		**-**	**12**	**51**	**87**	**118**	**150**	**85**	**104**
High pricing assumption		**-**	**17**	**77**	**131**	**177**	**226**	**127**	**156**
RoW XLH									
RoW Population	*2%*	12,989	13,249	13,514	13,784	14,060	14,341	14,628	14,920
Penetration			*2%*	*7%*	*12%*	*16%*	*20%*	*20%*	*20%*
# patients treated		-	265	946	1,654	2,250	2,868	2,926	2,984
Gross Price per patient ('000s)	*1.50%*								
Low pricing			50	51	52	52	53	54	55
Base pricing			100	102	103	105	106	108	109
High pricing			150	152	155	157	159	162	164
Net Price per patient ('000s)	*7%*								
Low pricing		-	47	47	48	49	49	50	51
Base pricing		-	93	94	96	97	99	100	102
High pricing		-	140	142	144	146	148	150	153
Total RoW Sales									
Low pricing assumption		-	12	45	79	109	142	147	152
Base pricing assumption		-	25	89	158	219	283	293	303
High pricing assumption		-	37	134	238	328	425	440	455
Growth				*262%*	*77%*	*38%*	*29%*	*4%*	*4%*

Source: J.P. Morgan estimates.

We assume patent protection through 2029, and there are currently no other products in development for XLH.

Intellectual Property

Ultragenyx shares rights with partner Kyowa Hakko Kirin to 20 issued patents, including 3 US patents and 1 pending US patent application relating to KRN23 for the treatment of XLH and other certain hypophosphatemic conditions. The terms of the issued patents in the US range from 2022-2029 and the projected term for the pending US patents is 2028. KRN23 also has orphan drug designation in the US (7yr exclusivity), and Ultragenyx plans on seeking orphan drug exclusivity in the EU (10yr exclusivity). There are currently no patents issued for KRN23 in Latin America.

Competitive Landscape

There are currently no approved therapies for the underlying cause of XLH, and as far as we are aware, there are no competing products in clinical development. Most patients manage the disease through oral phosphate replacement and vitamin D therapies; however, these approaches are only moderately effective at restoring bone formation and growth and are not well-tolerated. In addition, multiple doses are needed per day, and close monitoring is required due to the potential for spikes in phosphate levels. Although oral phosphate replacement and vitamin D therapies are relatively inexpensive, we believe patients will seek treatment that addresses the underlying cause of the disease, especially in the pediatric population, where XLH has a more devastating affect.

RARE is developing KRN23 in partnership with KHK, and the partnership terms/ responsibilities vary by geography. In sum, RARE receives ~1/3 of KRN23's NPV.

Partnership/Collaboration Overview

Ultragenyx entered into a license and collaboration agreement with Kyowa Hakko Kirin (KHK) in August 2013. Under the terms of the agreement, Ultragenyx and KHK will collaborate on the development of KRN23 in the US, Canada and EU, with Ultragenyx leading the development for XLH. The development costs are split 50/50 until five years post launch in the US and until launch in the EU; KHK will then retain full responsibility of the development costs.

In the US and Canada, Ultragenyx will jointly commercialize KRN23 and receive a 50% profit share for five years post launch. After the five-year period, KHK will assume full commercialization responsibility in the US, and Ultragenyx is entitled to receive a tiered double-digit revenue share in the mid- to high-twenty percent range. Marketing and promotional responsibilities will be shared between both parties.

Ultragenyx retains the right to develop and commercialize KRN23 in Mexico, Central and South America, and will pay KHK a low-single-digit royalty on net sales in these regions. In the EU and the rest of the world (excluding the aforementioned Latin American regions), KHK will commercialize KRN23, and Ultragenyx is entitled to receive a royalty of up to 10% of net sales. KHK is responsible for manufacturing and supplying the product both for clinical and commercial use worldwide, with Ultragenyx paying a supply price based on a set percentage of net sales.

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rhGUS for MPS 7

UX003 is an enzyme replacement therapy in development for the treatment of MPS 7.

Product Overview

UX003 (recombinant human beta-glucuronidase, or rhGUS) is an enzyme replacement therapy (ERT) being developed for the treatment of mucopolysaccharidosis 7 (MPS 7). Beta-glucuronidase is an enzyme located in the lysosome that catalyzes hydrolysis of (breaks down) glycosaminoglycans (GAGs). Patients lacking this enzyme develop MPS 7, a multi-systemic disease caused by the buildup of non-degraded GAGs in the lysosomes. As it has been shown to do in other MPS disorders, replacement of the missing enzyme via ERT is hypothesized to help clear GAGs from the cells and ameliorate the disease burden. Ultragenyx licensed exclusive worldwide rights to rhGUS related know-how and cell lines from St. Louis University in November 2010.

Disease Background

MPS 7, also known as Sly Syndrome, is a member of a group of rare genetic diseases caused by a deficiency of one of 10 lysosomal enzymes that are responsible for the stepwise breakdown of GAGs. It is an inherited autosomal recessive trait. MPS 7 patients lack beta-glucuronidase, an enzyme that is responsible for one of the last degradation steps of most GAGs. Improperly digested GAG fragments accumulate in the lysosomes throughout the body eventually causing progressive cellular and organ dysfunction. Undegraded GAG is also excreted in the urine.

Clinical manifestations of MPS 7 are heterogeneous in terms of system involvement and are similar to MPS I and II. Symptoms are progressively limiting in nature and include coarsened facial features, hepatosplenomegaly, airway obstruction, lung disease, cardiovascular complications, joint stiffness, short stature, and skeletal disease (dysostosis multiplex). Additionally, GAG build-up in the joint tissue can lead to significant stiffness and reduced mobility, which results in severe pain and inability to walk. Developmental delays are also a common feature of MPS 7, as is mental retardation, though it is usually moderate and non-progressive.

The severity of MPS 7 ranges widely, from very severe forms present at birth, as evidenced by symptoms such as hydrops fetalis (extreme swelling) and neonatal jaundice, in addition to facial dysmorphism and skeletal disease. Infants with hydrops fetalis rarely survive longer than a few weeks or months, and this most severe manifestation of the disease is thought to account for as much as half of the disease incidence. On the other hand, milder cases of MPS 7 with relatively normal life spans have been observed wherein some symptoms as benign as mild scoliosis or thoracic kyphosis were noted. The majority of MPS 7 patients die between their teenage years and 30s.

Figure 1: Mucopolysaccharidosis 7 Patients

Source: Company Presentation.

There is no currently approved therapy to specifically treat MPS 7, though a constellation of symptomatic therapies, supportive care, and treatment of complications have improved the quality of life and extended survival for MPS 7 patients. Bone marrow or hematopoietic stem cell transplants have been tested in some patients, but the skeletal and connective tissue disease was not effectively treated. Additionally, there is significant morbidity and mortality associated with these treatments, which is a key point of consideration in a pediatric population.

Data Review

rhGUS ERT has been evaluated in preclinical (mouse) studies, which show that chronic IV administration resulted in widespread tissue uptake and a reduction in pathology in a wide variety of tissues. Enzyme uptake was observed in a dose dependent manner in both visceral and CNS tissue.

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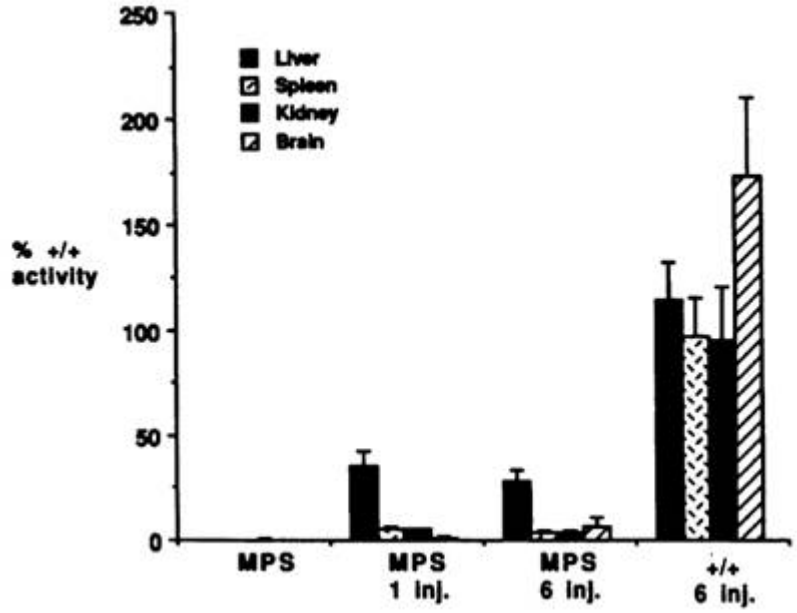
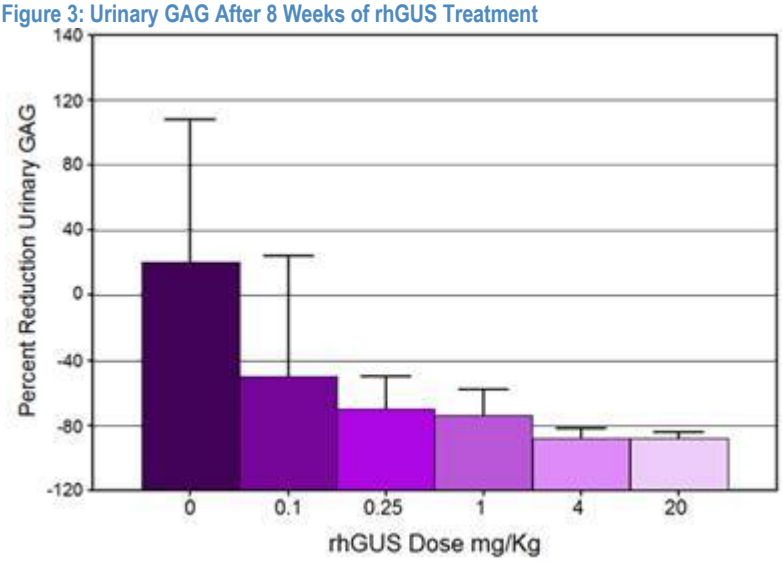
Figure 2: Beta-glucuronidase Levels 1 Week After Administration in the Liver, Spleen, Kidney and Brain of MPS 7 Mice



Source: Sands et al. J. Clin. Invest. 1994.93:2324-2331.

Treatment with the ERT resulted in a reduction in lysosomal GAG (or prevention accumulation when administered to newborns) and was correlated with improvements in bone length, longevity, hearing, immune function and cognitive ability. Treatment with rhGUS in MPS 7 mice also showed dose dependent reduction in urinary GAG excretion. Importantly, no-infusion associated reactions or clinical problems were noted in these preclinical trials.

Figure 3: Urinary GAG After 8 Weeks of rhGUS Treatment



Source: Company Presentation.

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Data recently presented on the first patient treated with UX003 showed signs of early efficacy and an acceptable safety/tolerability profile.

Data on the first patient treated with rhGUS (via an emergency IND) was presented at the World Lysosomal Disease Network Symposium in early Feb. 2014. The patient was 12 years old and had advanced multi-system MPS 7 with respiratory insufficiency and had been wheelchair dependent since Dec 2010. The patient was born in 2001 with hydrops fetalis and hepatosplenomegaly and was diagnosed at 17 mos with severe spinal cord compression that required cervical fusion. Data presented was after 14 weeks of treatment (8 infusions) at 2mg/kg. Preliminary observations show evidence of clearance of lysosomal storage, including reduction in urinary GAG and hepatosplenomegaly.

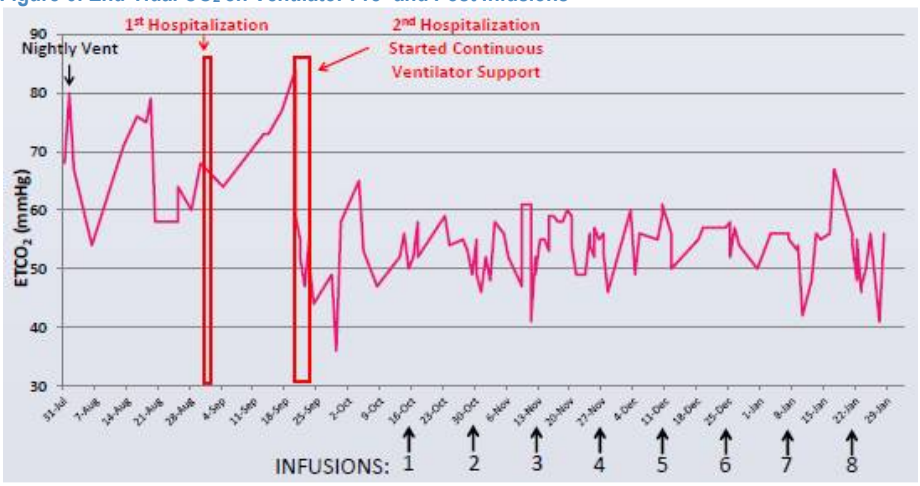
Figure 5: Physical Exam Findings in First Patient Treated with UX003 After 14 weeks (8 infusions)

	Liver	Spleen
Pre-treatment	~2 cm below umbilicus	Extended into groin
2 wks post-treatment	~1 cm above umbilicus	At umbilicus
8 wks post-treatment	~1 cm above umbilicus	At umbilicus
12 wks post-treatment	Above umbilicus	Above umbilicus

Source: Fox et al. 2014 - 10th Annual World Lysosomal Disease Network Symposium.

There is also evidence of improved pulmonary function based on reduced ventilator dependence and decreased CO_2 retention. Importantly, no IARs were observed during the first 14 weeks of treatment. The patient has also experienced a significant improvement in stamina, increased school attendance, and has started to manipulate his wheelchair independently.

Figure 6: End-Tidal CO_2 on Ventilator Pre- and Post Infusions



Source: Fox et al. 2014 - 10th Annual World Lysosomal Disease Network Symposium.

No infusion associated reactions were observed. Hive-like macuoles were observed within 24 hours after the first and second infusions, though were treatable. Temporary fatigue was also observed within the first 24-48 hours after infusions, which physicians speculate could be related to secondary fluid overload related to the patient's valve disease and improved with treatment.

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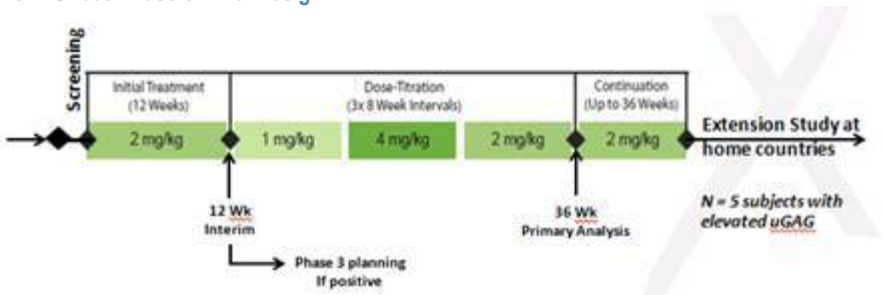
Development Status

In 4Q13, Ultragenyx initiated a Phase1/2 trial of UX003 in MPS patients. The trial is expected to enroll 5 patients who will receive UX003 via IV every other week for 36 weeks, with the option to continue for up to an additional 36 weeks. During the initial 12-week treatment period, patients will receive 2mg/kg UX003, and the subsequent 24 weeks will be a dose titration phase during which patients will receive 1mg/kg, 2mg/kg, or 4 mg/kg UX003.

Figure 4: UX003 Phase1/2 Trial Design



Source: Company Presentation.

If results from the initial 12-week treatment period are positive, Ultragenyx plans to initiate a pivotal trial involving ~12 patients. The trial is expected to have a reduction in urinary GAG primary endpoint; the EMA has signed off on the surrogate endpoint, and the FDA has requested additional data showing correlation with urinary GAG clinical endpoints. Ultragenyx also plans to study the ERT in MPS 7 patients under the age of 5, including in younger patients with hydrops fetalis.

Market Opportunity

MPS 7 is an ultra orphan disease, with an estimated WW prevalence of ~200 in the developed world.

Ultragenyx estimates that the prevalence of MPS 7 is ~200 patients in the developed world and ~20 patients per year worldwide are born with non-immune hydrops fetalis due to MPS 7 (MPS 7-NIHF patients typically die within the first year). To date, Ultragenyx has identified 91 MPS 7 patients worldwide, with 15 of these patients in the US. We believe the global prevalence could increase over time (as seen with other rare disease populations) with the availability of an effective and safe therapy.

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We model peak sales of $70M, which assumes a 70% peak penetration into US patients and a 50% penetration into ROW patients.

Revenue Build

We estimate ~$70 million in worldwide peak sales for rhGUS in MPS 7. We assume a worldwide prevalence of 200 patients, which grows slightly year over year. In this patient population, we then assume a peak penetration of 70% in the US and a slightly lower penetration rate of 50% ex-US, five years post launch. Regarding price, we assume a base case price of $450K per patient, which translates into peak sales of ~$70 million. With a more conservative pricing assumption of $400K per patient, we achieve peak sales of ~$65 million, and with a more aggressive pricing assumption of $500K per patient, we achieve peak sales of ~$80 million. We assume rhGUS launches in 2017, though we note that this timeline depends on the Phase 3 trial progressing as planned and FDA acceptance of Ultragenyx's pivotal trial design.

Figure 11: Our rhGUS Revenue Build for MPS 7

		2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E
MPS7 Prevalence worldwide										
	growth									
MPS7 Prevalence worldwide	*2%*	212	216	221	225	230	234	239	244	249
US MPS7										
US MPS7 Population	*2%*	35	36	36	37	38	39	39	40	41
	16%									
Penetration			*8%*	*28%*	*43%*	*55%*	*65%*	*70%*	*70%*	*70%*
# patients treated		-	3	10	16	21	25	28	28	29
Gross Price per patient (000s)	*1.50%*									
Low pricing			400	406	412	418	425	431	437	444
Base pricing			450	457	464	471	478	485	492	499
High pricing			500	508	515	523	531	539	547	555
Net Price per patient ('000s)	*0%*									
Low pricing		-	400	406	412	418	425	431	437	444
Base pricing		-	450	457	464	471	478	485	492	499
High pricing		-	500	508	515	523	531	539	547	555
Total US Sales										
Low pricing assumption		-	1	4	7	9	11	12	12	13
Base pricing assumption		-	1	5	7	10	12	13	14	14
High pricing assumption		-	1	5	8	11	13	15	15	16
Growth				*262%*	*59%*	*32%*	*22%*	*11%*	*4%*	*4%*
RoW MPS7										
RoW MPS7 Population	*2%*	177	181	184	188	192	196	200	204	208
Penetration			*8%*	*23%*	*33%*	*41%*	*46%*	*50%*	*50%*	*50%*
# patients treated		-	14	42	62	79	89	100	102	104
Gross Price per patient (000s)	*1.50%*									
Low pricing			400	406	412	418	425	431	437	444
Base pricing			450	457	464	471	478	485	492	499
High pricing			500	508	515	523	531	539	547	555
Net Price per patient ('000s)	*0%*									
Low pricing		-	400	406	412	418	425	431	437	444
Base pricing		-	450	457	464	471	478	485	492	499
High pricing		-	500	508	515	523	531	539	547	555
Total RoW Sales										
Low pricing assumption		-	6	17	26	33	38	43	45	46
Base pricing assumption		-	7	19	29	37	43	48	50	52
High pricing assumption		-	7	22	32	41	47	54	56	58
Growth				*198%*	*49%*	*29%*	*15%*	*14%*	*4%*	*4%*
Royalty to St. Louis University		0%	0%	3%	3%	3%	3%	3%	3%	3%
Royalty Amount										
Low pricing assumption		-	-	1	1	1	1	2	2	2
Base pricing assumption		-	-	1	1	1	2	2	2	2
High pricing assumption		-	-	1	1	2	2	2	2	2
Total Net Sales to Ultragenyx										
Low pricing assumption		**-**	**7**	**21**	**31**	**40**	**47**	**53**	**55**	**57**
Base pricing assumption		**-**	**8**	**23**	**35**	**45**	**53**	**60**	**62**	**64**
High pricing assumption		**-**	**9**	**26**	**39**	**50**	**59**	**67**	**69**	**71**

Source: J.P. Morgan estimates,

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rhGUS is currently not covered by any patents but has received orphan designation from the FDA and EMA (7-year exclusivity in the US and 10 years in the EU).

Intellectual Property

rhGUS is not currently covered by patents; however, Ultragenyx is in the process of filing patent applications related to compositions with specific characteristics useful in enzyme replacement therapies for the potential treatment of multi-system lysosomal storage disease. Ultragenyx also plans to file patents related to certain aspects of its therapy, such as formulation, manufacturing process, dosage, and potentially other aspects of the rhGUS therapy. rhGUS has orphan drug designation in the US and EU. We do not expect generic competition for a disease with such few patients, and thus, rhGUS could almost be viewed as a perpetuity, in our view.

Competitive Landscape

There are currently no approved therapies for MPS 7, and as far as we are aware, there are no competing products in clinical development. A few MPS 7 patients have received bone marrow or hematopoietic stem cell transplants; however, these transplants did not prove effective in treating the patients' skeletal and connective tissues and the transplant procedures could lead to high morbidity/mortality.

Partnership/Collaboration Overview

Ultragenyx licensed worldwide rights to rhGUS-related intellectual property from Saint Louis University in November 2010. Under the terms of the agreement, Ultragenyx paid a $10K up-front fee and Saint Louis University is eligible to receive a $100K approval milestone, as well as a low-single-digit royalty on net sales (we assume a 3% royalty) once $10M of cumulative worldwide sales is reached.

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Triheptanoin for LC-FAOD & Glut1 DS

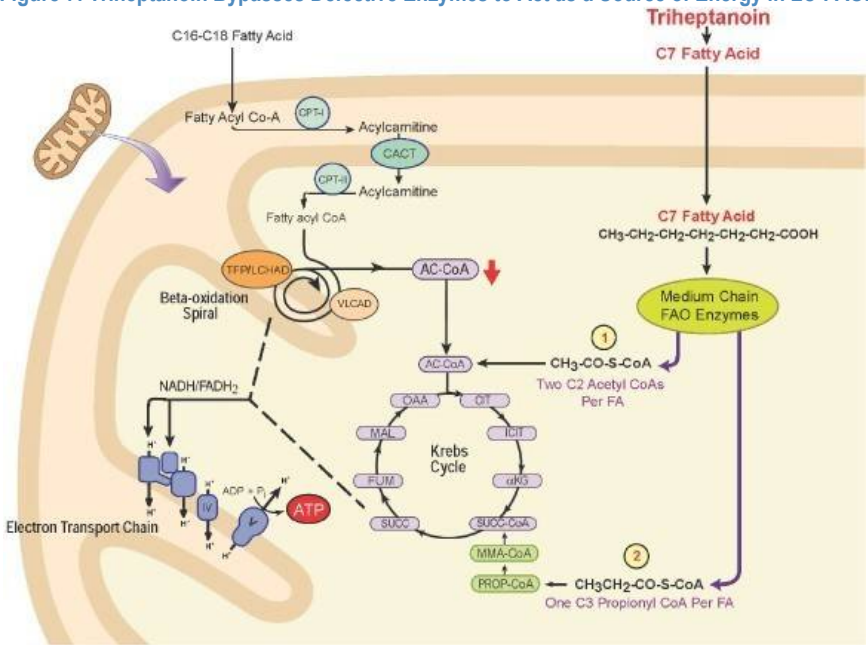
Triheptanoin is an oil that is composed of medium-chain fatty acids and is being developed as a substrate replacement therapy for patients with LC-FAOD and Glut1 DS.

Product Overview

UX007 (Triheptanoin, aka trihep) is a tasteless oil made up of medium-chain triglycerides of three seven-carbon fatty acids. It is designed to act as a substrate replacement, i.e., an alternative source of energy in patients who are unable to make use of typical energy production/utilization methods. Ultragenyx is currently developing the product as a treatment for long-chain fatty acid oxidation disorders (LC-FAOD) and glucose transporter type 1 deficiency syndrome (Glut1 DS).

In LC-FAOD, trihep is able to bypass the defective long-chain fatty acid metabolism. The metabolites (heptanoate and ketone bodies) are then converted to two-carbon units and three-carbon units in the mitochondria, which can then be used to fuel the Krebs cycle/energy production.

Figure 7: Triheptanoin Bypasses Defective Enzymes to Act as a Source of Energy in LC-FAOD



Source: Company reports.

In Glut1 DS, trihep's metabolites (heptanoate and ketone bodies) can bypass the defective Glut1 transporter (which normally transports glucose into the brain) to cross the blood-brain-barrier and act as an alternative energy source for the brain (which is typically dependent on glucose).

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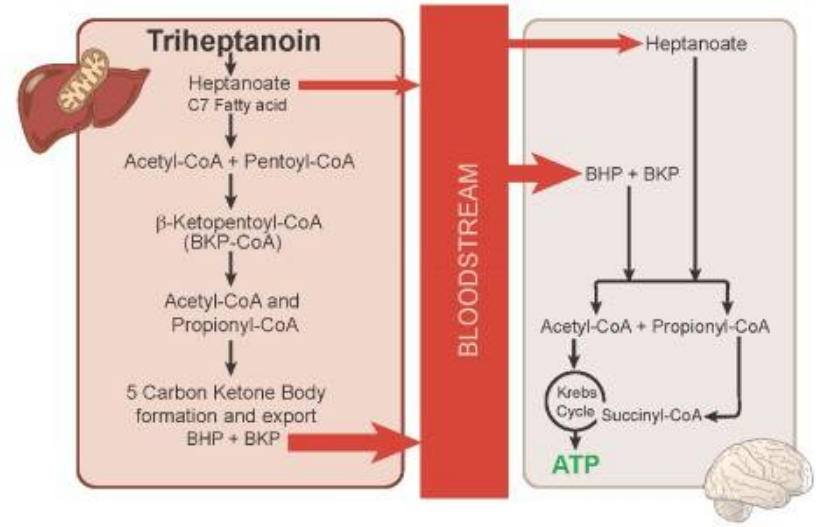
Figure 8: Triheptanoin Bypasses a Defective Transporter to Cross the BBB and Act as a Source of Energy in Glut1 DS



Source: Company reports.

Trihep has been studied for 13 years in ~130 patients in a variety of indications, including LC-FAOD (n=65), and multiple investigator sponsored trials have suggested a treatment benefit (+/- SOC). In Glut1 DS, anecdotal reports have indicated that treatment with trihep reduced the seizure rate and improved the development rate in some patients.

Ultragenyx recently initiated an open-label Phase 2 trial in patients with LC-FAOD disorders, and a randomized Phase 2 trial in Glut1 DS is enrolling. Ultragenyx has exclusive worldwide rights to triheptanoin (in-licensed from Baylor Research Institute in 2012).

Patients with LC-FAOD lack the enzymes necessary to break down large fat molecules from food into energy. Trihep metabolism bypasses those defective enzymes and can be metabolized to create energy by downstream processes.

LC-FAOD. Long chain fatty acid oxidation disorder describes a group of autosomal recessive metabolic disorders wherein the body is unable to break down fatty acids into energy. Specifically, LC-FAOD is caused by mutations in the genes that code for enzymes that are involved in the breakdown of long-chain fatty acids into smaller molecules that can be used for energy. The build-up of long-chain fatty acids is also thought to cause mitochondrial damage, which leads to a deficiency in intermediate compounds that are required for normal functioning of the Krebs cycle.

As a result, LC-FAOD patients are unable to adequately generate energy from fat metabolism, and instead depend on glucose metabolism. This can lead to the depletion of all glucose in the body and severe liver, heart and muscle disease. The imbalance in energy production may also leave patients with low muscle tone, weakness, exercise intolerance, muscle pain, fatigue, low-grade chronic rhabdomyolysis (muscle rupture), and severe acute episodes of rhabdomyolysis requiring hospitalization. Additionally, LC-FAOD is also known to be a cause of sudden infant death syndrome. As such, newborn screening for LC-FAOD has been implemented across the US and awareness/newborn screening ex-US is growing.

The current standard of care for LC-FAOD includes diligent prevention of fasting along with a low-fat/high carbohydrate diet. Carnitine (an amino acid derivative that

plays a critical role in energy production) supplementation and/or medium-chain triglyceride (MCT) oil supplementation are also used in some cases. MCT oil is composed of medium-chain fatty acids that can be metabolized by medium-chain fatty acid oxidation enzymes, thus also bypassing defective long-chain enzymes (like trihep). It does not provide odd-chain fatty acids to refill the Krebs cycle, however, and cannot be converted into glucose. As such, the current SOC does not prevent all hypoglycemic events, exercise intolerance, muscle weakness, rhabdomyolosis and cardiomyopathy in some patients. Furthermore, a mortality rate of 50% has been observed in these patients despite treatment with SOC.

Patients with Glut1 DS have a defect in the protein that is responsible for glucose transport across the blood-brain-barrier. Trihep's metabolites work around the defective transporter, and enter the brain where they can serve as an alternative energy source.

Glut1 DS. Glut1 DS is an autosomal dominant genetic disorder that impairs brain metabolism, specifically by preventing glucose transport into the brain, where it is normally the main source of energy. The disease is caused by a mutation in the gene that encodes for Glut1, a membrane spanning protein that facilitates the transport of glucose across plasma membranes. Without it, glucose is unable to cross the blood-brain-barrier resulting in a chronic state of energy deficiency for the brain.

Patients with Glut1 typically suffer from seizures (which begin within the first few months of life), developmental delays/intellectual disability and movement disorders. The majority of children with Glut1 DS experience problems with language (both in speaking and comprehension). Patients are suspected of having Glut1 DS based on symptoms and low glucose levels in the cerebrospinal fluid. Genetic testing is readily available and is the main diagnostic test, though diagnosis can also be confirmed using a red cell glucose uptake test (though this test is not universally available).

There are currently no drugs approved to treat Glut1 DS specifically. Patients may be treated with antiepileptic drugs (AEDs), though Glut1 DS seizures are generally thought to be resistant to AEDs. The current standard of care is a ketogenic diet, which is a high-fat diet (70-80% of daily calories in fat)/low-carbohydrate diet. This diet generates ketone bodies to serve as an alternative energy source and is generally effective in controlling or reducing seizures when patients are fully compliant. However, seizures are not always completely controlled in some patients, and compliance to the diet can be challenging. Furthermore, some patients still experience significant problems with developmental delay and motor dysfunction despite improvement in seizures.

Data Review

Trihep has been studied in academia in various LC-FAOD subtypes and has been shown to be effective and relatively well tolerated.

LC-FAOD. Trihep has been studied for 13 years in various investigator sponsored trials in LC-FAOD patients. Of the ~65 LC-FAOD patients treated over this time period, trihep was shown to be safe and relatively well tolerated in all subtypes of LC-FAOD. Common non-serious treatment emergent AEs include GI side effects and weight gain, which can be managed by titrating the dose at initiation of therapy and carefully managing caloric intake, respectively. Serious AEs reported have been consistent with the underlying disease and include muscle weakness/pain, myoglobinuria (muscle protein in the urine), muscle cell rupture, metabolic crisis, cardiomyopathy, hypoglycemia and elevated creatine kinase. Other SAEs included infections, fever and vomiting from unspecified cause, respiratory distress/breathing problems, falling oxygen saturation rate, seizure, or medical procedures. Of all SAEs, only 3 (6%) were considered possibly related to triheptanoin treatment and were muscle cell rupture and elevated CK reported in 2 patients, and myoglobinuria (in conjunction with exercise, suboptimal triheptanoin dose, and no fluid intake) in one patient.

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In 2006, a cumulative summary was reported for 48 patients, which showed that treatment with trihep on top of SOC resulted in a decrease in liver enlargement and low blood sugar in substantially all of the treated patients vs. their previous SOC alone regimen.

Figure 9: Event Rates in 2006 Triheptanoin Study

	# Symptomatic Patients	
Symptoms	**Before triheptanoin**	**After triheptanoin**
Cardiac	10	1
Muscle rupture	36	15
Weakness/fatigue	44	10
Low blood sugar	24	1
Liver enlargement	26	2
Retinopathy	3	3

Source: Company reports.

Additionally, Ultragenyx has conducted a retrospective review of patient data from a compassionate use trial at the University of Pittsburgh Medical Center. The analysis looked at the impact of trihep on the rate and extent of hospitalizations in 20 of 24 patients who were treated with trihep for up to 13 years. Data reviewed included 241 years of patient data and 319 hospitalizations. The annual pre-treatment and post-treatment rates were calculated by dividing the total number of events or hospital days by the total number of pre- or post-treatment years. The retrospective analysis showed that treatment with trihep resulted in a statistically significant decrease in mean total hospital days per year, in mean hypoglycemic events per year, and mean hospital days per year due to hypoglycemic events.

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Figure 10: Event Rates in 2006 Triheptanoin Study

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

(1) Excludes data for four infants dosed within first six months of life.
(2) Excludes hospitalizations with unknown discharge dates.
(3) Four infants were dosed within the first six months of life.
(4) Includes only those patients with hypoglycemia events prior to treatment.
(5) Includes only those patients with rhabdomyolysis events prior to treatment.

Source: Company reports.

Anecdotal reports describing the use of trihep in Glut1 DS indicate that it helps reduce the rate of seizures and improves the development rate in Glut1 DS patients.

Glut1 DS. Several publications provide data on the efficacy of trihep in animal models of epilepsy, and because trihep's metabolites are essential as sources of energy in Glut1 DS, they are not anticipated to be toxic. In two studies reported in the literature, toxicities were not observed aside from an increase in liver fat, which may have been related to the high fat diet. Additionally, long-term treatment with trihep in pediatric patients in other diseases support the safety of chronic dosing when administered at ~35% of daily caloric intake. Anecdotal reports from the use of trihep in Glut1 DS indicate reduced seizure rates and an improvement in development rate in some patients.

Development Status

Phase 2 trials are ongoing, one in LC-FAOD and one in Glut1 DS. Data from both trials are expected in 2015.

Ultragenyx recently began enrolling an open label Phase 2 trial in patients with LC-FAOD, which will be conducted at ~8 sites in the US and EU. The trial will enroll ~30 patients between the ages of 6 months to 35 years with severe LC-FAODs who exhibit clinical manifestations of their disease despite their current therapeutic regimen. After a 4-week baseline run-in period on standard treatment, patients will receive trihep dosed at ~25-35% of total daily caloric intake for 24 weeks. Patients will then have the option to continue treatment for an additional 54 weeks. The primary endpoint of the trial is to evaluate the impact of trihep on acute clinical pathophysiology associated with LC-FAODs (change from baseline in skeletal myopathy, hepatic disease and cardiac disease). Secondary measures include safety and effect on energy metabolism; the trial will also evaluate the impact of trihep on major clinical events associated with the disease. Ultragenyx is also supporting multiple compassionate use programs and ISTs in FAOD and other indications.

A Phase 2 trial in Glut1 DS is enrolling patients between the ages of 3 and 17 who are not on or are not fully compliant with a ketogenic diet. Patients must also experience an average of at least 5 seizures per month despite current or prior use of at least one anti-epileptic drug (AED) to enroll. Patients will be randomized to

receive a trihep dose equal to ~35% of their daily caloric intake or placebo for an initial 8-week double blind treatment period (2 week titration, 6 week stable dose). After 30 patients have completed the double blind treatment period, an independent DMC will review the efficacy/seizure reduction data to consider modifying the target enrollment. After the initial double blind enrollment period, patients will have the option of rolling over into an open-label extension through week 52 of treatment. The primary efficacy endpoint of the trial is to determine the reduction from baseline in frequency of generalized or partial onset seizures. Secondary endpoints include the frequency of seizure as measured by EEG, change in cognitive function per the CANTAB test, baseline change in 6MWT distance, and change in gross motor function.

Market Opportunity

RARE estimates there are 2,000 to 3,500 LC-FAOD patients and 3,000 to 8,000 Glut1 DS patients in the United States.

LC-FAOD. Ultragenyx estimates that there are anywhere from 2,000-3,500 LC-FAOD patients in the US, depending on the mortality rate assumed. Of this estimated patient population, it is difficult to identify the number currently diagnosed since the adoption of newborn screening across the US is relatively new. The prevalence of LC-FAOD patients ex-US is even harder to estimate because newborn screening is not consistently implemented. However, with newborn screening, patients may be identified before they are symptomatic and could stay asymptomatic from preventative treatment. To date, Ultragenyx has identified 1,300 LC-FAOD patients globally, with >600 of these patients in the US. However, until triheptanoin progresses further in clinical development and more data matures, it's unclear what subsets of diagnosed LC-FAOD patients will be treated with triheptanoin.

Glut1 DS. Ultragenyx estimates that there are 3,000-8,000 Glut1 DS patients in the US and 12,000-20,000 patients in the developed world, with birth incidence estimated at 1:90,000 (published literature estimates a range of 3,000-7,000 patients in the US based on the assessment of generalized or absence seizures and suggests that older patients could be discovered over time with increased recognition of alternative or variable motor forms of the disease). To date, Ultragenyx has identified >200 Glut1 DS patients globally, with >80 of these patients in the US. Ultragenyx believes that the identification of one patient can be helpful in discovering other affected relatives, given that Glut1 DS can be an inherited disease.

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We assume 70% peak penetration in both LC-FAOD and Glut1 DS, giving peak revenues of $300M and $850M, respectively.

Revenue Build

LC-FAOD. We estimate ~$300 million in worldwide peak sales for triheptanoin in LC-FAOD. We assume a worldwide prevalence of about 4,300 patients, which grows slightly year over year. Of this patient population, we assume a peak penetration of 70% is reached in both the US and ex-US. Because triheptanoin may be used in combination with the current standard of care diet, we believe a large percentage of the patient population could be penetrated. As for pricing, we assume a base case of $80K per patient. With a more conservative price of $40K per patient, we achieve peak sales of ~$150 million, and with a more aggressive price of $120K per patient, we achieve peak sales of ~$450 million. We assume triheptanoin for LC-FAOD launches at the end of 2017 (slightly ahead of the Glut1 DS indication), but this timeline depends on the recently initiated Phase 2 trial and other later-stage studies progressing as planned.

Figure 12: Our Triheptanoin Revenue Build for LC-FAOD

		2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E
LC-FAOD Prevalence worldwide										
	growth									
LC-FAOD Prevalence worldwide	*2%*	4,599	4,691	4,784	4,880	4,978	5,077	5,179	5,282	5,388.0
	217%									
US LC-FAOD										
US LC-FAOD Population	*2%*	2,122	2,165	2,208	2,252	2,297	2,343	2,390	2,438	2,487
Penetration			*1%*	*16%*	*28%*	*38%*	*48%*	*58%*	*65%*	*70%*
# patients treated		-	22	353	631	873	1,125	1,386	1,585	1,741
Gross Price per patient ('000s)	*1.50%*									
Low pricing			40	41	41	42	42	43	44	44
Base pricing			80	81	82	84	85	86	87	89
High pricing			120	122	124	125	127	129	131	133
Net Price per patient ('000s)	*7%*									
Low pricing		-	37	38	38	39	39	40	41	41
Base pricing		-	74	76	77	78	79	80	81	83
High pricing		-	112	113	115	117	118	120	122	124
Total US Sales										
Low pricing assumption		-	1	13	24	34	44	56	64	72
Base pricing assumption		-	2	27	48	68	89	111	129	144
High pricing assumption		-	2	40	73	102	133	167	193	216
Growth					*81%*	*41%*	*31%*	*25%*	*16%*	*11%*
RoW LC-FAOD										
RoW LC-FAOD Population	*2%*	2,476	2,526	2,576	2,628	2,680	2,734	2,789	2,844	2,901
Penetration			*1%*	*16%*	*28%*	*38%*	*48%*	*58%*	*65%*	*70%*
# patients treated		-	25	412	736	1,019	1,312	1,617	1,849	2,031
Gross Price per patient ('000s)	*1.50%*									
Low pricing			40	41	41	42	42	43	44	44
Base pricing			80	81	82	84	85	86	87	89
High pricing			120	122	124	125	127	129	131	133
Net Price per patient ('000s)	*7%*									
Low pricing		-	37	38	38	39	39	40	41	41
Base pricing		-	74	76	77	78	79	80	81	83
High pricing		-	112	113	115	117	118	120	122	124
Total RoW Sales										
Low pricing assumption		-	1	16	28	40	52	65	75	84
Base pricing assumption		-	2	31	56	79	104	130	150	168
High pricing assumption		-	3	47	85	119	155	194	226	252
Growth					*81%*	*41%*	*31%*	*25%*	*16%*	*11%*
Royalty payable to Baylor Research Institute	5%									
Low pricing assumption		-	0	1	3	4	5	6	7	8
Base pricing assumption		-	0	3	5	7	10	12	14	16
High pricing assumption		-	0	4	8	11	14	18	21	23
Total Net Sales to Ultragenyx										
Low pricing assumption		-	2	27	50	70	91	114	133	148
Base pricing assumption		-	3	55	99	140	183	229	265	296
High pricing assumption		-	5	82	149	210	274	343	398	444

Source: J.P. Morgan estimates

Glut1 DS. We estimate ~$850 million in worldwide peak sales for triheptanoin in Glut1 DS. We assume a worldwide prevalence of 12,000 patients, which grows slightly year over year. In this patient population, we then assume a peak penetration of 70% in both the US and ex-US. Once again, since triheptanoin may be used in combination with the current standard of care, we believe a large percentage of the patient population could be penetrated. Regarding price, we assume a base case price of $80K per patient. With a more conservative pricing assumption of $40K per patient, we achieve peak sales of ~$450 million, and with a more aggressive pricing assumption of $120K per patient, we achieve peak sales of ~$1.3 billion. We assume triheptanoin for Glut1 DS launches in 2018, but this timeline depends on the recently initiated Phase 2 trial and other later-stage studies progressing as planned.

Figure 13: Our Triheptanoin Revenue Build for Glut1 DS

	growth	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E
Glut1 DS Prevalence worldwide									
Glut1 DS Prevalence worldwide	*2%*	12,989	13,249	13,514	13,784	14,060	14,341	14,628	14,920
US Glut1 DS									
US Glut1 DS Population	*2%*	3,247	3,312	3,378	3,446	3,515	3,585	3,657	3,730
Penetration			*5%*	*20%*	*35%*	*45%*	*55%*	*65%*	*70%*
# patients treated		-	166	676	1,206	1,582	1,972	2,377	2,611
Gross Price per patient ('000s)	*1.50%*								
Low pricing			40	41	41	42	42	43	44
Base pricing			80	81	82	84	85	86	87
High pricing			120	122	124	125	127	129	131
Net Price per patient ('000s)	*7%*								
Low pricing		-	37	38	38	39	39	40	41
Base pricing		-	74	76	77	78	79	80	81
High pricing		-	112	113	115	117	118	120	122
Total US Sales									
Low pricing assumption		-	6	26	46	62	78	95	106
Base pricing assumption		-	12	51	92	123	156	191	212
High pricing assumption		-	18	77	139	185	234	286	319
Growth				*314%*	*81%*	*33%*	*27%*	*22%*	*11%*
RoW Glut1 DS									
RoW Glut1 DS Population	*2%*	9,742	9,937	10,135	10,338	10,545	10,756	10,971	11,190
Penetration			*5%*	*20%*	*35%*	*45%*	*55%*	*65%*	*70%*
# patients treated		-	497	2,027	3,618	4,745	5,916	7,131	7,833
Gross Price per patient ('000s)	*1.50%*								
Low pricing			40	41	41	42	42	43	44
Base pricing			80	81	82	84	85	86	87
High pricing			120	122	124	125	127	129	131
Net Price per patient ('000s)	*7%*								
Low pricing		-	37	38	38	39	39	40	41
Base pricing		-	74	76	77	78	79	80	81
High pricing		-	112	113	115	117	118	120	122
Total RoW Sales									
Low pricing assumption		-	18	77	139	185	234	286	319
Base pricing assumption		-	37	153	277	369	467	572	637
High pricing assumption		-	55	230	416	554	701	857	956
Growth				*314%*	*81%*	*33%*	*27%*	*22%*	*11%*
Royalty payable to Baylor Research Institute	5%								
Low pricing assumption		-	1	5	9	12	16	19	21
Base pricing assumption		-	2	10	18	25	31	38	42
High pricing assumption		-	4	15	28	37	47	57	64
Total Net Sales to Ultragenyx									
Low pricing assumption		-	23	97	176	234	296	362	404
Base pricing assumption		-	47	194	351	468	592	724	807
High pricing assumption		-	70	291	527	701	888	1,086	1,211

Source: J.P. Morgan estimates.

RARE currently has patent protection for triheptanoin through 2024 with pending patents that could extend to 2034.

Intellectual Property

Ultragenyx has an exclusive license from Baylor Research Institute relating to its patent portfolio for triheptanoin for the treatment of FAOD and Glut1 DS, among other diseases. More specifically, Ultragenyx licensed 24 issued patents, including 7 in the US, and 8 pending US applications, covering composition, formulation, use and manufacturing of triheptanoin and related odd carbon fatty acids. The terms of the issued patents in the US range from 2020-2024, and the projected terms for the pending US patents are 2020-2034. Ultragenyx plans on seeking orphan drug designation in the US and EU providing 7 and 10 yrs of exclusivity, respectively.

Competitive Landscape

There are currently no approved drugs for the treatment of LC-FAOD or Glut1 DS. The current standard of care for LC-FAOD is diet therapy and medium chain triglycerides. A ketogenic diet and anti-epileptic drugs are commonly administered for Glut1 DS. These treatments could potentially compete with triheptanoin, if approved. However, because they can be used in combination with triheptanoin, we don't foresee substantial market share being threatened by the current standard of care. Although Ultragenyx is developing triheptanoin as a drug therapy, other companies could attempt to sell the product through a nutraceutical or food pathway. As far as we are aware, there are no clinical development efforts underway for competing products for the treatment of LC-FAOD or Glut1 DS. B. Braun Medical Inc. has received orphan drug designation for triheptanoin in Europe, but has not proceeded further in development of triheptanoin.

Partnership/Collaboration Overview

In September 2012, Ultragenyx entered into an exclusive license agreement with Baylor Research Institute for rights to patents and know-how relating to triheptanoin. Under the terms of the agreement, Ultragenyx paid a $250K upfront fee for North American rights then exercised an option for rights outside of North America for an additional fee of $750K. Baylor Research Institute is eligible to receive a mid-single-digit royalty on net sales as well as up to $10.5M for certain development milestones and up to $7.5M for certain sales milestones.

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Sialic Acid Extended Release HIBM

SA-ER is an extended release formulation of sialic acid that is currently being developed to treat patients with HIBM, a genetic disease caused by a defect in the sialic acid biosynthetic pathway.

Product Overview

UX001 (SA-ER) is an extended release, oral formulation of sialic acid, an essential, naturally occurring amino sugar. Ultragenyx is developing SA-ER for the treatment of hereditary inclusion body myopathy (HIBM), or GNE myopathy, a genetic disease caused by a defect in the sialic acid biosynthetic pathway. SA-ER is intended as a substrate replacement to address the sialic acid deficiency and restore muscle function in these patients. Data from a Phase 2 trial in HIBM patients was released in Dec. 2013, and an extension study at a higher dose is currently ongoing with data expected in late 2014.

Disease Background

Hereditary inclusion body myopathy (HIBM), or GNE myopathy, is an autosomal recessive disorder caused by a mutation in the GNE gene (which encodes UDP-N-acetylglucosamine 2-epimerase/N-acetylmannosamine kinase). This enzyme is responsible for the first step in the biosynthesis of sialic acid (SA), and without it patients develop an SA deficiency. SA is needed by many proteins and lipids in the body for normal function, and SA deficiency in the muscle interferes with muscle function, leading to myopathy and atrophy.

HIBM usually presents in the late teens or early adulthood, often as weakness in the legs. The disease is characterized by progressive muscular myopathy, so while the legs are often the first muscles affected, nearly all muscles become progressively weaker over time. Most patients become wheelchair bound 10 to 20 years after disease onset. Interestingly, the quadriceps and certain facial and diaphragm muscles as well as cardiac muscles are often spared from severe disease.

There are currently no approved therapies for the treatment of HIBM, and instead treatment focuses on management of symptoms, e.g., foot drop bracing or other mechanical aids and nocturnal hypoventiliation to treat sleep apnea.

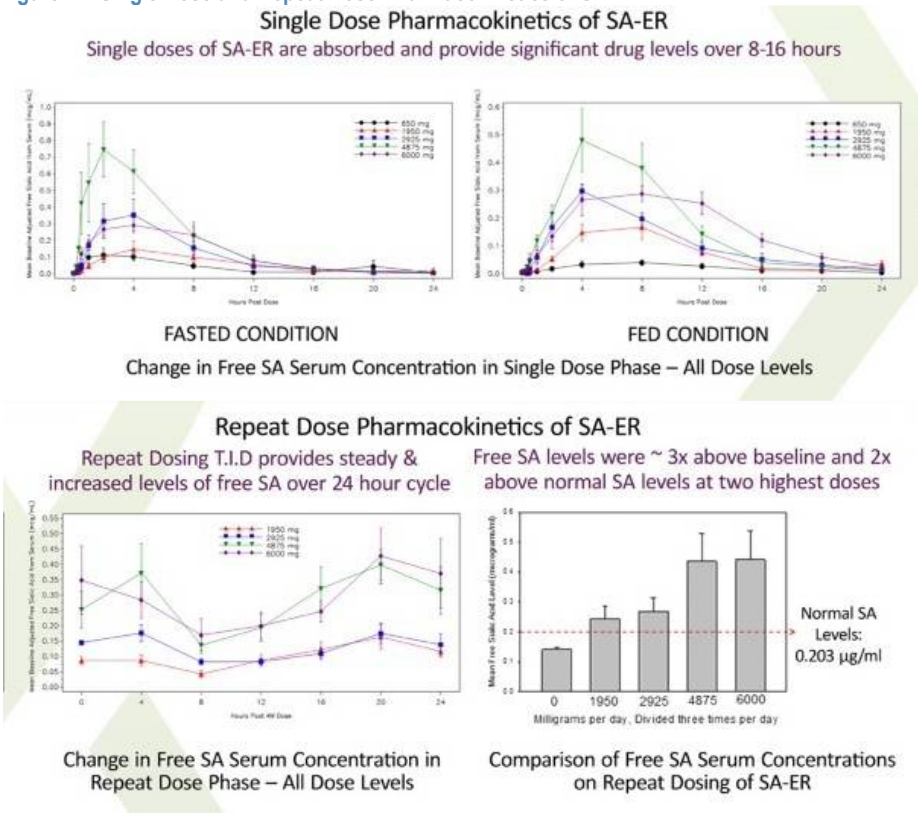
Data Review and Development Status

SA-ER is designed to replace deficient sialic acid and restore muscle function in HIBM patients. While there is some debate as to the underlying pathophysiology of HIBM (some hypothesize the enzyme may have other functions that could contribute to the disease), Ultragenyx believes the sialic acid deficiency is the key result of mutated GNE. Therefore, the company believes sialic acid substrate replacement could have a meaningful effect on disease progression. In support of that hypothesis is animal data showing that sialic acid substrate replacement in mice has beneficial effects on the phenotype, almost eliminating clinical symptoms/pathology.

Ultragenyx conducted a Phase 1 safety/PK study of SA-ER in 26 HIBM patients at doses up to 6g per day. The drug was shown to be safe and relatively well tolerated at the doses evaluated with no SAEs reported during the study. PK results were also promising, showing that the drug was absorbed and provided steady drug levels over a period of 8 to 16 hours post dose (depending on dose level). At the 6g/day dose, dosing with food appeared to extend the elevated drug levels. Additionally, at the higher dose levels, the concentrations of sialic acid were on average 2-3x higher than

normal levels. Repeat dosing TID showed steady and increased levels of free sialic acid over a 24-hour period.

Figure 11: Single Dose and Repeat Dose Pharmacokinetics of SE-ER



Source: Company reports.

Phase 2 data showed trends towards improving muscle strength at the highest dose tested, and an extension study at a higher dose is currently ongoing, with results anticipated in late 2014.

Ultragenyx is currently conducting a Phase 2 trial in 47 patients with HIBM. In the initial blinded phase, patients were randomized to receive either 3g/day, 6g/day or placebo for 24 weeks, after which patients in the placebo arm were randomized and crossed over to receive 3g/day or 6g/day and treatment continued for an additional 24 weeks. Interim data released after 24 weeks of treatment showed a dose dependent improvement in muscle strength (especially in the upper extremities at the higher dose) vs. a decline in muscle strength in placebo treated patients in some muscle groups. The results were statistically significant in some muscle groups and showed trends towards significance in others, though other clinical endpoints did not reveal changes. Importantly, the observed changes were more pronounced in a pre-defined subset of patients with greater walking ability/less severe disease at baseline (greater than 200m in the 6MWT). Creatine kinase levels showed a trend towards improvement in the 6g/day dose group compared to a trend towards worsening in the placebo treated group.

Data at 48 weeks (dose groups pooled to include crossed-over placebo patients) showed a modest increase in a composite of upper extremity muscle strength in the 6g/day group vs. a decline in the 3mg group. The difference was statistically significant. In the 6g group of patients treated for the full 48 weeks, the modest increase in upper extremity strength was maintained from the 24-week time point, whereas continued decline was observed in the 3g patients. Once again, the changes

observed were more pronounced in the subset of patients with less severe disease at baseline. The lower extremity composite did not show a statistically significant difference between the dose groups, but neither showed a significant decline. In a patient reported outcome (GNE Myopathy Functional Activity Scale) designed to asses the clinical meaningfulness of change in function, a trend towards positive effect was seen in the pooled 6g/day group vs. the 3g/day group. The sit-to-stand test also showed a trend towards positive effect, though other clinical endpoints (e.g., 6MWT) did not show a significant change in function (either upwards or downwards). SA-ER continued to be well tolerated, with no SAEs reported. Full 48 week data is expected to be presented at a scientific conference in 2014.

All patients from the initial period of the study opted to roll over into an open-label extension study, where a higher dose will be evaluated. Data from this portion of the trial is expected in late 2014. Ultragenyx is also working to develop pro-drugs of sialic acid (which could potentially have better penetration into muscle) and is also sponsoring a disease monitoring program to gather more information on the natural history of the disease.

Market Opportunity

Ultragenyx estimates there are 1,200-2,000 HIBM patients WW and has identified >800 WW.

Ultragenyx estimates that there are 300-400 HIBM patients in the US and 1,200-2,000 patients worldwide (~400 HIBM patients have been identified in published literature). To date, Ultragenyx has identified >800 HIBM patients worldwide, with >300 of these patients in the US. HIBM is prevalent in the Persian Jewish population (the disease is estimated in 1/1,600 persons of Persian Jewish descent) and patients of Asian Indian, European, Chinese, Japanese, Korean, and Middle Eastern descent have also been identified.

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Our model assumes a WW prevalence of 1,200 pts and a 65% peak penetration rate, giving peak WW revenues of $125M.

Revenue Build

We estimate ~$125 million in worldwide peak sales for SA-ER in HIBM. We assume a worldwide patient population of 1,200, which grows slightly year over year. In this patient population, we then assume a peak penetration of 65% in both the US and ex-US, five years post launch. Regarding price, we assume a base case price of $120K per patient. With a more conservative pricing assumption of $60K per patient, we achieve peak sales of ~$60 million, and with a more aggressive pricing assumption of $180K per patient, we achieve peak sales of ~$175 million. We assume a 2018 launch for SA-ER, but this is contingent upon signs of increased efficacy in the Phase 2 dose escalating extension study currently underway, successful progression through pivotal studies, and ultimately approval.

Figure 14: Our SA-ER Revenue Build for HIBM

		2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E
HIBM Prevalence worldwide									
	growth								
HIBM Prevalence worldwide	*2%*	1,299	1,325	1,351	1,378	1,406	1,434	1,463	1,492
US HIBM									
US HIBM Population	*2%*	325	331	338	345	351	359	366	373
Penetration			*10%*	*25%*	*40%*	*55%*	*65%*	*65%*	*65%*
# patients treated		-	33	84	138	193	233	238	242
Gross Price per patient ('000s)	*1.50%*								
Low pricing			60	61	62	63	64	65	66
Base pricing			120	122	124	125	127	129	131
High pricing			180	183	185	188	191	194	197
Net Price per patient ('000s)	*7%*								
Low pricing		-	56	57	57	58	59	60	61
Base pricing		-	112	113	115	117	118	120	122
High pricing		-	167	170	172	175	178	180	183
Total US Sales									
Low pricing assumption		-	2	5	8	11	14	14	15
Base pricing assumption		-	4	10	16	23	28	29	30
High pricing assumption		-	6	14	24	34	41	43	44
Growth				*159%*	*66%*	*42%*	*22%*	*4%*	*4%*
RoW HIBM									
RoW HIBM Population	*2%*	974	994	1,014	1,034	1,054	1,076	1,097	1,119
Penetration			*10%*	*25%*	*40%*	*55%*	*65%*	*65%*	*65%*
# patients treated		-	99	253	414	580	699	713	727
Gross Price per patient ('000s)	*1.50%*								
Low pricing			60	61	62	63	64	65	66
Base pricing			120	122	124	125	127	129	131
High pricing			180	183	185	188	191	194	197
Net Price per patient ('000s)	*7%*								
Low pricing		-	56	57	57	58	59	60	61
Base pricing		-	112	113	115	117	118	120	122
High pricing		-	167	170	172	175	178	180	183
Total RoW Sales									
Low pricing assumption		-	6	14	24	34	41	43	44
Base pricing assumption		-	11	29	48	68	83	86	89
High pricing assumption		-	17	43	71	102	124	129	133
Growth				*159%*	*66%*	*42%*	*22%*	*4%*	*4%*
Royalty to Nobelpharma	*5%*								
Low pricing assumption		-	0	1	2	2	3	3	3
Base pricing assumption		-	1	2	3	5	6	6	6
High pricing assumption		-	1	3	5	7	8	9	9
Total Net Sales to Ultragenyx									
Low pricing assumption		-	7	18	30	43	52	54	56
Base pricing assumption		-	14	36	60	86	105	109	112
High pricing assumption		-	21	55	90	129	157	163	169

Source: J.P. Morgan estimates

Our model assumes patent protection through 2028.

Intellectual Property

Ultragenyx has 10 pending patent applications in the US and owns patents/patent applications in other jurisdictions related to sialic acid for the treatment of HIBM, biomarkers useful for the treatment of HIBM, and the extended release formulation of sialic acid. SA-ER also has orphan drug designation in the US and EU.

Competitive Landscape

Although there are no approved drug therapies for the treatment of HIBM, there is a competing study at the National Institutes of Health evaluating N-acetyl mannosamine (ManNAc), another metabolite in the sialic acid pathway, for the treatment of HIBM. New Zealand Pharma licensed the program from the National Institutes of Health. ManNAc, a precursor compound for the biosynthesis of the sialic acids, completed a randomized, placebo-controlled, double-blind, escalating single-dose Phase 1 trial which evaluated how ManNAc is absorbed into/removed from the blood, as well as safety/tolerability.

Partnership/Collaboration Overview

RARE licensed WW rights relating to sialic acid (ex-Asia/Japan) from Nobelpharma, and has an exclusive WW license from AAI Pharma for patents and knowhow related to extended release versions of sialic acid.

Ultragenyx entered into a license and collaboration agreement with Nobelpharma for patents and know-how (includes a third-party patent) relating to sialic acid. Nobelpharma retains the rights to Japan and other countries in Asia, and Ultragenyx has rights for the rest of the world. Development efforts for SA-ER are conducted independently and Nobelpharma is responsible for supplying sialic acid API to Ultragenyx. As for the financial terms of the agreement, Ultragenyx paid an upfront fee of ~$110K, ~$495K in development milestones, and issued 76,567 shares of common stock to Nobelpharma. Ultragenyx is eligible to receive a mid-single-digit royalty on net sales from Nobelpharma's licensed territories in Asia (excluding Japan), and Ultragenyx is obligated to pay a high-single-digit royalty on rest of world net sales and a ~$2M approval milestone.

Ultragenyx has an exclusive license with AAI Pharma to patents and know-how relating to extended release formulations of sialic acid for HIBM or distal myopathy with rimmed vacuoles. This agreement is royalty-free; however, Ultragenyx is obligated to pay a mid-single-digit royalty related to any sublicense.

RARE also has an exclusive WW license agreement with HIBM Research Group related to the use of sialic acid for the treatment of HIBM.

Ultragenyx also has an exclusive worldwide license agreement with HIBM Research Group to patents and know-how relating to the use of sialic acid in treating HIBM and related conditions using substrate replacement therapy. Under the terms of the agreement, Ultragenyx paid a $25K upfront fee and HIBM Research Group is eligible to receive a royalty of <1% of net sales and up to $300K of development and approval milestones.

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rhPPCA for Galactosialidosis

UX004 is an ERT that is currently in preclinical development for the treatment of galactosialidosis, a disease similar to MPS.

Product Overview

UX004 (recombinant human protective protein cathepsin-A, rhPPCA) is an enzyme replacement therapy being developed for the treatment of galactosialidosis, a rare autosomal recessive lysosomal storage disease. Galactosialidosis is similar to MPS in that the disease afflicts both soft tissue storage in the liver, spleen, and other tissues, as well as connective tissue (bone and cartilage). Also, as with MPS 7, an enzyme deficiency causes a buildup of substrates (oligosaccharides, or short sugar chains) in the lysosomes, leading to skeletal and organ dysfunction, and death. rhPPCA is currently in preclinical development, which Ultragenyx plans to continue during 2014.

Disease Background

In galactosialidosis, cathepsin A (called a "protective protein," or PPCA), is missing. This protein typically functions as a lysosomal stabilizing agent for two other enzymes, sialidase and beta-galactosidase. The deficiency in the PPCA enzyme causes a buildup of oligosaccharides, or short sugar chains, which causes bone and organ dysfunction and can lead to death. There are three types of galactosialidosis disease, which are categorized based on time of onset. Type I (early infantile) occurs between birth and 3 months old, with one year being the average age of death. Type II (late infantile) occurs within the first few months of life and patient life expectancy can vary depending on the severity of symptoms (patients still alive in their early 20s have been reported). Type III (juvenile/adult) typically occurs at about 16 years of age, although symptoms can widely vary. About 70-80% of galactosialidosis patients have Type II and III, with reports of Type III mostly occurring in people of Japanese descent.

RARE plans to conduct preclinical POC studies in 2014/2015, and rhPPCA could enter the clinic in 2015/2016.

Data Review and Development Status

In vitro and mouse model studies have shown that the replacement of PPCA leads to a reduction of storage of oligosaccharides in multiple organs. Ultragenyx plans on conducting proof-of-concept preclinical studies in animal models during 2014-2015. If the results of these studies are positive, clinical studies of rhPPCA may begin in 2015/2016.

Market Opportunity

Ultragenyx estimates that there are ~300-500 galactosialidosis patients in the developed world (100 patients identified in published literature). We do not yet ascribe any value to rhPPCA due to the early stage nature of the drug.

Intellectual Property

Ultragenyx does not currently have issued patents or applications in process for rhPPCA but plans to file patent applications for compositions with specific characteristics useful in enzyme replacement therapies for the potential treatment of autosomal recessive lysosomal storage disease. Ultragenyx also plans to file patents related to certain aspects of its therapy, such as formulation, manufacturing process, dosage, and potentially other aspects of the rhPPCA therapy. In addition to the patent portfolio, Ultragenyx intends to seek orphan drug designation in the US and EU.

Competitive Landscape

There are currently no approved therapies for galactosialidosis, and as far as we are aware, no competing compounds in clinical development. Potential competing therapies for galactosialidosis are bone marrow and stem cell transplants, which have been used in other lysosomal storage diseases.

Partnership/Collaboration Overview

In September 2012, Ultragenyx licensed exclusive rights to IP and know-how related to rhPPCA for galactosialidosis and other monogenetic diseases from St. Jude Children's Research Hospital. Under the terms of the agreement, Ultragenyx paid a $10K up-front fee and St. Jude is eligible to receive a royalty of <1% of net sales.

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Financial Outlook

Ultragenyx is a development-stage biotechnology company with five programs expected to be in Phase 2 clinical studies by early 2014. The company does not expect to be profitable for the foreseeable future, and we currently do not anticipate profitability until 2019 (two years post first commercial launch).

We think RARE is well positioned, with cash through ~2015.

Ultragenyx Ended 3Q13 with $64 Million in Cash

The company's cash, cash equivalents, and marketable securities totaled ~$64 million as of September 30, 2013. However, this does not include ~$126 million of net proceeds from an initial public offering of common stock in February 2014. Including this capital, we estimate Ultragenyx will end 2014 with ~$125 million in cash. While R&D expense may increase slightly over time, the uptick should be modest, as the trials are going to be relatively small. As for SG&A, we expect this to ramp up as the company gets closer to commercialization. However, the cost of building a commercial infrastructure is not expected to be large, as rare disease sales forces are generally concentrated on a small number of specialty physicians.

Share Count

We estimate Ultragenyx currently has 36.2 million fully diluted shares outstanding (including 29.9 million common shares, 1.5 million stock options, 0.4 million warrants, and 4.4 million shares from equity incentive and stock purchase plans).

Figure 13: RARE Key Financial Metrics

Key Financial Metrics	2011A	2012A	2013E	2014E	2015E	2016E	2017E	2018E
In $ M								
December financial year-end								
Cash	10.6	86.2	56.5	127.2	205.8	114.3	154.0	130.6
Debt	-	-	-	-	-	-	-	-
CFOp + CapEx (burn)		(13.6)	(29.7)	(55.7)	(71.4)	(91.6)	(110.4)	(23.4)
Expected financing		-	-	126.4	150.0	-	150.0	-
Revenue		-	-	-	-	-	0.1	103.5
EPS		(2.04)	($1.22)	($1.60)	($1.82)	($2.27)	($2.47)	($0.57)
Average shares outstanding		9.9	23.3	29.9	34.2	35.2	39.5	40.5
Fully diluted shares outstanding		9.9	29.6	36.2	40.5	41.5	45.8	46.8

Source: Company reports and J.P. Morgan estimates.

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Valuation

We are initiating coverage at OW with a Dec14 price target of $66.

We are initiating coverage of RARE with an Overweight rating and a December 2014 price target of $66. Our target is based on a blended average of our proprietary probability-adjusted scenario analysis (50%) and a risk-adjusted NPV model (50%) We also believe that a comparable company analysis supports upside from current levels.

Figure 14: RARE Valuation Summary

Ultragenyx Valuation Summary

Discount rate	10.0%		
4Q14 Fully Diluted Shares (mm)	36.2		
		Peak WW sales est	
Main value drivers	Prob of approval	(avg. scenario)	Avg peak yr
KRN23	65%	$ 275	2024
rhGUS	75%	$ 75	2022
Tripheptanoin (FAOD)	45%	$ 300	2024
Tripheptanoin (Glut1 DS)	45%	$ 825	2024
SA-ER	30%	$ 125	2022

Valuation methodology	Value / share	Weighting	Adj. value/ share
Real options scenario analysis	$ 69.35	50%	34.68
Risk adjusted NPV analysis	$ 62.95	50%	31.48
Total		100%	$ 66.15
Catalyst/liquidity discount			0%
YE14 Price Target			**$ 66**

Source: J.P. Morgan estimates.

Proprietary Real Options SOTP Scenario Analysis (50% weighting)

Using this model, we estimate the value of the company's development programs by assigning a range of probabilities to six different commercial scenarios (ranging from an ineffective product that generates zero value to a breakthrough treatment option) and analyze them over several possible peak sales years (which we assume take longer to reach for orphan disease products). We also evaluate a range of price-to-peak sales/royalty multiples for each asset (biologics and longer-tailed assets earn higher multiples than easier to copy/manufacture small molecules). Additionally, we apply a discount rate of 10%, which we believe is appropriate given the prior probability adjustments.

Value contribution of each product

Below, we demonstrate our analysis for each product's contribution to our overall valuation. We assume a 65% probability that KRN23 will reach the market, 75% probability for rhGUS, 45% probability for triheptanoin, and a 30% probability for SA-ER. Below is our calculated value contribution from each product for a range of multiples if the drugs generate peak sales of ~$800 million for KRN23, ~$70 million for rhGUS, ~$300 million for triheptanoin in FAOD, ~$850 million for triheptanoin in Glut1, and ~$125 million for SA-ER.

Figure 15: RARE Scenario Analysis

Product:	KRN23			Peak year	2023			2024			2025			Average
Indication:	XLH			Discount period	9.0			10.0			11.0			prob-adj
Market:	WW													value
Ownership:	Partnered			Price/sales mult.	6	8	10	6	8	10	6	8	10	/share
	Prob.	Peak sales (millions)	Peak royalties (millions, 100%)				Value/share							
Ineffective	35%	$ -	$ -		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -
Disappointment	2%	133	$ 133		$ 9.3	$ 12.4	$ 15.6	$ 8.5	$ 11.3	$ 14.1	$ 7.7	$ 10.3	$ 12.9	0.23
Below average	4%	199	$ 199		$ 14.0	$ 18.7	$ 23.3	$ 12.7	$ 17.0	$ 21.2	$ 11.6	$ 15.4	$ 19.3	0.68
Average	35%	266	$ 266		$ 18.7	$ 24.9	$ 31.1	$ 17.0	$ 22.6	$ 28.3	$ 15.4	$ 20.6	$ 25.7	7.9
Above average	22%	398	$ 398		$ 28.0	$ 37.3	$ 46.7	$ 25.4	$ 33.9	$ 42.4	$ 23.1	$ 30.8	$ 38.6	7.5
Breakthrough	2%	531	$ 531		$ 37.3	$ 49.8	$ 62.2	$ 33.9	$ 45.2	$ 56.6	$ 30.8	$ 41.1	$ 51.4	0.9
Total	100%													$ 17.2

Product:	rhGUS			Peak year	2021			2022			2023			Average
Indication:	MPS 7			Discount period	7.0			8.0			9.0			prob-adj
Market:	WW													value
Ownership:	Unpartnered			Price/sales mult.	4	5	6	4	5	6	4	5	6	/share
	Prob.	Peak sales (millions)	Peak royalties (millions, 100%)				Value/share							
Ineffective	25%	$ -	$ -		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -
Disappointment	5%	50	$ 50		$ 2.8	$ 3.5	$ 4.2	$ 2.6	$ 3.2	$ 3.9	$ 2.3	$ 2.9	$ 3.5	0.16
Below average	15%	61	$ 61		$ 3.4	$ 4.3	$ 5.1	$ 3.1	$ 3.9	$ 4.7	$ 2.8	$ 3.5	$ 4.3	0.59
Average	30%	71	$ 71		$ 4.0	$ 5.0	$ 6.1	$ 3.7	$ 4.6	$ 5.5	$ 3.3	$ 4.2	$ 5.0	1.4
Above average	20%	125	$ 125		$ 7.1	$ 8.8	$ 10.6	$ 6.4	$ 8.0	$ 9.6	$ 5.8	$ 7.3	$ 8.8	1.6
Breakthrough	5%	178	$ 178		$ 10.1	$ 12.6	$ 15.1	$ 9.2	$ 11.5	$ 13.8	$ 8.3	$ 10.4	$ 12.5	0.6
Total	100%													$ 4.3

Product:	Tripheptanoin (FAOD)			Peak year	2023			2024			2025			Average
Indication:	LC-FAOD			Discount period	9.0			10.0			11.0			prob-adj
Market:	WW													value
Ownership:	Unpartnered			Price/sales mult.	3	4	5	3	4	5	3	4	5	/share
	Prob.	Peak sales (millions)	Peak royalties (millions, 100%)				Value/share							
Ineffective	55%	$ -	$ -		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -
Disappointment	3%	74	$ 74		$ 2.6	$ 3.5	$ 4.3	$ 2.4	$ 3.1	$ 3.9	$ 2.1	$ 2.9	$ 3.6	0.09
Below average	5%	148	$ 148		$ 5.2	$ 6.9	$ 8.7	$ 4.7	$ 6.3	$ 7.9	$ 4.3	$ 5.7	$ 7.2	0.32
Average	25%	296	$ 296		$ 10.4	$ 13.9	$ 17.3	$ 9.4	$ 12.6	$ 15.7	$ 8.6	$ 11.5	$ 14.3	3.2
Above average	10%	444	$ 444		$ 15.6	$ 20.8	$ 26.0	$ 14.2	$ 18.9	$ 23.6	$ 12.9	$ 17.2	$ 21.5	1.9
Breakthrough	2%	592	$ 592		$ 20.8	$ 27.7	$ 34.6	$ 18.9	$ 25.2	$ 31.5	$ 17.2	$ 22.9	$ 28.6	0.5
Total	100%													$ 6.0

Product:	Tripheptanoin (Glut1 DS)			Peak year	2023			2024			2025			Average
Indication:	Glut1 D			Discount period	9.0			10.0			11.0			prob-adj
Market:	WW													value
Ownership:	Unpartnered			Price/sales mult.	3	4	5	3	4	5	3	4	5	/share
	Prob.	Peak sales (millions)	Peak royalties (millions, 100%)				Value/share							
Ineffective	55%	$ -	$ -		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -
Disappointment	3%	209	$ 209		$ 7.3	$ 9.8	$ 12.2	$ 6.7	$ 8.9	$ 11.1	$ 6.1	$ 8.1	$ 10.1	0.27
Below average	10%	418	$ 418		$ 14.7	$ 19.6	$ 24.5	$ 13.3	$ 17.8	$ 22.2	$ 12.1	$ 16.2	$ 20.2	1.78
Average	25%	836	$ 836		$ 29.4	$ 39.1	$ 48.9	$ 26.7	$ 35.6	$ 44.5	$ 24.3	$ 32.3	$ 40.4	8.9
Above average	5%	1,254	$ 1,254		$ 44.0	$ 58.7	$ 73.4	$ 40.0	$ 53.4	$ 66.7	$ 36.4	$ 48.5	$ 60.6	2.7
Breakthrough	2%	1,671	$ 1,671		$ 58.7	$ 78.3	$ 97.8	$ 53.4	$ 71.2	$ 89.0	$ 48.5	$ 64.7	$ 80.9	1.4
Total	100%													$ 15.1

Product:	SA-ER			Peak year	2021			2022			2023			Average
Indication:	HIBM			Discount period	7.0			8.0			9.0			prob-adj
Market:	WW													value
Ownership:	Unpartnered			Price/sales mult.	3	4	5	3	4	5	3	4	5	/share
	Prob.	Peak sales (millions)	Peak royalties (millions, 100%)				Value/share							
Ineffective	70%	$ -	$ -		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -
Disappointment	2%	58	$ 58		$ 2.5	$ 3.3	$ 4.1	$ 2.2	$ 3.0	$ 3.7	$ 2.0	$ 2.7	$ 3.4	0.06
Below average	3%	87	$ 87		$ 3.7	$ 4.9	$ 6.2	$ 3.4	$ 4.5	$ 5.6	$ 3.1	$ 4.1	$ 5.1	0.14
Average	15%	116	$ 116		$ 4.9	$ 6.6	$ 8.2	$ 4.5	$ 6.0	$ 7.5	$ 4.1	$ 5.5	$ 6.8	0.9
Above average	5%	233	$ 233		$ 9.9	$ 13.2	$ 16.5	$ 9.0	$ 12.0	$ 15.0	$ 8.2	$ 10.9	$ 13.6	0.6
Breakthrough	5%	349	$ 349		$ 14.8	$ 19.8	$ 24.7	$ 13.5	$ 18.0	$ 22.5	$ 12.3	$ 16.4	$ 20.4	0.9
Total	100%													$ 2.6

Source: J.P. Morgan estimates.

Risk-Adjusted NPV Analysis (50% weighting)

In our risk-adjusted NPV analysis, we estimate the revenues and associated expenses (including taxes) over the expected patent life of a product. We complete this exercise for conservative, base case, and aggressive sales scenarios and then assign a range of probabilities to each of these outcomes as well as to the possibility that the product is ineffective and generates zero value. We assume patent protection until 2029 for KRN23, 2027 for rhGUS (RARE is in the process of filing patent applications for rhGUS; we assume patent protection for ten years after launch although we could argue that this is overly conservative), 2024 in the US and 2027 in the EU for Triheptanoin for FAOD and 2025 in the US and 2028 in the EU for Triheptanoin for Glut1 (driven by orphan exclusivity), and 2025 in the US and 2028 in the EU for SA-ER. We apply a discount rate of 10% and believe this is appropriate given the applied probability adjustments.

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What's the value of human capital and a diversified orphan disease business model?

This is arguably the most difficult aspect of valuing a stock such as RARE. What is clear is that many investors ascribe significant value to the team in place (CEO Emil Kakkis, in particular) in addition to the company's business model, which we believe is likely to continue accumulating additional orphan disease assets in the future. For example, we look to the recent history with BioMarin as a potential proxy. Many of the key value drivers today for BMRN were completely unknown to investors just a few years ago. We suspect this type of pipeline evolution is possible for Ultragenyx as well. After all, the company has already in-licensed five drug candidates for six indications in its brief history (going on four years). In an attempt to take this into account, we value RARE's orphan disease model and long-term pipeline (in both our proprietary probability-adjusted scenario analysis and risk-adjusted NPV model) at $500M and $250M, respectively.

Comp Analysis

For perspective, we've also looked at RARE relative to other publicly traded orphan disease companies, which we believe supports additional upside.

Figure 12: Trading Comparables ($ in millions, except per share data)

Ticker	Company	Price	% of 52-week high	Market Cap	Cash	EV	Key product / indication	Phase of development
		2/24/2014						
BMRN	Biomarin	**$82.07**	**98.9%**	**$11,671**	**$380**	**$11,370**	Aldurazyme / MPS1	**Marketed**
ALNY	Alnylam	**$87.68**	**77.9%**	**$5,604**	**$350**	**$5,254**	ALN-TTR02 / TTR Amyloidosis	**Phase 2**
ICPT	Intercept	**$373.12**	**75.1%**	**$7,214**	**$157**	**$7,058**	Obeticholic acid / Primary biliary cirrhosis	**Phase 3**
NPSP	NPS	**$39.06**	**98.8%**	**$4,015**	**$180**	**$3,984**	Gattex / Adult PN-dependent short bowel syndrome	**Marketed**
GEVA	Synageva	**$112.02**	**99.1%**	**$3,443**	**$435**	**$3,008**	Sebelipase alfa / Lysosomal acid lipase deficiency	**Phase 3**
AEGR	Aegerion	**$68.40**	**67.7%**	**$2,000**	**$126**	**$1,883**	Juxtapid / Hypercholesterolemia	**Marketed**
ITMN	Intermune	**$13.96**	**75.9%**	**$1,252**	**$429**	**$1,087**	Esbriet / IPF	**Ph 3 / Mkt**
RPTP	Raptor	**$16.67**	**94.1%**	**$1,022**	**$88**	**$983**	Procysbi / Nephropathic cystinosis	**Marketed**
SRPT	Sarepta	**$30.05**	**54.0%**	**$1,129**	**$274**	**$857**	Eteplirsen / Duchenne Muscular Dystrophy	**Phase 2b**
PTC	PTC Therapeutics	**$38.68**	**98.7%**	**$4,614**	**$260**	**$4,354**	Ataluren / DMD	**Phase 3**
HPTX	Hyperion	**$27.71**	**92.5%**	**$557**	**$109**	**$458**	Ravicti / Urea Cycle Disorders	**Marketed**
BLUE	bluebird bio	**$24.25**	**66.9%**	**$577**	**$217**	**$360**	Lenti-D / CCALD	**Phase 2/3**
	Mean		**83.3%**	**$3,592**	**$250**	**$3,388**		
	Median		**85.2%**	**$2,722**	**$238**	**$2,445**		
RARE	Ultragenyx	**$58**	**92.8%**	**$1,736**	**$186**	**$1,550**	UX023 (KRN23) / XLH	**Phase 2**

Source: Company reports and Bloomberg.

Management

The CEO, as well as other management team members, posses strong expertise in the rare disease space, deep relationships with the medical community and FDA, as well as an excellent reputation with investors.

Emil Kakkis, MD, PhD – Chief Executive Officer

Emil Kakkis is Ultragenyx' founder and has served as President and Chief Executive Officer and as a member of the board of directors since inception in April 2010. Prior to Ultragenyx, Dr. Kakkis served in various executive capacities, and ultimately as Chief Medical Officer, at BioMarin, from September 1998 to February 2009. Dr. Kakkis also serves as President and Founder of EveryLife Foundation for Rare Diseases, a non-profit organization he started in 2009 to accelerate biotechnology innovation for rare diseases. Dr. Kakkis is board certified in both Pediatrics and Medical Genetics. He holds a B.A. in Biology from Pomona College and received combined M.D. and Ph.D. degrees from the UCLA School of Medicine's Medical Scientist Training Program and received the Bogen prize for his research.

Tom Kassberg – Chief Business Officer

Thomas Kassberg has served as Chief Business Officer and Senior Vice President since November 2011. Prior to Ultragenyx, Mr. Kassberg worked as Vice President of Business Development at Corium International from July 2010 to October 2011. Prior to Corium International, Mr. Kassberg worked as an independent consultant in Corporate Development and Business Strategy and consulted with a number of companies from March 2009 to June 2010, including Corium International and Rib-X Pharmaceuticals. Before becoming a consultant, Mr. Kassberg worked at Proteolix, from January 2008 until February 2009, where he served as Senior Vice President of Corporate Development. Mr. Kassberg holds a B.A. in Business Administration from Gustavus Adolphus College and an M.B.A. from Northwestern University.

Shalini Sharp – Chief Financial Officer

Shalini Sharp has served as Chief Financial Officer and Senior Vice President since May 2012. Prior to Ultragenyx, Ms. Sharp served in various executive capacities, and ultimately as Chief Financial Officer, of Agenus, from August 2003 to May 2012. Prior to Agenus, Ms. Sharp held strategic planning and corporate finance roles and ultimately served as chief of staff to the chairman of the board at Elan, from August 1998 to August 1999 and September 2001 to August 2003. Prior to Elan, Ms. Sharp was a management consultant at McKinsey & Company and an investment banker at Goldman Sachs, specializing in pharmaceuticals and medical devices. Ms. Sharp has also served as a board member of Agenus since May 2012. Ms. Sharp holds a B.A. and an M.B.A. from Harvard University.

Steve Jungles – SVP Technical Operations

Steven Jungles has served as Senior Vice President, Technical Operations since August 2011. Prior to Ultragenyx, Mr. Jungles worked as Vice President, Supply Chain at BioMarin, from June 1999 to July 2011, was Associate Director of Operations at Harvard Gene Therapy Initiative from June 1997 until June 1999, and worked at Somatix Therapy Corporation, a research and development company in the

field of gene therapy that was acquired by Cell Genesys, from March 1993 to May 1997. Mr. Jungles holds a B.S. in Biology from the University of Iowa.

Cori Leonard – VP, Regulatory Affairs

Cordelia Leonard has served as Vice President, Regulatory Affairs and Quality Assurance since July 2011. Prior to Ultragenyx, Ms. Leonard was Senior Director, Regulatory Affairs at BioMarin, from October 2003 to July 2011. Prior to BioMarin, Ms. Leonard was the Manager, Regulatory Affairs at Cerus Corporation, from May 1999 to October 2003. Ms. Leonard received bachelor degrees in Chemistry and Biological Science from the University of California, Irvine and holds both U.S. and EU Regulatory Affairs Certifications.

Vimal Srivastava – VP, Program Development

Vimal Srivastava has served as Vice President, Program Development since August 2011. Before joining Ultragenyx, Mr. Srivastava was Senior Director, Portfolio and Project Management at Elan/Janssen Alzheimer Immunotherapy, from January 2008 to August 2011. He was also Director, Global Program Manager, Diabetes at Amgen, from September 2005 to January 2008 and Director, Program Management at BioMarin, from March 2003 to September 2005. Mr. Srivastava holds a B.S. in Pharmacy from Banaras Hindu University, an M.S. in Medicinal Chemistry from St. John's University and an M.A.S. in Management from Johns Hopkins University.

John Ditton – VP, Commercial Planning

John Ditton has served as Vice President, Commercial Planning since April 2011. Prior to Ultragenyx, Mr. Ditton was the Chief Operating Officer at EveryLife Foundation for Rare Diseases, from January 2009 to April 2011. Prior to working at the EveryLife Foundation, Mr. Ditton served as the Vice President of Marketing at Diamics, a maker of cancer diagnostics, from October 2006 to December 2008 and Director of Global Marketing at BioMarin, from March 2004 to March 2006. Mr. Ditton holds an M.B.A. from the University of Tasmania.

Michael Vellard, PhD – VP Research

Michael Vellard, Ph.D. has served as Vice President, Research since May 2013. Prior to joining Ultragenyx, Dr. Vellard worked as Head of Lysosomal Biology at BioMarin, from October 1999 to May 2013. He was a postdoctoral fellow in the pediatric department at UCLA Harbor Medical Center from September 1992 to June 1995. Dr. Vellard received his B.S. in Natural and Life Sciences and M.S. in Molecular and Cellular Genetics from the University of Lyon I, France. He obtained his Ph.D. in Virology from the Pasteur and Curie Institutes (Universities Paris VI, VII and XI), France.

Tony Koutsoukos, PhD – VP Biometrics

Tony Koutsoukos, Ph.D. has served as Vice President of Biometrics since October 2013. Prior to Ultragenyx, Mr. Koutsoukos worked as Vice President of Biometrics at Allos Therapeutics, from September 2007 to March 2013, which was acquired by Spectrum Pharmaceuticals. He was also Director of Biostatistics at Amgen, from May 2002 to September 2007. Prior to Amgen, Mr. Koutsoukos spent three years at Quintiles, a contract research company, as a Director of Biostatistics. His experience also includes five years at the FDA, Center for Drugs Evaluation and Research division and approximately four years at the National Cancer Institute, Biometric

Research Branch, CTEP, DCT. Dr. Koutsoukos received his Ph.D. and M.A., both in Mathematical Statistics from the University of Maryland, College Park.

Alison Skrinar, PhD – Sr. Director of Clinical Sciences

Alison Skrinar, Ph.D. has served as Senior Director, Clinical Sciences since March 2012. Prior to joining Ultragenyx, Dr. Skrinar worked as the Senior Director of Clinical Outcomes and Regulatory Affairs from February 2009 to February 2012 at Enobia Pharma, a private clinical stage orphan company focused on the development of an enzyme replacement therapy for hypophosphastasia, which was acquired by Alexion. Prior to Enobia Pharma, Dr. Skrinar was the Senior Director of Clinical Outcomes at Genzyme, from May 2001 to January 2009. In her close to 15 years in the biotechnology industry, Dr. Skrinar has worked exclusively on the clinical development and regulatory approval of ultra-orphan drugs. Dr. Skrinar received a B.B.A. from Emory University and a Ph.D. and a Master of Public Health degree from the University of Alabama.

Spencer Guthrie – Sr. Director of Clinical Operations

Spencer Guthrie has served as Senior Director, Clinical Operations since June 2012. Prior to Ultragenyx, Mr. Guthrie worked as Director of Clinical Operations and Project Team Leader at Elan Pharmaceuticals, and Janssen Alzheimer's Immunotherapy from September 2007 to June 2012. Prior to Elan and Janssen, Mr. Guthrie spent nine years with increasing responsibilities at Genentech in Clinical Operations and Market Planning. At Genentech, he worked on several innovative clinical programs, IND and BLA filings with Rituxan, Avastin, and Lucentis, including work on orphan indications. Mr. Guthrie also spent two years at ICON Clinical Research and a year at NASA's space science lab. Mr. Guthrie received his B.A. in Neuroscience from Vanderbilt University, an M.B.A. from the University of California, Irvine and he is certified as a Project Management Professional.

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Models

Figure 16: RARE Income Statement

	2011A	2012A	1Q13A	2Q13A	3Q13A	4Q13E	2013E	2014E	2015E	2016E	2017E	2018E
Total Product Revenue												
UX003 (rhGUS)									-	-	7.8	24.0
UX007 (FAOD)									-	-	3.5	57.8
UX007G (Glut-1)									-	-	-	49.3
UX023 / KRN23 (XLH)									-	-	-	47.7
UX001 (SA-ER)												
Total Product Revenue									**-**	**-**	**11.3**	**178.9**
Net Revenue to Ultragenyx												
UX003 (rhGUS)									-	-	7.8	23.3
UX007 (FAOD)									-	-	3.3	54.9
UX007G (Glut-1)									-	-	-	46.8
UX023 / KRN23 (XLH) - Profit Share							-	-	-	-	-	15.8
UX001 (SA-ER)												
License & Milestone		-								-	(10.6)	(2.0)
Total Revenues	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**0.5**	**138.9**
COGS										-	1.9	17.1
R&D	4.7	12.6	5.7	7.2	6.8	6.5	26.2	42.0	57.1	70.2	70.3	75.2
SG&A	1.8	3.3	1.1	1.0	1.0	1.0	4.2	15.9	16.7	24.0	41.3	41.7
Total Operating Expenses	**6.6**	**16.0**	**6.7**	**8.2**	**7.8**	**7.6**	**30.3**	**57.9**	**73.8**	**94.2**	**113.5**	**133.9**
Operating Income	**(6.6)**	**(16.0)**	**(6.7)**	**(8.2)**	**(7.8)**	**(7.6)**	**(30.3)**	**(57.9)**	**(73.8)**	**(94.2)**	**(113.0)**	**5.0**
Net interest & other income	(0.3)	(0.3)	0.0	(0.3)	(0.7)	(0.3)	(1.3)	-	-	-	-	-
Profit (Loss) before Income Tax	(6.8)	(16.3)	(6.7)	(8.6)	(8.4)	(7.9)	(31.7)	(57.9)	(73.8)	(94.2)	(113.0)	5.0
Income Tax benefit (expense)	-	-	-	-	-	-	-	-	-	-	-	(1.5)
Net Income	**(6.8)**	**(16.3)**	**(6.7)**	**(8.6)**	**(8.4)**	**(7.9)**	**(31.7)**	**(57.9)**	**(73.8)**	**(94.2)**	**(113.0)**	**3.5**
Accretion and dividends on convertible preferred stock	(0.6)	(4.0)	(1.1)	(1.1)	(1.1)	(1.1)	(4.3)					
Net Income attributable to common stockholders	**(7.5)**	**(20.3)**	**(7.8)**	**(9.7)**	**(9.5)**	**(9.0)**	**(35.9)**	**(57.9)**	**(73.8)**	**(94.2)**	**(113.0)**	**3.5**
Basic EPS	**(4.62)**	**(2.04)**			**(0.41)**	**(0.39)**	**(1.54)**	**(1.93)**	**(2.16)**	**(2.67)**	**(2.86)**	**0.09**
Diluted EPS	**(4.62)**	**(2.04)**			**(0.41)**	**(0.30)**	**(1.22)**	**(1.60)**	**(1.82)**	**(2.27)**	**(2.47)**	**0.07**

Source: Company reports and J.P. Morgan estimates.

Figure 17: RARE Balance Sheet

Ultragenyx Balance Sheet ($ millions)

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	2011A	2012A	2013E	2014E	2015E	2016E	2017E	2018E
Assets								
Cash and cash equivalents	10.6	86.2	56.5	127.2	205.8	114.3	154.3	161.0
Marketable Securities	-	-	-	-	-	-	-	-
Receivables due from related party	0.1	-	-	-	-	-	-	-
Prepaid expenses and other current assets	0.2	0.3	0.3	0.3	0.3	0.4	0.4	0.5
Total Current Assets	**11.0**	**86.4**	**56.8**	**127.5**	**206.2**	**114.7**	**154.7**	**161.4**
PPE, Net	0.8	1.4	1.5	1.6	1.8	2.0	2.2	2.4
Restricted Cash	0.4	0.5	0.5	0.6	0.6	0.7	0.8	0.8
Other Assets	0.0	0.0	0.0	0.0	0.0	0.0	0.1	0.1
Total Assets	**12.1**	**88.3**	**58.9**	**129.8**	**208.7**	**117.4**	**157.7**	**164.7**
Liabilities & Equity								
Accounts Payable	0.3	1.2	1.3	1.5	1.6	1.8	1.9	2.1
Accrued liabilities	0.7	1.9	2.1	2.3	2.5	2.8	3.1	3.4
Deferred rent	-	0.1	0.1	0.1	0.1	0.1	0.1	0.1
Total Current Liabilities	**1.0**	**3.2**	**3.5**	**3.9**	**4.2**	**4.7**	**5.1**	**5.6**
Convertible preferred stock warrant liability	0.2	0.5	0.5	0.5	0.5	0.5	0.5	0.5
Other liabilities	0.3	0.3	0.3	0.3	0.3	0.3	0.3	0.3
Total Liabilities	**1.5**	**4.0**	**4.3**	**4.6**	**5.0**	**5.5**	**5.9**	**6.4**
Series A Convertible preferred stock	18.6	37.5	37.5	-	-	-	-	-
Series B Convertible preferred stock	-	73.9	73.9	-	-	-	-	-
Common Stock	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Additional Paid-in capital	0.2	0.0	0.0	224.8	374.8	374.8	524.8	524.8
Accumulated deficit	(8.2)	(27.1)	(56.8)	(99.7)	(171.2)	(262.8)	(373.1)	(366.5)
Total Shareholders' Equity	**10.6**	**84.3**	**54.6**	**125.2**	**203.6**	**112.0**	**151.8**	**158.3**
Total Liabilities & Equity	**12.1**	**88.3**	**58.9**	**129.8**	**208.7**	**117.4**	**157.7**	**164.7**

Source: Company reports and J.P. Morgan estimates.

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Figure 18: RARE Cash Flow Statement

Ultragenyx Cash Flow Statement ($ millions)

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		2011A	2012A	2013E	2014E	2015E	2016E	2017E	2018E
Cash Flow from Operations									
Net Income		$ (6.8)	$ (16.3)	$ (31.7)	$ (57.9)	$ (73.8)	$ (94.2)	$ (113.0)	$ 3.5
Adjustments to reconcile net loss to net operating cash									
Depreciation and Amort		0.0	0.3	0.3	0.4	0.4	0.5	0.5	0.6
Noncash interest expense		0.3		-	-	-	-	-	-
Stock-based Compensation		0.3	0.9	1.0	1.1	1.2	1.3	1.4	1.6
Revaluation of convertible preferred stock warrant liab		0.0	0.3	0.3	0.4	0.4	0.4	0.5	0.5
Changes in operating assets and liabilities									
Contracts receivable		(0.0)	0.0	-	-	-	-	-	-
Prepaid expenses		(0.2)	(0.1)	(0.03)	(0.03)	(0.03)	(0.03)	(0.04)	(0.04)
Other current and LT assets		(0.0)	0.0	-	-	-	-	-	-
Accounts payable		0.3	0.9	0.1	0.1	0.1	0.2	0.2	0.2
Accrued expenses		0.5	1.5	0.2	0.2	0.2	0.3	0.3	0.3
Deferred rent				0.0	0.0	0.0	0.0	0.0	0.0
Cash Flow from Operations	**$ -**	**$ (5.8)**	**$ (12.5)**	**$ (29.7)**	**$ (55.7)**	**$ (71.4)**	**$ (91.6)**	**$ (110.1)**	**$ 6.6**
Purchase of short term investments		-	-	-	-	-	-	-	-
Proceeds from sales of short term investments		-	-	-	-	-	-	-	-
Purchase of PPE		(0.5)	(1.1)	-	-	-	-	-	-
Proceeds from sale of PPE		-	-	-	-	-	-	-	-
Increase in restricted cash		(0.4)	(0.1)	0.0	0.1	0.1	0.1	0.1	0.1
Cash Flow from Investing	**$ -**	**$ (0.9)**	**$ (1.2)**	**$ 0.0**	**$ 0.1**	**$ 0.1**	**$ 0.1**	**$ 0.1**	**$ 0.1**
Net proceeds from issuance of common stock		-	0.1	-	126.4	150.0	-	150.0	-
Net proceeds from issuance of convertible preferred stock		14.9	89.0	-	-	-	-	-	-
Proceeds from issuance of promissory notes		2.5	0.1	-	-	-	-	-	-
Cash Flow from Financing	**$ -**	**$ 17.3**	**$ 89.2**	**$ -**	**$ 126.4**	**$ 150.0**	**$ -**	**$ 150.0**	**$ -**
Total Change in Cash	-	10.6	75.5	(29.7)	70.7	78.6	(91.5)	40.0	6.7
Beginning Cash Balance		0.1	10.6	86.2	56.5	127.2	205.8	114.3	154.3
Ending Balance: Cash and Investments	**$ 0.1**	**$ 10.6**	**$ 86.2**	**$ 56.5**	**$ 127.2**	**$ 205.8**	**$ 114.3**	**$ 154.3**	**$ 161.0**

Source: Company reports and J.P. Morgan estimates.

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Ultragenyx: Summary of Financials

Income Statement - Annual	FY12A	FY13E	FY14E	FY15E
Revenues	-	0	0	0
Cost of products sold	-	0	0	0
Gross profit	-	-	-	-
SG&A	-	(4)	(16)	(17)
R&D	-	(26)	(42)	(57)
Operating income	-	(30)	(58)	(74)
EBITDA	-	(30)	(58)	(74)
Net interest (income) / expense	-	0	0	0
Other income / (expense)	-	(2)	0	0
Income taxes	-	0	0	0
Net income - GAAP	-	(32)	(58)	(74)
Net income - recurring	-	(36)	(58)	(74)
Diluted shares outstanding	10	30	36	41
EPS - excluding non-recurring	-	(1.22)	(1.60)	(1.82)
EPS - recurring	-	(1.22)	(1.60)	(1.82)

Balance Sheet and Cash Flow Data	FY12A	FY13E	FY14E	FY15E
Cash and cash equivalents	86	57	127	206
Accounts receivable	0	0	0	0
Inventories	-	-	-	-
Other current assets	0	0	0	0
Current assets	86	57	128	206
PP&E	1	1	2	2
Total assets	88	59	130	209
Total debt	-	-	-	-
Total liabilities	4	4	5	5
Shareholders' equity	84	55	125	204
Net income (including charges)	(16)	(32)	(58)	(74)
D&A	0	0	0	0
Change in working capital	2	0	0	0
Other	1	1	1	2
Cash flow from operations	(13)	(30)	(56)	(71)
Capex	(1)	0	0	0
Free cash flow	(14)	(30)	(56)	(71)
Cash flow from investing activities	(1)	0	0	0
Cash flow from financing activities	89	0	126	150
Dividends	-	-	-	-
Dividend yield	-	-	-	-

Income Statement - Quarterly	1Q13A	2Q13A	3Q13A	4Q13E
Revenues	-	-	-	-
Cost of products sold	-	-	-	-
Gross profit	-	-	-	-
SG&A	-	-	-	-
R&D	-	-	-	-
Operating income	-	-	-	-
EBITDA	-	-	-	-
Net interest (income) / expense	-	-	-	-
Other income / (expense)	-	-	-	-
Income taxes	-	-	-	-
Net income - GAAP	-	-	-	-
Net income - recurring	-	-	-	-
Diluted shares outstanding	0A	0A	23A	30
EPS - excluding non-recurring	-	-	-	-
EPS - recurring	-	-	-	-

Ratio Analysis	FY12A	FY13E	FY14E	FY15E
Sales growth	-	-	-	-
EBIT growth	-	-	90.8%	27.5%
EPS growth - recurring	-	-	31.5%	14.0%
Gross margin	-	-	-	-
EBIT margin	-	-	-	-
EBITDA margin	-	-	-	-
Tax rate	-	0.0%	0.0%	0.0%
Net margin	-	-	-	-
Net Debt / EBITDA	-	-	-	-
Net Debt / Capital (book)	-	-	-	-
Return on assets (ROA)	-	(48.8%)	(61.4%)	(43.6%)
Return on equity (ROE)	-	(51.8%)	(64.4%)	(44.9%)
Enterprise value / sales	-	-	-	-
Enterprise value / EBITDA	-	NM	NM	NM
Free cash flow yield	(2.4%)	(1.7%)	(2.7%)	(3.0%)

Source: Company reports and J.P. Morgan estimates.
Note: $ in millions (except per-share data).Fiscal year ends Dec

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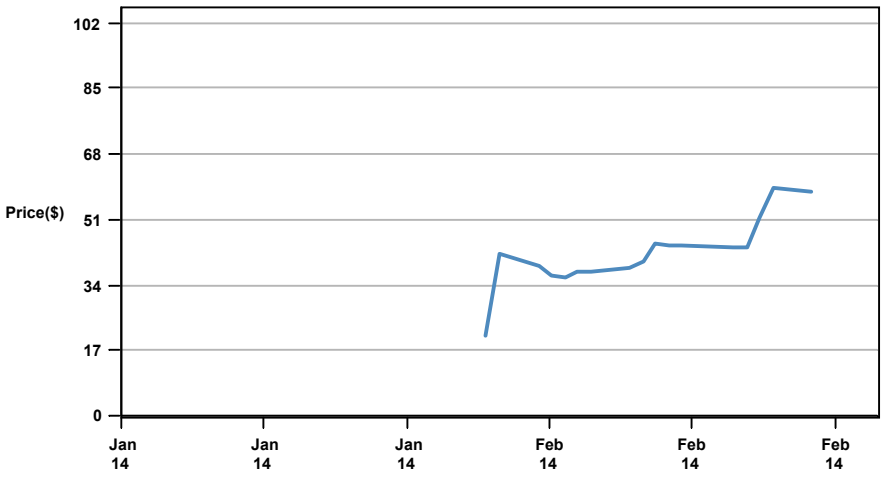
Ultragenyx (RARE, RARE US) Price Chart



Source: Bloomberg and J.P. Morgan; price data adjusted for stock splits and dividends.

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