Corporate Presentation

May 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 the company's business and operating results, smaller than anticipated market opportunities for our products and product candidates, our evolving commercial infrastructure, uncertainties related to insurance coverage and reimbursement approval for our products, manufacturing risks, the uncertainties inherent in the clinical drug development process, such as the regulatory approval process, the timing of our regulatory filings 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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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 investments at end of 1Q20 ▪ Net burn planned to decrease in 2020

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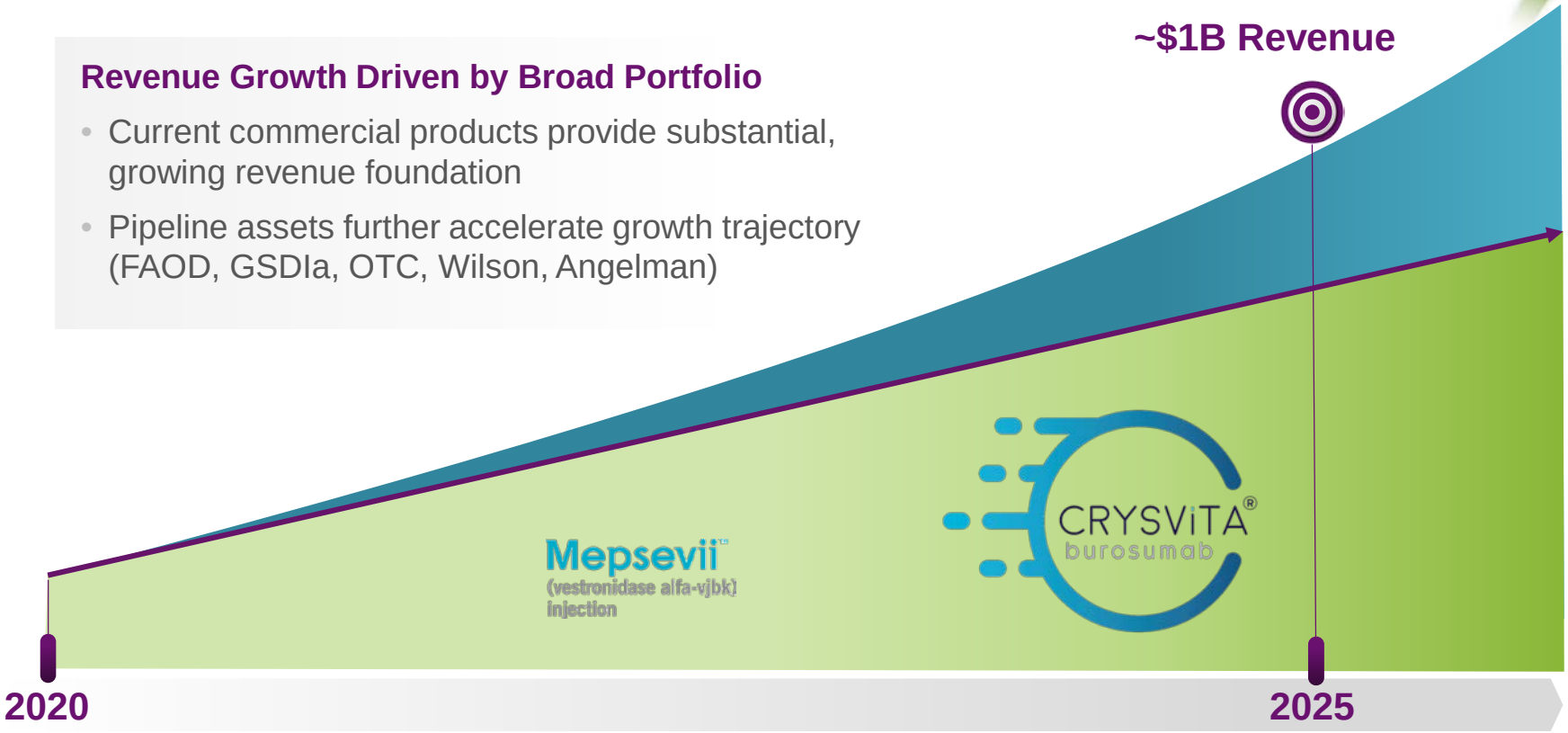
Building an Exceptional Rare Disease Company

	YE 2019	2025
Revenue[1]	$103.7M	Approaching $1B
Commercial Products	2	7+
Clinical Programs	2	5+
Treatable Patient Population	~25,000	150,000+
Manufacturing	CMO	Commercial Gene Therapy Plant & CMO

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	$125M to $140M
Adjusted YoY Growth[2]	*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 balance[3] 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 burn[4] in 2020
- Cash runway into at least mid-2023[5]

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-vjbk) 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	GSDIa						~6,000
BAYER DTX201	AAV-FVIII Gene Transfer	Hemophilia A						~144,000
UX701	AAV-ATP7B Gene Transfer	Wilson						~50,000
genetx 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
** Ultragenyx has an option to acquire GTX-102 from GeneTx

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Multiple Clinical Catalysts in 2020

Two Potential Approvals and Two Key Data Readouts

			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	✓	
		Cohort 4 (Prophylactic Steroid) Data		☐
DTX401 AAV8 Gene Therapy	GSDIa	Cohort 3 (Confirmatory) Data	☐	
		Phase 3 Initiation		☐
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

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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

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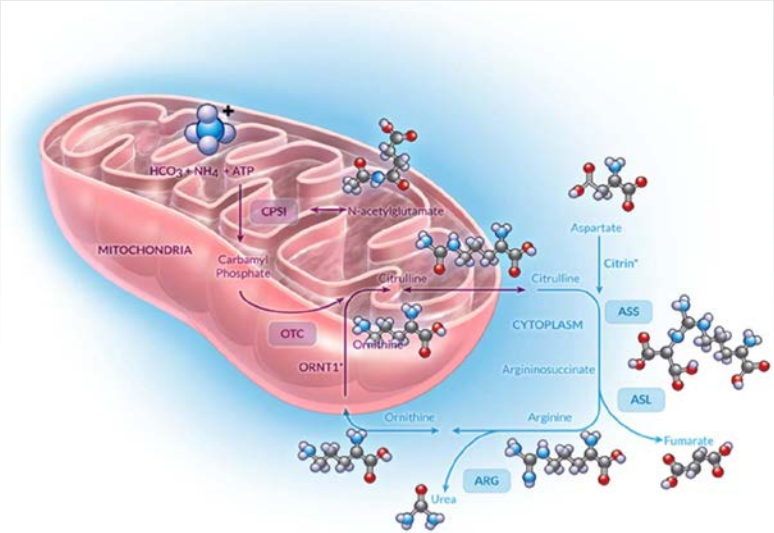
Gene Therapy Programs and Platform

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: Up to Six of Nine Patients Responding Including 3 Female Responders

- **Cohort 3**: **Responses from all three patients**
  - Patient 7: Complete responder (off NH3 scavenger drugs and diet)
  - Patient 8: Responder (not yet tapered medication or diet, still on steroids)
  - Patient 9: Potential responder (requires more follow-up past steroid treatment period)
- **Cohort 2, Patient 6: Additional new female responder**
  - Response began at Week 52 and was confirmed at Week 78
  - Started to taper alternate pathway medications and liberalize protein-restricted diet
- To date, three complete responders off all NH3 scavenger medications and diet
  - Sustained significant improvements in ureagenesis
  - Clinical and metabolically stable after discontinuing alternate medications and liberalizing protein-restricted diet

DTX301: Responses Observed in All Dose Cohorts

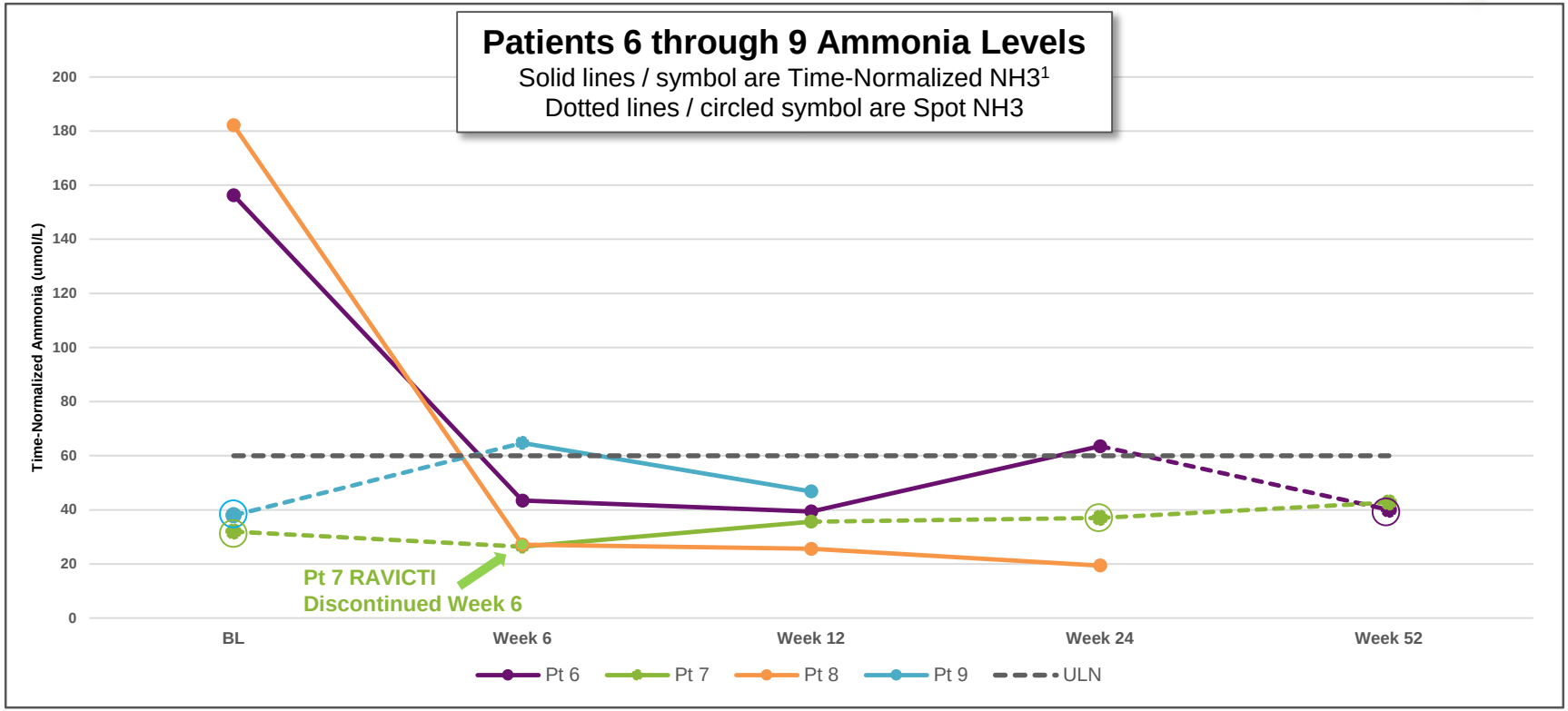
Up to 3 Responders at Cohort 3 Dose

Cohort / Dose (GC/kg)	Patient / Follow-Up Duration	Gender	% 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 (2e12 dose)	Patient 1 (Week 104)	M	**+81% (67% → 121%)**	Normal levels maintained	**Off medications Liberalized diet**	**Complete responder[3]**
	Patient 2 (Week 104)	F	+6% (52% → 55%)	**92% decrease (146 → 11)**	No change	No response (evaluating ammonia response)
	Patient 3 (Week 104)	M	**+81% (48% → 87%)**	Normal levels maintained	No change	No response (evaluating late ureagenesis response)
Cohort 2 (6e12 dose)	Patient 4 (Week 78)	M	**+79% (66% → 118%)**	Normal levels maintained	**Off medications Liberalized diet**	**Complete responder**
	Patient 5 (Week 78)	F	-38% (19% → 12%)	Normal levels maintained	No change	No response
	Patient 6 (Week 78)	F	**+218% (20% → 64%)**	**74% decrease (156 → 40)**	**Tapering medication Liberalizing diet**	**Responder (new)**
Cohort 3 (1e13 dose)	Patient 7 (Week 52)	F	**+79% (24% → 64% & 44%)**	Normal levels maintained	**Off medications Liberalized diet**	**Complete responder**
	Patient 8 (Week 24)	F	?%[2] (66% → 25%)	**90% decrease (184 → 19)**	No change yet	**Responder** (strong consistent ammonia reduction; clinical benefit noted; still on steroids)
	Patient 9 (Week 12)	M	**+123% (25%[4] → 56%)**	Normal levels maintained	No change yet	**Responder (potential)** (still on steroids; more time needed)

[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

Ammonia Levels Significantly Reduced or Controlled in Last 4 Patients

Including in Patient 7 after discontinuation of scavenger therapy at Week 6



1: Time normalized ammonia is defined as ammonia $AUC_{0\text{-}24\ hr}$ / 24hrs

DTX301: Safety Profile

- No infusion-related adverse events and no treatment-related serious adverse events
- All adverse events Grade 1 or 2
- All three patients in Cohort 3 had mild, clinically asymptomatic elevations in ALT levels, consistent with what has been observed in other AAV-based gene therapy programs
  - All have been responding to reactive tapering courses of steroids

DTX301: Next Steps

- Enrolling three additional patients in prophylactic steroid cohort at 1e13 dose
  - Additional cohort was planned prior to Cohort 3 data based on benefit observed in other gene therapy studies and our own lab work
- 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 2020

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

"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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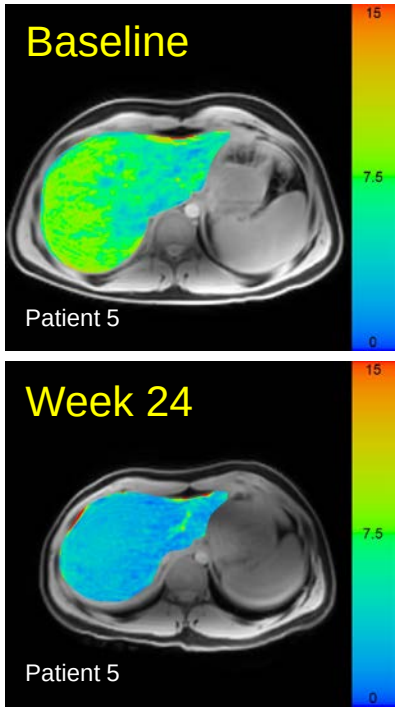
DTX401: Summary of Results from First Two Cohorts

- All six patients responded in first two cohorts
  - Increases in time to hypoglycemia
  - Significant reductions in cornstarch intake
- Cohort 2 showed greater transgene expression with more meaningful improvements across metabolic measures
  - Glycogen storage as measured by liver fat fraction
  - Reductions in lactate during fasting
- Strong safety profile
  - No treatment-related serious adverse events and no infusion-related adverse events

Reduced Glycogen Storage
(MRI Fat Fraction)



DTX401: Improvements and Acceptable Safety

	Cohort 1 at Week 24-52			Cohort 2 at Week 12-24		
Endpoint	Patient 1	Patient 2	Patient 3	Patient 4	Patient 5	Patient 6
Glucose Levels Fasting at week 6	↑	↑	↑	↑↑	↑↑	↑↑
Time to Hypoglycemia	↑103%	↑159%	↑20%	↑15%	↑22%	↑58%
Cornstarch Reduction	↓100%	↓56%	↓79%	↓69%	↓16%	↓80%
Liver Glycogen Week 12 MRI	↓6%	↓20%	↓37%	↓40%	↓39%	↓32%
Lactate During Fasting	+/-	+/-	+/-	↓	↓	↓

Cohort 2 has more consistent impact on other metabolic endpoints

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DTX401: Next Steps

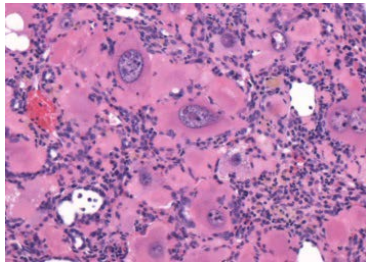
- Expansion cohort initiated to confirm 6e12 GC/kg dose
  - All three patients in expansion cohort have been dosed
  - Modified time to hypoglycemia challenge with reduced cornstarch regimen at baseline and post-treatment
- Data from expansion cohort expected in 1H20

Potential Phase 3 initiation in late 2020

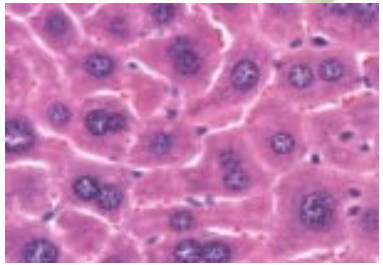
UX701 for Wilson Disease

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**

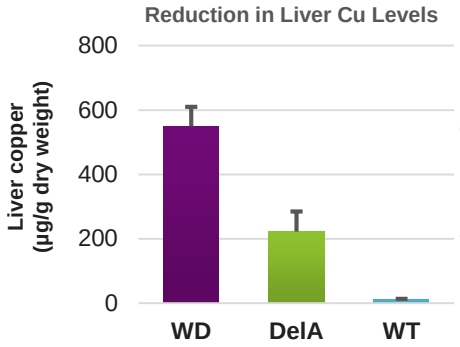


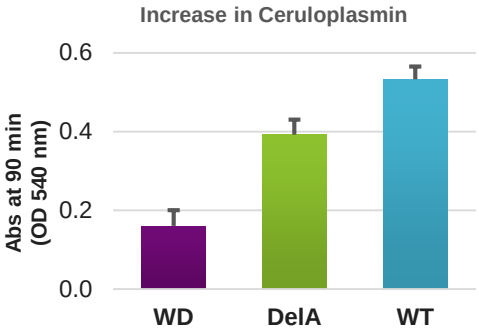
Untreated KO Mice



1x10^{11} GC Treated Mice

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



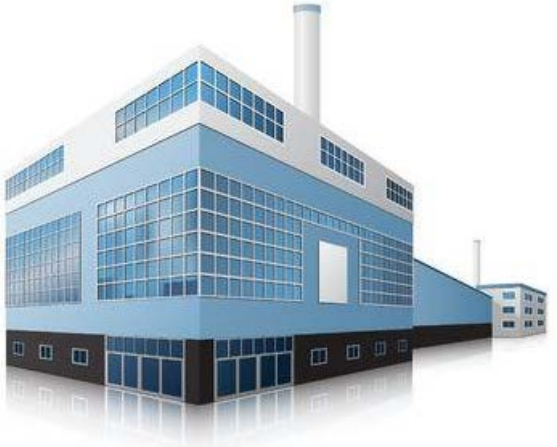


Gene Therapy Manufacturing Platforms: Optimized for Scalability and Efficiency

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

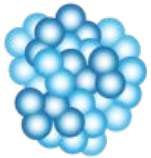
2,000L

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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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

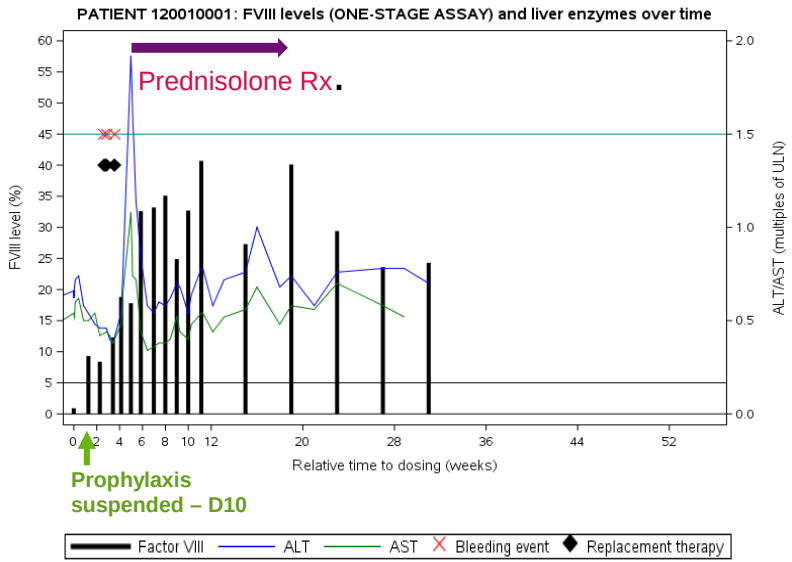
- Non-exclusive license to gene therapy manufacturing patents and know-how
  - Covers both HeLa PCL and HEK293 transient transfection platforms
- Excluded from developing for OTC, GSDIa, Wilson, and certain other indications
- Ultragenyx to provide strategic consultation on gene therapy and rare diseases

Positive, Clinically Effective HemA Data from the HeLa Platform

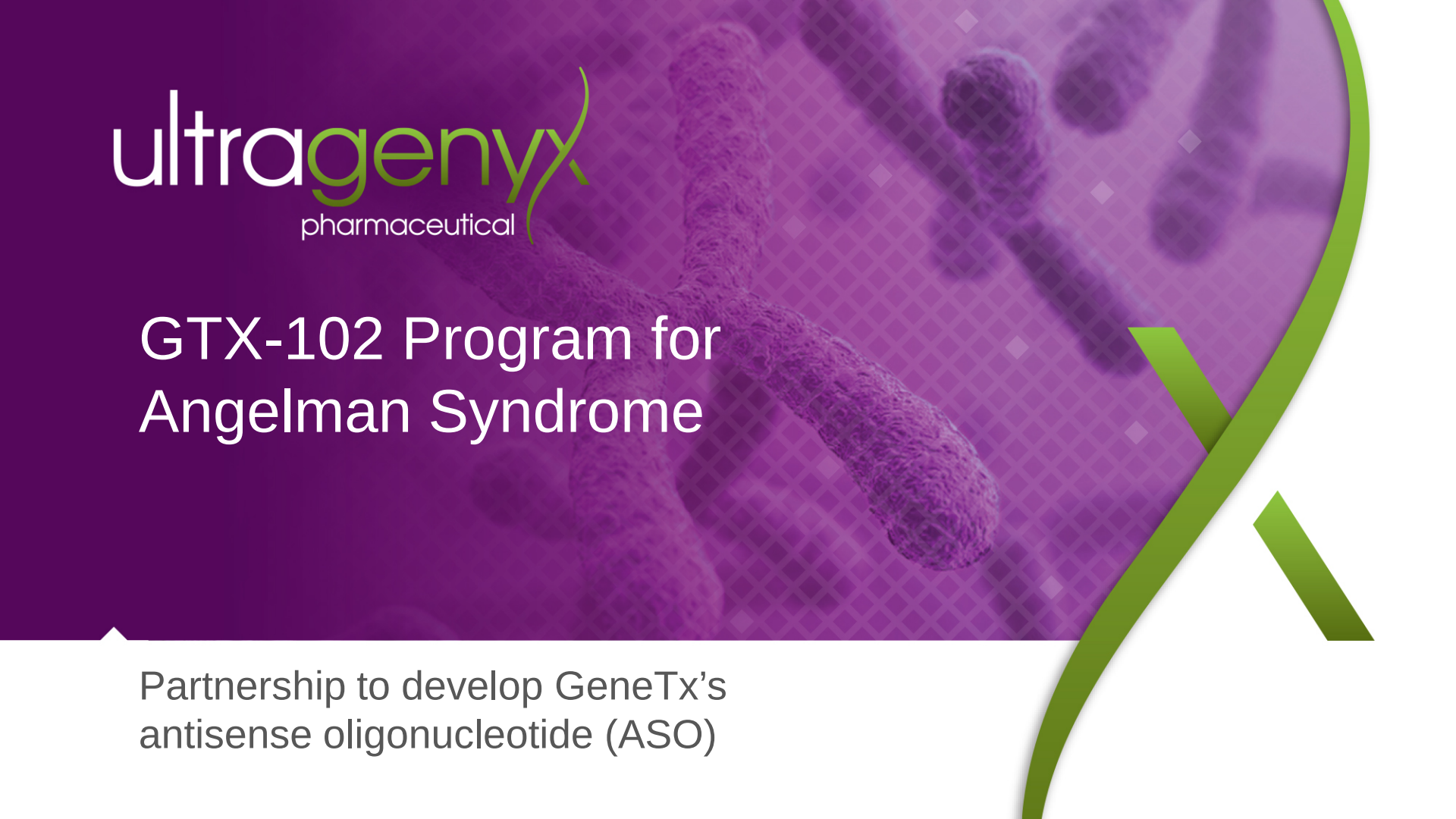
Out-licensed program to Bayer validates Ultragenyx HeLa system

- 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)



Pipe *et al.* 13th Annual Congress of the European Association for Haemophilia and Allied Disorders, January 2020



GTX-102 Program for Angelman Syndrome

Partnership to develop GeneTx's antisense oligonucleotide (ASO)

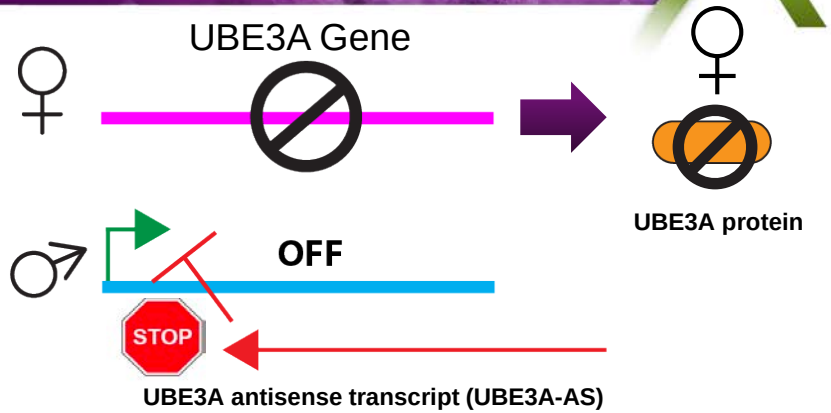
GTX-102 for Angelman Syndrome
ASO to activate paternal expression of missing enzyme

- **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

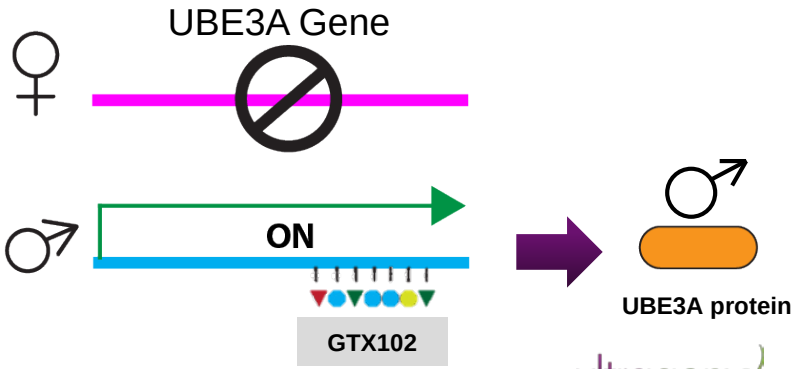
Before Tx with GTX-102

Angelman patients have a deletion or mutation preventing maternal gene expression leading to a loss of expression of UBE3A gene and father's copy is silenced (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

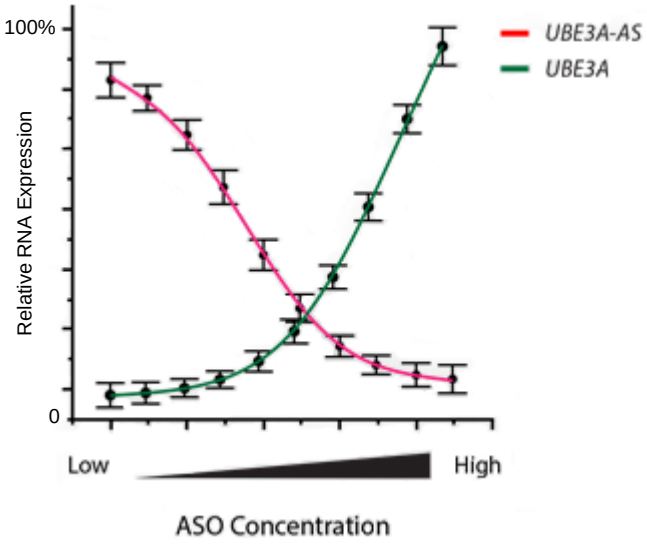


Preclinical Proof of Concept: Specific ASO Discovered with Potent Impact on Releasing Paternal Gene Expression

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.

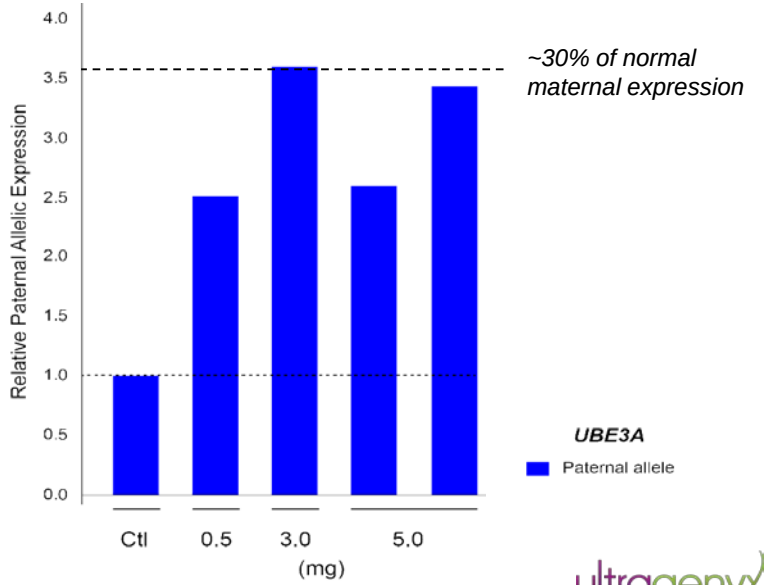
Relative Expression of RNA after GTX-102 Exposure in Cultured AS Neurons



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



Dindot et al., manuscript in preparation

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

- Crysvita TIO PDUFA (June 18, 2020)
- UX007 PDUFA (July 31, 2020)

Pipeline Development

- OTC and GSDIa data
- Angelman and Wilson Disease INDs

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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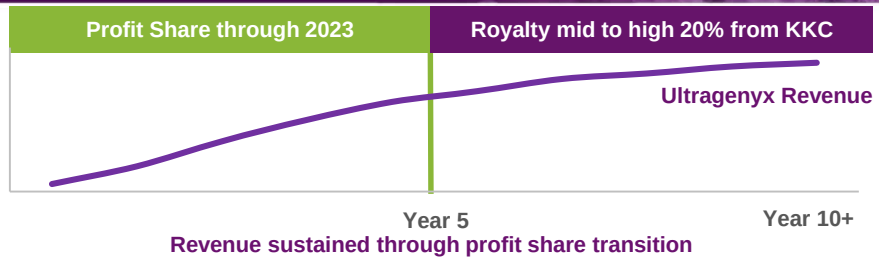
Appendix

Key Licenses & Intellectual Property

Product	License	US Intellectual Property Rights/Royalties
CRYSVITA® (XLH, TIO)	KHK	▪ Anti-FGF23 antibodies and use for treatment of XLH and TIO (2022-2032)[1] ▪ See discussion of KHK 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 (2020-2029/30)[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

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

UX007 Exclusivity Summary

NCE Exclusivity ~2025

LC-FAOD Orphan Exclusivity ~2027

UX007 CoM Patent 2029*

Ultrapure UX007 (Pending) 2034

2018 2020 2022 2024 2026 2028 2030 2032 2034 2036

Est. LC-FAOD Orphan & D&M Exclusivity

Ultrapure UX007 (Pending) 2034

*Includes projected U.S. PTE award