Corporate Deck

May 19, 2021

Legal Warning

Cautionary note regarding forward-looking statements: This presentation contains forward-looking statements, including, but not limited to, statements regarding our expectations and projections regarding our future operating results and financial performance, anticipated cost or expense reductions, plans with respect to commercializing our product and product candidates, our translational research program, expectations regarding our manufacturing capabilities, the expected timing of release of additional data for our product candidates, plans to initiate additional studies for product candidates and timing and design of these studies, plans regarding ongoing studies for existing programs, our liquidity position as of the most recent fiscal quarter end, expectations regarding the adequacy of clinical data to support marketing applications and approvals of product candidates, our intent to file, and potential timing and success of, marketing applications and other regulatory approvals, expectations regarding timing of receiving potential approval of product candidates, expectations regarding prevalence of patients, future regulatory interactions, and the value to be generated by our pipeline. Such forward-looking statements involve substantial risks and uncertainties that could cause our clinical development programs, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, our reliance on our third party partner, Kyowa Kirin Co., Ltd., for the supply of Crysvita, the effects from the COVID-19 pandemic on our clinical trial activities, business and operating results, smaller than anticipated market opportunities for our products and product candidates, manufacturing risks, competition from other therapies or products, uncertainties related to insurance coverage and reimbursement status of our newly approved products, our evolving integrated commercial organization, the uncertainties inherent in the clinical drug development process, such as the regulatory approval process, the timing of our regulatory filings the uncertainties inherent in the clinical drug development process, including the potential for substantial delays and risk that earlier study results may not be predictive of future study results, and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations, the availability or commercial potential of our product and product candidates, and our ability to integrate acquired businesses, which are more fully described in our most recent Form 10-Q or Form 10-K under the caption “Risk Factors” and elsewhere in such reports. 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. Except as required by law, we assume no obligation, and we disclaim any intent, to update these statements to reflect actual results.

This presentation concerns commercial products as well as discussion of investigational 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.

Building an Exceptional Rare Disease Company with Value Drivers Across Commercial, Clinical, and Platforms

Commercial Expansion	▪ Continued Crysvita growth ▪ Strong Dojolvi launch for LC-FAOD
Clinical Development Catalysts	▪ Initiate four pivotal studies (including three gene therapy) ▪ Data catalysts and study initiations
Gene Therapy Manufacturing	▪ HeLa PCL: High quality, commercial scale ▪ Supports larger indications with reduced COGS

2017-2020: Completing Four Approvals and Launches

Refilling the pipeline through efficient business development

Pipeline Approvals

Mepsevii™ (vestronidase alfa-vjbk) injection
MPS VII

CRYSVITA® burosumab
XLH

CRYSVITA® burosumab
TIO

DOJOLVI® TRIHEPTANOIN Oral Liquid
LC-FAOD

2017	2018	2019	2020	2021+

Pipeline Build

GENE THERAPY

DIMENSION THERAPEUTICS → **OTC AAV8** DTX301 → **GSDIa AAV8** DTX401

→ **Wilson AAV** UX701
→ **CDKL5 Def. AAV** UX055

SOLID BIOSCIENCES → **Duchenne** UX810

NUCLEIC ACID

ARCTURUS therapeutics → **GSD III mRNA/LNP** UX053

genetx → **Angelman-ASO** GTX102

MAb

Mereo BioPharma → **Osteogenesis Imperfecta** UX143

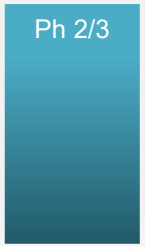
ultragenyx pharmaceutical

Three Therapeutic Areas Approaching $1 Billion of Product Revenue in the Next Five Years

CLINICAL

COMMERCIAL

BONE ENDOCRINE

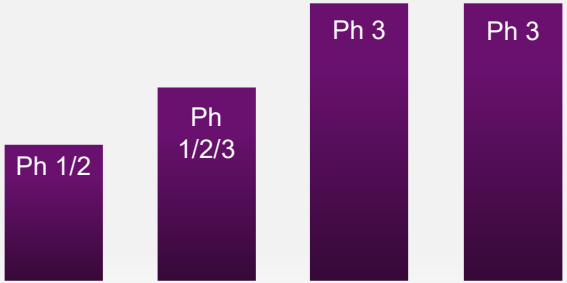


Setrusumab
for OI

CRYSVITA® burosumab
XLH

CRYSVITA® burosumab
TIO

METABOLICS



UX053 for GSDIII
UX701 for Wilson
DTX401 for GSDIa
DTX301 for OTC

Mepsevii™ (vestronidase alfa-vjbk) injection
MPS VII

DOJOLVI® TRIHEPTANOIN Oral Liquid
LC-FAOD

CNS / MUSCLE

GTX-102
for Angelman

ultragenyx pharmaceutical

Diverse Clinical Pipeline with Larger Indications to Drive Long-Term Growth

Candidate	Description	Pre-Clinical	IND	Phase 1	Phase 2	Phase 3	Approved	Est'd Patients in Dev. World
CRYSVITA® burosumab KYOWA KIRIN	Anti-FGF23 Monoclonal Antibody	XLH						~48,000
		TIO						~2,000 - 4,000
Mepsevii™ (vestronidase alfa-vjbk) injection	Enzyme Replacement	MPS 7						~200
Mereo BioPharma Setrusumab	Anti-sclerostin Monoclonal Antibody	Osteogenesis Imperfecta						~60,000
DOJOLVI® TRIHEPTANOIN Oral Liquid	Substrate Replacement	LC-FAOD						~8,000 - 14,000
DTX401	AAV8-G6Pase Gene Therapy	GSDIa						~6,000
DTX301	AAV8-OTC Gene Therapy	OTC						~10,000
BAYER DTX201	AAVhu37-FVIII Gene Therapy	Hemophilia A						~144,000
UX701	AAV9-ATP7B Gene Therapy	Wilson						~50,000
UX810	Microdystrophin Gene Therapy	Duchenne						~40,000
genetx GTX-102[1]	Antisense Oligonucleotide	Angelman						~60,000
UX053	mRNA/LNP	GSDIII						~10,000

Protein Biologic | Small Molecule | Gene Therapy | ASO / mRNA

1: Ultragenyx has an option to acquire GTX-102 from GeneTx

ultragenyx pharmaceutical

Commercial Performance and Strong Capital Position Drive Future Growth

1Q21 Revenue ($M)	
Crysvita in Ultragenyx Territories[1]	$42.1
Dojolvi	7.0
Mepsevii	3.6
Daiichi Sankyo	42.8
Non-Cash EU Royalty	3.9
Total Revenue	$99.4

2021 Crysvita Revenue Guidance	
Crysvita in Ultragenyx Territories[1]	$180M to $190M

Strong Capital Position: Strategic Investments and Financial Discipline

- Cash balance[2] as of 1Q21 approximately $1.0 billion
  - Modest YoY OpEx growth in 2021 to support advancing clinical pipeline and commercial launches, excluding the $50M upfront payment to Mereo in 1Q21

1: Crysvita in Ultragenyx Territories, which excludes royalty in the European territory
2: Cash, cash equivalents, and marketable debt securities as of March 31, 2021

ultragenyx pharmaceutical

2021 Clinical Milestones
Initiating Four Pivotal Clinical Studies

GENE THERAPY			1H21	2H21
DTX401 AAV8 Gene Therapy	**GSDIa**	Longer-Term Phase 1/2 Data	✓	
		Phase 3 Initiation		☐ *Early 2H*
DTX301 AAV8 Gene Therapy	**OTC**	Longer-Term Phase 1/2 Data	✓	
		Phase 3 Initiation		☐
UX701 AAV9 Gene Therapy	**Wilson Disease**	IND Cleared	✓	
		Phase 1/2/3 Initiation		☐ *Early 2H*

ASO/mRNA			1H21	2H21
GTX-102 ASO	**Angelman Syndrome**	Resume Phase 1/2 Study	*Late 1H21 or early 2H21*	
		Updated Phase 1/2 Data		☐
UX053 mRNA/LNP	**GSDIII:** **Debrancher Deficiency**	IND Cleared	✓	
		Phase 1/2 Initiation		☐

PROTEIN BIOLOGIC			1H21	2H21
Setrusumab (UX143) Monoclonal Antibody	**Osteogenesis Imperfecta**	Pediatric Phase 2/3 Initiation		☐

ultragenyx pharmaceutical

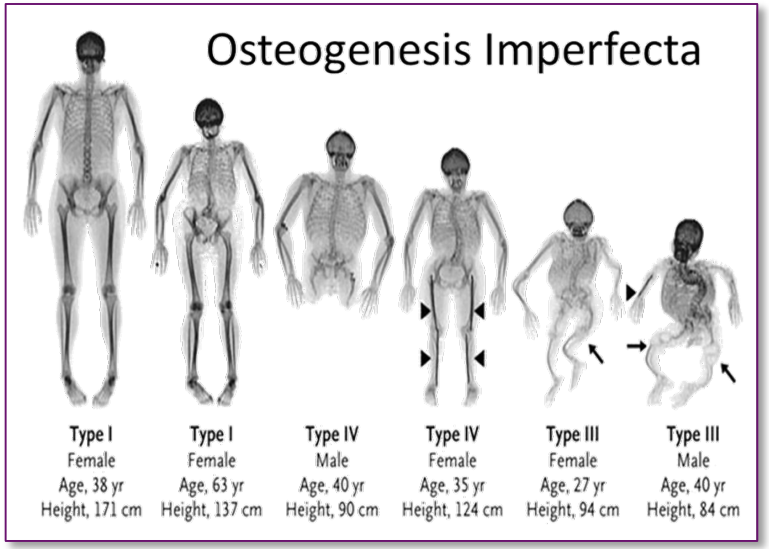
ultragenyx pharmaceutical

Setrusumab (UX143) for Osteogenesis Imperfecta

Phase 2/3 monoclonal antibody in large genetic bone disease

Setrusumab for Osteogenesis Imperfecta (OI)
Large genetic bone disorder with positive Phase 2b adult data

- **OI:** Reduced or abnormal collagen causes increased bone breakdown and decreased bone mass and strength
- **Setrusumab**: Fully human anti-sclerostin antibody that increases bone formation and density
- **Key symptoms:** Bone fragility, fractures, deformities, stiffness, pain, short stature, loss of mobility
- **No approved treatments;** bisphosphonates anti-resorptive treatments are standard of care off-label
- **WW prevalence:** ~60,000 (targeting types I/III/IV)
- **Partnership:** Ultragenyx leads development and has commercial rights ex-EU (receives royalty in EU)
- **Development:** Phase 2b completed in adult OI; pivotal studies in pediatric and adult patients planned



ultragenyx pharmaceutical

Setrusumab: Phase 2b Data in Adults with OI

- Open-label study (n=90) with 3 escalating dose cohorts, dosed monthly for 12 months
  - Studied types I, III, and IV (~90% of prevalent population)
- Bone mineral density increases observed across multiple measures
  - Dose dependent and across anatomical sites
- P1NP biomarker of collagen formation and bone production improvements
  - Seen consistently across disease types
- Bone strength and stiffness potential benefits
  - Improvements in wrist bone failure load and trend to improvement in ankle bone failure load at highest dose
- Setrusumab well-tolerated
  - No cardiac-related safety issues

Setrusumab: Next Steps

- Planning Phase 2/3 study in pediatric patients with OI
  - Phase 2 portion: determine optimal dose based on increases in collagen production using serum P1NP levels
  - Phase 3 portion: evaluate fractures over 15-24 months
  - Aim to initiate study in late-2021, pending regulatory feedback
- Concurrent path forward in adult patients with OI
  - Separate pivotal study in earlier stages of preparation

Development program to be led by development organization that achieved rapid approval for Crysvita in XLH and TIO

ultragenyx pharmaceutical

GTX-102 Program for Angelman Syndrome

Positive interim Phase 1/2 efficacy data from ASO program in Angelman syndrome

GTX-102 for Angelman Syndrome
Large neurodevelopmental disorder

- **Angelman syndrome:** Neurogenetic disorder caused by loss of expression of UBE3A gene
- **GTX-102**: Antisense oligonucleotide (ASO) that targets regulatory RNA to activate paternal UBE3A expression
- **Key symptoms/prognosis:** Motor dysfunction, lack of speech, cognitive impairment, sleep disorder
- **No approved treatments**
- **WW prevalence:** ~60,000
- **Partnership:** Ultragenyx has option to acquire collaborator GeneTx after Phase 1/2 completion
- **Phase 1/2 study design:** Intrathecal intra-patient dose escalating, open-label study in deletion patients (most common genotype and most severe phenotype)

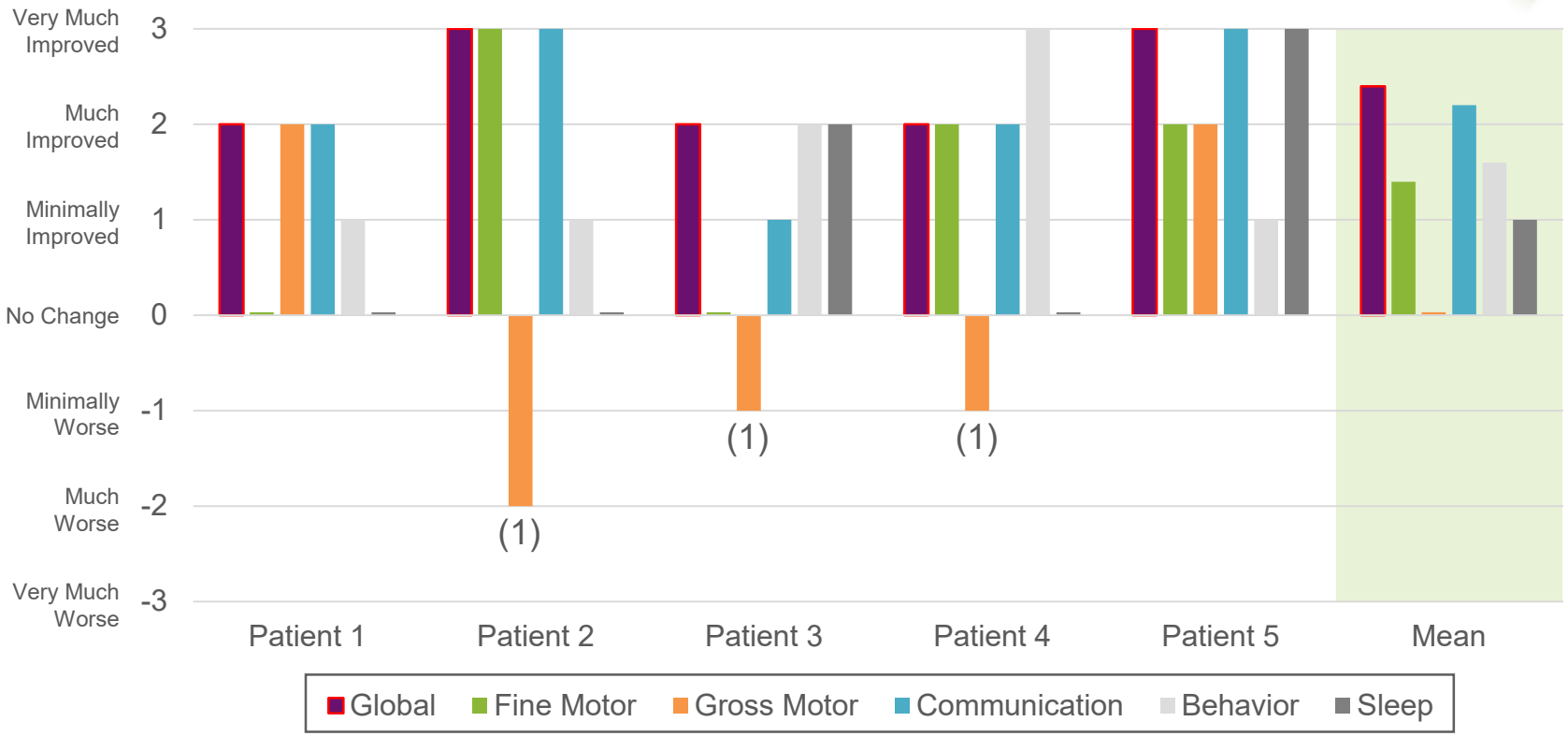


GTX-102 Efficacy and Safety Summary

- Five patients treated with GTX-102 in open label, intra-patient dose escalation safety and efficacy Phase 1/2 study
- All five patients showed substantial improvements in multiple domains
- All five patients had a grade 1 or 2 serious adverse event of lower extremity weakness
  - SAE has fully resolved in all patients

CGI-I-AS* Ratings of Change by Patient at Day 128

Mean global score of +2.4 across patients (scale of -3 to +3)



*CGI-I-AS: Clinical Global Impression of Improvement-Angelman Syndrome; domains are fine motor, gross motor, communication, behavior sleep

(1) Patients 2, 3, and 4 had gross motor impairment at time of assessment due to ongoing SAE

GTX-102 Regulatory Progress

Ex-US Strategy

- **European National Regulator**: Completed pre-application meeting to review efficacy and safety data as well as proposal for starting dose and dose titration
  - The authority agreed in principle on the expansion of the trial in that country
  - Application to enroll clinical studies in this region was recently submitted
- **Canada**: Amendment which included the additional data and updated protocol was cleared by Health Canada. Enrollment to begin early in 2H21

US Strategy

- Discussions are continuing with the FDA and a request for a meeting has been submitted. Meeting expected to take place in 2Q21

Clinical data anticipated before the end of 2021

ultragenyx pharmaceutical

Gene Therapy Program, Platform and Partnerships

Gene Therapy Overview

Programs

Clinical
- DTX301 for OTC
- DTX401 for GSDIa
- UX701 for Wilson

Preclinical
- UX810 for Duchenne
- UX055 for CDD

Partnerships

Program

BAYER

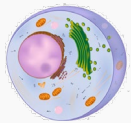
DTX201 for Hem A

Manufacturing

Daiichi-Sankyo

HeLa PCL Tech Transfer

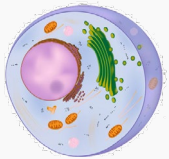
HeLa PCL Manufacturing Platform



HeLa 2.0

Higher fill ratio, 10x yield, better speed via refined high throughput screening

UX701 for Wilson



HeLa 3.0

Further improved yield, speed, and lower COGS

UX810 for Duchenne

Expected to open in 2023

Large Scale Plant Under Construction

ultragenyx pharmaceutical

DTX401: AAV8 for Glycogen Storage Disease Type Ia

- **GSDIa:** Defect in liver's ability to release glucose to the circulation due to G6Pase deficiency
- **Key symptoms/prognosis**
  - Severe life-threatening hypoglycemia
  - Significant morbidity and mortality
  - Long-term liver and renal disease
  - Impaired growth and delayed puberty
  - Severe long-term complications (70-80% patients)
- **Treatment:** Diet and cornstarch only
  - Keeps patients alive but not normal
  - Only curative approach is liver transplantation
- **WW prevalence:** 6,000

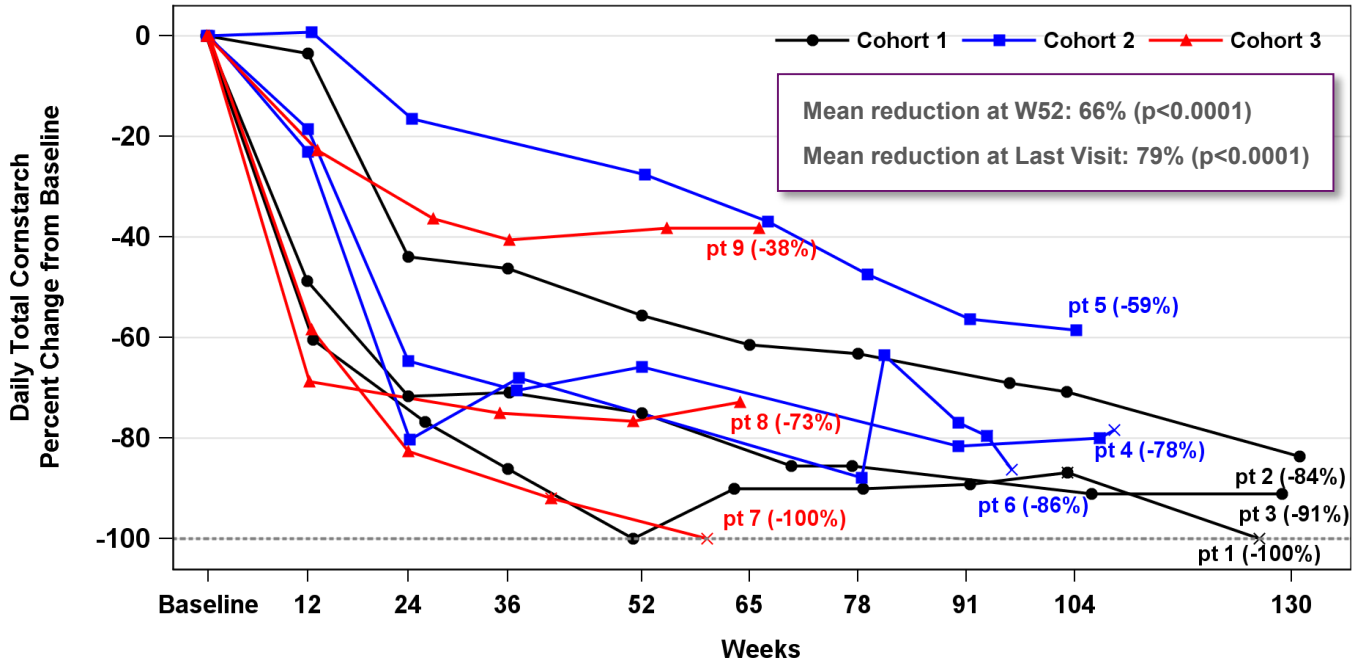
Patient 3 Cornstarch when Travelling

Before **After**

"I don't think people can understand how fast the blood sugars fall. And the stress that these families have, knowing that if you oversleep or you miss your alarm clock, your child can die or have a seizure."
-David Weinstein, former Director-Glycogen Storage Disease Program, Connecticut Children's Medical Center

ultragenyx pharmaceutical

All Patients Reduced Cornstarch Therapy While Maintaining or Improving Time in Euglycemia



Cohort 3: Cornstarch reduced 61%, while time in euglycemia increased by 8%

ultragenyx pharmaceutical

DTX401: Regulatory Status and Phase 3 Design

Regulatory Status

- EOP2 and Scientific Advice meetings held with both FDA and EMA, respectively
- Finalizing Phase 3 design and endpoints with regulators
- Expect to enroll first patient early in 2H21

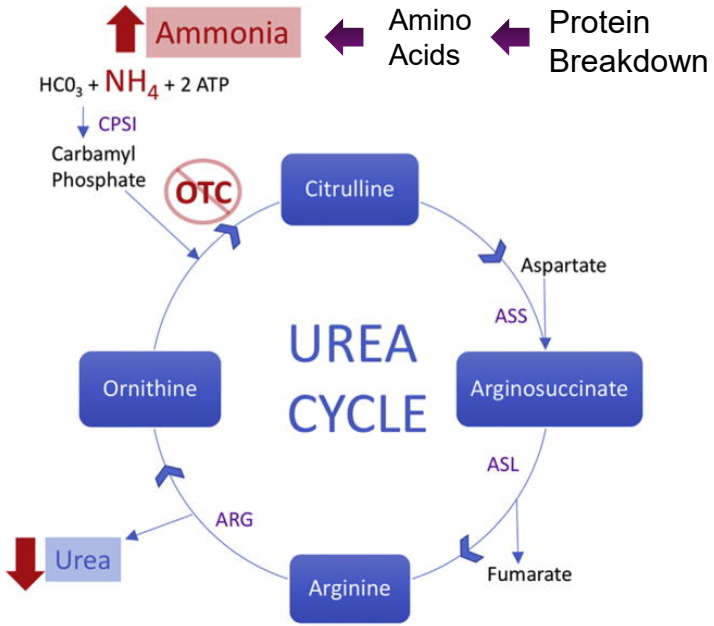
Planned Phase 3 Study Design

- 50 patients, randomized 1:1 DTX401 (1.0×10^{13} GC/kg) to placebo
  - Patients on placebo will cross over to gene therapy arm
- 48-week duration
  - Longer-term Phase 1/2 data to be included in submission to support durability
- Primary endpoint: reduction in oral glucose replacement therapy (cornstarch) while maintaining or improving glucose control by CGM

DTX301: AAV8 for OTC Deficiency

AAV8 gene therapy for stable expression of OTC

- **OTC Deficiency:** X-linked urea cycle disorder, genetic defect in ammonia detoxification
- **Key symptoms/prognosis:**
  - Acute hyperammonemic episodes
  - Adverse cognitive & neurological effects
  - Hospitalizations
  - Death
- **Treatment limited:** Liver transplantation only curative, ammonia scavengers, protein restricted diet
- **WW prevalence:** ~10,000, 80% late-onset



S. Harris, et al., Obstetrics and Gynecology Clinics of North America (2018)

Durable Metabolic Control and Sustained Responses Lasting More than 3 Years Post-Treatment

Overall Response

- 6 of 9 patients treated to date responded to DTX301
  - Includes all 3 patients in highest dose cohort
- 3 complete responders who have discontinued alternative medications and protein-restricted diets without ammonia issues
  - 3 other responders with stable ammonia; all started tapering medications and 2 liberalizing diets

Prophylactic Steroid Cohort Update

- Both patents dosed and doing well
  - First patient has demonstrated a response
  - Second patient responder status will be evaluated after patient finishes steroid regimen

DTX301: Regulatory Status and Phase 3 Design

Regulatory Status

- EOP2 and Scientific Advice meetings held with FDA and EMA, respectively
- Expect to enroll first patient in 2H21

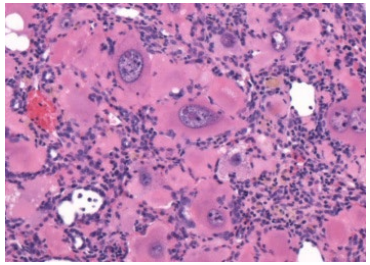
Phase 3 Study Design

- 50 patients, randomized 1:1 DTX301 (1.7×10^{13} GC/kg) to placebo
  - Patients on placebo will cross over to gene therapy arm
- 64-week duration
  - Longer-term Phase 1/2 data to be included in submission to support durability
- Co-primary endpoints
  - Change in 24-hour plasma ammonia levels
  - Percent of patients who achieve a response as measured by discontinuation or reduction in baseline disease management

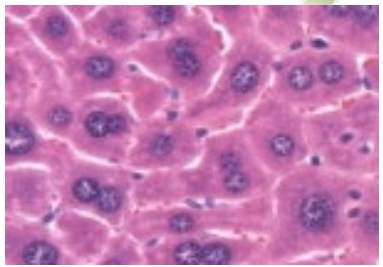
UX701: AAV9 for Wilson Disease

Second clinical program to utilize HeLa manufacturing system

- **Wilson Disease:** Causes copper to accumulate in liver, brain and other vital organs
- **Key symptoms/prognosis:** Liver failure, neurological deterioration, death
- **Standard of Care:** Chelation therapy and dietary restriction
  - Many patients still experience liver and neurological deterioration
- **WW prevalence:** >50,000
- **Status:** IND cleared; plan to initiate seamless single-protocol Ph1/2/3 early in 2H21

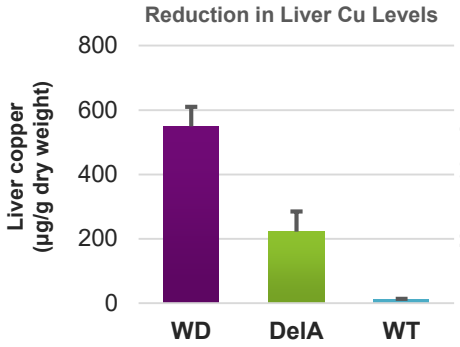


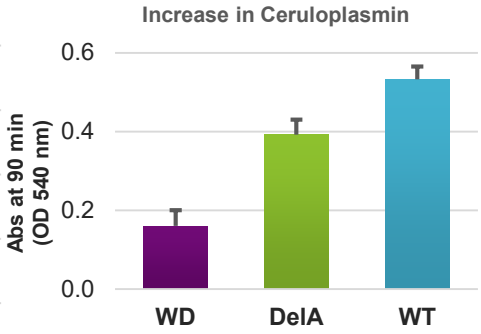
Untreated KO Mice



1x10^{11} GC Treated Mice

Reduced liver copper accumulation leading to improved liver pathology in preclinical models





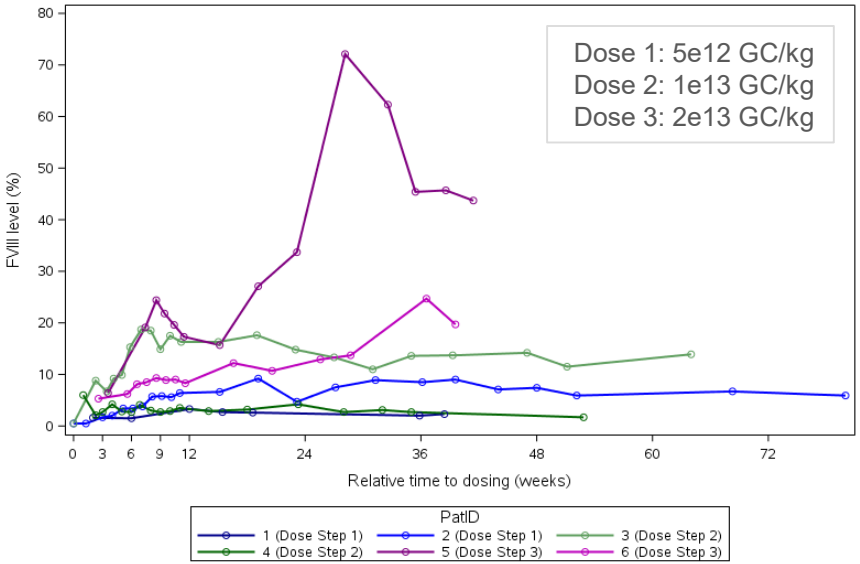
Positive, Clinically Effective Hem A Data from the HeLa Platform

Out-licensed program to Bayer validates Ultragenyx HeLa system

- Positive data from first three dose cohorts
  - Data from six patients, two at each dose
- Clinically meaningful Factor VIII levels in five of six patients
  - Sustained FVIII levels up to >18 months with no evidence of loss of expression
  - No spontaneous bleeds were reported after achieving protective FVIII levels (>15 IU/dL)
- Favorable safety results
  - ALT/AST elevations observed in one patient in Cohort 2 and both in Cohort 3
  - Managed with tapering course of corticosteroids
- Dose escalation ongoing

Factor VIII Expression Levels

(Chromogenic assay; % normal)



Pipe *et al.* American Society of Hematology (ASH), December 2020

Building a Commercial Rare Disease Company with Accelerating Growth

4 approvals in first 10 years

Advance clinical pipeline and commercialize larger rare disease indications

Mepsevii™ (vestronidase alfa-vjbk) injection
MPSVII

CRYSVITA® burosumab
XLH & TIO

DOJOLVI® TRIHEPTANOIN Oral Liquid
LC-FAOD

GSDIa
OTC
Angelman
Osteogenesis Imperfecta
Wilson
GSDIII
Duchenne

Protein Biologic	Small Molecule
Gene Therapy	ASO / mRNA

ultragenyx pharmaceutical

ultragenyx pharmaceutical

Appendix

Key Licenses & Intellectual Property

Product	License	US Intellectual Property Rights/Royalties
CRYSVITA® (XLH, TIO)	Kyowa Kirin Co. (KKC)	▪ Anti-FGF23 antibodies and use for treatment of XLH and TIO (2022-2032)[1] ▪ Q2W dosing for treatment of FGF23-associated hypophosphatemic disorders (2035) ▪ See discussion of KKC license and collaboration in annual report for royalty summary
MEPSEVII® (MPS 7)	St. Louis University (Know-How)	▪ Low single-digit royalty until expiration of orphan drug exclusivity
	N/A (IP Owned by Ultragenyx)	▪ Recombinant human GUS (rhGUS) and use for treatment of MPS7 (2035)
DOJOLVI® (LC-FAOD)	Baylor Research Institute (BRI)	▪ Compositions comprising triheptanoin (2025-2029)[1] ▪ Mid single-digit royalty
DTX301 (OTC Deficiency)	Sub-License from REGENXBIO of UPENN IP	▪ AAV8 Capsid (2022-2024) ▪ Recombinant vectors comprising codon-optimized OTC gene (2035) ▪ Low to mid single-digit royalty
DTX401 (GSDIa)	Sub-License from REGENXBIO of UPENN IP	▪ AAV8 Capsid (2022-2024) ▪ Low to mid single-digit royalty
	NIH (Non-Exclusive)	▪ Recombinant vectors comprising codon-optimized G6Pase gene (2034) ▪ Low single-digit royalty
UX143 (Osteogenesis Imperfecta)	Mereo Biopharma	▪ Setrusumab antibody (2028) ▪ Use of anti-sclerostin antibodies including setrusumab for treatment of OI (Pending; 2037) ▪ Tiered double-digit royalty on ex-EU sales
DTX201 (Hemophilia A)	Sub-License from REGENXBIO of UPENN IP	▪ Hu37 Capsid (2024) ▪ Recombinant vectors comprising codon-optimized Factor VIII gene (2037) ▪ Low to mid single-digit royalty
GTX-102 (Angelman Syndrome)	Option to Acquire GeneTx (Exclusive Licensee of TAMU's GTX-102 IP)	▪ UBE3A-ATS antisense oligonucleotides including GTX-102 and their use in treatment of AS (Pending; 2038) ▪ Development and commercial milestones plus royalties

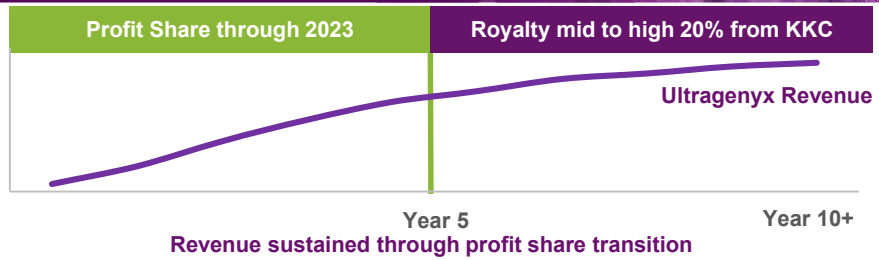
1: Includes projected U.S. patent term extension

ultragenyx pharmaceutical

Revenue to Ultragenyx Maintained After Transition from Profit Share to Royalty in U.S.

CRYSVITA® burosumab

U.S. Prevalence: 12,000



	U.S. AND CANADA	LATIN AMERICA	EUROPE
Commercialization	▪ KKC books sales ▪ 50/50 profit share for 5 years then tiered revenue share ▪ Shared commercial activities over time	Ultragenyx commercializes and books sales	KKC commercializes and books sales
Royalties	After 5 years, tiered revenue share in mid to high 20% range to Ultragenyx after profit share period	Low single-digit royalty to KKC	Up to 10% non-cash revenue[1] to Ultragenyx after Royalty Pharma transaction
Commercial supply	KKC supplies: 35% of net sales through 2022, 30% thereafter	KKC supplies: 35% of net sales through 2022, 30% thereafter	NA

1: Beginning January 1, 2020, the company no longer receives cash payments from the EU territory royalty until the respective threshold amount is met pursuant to the Royalty Pharma transaction. The company remains, however, contractually entitled to the royalties from KKC and will continue recognizing the Crysvita EU territory royalties in total revenues as “non-cash revenue” since the associated cash proceeds will be remitted to Royalty Pharma.

ultragenyx pharmaceutical

CRYSVITA® Exclusivity Summary

XLH Orphan Exclusivity — 2025

TIO Orphan Exclusivity — 2027

Biologics Exclusivity — 2030

Crysvita CoM Patent — 2032*

Q2W Dosing Patent — 2035

2018 2020 2022 2024 2026 2028 2030 2032 2034 2036

2028 — XLH Orphan + D&M Exclusivity

2033* — Crysvita CoM Patent

2035 — Q2W Dosing Patent

*Includes projected US PTE and EU SPC awards

MEPSEVII® Exclusivity Summary

Mepsevii™
(vestronidase alfa-vjbk)
injection, for intravenous use

MPS7 Orphan Exclusivity
2024

Biologics Exclusivity
2029

Mepsevii CoM Patent
2035

2018 2020 2022 2024 2026 2028 2030 2032 2034 2036

2028
MPS7 Orphan + D&M Exclusivity

2035
Mepsevii CoM Patent

ultragenyx pharmaceutical

DOJOLVI® Exclusivity Summary

DOJOLVI® TRIHEPTANOIN Oral Liquid

NCE Exclusivity — 2025

LC-FAOD Orphan Exclusivity — 2027

Dojolvi CoM Patent — 2029*

Ultrapure Dojolvi (Pending) — 2034

2018 2020 2022 2024 2026 2028 2030 2032 2034 2036

Est. LC-FAOD Orphan & D&M Exclusivity — 2034

Ultrapure Dojolvi (Pending)

*Includes projected U.S. PTE award

ultragenyx pharmaceutical