



Corporate Presentation

June 2020

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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, 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, 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.

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Positioned for Significant Value Growth



Strong Revenue Drivers

- Exceptional Crysvita launch continues
- Growth potential with Mepsevii and UX007

Diverse Portfolio

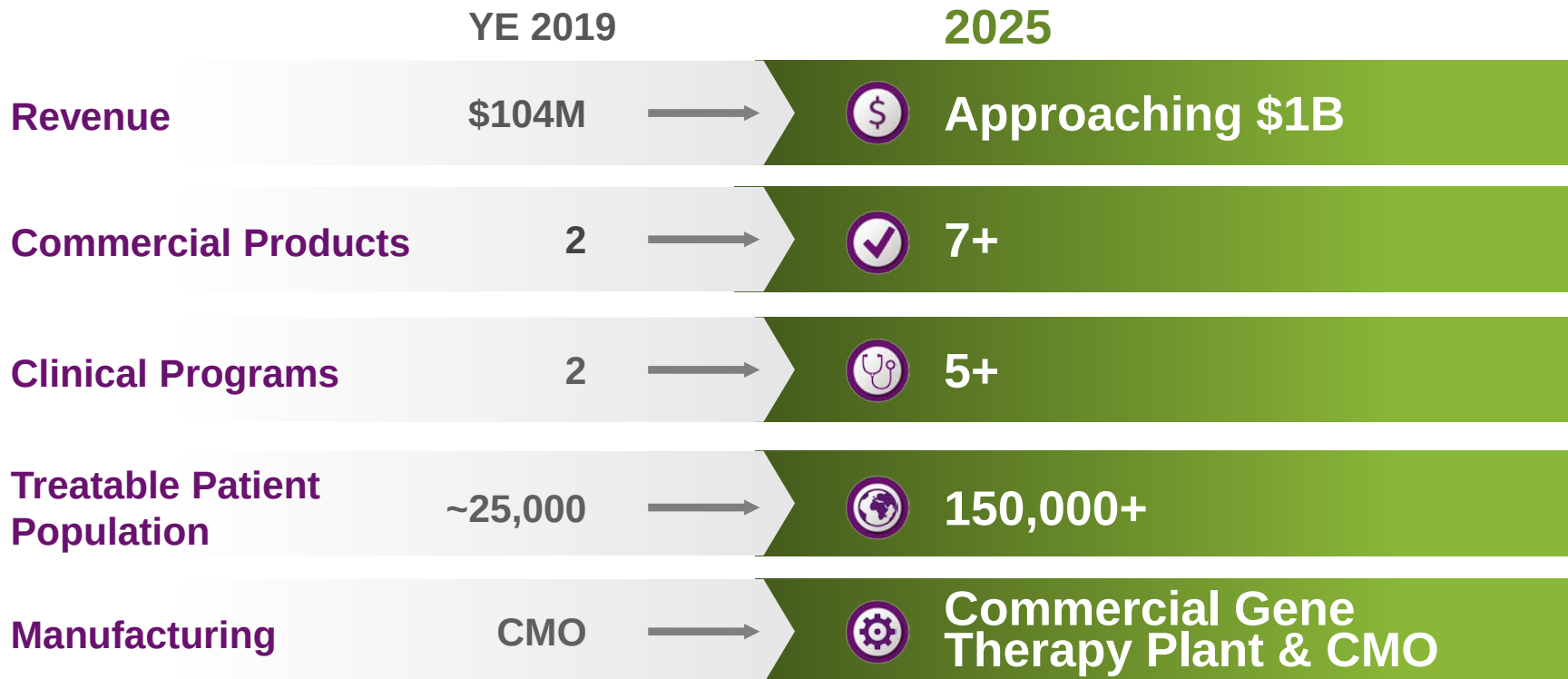
- Broad clinical and preclinical pipeline
- Gene therapy and mRNA platforms

Financial Strength

- \$705M cash and investments¹ at end of 1Q20
- Net burn planned to decrease in 2020

1: Excludes \$125M upfront license payment from Daiichi Sankyo, received April 2020

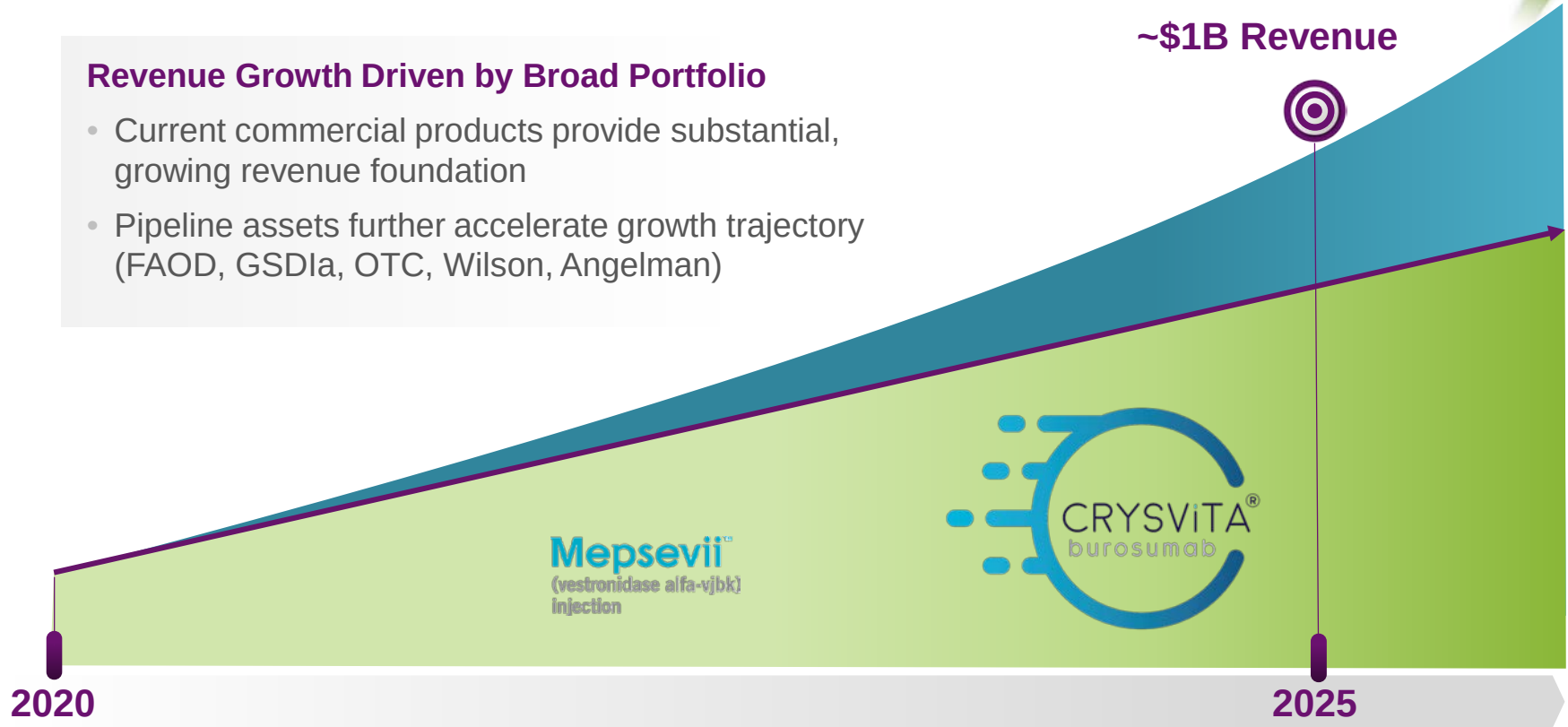
Building an Exceptional Rare Disease Company



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 (FAOD, GSDIa, OTC, Wilson, Angelman)



Strong Crysvita Performance and Solid Financial Base Drive Future Growth

2019 Revenue	
Ultragenyx Crysvita Revenue	\$87.3M
North America Profit Share	74.9M
LatAm Product Sales	4.3M
EU Royalty Revenue	8.1M
Total Company Revenue	\$103.7M

2020 Crysvita Revenue Guidance ¹	
Crysvita in Ultragenyx Regions <i>Adjusted YoY Growth</i> ²	\$125M to \$140M 58% to 77%

1: Crysvita Revenue guidance is for Ultragenyx regions, which excludes non-cash royalty revenue in EU

2: Excludes EU royalty revenue in 2019 and non-cash EU royalty revenue in 2020

Strong Capital Position Supported by Financial Discipline and New Partnerships

- Cash balance³ as of 1Q20: \$705.0 million
 - Excludes \$125.0 million upfront license payment from the Daiichi Sankyo manufacturing partnership which was received in April 2020
- 20%+ reduction in net cash burn⁴ in 2020
- Cash runway into at least mid-2023⁵

3: Cash, cash equivalents, and available-for-sale investments as of March 31, 2020

4: Net cash used in operations plus capital expenditures

5: Based on current business, excluding potential GeneTx option exercise

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-rj) [k] injection	Enzyme Replacement	MPS 7						~200
UX007	Substrate Replacement	LC-FAOD						~8,000 - 14,000
DTX301	AAV8-OTC Gene Transfer	OTC						~10,000
DTX401	AAV8-G6Pase Gene Transfer	GSD1a						~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

* Crysvita is approved in the U.S., Canada, EU, and Brazil

* Mepsevii is approved in the U.S., EU, and Brazil

** Ultragenyx has an option to acquire GTX-102 from GeneTx

Protein Biologic

Small Molecule

Gene Therapy

ASO / mRNA

Multiple Clinical Catalysts in 2020

			1H20	2H20
Crysvita Anti-FGF23 MAb	TIO	FDA Regulatory Decision (PDUFA June 18)	☒	
UX007 Substrate Replacement	LC-FAOD	FDA Regulatory Decision (PDUFA July 31)		☐
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 (by end of 2020)		☐ ☐
GTX-102* ASO	Angelman Syndrome	IND Submission	☒	
		Phase 1 Initiation	☒	
UX701 Gene Therapy	Wilson Disease	IND Submission		☐
Partnership	GT Manufacturing Technology	Daiichi Sankyo Partnership Announced	☒	

*Ultragenyx has an option to acquire
GeneTx (GTX-102)

Protein Biologic

Small Molecule

Gene Therapy

ASO / mRNA

Potential for Two Commercial Launches in 2020



Tumor-Induced Osteomalacia Indication

- Prescription Drug User Fee Act (PDUFA) date of June 18, 2020
- ~2,000 – 4,000 patients in developed world

UX007 for LC-FAOD

- PDUFA date of July 31, 2020
- Potential revenue expected to be modest in 2020 and build over time
- ~8,000 – 14,000 patients in developed world

Both programs will leverage existing commercial infrastructure with minor additional expense

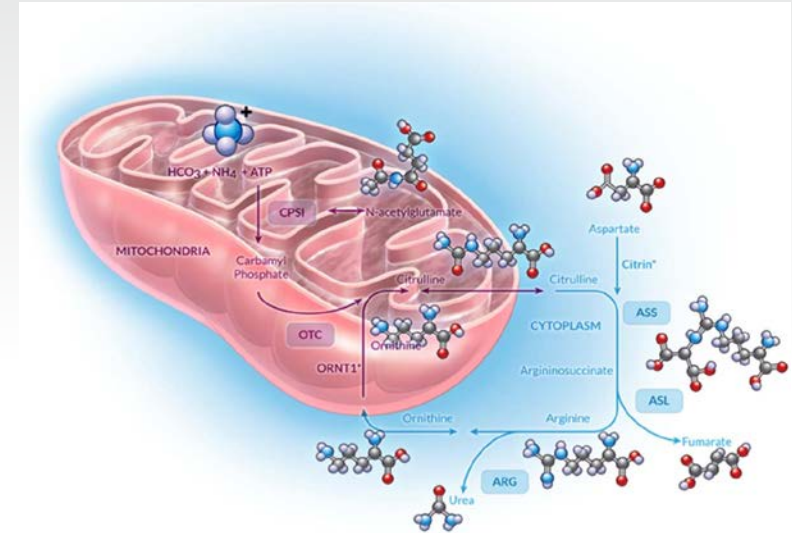


Gene Therapy Programs and Platform

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 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 responder³
	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 responder³
	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 responder³
	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.

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
expected in second half of 2020¹**

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



"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

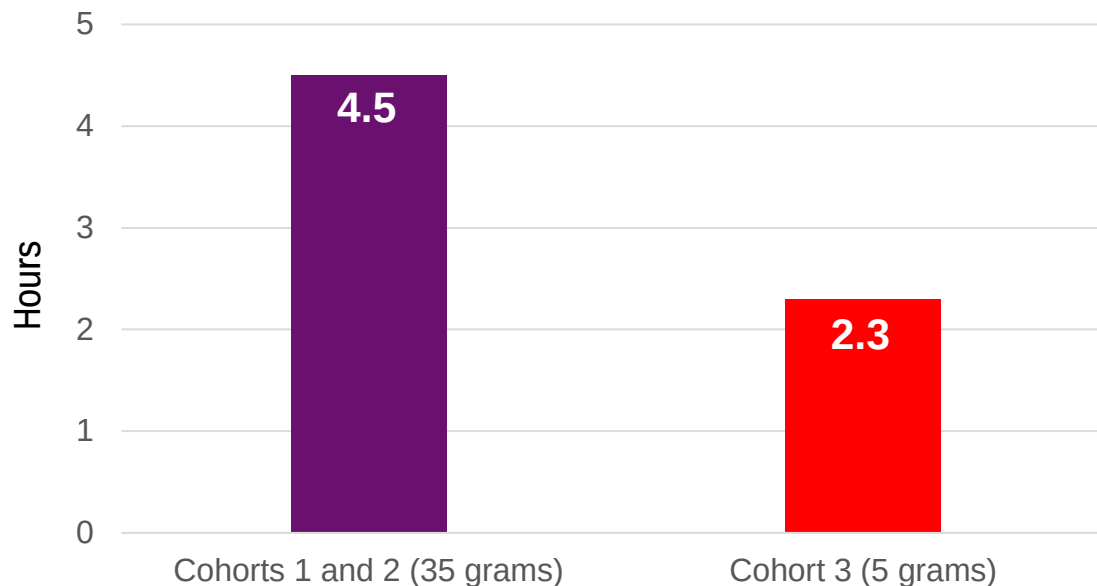
DTX401: Treatment Protocol Changes

Lessons learned from earlier cohorts prompted the following changes to the protocol prior to dosing of patients in Cohort 3 (6×10^{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

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

Mean duration of baseline controlled fasting challenge, hours

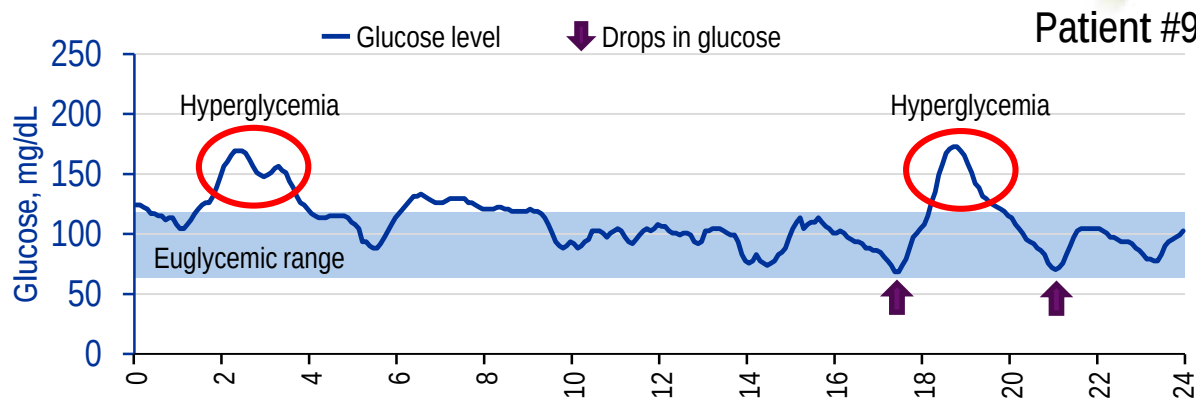


- Reduced cornstarch dose at the start of the controlled fasting challenge in Cohort 3 avoided hyperinsulinemic responses observed in Cohorts 1 and 2

Early Transgene Expression Revealed by CGM Data

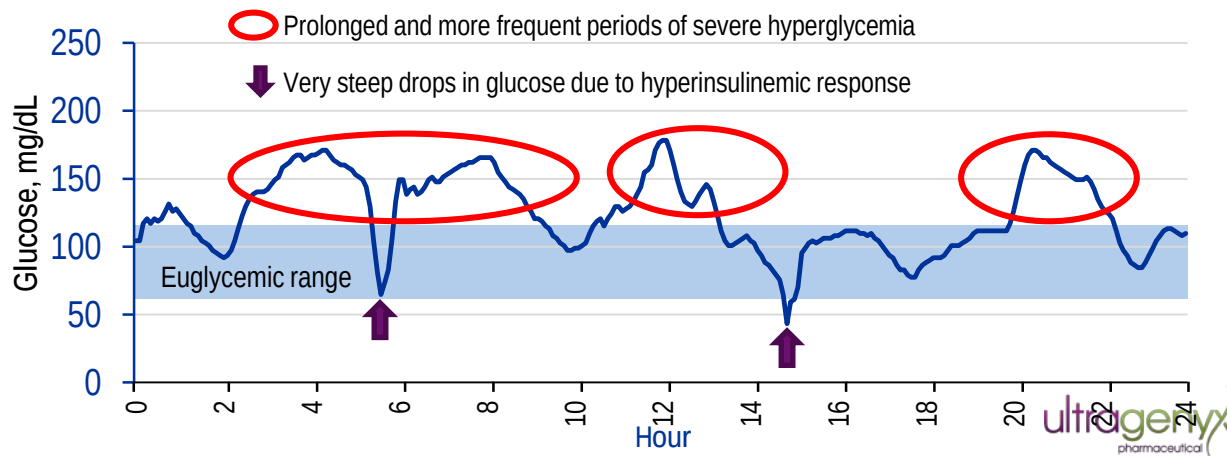
Day -3 Prior to DTX401 Dose

- Periods of hyperglycemia followed by drops in glucose

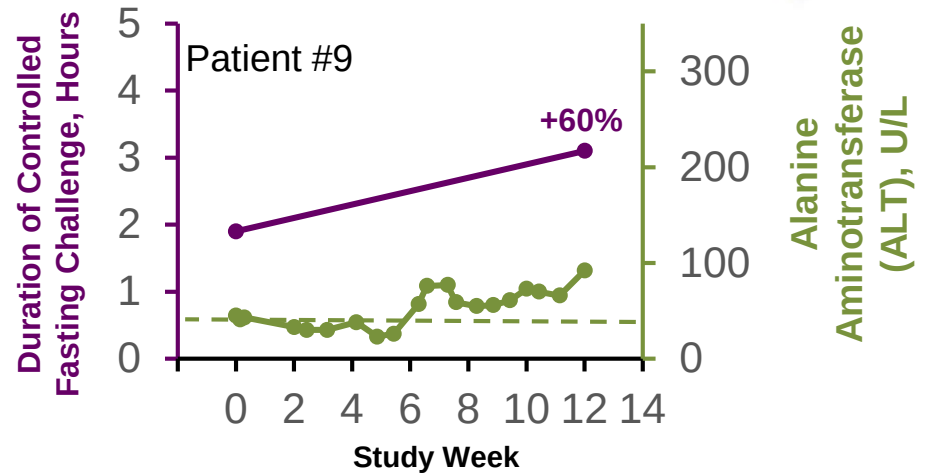
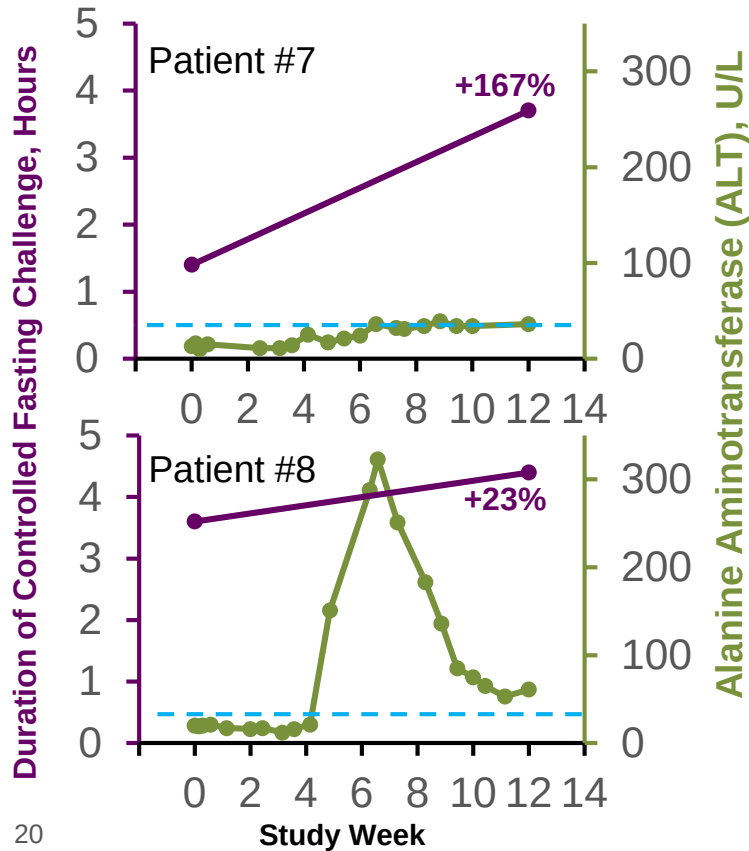


Day +4 After DTX401 Dose

- 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



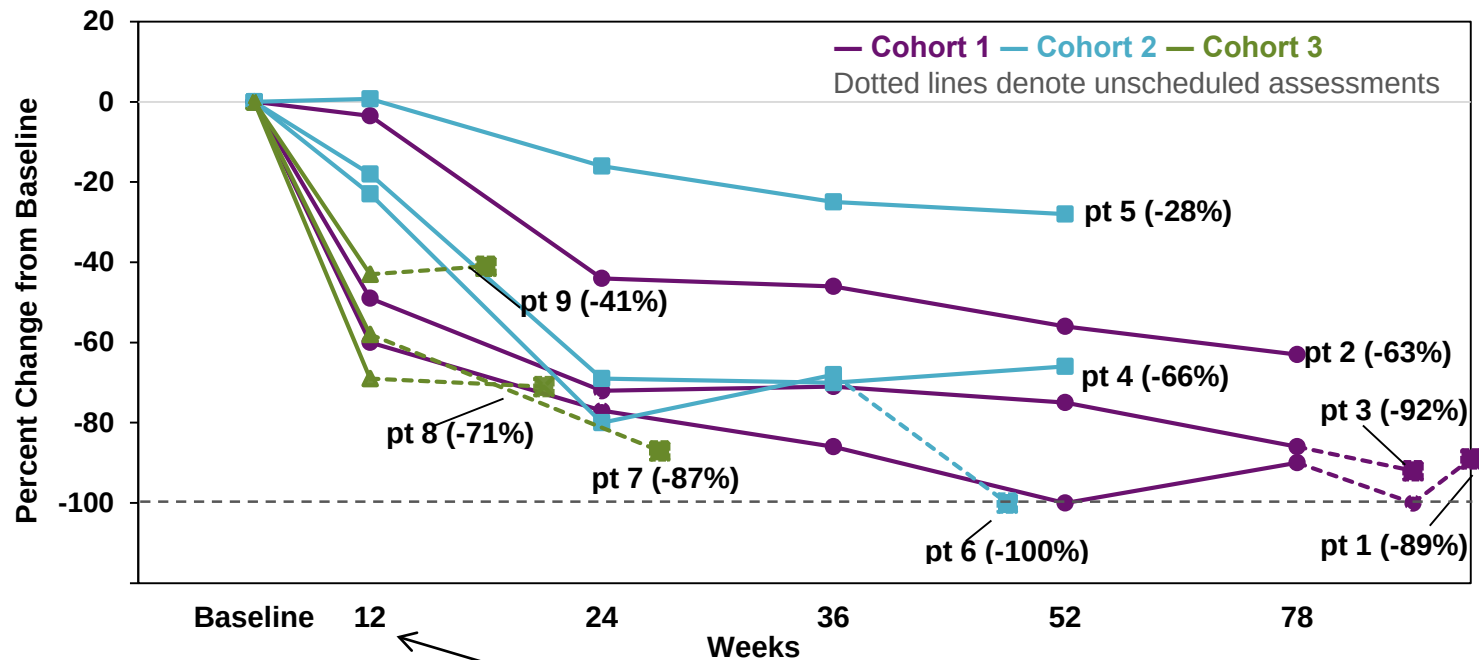
- All three patients received an optimized reactive steroid regimen at approximately week 4
- Patient 8 had an asymptomatic and transient rise in ALT

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

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

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 fast challenge, number of hypoglycemic events through CGM data, all important in evaluating glucose control

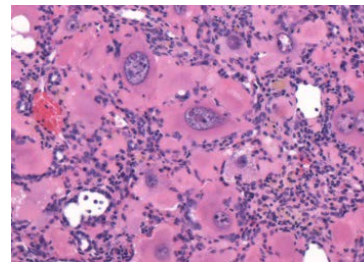
**Longer-term Cohort 3 data
expected in second half of 2020¹**

1: Barring potential delays due to COVID 19

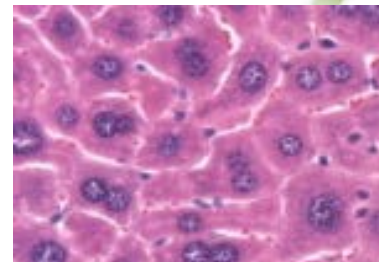
UX701 for Wilson Disease

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**

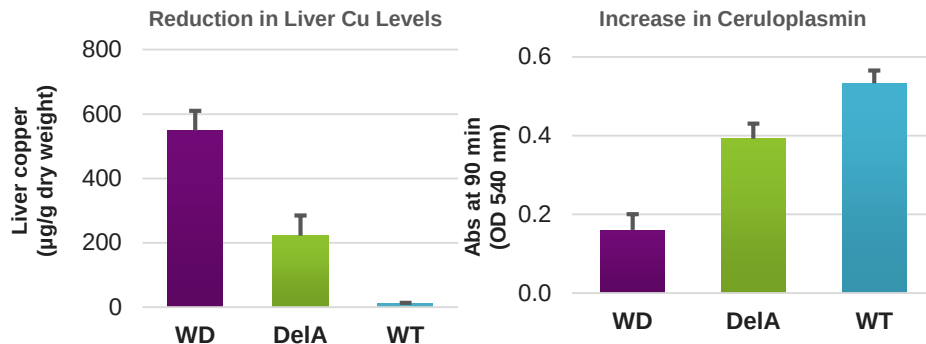


Untreated KO Mice



1x10¹¹ GC Treated Mice

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



Gene Therapy Manufacturing Platforms: Optimized for Scalability and Efficiency

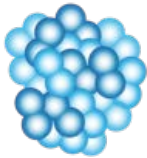
HeLa PCL enables reproducible and consistent
commercial-scale manufacturing at lower COGS



2,000L

HemA in the clinic and
planned for Wilson

HEK293 Suspension/Transfection



4 x 200L

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



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

Gene Therapy Manufacturing Platform: Strategic Partnership with Daiichi Sankyo



- Initial \$200M upfront
 - \$125M cash and \$75M via equity investment
- Additional \$25M in milestones upon completion of tech transfer
- Option to co-develop and co-commercialize Daiichi Sankyo's rare disease programs in this partnership
- Retained the right to use manufacturing technology for current and future indications, including additional partnering

- Non-exclusive license to gene therapy manufacturing patents and know-how
 - Covers both HeLa PCL and HEK293 transient transfection platforms
- Excluded from developing for OTC, GSDIa, Wilson, and certain other indications
- Ultragenyx to provide strategic consultation on gene therapy and rare diseases

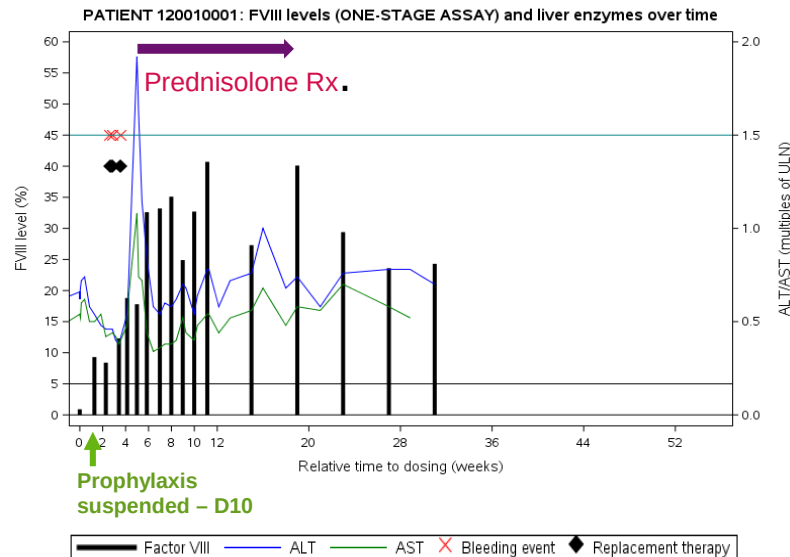
Positive, Clinically Effective HemA Data from the HeLa Platform

Out-licensed program to Bayer validates Ultragenyx HeLa system



- Positive data from lowest two dose cohorts
 - Data from four patients, two at each dose
 - 5e12 and 1e13 GC/kg dose levels of AAVhu37 (DTX201 / BAY 2599023)
- Clinically meaningful Factor VIII levels in one patient in Cohort 1 and both patients in Cohort 2
 - Patient 4 (Cohort 2) bleed-free and replacement therapy-free for 7 months as of data cut-off
- Favorable safety results
 - ALT/AST elevations observed in one patient, managed with tapering course of corticosteroids
- Dose escalation currently ongoing

Patient 4 – Cohort 2 (1e13 GC/kg Dose)





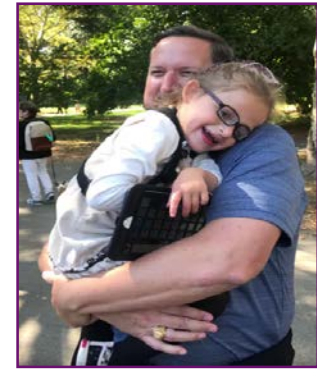
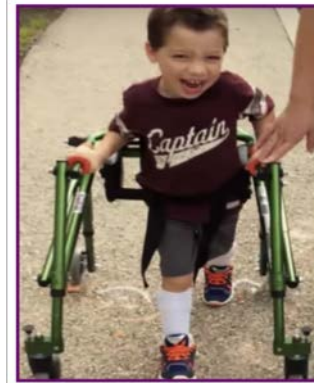
GTX-102 Program for Angelman Syndrome

Partnership to develop GeneTx's
antisense oligonucleotide (ASO)

GTX-102 for Angelman Syndrome

ASO to activate paternal expression of missing enzyme

- **Angelman Syndrome:** Neurogenetic disorder caused by loss of expression of UBE3A gene
- **Key symptoms/prognosis:** Lack of speech, cognitive impairment, motor dysfunction, seizures, sleep disorder
 - Not neurodegenerative, potential for reversal of symptoms
- **No approved treatments**
- **WW prevalence:** ~60,000
- **Partnership:** Ultragenyx has option to acquire GeneTx after Phase 1/2 completion
- **Phase 1/2 Study underway:** First in human, intrathecal intra-patient dose escalating, open-label study
 - Initial data anticipated first half of 2021

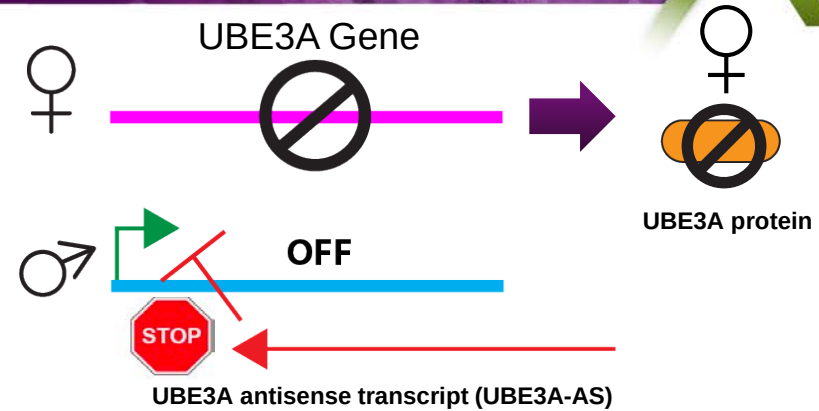


GTX-102 for Angelman

ASO designed to activate the paternal gene

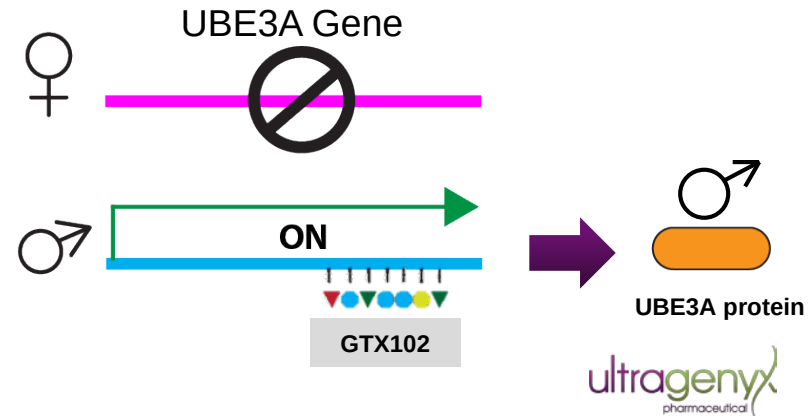
Before Tx with GTX-102

- Deletion or mutation preventing maternal gene expression
- Loss of expression of UBE3A gene
- Father's copy silent (not expressed)



Post Tx with GTX-102

ASO activates the normally silenced paternal UBE3A gene to make UBE3A protein from the father's copy of the gene

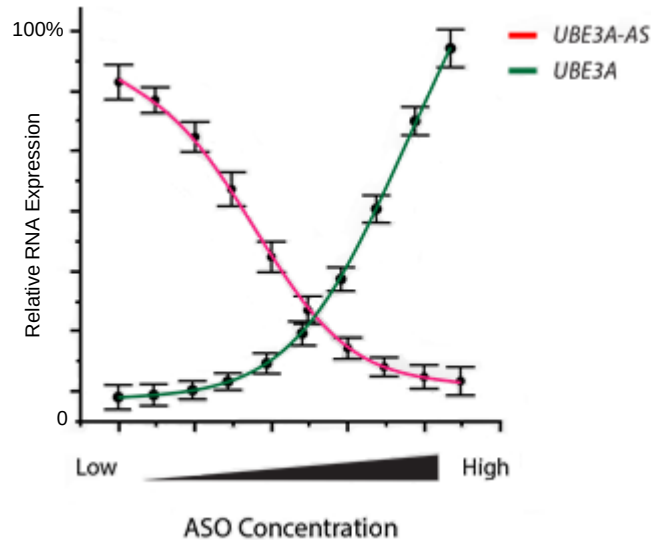


Preclinical Proof of Concept: Specific ASO Discovered with Potent Impact on Releasing Paternal Gene Expression

Human Neuronal Stem Cells

UBE3A-AS knockdown by nearly 100% in human AS neurons after treatment with GTX-102 in vitro. Direct correlation with UBE3A RNA supporting robust reactivation of the paternal UBE3A gene.

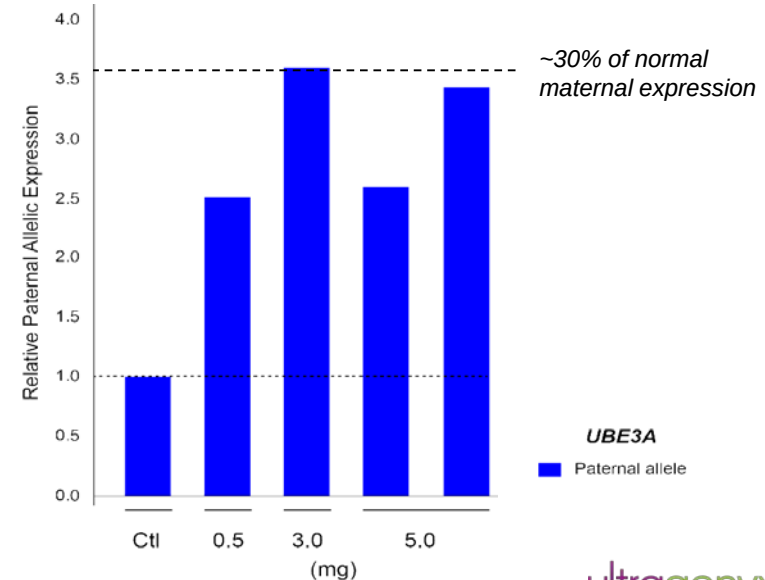
Relative Expression of RNA after GTX-102 Exposure in Cultured AS Neurons



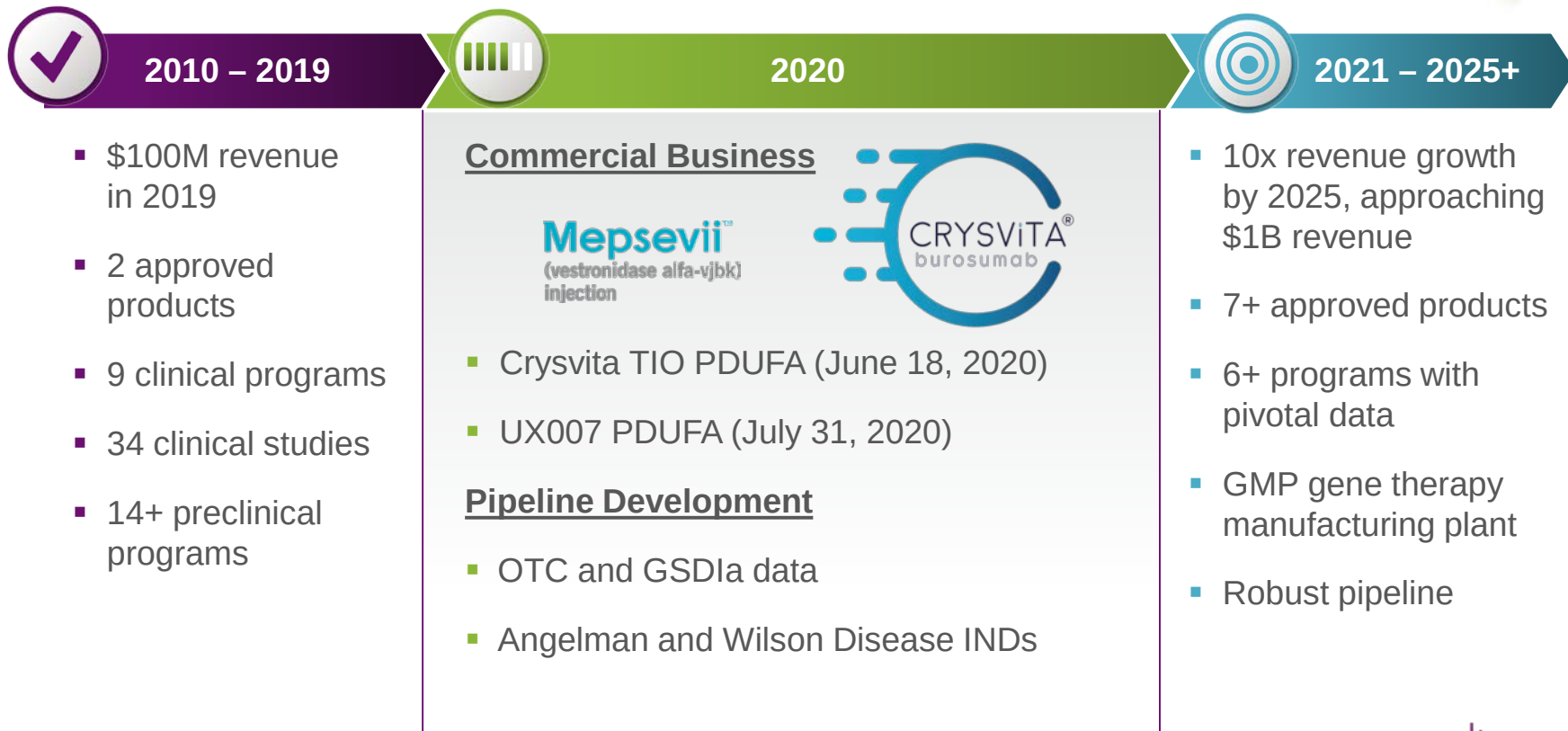
Non-Human Primates

Single-dose of GTX-102 in wild type monkeys demonstrates substantially increased paternal UBE3A gene expression in key brain region. Additional NHP data show broad brain distribution of antisense knockdown.

Relative Increase of Paternal UBE3A Expression in Motor Cortex after Single Dose of GTX-102



Building a Diversified Commercial Rare Disease Company





Appendix

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)
UX007 (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

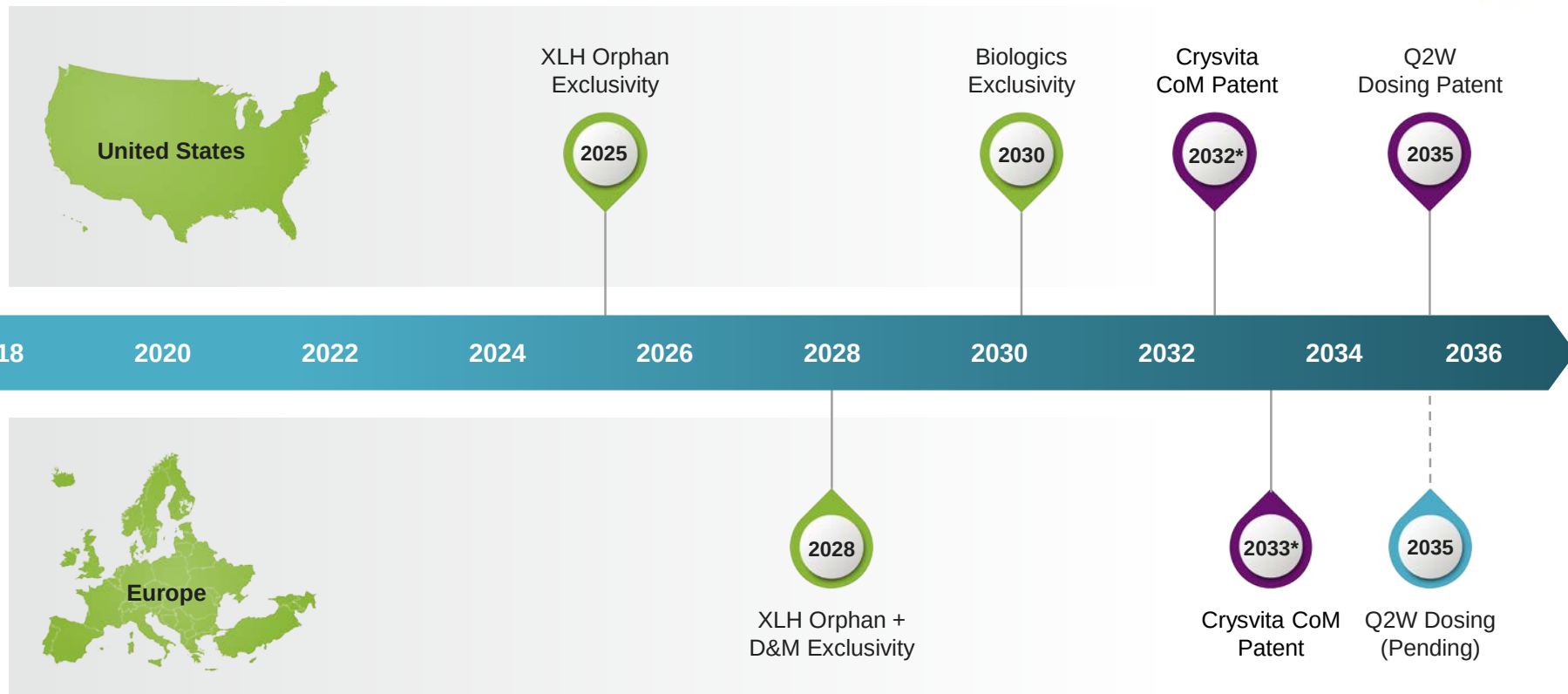
Revenue to Ultragenyx Maintained After Transition from Profit Share to Royalty in U.S.



	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

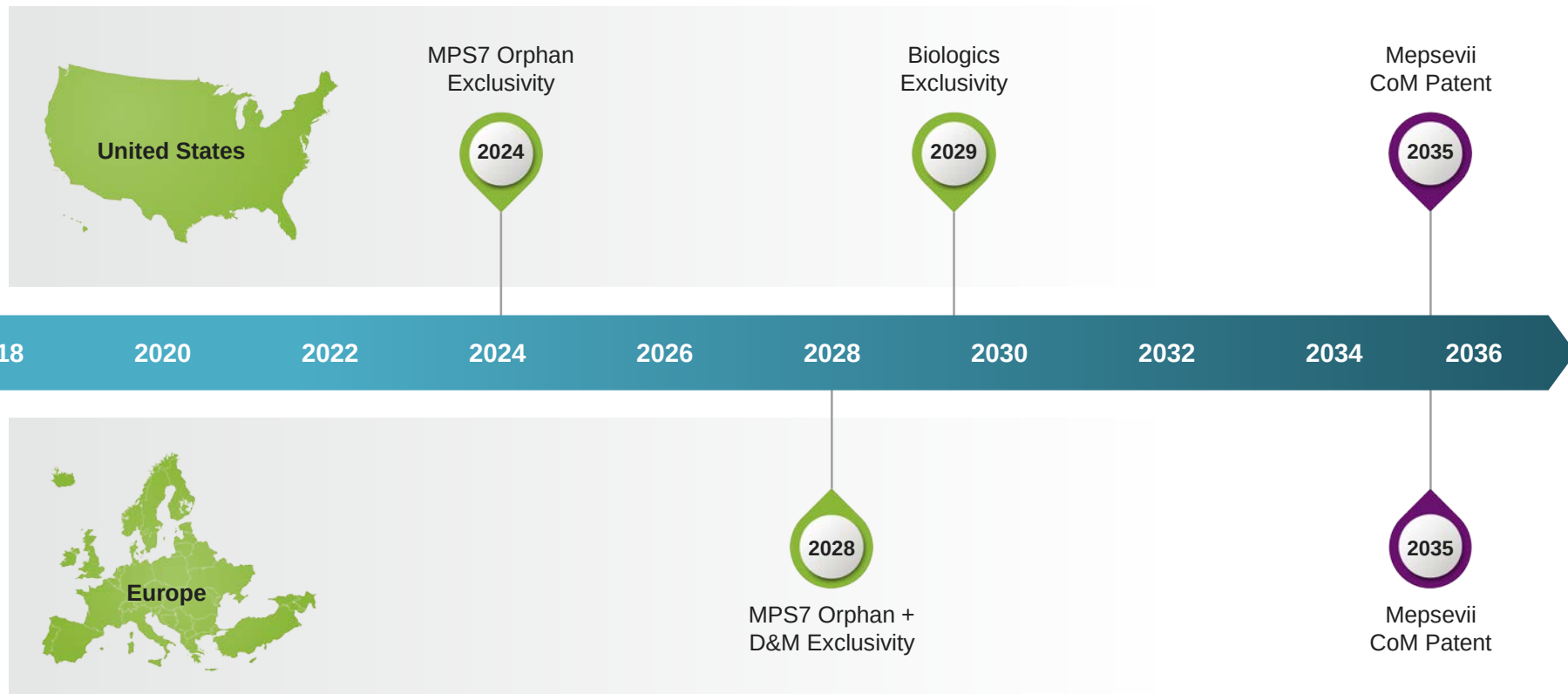
1: Beginning January 1, 2020, the company no longer receives cash payments from the EU territory royalty until the respective threshold amount is met pursuant to the Royalty Pharma transaction. The company remains, however, contractually entitled to the royalties from KKC and will continue recognizing 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



Mepsevii™ Exclusivity Summary

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



UX007 Exclusivity Summary

