



Corporate Presentation

January 2025

Forward Looking Statements

Cautionary note regarding forward-looking statements: This presentation contains forward-looking statements, including, but not limited to, statements regarding our expectations, estimates, assumptions, and projections regarding our future operating results and financial performance, including our expectations for profitability in 2027, anticipated cost or expense management, plans with respect to commercializing our product and product candidates, 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 or commercializing 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 Crysvida, fluctuations in buying or distribution patterns from distributors and specialty pharmacies, the transition back to Kyowa Kirin of our exclusive rights to promote Crysvida in the United States and Canada and unexpected costs, delays, difficulties or adverse impact to revenue related to such transition, 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, uncertainties in the regulatory approval process and 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, risks related to adverse side effects, the

ability for us to successfully develop our pipeline product candidates, our ability to achieve our projected development goals in the expected time frames, the potential for any license or collaboration agreement to be terminated, 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, actual results or outcomes may materially differ from our current expectations, estimates, assumptions and projections. Given these uncertainties, you should not place undue reliance on these forward-looking statements.

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Who we are

Next generation rare disease company dedicated to the development and delivery of transformative treatments where none exist



Our differentiated approach to rare diseases



Research

Pursue high potential programs

- Potent biology in severe diseases
- Treating underlying cause
- Best modality for each disease



Clinic

Accelerate to drive value

- Adaptive trial designs
- Novel endpoints
- High unmet medical need supporting expedited enrollment



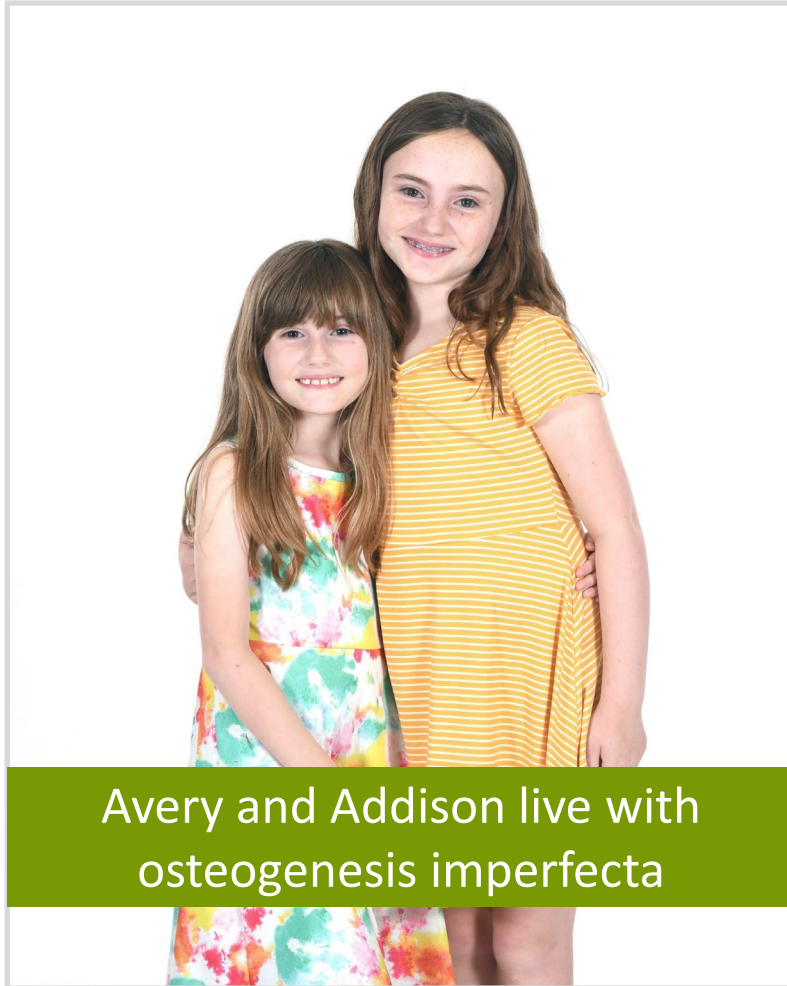
Commercial

Patient-centric approach



- Lean commercial team
- Emphasize patient find/support
- Reduced post-approval R&D costs



Find right opportunities at reasonable cost, develop rapidly with adaptive designs, and commercialize efficiently and effectively

Creating a *successful* and *profitable* rare disease company





4 commercial products

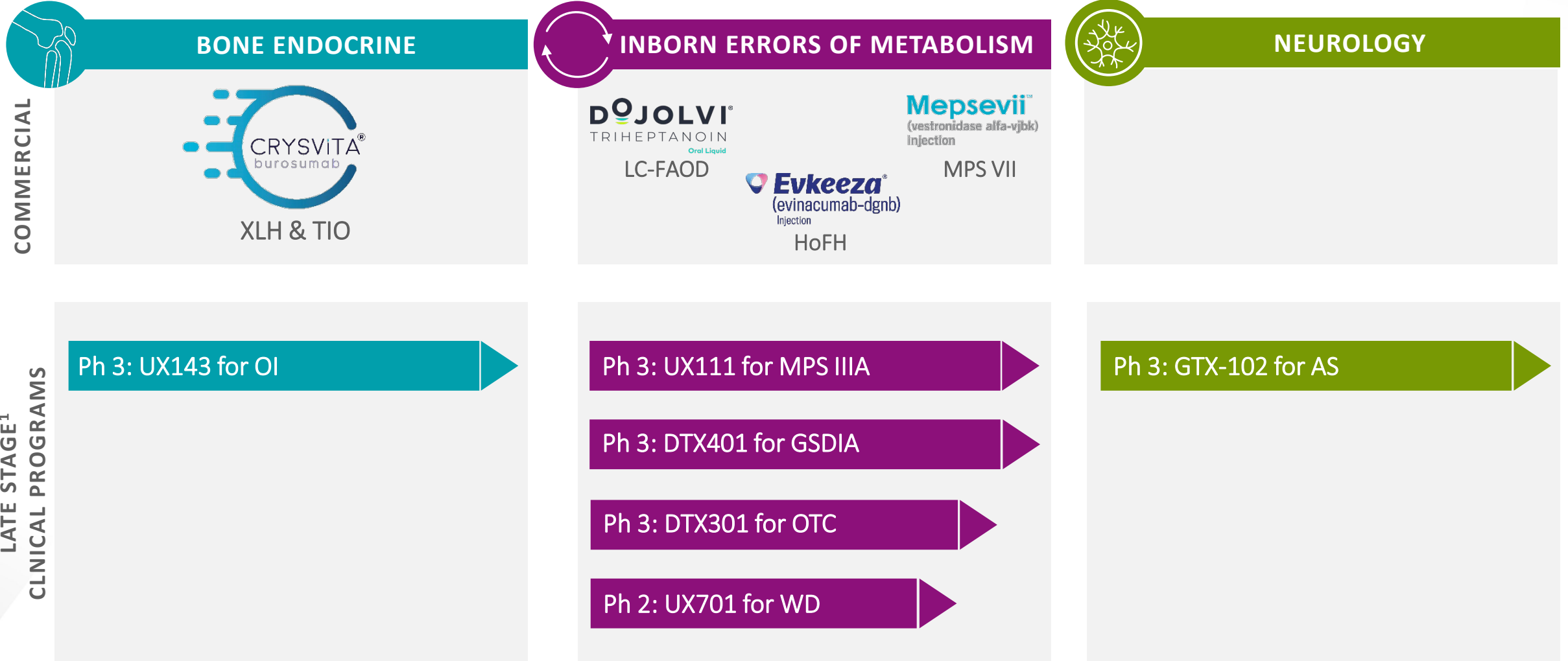
 

Largest clinical pipeline in rare disease

 Phase 2/3 studies →  Near-term approvals

Focused on three therapeutic areas

Late-stage pipeline will leverage successful global commercial organization



1: Clinical pipeline available in Appendix

Drivers of value creation in 2025

1 UX143 for OI
Ph3 data readout



Amber and her daughter live with osteogenesis imperfecta

2 GTX-102 for AS
Ph3 enrollment completion



Mason lives with Angelman syndrome

3 Revenue expansion &
near-term launches



Aly lives with XLH

UX143 for osteogenesis imperfecta (OI)

Fully human monoclonal antibody; Ph3 data readout expected in 2025

Osteogenesis Imperfecta: Collagen defect leading to brittle bone, low bone mineral density (BMD), and frequent fractures

- Associated with pain and decreased mobility, often with ambulatory aid
- Prevalence*: ~60,000 (Types I/III/IV)
- Treatment: No globally approved therapies; bisphosphonates used off label

**Prevalence in commercially accessible geographies*

UX143: Fully human mAb to inhibit sclerostin and turn on bone production via normal pathway

- Phase 3 data expected in 2025
- Investing in commercial supply
- Extensive launch expertise in bone/endocrine from Crysvida
- Priority Review Voucher (PRV) eligible



Matthew lives with osteogenesis imperfecta

"I have not yet encountered a patient with a fragility fracture while on setrusumab, and this may result from setrusumab's effects on the skeleton, improving the rate of new bone formation and bone quality."

Gary Gottesman, MD

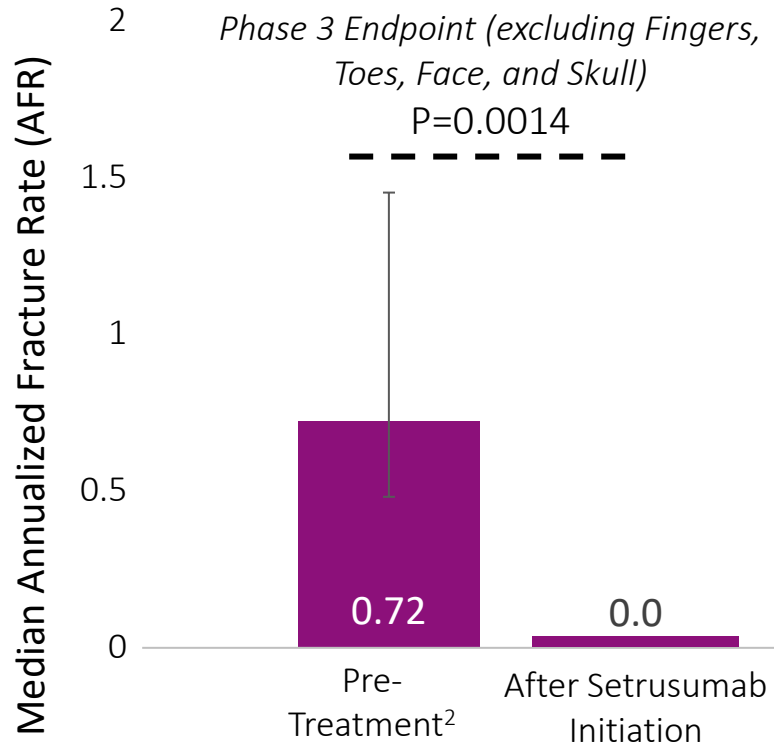
Professor of Pediatrics and Medicine

Washington University School of Medicine

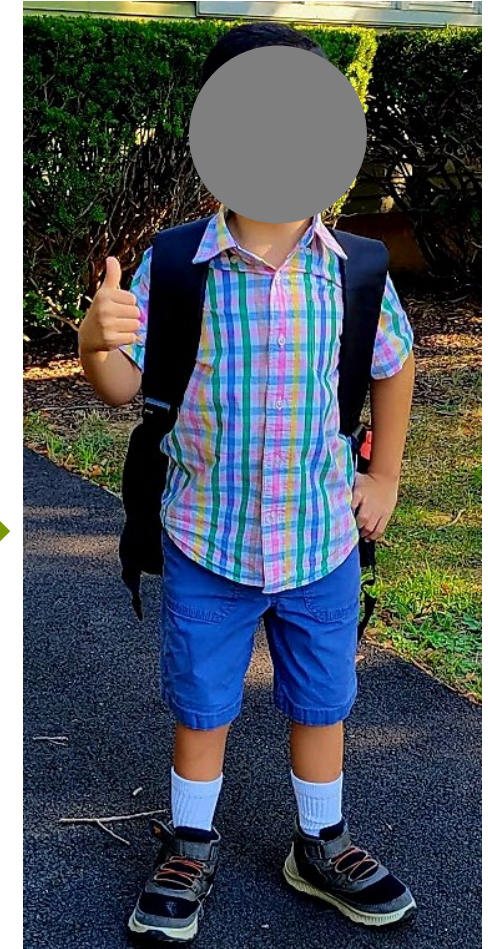
In reference to October 14, 2023 Phase 2 data presentation

UX143 for OI: Updated Phase 2 data showed 67% reduction¹ in annualized fracture rate (AFR) post-treatment

Radiographically Confirmed Fractures¹

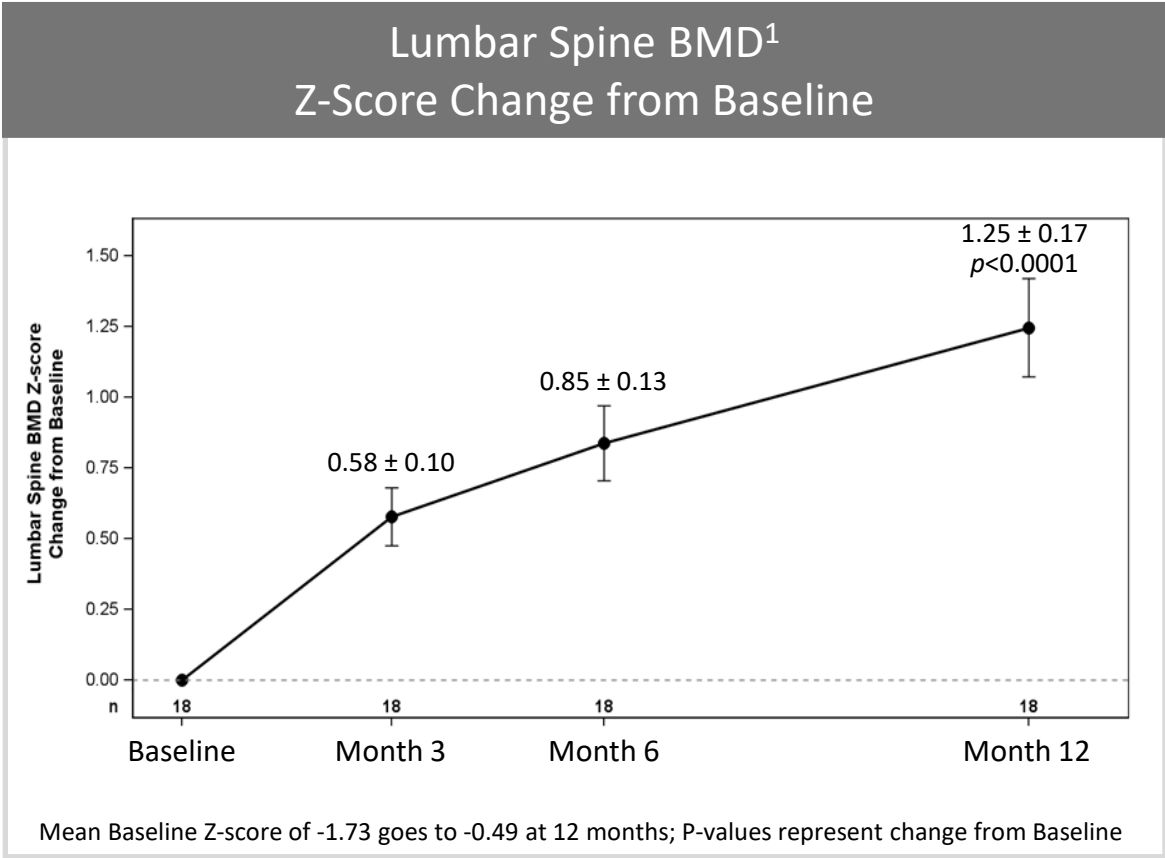
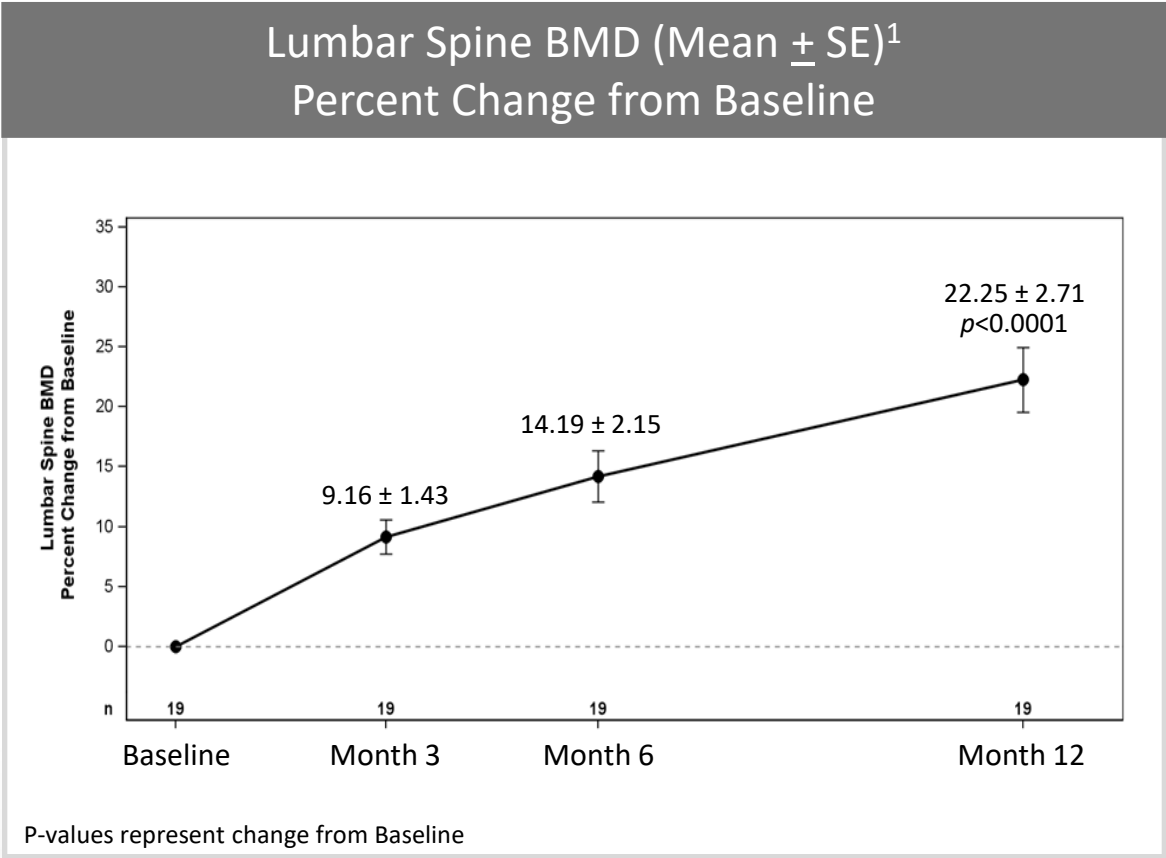


1: Interim data as of May 24, 2024 and includes a mean follow-up of 16 months.
67% reduction = Median(AFR Post-Tx Initiation - Pre-Tx) ÷ Median(AFR Pre-Tx)
2: Pre-Treatment period includes fractures in the two years before screening based on medical record review and patient report, and fractures between screening and first dose



6 y/o male patient with Type IV OI, increased mobility after 17 months on study

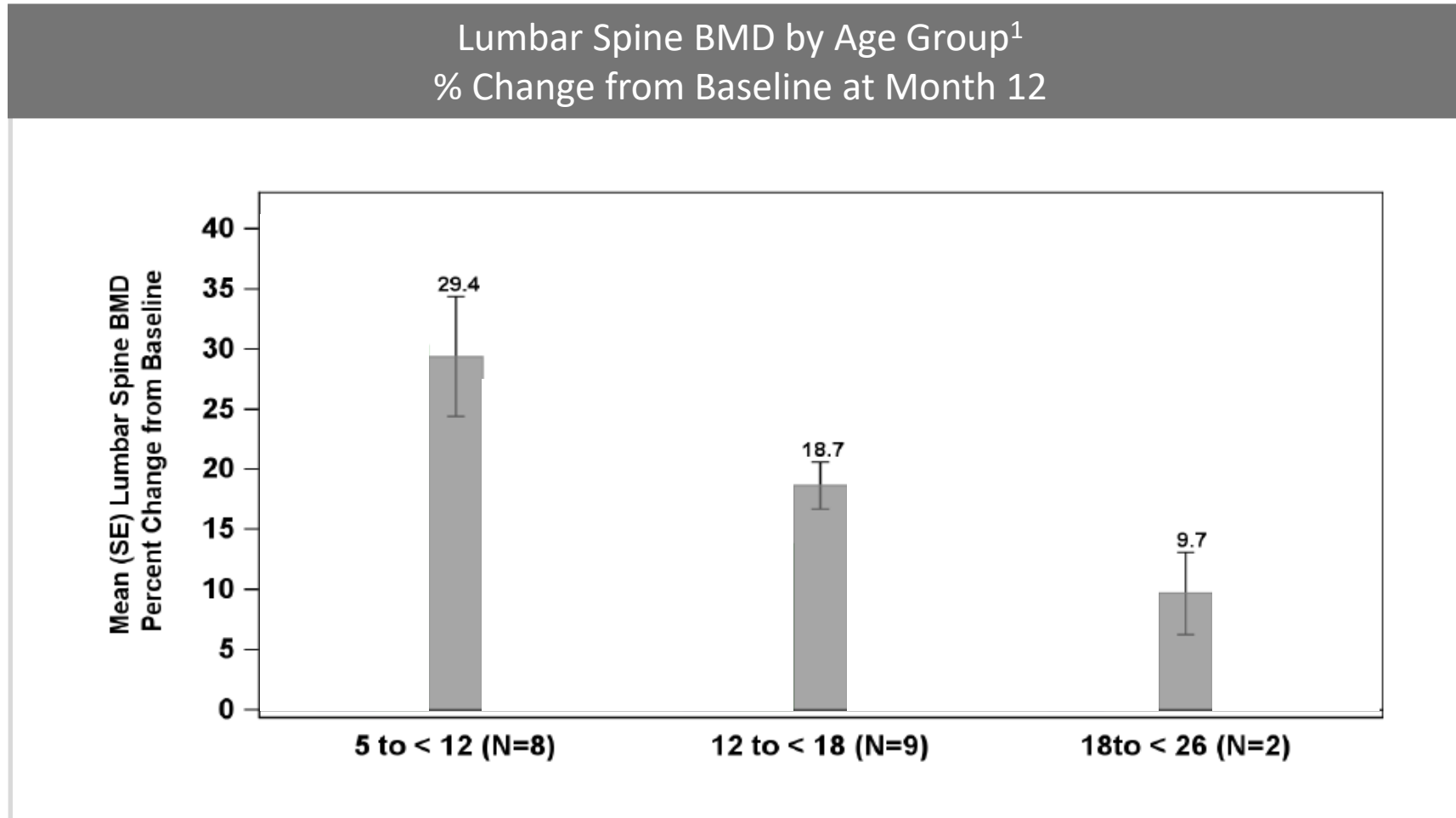
UX143 for OI: Phase 2 data demonstrated increase in lumbar spine BMD and Z-score observed at >14 months



¹ Interim data as of May 24, 2024



UX143 for OI: Younger patients in Phase 2 showed very large BMD improvement at Month 12



¹ Interim data as of May 24, 2024

UX143 for OI: Phase 2 safety data¹ consistent through Month 14



No drug-related hypersensitivity reactions

No treatment-related SAEs

No unexpected adverse events or safety concerns

No patients discontinued treatment for any adverse event

1: As of a May 24, 2024 cutoff

UX143 for (OI): Orbit Phase 2/3 comparison

Orbit Phase 2

Open-label 20 vs 40 mg/kg for pivotal dose selection

- Purpose: Compare 20 and 40 mg/kg doses to determine optimal Ph3 dose in 5-25 y/o patients
- Enrollment: 5-25 y/o; N=24; OI Type 1, 3, 4; into a low or high dose cohort
- Primary Endpoint: Percent change in P1NP¹ at Mo 1
- Phase 2 Results:
 - Significant increases in P1NP in both 20 and 40 mg/kg cohort
 - Increasing BMD and Z-score at >14 Months
 - 67% reduction in median AFR² (p=0.0014)
 - Wilcoxon non-parametric model used to evaluate median change pre- to post-Tx AFR

Orbit Phase 3

Randomized, placebo controlled to lead BLA filing

- Purpose: Replicate strong Ph2 data in a larger, placebo controlled study to support BLA filing
- Enrollment: 5-25 y/o; N=159; OI Type 1, 3, 4; randomized 2:1 UX143 to placebo
- Primary Endpoint: Annualized Fracture Rate²
- Phase 3 Readout:
 - Interim Analysis 2 or Final Analysis
 - Negative Binomial regression model will be used to compare placebo and UX143 AFR

1: Procollagen type I N propeptide (P1NP), a sensitive marker of bone formation

2: Annualize fracture rate (AFR) of radiographically confirmed clinical fractures associated with pain. Phase 3 primary endpoint excludes fingers, toes, face, and skull

GTX-102 for Angelman syndrome (AS)

Antisense oligonucleotide; Phase 3 expected to complete enrollment in 2H-2025

Angelman Syndrome: Loss-of-function of maternal *UBE3A* gene

- Cognitive, communication, motor, behavior, and sleep impairment and seizures
- Requires continuous care
- Prevalence*: ~60,000
- Treatment: No approved therapies

**Prevalence in commercially accessible geographies*



Conner lives with Angelman syndrome

GTX-102: Antisense oligonucleotide (ASO) to activate paternal expression of *UBE3A*

- Phase 3 *Aspire* Study in deletion patients expected to complete enrollment in 2H-2025
- Phase 2/3 *Aurora* study in other genotypes and ages expected to begin in 2025

*“Angelman syndrome affects cognitive and motor function, making walking, communicating, and performing many everyday tasks more difficult...The initiation of the Phase 3 *Aspire* study by Ultragenyx is a significant achievement and something the community should celebrate.”*

Joint statement from **Amanda Moore**, chief executive officer at the Angelman Syndrome Foundation (ASF) and **Ryan Fischer**, chief operating officer at Foundation for Angelman Syndrome Therapeutics (FAST)

GTX-102 for AS: Overview of Phase 1/2 long-term safety and efficacy



Participants have made consistent developmental gains with sustained improvements across multiple symptom domains up to 3 years on therapy



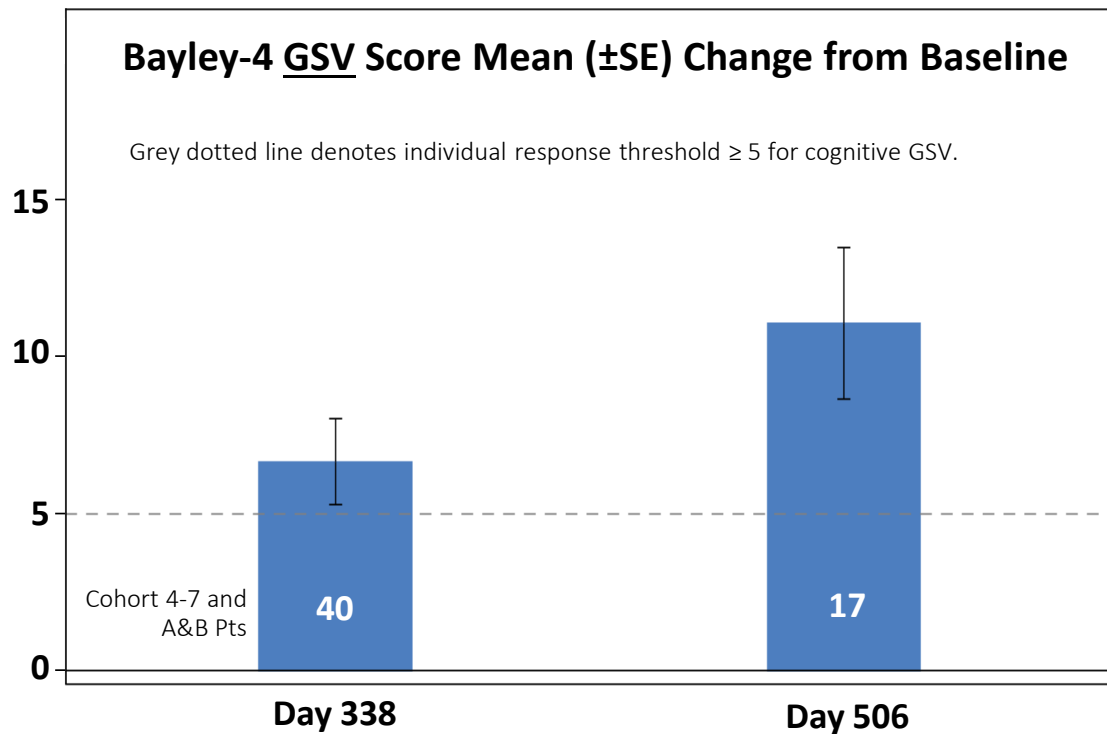
No additional cases of lower extremity weakness; safety profile is understood and remains consistent



Phase 3 study *Aspire* initiated in December 2024

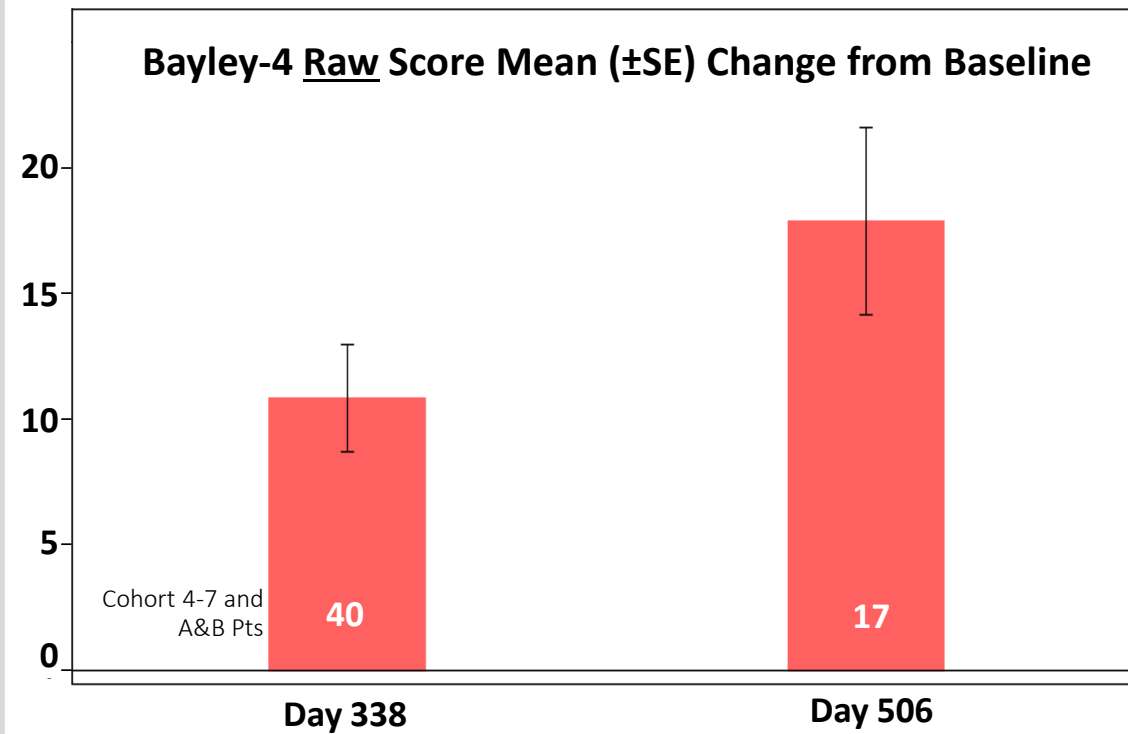
GTX-102 for AS: Cognition by Bayley-4 GSV and raw scores show ample power for Phase 3 trial

Bayley-4 Cognition GSV scores show significant gains at Day 338 that continue through Day 506



GTX-102 Arm Mean (SD)	3x NHS2 Data [§] Mean (SD)	Hypothesized Difference	SD*	Power [^] ($\alpha = 0.045$)
6.7 (8.6)	0.9 (4.8)	5.8	8.6	92.9%

Ph3 Primary Endpoint: Bayley-4 Cognition raw score trend comparable to GSV and is also well powered



GTX-102 Arm Mean (SD)	3x NHS2 Data [§] Mean (SD)	Hypothesized Difference	SD*	Power [^] ($\alpha = 0.045$)
10.9 (13.5)	1.2 (9.5)	9.7	13.5	95.5%

[§]Natural History data: Linking Angelman and Dup15q Data for Expanded Research (LADDER) at Day 365. ^{*}Conservative SD assumption.

[^]N=108 completers out of 120 randomized (1:1) with 10% drop out rate.

Data shared via press release on November 9, 2024

GTX-102 for AS:

Changes in dose administration provided acceptable safety profile

- No unexpected serious adverse events
- Two patients from Expansion Cohorts (N=53; previously disclosed in April 2024) had serious adverse events of transient lower extremity weakness assessed as related to study treatment
 - Both resolved rapidly without sequelae and remain in the study without ongoing safety concerns
- Patients redosed with multiple doses following resolution of lower extremity weakness
 - Five original patients from Cohorts 1-3 (previously disclosed in October 2020) safely re-dosed multiple times and are receiving maintenance treatment without recurrence
 - The Cohort 7 patient (previously disclosed in January 2023) has also re-dosed safely multiple times and is receiving maintenance treatment without recurrence

FDA and other regulators notified of safety events;
no issues raised and no additional actions requested

GTX-102 for AS: Phase 3 development plans

*Aspire: Phase 3 Study*¹

- Randomized, controlled study in deletion patients
- Sample size: ~120 patients; ages 4 to <18 years
- 48-week primary efficacy period
- Primary Endpoint: Bayley-4 Cognition raw score
- Key Secondary: MDRI across cognition, receptive communication, behavior, gross motor, and sleep
- Additional, individual secondary endpoints for domains of communication, behavior, gross motor, and sleep

Aurora: Additional Genotype and Ages Study

- Open label
- Ages <4 and >18 years of age
- Non-deletion types
- Duration, endpoints and other details to be determined with regulatory agencies

Aspire initiated December 2024;
expect *Aurora* to initiate in 2025

1: Based on EOP2 meeting with FDA; disclosed July 17, 2024

UX111 for Sanfilippo syndrome (MPS IIIA) *AAV9 gene therapy; anticipate 2025 Launch*

MPS IIIA: Fatal lysosomal storage disease of CNS

- Early childhood onset
- Rapid neurodegeneration
- Prevalence*: ~3,000 to 5,000
- Treatment: No approved therapies

** Prevalence in commercially accessible geographies*

UX111: Gene therapy to restore *SGSH* gene in CNS and peripheral organs

- BLA submitted in December 2024, anticipate 2025 launch
- Investing in commercial supply
- Leverage existing inborn errors of metabolism field team
- PRV eligible



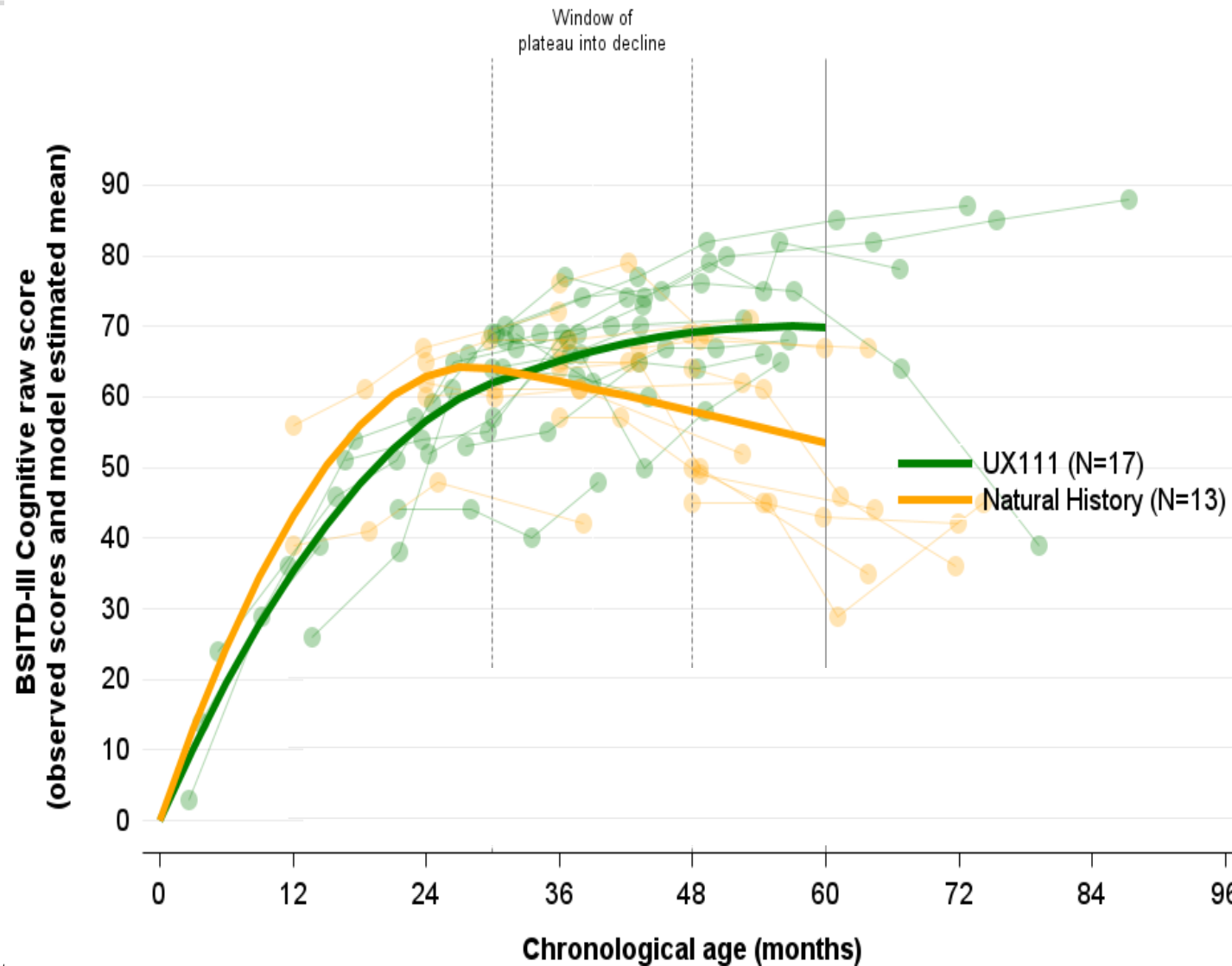
Sadie lives with MPSIII A

“It’s impressive to see how our study patients treated with UX111 have maintained their communication skills despite being the age in which regression begins to occur...improving behavioral problems and thus family daily life.”

Mireia del Toro, M.D.

*Coordinator of the Metabolic Unit, Pediatric Neurology Department,
Hospital Universitari Vall d’Hebron, Barcelona
In reference to data presented at WORLDSymposium in February 2024*

UX111 for MPS IIIA: Divergence emerging at 48 Months using estimated mean BSITD-III cognitive raw scores



BSITD-III Cognitive raw score difference from natural history was mean (95% CI) +22.7 (12.6, 32.9), p-value = 0.0001

Control group from the published natural history study by Shapiro and colleagues (Shapiro et al., 2016). mITT group defined as subjects ≤ 2 years of age at enrollment or > 2 years of age at enrollment with a baseline BSITD-III Cognitive DQ ≥ 60 . BSITD-III, Bayley Scales of Infant and Toddler Development, Third Edition; DQ, developmental quotient; mITT, modified intention-to-treat

UX111 for MPS IIIA: Data showed meaningful accumulated substrate reduction and improved clinical function in pediatric patients

- Reduction in CSF HS¹ exposure predicts increase in BSITD-III Cognitive raw scores
 - Rapid and sustained reduction ($\geq 50\%$) in toxic CSF HS exposure over a median follow-up period of approximately 2 years (23.9 months) after treatment with UX111
 - Gain or stability in BSITD-III Cognitive raw scores observed during the expected window of plateau into decline in the majority of patients
- Treatment with UX111 led to reduction in CSF gangliosides (GM2 and GM3) in line with results for reduction in CSF HS
- Total cortical volume on brain MRI demonstrated stabilization over time and stayed within normal limits in the majority of UX111 treated patients
- Most frequently reported related TEAEs were elevations in liver enzymes
- Promising results suggest a favorable benefit-risk profile of UX111 for the treatment of pediatric patients with MPS IIIA

BLA filed in December 2024

1: cerebrospinal fluid (CSF) heparan sulfate (HS)

DTX401 for glycogen storage disease type Ia (GSDIa)

AAV8 gene therapy; BLA submission expected in mid-2025, launch 2026

GSDIa: Life-threatening defect in liver's ability to release glucose due to *G6Pase* deficiency

- Severe hypoglycemia
- Long-term liver and renal disease
- Prevalence*: ~6,000
- Treatment: Modified diet, cornstarch slurries every few hours around the clock, or liver transplantation

**Prevalence in commercially accessible geographies*

DTX401: Gene therapy designed for stable expression of G6Pase- α

- BLA submission expected in mid-2025, launch 2026
- Manufacturing in-house at our Bedford, MA plant
- Leverage existing inborn errors of metabolism field team
- PRV eligible

Daily cornstarch consumption



"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*

DTX401 for GSDIa: Phase 3 successful across primary and key secondary endpoints

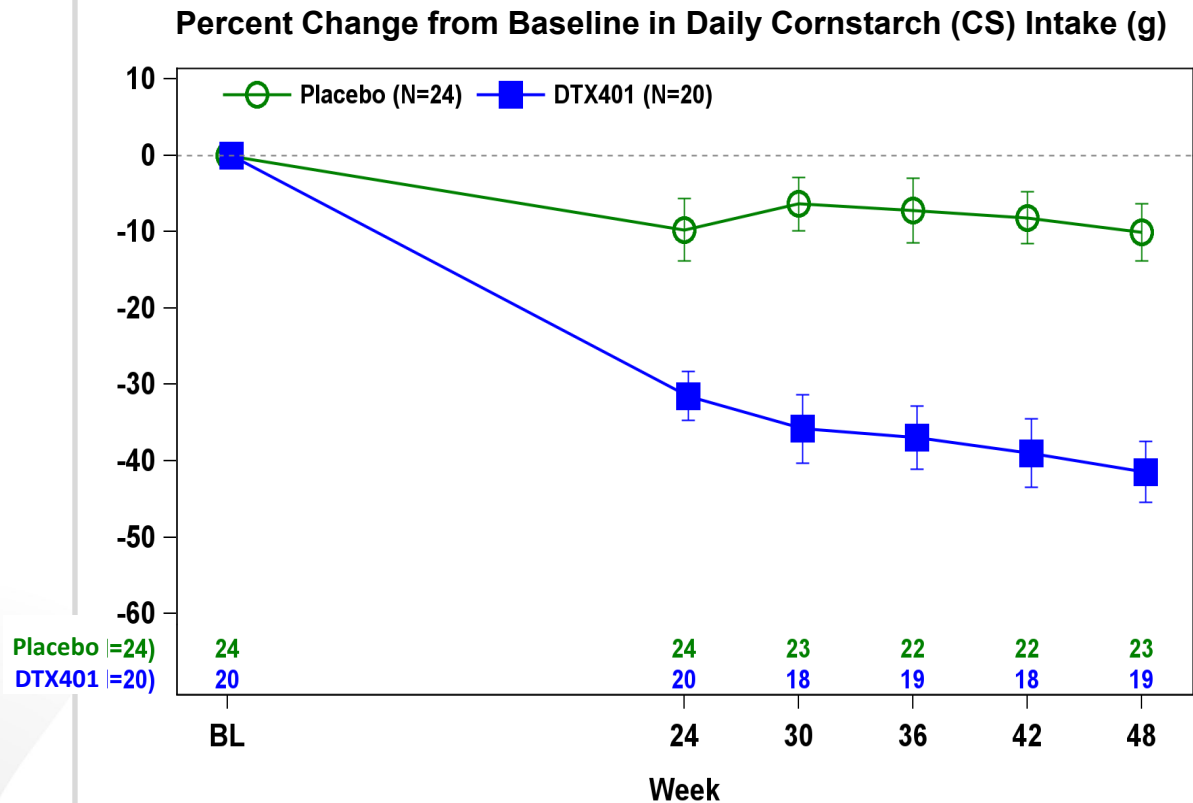
		p-value
Primary Endpoint	%Δ daily cornstarch intake	<0.0001
Key Secondary Endpoints	# of total daily doses of cornstarch	0.0011
	%Δ glucose values in hypoglycemic range (<70 mg/dL), assessed for non-inferiority	<0.0001
	Patient Global Impression of Change score at Week 48 (median)	0.132

Key Takeaways

- GSDIa is a severe, life-threatening metabolic disease, with long term complications due to inability to control glucose
- Phase 3 data demonstrated DTX401 significantly reduced patients dependence on cornstarch, while maintaining glucose control
- Substantial unmet need and we have extensive experience commercializing rare disease medicines

DTX401 for GSDIa: Phase 3 patients continued improving at Week 78 in crossover and originally treated arms

Week 48: Statistically significant reduction (41%) in daily cornstarch intake ($p < 0.0001$)



Week 78: Crossover and original treatment arms continued reducing daily cornstarch (CS)

- Crossover patients (N=12) at Week 30 post-treatment had substantial 62% mean reduction of daily cornstarch (CS)
 - 2x the rate of decrease compared to the original DTX401 treatment arm
 - Patients able to titrate CS much more rapidly once treatment confirmed with DTX401 and with timely, direct access to their glucose levels
- DTX401 treatment arm continued to reduce daily CS intake, while maintaining glycemic control
- DTX401 demonstrated a consistent and acceptable safety profile as of the data cut-off

DTX401 for GSDIa: Patients treated showed significant reduction in frequency and quantity of day and nighttime cornstarch vs placebo

Total Daily Cornstarch (CS) Doses

Total Daily CS Doses (n)	Placebo N=24	DTX401 N=20	p-value
Baseline Mean (SD)	5.1 (1.4)	5.8 (1.4)	
Δ BL to W48 Mean (SD)	-0.1 (0.6)	-1.1 (0.9)	
Δ BL to W48 LS Mean (SE)	-0.2 (0.2)	-1.1 (0.2)	0.0011

“With these Phase 3 results, the significant reduction in cornstarch intake with continued management of glucose control has the potential to offer meaningful benefit to patients while improving quality of life on a daily basis.”

Rebecca Riba-Wolman, M.D.

Director of the Glycogen Storage Disease Program & Disorders of Hypoglycemia at Connecticut Children’s Medical Center and investigator on the study

Nighttime Cornstarch (CS) Doses and Grams

Nighttime CS Doses (n)	Placebo N=17	DTX401 N=17	p-value
Baseline Mean (SD)	1.8 (1.1)	1.7 (0.7)	
Δ BL to W48 Mean (SD)	+0.3 (1.4)	-0.4 (0.6)	
Δ BL to W48 LS Mean (SE)	+0.4 (0.3)	-0.4 (0.3)	0.0410

Changes from baseline for patients who required nighttime CS at baseline

Nighttime CS Intake (g)	Placebo N=17	DTX401 N=17	p-value
Baseline Mean (SD)	100 (74.4)	87.4 (37.0)	
%Δ BL to W48 Mean (SD)	+8.5 (69.3)	-42.4 (29.3)	
%Δ BL to W48 LS Mean (SE)	+6.9 (14.5)	-44.1 (15.0)	0.0091

Changes from baseline for patients who required nighttime CS at baseline

UX701 for Wilson disease (WD)

AAV9 gene therapy; Stage 1 Cohort 4 Data Expected in 2H-2025

Wilson disease: Life-threatening defect in liver's ability to metabolize copper due to *ATP7B* mutation

- Liver failure
- Neurologic deterioration
- Death, if untreated
- Prevalence*: ~50,000
- Treatment: Modified diet, chelation therapy, or liver transplantation

**Prevalence in commercially accessible geographies*

UX701: Gene therapy designed for stable expression of *ATP7B* gene

- Stage 1, Cohort 4 data expected in 2H-2025
- Manufacturing in-house at our Bedford, MA plant



UX701 for WD: Clinical activity observed in Stage 1 with 6 of 15 patients completely off chelators and/or zinc therapy¹

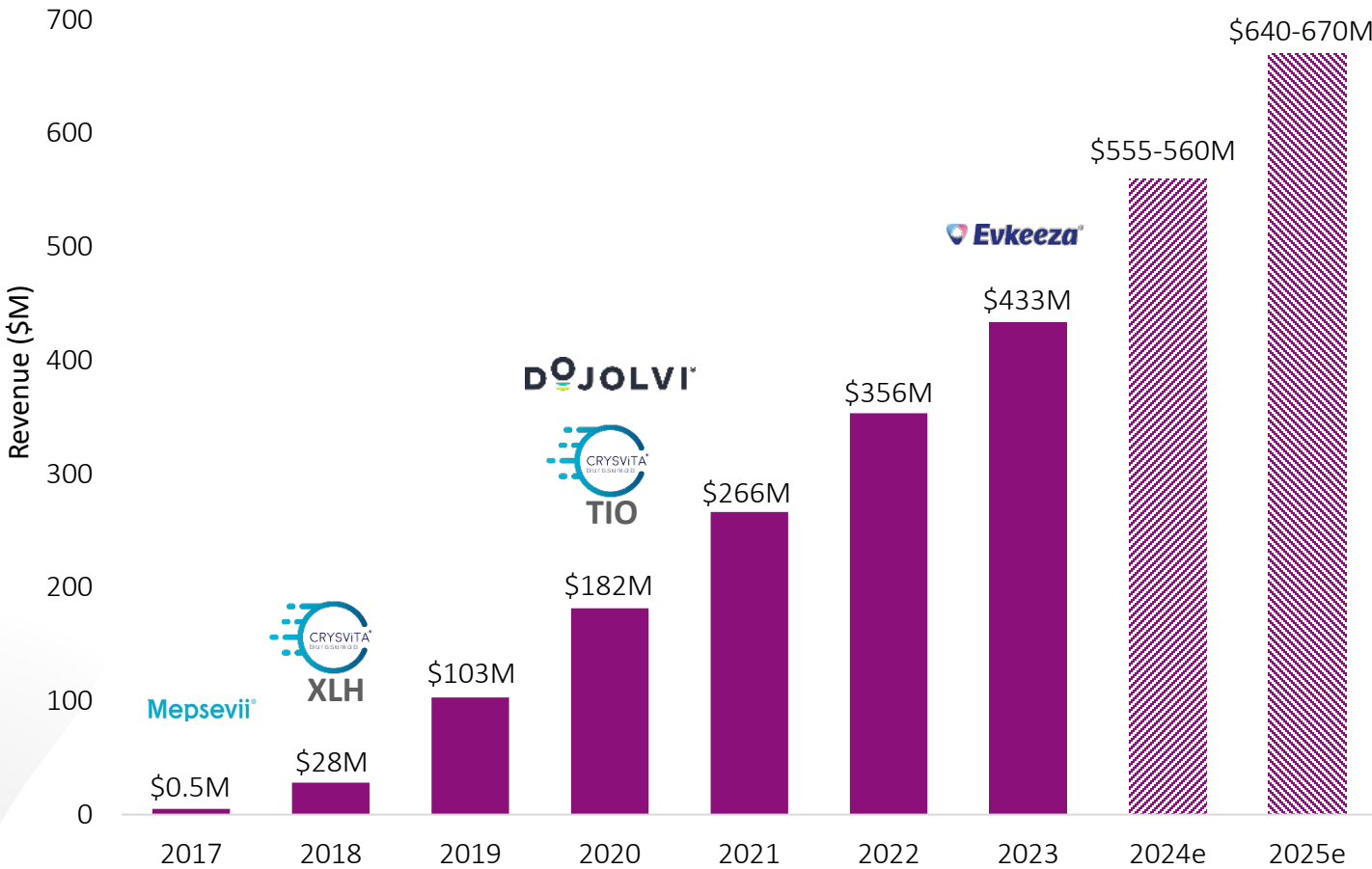
- Clinical activity observed across all three dose cohorts in Stage 1
 - 6 of 15 patients completely off chelators and/or zinc therapy
 - 1 additional patient tapering standard of care
 - In responders, non-ceruloplasmin bound copper (NCC) stabilized to normal, healthy levels
 - Some patients demonstrated increased ceruloplasmin-copper activity consistent with improved loading of copper on ceruloplasmin by *ATP7B* function
- UX701 well tolerated, with no unexpected related treatment emergent adverse events

Plan to enroll additional cohort at moderately increased dose and with optimized immunomodulation

1: Data disclosed in press release on October 3, 2024

2024 total revenue expected to exceed guidance and 2025 total revenue expected to grow 14-20%

Annual Revenue Growth¹



Product	2024 Flash Estimate ³	2025 Guidance
Crysvita ¹	\$405-410M	Expect to be shared on 4Q/FY24 earnings call
Dojolvi	\$87-89M	
Total Revenue²	\$555-560M	\$640-670M

1 Total Crysvita revenue, including North America, Latin America, and Europe
 2 Total Revenue includes Crysvita, Dojolvi, Mepsevii, and Evkeeza
 3 Preliminary, unaudited estimate

¹ Excluding Bayer and Daiichi collaboration revenue, estimates for 2024 and 2025. Logos indicate launch year.



Project full year GAAP profitability in 2027

Core Assumptions

- **Revenue:** Continued double-digit growth from current products and contribution from three upcoming launches
- **Operating expense:** Continued expense management, incorporating investments to maximize launch success (e.g., pre-launch inventory)
- **Cash:**
 - Declining YoY cash used in operations
 - Planned monetization of PRVs from UX111, DTX401, and UX143
 - \$745M in estimated cash, cash equivalents, and marketable debt securities as of December 31, 2024

We expect 2025 to be a transformative year:

Key clinical and regulatory catalysts

PROGRAM	OBJECTIVE	ANTICIPATED TIMING
UX143 Osteogenesis imperfecta	Phase 3 <i>Orbit</i> interim analysis 2 (threshold: $p < 0.01$) Phase 3 <i>Orbit</i> final analysis (threshold: $p < 0.04$)	Mid-2025 4Q-2025
GTX-102 Angelman syndrome	Phase 3 <i>Aspire</i> study initiation Phase 3 <i>Aspire</i> enrollment completion Phase 2/3 <i>Aurora</i> study initiation	✓ 2H-2025 2025
UX111 Sanfilippo Syndrome	BLA submission PDUFA decision and launch	✓ 2H-2025
DTX401 GSDIa	BLA filing	Mid-2025
UX701 Wilson disease	Stage 1, Cohort 4 enrollment completion	2H-2025
DTX301 OTC deficiency	Phase 3 enrollment completion	Early 2025

We are creating a *successful* and *profitable* rare disease company



History of outstanding commercial and clinical execution



Near-term catalysts from 6 Phase 2/3 studies and 3 potential approvals



Revenue growth plus expense management to achieve expected full-year GAAP profitability in 2027



Appendix

Diverse late-stage clinical pipeline

Candidate	Description	Phase 1	Phase 2	Phase 3	Route of Admin	Prevalence ¹
UX143 (setrusumab)	Anti-Sclerostin monoclonal antibody		Osteogenesis Imperfecta (OI)		Intravenous (IV) Infusion	~60,000
UX111	AAV9 SGSH gene therapy		Sanfilippo Syndrome (MPS IIIA)		IV Infusion	~3,000 – 5,000
DTX401	AAV8-G6Pase gene therapy		Glycogen Storage Disease Type Ia (GSDIa)		IV Infusion	~6,000
DTX301	AAV8-OTC gene therapy		Ornithine Transcarbamylase (OTC)		IV Infusion	~10,000
UX701	AAV9-ATP7B gene therapy		Wilson Disease (WD)		IV Infusion	~50,000
GTX-102	ASO activating paternal expression of UBE3A		Angelman Syndrome (AS)		Intrathecal (IT) Infusion	~60,000

Therapeutic Area:

Bone/Endo

Inborn Error of Metabolism

CNS/Muscle

1: Prevalence in commercially accessible geographies

Key licenses & intellectual property – commercial products

Product	License	<u>United States</u> Intellectual Property Rights/Royalties
CRYSVITA® (XLH, TIO)	Kyowa Kirin Co. (KKC)	<ul style="list-style-type: none"> • Anti-FGF23 antibodies and use for treatment of XLH and TIO (2028-2032)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® (MPS7)	St. Louis University (Know-How)	<ul style="list-style-type: none"> • Low single-digit royalty until expiration of orphan drug exclusivity
	N/A (IP Owned by Ultragenyx)	<ul style="list-style-type: none"> • Recombinant human GUS (rhGUS) and use for treatment of MPS7 (2035)
DOJOLVI® (LC-FAOD)	Baylor Research Institute (BRI)	<ul style="list-style-type: none"> • Compositions comprising triheptanoin (2025-2029)¹ • Mid single-digit royalty
	N/A (IP Owned by Ultragenyx)	<ul style="list-style-type: none"> • Ultrapure triheptanoin and use in treatment of FAOD (Pending; 2034)

Product	License	<u>Europe</u> Intellectual Property Rights/Royalties + Milestones
EVKEEZA® (HOFH)	Regeneron	<ul style="list-style-type: none"> • Evkeeza antibody and use for treatment of HOFH (2036)² • Evkeeza antibody in combination with other agents for treatment of HOFH (Pending; 2037) • Stabilized formulations of Evkeeza (Pending; 2041) • Regeneron supplies product and charges Ultragenyx a transfer price from the low 20% range up to 40% on net sales • Ultragenyx to pay up to \$63M in potential regulatory and sales milestones

¹Includes granted U.S. patent term extension

²Includes projected extension via supplementary protection certificates (SPCs)

Key licenses & intellectual property – clinical programs

Product	License	US Intellectual Property Rights/Royalties + Milestones
UX143 (Osteogenesis Imperfecta)	Mereo Biopharma	<ul style="list-style-type: none"> • Setrusumab antibody (2028) • Use of anti-sclerostin antibodies including setrusumab for treatment of OI (2037) • Tiered double-digit royalty on ex-EU sales and clinical, regulatory, and commercial milestones to Mereo • Fixed double-digit royalty on EU sales to Ultragenyx
DTX401 (GSDIa)	NIH (Non-Exclusive)	<ul style="list-style-type: none"> • Recombinant vectors comprising codon-optimized G6Pase gene (2034) • Low single-digit royalty
UX111 / ABO-102 (MPS IIIA)	Nationwide Children’s Hospital (NCH)	<ul style="list-style-type: none"> • Recombinant vectors comprising SGSH gene (Pending; 2032) • Development milestones up to \$1M plus low single-digit royalty
	Abeona Therapeutics	<ul style="list-style-type: none"> • Commercial milestones up to \$30M plus tiered royalty up to 10%
DTX301 (OTC Deficiency)	Sub-License from REGENXBIO of UPENN IP	<ul style="list-style-type: none"> • Recombinant vectors comprising codon-optimized OTC gene (2035) • Low to mid single-digit royalty and development milestones
UX701 (Wilson Disease)	Sub-License from REGENXBIO of UPENN IP	<ul style="list-style-type: none"> • AAV9 Capsid (2026) • Mid to high single-digit royalty and up to \$9M in development milestones
	UPENN	<ul style="list-style-type: none"> • Recombinant vectors comprising certain regulatory and coding sequences packaged in UX701 (2039) • Development up to \$5M and commercial milestones up to \$25M plus low to mid single-digit royalty
	N/A (IP Owned by Ultragenyx)	<ul style="list-style-type: none"> • Recombinant vectors expressing a novel truncated version of ATP7B protein produced by UX701 (Pending; 2040)
GTX-102 (Angelman Syndrome)	Texas A&M University	<ul style="list-style-type: none"> • Use of UBE3A-ATS antisense oligonucleotides including GTX-102 for treatment of AS (2038) • Development and commercial milestones plus mid single-digit royalty
	GeneTx	<ul style="list-style-type: none"> • Development, regulatory, and commercial milestones up to \$190M plus mid to high single-digit royalty

Crysvita partnership revenue recognition

	Product Sales: Latin America & Turkey	Revenue in Profit-Share Territory: U.S. and Canada	Royalty revenue in European Territory
Commercialization	Ultragenyx	KKC	KKC
Revenue	Ultragenyx books sales and pays low single-digit royalty to KKC	KKC books sales and pays revenue share calculated using annual revenue tiers ranging from the mid-20% up to 30% to Ultragenyx	KKC books sales and pays up to 10% royalty to Ultragenyx
Product supply	KKC supplies; price is double-digit percentage of net sales recorded to cost of sales	NA	NA

CRYSVITA[®] exclusivity summary



United States

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



Europe

2028

XLH Orphan +
D&M Exclusivity

2033*

Crysvita CoM
Patent

2035

Q2W
Dosing Patent

*Includes US PTE and EU SPC awards

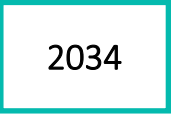
DOJOLVI® exclusivity summary



United States



Europe



Ultrapure Dojolvi (Pending)

*Includes US PTE award



MEPSEVII[®] exclusivity summary

Mepsevii
(vestronidase alfa-vjbk)
injection, for intravenous use
10 mg/5 mL (2 mg/mL)



United States

MPS7 Orphan
Exclusivity

2024

Biologics
Exclusivity

2029

Mepsevii
CoM Patent

2035

2018

2020

2022

2024

2026

2028

2030

2032

2034

2036



Europe

2028

MPS7 Orphan +
D&M Exclusivity

2035

Mepsevii
CoM Patent

EVKEEZA[®] exclusivity summary



Data & Marketing
Exclusivity

2031

Evkeeza
Ab Patent

2036*

2022

2024

2026

2028

2030

2032

2034

2036

2038

2040

Exemplary additional patent applications pending:

- Evkeeza w/ PCSK9 Ab
- Evkeeza w/ statins
- Evkeeza formulations

Projected expiration dates between 2037-2041

*Includes EU SPC award