Corporate Presentation

November 2020

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.

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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.

Positioned for Significant Value Growth

Four Medicines Developed and Approved in 10 Years

Strong Revenue Drivers

- Exceptional Crysvita launch continues
- Growth potential with Dojolvi and Mepsevii

Diverse Portfolio

- Broad clinical and preclinical pipeline
- Gene therapy and mRNA platforms

Financial Strength

- $766M cash and investments at end of 3Q20
- $180M revenue YTD through 3Q 2020

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Building an Exceptional Rare Disease Company

	YE 2019	2025
Revenue	$104M	Approaching $1B
Commercial Products	2	7+
Clinical Programs	2	5+
Treatable Patient Population	~25,000	150,000+
Manufacturing	CMO	Commercial Gene Therapy Plant & CMO

Four Approvals in Three Years, Two in June 2020

CRYSVITA® burosumab

XLH

~48,000 patients in developed world

CRYSVITA® burosumab

TIO

~2,000-4,000 patients in developed world

Bone / Endocrinology

2017 2018 2019 2020

Metabolic Genetics

Mepsevii™ (vestronidase alfa-vjbk) injection

MPS VII

~200 patients in developed world

DOJOLVI® TRIHEPTANOIN Oral Liquid

LC-FAOD

~8,000-14,000 patients in developed world

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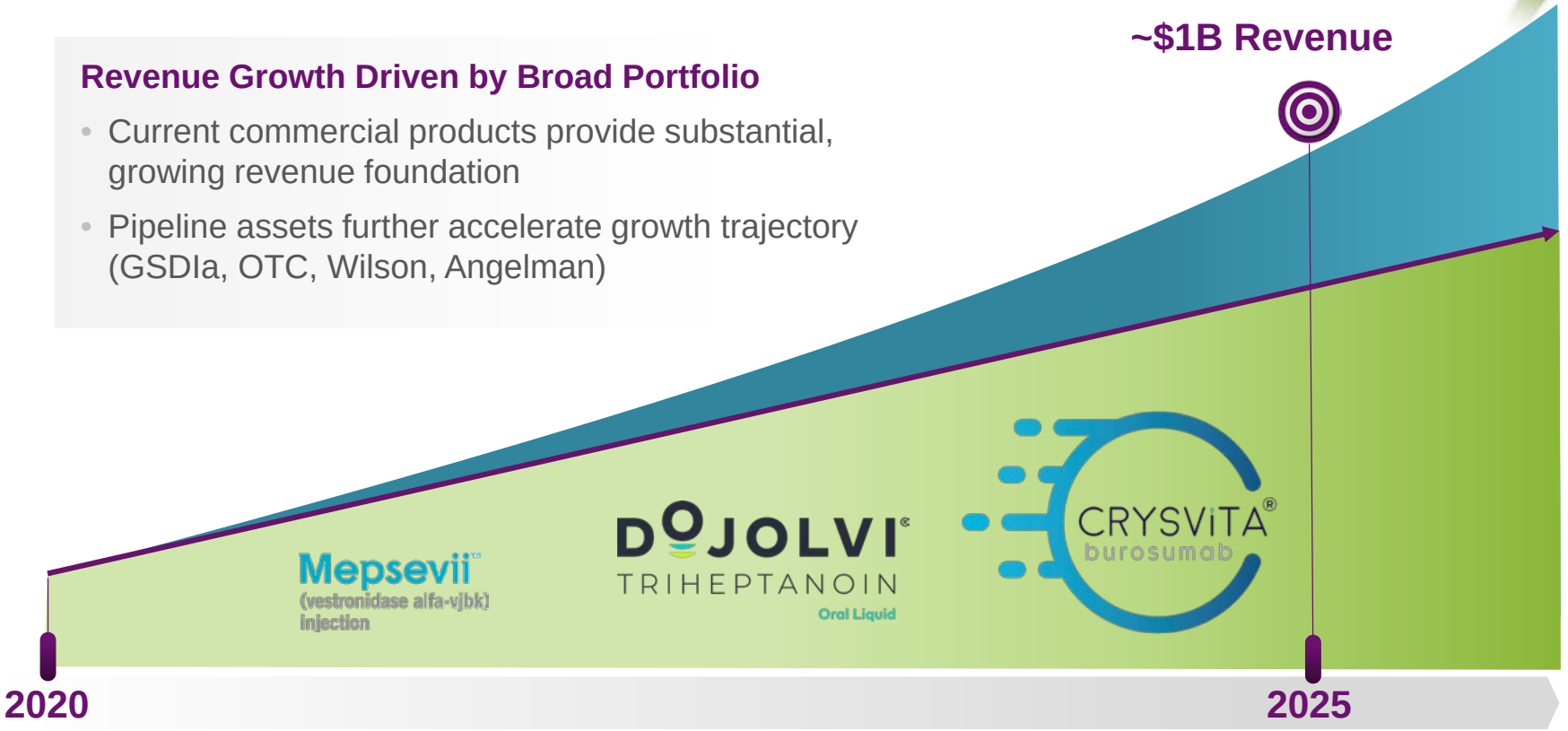
Ultragenyx in 2025:
Potential for ~10x revenue growth in 5 years

Revenue Growth Driven by Broad Portfolio

- Current commercial products provide substantial, growing revenue foundation
- Pipeline assets further accelerate growth trajectory (GSDIa, OTC, Wilson, Angelman)



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Strong Crysvita Performance and Solid Financial Base Drive Future Growth

YTD through Q3 Revenue		YoY Change
Crysvita in Ultragenyx Territories	**$98.5M**	**91%**
North America Profit Share	*$91.1M*	*87%*
LatAm Product Sales	*$7.4M*	*172%*
Mepsevii	**$11.7M**	**41%**
Dojolvi	**$6.6M**	**225%**
Total Company Revenue[1]	**$179.5M**	**163%**

2020 Crysvita Revenue Guidance[2]	
Crysvita in Ultragenyx Territories	**$130M to $140M**

Lower end of guidance increased from $125M to $130M

1: Includes Crysvita in EU territory, which is primarily non-cash, and revenue from agreement with Daiichi Sankyo, which has primarily been received upfront

2: Crysvita Revenue guidance is for Ultragenyx regions, which excludes royalty revenue in EU

Strong Capital Position Supported by Financial Discipline

- Cash balance[3] as of 3Q20: $766 million
  - Additional $435 million net proceeds from public stock offering[4]
- 20%+ reduction in net cash burn[5] in 2020

3: Cash, cash equivalents, and available-for-sale investments as of September 30, 2020
4: Underwritten public offering closed on November 2, 2020
5: Net cash used in operations plus capital expenditures

Diverse Clinical Pipeline Across Metabolic Indications

Additional >15 Preclinical Programs

Candidate	Description	IND	Phase 1	Phase 2	Phase 3	Regulatory Review	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
DOJOLVI® TRIHEPTANOIN Oral Liquid	Substrate Replacement	LC-FAOD						~8,000 - 14,000
DTX301	AAV8-OTC Gene Transfer	OTC						~10,000
DTX401	AAV8-G6Pase Gene Transfer	GSDIa						~6,000
DTX201	AAV-FVIII Gene Transfer	Hemophilia A						~144,000
UX701	AAV-ATP7B Gene Transfer	Wilson						~50,000
GTX-102**	Antisense Oligonucleotide	Angelman						~60,000

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

* Crysvita is approved in the U.S., Canada, EU, and Brazil
* Mepsevii is approved in the U.S., EU, and Brazil
* Dojolvi is approved in the U.S.
** Ultragenyx has an option to acquire GTX-102 from GeneTx

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Two Commercial Approvals in June 2020, Additional Clinical Milestones in 2H20

			1H20	2H20[1]
Crysvita Anti-FGF23 MAb	TIO	U.S. FDA Approval (June 18)	✓	
Dojolvi Substrate Replacement	LC-FAOD	U.S. FDA Approval (June 30)	✓	
DTX301 AAV8 Gene Therapy	OTC	Cohort 3 Data	✓	
		End of Phase 2 Meeting Cohort 4 (Prophylactic Steroid) Data		☐
DTX401 AAV8 Gene Therapy	GSDIa	Cohort 3 (Confirmatory) Data	✓	☐
		End of Phase 2 Meeting Phase 3 Initiation		☐ *1H21*
GTX-102[2] ASO	Angelman Syndrome	Phase 1/2 Study	✓ *Initiation*	✓ *Interim Data*
UX701 Gene Therapy	Wilson Disease	IND Submission		☐
Gene Therapy Platform	New Partnerships	Daiichi Sankyo: Manufacturing Technology	✓	
		Solid: Duchenne Microdystrophin		✓

1: Barring potential delays due to COVID-19
2: Ultragenyx has an option to acquire GeneTx (GTX-102)

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

New Gene Therapy Program Leveraging HeLa PCL for Duchenne Muscular Dystrophy

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Best-in-Class Microdystrophin

- nNOS binding restoration
- Functional benefit in preclinical models
- Expertise in Duchenne and muscle biology

HeLa PCL Platform

- Commercial-grade 2,000L large-scale manufacturing
- Lower cost for high-prevalence and high-dose requirements

AAV8 Variant

- Demonstrated favorable immune profile in clinic
- Successfully made at 2,000L scale

Leveraging complementary technology and capabilities to develop high-quality AAV therapy for large rare disease

Two Products Approved by U.S. FDA in June 2020

DOJOLVI® TRIHEPTANOIN Oral Liquid

- **Approved** by FDA on June 30, 2020 for the treatment of pediatric and adult patients with molecularly confirmed LC-FAOD
- Potential revenue expected to be modest in 2020 and will build over time
- ~2,000 – 3,500 patients in the U.S.

CRYSVITA® burosumab — Tumor-Induced Osteomalacia Indication

- **Approved** by FDA on June 18, 2020 for patients with TIO where the tumor cannot be surgically removed
- Second indication for Crysvita following approval for XLH in 2018
- ~500 – 1,000 patients in the U.S.

Launches leverage existing commercial infrastructure with minor incremental expense

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Dojolvi: First FDA-Approved Therapy for the Treatment of LC-FAOD

DOJOLVI® TRIHEPTANOIN Oral Liquid

- **Indication:** Treatment of pediatric and adult patients with molecularly confirmed LC-FAOD
- **Key symptoms/prognosis:**
  - Hypoglycemia, muscle rupture, heart failure
  - Mortality of >50%[1]; a cause of SIDS (newborn screened in U.S.)
- **Treating physicians:** Metabolic geneticists, across 160 centers in the U.S.
- **U.S. prevalence:** ~2,000 – 3,500
- **Regulatory Submissions:** Canada, Brazil, EU discussions on going

[1] J Inherit Metab Dis 2013;36:795-803

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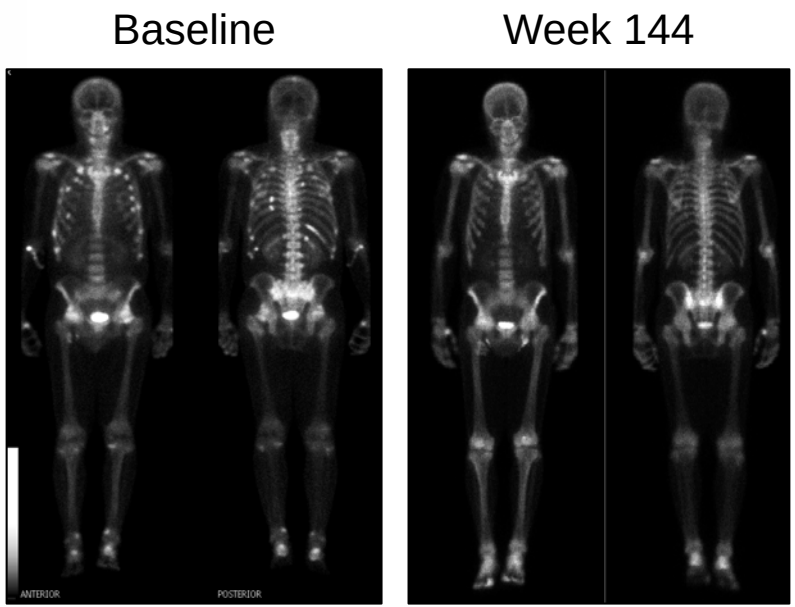
Crysvita for Tumor Induced Osteomalacia (TIO)

- **First approved therapy** in the U.S. for patients with TIO who cannot undergo surgical removal of tumors
- **Second FDA-approved indication** for Crysvita, also approved for XLH
- **Key symptoms:**
  - Muscle weakness, fatigue, bone pain, fractures
- **U.S. prevalence:** ~500 – 1,000



Bone scan of a 52-year old man with TIO shows decreased uptake over 144 weeks of burosumab treatment

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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 with positive interim data

- **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)

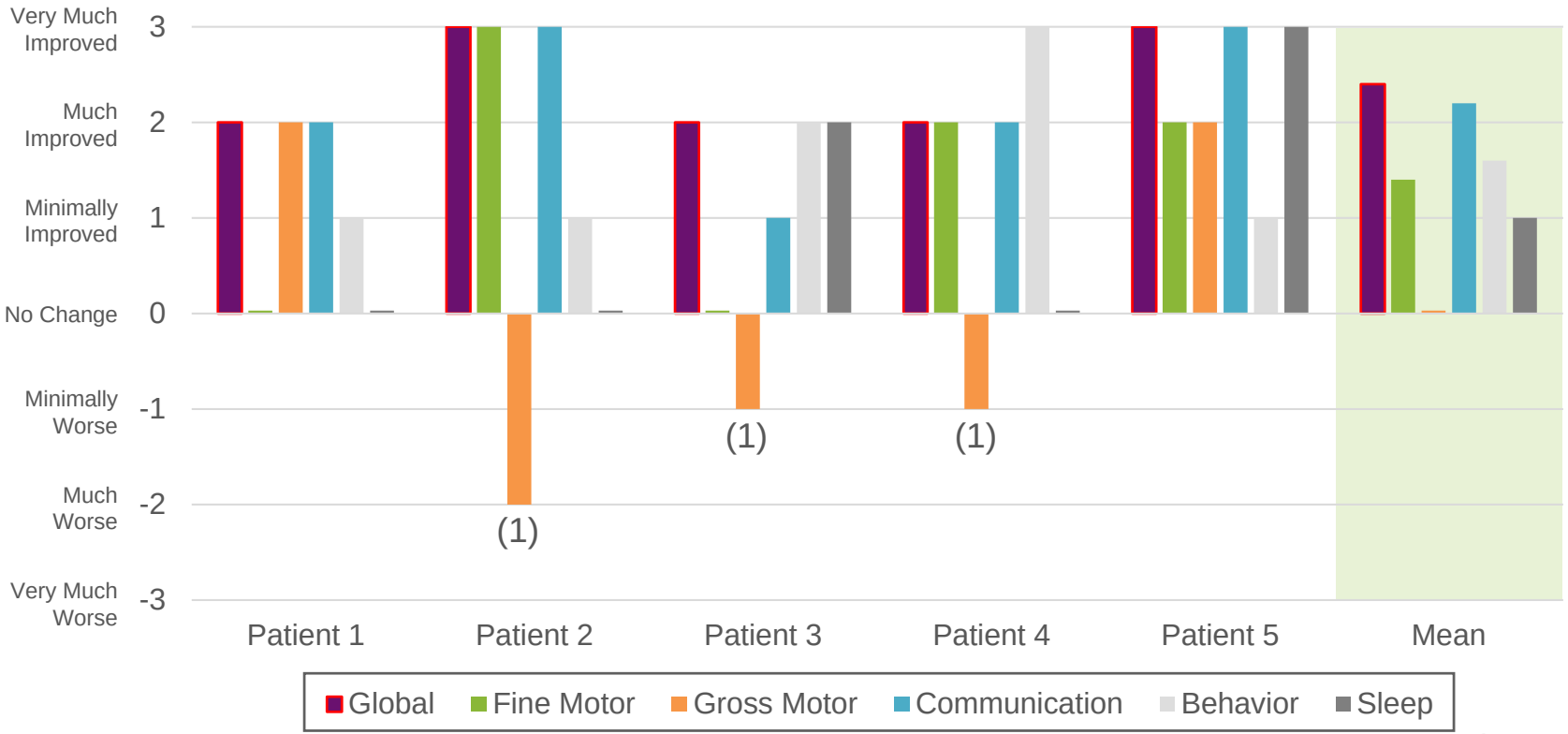
All 5 Initial Patients Improved Across Multiple Angelman Syndrome Domains

- Interim results indicate that all 5 initial patients had substantial improvements in multiple disease domains as assessed by CGI-I-AS*
  - Mean global CGI-I-AS score of +2.4 across patients (scale of -3 to +3)
- Positive CGI-I-AS results supported by other indications of benefit
  - Increases in Bayley-4 expressive/receptive communication scales and in 3 patients in Observer Reported Communication Ability (ORCA) measure
  - Improvements reported by patient caregivers
- Improvements generally began early following initial treatment
  - Some patients showed response within a few weeks and all within 4 months
  - Responses began after lowest doses in some patients

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

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

All patients had positive response in at least three domains



*Day 86 for patient 5

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

Caregivers Report a Range of Improvements

Patient ages between 5 and 15 years old

- Acquisition of spoken words, signs and gestures, and augmentative and alternative communication abilities
  - Two previously nonverbal patients began using words, one reaching 9 words
- Ability to respond to their name, follow commands, and focus on tasks
- Acquisition of independent capabilities, such as self-feeding with a fork
- Increased abilities in physical activities, such as patients swimming on own and catching/throwing a ball
- Dramatically improved sleep
- Decreased maladaptive behaviors
- Increased social engagement
- Improved gait and posture

GTX-102 Phase 1/2: Safety & Next Steps

- Grade 1 or 2 SAE of lower extremity weakness seen in all 5 initial patients
  - Rate as mild to moderate, but significant weakness in 3 and unable to walk in 2 subjects
  - Of 4 doses studied across 10-fold range, SAE occurred at highest dose in 4 patients and second highest dose in 1 patient
  - Dosing paused after first onset observed
  - Completely resolved in 4 patients and near complete resolution in 1 patient
  - Time course of resolution faster while clinical improvements were sustained longer
- Evaluations consistent with lower back inflammation due to GTX-102 intrathecal delivery
  - MRI signals consistent with inflammation in lumbosacral meninges and nerve roots
  - CSF protein increases similar to seen in other ASO treatments
- No other SAEs reported to date; other AEs include transient ataxia, headache, fatigue
- Amending study design and seeking agreement with the FDA prior to resuming enrollment and dosing
  - Focusing on lower end of dosing range: within efficacious doses but below SAE-associated doses
  - Modifying administration process to minimize duration of exposure at site of administration

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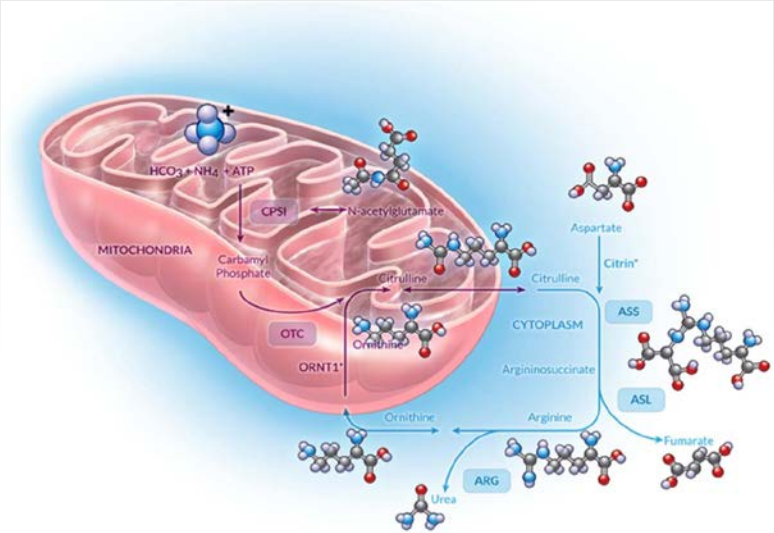
Gene Therapy Programs and Platform

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



DTX301: Six of Nine Patients Responding Now Including all 3 Patients in Cohort 3

- **Cohort 3**: **Responses from all three patients**
  - Patient 7: Complete responder (off NH3 scavenger drugs and diet)
  - Patient 8: Responder (discontinued of one of two ammonia scavengers and modified diet)
  - Patient 9: Confirmed Responder (confirmed at week 24, not yet tapered medication or diet)
- **Cohorts 1 and 2: Long-term follow-up of complete responders**
  - Ureagenesis greater than 100% for 2 years and 1.5 years, respectively
  - Restricted protein diet and alternate pathway drugs discontinued for more than one year
  - Ammonia maintained within normal parameters throughout the long-term follow-up period
  - Excellent clinical condition with no significant adverse events, hospitalizations, or events related to urea cycle disorders

DTX301: Responses Observed in All Dose Cohorts and Three Responders at Cohort 3 Dose

Cohort / Dose (GC/kg)	**Patient # (Gender) / Follow-Up Duration**	**% Change in Ureagenesis** (baseline → after treatment, % normal[1])	**% Change in Ammonia Levels** (baseline → after treatment, umol/L)	**Alternate Pathway Medication and Diet Status**	**Response Status**
Cohort 1 ($2x10^{12}$ dose)	1 (Male) 130 Weeks	**+53% (67% → 102%)**	Normal levels maintained	**Off medications Liberalized diet**	**Complete responder[3]**
	2 (Female) 104 Weeks	+6% (52% → 55%)	**92% decrease (146 → 11)**	No change	No response
	3 (Male) 104 Weeks	**+81% (48% → 87%)**	Normal levels maintained	No change	No response
Cohort 2 ($6x10^{12}$ dose)	4 (Male) 78 Weeks	**+79% (66%[4] → 118%)**	Normal levels maintained	**Off medications Liberalized diet**	**Complete responder[3]**
	5 (Female) 78 Weeks	-38% (19% → 12%)	Normal levels maintained	No change	No response
	6 (Female) 78 Weeks	**+218% (20% → 64%)**	**80% decrease (156 → 31 [Week 78])**	**Tapering medication Liberalizing diet**	**Responder**
Cohort 3 ($1x10^{13}$ dose)	7 (Female) 52 Weeks	**+79% (24% → 44%)**	Normal levels maintained	**Off medications Liberalized diet**	**Complete responder[3]**
	8 (Female) 36 Weeks	?%[2] (66% → 25%)	**90% decrease (184 → 19 [Week 24])**	**Increased protein intake and discontinuation of one of two ammonia scavengers**	**Responder** (consistent ammonia reduction; clinical benefit noted)
	9 (Male) 24 Weeks	**+188% (25%[4] → 73%)**	Normal levels maintained	No change yet	**Responder (confirmed)** (still on steroids)

[1] Normal rate of ureagenesis = 300 umol*kg/hr. [2] Aberrant high baseline ureagenesis values inconsistent with patient clinical severity making ureagenesis not interpretable.
[3] Complete responder = biochemical effect sustained after discontinuation of alternate pathway medications and diet liberalization. [4] Baseline ureagenesis based on screening value.

DTX301: Safety Profile

- No infusion-related or serious adverse events (AEs) have been reported to date
- All reported AEs were grade 1-2
- Six patients (two in Cohort 1, one in Cohort 2, and three in Cohort 3) experienced mild, asymptomatic ALT increases consistent with those seen in other AAV gene transfer clinical trials
  - ALT increases were managed and resolved with a protocol-specified, reactive, tapering regimen of oral corticosteroids administered in the outpatient setting

DTX301: Next Steps

- Enrolling three additional patients in prophylactic steroid cohort at 1e13 dose
  - Dosing in this cohort is currently on hold due to COVID-19
- Planning for Phase 3 study and continuing FDA discussions
  - Ammonia expected to be a primary endpoint based on FDA feedback

Prophylactic steroid cohort (1e13 dose) update around the end of 2020[1]

1: Barring potential delays due to COVID 19

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, Director-Glycogen Storage Disease Program, Connecticut Children's Medical Center

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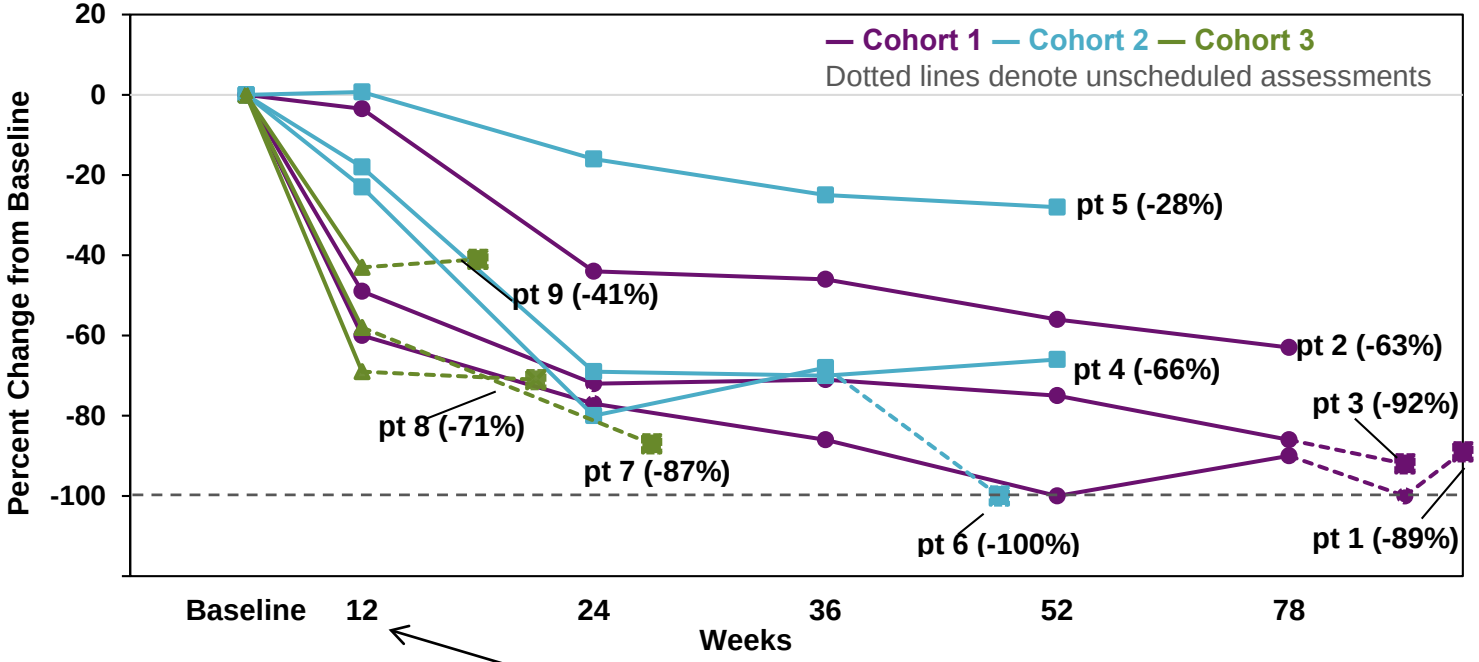
DTX401: Treatment Protocol Changes

Lessons learned from earlier cohorts prompted the following changes to the protocol prior to dosing of patients in Cohort 3 ($6x10^{12}$ GC/kg):

- Reduced cornstarch dose at the start of the controlled fasting challenge (decreased from 35 grams to 5 grams)
- Use of continuous glucose monitoring (CGM)
- Implementation of an ‘optimized’ reactive steroid regimen

Substantial Reduction in Cornstarch Requirements for All Patients
More Rapid Reductions in Cohort 3 by 12 Weeks



Week 12 Mean Reduction in Cornstarch

Cohort 1	38%
Cohort 2	14%
Cohort 3	57%

DTX401: Summary of Data

Dose Cohort 3

- More rapid reductions in cornstarch requirements
- CGM confirms early transgene expression and allows for timely and more accurate cornstarch reduction
- ‘Optimized’ reactive steroid regimen more effectively mitigated ALT elevations

All Cohorts

- All patients (n=9) have shown an improved response in time to hypoglycemia and decreased cornstarch requirements
- Consistent and acceptable safety profile across all patients

DTX401: Next Steps

- Collecting longer-term data from confirmatory Cohort 3
- Planning for Phase 3 study and continuing FDA discussions
  - Cornstarch requirements, time to hypoglycemia during fasting challenge, number of hypoglycemic events through CGM data, all important in evaluating glucose control

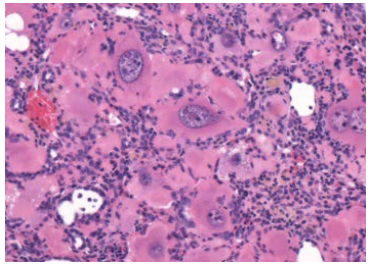
Longer-term Cohort 3 data expected in second half of 2020[1]

1: Barring potential delays due to COVID 19

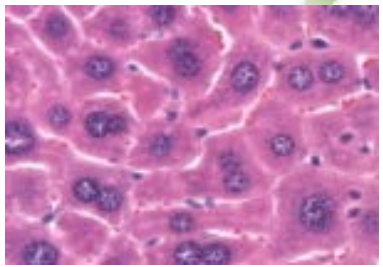
UX701 for Wilson Disease

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
- **IND planned by end of 2020**

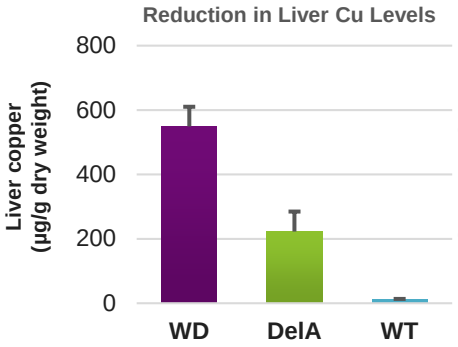


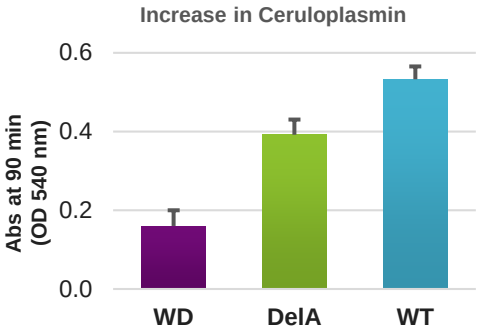
Untreated KO Mice



1x10^{11} GC Treated Mice

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





Gene Therapy Manufacturing Platforms: Optimized for Scalability and Efficiency

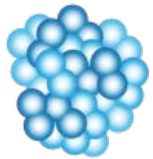
HeLa PCL enables reproducible and consistent commercial-scale manufacturing at lower COGS

2,000L

HemA in the clinic and planned for Wilson

GSDIa and OTC will transition to in-house manufacturing facility in early commercial stages

HEK293 Suspension/Transfection



4 x 200L

OTC and GSDIa in the clinic; GSDIa will transition to HeLa

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HeLa PCL Platform: Unlocking Value Across Multiple Programs and Companies

Broad Platform-Based Partnership

Daiichi-Sankyo

- Non-exclusive license to use manufacturing technologies initiated in 2020
- $125M upfront plus $75M equity investment

Internally-Originated Program

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- IND expected by year-end for Wilson disease
- First in-house program to utilize HeLa platform
- Additional earlier-stage programs on track

Program-Based Partnership

BAYER

- License to HeLa-based program in Hemophilia A
- Clinical program using HeLa at 2,000L commercial scale

Tech Collaboration for Internal Program

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- License to use novel microdystrophin with AAV8
- Utilize HeLa platform for cost-effective manufacturing in Duchenne muscular dystrophy

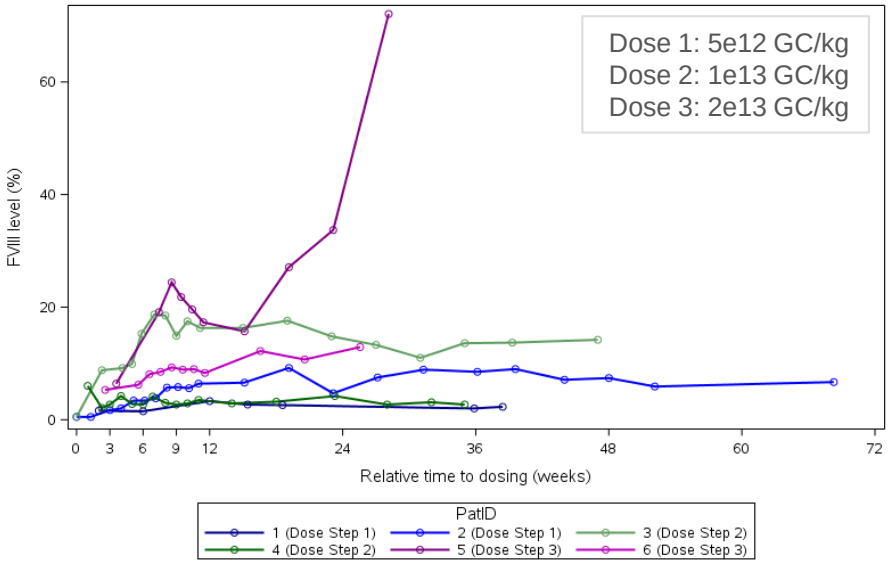
Positive, Clinically Effective HemA Data from the HeLa Platform

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 one patient in Cohort 1 and all patients at higher doses
  - No spontaneous bleeds in Cohort 3 after reaching full expression and discontinuing prophylaxis
- 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.* International Society on Thrombosis and Haemostasis (ISTH), July 2020

Building a Diversified Commercial Rare Disease Company

2010 – 2019

- $100M revenue in 2019
- 2 approved products
- 9 clinical programs
- 34 clinical studies
- 14+ preclinical programs

2020

Commercial Business

Mepsevii™ (vestronidase alfa-vjbk) injection

CRYSVITA® burosumab

DOJOLVI® TRIHEPTANOIN Oral Liquid

- Crysvita TIO and Dojolvi approved in June
- $180M revenue through Q3 YTD

Pipeline Development

- Angelman, OTC, and GSDIa data
- New preclinical Duchenne gene therapy program and Wilson gene therapy IND

2021 – 2025+

- 10x revenue growth by 2025, approaching $1B revenue
- 7+ approved products
- 6+ programs with pivotal data
- GMP gene therapy manufacturing plant
- Robust pipeline

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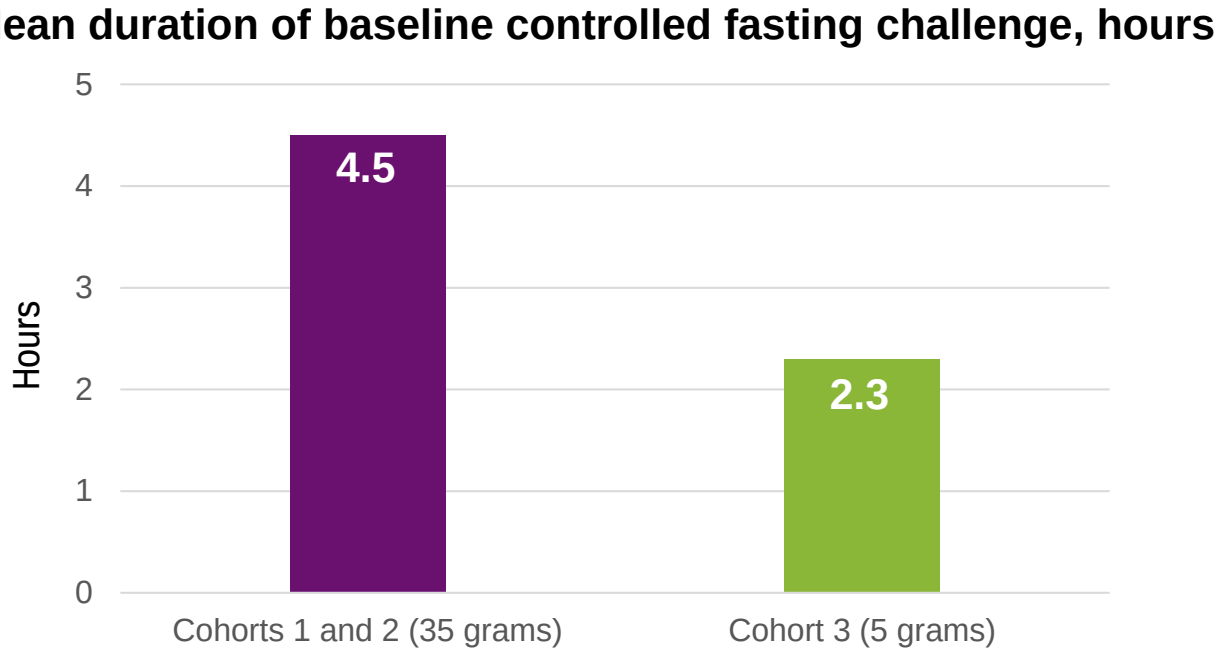
Appendix

DTX401: Cohort 3 Baseline Time to Hypoglycemia 48% Less Than Baseline of Prior Cohorts



- Reduced cornstarch dose at the start of the controlled fasting challenge in Cohort 3 avoided hyperinsulinemic responses observed in Cohorts 1 and 2

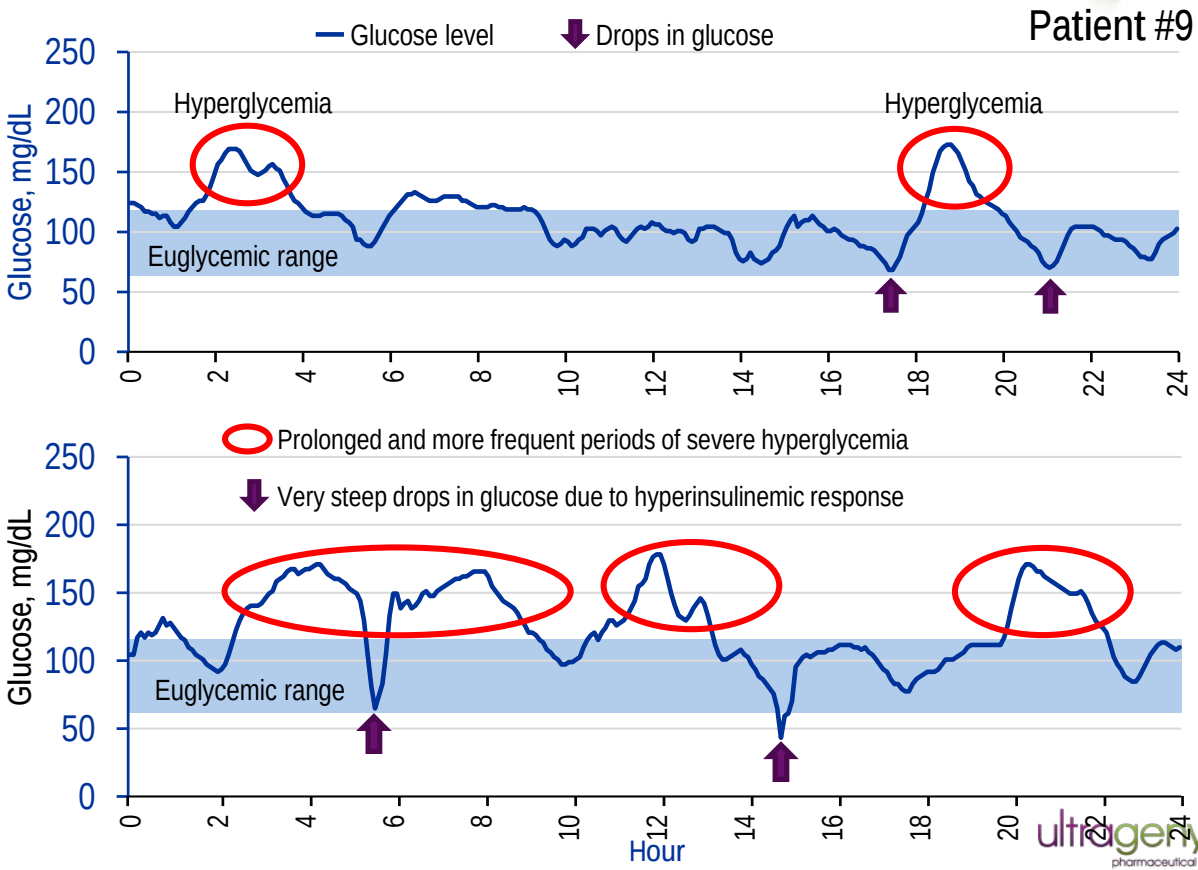
DTX401: Early Transgene Expression Revealed by CGM

Day -3 Prior to DTX401 Dose

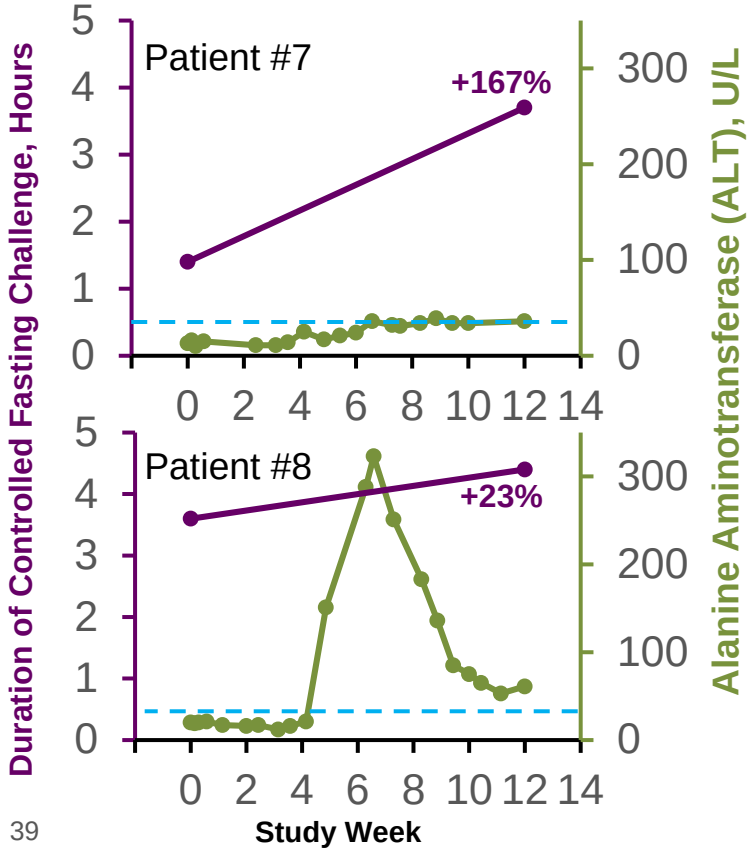
- Periods of hyperglycemia followed by drops in glucose

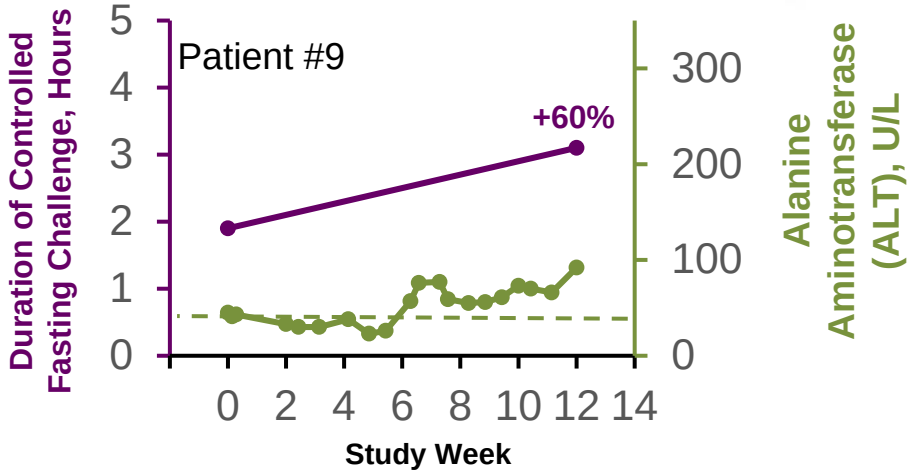
Day +4 After DTX401 Dose

- As transgene expression begins post-DTX401 dose, prolonged periods of severe hyperglycemia are followed by hyperinsulinemic responses resulting in severe drops in glucose



DTX401: All Patients in Cohort 3 Experienced Increased Time To Hypoglycemia





- All three patients received an optimized reactive steroid regimen at approximately week 4
- Patient 8 had an asymptomatic and transient rise in ALT

– – – Female ALT Upper Limit of Normal, 33 U/L

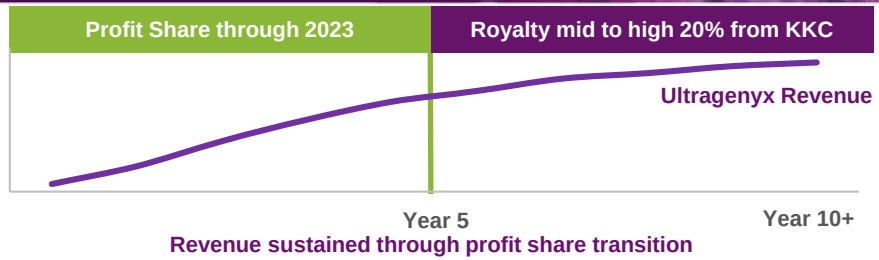
– – – Male ALT Upper Limit of Normal, 40 U/L

Key Licenses & Intellectual Property

Product	License	US Intellectual Property Rights/Royalties
CRYSVITA® (XLH, TIO)	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 (2020-2029)[1] ▪ Use of triheptanoin for treatment of LC-FAOD (2020) ▪ 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
DTX201 (Hemophilia A)	Sub-License from REGENXBIO of UPENN IP	▪ Hu37 Capsid (2024) ▪ Recombinant vectors comprising codon-optimized Factor VIII gene (Pending; 2037) ▪ Low to mid single-digit royalty

[1]Includes projected U.S. patent term extension

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



	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.

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 (Pending)

*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

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

Ultrapure Dojolvi (Pending) — 2034

*Includes projected U.S. PTE award