UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934

Date of Report (Date of Earliest Event Reported): August 14, 2014

Ultragenyx Pharmaceutical Inc.
(Exact name of registrant as specified in its charter)

Delaware	**001-36276**	**27-2546083**
(State or other jurisdiction of incorporation)	**(Commission File Number)**	**(I.R.S. Employer Identification No.)**

60 Leveroni Court, Novato, California	**94949**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (415) 483-8800

Not Applicable
Former name or former address, if changed since last report

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Item 8.01. Other Events.

On August 14, 2014, the Company updated its corporate presentation, a copy of which is attached hereto as Exhibit 99.1 and incorporated by reference herein.

(d) Exhibits

Exhibit No.	Description
99.1	Corporate Presentation, dated August 14, 2014

* * *

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: August 14, 2014

Ultragenyx Pharmaceutical Inc.

By: /s/ Shalini Sharp
Shalini Sharp
Senior Vice President, Chief Financial Officer

Exhibit Index

Exhibit No.	Description
99.1	Corporate Presentation, dated August 14, 2014

Exhibit 99.1

ultragenyx pharmaceutical

Transforming good science into great medicine for rare diseases

August 14, 2014

www.ultragenyx.com

Legal Warning

- **Cautionary note regarding forward-looking statements**: The following information contains forward-looking statements, including statements regarding our expectations regarding the timing of reporting results from our clinical studies of KRN23, rhGUS, triheptanoin (in LC-FAOD and Glut1 DS), and SA-ER; our expectations regarding the timing of commencing clinical studies with respect to KRN23 and rhGUS; the design of studies for our product candidates; the likelihood of regulatory approvals for our product candidates; the potential market opportunities for commercializing our product candidates; our expectations regarding the potential market size and the size of the patient populations for our product candidates, if approved for commercial use; and other similar statements. Any forward-looking statements made by us reflect our current views with respect to future events or to our future financial performance and involve known and unknown risks, uncertainties, and other factors that may cause our actual results, performance, or achievements to be materially different from any future results, performance, or achievements expressed or implied by these forward-looking statements. Accordingly, our actual results may materially differ from our current expectations, estimates, and projections. Given these uncertainties, you should not place undue reliance on these forward-looking statements.
- Any forward-looking statements made by us in this presentation speak only as of the date of this presentation and represent our estimates and assumptions only as of the date of this presentation. As required by law, we assume no obligation to update these statements publicly, or to update the reasons actual results could differ materially from those anticipated in these statements, even if new information becomes available in the future.
- This presentation concerns drugs that are under preclinical and/or clinical investigation and which have not yet been approved for marketing by the U.S. Food and Drug Administration (FDA). They are currently limited by Federal law to investigational use, and no representations are made as to their safety or effectiveness for the purposes for which they are being investigated.
- Ultragenyx, Ultragenyx Pharmaceutical, and our logo are our trademarks. Any other trademarks appearing in these slides are the property of their respective holders.

Ultragenyx Pharmaceutical Inc.

A rare disease company by design

- Serious metabolic genetic disorders
- Five clinical stage programs
- Proven team with rare disease expertise
- Commercialization worldwide
- Well financed with $182M[1] raised year-to-date

[1]Net proceeds

A Next-Generation Rare Disease Company

Novel approaches to finding and developing products

- **Open minds** to undiscovered discoveries
- **Drugs with clear mechanisms**
- **Risk-managed development** strategies
- **Creative solutions** to rare disease challenges
- **Capital efficient and rapid** execution

Development for rare diseases is different

Experienced Management Team

Multiple rare disease product approvals

Emil Kakkis, MD, PhD, CEO	BioMarin (CMO)
Tom Kassberg, CBO	Proteolix, InterMune, Plexxikon, SUGEN, BMS
Shalini Sharp, CFO	Agenus, Elan, Goldman, McKinsey
Sunil Agarwal, MD, CMO	Roche/Genentech, MedImmune, Guilford, Kosan
Steve Jungles, SVP Technical Ops	BioMarin, Harvard Gene Therapy , Somatix, Baxter
Cori Leonard, VP Regulatory Affairs	BioMarin, Cerus, Boehringer Mannheim, Genentech
Vimal Srivastava, VP Program Dev	Janssen/Elan, Amgen, BioMarin, Wyeth
John Ditton, VP Commercial Planning	BioMarin, Merck/Dey, Diamics
Michael Vellard, PhD, VP Research	BioMarin, University Paris/Pasteur
Javier San Martin, MD, VP Clin Dev	Alder, Amgen, Lilly
Tony Koutsoukos, PhD, VP Biometrics	Allos/Spectrum, Amgen, Quintiles, FDA, NCI/NIH
Michael Cohrs, VP Quality	Medicines 360, Sunesis, Transcept, InterMune, Chiron
Ali Skrinar, PhD, Sr. Dir. Clin Sciences	Enobia, Genzyme, PwC, Andersen

Ultragenyx Pipeline

Developing multiple clinical-stage programs in parallel

Candidate	Description	Indication	Pre-clinical	Phase 1	Phase 1/2 or Phase 2	Phase 3 or Pivotal	Ultragenyx Commercial Rights
KRN23 (UX023)	Anti-FGF23 monoclonal antibody	XLH					▪ U.S. and Canada: Joint with KHK* (profit share) ▪ Mexico, Central & South America
rhGUS (UX003)	Enzyme replacement	MPS 7					▪ Worldwide
rhPPCA (UX004)	Enzyme replacement	Galactosialidosis					▪ Worldwide
Triheptanoin (UX007)	Substrate replacement	LC-FAOD					▪ Worldwide
Triheptanoin (UX007)	Substrate replacement	Glut1 DS					▪ Worldwide
SA-ER (UX001)	Substrate replacement	HIBM					▪ Worldwide (excluding Japan and certain other Asian territories)

*Kyowa Hakko Kirin

Biologic | Small Molecule

KRN23 for X-Linked Hypophosphatemia* (XLH)

Phase 2 fully human monoclonal antibody against FGF23 (SC injection)

*Also known as X-linked hypophosphatemic rickets or vitamin D-resistant rickets

KRN23 MAb Against FGF23 for XLH

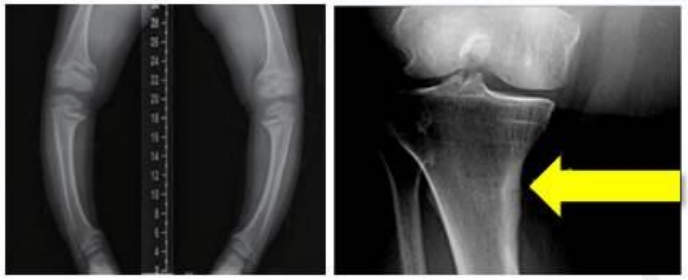
Increases low serum phosphate, which causes bone disease

- **XLH Disease:** Excess FGF23* causes excess phosphate loss into urine leading to low serum phosphate and poor bone mineralization
- **Key symptoms:** Rickets/deformity, short stature, fractures/pain
- **Diagnosis:** Clinical presentation, x-ray, urine/blood tests
- **US prevalence:** Estimated 3,000 pediatric and 9,000 adult
- **Standard of care:** Oral phosphate + Vitamin D (nephrocalcinosis risk)

KRN23: Binds elevated FGF23, reducing excess urinary phosphate loss and increasing serum phosphate and Vitamin D levels

Clinical presentation of XLH Children (left) and Adults (right)

Bowing of legs



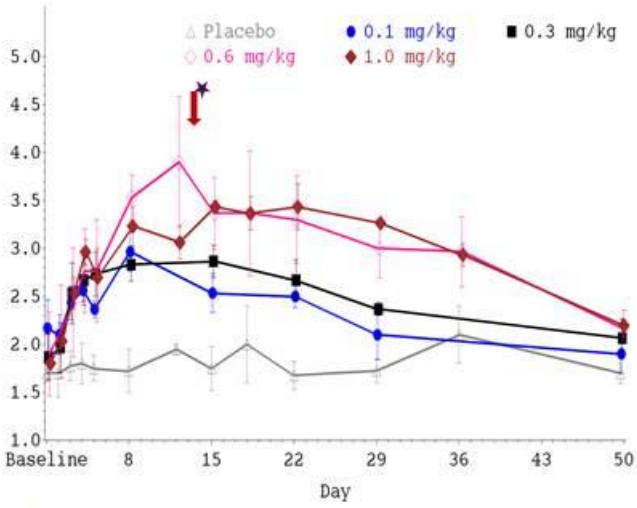
Fractures

*Fibroblast growth factor 23

KRN23 Increases Phosphate and Vitamin D

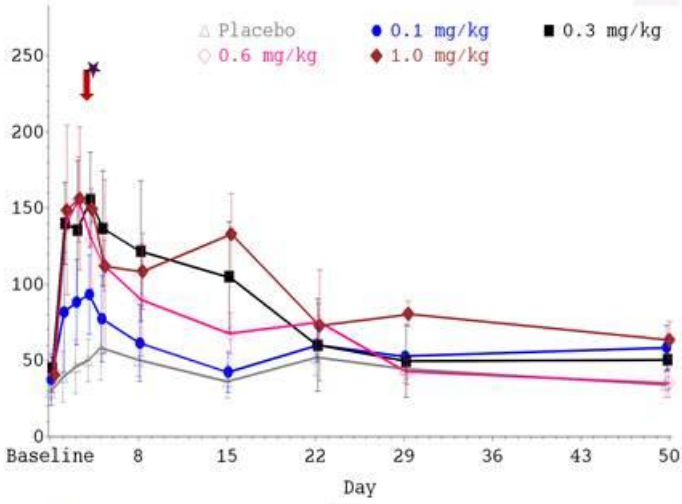
Phase 1 single-dose data presented at ASBMR* and in JCI**

Serum Pi (mg/dL)
SC administration



- *At 0.3 – 1 mg/kg, increase in serum Pi > placebo; $p < 0.05$
- Peak at day 8-15, persists ~6-8 weeks

1,25 (OH)2D (pg/mL)
SC administration



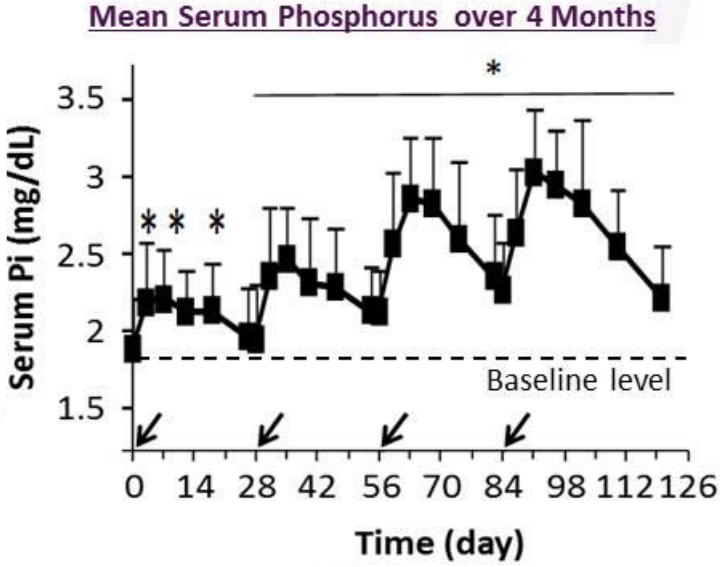
- *At 0.3, 0.6 and 1 mg/kg, increase in 1,25(OH)2D > placebo; $p < 0.001$
- Peak: day 4, persists through day 15 (29 in 1 mg/kg group)

*American Society for Bone and Mineral Research, October 2013
Carpenter, et al, 2013; Randomized, double-blind, placebo-controlled, single dose in XLH adults (n=38); placebo or KRN23 via SC or IV administration at different dose levels
**Journal of Clinical Investigation 2014; 10.1172/JCI72829

Dose-Escalation Phase 1/2 Data at ENDO*

Sustained increase in serum phosphorus with repeat KRN23

- **Study design and conduct**
  - Open-label; 4 escalating doses (0.05 mg/kg to 0.6 mg/kg)
  - Monthly subcutaneous injection
  - 28 adult XLH patients enrolled
- **Biochemical results**
  - 60% increase in serum phosphorus from baseline after 4th dose
  - Mean peak serum phosphorus of 3.03 ± 0.42 mg/dL after 4th dose
  - 89% subjects reach low normal phosphorus range; 100% had an increase
  - Comparable increases observed in reabsorption of phosphate from the urine (TmP/GFR) and serum 1,25 Vit. D



*ICE/ENDO; Imel, et al, 2014; The First Multi-Dose Trial of a Human Anti-FGF23 (Fibroblast Growth Factor 23) Antibody (KRN23) in Adults with X-Linked Hypophosphatemia (XLH)

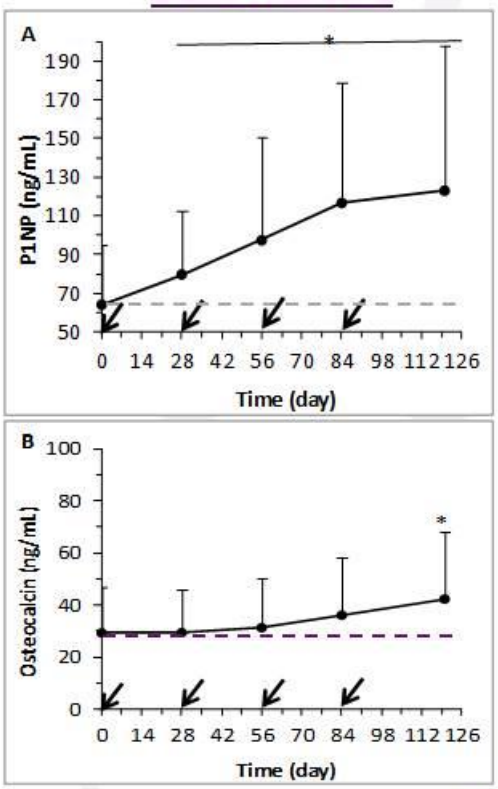
Dose-Escalation Phase 1/2 Data at ENDO

Statistically significant increases in bone markers and QOL

- Increases in bone remodeling markers (P1NP, osteocalcin) support impact of improved phosphate metabolism on bone remodeling
- Increases in certain QOL measures* in SF-36 & WOMAC physical functioning
- To be evaluated in future controlled studies
- Safety
  - No significant changes in parathyroid hormone or calcium or SAEs related to calcification
  - Common AEs: nasopharyngitis, joint pain, diarrhea, back pain, restless legs syndrome
  - One discontinuation due to injection site reaction
  - No anti-KRN23 antibodies observed
- Data from 12 additional doses to be presented at ASBMR in September 2014

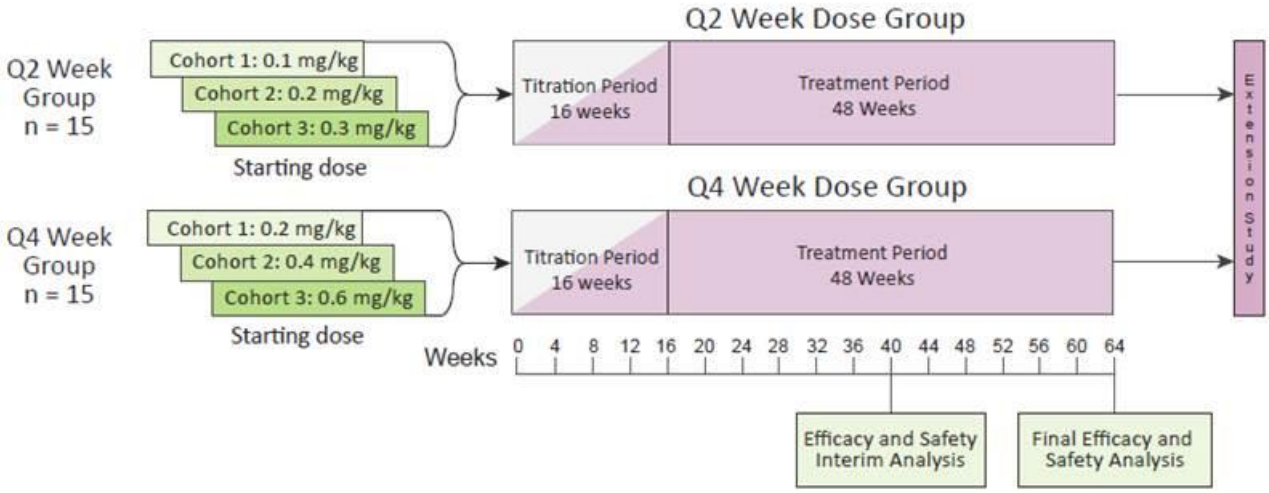
*SF-36v2 role limitations due to physical health, bodily pain, and physical component summary; WOMAC physical functioning and stiffness

Bone Markers



KRN23 Development Status and Plan

Focus on pediatric population with maximum need



- Complete Phase 2 pediatric study initiated in July 2014
  - Establish dose range/regimen, preliminary efficacy, and safety
  - Evaluate changes in baseline bone disease/clinical measures
  - Expect 30 prepubertal patients, age 5-12
- Design and conduct Phase 3 pediatric study
- Conduct parallel adult Phase 2b study

Recombinant Human β-glucuronidase (rhGUS) for Mucopolysaccharidosis 7 (MPS 7): Sly Syndrome

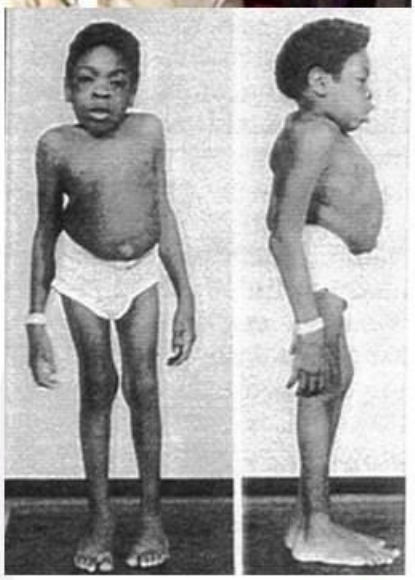
Phase 1/2 enzyme replacement therapy (IV infusion)

rhGUS ERT in MPS 7: Sly Syndrome

Disease and ERT treatment similar to MPS I, II, IVA & VI

- **MPS 7 Disease:** Deficiency of lysosomal enzyme β-glucuronidase leads to storage of glycosaminoglycans (GAG)
- **Key symptoms/prognosis**
  - Large liver/spleen, airway/pulmonary disease, joint stiffness, etc.
  - Death: teens-30s; hydrops* < 1 year
- **Diagnosis:** Via leukocyte enzyme assays
- **Market:** ~200 patients in developed world
- **Treatment:** No approved drug therapy

rhGUS: Replaces deficient enzyme to clear GAG accumulation



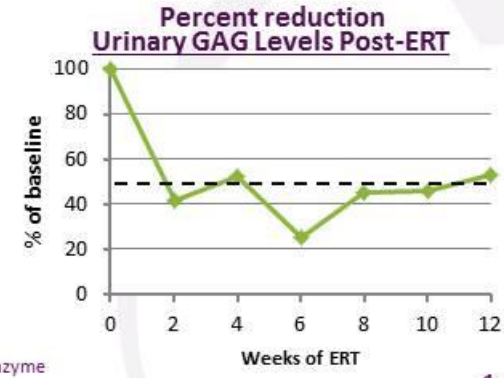
*The most severe form of the disease can uniquely present at birth with non-immune hydrops fetalis, a very severe neonatal condition in which the child retains an enormous amount of fluid throughout the body

Single-Patient eIND Data at LDN World*

12 year old patient with recent pulmonary decline

- **Patient treated under emergency IND (eIND)**
  - Sponsored by Steven and Alexandra Cohen Children's Medical Center
- **Symptoms**
  - Large liver/spleen, hearing loss, heart valve abnormalities, fatigue
  - Recent worsening pulmonary status and ventilator dependence
- **After 14 weeks of treatment**
  - Reduction in liver and spleen size
  - Reduction in urine GAG of 50%
  - Improvement in pulmonary function
  - Improved stamina and increased time in school
  - No infusion-associated reactions

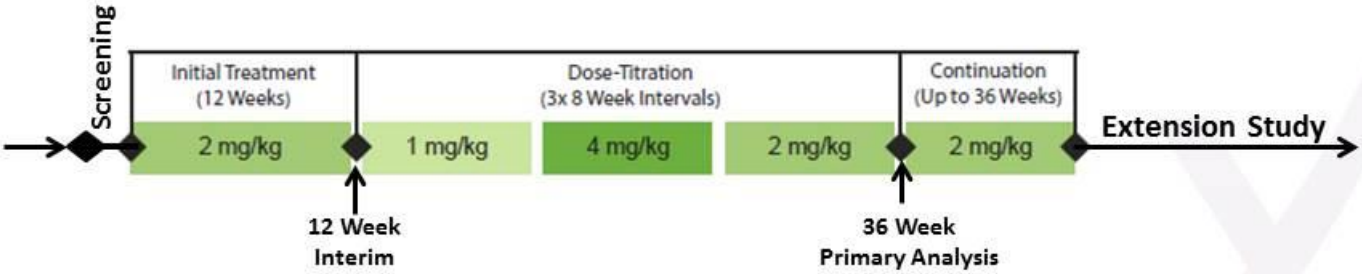
TIME	LIVER	SPLEEN
PRE	~2 cm below umbilicus	Tip in groin
2 wks	~1 cm above umbilicus	At umbilicus
8 wks	~1 cm above umbilicus	At umbilicus
12 wks	Above umbilicus	Above umbilicus



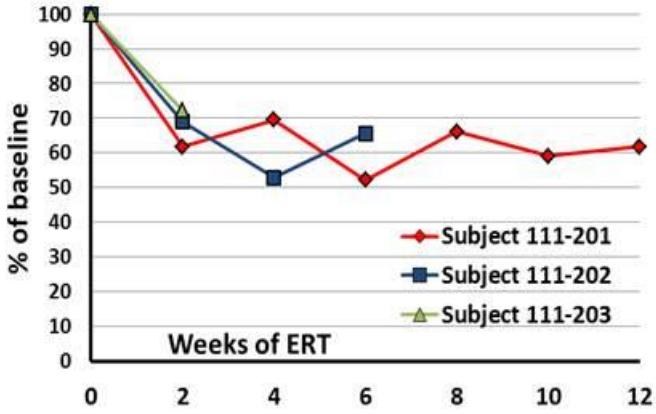
*10th Annual Lysosomal Disease Network World Symposium, February 2014
Fox JE, Volpe L, Bullaro J, Kakkis ED, Sly WS, 2014.; Recombinant Human Beta-Glucuronidase Enzyme Replacement Therapy for Mucopolysaccharidosis Type VII: Report of the First Patient Treated

rhGUS Phase 1/2 Study Interim Results

Trial initiated December 2013; 6-12 week data in first 2 subjects



Urinary Chondroitin/Dermatan Sulfate (NREs) Rapidly Decline on Therapy



Reduction in Hepatomegaly in 1st Subject

- Liver excess size in 111-201 patient reduced 46.5% by ultrasound
- 4.46% of body weight to 3.81%
- Normal is 3.13%

Safety Observations To Date

- No SAEs up to 12 wks of treatment
- No IARs in total 13 infusions

rhGUS Clinical Program Status

- Continue Phase 1/2 study
  - At 12 weeks assess PK/efficacy/safety
  - Dose titration for dose-response after 12 weeks
  - Decide timing for progression into Phase 3
- Phase 3 blind-start design with uGAG primary endpoint
  - Agreed N=12 and uGAG primary endpoint with EMA
- Start of Phase 3 study expected in US in 2014
  - Once dose/regimen/safety confirmed in Phase 1/2
  - Phase 3 study planning underway, protocol completed
- Plan to consult with FDA regarding US pathway

Triheptanoin for Long-Chain Fatty Acid Oxidation Disorders* (LC-FAOD)

Phase 2 substrate replacement therapy (oral liquid)

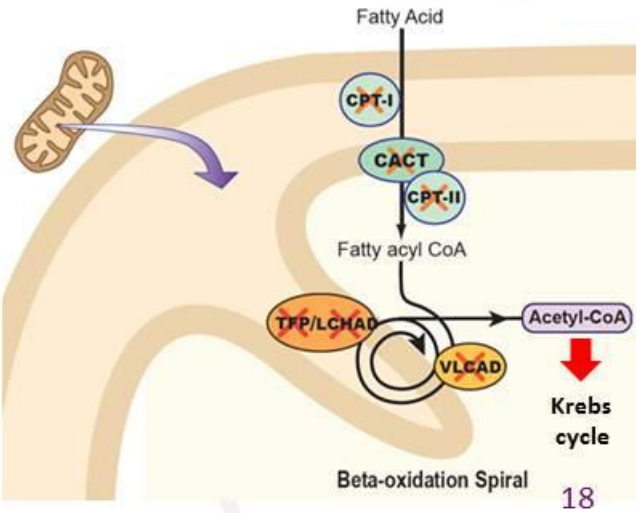
*Includes VLCAD, LCHAD, CPT-I, CPT-II, TFP, CACT

Triheptanoin for LC-FAOD

Triglyceride of C7: alternative energy source to long-chain fat

- **LC-FAOD disease mechanism**
  - Cannot convert fat into energy due to mitochondrial enzyme deficiency
  - Critical energy shortage during exercise, fasting, and illness
- **Key symptoms/prognosis**
  - Hypoglycemia, acute muscle rupture, heart failure
  - Reported mortality: ~50%*; a cause of SIDS
- **US prevalence**
  - Newborn screened
  - ~2,000 – 3,500
- **Standard of care**
  - Avoid fasting, high-carb/low-fat diet
  - Supplement with MCT oil**

Triheptanoin: Designed to bypass energy block and repair Krebs cycle



*J Inherit Metab Dis 2013;36:795-803 **Medium chain triglycerides

Multi-Symptom Improvement

Historical studies of triheptanoin in LC-FAOD for up to 13 years

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

*n=48; CPT-I, CPT-II, CACT, VLCAD, LCHAD, TFP, SCAD
**Patients on MCT oil and low-fat, high carb diet

Roe and Mochel, 2006

Reduction in Major Medical Events

Retrospective study data of 20 compassionate use patients

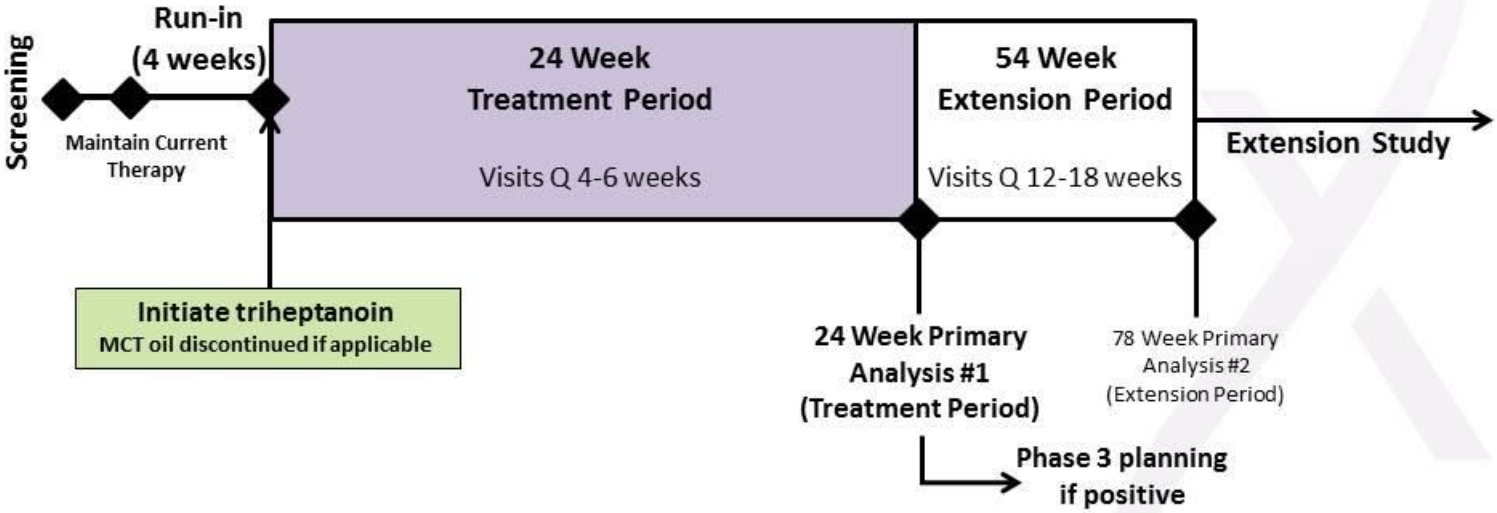
Description	Pre-treatment	Post-treatment	% Decrease	n	p value
Mean total hospital days/year[1,2]	17.55	5.40	69%	15	**0.0242**
Mean total hospitalizations/year[1]	1.94	1.26	36%	16	0.1126
Mean infant total hospitalizations/year[3]	13.01	1.37	89%	4	0.0892
Mean hypoglycemia total hospital days/year[1,2,4]	8.42	0.18	98%	9	**0.0257**
Mean hypoglycemia events/year[1,4]	0.92	0.04	96%	9	**0.0091**
Mean rhabdomyolysis total hospital days/year[1,5]	5.94	2.16	64%	9	0.1224
Mean rhabdomyolysis events/year[1,5]	1.05	0.68	35%	11	0.4604
Mean peak creatine kinase (units) for rhabdomyolysis events[1,5]	85,855	25,797	68%	7	0.1279

[1] Excludes data for 4 infants dosed within first 6 months of life.
[2] Excludes hospitalizations with unknown discharge dates.
[3] Four infants were dosed within the first 6 months of life.
[4] Includes only those patients with hypoglycemia events prior to treatment.
[5] Includes only those patients with rhabdomyolysis events prior to treatment.

- Data presented at ICIEM September 2013
- 20 patients treated for up to 13 years
- 319 hospitalizations, 120 charts, 241 years

LC-FAOD Phase 2 Study Ongoing

Goal to determine endpoints and patient selection for Phase 3



- N = ~30 severe subjects, first patient enrolled in February 2014
- Dosed at 25-35% of daily caloric intake
- Evaluate exercise tolerance (cycle erg, 12MWT, muscle strength, event rate, CK, etc.), hypoglycemia, liver size, cardiac disease
- Interim data expected in 2015

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Triheptanoin for Glucose Transporter Type 1 Deficiency Syndrome (Glut1 DS)

Phase 2 substrate replacement therapy (oral liquid)

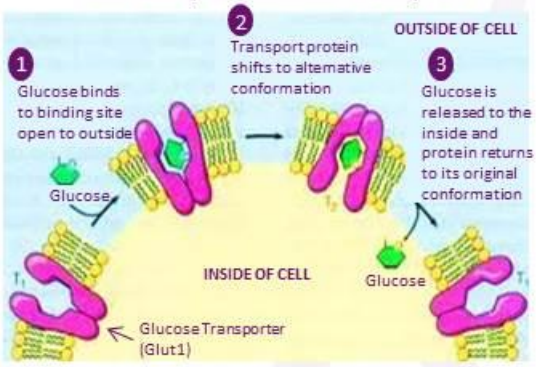
Triheptanoin for Glut1 DS

Alternative energy source for the brain

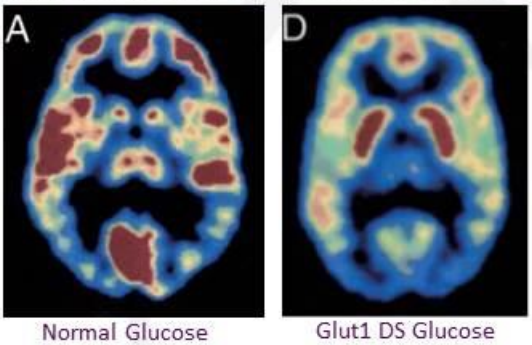
- **Glut1 DS mechanism:** Glucose transport defect causes brain energy deficiency
- **Key symptoms/prognosis**
  - Seizures
  - Developmental delay
  - Movement disorder
- **Diagnosis:** CSF, red cell, genetic tests
- **U.S. prevalence:** ~3,000 – 7,000
- **Standard of care:** Ketogenic diet (70-80% of calories in fat, <10% carbs) and anti-epileptic drugs

Triheptanoin: Triglyceride of C7 intended to bypass defective transport to provide alternate energy source

Deficiency of Glucose Transport



Less glucose uptake (red color) in Glut1 DS



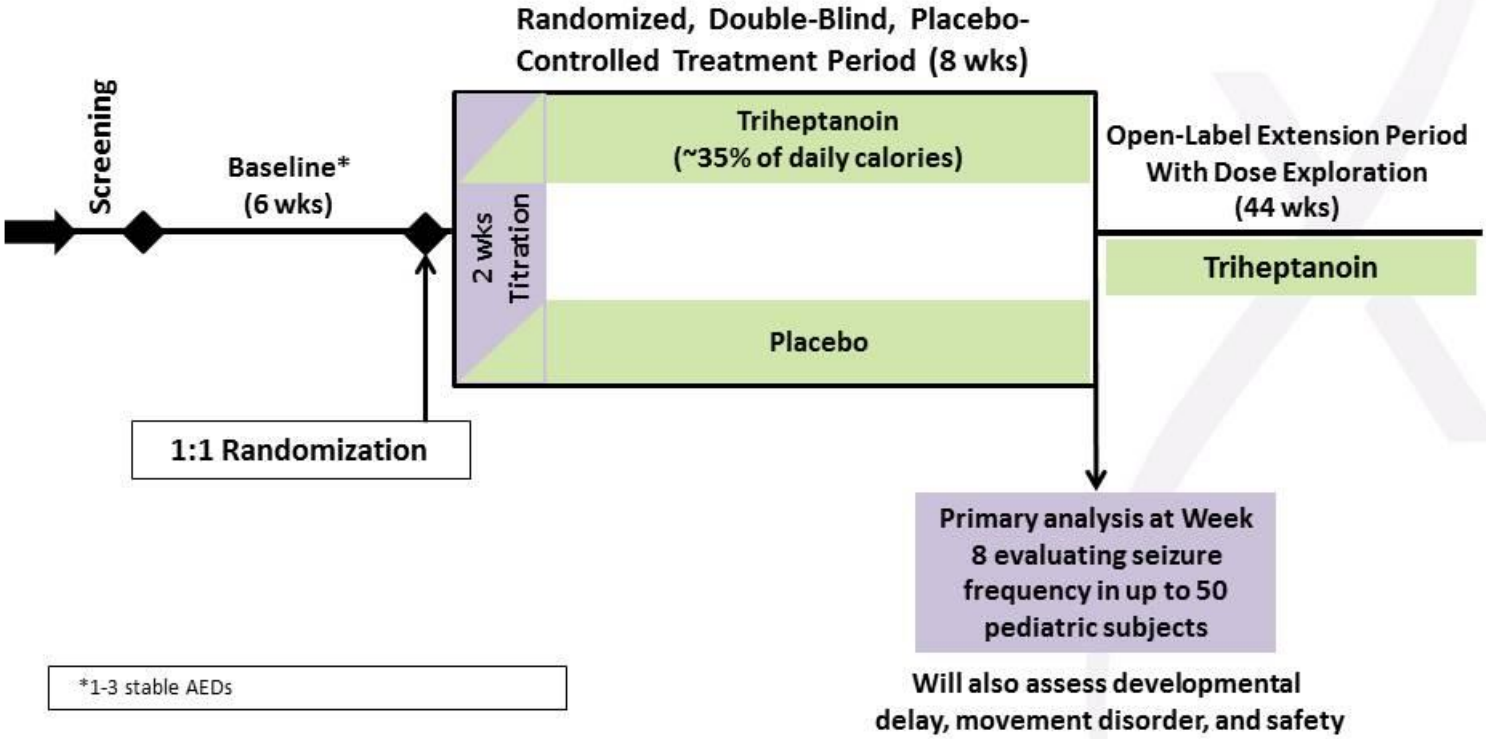
Normal Glucose Uptake

Glut1 DS Glucose Uptake

Ann Neurol 2002;52:458–464

Glut1 DS Phase 2 Study Ongoing

Study goal to evaluate potential reduction in seizure frequency



*1-3 stable AEDs

ultragenyx pharmaceutical

Sialic Acid Extended Release (SA-ER) for Hereditary Inclusion Body Myopathy (HIBM)*

Phase 2 substrate replacement therapy (oral tablet)

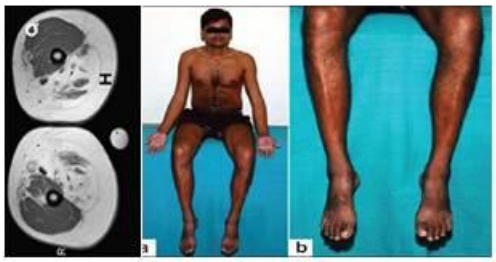
*Also known as distal myopathy with rimmed vacuoles (DMRV), Nonaka disease, GNE myopathy

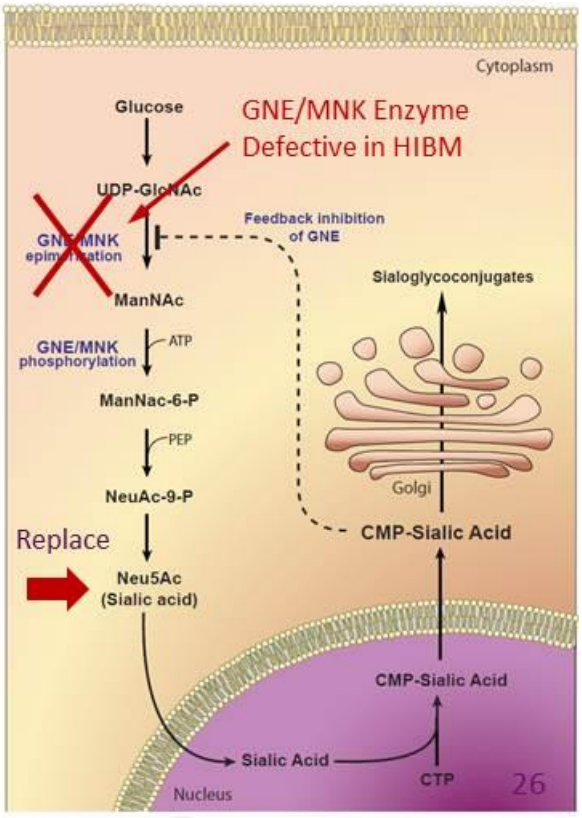
SA-ER Substrate Replacement for HIBM

Sialic acid deficiency leads to progressive muscle atrophy

- **HIBM mechanism:** Sialic acid deficiency caused by defect in the biosynthetic pathway
- **Key symptoms/prognosis:**
  - Onset typically at 18-30 years old
  - Loss of major muscle function ~10–20 years from diagnosis
- **Diagnosis**: Muscle biopsy/sequencing
- **Prevalence:** ~1,200–2,000 patients in developed world
- **Standard of care:** No approved therapy

SA-ER: Designed to replace deficient sialic acid

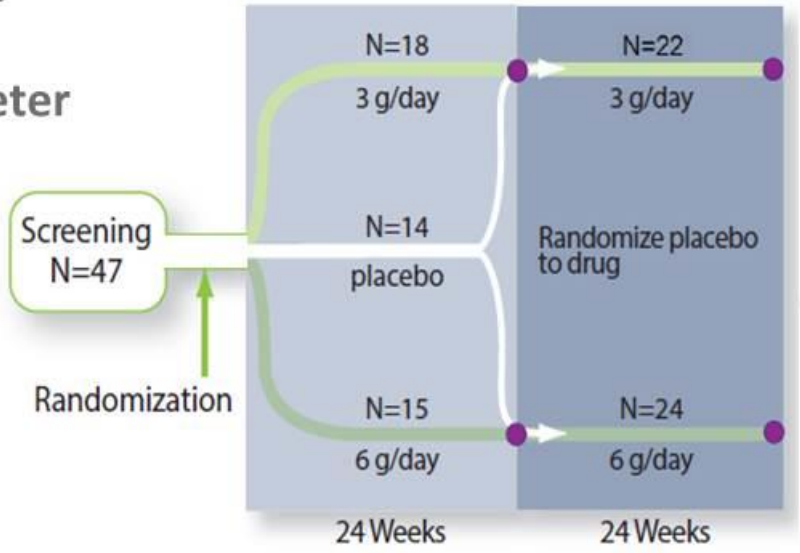




SA-ER: Positive Phase 2 Results at AAN*

SA-ER stabilizes upper extremity muscle strength

- **Randomized controlled study**
  - Placebo, 3g and 6g
  - Cross-over of placebo to 3 or 6g at 24 weeks
- **Biochemistry/PK**
- **Muscle strength/dynamometer**
  - Upper extremity composite
  - Lower extremity composite
- **Other clinical measures**
- **Patient-reported outcomes**
  - GNEM-FAS functional scale
- **Safety and tolerability**
  - AEs and laboratory measures

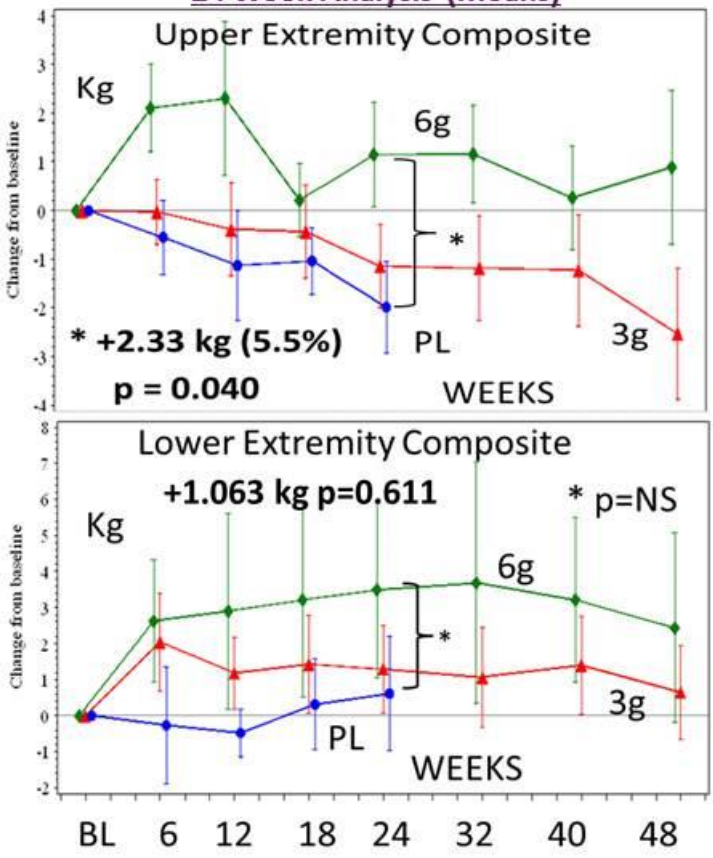


*American Academy of Neurology Annual Meeting; April 30, 2014

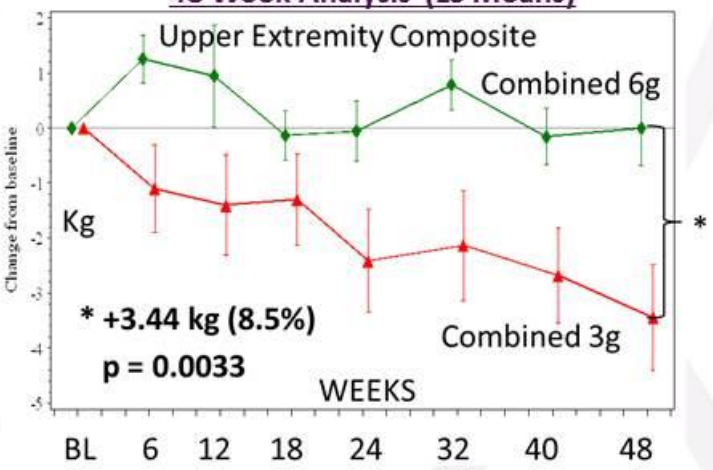
SA-ER: Phase 2 Results Presented at AAN

Upper extremity muscle strength preserved over 48 weeks on 6g

24 Week Analysis (Means)



48 Week Analysis (LS Means)



Upper Extremity Composite (UEC) Change

	6g vs Placebo (24 weeks)	Combined 6g vs Combined 3g (48 weeks)	Combined 6g vs Combined 3g (48 weeks)
Predefined subset	n/a	n/a	≥200M 6MWT at baseline
UEC Change	+2.33 kg (+5.5%)	+3.44 kg (+8.5%)	+4.69 kg (+9.6%)
P-value	0.040	0.0033	0.00055

Patient-Reported Data Consistent with UEC

Patient-Reported Outcomes Consistent with Preservation of Muscle Strength at 6g

FAS endpoints	Combined 6g vs. 3g groups[1]	P value (ANCOVA[2])	P value (GEE[3])
Total	**4.032**	**0.0859**	**0.020**
Mobility	1.772	0.0865	0.030
Upper Extremity	1.856	0.0955	0.050
Self Care	0.075	0.3971	0.30

[1]After 48-weeks
[2]ANCOVA = Analysis of covariance
[3]GEE = General Estimator Equation Statistical Model

Safety Results Indicate SA-ER Is Well-Tolerated

- No Serious Adverse Events occurred
- No dose-dependent treatment emergent Adverse Events (AEs) identified in the study
- Most common AEs:
  - Gastrointestinal-related
  - Procedural pain related to muscle biopsy

48 Week Conclusions

- Upper extremity composite (UEC) stabilized in 6g versus 3g dose or placebo
- FAS score supports muscle strength result
- Lower extremities showed similar pattern but not statistically significant; neither group showed decline
- SA-ER is well tolerated
- Higher dose under study, results expected in late 2014

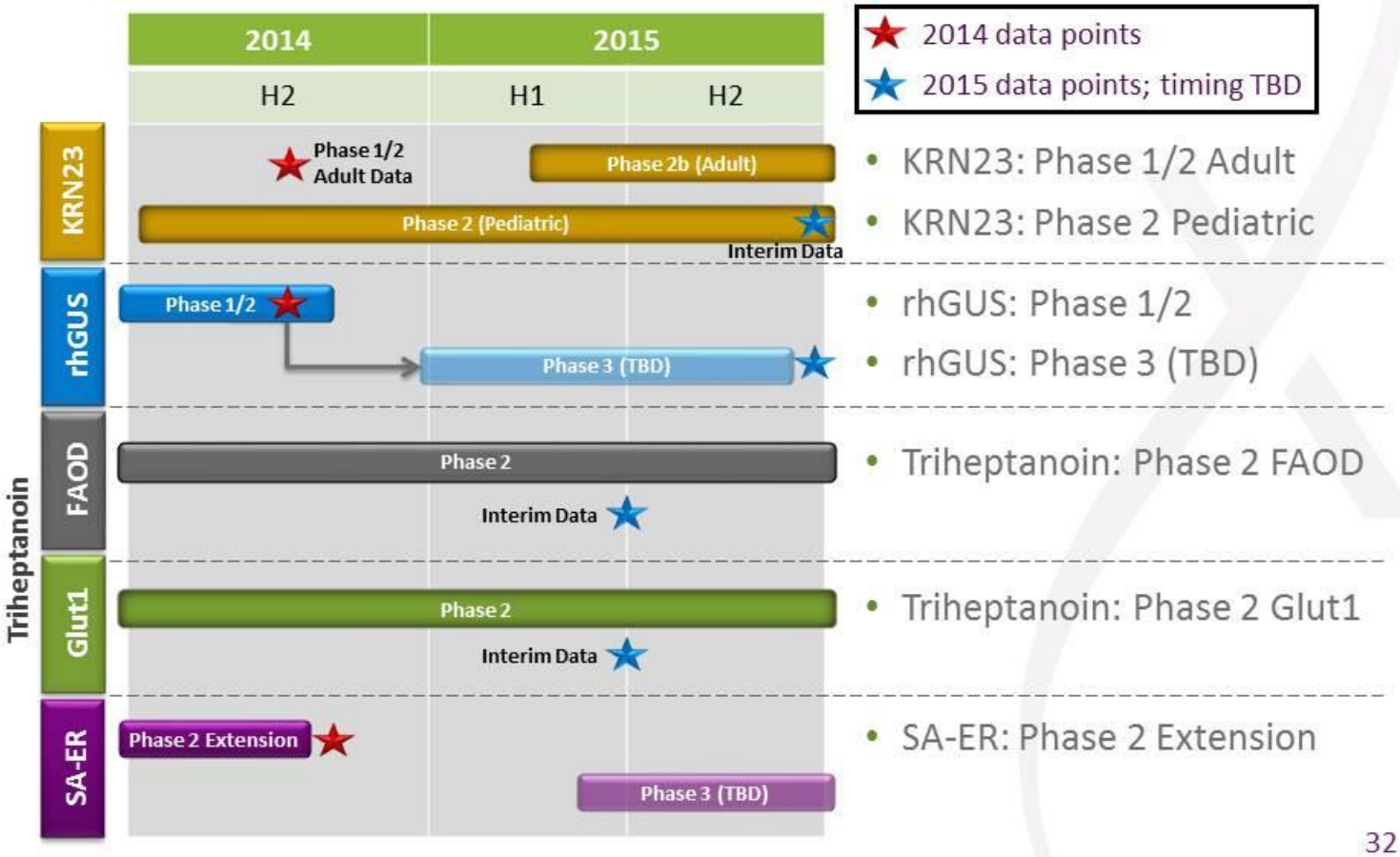
Business Summary

Financial Overview

- Cash, cash equivalents, and short-term investments as of Q2 2014: $153.3 million
- Total operating loss for 1H 2014: $24.0 million
- Completed $60.2 million follow-on in July 2014[1]
- Expect existing cash to fund operations through 2016 with current operating plan
- No debt

[1]Net proceeds

Five Program Data Readouts in 2014-2015



Investment Thesis for Ultragenyx

A rare disease company by design

- Proven rare disease track record for development
- Efficiently transforming unrecognized science into effective rare disease products
- Deep and diversified product pipeline
- Rich set of value drivers in five programs
- A next-generation rare disease company

Appendix

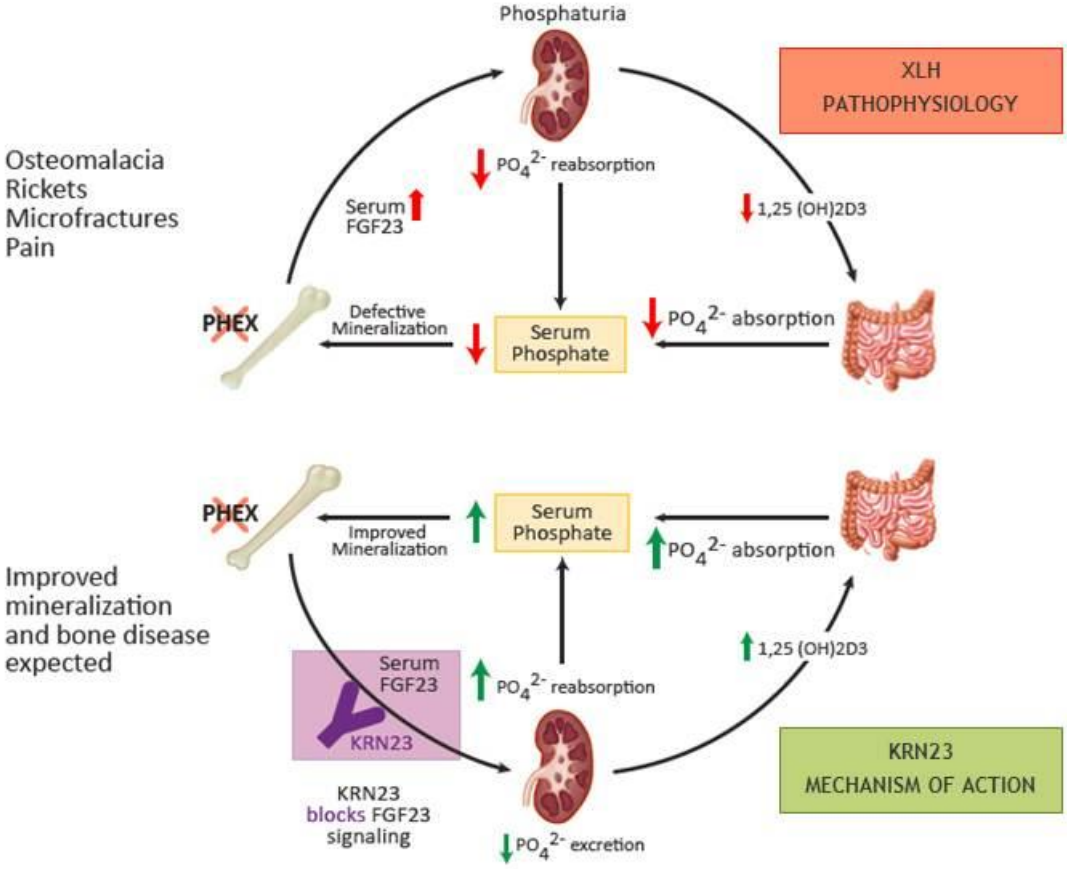
Rapidly Building a Pipeline and the Company

Company founded in 2010; IPO in February 2014

	2010-2012	2013	2014
KRN23		• In-licensed from KHK • Phase 1 adult data	• IND amend./CTA filings • Phase 1/2 adult data • Initiated Ph. 2 pediatric
rhGUS	• In-licensed from SLU • Initiated dev. 2012	• CTA filing • Initiated Phase 1/2	• Phase 1/2 data • Initiate Phase 3*
Triheptanoin	• In-licensed from BRI	• FAOD IND filing • FAOD retro data • Glut1 IND filing	• Initiated Phase 2 FAOD • Initiated Phase 2 Glut1
SA-ER	• In-licensed 2010 • IND Filed June 2011 • Phase 1 initiated 2011	• Phase 2 24/48-wk data • Initiated DMP	• Phase 2 extension data

*If Phase 1/2 is positive

KRN23 Mechanism of Action



KRN23 Appears Safe and Well-Tolerated

Phase 1 single-dose data presented at ASBMR and in JCI

- No SAEs, deaths, or AEs leading to withdrawal
- AEs: KRN23 (82%) vs placebo (44%); most were mild
- Treatment emergent AEs: KRN23 (24/29) vs placebo (4/9). Most common were:
  - SC: elevated serum amylase and back pain (17%)
- No maximum tolerated single-dose reached
- No anti-KRN23 antibodies detected
- No hypersensitivity/infusion reactions

Carpenter, et al, IBID 2013 and 2014

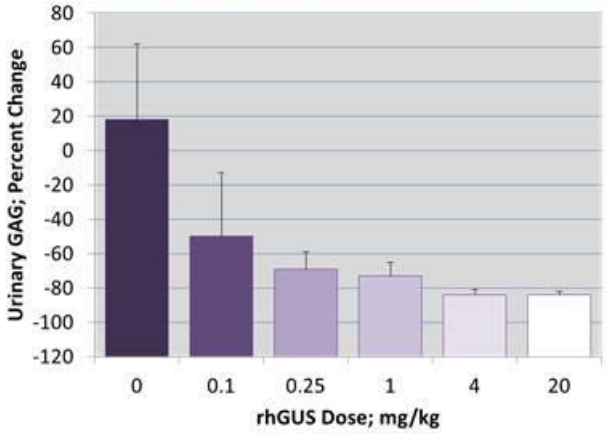
rhGUS Treatment in MPS 7 Mouse

Enzyme distribution, urine GAG, and tissue pathology

- **Goal:** To verify the enzyme uptake and activity of Ultragenyx's rhGUS
- **Design:** 5 mice/group treated with 0 – 20 mg/kg UX003 IV weekly for 8 weeks
- **Results:**
  - Dose-dependent enzyme uptake in wide array of tissues (not shown)
  - Dose-dependent urine GAG reduction reaching 80%
  - Clearance of storage in tissues

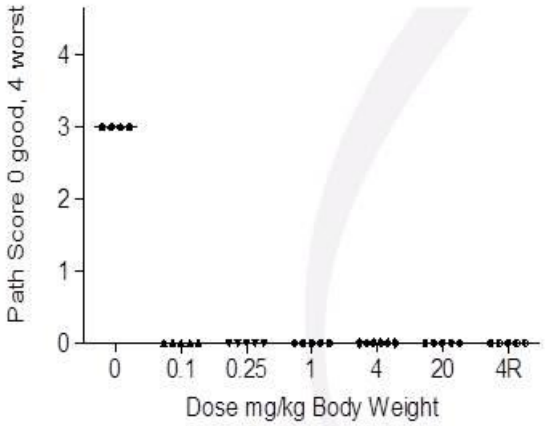
uGAG reduction

(after 8 weeks rhGUS treatment)



Dose vs. Lysosomal Pathology

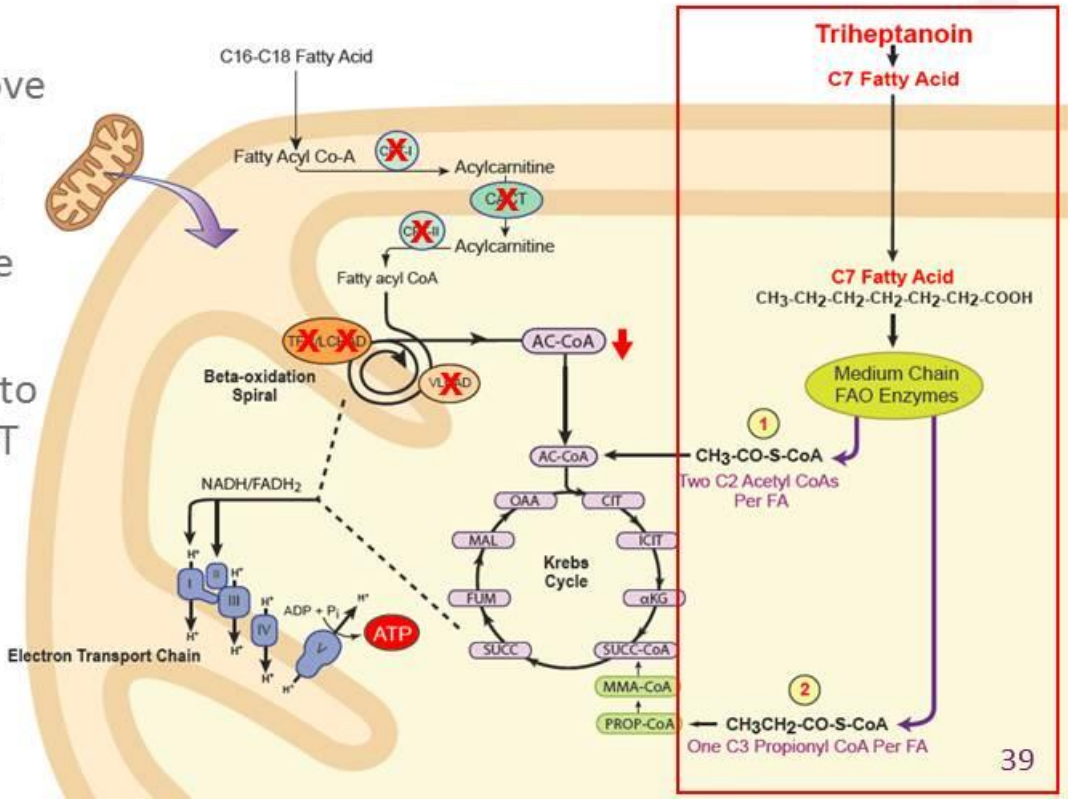
Liver



Triheptanoin for LC-FAOD

Synthetic triglyceride of C7 fatty acids

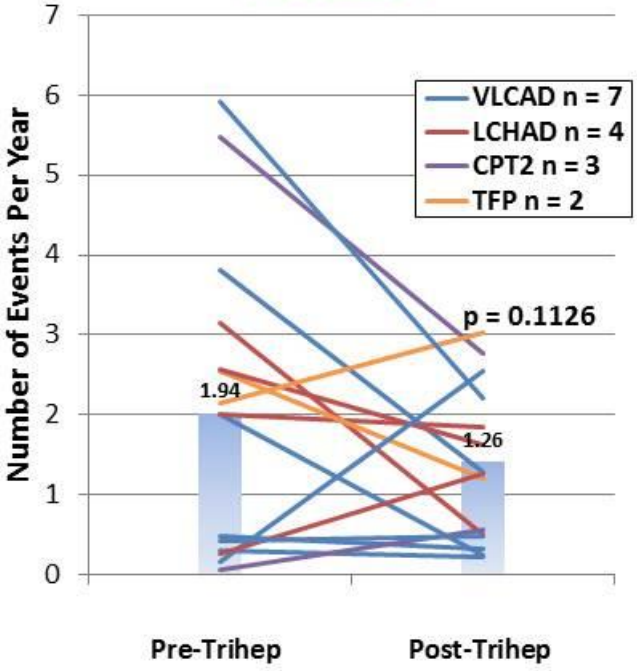
- Designed to improve Krebs cycle fat-to-energy conversion
- Appears to provide **TWO** substrates
- Can be converted to glucose unlike MCT



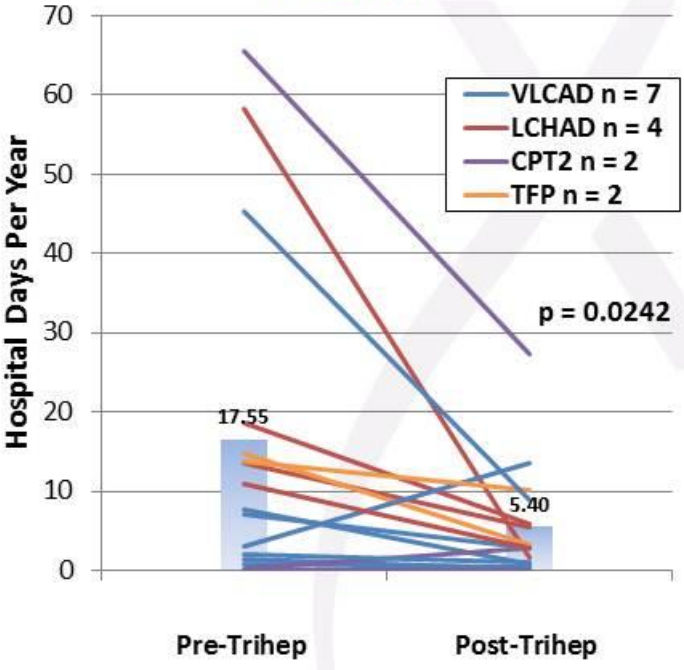
Reduction in Hospital Utilization

Protocol-driven retrospective study of compassionate use patients

Mean Hospitalizations/Year: 36% reduction[1]



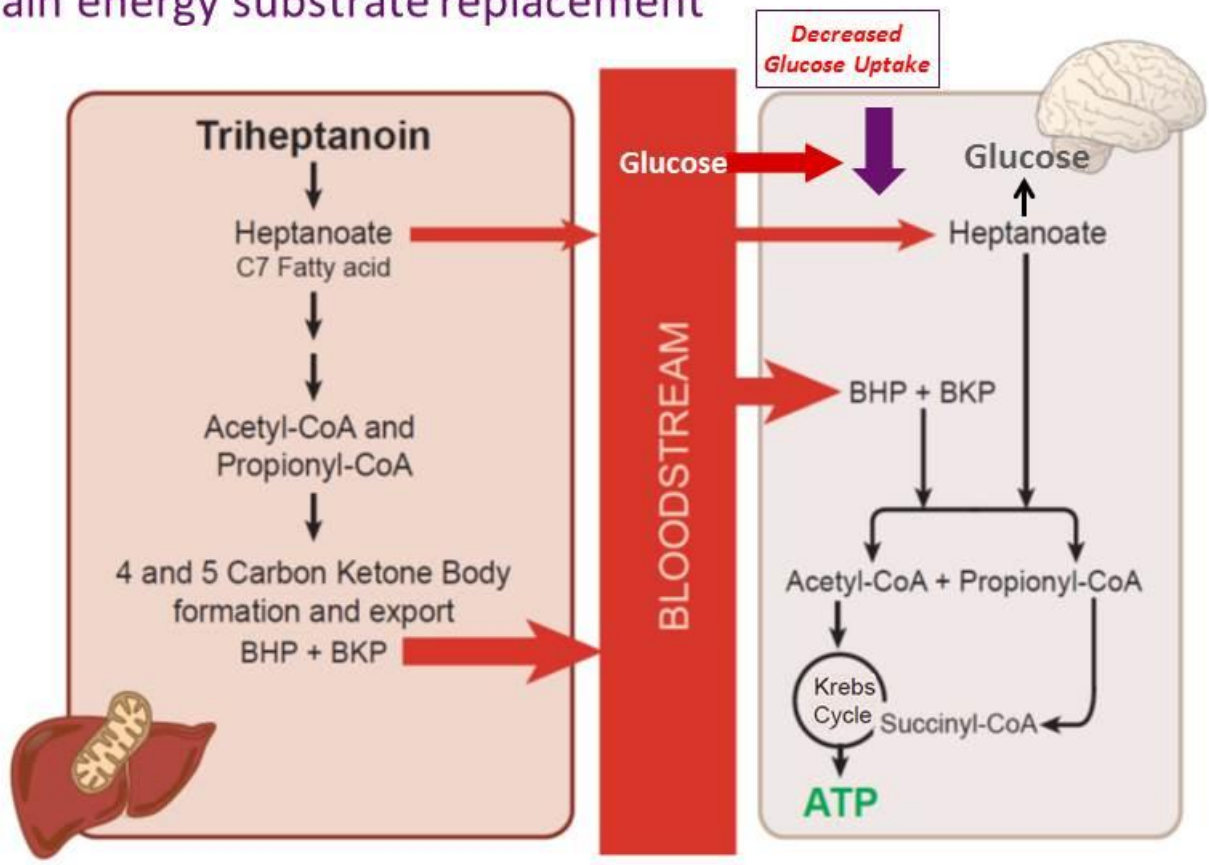
Mean Hospital Days/Year: 69% reduction[1][2]



[1]Excludes 4 infants dosed within 6 months of life
[2]Excludes hospitalizations w/unknown discharge dates

Triheptanoin Mechanism in Glut1 DS

Brain energy substrate replacement



License and Collaboration with KHK

Ultragenyx leads development with costs shared 50/50

Key Terms	US and Canada	Europe	Latin America
Commercialization	• Ultragenyx launches • KHK books sales • 50/50 profit share for 5 years then tiered revenue share • Shared commercial activities over time	KHK commercializes and books sales	Ultragenyx commercializes and books sales
Royalties	After 5 years, tiered revenue share in mid to high 20% range to Ultragenyx after profit share period	Up to 10% royalty to Ultragenyx	Low single-digit royalty to KHK
Commercial supply	KHK supplies; price is double-digit percentage of net sales	NA	KHK supplies; price is double-digit percentage of net sales

Key Licenses & Intellectual Property

Product	License	Intellectual Property/Royalties
KRN23 (XLH)	KHK	• Shared rights to US patents to generic and specific antibodies and use for the treatment of XLH (2022-2029)[1] • See summary of collaboration
rhGUS (MPS 7)	St. Louis University	• IP in progress • Low single-digit royalty
rhPPCA (Galactosialidosis)	St. Jude Children's Hospital	• IP in progress • <1% royalty
Triheptanoin (LC-FAOD)	Baylor Research Institute	• Composition and use in FAOD (2025)[1] • Mid-single digit royalty
Triheptanoin (Glut1 DS)	Baylor Research Institute	• Composition (2025)[1,2] • Mid-single digit royalty
SA-ER (HIBM)	Nobelpharma & HIBM Research Group	• Applications on PK, formulation, use of sialic acid or substrate replacement in HIBM (2028-2034)[1] • High single digit royalty (Nobelpharma) • <1% royalty (HIBM Research Group)

[1]Without patent term extension of up to five years
[2]Additional patent application related to triheptanoin use in seizures (2031) licensed from UniQuest and announced 8/5/14

Selected Quarterly Financial Information
(Unaudited)

The following tables present certain unaudited quarterly financial information. This information has been prepared on the same basis as the audited financial statements and includes all adjustments (consisting only of normal recurring adjustments) necessary to present fairly the unaudited quarterly results of operations set forth herein. The operating results for any quarter are not necessarily indicative of results for any future period. Net loss per share for all periods presented has been retroactively adjusted to reflect the 1-for-3.1345 reverse stock-split effected on January 17, 2014. All data is in thousands except per share data.

	2014	
	Q1	Q2
Operating expenses	$ 10,339	$ 13,661
Net loss	$ (13,630)	$ (13,585)
Net loss attributable to common stockholders	$ (18,438)	$ (13,585)
Net loss per share applicable to common stockholders, basic and diluted	$ (0.85)	$ (0.45)

	2013			
	Q1	Q2	Q3	Q4
Operating expenses	$ 6,747	$ 8,247	$ 7,761	$ 9,525
Net loss	$ (6,735)	$ (8,590)	$ (8,428)	$ (11,317)
Net loss attributable to common stockholders	$ (8,205)	$ (10,829)	$ (12,590)	$ (18,665)
Net loss per share applicable to common stockholders, basic and diluted	$ (2.84)	$ (3.32)	$ (3.48)	$ (4.98)

	2012			
	Q1	Q2	Q3	Q4
Operating expenses	$ 3,092	$ 3,571	$ 4,644	$ 4,678
Net loss	$ (3,103)	$ (3,672)	$ (4,629)	$ (4,930)
Net loss attributable to common stockholders	$ (3,384)	$ (4,063)	$ (5,302)	$ (6,812)
Net loss per share applicable to common stockholders, basic and diluted	$ (6.96)	$ (5.72)	$ (2.81)	$ (2.83)