Ultragenyx Pharmaceutical

RARE : NASDAQ : US$58.01

BUY

Target: $85.00

Salveen Richter, CFA - Canaccord Genuity Inc. (US)
srichter@canaccordgenuity.com 212.389.8052
Andrew Goldsmith, PhD - Canaccord Genuity Inc. (US)
agoldsmith@canaccordgenuity.com 212.389.8053

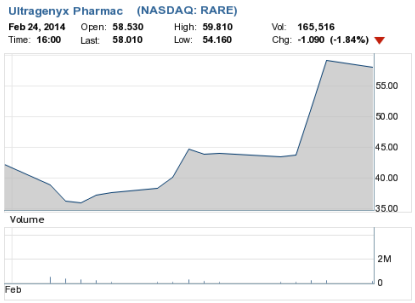
COMPANY STATISTICS:

52-week Range:	35.15 - 62.48
Market Cap (M):	US$1686
Avg. Daily Vol. (000s):	468
Shares Out (M):	29.062
Cash (M):	US$86.19

EARNINGS SUMMARY:

FYE Dec		2012A	2013E	2014E
EPS:		(14.20)	(1.42)	(1.87)
Revenue (M):	Q1	-	0.0A	0.0
	Q2	-	0.0A	0.0
	Q3	-	0.0A	0.0
	Q4	-	0.0	0.0
Total		0.0	0.0	0.0
EPS:	Q1	-	(3.36)A	(0.37)
	Q2	-	(3.23)A	(0.43)
	Q3	-	(2.58)A	(0.50)
	Q4	-	(0.41)	(0.57)
Total		(14.20)	(1.42)	(1.87)

SHARE PRICE PERFORMANCE:



Source: Interactive Data Corporation

COMPANY DESCRIPTION:
Ultragenyx is a clinical-stage biopharmaceutical company focused on developing and commercializing a broad pipeline of biologics and small molecules for the treatment of rare and ultra-rare serious metabolic genetic diseases. Their lead products are KRN23 for XLH, triheptanoin for Glut1 DS and LC-FAOD, rhGUS for MPS VII and SA-ER for HIBM.

All amounts in $US unless otherwise noted.

Life Sciences -- Biotechnology

A RARE OPPORTUNITY; INITIATING WITH A BUY AND $85 PRICE TARGET

Ultragenyx has a broad and promising pipeline consisting of four therapies for five rare and serious genetic diseases. We like the orphan-disease-focused strategy for early proof-of-concept, short time-to-clinic, exclusivity and pricing power and particularly the leveragability of this model. The key value drivers are KRN23 for the most common genetic form of rickets and triheptanoin for two diseases where patients' cells receive inadequate energy leading to neurological or liver/cardiac/muscle issues. The other two pipeline products (rhGUS and SA-ER) are low-risk but represent smaller opportunities. We estimate ~$1B+ in total peak WW revenue to Ultragenyx, noting high chances of securing regulatory approval on good data, with substantial uptake given most targeted indications are severe with no to few/poor existing therapies. In addition, we view the preclinical asset (rhPPCA) as a free call option and expect a continued focus on early-stage R&D. We expect RARE to trade higher on multiple data catalysts in H1+ through four product launches in 2017-18.

- **Triheptanoin and KRN23 are the key value drivers, with upside potential:** Triheptanoin is a substrate replacement to treat two diseases where a patient's cells do not get enough glucose: long-chain fatty acid oxidation disorder (LC-FAOD – not enough energy is produced by a metabolic pathway) and glucose transporter type-1 deficiency syndrome (Glut1 DS – energy does not get into the brain). KRN23 is an antibody that reduces levels of the FGF23 protein, the cause of soft bones in genetic rickets (XLH). We model for peak revenue to RARE from triheptanoin and KRN23 of $601M and $298M in 2028, respectively, and view triheptanoin as a pipeline within a product with potential use in other neurological diseases (e.g., seizures, etc.).
- **Ultragenyx has multiple data catalysts in 2014+:** We expect three positive data catalysts in 2014: 1) Phase 1/2 rhGUS 12-week data (H1), which we expect to demonstrate improvements in spleen/liver size and stamina; 2) Phase 1/2 KRN23 data in adults (mid-2014), where bone density/quality improvements should build on PK data; and 3) Phase 2 SA-ER extension data (H2), where we expect improvements over 48-week data (improvements in upper extremity strength) given higher dosing. Post 2014, we expect 2-3 positive data readouts per year.

INVESTMENT THESIS

Ultragenyx has multiple high-probability shots on goal in the ultra-rare disease space. Ultragenyx has four drugs in mid-stage clinical development for five rare diseases – a field distinguished by early proof-of-concept, short time-to-clinic, orphan exclusivity and high pricing opportunity. The key value drivers are: 1) triheptanoin for glucose transporter type-1 deficiency syndrome (Glut1DS) and long-chain fatty acid oxidation disorder (LC-FAOD) - diseases where a patient's cells do not receive enough energy, causing neurological and muscle/liver issues, respectively; and 2) KRN23 for X-linked hypophosphatemia (XLH), the most common form of inherited rickets. In addition, Ultragenyx is developing rhGUS for MPS VII (mucopolysaccharidosis 7, Sly Syndrome – a very severe lysosomal storage disease where the life expectancy is ~20 years of age), and SA-ER for hereditary inclusion body myopathy (HIBM, progressive weakness that can lead to muscle immobilization) – two relatively low-risk shots on goal. We view triheptanoin and KRN23 as the key value drivers due to the potential market sizes (~55K patients WW for KRN23; ~33K patients for triheptanoin). Ultragenyx has clinical data in four indications (all but triheptanoin for Glut1DS). Our review of existing literature and data along with physician diligence leads us to believe that clinical and commercial success in each program is likely (we have the most conviction in rhGUS for MPS VII, with potential commercial outlook questions for triheptanoin. We like the diversified nature of the pipeline, which is rare so early in an orphan company's existence. Of note, we expect the broad pipeline to produce steady news flow, with two to three data catalysts/year prior to product launches in 2017+. We particularly like Ultragenyx given the proven track record of management (CEO Dr. Emil Kakkis is a leader in the development of drugs for ultra-rare diseases; management has developed/commercialized Aldurazyme, Naglazyme, Kuvan and Vimizim (BMRN), Lumizyme/Myozyme (SNY/Genzyme) and asfotase alfa (ALXN/Enobia)).

We see a high likelihood of success for the Ultragenyx pipeline given clean mechanisms of action and clinical data. We like the odds for rhGUS success given a well-established track record of ERTs in treating MPS diseases, mouse data indicating a dose-dependent reduction of urinary GAGs (>80%) and the initial experience of one patient treated under an emergency IND, who demonstrated improvements in spleen/liver size, ETCO2 and stamina following 14 weekly rhGUS infusions. As such, we assign rhGUS an 85% chance of success. We are also encouraged by KRN23, particularly the elevations in vitamin D and phosphate to clinically-meaningful levels following a single dose of drug, and expect these results to be repeated/improved in a multi-dose trial (data mid-14) – allocating a 75% chance of success to KRN23. For SA-ER, Phase 2 data demonstrates preliminary signs of efficacy – with significant improvements in upper extremity muscle strength following 6g/day SA-ER, particularly in healthier patients. Given the relatively clean safety profile, we see further efficacy possible with dosing >6g/day and we assign SA-ER a 75% chance of success. Although there is extensive data on triheptanoin for treatment of LC-FAOD patients (significant improvements in the number of hospitalizations per year following triheptanoin treatment), there is no published clinical data for Glut1 DS – although the mechanism of this disease is encouraging. Given the lack of controlled clinical data in these indications and existing therapies (ketogenic diet, avoidance of fasting), we are conservatively giving a 65% chance of success to triheptanoin.

Our physician feedback is positive on Ultragenyx's therapies. Our physician feedback is positive on the chance for clinical success for the products, with likely widespread uptake

per drug on approval given the severity in disease nature and no treatment options (MPS VII, HIBM) or poor treatment options (XLH, Glut1 DS, LC-FAOD). Specifically, physicians like rhGUS for MPS VII (proven track record of ERTs to treat LSDs; most patients do not live past the age of 20), KRN23 for XLH (well-understood mechanism; clean safety; serum phosphate and vitamin D levels react to KRN23 as predicted) and SA-ER for HIBM (nice mouse data; clinical data indicates disease reversal possible). On tripeheptonion, physicians note the inadequacy of existing therapies for Glut1 DS and LC-FAOD (ketogenic diet and avoidance of fasting, respectively), but look to placebo-controlled data demonstrating triheptanoin efficacy. As with many rare diseases, physicians also see most of these diseases as underdiagnosed, with the potential for more patients to be found with an approved therapy. In addition, physicians see Ultragenyx's therapies as likely the first to market to directly address the causes of the diseases – although they note pre-clinical academic development is underway for therapies to treat XLH (C-terminal FGF23 and D6R) and HIBM (ManNAc).

With four products in the clinic, Ultragenyx should have nice news flow in 2014 through launches in 2017+: Starting with three data readouts expected in 2014 (Phase 1/2 rhGUS in Q1; Phase 1/2 triheptanoin for LC-FAOD in mid-14; Phase 2 SA-ER extension data in Q4), we look for two to three additional data catalysts per year in 2015, 2016 and 2017. We expect these catalysts will drive shares higher given the likelihood of success while drawing investor interest as the story shifts to one of commercial execution with four product launches in 2017-18. With two wholly-owned products (rhGUS and triheptanoin) and Ultragenyx retaining substantial rights to the others (Ultragenyx owns the Latin American rights to KRN23, receives a 10% royalty on sales in E.U. and receives a profit share/royalty on U.S./Canadian sales; Ultragenyx receives a royalty on Asian sales of SA-ER ex-Japan and owns commercialization rights ex-Asia), we like the commercial strategy and note substantial leveragability with the ultra-orphan model.

We model for peak revenue to Ultragenyx of ~$1B in 2028+. We model for rhGUS approval/launch in mid-2017, with peak sales of $71M in 2028 at pricing of $400K/year and peak penetrations of 93% (U.S.) and 80% (ROW) into the ~200 cases in the developed world. For KRN23, we assume early-2018 launch and peak WW sales estimate of $915M in 2028 from launch pricing of $100K/year and peak pediatric/adult penetrations of 41%/18% (U.S.), 30%/14% (E.U.) and 10%/5% (Latin America) - with Ultragenyx recognizing $140M as revenue and $158M as a royalty. We model for triheptanoin launch for both LC-FAOD and Glut1 DS in H2/18 and peak sales of $601M in 2028 at $75K/year in pricing and penetrations of 25%, 24% and 19% into LC-FAOD in U.S., E.U., and ROW, respectively and penetrations of 29%, 26% and 20% into Glut1 DS. Given the large population sizes, we view triheptanoin and KRN23 as the key value drivers, but view our penetration rates as conservative. For SA-ER, we assume WW launches in early 2018+, annual launch pricing of $50K and peak penetrations of 86% (U.S.), 83% (E.U.) and 67% (ROW) – for peak sales of $71M in 2028 of which $53M is recognized as revenue (sales in U.S. and E.U.) and ~$1M as a royalty (Asia).

Pre-clinical rhPPCA and other assets represent potential upside. Ultragenyx is also developing rhPPCA, an enzyme replacement therapy for the lysosomal storage disorder galactosialidosis (IND expected 2015/16). Given the significant R&D focus with all four products in the clinic expected to come to market in 2017-18, we applaud the continued interest in earlier-stage assets. We look for additional pipeline growth through licensing/business development efforts. We do not model for sales of rhPPCA.

Ultragenyx has an estimated $170M in cash following the IPO. At the end of Q3/13, Ultragenyx had $63.7M in cash and equivalents, not including net proceeds from the IPO of $126.4M (6.6M shares at $21/share). We believe this position will be sufficient to fund operations into early 2016 and model for two equity offerings: $150M in mid-15 and $200M in mid-17.

VALUATION

We arrive at our 12-month price target of $85 by blending two valuation methodologies: 1) a sum-of-the-parts discounted cash flow analysts (50% weight) equating to $96/share, which ascribes $29/share from KRN23, $5/share from rhGUS, $56/share from triheptanoin, $1/share from SA-ER and $5/share in cash, with the following assumptions: we assign KRN23 a 75% chance of success, rhGUS an 85% chance of success, triheptanoin a 65% chance of success, and SA-ER a 75% chance of success, and we assign a WACC of 8.5% and a 2% terminal growth rate; and 2) a discounted EPS (50% weight), equating to $74/share by applying a 35x multiple of our fully-diluted FY22 GAAP EPS estimate of $10.65, discounted back to YE14 at 24%.

INVESTMENT RISKS

The primary risks for Ultragenyx include the following:

1. Clinical development risk: efficacy per product (will the preclinical/early-stage clinical results be recapitulated in late-stage trials), particularly for those indications (rhGUS for MPS VII; triheptanoin for Glut1 DS) where there is limited or no clinical data to date.
2. Commercial risk, including the possibility that each product does not achieve the peak commercial revenue estimates in our model (due to patient identification, market size, penetration rates and/or pricing/reimbursement).
3. Regulatory risk, including failure to secure U.S./E.U./ROW approval for each product.
4. Product competition: although there are few (XLH, LC-FAOD, Glut1 DS) or no (MPS VII, HIBM) approved therapies to treat Ultragenyx's indications and there are few pipeline treatments for these indications, emergence of competing therapies could impact the uptake of Ultragenyx's products.
5. Partnership risk: Ultragenyx is relying on the manufacturing and commercialization efforts of partners KHK (KRN23 clinical supply and E.U. commercialization) and Nobelpharma (SA-ER manufacturing and commercialization in Japan and other Asian territories).
6. Financing risk: we model for two equity offerings of $150M (1.76M shares at $85/share) in mid-2015 and $200M (2M shares at $100/share) in mid-2017.

PIPELINE SUMMARY

Ultragenyx is a clinical stage biopharmaceutical company focused on developing and commercializing a broad pipeline of biologics and small molecules for the treatment of rare and ultra-rare serious metabolic genetic diseases.

The four lead products (for five indications) are:

1) KRN23 for X-linked hypophosphatemia (XLH) - currently being evaluated in a Phase 1/2 trial in adults (Phase 2 pediatric trial to initiate in H2/14);

2) triheptanoin for long-chain fatty acid oxidation disorder (LC-FAOD) and glucose transporter type-1 deficiency syndrome (Glut1 DS) – currently in a Phase 2 trial for LC-FAOD and about to enter Phase 2 for Glut1 DS;

3) rhGUS for MPS VII/Sly Syndrome – currently in a Phase 1/2 trial (Phase 3 to potentially start in H2/14); and

4) extended-release sialic acid (SA-ER) for hereditary inclusion body myopathy (HIBM) – currently in a Phase 2 trial (extension data expected in H2/14).

Ultragenyx is also developing the pre-clinical asset recombinant human protein protective cathepsin-A (rhPPCA) for galactosialidosis, with an IND expected in 2015/16.

Figure 1: Ultragenyx pipeline

Product	Description	Indication	Commercial rights	Status: Pre-clinical	Phase 1/2	Phase 2
Recombinant human protein protective cathepsin-A (rhPPCA, UX004)	Enzyme replacement therapy	Galactosialidosis	Worldwide			
KRN23 (UX023)	Anti-FGF antibody	X-linked hypophosphatemia (XLH)	Kyowa Hakko Kirin (KHK) U.S. / Canada (profit share); E.U. (royalty) Mexico, Central/South America (full rights)			
Recombinant human β-glucuronidase (rhGUS, UX003)	Enzyme replacement therapy	Mucopolysaccharidosis 7 (MPS 7/Sly syndrome)	Worldwide			
Triheptanoin (UX007)	Susbtrate replacement	Glucose transporter type-1 deficiency syndrome (Glut1 DS)	Worldwide			
Triheptanoin (UX007)	Susbtrate replacement	Long-chain fatty acid oxidation disorders (LC-FAOD)	Worldwide			
Extended-release sialic acid (SA-ER, UX001)	Susbtrate replacement	Hereditary inclusion body myopathy (HIBM)	Nobelpharma Partnership ex-Japan			

Source: Company reports, Canaccord Genuity

Figure 2: Upcoming expected milestones

Product	Indication	Timing	Milestone
rhGUS (UX003)			
rhGUS	Mucopolysaccharidosis 7 (MPS 7/Sly)	1H14	Phase 1/2 12-week data
rhGUS	Mucopolysaccharidosis 7 (MPS 7/Sly)	2H14	Phase 3 initation
KRN23 (UX023)			
KRN23	X-linked hypophosphatemia (XLH)	mid-14	Phase 1/2 data
KRN23	X-linked hypophosphatemia (XLH)	2H14	Phase 2 (pediatric) clinical initiation
KRN23	X-linked hypophosphatemia (XLH)	2015	Phase 2b (adult) initiation
KRN23	X-linked hypophosphatemia (XLH)	2015	Phase 2 (pediatric) data
Triheptanoin (UX007)			
Triheptanoin	Glucose transporter type-1 deficiency syndrome (Glut1 DS)	1Q14	Phase 2 initiation
Triheptanoin	Long-chain fatty acid oxidation disorders (LC-FAOD)	1H15	Phase 2 data
Triheptanoin	Glucose transporter type-1 deficiency syndrome (Glut1 DS)	2015	Phase 2 data
Extended-release sailic acid (SA-ER / UX001)			
SA-ER	Hereditary inclusion body myopathy (HIBM)	2H14	Extension data
SA-ER	Hereditary inclusion body myopathy (HIBM)	2015	Phase 3 initation

Source: Company reports, Canaccord Genuity

ULTRAGENYX HAS FOUR PRODUCTS IN CLINIC FOR SEVERE AND RARE DISORDERS

Ultragenyx has a broad pipeline with four products in the clinic to treat five rare and ultra-rare serious metabolic genetic diseases: 1) KRN23 (antibody) for X-linked hypophosphatemia (XLH), currently in a Phase 1/2 clinical trial; 2) rhGUS (enzyme replacement therapy) for MPS VII/Sly Syndrome, currently in Phase 1/2; 3) triheptanoin (small-molecule substrate replacement therapy) for glucose transporter type-1 deficiency syndrome (Glut1 DS) and long-chain fatty acid oxidation disorder (LC-FAOD), with Phase 2 trials ongoing and about to initiate, respectively; and 4) extended release sialic acid (SA-ER; small-molecule substrate replacement therapy) for hereditary inclusion body myopathy (HIBM), currently in Phase 2.

The multiple shots on goal allow for diversification, while staying within the well-trodden rare-disease field (early proof-of-concept, short time to clinic, orphan exclusivity and high pricing) and allowing for multiple data catalysts in 2014-2016. While we are optimistic about the prospects for each product, we believe rhGUS for MPS VII has the highest probability of success given the simple and well-understood mechanism of action and the track record for ERTs in mucopolysaccharidosis disorders (CEO Dr. Kakkis developed Aldurazyme for MPS 1 at BMRN).

With Ultragenyx retaining worldwide rights/key commercialization rights in most territories, we expect the story to shift to a commercial/launch play as all four products enter the market in the 2017-2018 timeframe. Given the management track record (management developed/commercialized Aldurazyme, Naglazyme, Kuvan and Vimizim (BMRN); Lumizyme/Myozyme (SNY/Genzyme); and asfotase alfa (ALXN/Enobia)) and relatively small sales forces required to launch orphan drugs, we like the commercial prospects and leveragability (of multiple approved drugs) on the model/income statement.

In addition, we look to the pipeline to further develop, with an IND expected for lead pre-clinical asset, recombinant human protein protective cathepsin-A (rhPPCA) for galactosialidosis in 2015/16 – all the while maintaining a focus on rare and ultra-rare diseases.

We see the simplest path to market for rhGUS for MPS VII: The enzyme replacement therapy (ERT) rhGUS (recombinant human beta-glucuronidase) recently entered into a Phase 1/2 trial in 5 patients with MPS (mucopolysaccharidosis) VII/Sly Syndrome – a severe and ultra-rare genetic disease characterized by defects in lysosomal processing. In its most severe form, MPS VII can cause death *in utero* or in newborns due to fluid accumulation. Even less severe patients develop mental retardation, loss of vision, hydrocephalus, coarse facial features, liver and spleen enlargement, and various bone defects (e.g., poorly formed pelvis, protruding sternum, enlarged skull). Data from one patient treated for 14 weeks under an emergency IND demonstrated improvements in hepatosplenomegaly, pulmonary function and stamina. We have a high level of confidence in the ongoing program, given: 1) strong preclinical data (urinary GAG levels were reduced up to 80% in a dose-dependent manner and liver pathology was eliminated in a mouse model of MPS VII treated with rhGUS); 2) a simple mechanism of action (replace the defective enzyme); and 3) history of success with ERT treatments of MPS diseases (e.g., Aldurazyme for MPS I, Elaprase for MPS II, Naglazyme for MPS VI and Vimizim for MPS IIIB). Accordingly, we model for an 85% chance of success for the rhGUS revenue stream in our sum-of-the-parts DCF – although we note the commercial opportunity is limited given the small population size. We estimate peak WW sales of $71M in 2028 given ~200 patients WW.

Triheptanoin has two shots on goal with LC-FAOD and Glut1 DS, but also the longest odds to market: Both long-chain fatty acid oxidation disorder (LC-FAOD) and glucose transporter type-1 deficiency syndrome (Glut1 DS) are diseases of energy metabolism where genetic defects prevent the correct production or transport of energy-bearing molecules. Patients with LC-FAOD are prone to liver and muscle diseases, triggered by fasting (they rely on glucose to meet their energy needs) while patients with Glut1 DS (also known as De Vivo disease) have a defect in a protein that transfers glucose into the brain, inducing neuronal defects such as developmental delay and seizures. Glut1 DS is likely underdiagnosed due to difficulty (doctors are reluctant to perform a spinal tap to measure glucose levels in CSF) or rarity (the De Vivo lab at Columbia performs an assay measure glucose transport into red blood cells, but it is not widely available) of existing diagnostic tests. Triheptanoin is downstream of the genetic defects – so providing it as a substrate replacement bypasses the error and serves as a source of energy for patients. Although triheptanoin has been used extensively for LC-FAOD – retrospective data indicates significant reductions in the number of hospital days and hypoglycemia events per year – evidence for its efficacy in Glut1 DS is derived from mechanism-based explanations and preclinical (mouse) data, but has the potential to improve cognitive ability. Given the nice retrospective data in LC-FAOD but the lack of human data in Glut1 DS, plus the presence of existing therapies (ketogenic diet, avoidance of fasting), we conservatively ascribe a 65% chance of success to triheptanoin (blended for the two indications). We expect Ultragenyx to commercialize triheptanoin alone WW and model for peak sales of $601M in 2028.

KRN23 for XLH has a simple mechanism of action and nice clinical data: KRN23 is an antibody against the FGF23 protein currently under evaluation in a Phase 1/2 trial for treatment of X-linked hypophosphatemia (XLH). XLH is a dominant genetic form of rickets,

or weakened/malformed bones that is caused by increased levels of the hormone FGF23, which in turn increases urinary excretion of phosphate. Given the relatively clean mechanism of action (KRN23 treatment reduces FGF23 activity the most important regulator of phosphate and active vitamin D levels) and Phase 1 clinical data (a single dose of KRN23 induces increases in serum phosphate and vitamin D levels with clean safety), we are reasonably confident that these results will be repeated in the Phase 1/2 and following Phase 2 trials. We assume a 75% chance of success for KRN23 and believe it will become a leading treatment option for XLH. However, given existing therapies (oral calcium and vitamin D), potential competitors and underdiagnosis, we model for conservative penetration (peak of 41%, 30% and 10% in the pediatric populations in U.S./Canada, E.U., and Latin America, respectively; peak of 18%, 14% and 5% in the adult populations) for peak WW sales of $915M in 2028, of which Ultragenyx recognizes $140M as sales and $158M as royalty/profit share (given collaboration with partner KHK).

SA-ER for HIBM shows early efficacy – particularly in a pre-specified subgroup but the opportunity is relatively small: Ultragenyx is currently evaluating a substrate replacement therapy, sialic acid extended-release (SA-ER), for hereditary inclusion body myopathy (HIBM) in a Phase 2 trial. SA-ER is a genetic disease in which patients lose strength in their muscles – starting at the distal limbs and progressing to the proximal. In a Phase 2 trial, SA-ER demonstrates signs of activity following 48 weeks of dosing via a significant difference in measures of muscle strength of the upper extremities in patients treated with the highest dose (6g /day) vs. low dose (3g/day). While lower extremities and other measurements (Functional Activity Score, sit-to-stand test, 6MWD) did not demonstrate a significant difference, there were trends to efficacy in some measurements. We are optimistic on the chances for SA-ER given the preliminary signs of efficacy, the potential for better efficacy in a pre-defined subgroup (patients with >200m baseline walking ability demonstrated larger delta), and the clean mechanism (patients with HIBM have defects in sialic acid production – SA-ER is a direct replacement) and assign SA-ER a 70% chance of success. However, the impacts to the bottom line may be limited given the relatively small number of patients (≤2K WW), the anticipated launch price of $50K/year, the potential for competitors (the NIH recently completed a Phase 1 trial of ManNAc/DEX-M74, a sialic acid precursor that is used by individual patients off-label, but questions remain on bioavailability it is not found in high concentrations in humans) and the economics to Ultragenyx (Ultragenyx receives a mid-single-digit royalty from partner Nobelpharma for SA-ER sales in Asia ex-Japan and certain other territories). Accordingly, we model for peak WW SA-ER sales of $71M in 2028, with Ultragenyx recognizing $53M as sales and ~$1M in royalties.

PHYSICIANS LIKE THE ODDS OF SUCCESS PER PRODUCT, BUT ARE MOST OPTIMISTIC ON RHGUS FOR MPS VII

Our physician feedback for most of Ultragenyx's therapies and indications are positive, with common themes including: 1) many of the diseases are underdiagnosed given rarity and lack of good treatment options; 2) the therapies are largely safe, with well-balanced risk profiles; 3) some risk on efficacy, particularly for diseases with little/no clinical data (e.g., triheptanoin for Glut1 DS); 4) most existing therapies are inadequate due to poor efficacy or convenience; and 5) most indications have few potential pipeline therapies ex-Ultragenyx – and those that do are behind Ultragenyx. Net-net, physicians are optimistic

on the profile of Ultragenyx's drugs and are eager to use them pending data in most or all of their patients.

Given the clean mechanism of action, physicians like rhGUS for MPS VII/Sly: Physicians are eager for a therapy for patients with MPS VII, given no approved therapies and disease severity (15-20 year life expectancy; most severe patients die as infants). They are optimistic on the chances for rhGUS to demonstrate efficacy in upcoming clinical trials, given preclinical data in mice and dogs, history of other ERTs for treatment of MPS disorders, and experience from the first patient treated under an emergency IND (results to date are "pretty impressive," according to one doctor). While they note a risk to the trial from enrolling older/sicker patients who may not have reversible disease, they anticipate that an ongoing registry will help in patient identification and note that the FDA is willing to look at efficacy via totality of data (one physician cites Elaprase for MPS II, which was approved following a miss on one component of a primary endpoint). Additionally, reducing liver and spleen size (as seen in the first patient treated) is expected to improve breathing status – including FVC and 6MWD. While physicians know of ~150 patients WW who have been born with MPS VII (~1/2 alive today), they have difficulty estimating WW incidence but note that newborn screening for MPS diseases (rolling out state-by-state) will aid in identifying MPS VII patients.

FGF23 would be a candidate for virtually all patients with XLH, which is likely underdiagnosed: Physicians are moderately satisfied with current regiments to treat XLH (phosphate, Calcitriol), although they note key issues with: 1) compliance - phosphate tastes bad, so children are reluctant to stay on therapy; 2) not treating early – if children are treated >5 years of age, there is a potential for poor outcomes that may require orthopedic surgery; 3) finding patients – not all physicians know how to look for characteristic X-ray images and will not necessarily order the correct tests; and 4) long-term complications from therapy - particularly hyperparathyroidism that may require treatment with cinacalcet or paricalcitol. Although they believe many patients are undiagnosed, they estimate there are ~15K – 22K patients in the U.S., in-line with Ultragenyx's estimate of 12K – 16K patients. They also see KRN23 as relatively safe, noting one patient with a skin reaction and no anti-drug antibodies to date, although hypermineralization is a risk with excess dosing (as with oral phosphate therapy). In terms of KRN23, they are optimistic that the single-dose data will be recapitulated or improved in multi-dose trials, noting that the Vitamin D levels seen in the single-dose trials are in a clinically-meaningful range. On approval, they anticipate using FGF23 in all children, while noting that adults have symptoms (bony pain, muscle weakness), which could be improved with treatment (2/3 to 3/4 of adult patients treated with phosphate and Calcitriol saw symptomatic benefit). Although they note the potential competitor hexa-D-arginine (D6R; activates an enzyme that downregulates FGF23; academics at U. Wisconsin hold a patent for its used in XLH treatment), they note KRN23 has clinical data, vs. the preclinical D6R.

Triheptanoin has nice potential in Glut1 DS, but the ketogenic diet is currently SOC: Our physician feedback indicates that the principal treatment for Glut1 DS, the ketogenic diet, appears to be reasonably effective in preventing epilepsy and movement disorders in children, although there is no placebo-controlled data demonstrating a benefit. The main issue with the ketogenic diet is compliance, particularly with adolescents who no longer want to eat the same food every day. Physicians note triheptanoin has a "similar mechanism" to the ketogenic diet, but would allow for more freedom versus a restricted diet (patients with other diseases that require a restricted diet, such as PKU, have

significant compliance issues). Although they are not aware of any published clinical data on triheptanoin for Glut1 DS, they are eager for a trial to be run using the ketogenic diet as a control – noting any safety concerns (primary GI, e.g., cramping or diarrhea) may differentiate triheptanoin negatively vs. the keto diet. On prevalence, they see the Ultragenyx estimate of 3K to 7K patients as reasonable based on number of patients they treat and published estimates (1/50K – 100K), although they see the disease as underdiagnosed given: 1) 90% of patients with Glut1 DS have a sporadic mutation (i.e. they will not necessarily find affected families); 2) genetic sequencing methods are expensive; 3) the red blood cell assay is only performed at Dr. De Vivo's lab at Columbia; and 4) doctors are reluctant to utilize CSF testing due to the requirement for a spinal tap. On the competitive front – other than the keto diet – they note gene therapy approaches (add in a copy of the defective gene) are being developed by academia but are early.

Triheptanoin for LC-FAOD will be tried by physicians in most patients: Although physicians note extensive retrospective data on the use of triheptanoin for LC-FAOD, they look to prospective, controlled trials that would stratify by patient severity to get a better handle on efficacy. However, they currently use triheptanoin in 10-20% of their LC-FAOD patients and assess response per patient – and would anticipate using in more patients on approval (biased toward more severe patients), expecting that most patients could benefit. In the recently-initiated Phase 2 trial, they like the size (~30 patients) and length (24 weeks), noting they currently treat for 3-6 months to assess benefit – and will look for improvements in number of hospitalizations, weight, exercise tests (e.g., 6MWD), biomarkers (LFTs) and ultrasound. They see triheptanoin as a very safe drug, noting no specific adverse events as concerns. On prevalence, they see the disease as underdiagnosed, but note efforts on E.U. are ramping up (particularly in Germany and U.K.). While they cite gene therapy as a potential competitor (as in Glut1 DS), they do not see it as a near-term threat to tripheptanoin.

SA-ER for HIBM has nice early data: Physicians understand the mechanism of sialic acid for HIBM, pointing to a mouse model of HIBM treated with SA that demonstrated an absence of pathology. However, a key issue in early SA clinical trials was that the drug was not detected in the blood (i.e. may indicate that it is not absorbed, but is not a measure of efficacy). In contrast, they are pleased with the extended-release formulation, describing the PK/PD data as "very convincing." They also like the 48-week Phase 2 data – particularly the improvement in upper extremity muscle strength in healthier patients, expressing surprise that that muscle fiber damage is reversible and patients could demonstrate improvements in strength (i.e. physicians anticipated that SA-ER would only prevent further decline). They do not see any red flags in the data, but are monitoring gastric discomfort, particularly given the amount of drug (6g+ per day) patients are taking. However, given sialic acid is found in everyday food, they do not see much risk on safety and want to see 6MWD tested in Phase 3. On approval, they would prescribe SA-ER to all their patients, preferring those with less-severe disease, but those with more disease progression (in wheelchairs) will also be treated given no other therapies and the potential to at least save remaining muscle strength. Although they see the estimate of ~1-2K patients in the developed world as reasonable, they do not know the exact number given the ultra-rare nature of the disease, but believe that neurologists in countries with many patients (e.g., Japan; however, Ultragenyx does not have any economics on Japan) are well-trained to find myopathies and will not overlook HIBM (>250 patients in Japan have been diagnosed). On the competitive front, they point to ManNAc (NIH ran a Phase 1 trial),

noting that ManNAc is likely metabolized/excreted more slowly than SA-ER, providing a benefit on PK/PD (it is "already like" an extended-release form) – but data from the NIH trial and commercial development plans are unclear.

WHAT ARE THE KEY RISKS FOR ULTRAGENYX?

- **Clinical failure via lack of efficacy, per product:** We acknowledge the potential risk of clinical failure for any of the assets under development – with more risk on efficacy vs. safety given the relatively clean profile per drug (see below). Specifically, when ranked, we see the greatest clinical risk with triheptanoin, particularly for Glut1 DS given the early-stage with absence of published clinical data to date, but we like the odds for LC-FAOD given extensive use and a significant reduction in hospitalizations in retrospective trials. We also view some risk associated with SA-ER given mixed Phase 2 data, with lower extremities not demonstrating a significant improvement in muscle strength, although we look to higher dosing and more details on efficacy per subset (healthier patients appear to respond more robustly). With KRN23, while we have seen reductions in serum phosphate and vitamin D levels from a single dose, we look to see these results replicated in multi-dose data and for more clinically meaningful endpoints. Despite the lack of clinical data, we assign the least risk on efficacy to rhGUS given the very clean preclinical data (dose-dependent uGAG reduction and elimination of liver lysosomal pathology), the history of success with ERT for MPS diseases and the preliminary clinical experience from one patient.
- **Emergence of a safety signal:** For KRN23, the most common adverse events in the Phase 1 trial were elevated serum amylase and back pain (17% each), although we look to multi-dose/chronic use for any possible development of anti-KRN23 antibodies or hypersensitivity reactions (not see in Phase 1). We do not see a significant risk of safety with rhGUS given MPS VII disease severity and history of ERTs success in the MPS disorders, although hypersensitivity is a possibility (common in labels for ERTs in MPS). Given the extensive history of triheptanoin use (two retrospective studies of LC-FAOD includes >65 patients; treatment of up to 13 years has been reported), we see emergence of a previously unknown safety signal as highly unlikely. For SA-ER, no serious adverse events were reported in the 48-week Phase 2 data, with GI symptoms (mostly mild-moderate) as the greatest safety risk, particularly given the slow disease onset (patients are wheelchair-bound 10-20 years post-diagnosis and may not see a dire need of treatment).
- **Commercial failure – are there enough patients and can they be found?** By nature of the diseases, there are few patients for any of Ultragenyx's therapies with uncertainty regarding the existing number of patients. For KRN23, prevalence estimates (~3K pediatric, ~9K adult in the U.S.) are based on a Danish epidemiological study and may not be recapitulated WW, and uptake – particularly in the less-severe adult form – may be limited. Given the ultra-rare nature of MPS VII (~200 cases WW), with potential that patients die *in utero* or shortly after birth, finding patients will require work – but we expect screening in most states to be adopted on approval of a therapy and help on this front. Ultragenyx puts the LC-FAOD and Glut1 DS prevalences at 2.5K to 3K and 3K to 7K patients, respectively (vs. our physician diligence estimates of ~3.3K and ~5K) – although both diseases may be underdiagnosed and we expect deeper penetration into younger populations for both diseases. For HIBM, the estimate of 1K

to 2K patients in the developed world is a rough estimate, but our physician feedback indicates many neurologists know to look for myopathies, including HIBM.

- **Will payors continue to pay for high-priced drugs?** As with any diseases for rare and ultra-rare conditions, potential commercial or government payor resistance to high prices remains a concern. However, we view this risk as minimal, given Ultragenyx's diseases are very rare, so no one payor will bear a large individual cost and Ultragenyx does not anticipate pushing the envelope for pricing (particularly for SA-ER, triheptanoin, KRN23 – where we model for annual prices at launch of $50K, $75K and $100K, respectively). We note a tolerance for high prices for severe, ultra-rare diseases; for example, annual WAC pricing for BMRN's ERT Vimizim for MPS IVA was recently set at $500K per patient.
- **Regulatory risks:** While regulatory risk remains for any drug in development, we see minimal risk to approval on significant improvements in efficacy in pivotal trials given regulators are amenable to approving orphan drugs for serious conditions – particularly for IV ERTs, where approval is the rule, not the exception, even on mixed results. We also note the granting of a rapid emergency IND for rhGUS as a favorable indicator, and see Breakthrough Designation (for "serious or life-threatening conditions" with "preliminary clinical evidence that demonstrates the drug may have substantial improvement on at least one clinically significant endpoint over available therapy," according to the FDA) as a possibility for all four lead products.
- **Competition from existing therapies…:** Of Ultragenyx's five lead indications, three have existing treatment options: phosphates/Calcitriol for XLH, avoidance of fasting and high-fat diet for LC-FAOD, and high-fat (specifically ketogenic diet) for Glut1 DS. While our feedback indicates dissatisfaction with these therapies given middling efficacy or side effect profiles, they may be some level of competition with Ultragenyx's therapies on approval.
- **…and emerging therapies:** Pipeline therapies are in development for XLS, Glut1 DS/FAOD and HIBM. Specifically, two academic treatments for XLH are in development: hexa-D-arginine and a C-terminal FGF23 tail. Given neither of these have entered clinical development, FGF23 has a dominant position and we see minimal risk on the competitive front, particularly in the near term. For Glut1 DS and LC-FAOD, academic groups are developing gene therapy approaches (add a copy of the defective gene), but we are not aware of clinical trials/data or plans for commercialization. A competing therapy for HIBM is ManNAc (N-Acetyl-D-mannosamine), which has demonstrated symptom progression in a mouse model and has been evaluated in a Phase 1 trial by the NIH. However, this trial has not been published (completion May 2013) and we are not aware of any further development, putting SA-ER closer to commercialization.

WE ESTIMATE PEAK WW REVENUE TO RARE OF ~$1B

rhGUS: For rhGUS, we assume the ongoing Phase 1/2 12-week data will enable initiation of a 12-patient trial in H2, with data in H2/16 and approval/launch in mid-2017. From pricing at launch of $400K/year (given the disease severity and low prevalence rate) and 3% price increases in the U.S., with peak penetration of 93% (U.S.) and 80% (ROW) into the ~200 cases in the developed world, we arrive at peak WW rhGUS revenue of $71M in 2028. Our sum of the part DCF includes an 85% chance of success for the rhGUS revenue stream. Given the asset is wholly-owned ($100K milestone on approval and low-single-digit royalty on sales owed to St. Louis University) and the small number of MPS VII patients, we expect Ultragenyx will commercialize rhGUS alone worldwide.

KRN23: For KRN23, we assume pediatric data in H1/15, with Phase 3 initiation in mid-15; for adults, we assume Phase 2b initiation in H1. We expect data from both trials in H2/16-H1/17, with early 2018 launch. Ultragenyx has partnered with Kyowa Hakko Kirin (KHK), with development/commercialization split in three regions: 1) U.S./Canada: 50/50 split in development costs; Ultragenyx launches and KHK books sales; 50/50 profit share over five years, with KHK paying a mid-high 20% royalty to Ultragenyx in years 6+ of the launch; 2) Europe: 50/50 split in development costs; KHK commercializes/books sales and pays a 10% royalty to Ultragenyx; and 3) Latin America: Ultragenyx pays the development costs, commercializes alone and pays a low-single-digit royalty to KHK. From projected peak pediatric/adult penetrations of 40%/18% (U.S.), 30%/14% (E.U.) and 10%/5% (Latin America), $100K annual pricing and 3% annual increases in the U.S., and ~56K pts WW (~25% pediatric), we arrive at our peak WW sales estimate of ~$915M in 2028 – with Ultragenyx recognizing $140M as revenue and $158M as a royalty. We model for a 75% chance of success for KRN23 in our DCF.

Triheptanoin: For triheptanoin, we expect data from both Phase 2 trials (LC-FAOD and Glut1 DS) in 2015, with a parallel Phase 3 trials initiation in H2/15. Following data in H1/17 and regulatory filings in H2/17, we expect launches in both indications in H2/18. We model for ~13K and ~20K LC-FAOD and Glut1DS patients WW, respectively, with peak triheptanoin penetrations of 25%, 24% and 19% into LC-FAOD in U.S., E.U., and ROW, respectively, and penetrations of 29%, 26% and 20% into Glut1 DS. From launch pricing of $75K and 3% annual price increases in the U.S., we arrive at our peak triheptanoin sales estimate of $600M in 2028 and probability-adjust triheptanoin revenue by 65% (blended for both indications) in our DCF. Other than a mid-single-digit royalty (we assume 4%) to Baylor Research Institute on sales, triheptanoin is wholly-owned by Ultragenyx, and we expect them to commercialize alone for both indications worldwide.

SA-ER: For SA-ER, Phase 2 extension data in H2 should inform the design of the Phase 3 trial, which we expect to initiate in H1/15. Following data at YE16, we assume regulatory filings in early 2017 and WW launches in early 2018+. We model for ~1.5K WW patients with HIBM (~25% in the U.S). From launch pricing of $50K/year, 3% annual increases in the U.S., and peak SA-ER penetrations of 86% (U.S.), 83% (E.U.) and 67% (ROW), we arrive at our peak WW sales estimate of $71M in 2028. Ultragenyx owns a license to commercialization rights ex-Asia, and partner Nobelpharma owns the rights for commercialization in Japan and other Asian countries. We model for $53M of peak SA-ER sales recognized as revenue (U.S. and E.U.) and ~$1M as a royalty (Asia, ex-Japan) in 2028 and assign a 75% chance of success to SA-ER in our DCF.

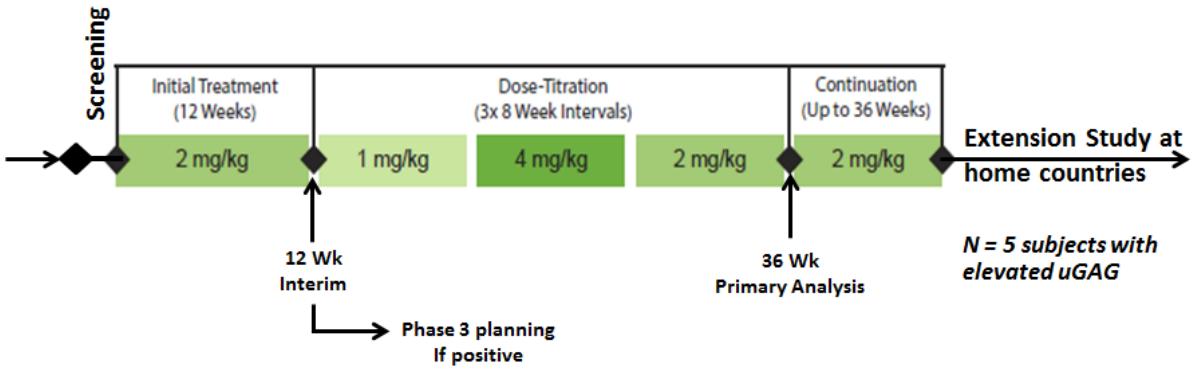
WE EXPECT 2-3 POSITIVE DATA CATALYSTS PER YEAR WITH PRODUCT LAUNCHES IN 2017+

We expect nice news flow from Ultragenyx's four lead products across five indications (three data readouts expected in 2014 alone) until product launches in 2017-18. For rhGUS, interim Phase 1/2 12-week data from 5 patients with MPS VII is expected in H1, with patients rolling onto a 24-week dose-titration portion and Phase 3 initiation in H2 likely on positive 12-week data. Phase 1/2 KRN23 repeat-dose data in adult patients with XLH is expected in mid-2014, in front of pediatric Phase 2 trial initiation in H2 and adult Phase 2b trial initiation in 2015. We expect a Phase 2 initiation utilizing triheptanoin for Glut1 DS in Q1 along with an ongoing Phase 2 trial for LC-FAOD, with data from both trials in 2015. Rounding out the timeline is SA-ER, which is currently in a Phase 2 trial for HIBM, with extension data in H2 and a Phase 3 initiation expected in 2015. Net-net, we expect all four lead programs to succeed, with rhGUS launching in mid-17 and SA-ER, KRN23 and triheptanoin coming to market in 2018. Beyond the lead products, Ultragenyx has another asset, rhPPCA for galactosialidosis, in pre-clinical development, with an IND expected 2015/16.

rhGUS for MPS VII is a low-risk opportunity with small trial requirements

Ultragenyx initiated a Phase 1/2 trial in Q4/13 to assess the safety and efficacy of rhGUS in five patients with MPS VII. The trial consists of an initial treatment period at 2 mg/kg, with an interim look at 12 weeks (expected in H1; see Figure 3). The primary endpoints are safety/tolerability, and urinary GAG (uGAG) reduction, each at 36 weeks; secondary endpoints include 6MWT, 3-minute stair climb, FVC, FEV1, MVV1, height/weight velocity, shoulder range of motion total uGAG excretion (to determine appropriate dosing).

Figure 3: rhGUS Phase 1/2 trial design for MPS VII



Source: Company presentation

We expect the 12-week data to be in-line with the 1 mg/kg to 4 mg/kg dosing at eight weeks in a mouse model of MPS VII (i.e. ~50%+ uGAG reductions) and no safety signal. On good 12-week data, the patients will continue into a 24-week dose titration portion (3 x 8 weeks) and a continuation/extension portion to follow. Simultaneously, a Phase 3 registration trial of 12 patients is expected to initiate in H2, with the primary endpoint of uGAG reduction. We assume data from the Phase 3 trial will be ready H2/16 (data from the

five patients in the Phase 1/2 trial will be included in the regulatory submissions), with approval/launch at mid-2017 (U.S.) and YE17+ (ROW).

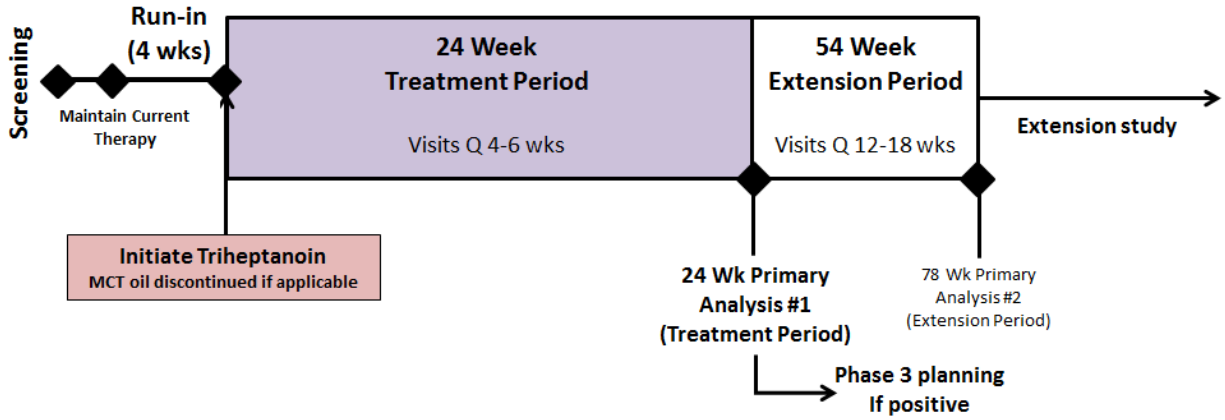
KRN23 for XLH has demonstrated efficacy in single-dose treatment; we expect improvements in multi-dose

rhGUS is currently being evaluated in Phase 1/2 clinical trials (primary endpoints safety and efficacy, via serum phosphate levels) with a pediatric Phase 2 and adult Phase 2b trials upcoming. Given the clean mechanism of action (excess FGF23 causes phosphate loss and reduced bone mineralization) and clinical data to date (a single dose of KRN23 elevates serum phosphate and vitamin D levels), we are optimistic that the multi-dose Phase 1/2 data in mid-14 will be in-line to better than the single-dose data (~50%+ serum phosphate reduction) with clean safety and will allow for a pediatric Phase 2 and adult Phase 2b trials to initiate in H2 and 2015, respectively. We expect data from the pediatric trial in H1/15, with Phase 3 initiation mid-2015 and data in H1/17. For adults, we expect a Phase 2b trial to initiate in H1, with data in late 2016/early 2017 and launch in both the pediatric and adult populations in early 2018.

Triheptanoin has two shots on goal with LC-FAOD and Glut1 DS, but there is no published clinical data on efficacy in Glut1 DS

Triheptanoin is currently being evaluated in an open-label Phase 2 trial for 20-30 patients with LC-FAOD, with the primary endpoint the effect of treatment on skeletal myopathy, hepatic disease and cardiac disease. Patients will be analyzed following 24 weeks of triheptanoin treatment (25-35% caloric daily intake); see Figure 4.

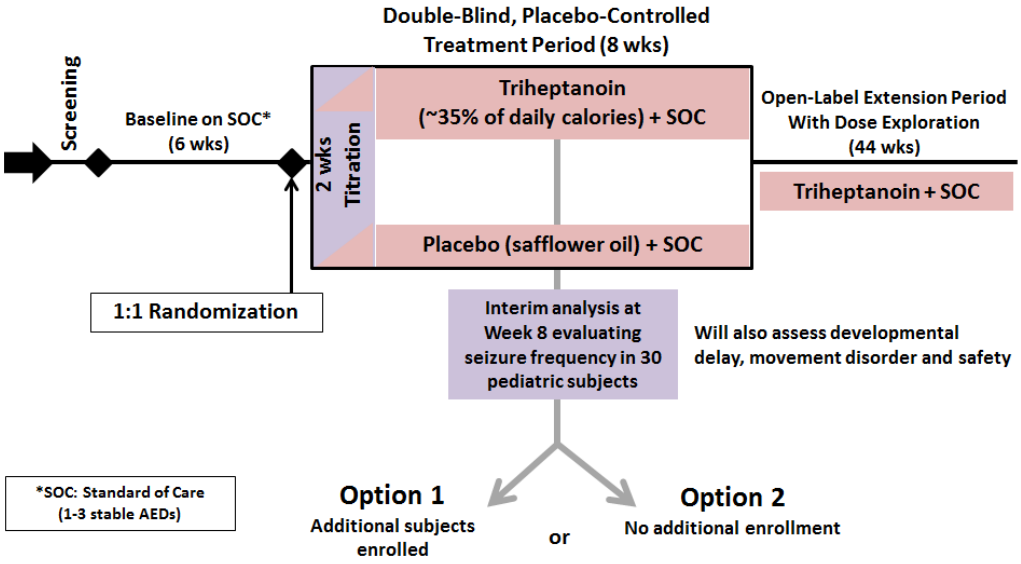
Figure 4: Triheptanoin Phase 2 trial design for LC-FAOD



Source: Company presentation

For Glut1 DS, triheptanoin will be evaluated in a randomized, double-blind Phase 2 trial. Specifically, 50 patients <18 years of age will be randomized to receive ~35% of their calories from triheptanoin or placebo. Following eight weeks of treatment (including a two-week titration), safety and efficacy will be measured via reduction from baseline in seizure frequency, development delay and movement disorders, and all patients will be rolled onto an open-label extension for an additional 44 weeks; see Figure 5. Safety and secondary endpoints (including number of patients with >=50% reduction in seizures, EEG abnormalities, cognitive ability, 6MWD) will be measured at 52 weeks.

Figure 5: Triheptanoin Phase 2 trial design for Glut1 DS



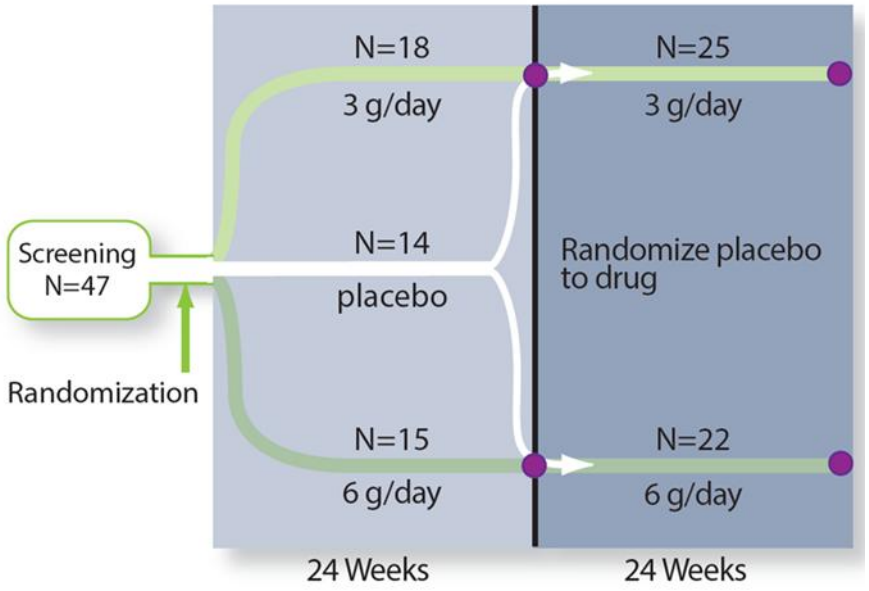
Source: Company presentation

Given historic data from patients with LC-FAOD treated with triheptanoin, we like the odds for clinical and commercial success but have more questions regarding uptake in Glut1 DS – particularly given the absence of clinical data in this indication and a current treatment option (Ketogenic diet). We expect data from both Phase 2 trials (LC-FAOD and Glut1 DS) in 2015, with parallel Phase 3 trials to initiate in H2/15. Following data in H1/17 and regulatory filings in H2/17, we expect launches in both indications in H2/18.

SA-ER for HIBM has demonstrated efficacy following 48 weeks of treatment, with higher dosing being utilized

Ultragenyx is currently running a Phase 2 trial utilizing SA-ER for HIBM. The first 24 weeks were randomized and placebo-controlled, placebo vs. 3g/day vs. 6g/day. At 24 weeks, patients on placebo were randomized to either drug arm, and treatment was continued through 48 weeks (see Figure 6). 48-week data was recently released, demonstrating signs of efficacy, particularly in the upper extremities in the healthier patients (>200m baseline walking ability) in those patients treated with 6g/day. Ultragenyx will test higher doses (up to 12g/day) and present full data from this trial in H2/14. We assume a Phase 3 trial will initiate in early 2015, with data at YE16, regulatory filings in early 2017 and approval/launch in early 2018.

Figure 6: Phase 2 SA-ER trial design



Source: Company presentation

rhPPCA for galactosialidosis will be the fifth asset in the pipeline – which we view as upside

Ultragenyx is developing recombinant human protein protective cathepsin-A (rhPPCA) for the treatment of galactosialidosis, an ultra-rare lysosomal storage disease caused by mutations in the *CTSA* gene that can be categorized as early infantile, late infantile and juvenile/adult. While the infantile forms are characterized by fluid accumulation, defects in skeletal development and shortened lifespan, the adult form is characterized by coarse features, skeletal deformities, epilepsy and ataxia, but normal lifespan.

The *CTSA* genes encode the protective protein/cathepsin A (PPCA) protein, and rhPPCA (recombinant human PPCA) is an enzyme replacement therapy to provide a working copy of the normally defective protein. Data in a mouse model of the related disease sialidosis demonstrates improvements in lysosomal storage following treatment with an ERT. Ultragenyx intends to generate proof-of-concept rhPPCA data in animals in 2014/15, and file an IND in 2015/16. Given the pre-clinical nature and lack of data with this program, we do not model for the asset; however, we note the severity of the disease and see the prevalence as possibly in-line with MPS VII (>100 cases of galactosialidosis have been described, vs. ~150 MPS VII).

GETTING UP TO SPEED ON ULTRAGENYX'S DISEASES, TREATMENTS AND DATA

X-linked Hypophosphatemia (XLH)

X-linked hypophosphatemia (XLH) is a genetic (dominant – affected parents will pass on the disease to 50% of their children) disease that causes numerous skeletal issues as the most common form of inherited rickets. Although disease severity varies from patient to patient, most individuals with XLH present with bowed legs in childhood. Additional symptoms include short stature and tibial (shin bone) rotation/fracture (see Figure 7) in children, with dental issues, arthritis and tendon/ligament calcification in adulthood. Despite the short stature and other symptoms, lifespan is relatively normal.

Figure 7: Untreated XLH can result in bone (tibia) fractures

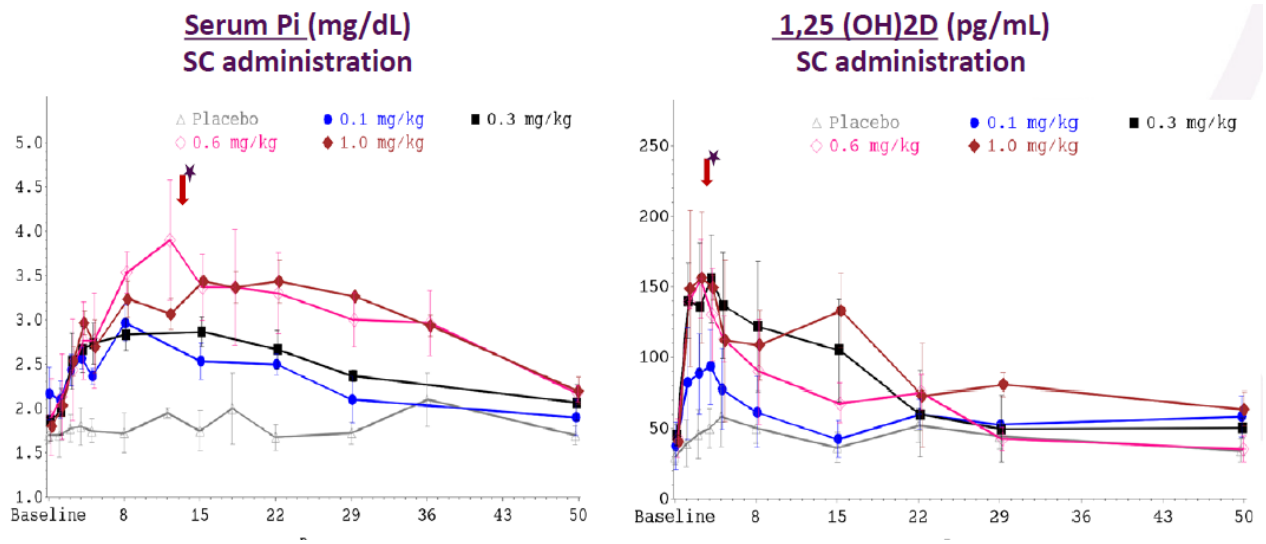
Source: Carpenter *et al*., 2010

XLH is caused by mutations in the gene known as PHEX (phosphate-regulating gene with homology to endopeptidases located on the X chromosome) that causes increased levels of the hormone FGF23 through partially unknown mechanism. Excess FGF23 causes phosphate to be excreted through the urine, which lowers the serum phosphate levels and reduces bone mineralization, causing weak bones. Treatment consists primarily of oral phosphate and activated vitamin D (Calcitriol; increases calcium uptake from the digestive tract) to treat the primary cause, although these can cause complications such as abdominal pain/diarrhea and ultimately hyperparathyroidism and nephrocalcinosis (calcium deposits in the kidneys). Other treatments for children include surgeries to correct leg deformities and growth hormone (GH) treatment, although GH appears to be less effective in patients who are not extremely short. Treatment for adults (often misdiagnosed as skeletal dysplasia) is centered on pain reduction and fracture recovery, although there is

not much evidence regarding treatment efficacy in the adult population. The incidence of XLH has been estimated at 3.9 – 5 per 100K live births, or ~12-16K patients in the U.S. assuming a normal lifespan.

The anti-FGF23 antibody KRN23 (UX023) increases phosphate and Vitamin D levels: KRN23 is an antibody against FGF23, which has demonstrated efficacy in a Phase 1 trial (see Figure 8). Specifically, a single dose produced significant elevations in 29 patients treated with KRN23 vs. 9 patients treated with placebo in phosphate levels (peak at day 8-15, $p<0.05$) and vitamin D levels (peak at day 4, $p<0.001$) with some evidence of dose-dependent efficacy persistence through 6-8 weeks (phosphate) or 15 days (vitamin D). On safety, there were no serious adverse events (AEs) or AEs leading to withdrawal, with elevated serum amylase and back pain the most common AE (17% each). The MTD was not reached and no anti-KRN23 antibodies were detected. Ultragenyx is looking to replicate and improve on these results in a Phase 1/2 trial in adults (mid-2014E data; bone density/quality is an endpoint) and a Phase 2 pediatric trial (H2 initiation; bone disease will also be an endpoint) ahead of Phase 2b adult and Phase 3 pediatric trials.

Figure 8: FGF23 treatment increases serum phosphate and vitamin D levels



Source: Company report and Carpenter *et al.*, 2013 ASBMR

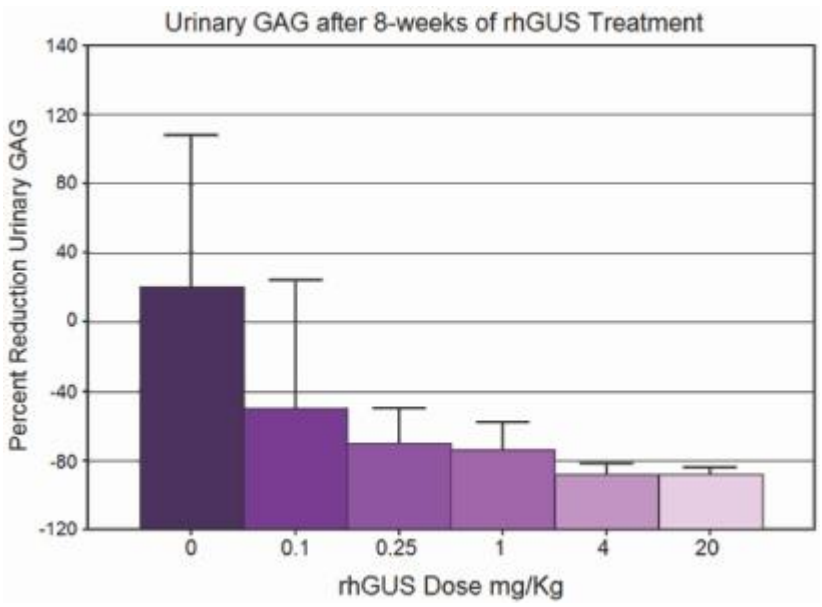
MPS VII/Sly Syndrome

MPS (mucopolysaccharidosis) VII, also known as Sly Syndrome, is one of the least common lysosomal storage disorders, with an incidence of ~1/250K births and ~200 patients in the developed world. It is a progressive disease with similar presentation as some other MPS diseases (e.g., MPS I/Hurler), including mental retardation, loss of vision, hydrocephalus, coarse facial features, liver and spleen enlargement, and various bone defects (e.g., poorly formed pelvis, protruding sternum, enlarged skull). In its most severe form, MPS VII can result in death *in utero* or shortly after birth due to fluid accumulation (hydrops fetalis), although less severe patients can survive to teens/20s. There is no curative treatment, although physiotherapy and hydrotherapy are used to encourage activity. MPS VII is a recessive genetic disease, caused by mutations in the beta-glucuronidase gene GUSB

(required to degrade a particular glycosaminoglycan/GAG) that allows accumulation of these GAGs in cell's lysosomes.

rhGUS (UX003) is a typical enzyme replacement therapy for treatment of MPS VII: Recombinant human beta-glucuronidase (rhGUS) is a replacement for the protein GUS that is defective in MPS VII patients. As with several other MPS diseases, enzyme replacement therapy (ERT) is an attractive treatment option (e.g., Aldurazyme for MPS I, Naglazyme for MPS VI and Vimizim for MPS IIIB) for patients with MPS VII. Preclinical work has demonstrated that rhGUS is taken up by cells, a necessary step for the protein to function properly. In a mouse model of MPS VII, 8 weeks of treatment with rhGUS is sufficient to reduce levels of GAG in the urine, as predicted (see Figure 9).

Figure 9: rhGUS reduces urinary GAG in a mouse model



Source: Company presentation

Data from one patient treated under an emergency IND was recently presented: following 14 weekly infusions, improvements were seen in spleen and liver size (the liver was 2 cm below the umbilicus pre-treatment, but above at 14 weeks), pulmonary function, ETCO2, stamina and number of hospitalizations (no hospitalization while on therapy, vs. three in the prior three months). No infusion-associated reactions were seen, although there was some temporary fatigue 24-48 hours post-infusions – likely due to fluid overload. Following 12-week data from an ongoing Phase 1/2 trial of five patients with MPS VII in H1, Ultragenyx intends to initiate a Phase 3 trial with 12 patients with MPS VII.

Long-chain fatty acid oxidation disorder (LC-FAOD)

Long-chain fatty acid oxidation disorder (LC-FAOD) is a disease of energy metabolism, where patients have defects in the Krebs cycle (a chemical process in which energy is produced from fats and carbohydrates) and fatty acid production. Patients with LC-FAOD cannot produce enough energy from fatty acids, making these patients reliant on glucose for their energy needs. As a result, these patients have low glucose levels (hypoglycemia) and related liver and muscle disease – with rhabdomyolysis potentially triggered by

prolonged fasting or illness. A retrospective analysis of 187 French children with FAOD (Baruteau *et al.*, 2013) found the most common symptoms to be hepatomegaly (92%), steatosis (lipid retention; 88%) and ALT elevations (82%), with a 48% mortality rate. Survival rates are correlated with older age – patients who are diagnosed earlier in life have much worse prognoses. While there is no curative therapy for LC-FAOD, treatments include avoiding fasting and various dietary treatments, such as low-fat/high-carbohydrate and medium-chain triglyceride oil. LC-FAOD is caused by genetic defects in various genes that encode proteins involved in energy metabolism. Newborns are screened for LC-FAOD – in the U.S., Ultragenyx has analyzed data from the National Newborn Screening Information System that indicates ~2K – 3.5K patients in the U.S. have LC-FAOD.

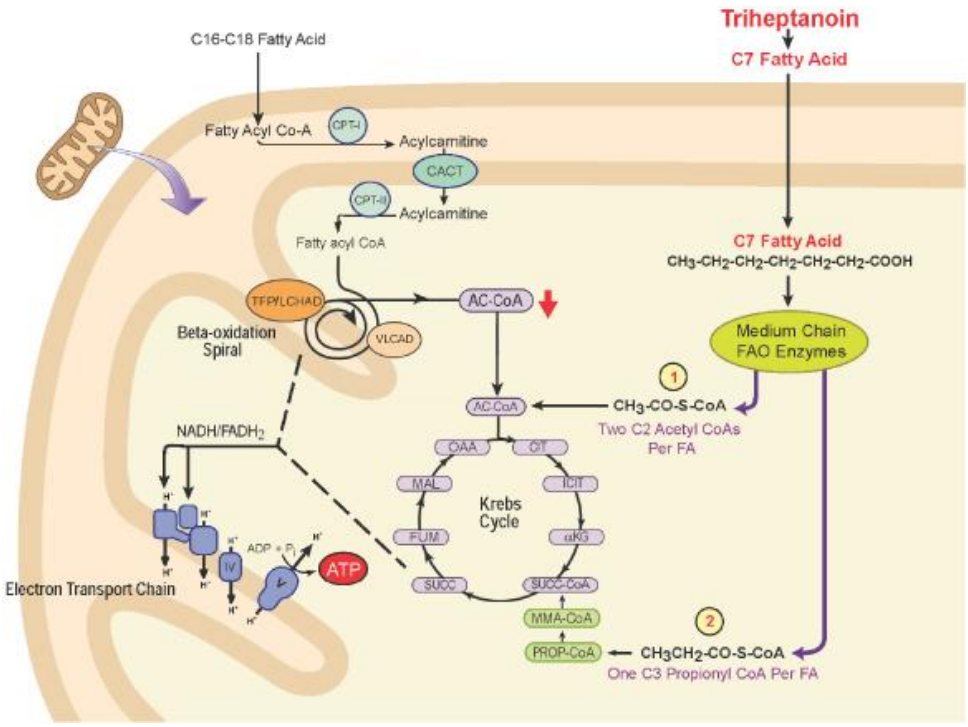
Glucose transporter type-1 deficiency syndrome (Glut1 DS) / De Vivo disease

Like LC-FAOD, glucose transporter type-1 deficiency syndrome (Glut1 DS) is a disease of energy metabolism. It is caused by a dominant mutation in the Glut1 gene that encodes the GLUT1 protein, which transports glucose into the brain. Defects in this protein prevent the brain from receiving sufficient energy, which causes several neural-specific symptoms, such as developmental delay, seizures, microcephaly and spasticity. The long-term prognoses of children rely on the degree of brain nutrition at early ages – patients who were not treated early do worse. Also similar to LC-FAOD, Glut1 DS can be treated with a special diet, specifically the ketogenic diet (high-fat, low carbohydrate) that generates ketone bodies as an alternate source of energy for the brain. Antiepileptic drugs are also used to treat Glut1 DS. Although prevalence is hard to estimate, one study (Comen *et al.*, 2006) estimates 1:90K incidence in Queensland, Australia, and another (Klepper and Leinendecker, 2007) notes 33 patients diagnosed in five years in Germany/Benelux/UK for an incidence rate of >1:260K (not all patient with the disease are reported). These rates are in-line with Ultragenyx's estimate of 3,000 to 7,000 patients in the U.S. (1:45K-106K prevalence), although work finding patients is underway.

Triheptanoin (UX007) is substrate replacement for both LC-FAOD and Glut1 DS:

Trihepatanoin is an orally-administered triglyceride with three seven-carbon arms that can be broken down into smaller components. For the treatment of LC-FAOD, triheptanoin acts as a substrate replacement – it is converted into heptanoate and ketone bodies, which are in turn broken down into byproducts that bypass the defective steps in the Krebs cycle and allow energy production (see Figure 10).

Figure 10: Tripheptanoin bypasses genetic defects to provide substrates for the Krebs cycle



Source: Company presentation

Triheptanoin as a treatment of LC-FAOD has been used extensively in an academic setting, with striking results. Specifically, 48 patients diagnosed with various forms of LC-FAOD demonstrated improvements in multiple symptoms following triheptanoin treatment (see Figure 11; C7/heptanoin is a component of triheptanoin). This study also found that 6% of patients treated with triheptanoin died, vs. 51% in a previous trial with conventional therapy, demonstrating a likely survival benefit. Another trial presented at ICIEM 2013 found a significant 69% reduction ($p = 0.02$) in number of hospital days per year in 20 LC-FAOD patients treated with triheptanoin in a compassionate use program. This also found that a non-significant ($p = 0.11$) reduction in the number of events per year indicating that triheptanoin may be mediating its effects through reducing event severity. Ultragenyx recently initiated a 52-week Phase 2 trial using triheptanoin for treatment of ~20 LC-FAOD patients and expects data in 2015.

Figure 11: Multiple symptoms are reduced following triheptanoin treatment of LC-FAOD patients

	Cardiac		Rhabdomyolysis		Weakness/fatigue		Hypoglycaemia		Hepatomegaly		Retinopathy	
Disorder (no.)	Conv.[a]	C_7[b]	Conv.	C_7	Conv.	C_7	Conv.	C_7	Conv.	C_7	Conv.	C_7
CPT I (2)	0	0	0	0	2	0	2	0	2	0	0	0
CACT (1)	Intervened at birth, asymptomatic by 7 months, died with rotavirus											
CPT II (7)	1	0	6	1	7	0	4	0	2	0	0	0
VLCAD (19)	8	1	18	10	18	3	11	1	13	1	0	0
LCHAD (9)	0	0	7	1	8	1	4	0	5	1	3	3
TFP (5)	1	0	5	3	5	4	1	0	1	0	0	0
'SCAD' (5)	0	0	0	0	4	2	2	0	3	0	0	0
Total (48)	10	1	36	15	44	10	24	1	26	2	3	3

[a]Conv = conventional diet (Mct and/or low-fat, high-carbohydrate
[b]C_7 = heptanoate

Source: Roe and Mochel., 2006

Evidence of triheptanoin efficacy to treat Glut1 DS is limited to mechanistic explanations (triheptanoin is metabolized to heptanoate, which should bypass the need for GLUT1-mediated entry into the brain), preclinical evidence (a diet containing triheptanoin reduces the number of seizures in a mouse with a genetic proclivity) and anecdotal evidence in humans. Ultragenyx recently initiated a Phase 2 trial of triheptanoin for ~50 pediatric patients with Glut1DS, with data expected in 2015.

Hereditary inclusion body myopathy (HIBM)/GNE myopathy

Hereditary inclusion body myopathy (HIBM) is a genetic disease characterized by adult-onset (late-teens/20s) muscle weakness that usually starts in the distal muscles of the legs and spreads to the more central muscles (with the exception of the quadriceps) – although Japanese patients often have earlier onset, and Jewish patients later. It is caused by recessive mutations in the gene GNE that encodes the protein udp-n-acetylglucosamine 2-epimerase/n-acetylmannosamine kinase, which is involved in the production of sialic acid – the most common V572L mutation is the most severe. In patients with HIBM, sialic acid production is impaired, which causes death of muscle cells through mechanisms that are not fully understood. However, most patients with HIBM become wheelchair-bound within 20 years of diagnosis. Diagnosis is by muscle biopsy (looking for characteristic pathology) and genetic sequencing. There is no curative treatment for HIBM at present, with most therapies being palliative (e.g., physical therapy). Prevalence estimates are ~1-3/1M WW (Khademian *et al.*, 2012), with higher concentrations found in Persian Jewish (~1-2/1K) and Japanese populations (~300-400 patients). Ultragenyx estimates ~1.2K – 2K patients in the developed world have HIBM.

Extended-release sialic acid (SA-ER) demonstrates preliminary efficacy in a Phase 2 trial: Sialic acid (SA) is a natural choice for treatment, given the reduction of sialic acid causes the muscle weakness that is characteristic of HIBM. SA-ER is SA formulated to release more slowly in the gut. SA-ER is being evaluated in a Phase 2 trial of 47 patients with HIBM. The first 24 weeks were randomized and placebo-controlled, placebo vs. 3g/day vs. 6g/day. At 24 weeks, patients on placebo we randomized to either drug arm, and treatment was continued through 48 weeks. At 48 weeks, SA-ER efficacy was evaluated via

measurements of upper and lower extremity muscle strength tests, with the patients on the 6g dose group demonstrating a "modest" increase in upper extremity muscle strength vs. a decline in the 3g group. For measurements of lower extremities, neither the 3g nor the 6g group demonstrated decline, and there was no statistical difference between the groups. Healthier patients (>200m walking ability at baseline) demonstrated better results. Ultragenyx will test higher doses (12g/day) and present full data from this trial in 2014.

The competitive landscape is relatively clean for Ultragenyx

Of Ultragenyx's five lead indications, we see potential pipeline competitors in all but MPS VII and LC-FAOD:

- **XLH:** Two drugs have demonstrated early, pre-clinical efficacy for XLH: Hexa-D-arginine (D6R) and a C-terminal FGF23 tail. D6R works via activation of the SPC2 protein (subtilisin-like protein convertase 2), which in turn inhibits FGF23. The C-terminal FGF23 competes with normal FGF23, inhabiting complex formation and reducing FGF23 activity. Both have demonstrated activity in academic pre-clinical models via serum phosphate normalization in the same mouse model of XLH (the *Hyp* mouse). However, given both molecules work through similar mechanisms as FGF23 and neither has moved into the clinic, we see FGF23 as having the advantage.
- **Glut 1 DS:** While the ketogenic diet is the standard of care for patients with Glut 1 DS and is acceptable to infants who will eat the same food on a daily basis, compliance becomes an issue in adolescence – a critical point in neuronal development. While our physician feedback indicates academics are pursuing gene therapy to treat this disease, we are not aware of any published data on this approach. Given the nascent state of commercial-stage gene therapy and the clinical timeframe, we do not anticipate a gene therapy approach will be competitive with triheptanoin for Glut1 DS in the near term.
- **HIBM:** The NIH has run a Phase 1 trial using DEX-M74/ManNAc, a sialic acid precursor, for treatment of HIBM. Although our physician feedback found support for this approach – noting efficacy in a mouse model and a potentially better PK/PD profile vs. SA-ER we are not aware of clinical data from this trial or commercial development of ManNAc. As a result, we like the relative competitive position for SA-ER, but will continue to monitor ManNAc development.

INTELLECTUAL PROPERTY – ULTRAGENYX HAS LICENSED OR IS FILING FOR EXTENSIVE PATENT PROTECTION

Ultragenyx holds patents, including a license for the WW commercialization rights for rhGUS, on which Ultragenyx owes St. Louis University a mid-single-digit royalty on net sales. Ultragenyx is in the process of filing for patents related to rhGUS composition and treatment therapy (e.g., dosing, regiment, formulation and manufacturing). For KRN23, Ultragenyx has a collaboration and license agreement with Kyowa Hakko Kirin (KHK) that includes three regions: 1) U.S./Canada, where Ultragenyx receives a 50:50 profit share on the first five years of the launch and a tiered mid-to-high-20% royalty on net sales thereafter; 2) E.U., Switzerland and Turkey, where Ultragenyx receives a 10% royalty on net sales; and 3) Mexico and Latin America, where Ultragenyx commercializes KRN23 and pays KHK a low single-digit royalty on net sales. The 20 issued patents in the U.S. covering KRN23 expire in 2022-2029 without extensions. Ultragenyx has licensed 24 patents related to triheptanoin from Baylor Research Institute that expire in 2020-2024; licensed patent

applications expire in 2020-2034. Ultragenyx owes a mid-single-digit royalty on net sales. For SA-ER, Ultragenyx and Nobelpharma granted each other a WW exclusive license to IP related to SA-ER, where Ultragenyx is required to pay a high single-digit royalty on sales in its territories (ex-Asia), and Nobelpharma is required to pay a mid-single-digit royalty on sales ex-Japan. SA-ER patents in the U.S. expire from 2028-2033.

FINANCIALS – CASH ON HAND SUFFICIENT THROUGH EARLY 2016

In Q3/13, Ultragenyx reported no revenue and a net loss of $8.4M, or $2.58 per share. The company has 23.3M shares outstanding, not including 4.7M shares issuable upon exercise of options, warrants and equity awards or the 6.6M shares issues in the IPO in February.

As of September 30, 2013, Ultragenyx had $63.7M in cash and equivalents, not including $126.4M net proceeds from the IPO. At the current run rate, we believe Ultragenyx's cash balance is sufficient to fund operations through early-2016. Due to the costs associated with clinical development of five products, we model for two equity offerings: $150M in mid-2015 (1.76M shares at $85/share) and $200M in mid-2017 (2M shares at $100/share).

Figure 12: KRN23 and rhGUS revenue builds

KRN23 market

U.S. / Canada		FY 2011A	FY 2012A	FY 2013E	Mar 1Q14E	Jun 2Q14E	Sep 3Q14E	Dec 4Q14E	FY 2014E	FY 2015E	FY 2016E	FY 2017E	FY 2018E	FY 2019E	FY 2020E	FY 2021E	FY 2022E	FY 2023E	FY 2024E	FY 2025E	FY 2026E	FY 2027E	FY 2028E
Total U.S. + Canada population ('000)	1%	345,067	348,518	352,003	352,880	353,758	354,640	355,523	355,523	359,078	362,669	366,296	369,959	373,658	377,395	381,169	384,980	388,830	392,718	396,646	400,612	404,618	408,664
Prevalence of XLH		*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*
Number of pediatric XLH patients	25%	3,278	3,311	3,344	3,352	3,361	3,369	3,377	3,377	3,411	3,445	3,480	3,515	3,550	3,585	3,621	3,657	3,694	3,731	3,768	3,806	3,844	3,882
Penetration into pediatric XLH population		*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*1.5%*	*14.0%*	*22.2%*	*27.0%*	*31.0%*	*34.5%*	*37.0%*	*38.5%*	*39.5%*	*40.3%*	*40.9%*
Number of adult XLH patients		9,834	9,933	10,032	10,057	10,082	10,107	10,132	10,132	10,234	10,336	10,439	10,544	10,649	10,756	10,863	10,972	11,082	11,192	11,304	11,417	11,532	11,647
Penetration into adult XLH population		*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.3%*	*7.0%*	*10.4%*	*13.0%*	*14.2%*	*15.3%*	*16.0%*	*16.6%*	*17.2%*	*17.7%*	*18.2%*
Total number of XLH patients on KRN23		-	-	-	-	-	-	-	-	-	-	-	84	1,242	1,915	2,390	2,692	2,970	3,171	3,327	3,467	3,590	3,708
Gross cost per treatment per patient		nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 85,000	$ 87,550	$ 90,177	$ 92,882	$ 95,668	$ 98,538	$ 101,494	$ 104,539	$ 107,675	$ 110,906	$ 114,233
Net cost per treatment per patient	85%	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 85,000	$ 87,550	$ 90,177	$ 92,882	$ 95,668	$ 98,538	$ 101,494	$ 104,539	$ 107,675	$ 110,906	$ 114,233
Total U.S. + Canada KRN23 sales ($'000)		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ 7,170	$ 108,773	$ 172,645	$ 221,981	$ 257,518	$ 292,647	$ 321,859	$ 347,830	$ 373,321	$ 398,171	$ 423,531
COGS (% of Sales)		nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	5.0%	15.0%	15.0%	15.0%	15.0%						
COGS ('000)		nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$358	$16,316	$25,897	$33,297	$38,628						
Gross Profit		nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 6,811	$ 92,457	$ 146,748	$ 188,684	$ 218,890						
Sales and Marketing (% of Sales)		nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	50.0%	45.0%	35.0%	30.0%	28.0%						
Sales and Marketing ('000)		nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$3,585	$48,948	$60,426	$66,594	$72,105						
R&D (% of Sales)		nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	20.0%	15.0%	13.0%	12.0%	11.0%						
R&D ('000)		nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$1,434	$16,316	$22,444	$26,638	$28,327						
Net Income ('000)		nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 1,792	$ 27,193	$ 63,879	$ 95,452	$ 118,458	*25%*	*26%*	*27%*	*28%*	*29%*	*29%*
Total U.S./Canada KRN23 royalty/profit share to RARE ($'0		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ 896	$ 13,597	$ 31,939	$ 47,726	$ 59,229	$ 73,162	$ 83,683	$ 93,914	$ 104,530	$ 115,470	$ 122,824
													Profit share					**Royalty**						

E.U.		FY 2011A	FY 2012A	FY 2013E	Mar 1Q14E	Jun 2Q14E	Sep 3Q14E	Dec 4Q14E	FY 2014E	FY 2015E	FY 2016E	FY 2017E	FY 2018E	FY 2019E	FY 2020E	FY 2021E	FY 2022E	FY 2023E	FY 2024E	FY 2025E	FY 2026E	FY 2027E	FY 2028E
Total E.U. population ('000)	1%	508,857	513,946	519,085	520,378	521,674	522,973	524,276	524,276	529,519	534,814	540,162	545,564	551,019	556,529	562,095	567,716	573,393	579,127	584,918	590,767	596,675	602,642
Prevalence of XLH		*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*
Number of pediatric XLH patients	25%	4,834	4,882	4,931	4,944	4,956	4,968	4,981	4,981	5,030	5,081	5,132	5,183	5,235	5,287	5,340	5,393	5,447	5,502	5,557	5,612	5,668	5,725
Penetration into pediatric XLH population		*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*2.0%*	*8.0%*	*15.0%*	*20.0%*	*23.6%*	*26.0%*	*27.6%*	*29.0%*	*29.8%*	*30.4%*
Number of adult XLH patients		14,502	14,647	14,794	14,831	14,868	14,905	14,942	14,942	15,091	15,242	15,395	15,549	15,704	15,861	16,020	16,180	16,342	16,505	16,670	16,837	17,005	17,175
Penetration into adult XLH population		*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*2.0%*	*5.8%*	*8.4%*	*10.0%*	*11.4%*	*12.6%*	*13.4%*	*13.8%*	*14.0%*
Total number of XLH patients on KRN23		-	-	-	-	-	-	-	-	-	-	-	-	105	740	1,730	2,438	2,920	3,312	3,634	3,884	4,036	4,145
Gross cost per treatment per patient		nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 100,000	$ 100,000	$ 100,000	$ 100,000	$ 100,000	$ 100,000	$ 100,000	$ 100,000	$ 100,000	$ 100,000	$ 100,000
Net cost per treatment per patient	85%	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 85,000	$ 85,000	$ 85,000	$ 85,000	$ 85,000	$ 85,000	$ 85,000	$ 85,000	$ 85,000	$ 85,000	$ 85,000
Total E.U. KRN23 sales ($'000)		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ 8,899	$ 62,916	$ 147,061	$ 207,211	$ 248,176	$ 281,522	$ 308,898	$ 330,115	$ 343,052	$ 352,322
Total E.U. KRN23 royalty to RARE ($'000)		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ 890	$ 6,292	$ 14,706	$ 20,721	$ 24,818	$ 28,152	$ 30,890	$ 33,011	$ 34,305	$ 35,232

ROW (Latin America)		FY 2011A	FY 2012A	FY 2013E	Mar 1Q14E	Jun 2Q14E	Sep 3Q14E	Dec 4Q14E	FY 2014E	FY 2015E	FY 2016E	FY 2017E	FY 2018E	FY 2019E	FY 2020E	FY 2021E	FY 2022E	FY 2023E	FY 2024E	FY 2025E	FY 2026E	FY 2027E	FY 2028E
Total Latin America population ('000)	1%	581,400	587,214	593,086	594,563	596,044	597,529	599,017	599,017	605,007	611,057	617,169	623,339	629,573	635,869	642,227	648,650	655,136	661,687	668,304	674,987	681,737	688,555
Prevalence of XLH		*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*	*0.004%*
Number of pediatric XLH patients	25%	5,523	5,579	5,634	5,648	5,662	5,677	5,691	5,691	5,748	5,805	5,863	5,922	5,981	6,041	6,101	6,162	6,224	6,286	6,349	6,412	6,477	6,541
Penetration into pediatric XLH population		*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.5%*	*5.0%*	*7.0%*	*7.9%*	*8.6%*	*9.0%*	*9.3%*	*9.6%*	*9.8%*	*10.1%*
Number of adult XLH patients		16,570	16,736	16,903	16,945	16,987	17,030	17,072	17,072	17,243	17,415	17,589	17,765	17,943	18,122	18,303	18,487	18,671	18,858	19,047	19,237	19,430	19,624
Penetration into adult XLH population		*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*1.1%*	*2.8%*	*3.7%*	*4.2%*	*4.5%*	*4.7%*	*4.8%*	*4.9%*	*5.0%*
Total number of XLH patients on KRN23		-	-	-	-	-	-	-	-	-	-	-	-	30	501	940	1,171	1,319	1,414	1,486	1,539	1,587	1,642
Gross cost per treatment per patient		nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 100,000	$ 100,000	$ 100,000	$ 100,000	$ 100,000	$ 100,000	$ 100,000	$ 100,000	$ 100,000	$ 100,000
Net cost per treatment per patient	85%	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 85,000	$ 85,000	$ 85,000	$ 85,000	$ 85,000	$ 85,000	$ 85,000	$ 85,000	$ 85,000	$ 85,000
Total ROW KRN23 sales ($'000)		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ 2,542	$ 42,618	$ 79,864	$ 99,519	$ 112,153	$ 120,220	$ 126,279	$ 130,813	$ 134,873	$ 139,558
TOTAL KRN23 sales - WW ($'000)		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ 7,170	$ 120,214	$ 278,178	$ 448,906	$ 564,248	$ 652,976	$ 723,602	$ 783,007	$ 834,249	$ 876,097	$ 915,411

rhGUS market

U.S.		FY 2011A	FY 2012A	FY 2013E	Mar 1Q14E	Jun 2Q14E	Sep 3Q14E	Dec 4Q14E	FY 2014E	FY 2015E	FY 2016E	FY 2017E	FY 2018E	FY 2019E	FY 2020E	FY 2021E	FY 2022E	FY 2023E	FY 2024E	FY 2025E	FY 2026E	FY 2027E	FY 2028E
Total U.S. population ('000)	1%	311,590	314,706	317,853	318,645	319,438	320,234	321,031	321,031	324,242	327,484	330,759	334,067	337,407	340,781	344,189	347,631	351,107	354,618	358,165	361,746	365,364	369,017
Prevalence of MPS 7		*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*
Total number of cases of MPS 7		34	35	35	35	35	35	35	35	36	36	36	37	37	37	38	38	39	39	39	40	40	41
Penetration into eligible MPS 7 population		*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*5.0%*	*51.1%*	*76.3%*	*85.0%*	*88.3%*	*90.0%*	*91.1%*	*92.0%*	*92.4%*	*92.6%*	*92.7%*	*92.8%*
Total number of MPS 7 patients on rhGUS		-	-	-	-	-	-	-	-	-	-	-	19	28	32	33	34	35	36	36	37	37	38
Gross cost per treatment per patient		nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 400,000	$ 412,000	$ 424,360	$ 437,091	$ 450,204	$ 463,710	$ 477,621	$ 491,950	$ 506,708	$ 521,909	$ 537,567	$ 553,694
Net cost per treatment per patient	85%	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 340,000	$ 350,200	$ 360,706	$ 371,527	$ 382,673	$ 394,153	$ 405,978	$ 418,157	$ 430,702	$ 443,623	$ 456,932	$ 470,640
Total U.S. rhGUS sales ($'000)		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ 619	$ 6,576	$ 10,215	$ 11,838	$ 12,793	$ 13,565	$ 14,284	$ 15,007	$ 15,679	$ 16,346	$ 17,024	$ 17,729

ROW		FY 2011A	FY 2012A	FY 2013E	Mar 1Q14E	Jun 2Q14E	Sep 3Q14E	Dec 4Q14E	FY 2014E	FY 2015E	FY 2016E	FY 2017E	FY 2018E	FY 2019E	FY 2020E	FY 2021E	FY 2022E	FY 2023E	FY 2024E	FY 2025E	FY 2026E	FY 2027E	FY 2028E
Total ROW population ('000)	1%	1,500,000	1,515,000	1,530,150	1,533,961	1,537,782	1,541,612	1,545,452	1,545,452	1,560,906	1,576,515	1,592,280	1,608,203	1,624,285	1,640,528	1,656,933	1,673,503	1,690,238	1,707,140	1,724,211	1,741,453	1,758,868	1,776,457
Prevalence of MPS 7		*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*	*0.000011%*
Total number of cases of MPS 7		165	167	168	169	169	170	170	170	172	173	175	177	179	180	182	184	186	188	190	192	193	195
Penetration into eligible MPS 7 population		*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*12.3%*	*41.1%*	*59.1%*	*68.3%*	*72.4%*	*74.6%*	*76.3%*	*77.8%*	*79.0%*	*80.0%*	*80.4%*
Total number of MPS 7 patients on rhGUS		-	-	-	-	-	-	-	-	-	-	-	22	73	107	124	133	139	143	148	151	155	157
Gross cost per treatment per patient		nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 400,000	$ 400,000	$ 400,000	$ 400,000	$ 400,000	$ 400,000	$ 400,000	$ 400,000	$ 400,000	$ 400,000	$ 400,000
Net cost per treatment per patient	85%	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 340,000	$ 340,000	$ 340,000	$ 340,000	$ 340,000	$ 340,000	$ 340,000	$ 340,000	$ 340,000	$ 340,000	$ 340,000
Total U.S. rhGUS sales ($'000)		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ 7,398	$ 24,968	$ 36,261	$ 42,325	$ 45,314	$ 47,158	$ 48,715	$ 50,170	$ 51,453	$ 52,625	$ 53,417
TOTAL rhGUS sales - WW ($'000)		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ 619	$ 13,974	$ 35,182	$ 48,099	$ 55,118	$ 58,879	$ 61,442	$ 63,722	$ 65,849	$ 67,799	$ 69,649	$ 71,146

Source: Company reports, Canaccord Genuity estimates

Figure 13: Triheptanoin and SA-ER revenue builds

Triheptanoin market

U.S.		FY 2011A	FY 2012A	FY 2013E	Mar 1Q14E	Jun 2Q14E	Sep 3Q14E	Dec 4Q14E	FY 2014E	FY 2015E	FY 2016E	FY 2017E	FY 2018E	FY 2019E	FY 2020E	FY 2021E	FY 2022E	FY 2023E	FY 2024E	FY 2025E	FY 2026E	FY 2027E	FY 2028E
Total U.S. population ('000)		311,590	314,706	317,853	318,645	319,438	320,234	321,031	321,031	324,242	327,484	330,759	334,067	337,407	340,781	344,189	347,631	351,107	354,618	358,165	361,746	365,364	369,017
Prevalence of LC-FAOD		*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*
Total number of cases of LC-FAOD		2,493	2,518	2,543	2,549	2,556	2,562	2,568	2,568	2,594	2,620	2,646	2,673	2,699	2,726	2,754	2,781	2,809	2,837	2,865	2,894	2,923	2,952
Penetration into LC-FAOD population		*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.2%*	*0.5%*	*12.5%*	*17.5%*	*20.3%*	*22.9%*	*23.4%*	*23.9%*	*24.4%*	*24.9%*	*25.4%*
Prevalence of Glut1 DS		*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*
Total number of cases of Glut1 DS		4,051	4,091	4,132	4,142	4,153	4,163	4,173	4,173	4,215	4,257	4,300	4,343	4,386	4,430	4,474	4,519	4,564	4,610	4,656	4,703	4,750	4,797
Penetration into Glut1DS population		*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.1%*	*3.5%*	*10.5%*	*16.5%*	*20.0%*	*23.0%*	*24.8%*	*26.4%*	*27.6%*	*28.4%*	*28.8%*
Total number of patients on triheptanoin		-	-	-	-	-	-	-	-	-	-	-	10	329	806	1,220	1,468	1,690	1,807	1,914	2,004	2,077	2,131
Gross cost per treatment per patient		nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 75,000	$ 77,250	$ 79,568	$ 81,955	$ 84,413	$ 86,946	$ 89,554	$ 92,241	$ 95,008	$ 97,858	$ 100,794
Net cost per treatment per patient	85%	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 63,750	$ 65,663	$ 67,632	$ 69,661	$ 71,751	$ 73,904	$ 76,121	$ 78,404	$ 80,757	$ 83,179	$ 85,675
Total U.S. Triheptanoin sales ($'000)		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ 618	$ 21,601	$ 54,508	$ 84,997	$ 105,359	$ 124,914	$ 137,561	$ 150,069	$ 161,842	$ 172,741	$ 182,611

E.U.		FY 2011A	FY 2012A	FY 2013E	Mar 1Q14E	Jun 2Q14E	Sep 3Q14E	Dec 4Q14E	FY 2014E	FY 2015E	FY 2016E	FY 2017E	FY 2018E	FY 2019E	FY 2020E	FY 2021E	FY 2022E	FY 2023E	FY 2024E	FY 2025E	FY 2026E	FY 2027E	FY 2028E
Total E.U. population ('000)		508,857	513,946	519,085	520,378	521,674	522,973	524,276	524,276	529,519	534,814	540,162	545,564	551,019	556,529	562,095	567,716	573,393	579,127	584,918	590,767	596,675	602,642
Prevalence of LC-FAOD		*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*
Total number of cases of LC-FAOD		4,071	4,112	4,153	4,163	4,173	4,184	4,194	4,194	4,236	4,279	4,321	4,365	4,408	4,452	4,497	4,542	4,587	4,633	4,679	4,726	4,773	4,821
Penetration into LC-FAOD population		*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*5.6%*	*13.5%*	*18.5%*	*20.0%*	*21.3%*	*22.0%*	*22.8%*	*23.4%*	*23.9%*	*24.2%*
Prevalence of Glut1 DS		*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*
Total number of cases of Glut1 DS		6,615	6,681	6,748	6,765	6,782	6,799	6,816	6,816	6,884	6,953	7,022	7,092	7,163	7,235	7,307	7,380	7,454	7,529	7,604	7,680	7,757	7,834
Penetration into Glut1DS population		*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.9%*	*6.5%*	*15.5%*	*19.8%*	*21.6%*	*23.0%*	*24.0%*	*24.8%*	*25.4%*	*25.8%*
Total number of patients on triheptanoin		-	-	-	-	-	-	-	-	-	-	-	-	311	1,071	1,965	2,370	2,587	2,751	2,892	3,011	3,111	3,188
Gross cost per treatment per patient		nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 75,000	$ 75,000	$ 75,000	$ 75,000	$ 75,000	$ 75,000	$ 75,000	$ 75,000
Net cost per treatment per patient	85%	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 63,750	$ 63,750	$ 63,750	$ 63,750	$ 63,750	$ 63,750	$ 63,750	$ 63,750
Total E.U. Triheptanoin sales ($'000)		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ 125,238	$ 151,065	$ 164,931	$ 175,367	$ 184,354	$ 191,923	$ 198,330	$ 203,233

ROW		FY 2011A	FY 2012A	FY 2013E	Mar 1Q14E	Jun 2Q14E	Sep 3Q14E	Dec 4Q14E	FY 2014E	FY 2015E	FY 2016E	FY 2017E	FY 2018E	FY 2019E	FY 2020E	FY 2021E	FY 2022E	FY 2023E	FY 2024E	FY 2025E	FY 2026E	FY 2027E	FY 2028E
Total ROW population ('000)	1%	706,400	713,464	720,599	722,393	724,193	725,996	727,805	727,805	735,083	742,433	749,858	757,356	764,930	772,579	780,305	788,108	795,989	803,949	811,989	820,108	828,310	836,593
Prevalence of LC-FAOD		*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*	*0.0008%*
Total number of cases of LC-FAOD		5,651	5,708	5,765	5,779	5,794	5,808	5,822	5,822	5,881	5,939	5,999	6,059	6,119	6,181	6,242	6,305	6,368	6,432	6,496	6,561	6,626	6,693
Penetration into LC-FAOD population		*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*4.3%*	*9.0%*	*12.3%*	*14.8%*	*16.4%*	*17.4%*	*18.0%*	*18.4%*	*18.7%*
Prevalence of Glut1 DS		*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*	*0.0013%*
Total number of cases of Glut1 DS		9,183	9,275	9,368	9,391	9,415	9,438	9,461	9,461	9,556	9,652	9,748	9,846	9,944	10,044	10,144	10,245	10,348	10,451	10,556	10,661	10,768	10,876
Penetration into Glut1DS population		*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*1.3%*	*6.7%*	*10.0%*	*12.6%*	*14.6%*	*16.3%*	*17.8%*	*19.0%*	*19.5%*
Total number of patients on triheptanoin		-	-	-	-	-	-	-	-	-	-	-	-	-	396	1,241	1,800	2,246	2,581	2,851	3,079	3,265	3,372
Gross cost per treatment per patient		nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 75,000	$ 75,000	$ 75,000	$ 75,000	$ 75,000	$ 75,000	$ 75,000	$ 75,000
Net cost per treatment per patient	85%	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 63,750	$ 63,750	$ 63,750	$ 63,750	$ 63,750	$ 63,750	$ 63,750	$ 63,750
Total ROW Triheptanoin sales ($'000)		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ 79,143	$ 114,752	$ 143,200	$ 164,518	$ 181,744	$ 196,266	$ 208,156	$ 214,984
TOTAL Triheptanoin sales - WW ($'000)		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ 618	$ 21,601	$ 54,508	$ 289,379	$ 371,176	$ 433,046	$ 477,446	$ 516,167	$ 550,031	$ 579,227	$ 600,829

SA-ER market

U.S.		FY 2011A	FY 2012A	FY 2013E	Mar 1Q14E	Jun 2Q14E	Sep 3Q14E	Dec 4Q14E	FY 2014E	FY 2015E	FY 2016E	FY 2017E	FY 2018E	FY 2019E	FY 2020E	FY 2021E	FY 2022E	FY 2023E	FY 2024E	FY 2025E	FY 2026E	FY 2027E	FY 2028E
Total U.S. population ('000)		311,590	314,706	317,853	318,645	319,438	320,234	321,031	321,031	324,242	327,484	330,759	334,067	337,407	340,781	344,189	347,631	351,107	354,618	358,165	361,746	365,364	369,017
Prevalence of HIBM		*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*
Total number of eligible new cases of HIBM		343	346	350	351	351	352	353	353	357	360	364	367	371	375	379	382	386	390	394	398	402	406
Penetration into eligible HIBM population		*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*5.0%*	*15.0%*	*35.0%*	*55.5%*	*70.5%*	*80.3%*	*82.4%*	*83.9%*	*84.9%*	*85.5%*	*85.7%*
Total number of HIBM patients on SA-ER		-	-	-	-	-	-	-	-	-	-	-	18	56	131	210	270	310	321	331	338	344	348
Gross cost per treatment per patient		nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 50,000	$ 51,500	$ 54,636	$ 56,275	$ 57,964	$ 59,703	$ 61,494	$ 63,339	$ 65,239	$ 67,196	$ 69,212
Net cost per treatment per patient	85%	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 42,500	$ 43,775	$ 45,088	$ 46,441	$ 47,834	$ 49,269	$ 50,747	$ 52,270	$ 53,838	$ 55,453	$ 57,116	$ 58,830
Total U.S. SA-ER sales ($'000)		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ 804	$ 2,510	$ 6,093	$ 10,051	$ 13,282	$ 15,738	$ 16,801	$ 17,796	$ 18,734	$ 19,627	$ 20,465

E.U.		FY 2011A	FY 2012A	FY 2013E	Mar 1Q14E	Jun 2Q14E	Sep 3Q14E	Dec 4Q14E	FY 2014E	FY 2015E	FY 2016E	FY 2017E	FY 2018E	FY 2019E	FY 2020E	FY 2021E	FY 2022E	FY 2023E	FY 2024E	FY 2025E	FY 2026E	FY 2027E	FY 2028E
Total E.U. population ('000)		508,857	513,946	519,085	520,378	521,674	522,973	524,276	524,276	529,519	534,814	540,162	545,564	551,019	556,529	562,095	567,716	573,393	579,127	584,918	590,767	596,675	602,642
Prevalence of HIBM		*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*
Total number of eligible new cases of HIBM		560	565	571	572	574	575	577	577	582	588	594	600	606	612	618	624	631	637	643	650	656	663
Penetration into eligible HIBM population		*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*1.0%*	*10.4%*	*27.5%*	*45.5%*	*60.4%*	*70.0%*	*75.0%*	*79.0%*	*81.0%*	*82.0%*	*83.0%*
Total number of HIBM patients on SA-ER		-	-	-	-	-	-	-	-	-	-	-	6	63	168	281	377	442	478	508	526	538	550
Gross cost per treatment per patient		nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 50,000	$ 51,500	$ 54,636	$ 56,275	$ 57,964	$ 59,703	$ 61,494	$ 63,339	$ 65,239	$ 67,196	$ 69,212
Net cost per treatment per patient	85%	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 42,500	$ 43,775	$ 45,088	$ 46,441	$ 47,834	$ 49,269	$ 50,747	$ 52,270	$ 53,838	$ 55,453	$ 57,116	$ 58,830
Total E.U. SA-ER sales ($'000)		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ 263	$ 2,842	$ 7,818	$ 13,457	$ 18,584	$ 22,406	$ 24,973	$ 27,365	$ 29,189	$ 30,740	$ 32,369

ROW		FY 2011A	FY 2012A	FY 2013E	Mar 1Q14E	Jun 2Q14E	Sep 3Q14E	Dec 4Q14E	FY 2014E	FY 2015E	FY 2016E	FY 2017E	FY 2018E	FY 2019E	FY 2020E	FY 2021E	FY 2022E	FY 2023E	FY 2024E	FY 2025E	FY 2026E	FY 2027E	FY 2028E
Total ROW population (ex-Japan; '000)	1%	500,000	505,000	510,050	511,320	512,594	513,871	515,151	513,234	518,366	523,550	528,785	534,073	539,414	544,808	550,256	555,759	561,316	566,929	572,599	578,325	584,108	589,949
Prevalence of HIBM		*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*	*0.00011%*
Total number of eligible new cases of HIBM		550	556	561	562	564	565	567	565	570	576	582	587	593	599	605	611	617	624	630	636	643	649
Penetration into eligible HIBM population		*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*2.0%*	*10.5%*	*27.5%*	*45.0%*	*56.0%*	*60.1%*	*63.2%*	*64.8%*	*65.8%*	*66.3%*	*66.7%*
Total number of HIBM patients on SA-ER		-	-	-	-	-	-	-	-	-	-	-	12	62	165	272	342	371	394	408	419	426	433
Gross cost per treatment per patient		nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 50,000	$ 50,000	$ 50,000	$ 50,000	$ 50,000	$ 50,000	$ 50,000	$ 50,000	$ 50,000	$ 50,000	$ 50,000
Net cost per treatment per patient	85%	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	$ 42,500	$ 42,500	$ 42,500	$ 42,500	$ 42,500	$ 42,500	$ 42,500	$ 42,500	$ 42,500	$ 42,500	$ 42,500
Total ROW SA-ER sales ($'000)		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ 499	$ 2,648	$ 7,004	$ 11,576	$ 14,550	$ 15,771	$ 16,750	$ 17,346	$ 17,790	$ 18,105	$ 18,396
												Royalty	5%	5%	5%	5%	5%	5%	5%	5%	5%	5%	5%
Total ROW KRN23 royalty/profit share to RARE ($'000)		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ 25	$ 132	$ 350	$ 579	$ 727	$ 789	$ 838	$ 867	$ 890	$ 905	$ 920
TOTAL SA-ER sales - WW ($'000)		$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ 1,566	$ 8,000	$ 20,916	$ 35,084	$ 46,416	$ 53,915	$ 58,525	$ 62,508	$ 65,713	$ 68,471	$ 71,230

Source: Company reports, Canaccord Genuity estimates

Figure 14: Ultragenyx income statement

($thousands, except per share data)

	FY 2011A	FY 2012A	Mar 1Q13A	Jun 2Q13A	Sep 3Q13A	Dec 4Q13E	FY 2013E	Mar 1Q14E	Jun 2Q14E	Sep 3Q14E	Dec 4Q14E	FY 2014E	FY 2015E	FY 2016E	FY 2017E	FY 2018E	FY 2019E	FY 2020E	FY 2021E	FY 2022E	FY 2023E	FY 2024E
Revenue																						
KRN23 revenue	$ -	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	2,542	42,618	79,864	99,519	112,153	120,220
rhGUS revenue	$ -	-	-	-	-	-	-	-	-	-	-	-	-	-	619	13,974	35,182	48,099	55,118	58,879	61,442	63,722
Triheptanoin revenue	$ -	-	-	-	-	-	-	-	-	-	-	-	-	-	-	618	21,601	54,508	289,379	371,176	433,046	477,446
SA-ER revenue	$ -	-	-	-	-	-	-	-	-	-	-	-	-	-	-	1,067	5,352	13,911	23,508	31,866	38,144	41,774
Total product revenue	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ 619**	**$ 15,659**	**$ 64,678**	**$ 159,136**	**$ 447,870**	**$ 561,441**	**$ 644,785**	**$ 703,162**
KRN23 U.S./Canada profit share/royalty	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	896	13,597	31,939	47,726	59,229	73,162	83,683
KRN23 E.U. royalty	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	890	6,292	14,706	20,721	24,818	28,152
SA-ER ex-U.S. royalty	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	25	132	350	579	727	789	838
Total profit share / royalty	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ 921**	**$ 14,619**	**$ 38,581**	**$ 63,011**	**$ 80,678**	**$ 98,768**	**$ 112,673**
Total Revenue	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ 619**	**$ 16,580**	**$ 79,297**	**$ 197,717**	**$ 510,880**	**$ 642,119**	**$ 743,553**	**$ 815,835**
COGS	-	-	-	-	-	-	-	-	-	-	-	-	-	-	100	10,749	2,179	15,014	55,921	77,634	94,218	102,488
Gross profit	-	-	-	-	-	-	-	-	-	-	-	-	-	-	519	5,831	77,117	182,704	454,959	564,484	649,334	713,347
Operating expense																						
R&D (GAAP)	4,717	12,641	5,664	7,120	6,762	7,511	27,056	9,450	10,779	12,801	14,374	47,404	59,103	66,129	73,120	80,181	88,179	96,330	104,216	112,667	120,556	128,294
SG&A (GAAP)	1,844	3,344	1,083	1,048	999	1,286	4,416	1,780	2,264	2,511	3,109	9,664	12,785	17,204	22,212	26,720	29,644	32,601	35,441	38,591	41,698	44,892
Total operating expense (GAAP)	6,561	15,985	6,747	8,168	7,761	8,797	31,472	11,230	13,043	15,312	17,483	57,068	71,888	83,333	95,332	106,901	117,823	128,931	139,657	151,258	162,254	173,186
Operating income (loss)	**(6,561)**	**(15,985)**	**(6,747)**	**(8,168)**	**(7,761)**	**(8,797)**	**(31,472)**	**(11,230)**	**(13,043)**	**(15,312)**	**(17,483)**	**(57,068)**	**(71,888)**	**(83,333)**	**(94,813)**	**(101,070)**	**(40,706)**	**53,773**	**315,302**	**413,226**	**487,080**	**540,161**
Interest income	4	1	26	63	68	59	216	113	164	150	134	561	993	1,319	1,604	1,835	1,285	1,543	4,473	11,064	20,717	33,507
Interest expense	(270)	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Other income (expense), net	(22)	(350)	(14)	(407)	(735)	(750)	(1,905)	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Total other (expense) income, net	(288)	(349)	12	(343)	(666)	(691)	(1,689)	113	164	150	134	561	993	1,319	1,604	1,835	1,285	1,543	4,473	11,064	20,717	33,507
Net gain (loss) before taxes	**(6,849)**	**(16,334)**	**(6,735)**	**(8,511)**	**(8,427)**	**(9,488)**	**(33,161)**	**(11,117)**	**(12,879)**	**(15,162)**	**(17,349)**	**(56,507)**	**(70,895)**	**(82,014)**	**(93,210)**	**(99,235)**	**(39,421)**	**55,316**	**319,775**	**424,291**	**507,797**	**573,668**
Income Tax Provision	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	12,729	35,546	68,840
Net income (loss) attributable to common stockholders	**$ (7,466)**	**$ (19,561)**	**$ (6,735)**	**$ (8,511)**	**$ (8,427)**	**$ (9,488)**	**$ (33,161)**	**$ (11,117)**	**$ (12,879)**	**$ (15,162)**	**$ (17,349)**	**$ (56,507)**	**$ (70,895)**	**$ (82,014)**	**$ (93,210)**	**$ (99,235)**	**$ (39,421)**	**$ 55,316**	**$ 319,775**	**$ 411,562**	**$ 472,251**	**$ 504,828**
EPS (basic and diluted)	**$ (4.62)**	**$ (14.20)**	**$ (3.36)**	**$ (3.23)**	**$ (2.58)**	**$ (0.41)**	**$ (1.42)**	**$ (0.37)**	**$ (0.43)**	**$ (0.50)**	**$ (0.57)**	**$ (1.87)**	**$ (2.20)**	**$ (2.52)**	**$ (2.67)**	**$ (2.82)**	**$ (1.11)**	**$ 1.51**	**$ 8.45**	**$ 10.65**	**$ 11.98**	**$ 12.56**
Weighted shares outstanding diluted	1,617	1,377	2,005	2,633	3,260	23,302	23,418	29,926	30,076	30,226	30,377	30,151	32,217	32,540	34,865	35,214	35,566	36,710	37,865	38,638	39,419	40,207
Margin Analysis:																						
Cost of goods sold	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	16%	69%	3%	9%	12%	14%	15%	15%
KRN23	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	0%	0%	3%	4%	15%	17%	18%	18%
rhGUS	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	0%	1%	2%	14%	16%	18%	18%	18%
Triheptanoin	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	0%	3%	4%	9%	11%	12%	13%	13%
SA-ER	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	0%	9%	10%	12%	14%	18%	18%	18%
Gross margin	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	31%	97%	91%	88%	86%	85%	85%
R&D (GAAP)	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	484%	111%	49%	20%	18%	16%	16%
SG&A (GAAP)	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	161%	37%	16%	7%	6%	6%	6%
Total operating expense	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	645%	149%	65%	27%	24%	22%	21%
Operating margin	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	62%	64%	66%	66%
Income tax provision	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	3%	7%	12%
Net margin (GAAP)	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	63%	64%	64%	62%
Y/Y change:																						
Total revenue	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	2581%	378%	149%	158%	26%	16%	10%
KRN23 revenue	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	1577%	87%	25%	13%	7%
rhGUS revenue	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	2159%	152%	37%	15%	7%	4%	4%
Triheptanoin revenue	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	3398%	152%	431%	28%	17%	10%
SA-ER revenue	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	402%	160%	69%	36%	20%	10%
Total profit share / royalty	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	1487%	164%	63%	28%	22%	14%
KRN23 U.S. / Canada profit share / royalty	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	1417%	135%	49%	24%	24%	14%
KRN23 E.U. royalty	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	607%	134%	41%	20%	13%
SA-ER ex-U.S. royalty	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	430%	165%	65%	26%	8%	6%
R&D (GAAP)	nm	168%	138%	153%	84%	99%	114%	n/a	n/a	n/a	n/a	75%	25%	12%	11%	10%	10%	9%	8%	8%	7%	6%
SG&A (GAAP)	nm	81%	52%	38%	3%	42%	32%	n/a	n/a	n/a	n/a	119%	32%	35%	29%	20%	11%	10%	9%	9%	8%	8%
Total operating expense	nm	144%	118%	129%	67%	88%	97%	n/a	n/a	n/a	n/a	81%	26%	16%	14%	12%	10%	9%	8%	8%	7%	7%
Operating income	nm	144%	118%	129%	67%	88%	97%	n/a	n/a	n/a	n/a	81%	26%	16%	14%	7%	-60%	-232%	486%	31%	18%	11%
Net income (loss)	nm	162%	90%	107%	66%	39%	70%	n/a	n/a	n/a	n/a	70%	25%	16%	14%	6%	-60%	-240%	478%	29%	15%	7%
GAAP EPS (diluted)	nm	208%	35%	-4%	-47%	-92%	-90%	n/a	n/a	n/a	n/a	32%	17%	15%	6%	5%	-61%	-236%	460%	26%	12%	5%
Shares outstanding - GAAP	nm	-15%	41%	115%	216%	1592%	1600%	n/a	n/a	n/a	n/a	29%	7%	1%	7%	1%	1%	3%	3%	2%	2%	2%

Source: Company reports, Canaccord Genuity estimates

APPENDIX: IMPORTANT DISCLOSURES

Analyst Certification: Each authoring analyst of Canaccord Genuity whose name appears on the front page of this research hereby certifies that (i) the recommendations and opinions expressed in this research accurately reflect the authoring analyst's personal, independent and objective views about any and all of the designated investments or relevant issuers discussed herein that are within such authoring analyst's coverage universe and (ii) no part of the authoring analyst's compensation was, is, or will be, directly or indirectly, related to the specific recommendations or views expressed by the authoring analyst in the research.

Analysts employed outside the US are not registered as research analysts with FINRA. These analysts may not be associated persons of Canaccord Genuity Inc. and therefore may not be subject to the NASD Rule 2711 and NYSE Rule 472 restrictions on communications with a subject company, public appearances and trading securities held by a research analyst account.

Compendium Report: If this report covers six or more subject companies, it is a compendium report and Canaccord Genuity and its affiliated companies hereby direct the reader to the specific disclosures related to the subject companies discussed in this report, which may be obtained at the following website (provided as a hyperlink if this report is being read electronically) http://disclosures.canaccordgenuity.com/EN/Pages/default.aspx; or by sending a request to Canaccord Genuity Corp. Research, Attn: Disclosures, P.O. Box 10337 Pacific Centre, 2200-609 Granville Street, Vancouver, BC, Canada V7Y 1H2; or by sending a request by email to disclosures@canaccordgenuity.com. The reader may also obtain a copy of Canaccord Genuity's policies and procedures regarding the dissemination of research by following the steps outlined above.

Site Visit: An analyst has not visited Ultragenyx Pharmaceutical's material operations.

Distribution of Ratings:
Global Stock Ratings
(as of 31 December 2013)

	Coverage Universe		IB Clients
Rating	#	%	%
Buy	564	57.0%	38.1%
Speculative Buy	47	4.7%	42.6%
Hold	325	32.8%	11.4%
Sell	50	5.1%	6.0%
	990	100.0%	

*Total includes stocks that are Under Review

Canaccord Genuity Ratings System:
BUY: The stock is expected to generate risk-adjusted returns of over 10% during the next 12 months.
HOLD: The stock is expected to generate risk-adjusted returns of 0-10% during the next 12 months.
SELL: The stock is expected to generate negative risk-adjusted returns during the next 12 months.
NOT RATED: Canaccord Genuity does not provide research coverage of the relevant issuer.

"Risk-adjusted return" refers to the expected return in relation to the amount of risk associated with the designated investment or the relevant issuer.

Risk Qualifier: **SPECULATIVE:** Stocks bear significantly higher risk that typically cannot be valued by normal fundamental criteria. Investments in the stock may result in material loss.

Canaccord Genuity Research Disclosures as of 25 February 2014

Company	Disclosure
Ultragenyx Pharmaceutical	1A, 2, 3, 5, 7

1	The relevant issuer currently is, or in the past 12 months was, a client of Canaccord Genuity or its affiliated companies. During this period, Canaccord Genuity or its affiliated companies provided the following services to the relevant issuer: A. investment banking services. B. non-investment banking securities-related services. C. non-securities related services.
2	In the past 12 months, Canaccord Genuity or its affiliated companies have received compensation for Corporate Finance/Investment Banking services from the relevant issuer.
3	In the past 12 months, Canaccord Genuity or any of its affiliated companies have been lead manager, co-lead manager or co-manager of a public offering of securities of the relevant issuer or any publicly disclosed offer of securities of the relevant issuer or in any related derivatives.
4	Canaccord Genuity acts as corporate broker for the relevant issuer and/or Canaccord Genuity or any of its affiliated companies may have an agreement with the relevant issuer relating to the provision of Corporate Finance/Investment Banking services.

5	Canaccord Genuity or one or more of its affiliated companies is a market maker or liquidity provider in the securities of the relevant issuer or in any related derivatives.
6	In the past 12 months, Canaccord Genuity, its partners, affiliated companies, officers or directors, or any authoring analyst involved in the preparation of this research has provided services to the relevant issuer for remuneration, other than normal course investment advisory or trade execution services.
7	Canaccord Genuity or one or more of its affiliated companies intend to seek or expect to receive compensation for Corporate Finance/Investment Banking services from the relevant issuer in the next six months.
8	The authoring analyst, a member of the authoring analyst's household, or any individual directly involved in the preparation of this research, has a long position in the shares or derivatives, or has any other financial interest in the relevant issuer, the value of which increases as the value of the underlying equity increases.
9	The authoring analyst, a member of the authoring analyst's household, or any individual directly involved in the preparation of this research, has a short position in the shares or derivatives, or has any other financial interest in the relevant issuer, the value of which increases as the value of the underlying equity decreases.
10	Those persons identified as the author(s) of this research, or any individual involved in the preparation of this research, have purchased/received shares in the relevant issuer prior to a public offering of those shares, and such person's name and details are disclosed above.
11	A partner, director, officer, employee or agent of Canaccord Genuity or its affiliated companies, or a member of his/her household, is an officer, or director, or serves as an advisor or board member of the relevant issuer and/or one of its subsidiaries, and such person's name is disclosed above.
12	As of the month end immediately preceding the date of publication of this research, or the prior month end if publication is within 10 days following a month end, Canaccord Genuity or its affiliated companies, in the aggregate, beneficially owned 1% or more of any class of the total issued share capital or other common equity securities of the relevant issuer or held any other financial interests in the relevant issuer which are significant in relation to the research (as disclosed above).
13	As of the month end immediately preceding the date of publication of this research, or the prior month end if publication is within 10 days following a month end, the relevant issuer owned 1% or more of any class of the total issued share capital in Canaccord Genuity or any of its affiliated companies.
14	Other specific disclosures as described above.

"Canaccord Genuity" is the business name used by certain wholly owned subsidiaries of Canaccord Genuity Group Inc., including Canaccord Genuity Inc., Canaccord Genuity Limited, Canaccord Genuity Corp., and Canaccord Genuity (Australia) Limited, an affiliated company that is 50%-owned by Canaccord Genuity Group Inc.

The authoring analysts who are responsible for the preparation of this research are employed by Canaccord Genuity Corp. a Canadian broker-dealer with principal offices located in Vancouver, Calgary, Toronto, Montreal, or Canaccord Genuity Inc., a US broker-dealer with principal offices located in New York, Boston, San Francisco and Houston, or Canaccord Genuity Limited., a UK broker-dealer with principal offices located in London (UK) and Dublin (Ireland), or Canaccord Genuity (Australia) Limited, an Australian broker-dealer with principal offices located in Sydney and Melbourne.

The authoring analysts who are responsible for the preparation of this research have received (or will receive) compensation based upon (among other factors) the Corporate Finance/Investment Banking revenues and general profits of Canaccord Genuity. However, such authoring analysts have not received, and will not receive, compensation that is directly based upon or linked to one or more specific Corporate Finance/Investment Banking activities, or to recommendations contained in the research.

Canaccord Genuity and its affiliated companies may have a Corporate Finance/Investment Banking or other relationship with the issuer that is the subject of this research and may trade in any of the designated investments mentioned herein either for their own account or the accounts of their customers, in good faith or in the normal course of market making. Accordingly, Canaccord Genuity or their affiliated companies, principals or employees (other than the authoring analyst(s) who prepared this research) may at any time have a long or short position in any such designated investments, related designated investments or in options, futures or other derivative instruments based thereon.

Some regulators require that a firm must establish, implement and make available a policy for managing conflicts of interest arising as a result of publication or distribution of research. This research has been prepared in accordance with Canaccord Genuity's policy on managing conflicts of interest, and information barriers or firewalls have been used where appropriate. Canaccord Genuity's policy is available upon request.

The information contained in this research has been compiled by Canaccord Genuity from sources believed to be reliable, but (with the exception of the information about Canaccord Genuity) no representation or warranty, express or implied, is made by Canaccord Genuity, its affiliated companies or any other person as to its fairness, accuracy, completeness or correctness. Canaccord Genuity has not independently verified the facts, assumptions, and estimates contained herein. All estimates, opinions and other information contained

in this research constitute Canaccord Genuity's judgement as of the date of this research, are subject to change without notice and are provided in good faith but without legal responsibility or liability.
Canaccord Genuity's salespeople, traders, and other professionals may provide oral or written market commentary or trading strategies to our clients and our proprietary trading desk that reflect opinions that are contrary to the opinions expressed in this research. Canaccord Genuity's affiliates, principal trading desk, and investing businesses may make investment decisions that are inconsistent with the recommendations or views expressed in this research.
This research is provided for information purposes only and does not constitute an offer or solicitation to buy or sell any designated investments discussed herein in any jurisdiction where such offer or solicitation would be prohibited. As a result, the designated investments discussed in this research may not be eligible for sale in some jurisdictions. This research is not, and under no circumstances should be construed as, a solicitation to act as a securities broker or dealer in any jurisdiction by any person or company that is not legally permitted to carry on the business of a securities broker or dealer in that jurisdiction. This material is prepared for general circulation to clients and does not have regard to the investment objectives, financial situation or particular needs of any particular person. Investors should obtain advice based on their own individual circumstances before making an investment decision. To the fullest extent permitted by law, none of Canaccord Genuity, its affiliated companies or any other person accepts any liability whatsoever for any direct or consequential loss arising from or relating to any use of the information contained in this research.

For Canadian Residents: This research has been approved by Canaccord Genuity Corp., which accepts sole responsibility for this research and its dissemination in Canada. Canadian clients wishing to effect transactions in any designated investment discussed should do so through a qualified salesperson of Canaccord Genuity Corp. in their particular province or territory.

For United States Residents: Canaccord Genuity Inc., a US registered broker-dealer, accepts responsibility for this research and its dissemination in the United States. Arg. This research is intended for distribution in the United States only to certain US institutional investors. US clients wishing to effect transactions in any designated investment discussed should do so through a qualified salesperson of Canaccord Genuity Inc. Analysts employed outside the US, as specifically indicated elsewhere in this report, are not registered as research analysts with FINRA. These analysts may not be associated persons of Canaccord Genuity Inc. and therefore may not be subject to the NASD Rule 2711 and NYSE Rule 472 restrictions on communications with a subject company, public appearances and trading securities held by a research analyst account.

For United Kingdom and European Residents: This research is distributed in the United Kingdom and elsewhere Europe, as third party research by Canaccord Genuity Limited, which is authorized and regulated by the Financial Conduct Authority. This research is for distribution only to persons who are Eligible Counterparties or Professional Clients only and is exempt from the general restrictions in section 21 of the Financial Services and Markets Act 2000 on the communication of invitations or inducements to engage in investment activity on the grounds that it is being distributed in the United Kingdom only to persons of a kind described in Article 19(5) (Investment Professionals) and 49(2) (High Net Worth companies, unincorporated associations etc) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005 (as amended). It is not intended to be distributed or passed on, directly or indirectly, to any other class of persons. This material is not for distribution in the United Kingdom or elsewhere in Europe to retail clients, as defined under the rules of the Financial Conduct Authority.

For Jersey, Guernsey and Isle of Man Residents: This research is sent to you by Canaccord Genuity Wealth (International) Limited (CGWI) for information purposes and is not to be construed as a solicitation or an offer to purchase or sell investments or related financial instruments. This research has been produced by an affiliate of CGWI for circulation to its institutional clients and also CGWI. Its contents have been approved by CGWI and we are providing it to you on the basis that we believe it to be of interest to you. This statement should be read in conjunction with your client agreement, CGWI's current terms of business and the other disclosures and disclaimers contained within this research. If you are in any doubt, you should consult your financial adviser.
CGWI is licensed and regulated by the Guernsey Financial Services Commission, the Jersey Financial Services Commission and the Isle of Man Financial Supervision Commission. CGWI is registered in Guernsey and is a wholly owned subsidiary of Canaccord Genuity Group Inc.

For Australian Residents: This research is distributed in Australia by Canaccord Genuity (Australia) Limited ABN 19 075 071 466 holder of AFS Licence No 234666. To the extent that this research contains any advice, this is limited to general advice only. Recipients should take into account their own personal circumstances before making an investment decision. Clients wishing to effect any transactions in any financial products discussed in the research should do so through a qualified representative of Canaccord Genuity (Australia) Limited. Canaccord Genuity Wealth Management is a division of Canaccord Genuity (Australia) Limited.

For Singapore Residents:

This research is distributed pursuant to 32C of the Financial Advisers under an arrangement between each of the Canaccord Genuity entities that publish research and Canaccord Genuity Singapore Pte. Ltd who are an exempt financial adviser under section 23(1)(d) of the Financial Advisers Act. This research is only intended for persons who fall within the definition of accredited investor, expert investor or institutional investor as defined under section 4A of the Securities and Futures Act It is not intended to be distributed or passed on, directly or indirectly, to any other class of persons. Recipients of this report can contact Canaccord Genuity Singapore Pte. Ltd. (Contact Person: Tom Gunnersen's tel # is +852 3919 2561) in respect of any matters arising from, or in connection with, the [analyses or report].

For Hong Kong Residents:

This research is distributed in Hong Kong by Canaccord Genuity (Hong Kong) Limited who is licensed by the Securities and Futures Commission. This research is only intended for persons who fall within the definition of professional investor as defined in the Securities and Futures Ordinance. It is not intended to be distributed or passed on, directly or indirectly, to any other class of persons. Recipients of this report can contact Canaccord Genuity (Hong Kong). Ltd. (Contact Person: Tom Gunnersen's tel # is +852 3919 2561) in respect of any matters arising from, or in connection with, the research.

Additional information is available on request.