Corporate Presentation

May 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, fluctuations in buying or distribution patterns from distributors and specialty pharmacies, 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, risks related to reliance on third parties to conduct certain activities on the company's behalf, our limited experience in generating revenue from product sales, our dependence on Kyowa Kirin for the commercialization of Crysvita in certain major markets, including the U.S. and Canada, and for commercial supply of Crysvita in those markets, 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.

Any forward-looking statements made by us in this presentation speak only as of the date of this presentation and represent our expectations, estimates, assumptions and projections only as of the date of this presentation. Except as required by law, we assume no obligation, and we disclaim any intent, to update these statements to reflect actual results or outcomes.

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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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 is yielding great results

Research

Pursue high potential programs

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

Development

Accelerate to drive value

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

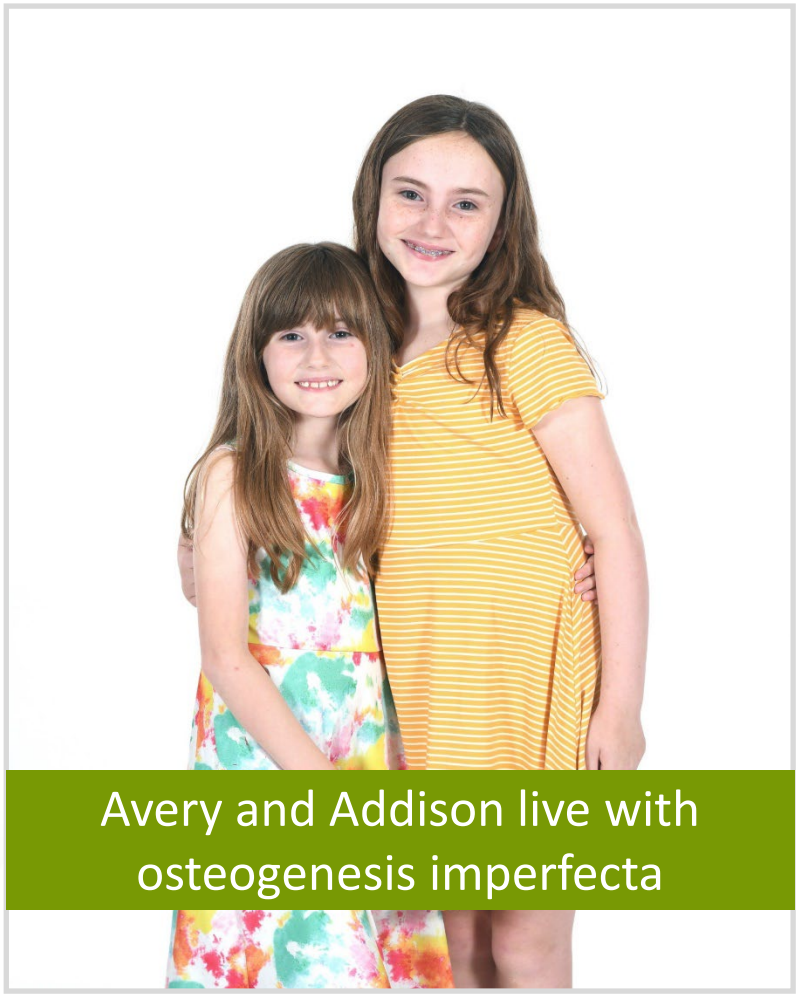
Commercial

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



Avery and Addison live with osteogenesis imperfecta

4 commercial products

CRYSVITA® burosumab

DOJOLVI®

Evkeeza®

Mepsevii®

Largest clinical pipeline in rare disease

6 Phase 2/3 studies → 3 Near-term approvals

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Focused on three therapeutic areas

Late-stage pipeline will leverage successful global commercial organization

	BONE-ENDOCRINE	INBORN ERRORS OF METABOLISM	NEUROGENETIC
COMMERCIAL	CRYSVITA® burosumab — XLH & TIO	DOJOLVI® TRIHEPTANOIN Oral Liquid — LC-FAOD Evkeeza® (evinacumab-dgnb) Injection — HoFH Mepsevii™ (vestronidase alfa-vjbk) injection — MPS VII	
LATE STAGE[1] CLNICAL PROGRAMS	Ph 3: UX143 for OI	Ph 3: UX111 for MPS IIIA Ph 3: DTX401 for GSDIA Ph 3: DTX301 for OTC Ph 2: UX701 for WD	Ph 3: GTX-102 for AS

1: Clinical pipeline available in Appendix

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Diverse late-stage clinical pipeline

Therapeutic Area: Bone/Endo | Neurogenetic | Inborn Errors of Metabolism

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**
GTX-102	ASO activating paternal expression of UBE3A	Angelman Syndrome (AS)			Intrathecal (IT) 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**

1: Prevalence in commercially accessible geographies

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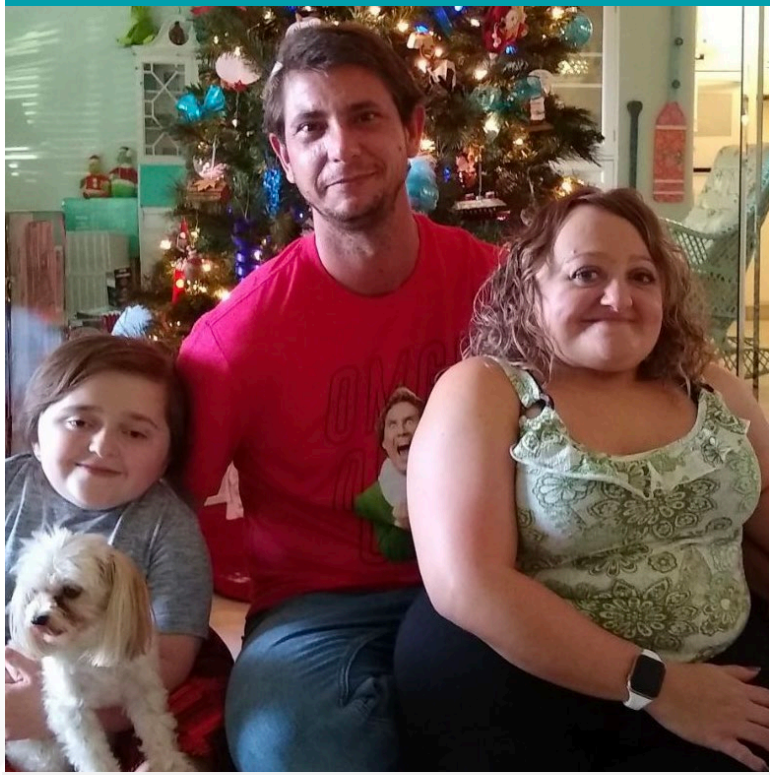
Drivers of value creation in 2025

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 low bone mineral density (BMD) and frequent fractures

- Associated with pain and decreased mobility

- Treatment: No globally approved therapies; bisphosphonates used off label
- Prevalence*: ~60,000 (Types I/III/IV)

**Prevalence in commercially accessible geographies*

Matthew lives with osteogenesis imperfecta

UX143 Setrusumab:

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

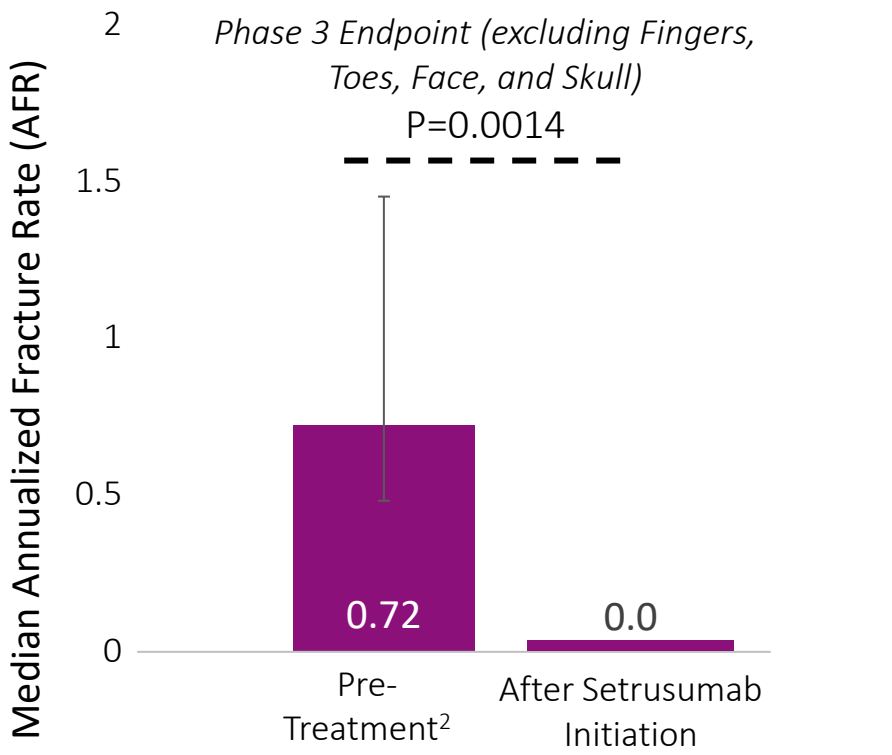
- Phase 3 data expected in 2025
- Investing in commercial supply
- Extensive launch expertise in bone/endocrine from Crysvita
- Priority Review Voucher (PRV) eligible

"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:
Phase 2 data: 67% reduction[1] in annualized fracture rate (AFR) P=0.0014

Radiographically Confirmed Fractures[1]

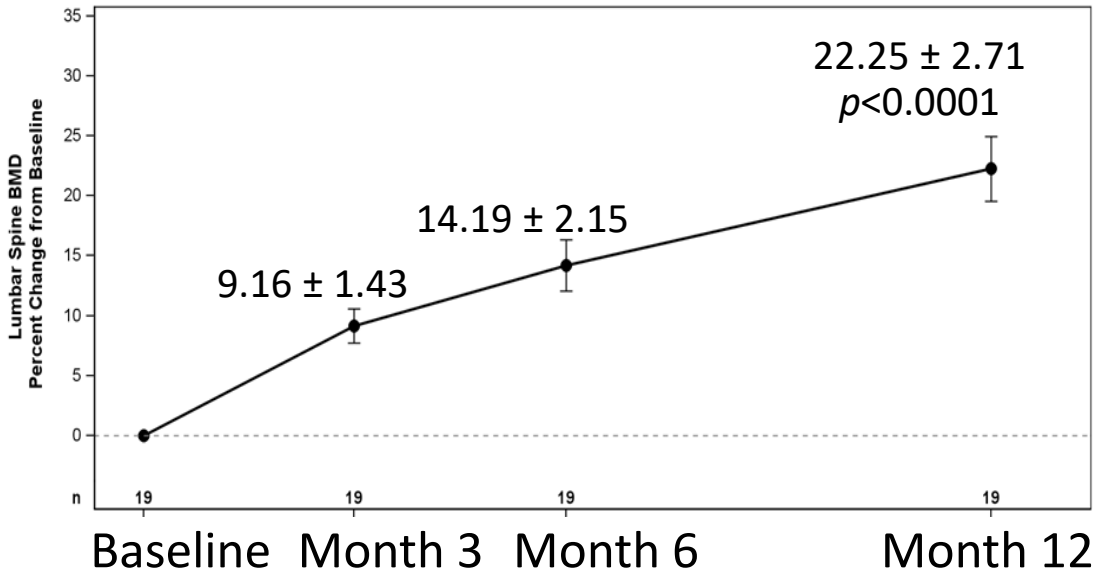


1: Interim data as of May 24, 2024 and includes a mean follow-up of 16 months.
67% reduction = Median(AFR Post-Tx Initiation - Pre-Tx) ÷ Median(AFR Pre-Tx)
2: Pre-Treatment period includes fractures in the two years before screening based on medical record review and patient report, and fractures between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

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

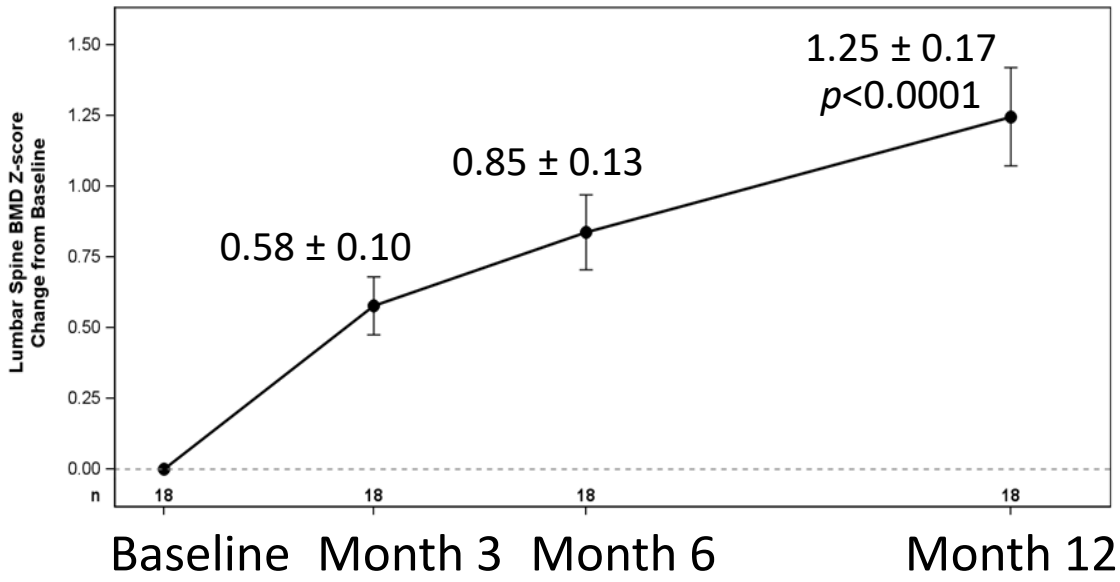
UX143 for OI: Phase 2 data demonstrated increase in lumbar spine BMD and Z-score observed at 12 months

Lumbar Spine BMD (Mean ± SE)[1] Percent Change from Baseline



P-values represent change from Baseline

Lumbar Spine BMD[1] Z-Score Change from Baseline

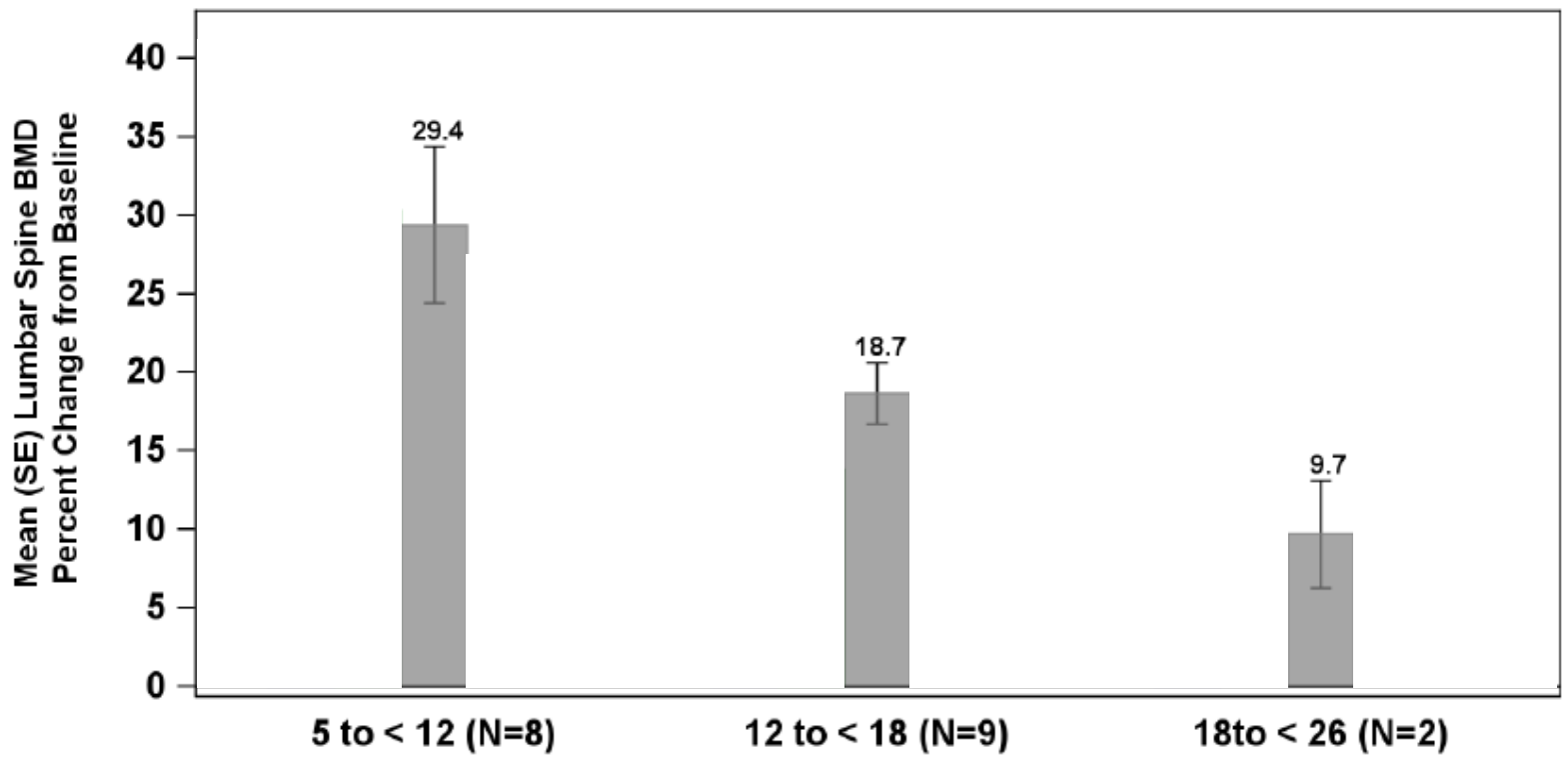


Mean Baseline Z-score of -1.73 goes to -0.49 at 12 months;
P-values represent change from Baseline

1 Interim data as of May 24, 2024

UX143 for OI: Younger patients in Phase 2 showed a very large 29% increase in BMD at Month 12

Lumbar Spine BMD by Age Group[1]
% Change from Baseline at Month 12



1 Interim data as of May 24, 2024

UX143 for OI: Phase 2 safety data[1] 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 P1NP[1] 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 AFR[2] (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 rate[2]
- 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

- Treatment: No approved therapies
- Prevalence*: ~60,000

**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

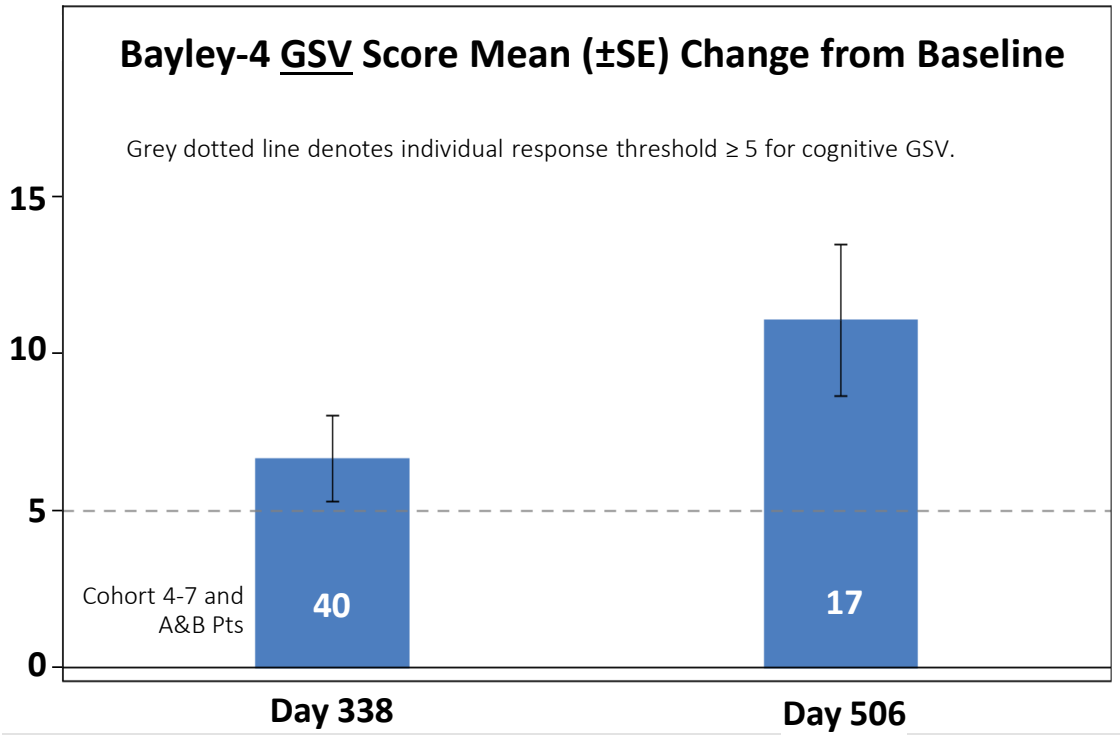
No additional cases of lower extremity weakness; safety profile is understood and remains consistent

Phase 3 study *Aspire* initiated in December 2024

Data shared via press release on November 9, 2024

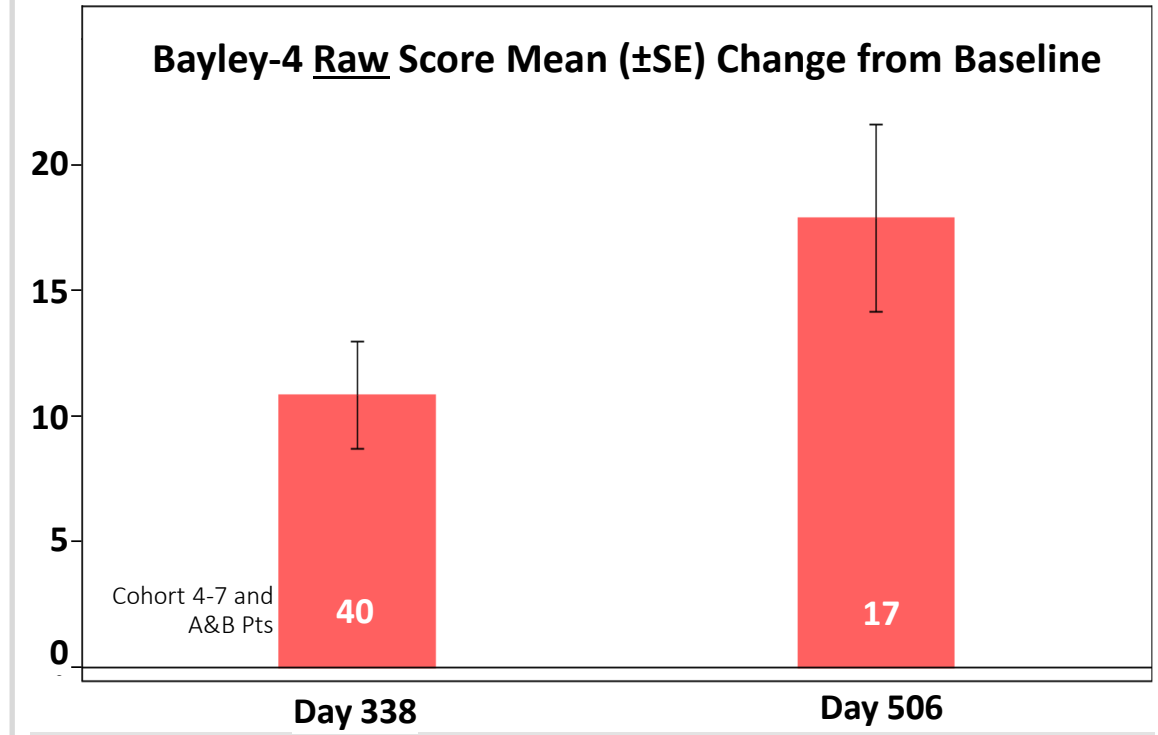
GTX-102 for AS: Cognition by Bayley-4 GSV and raw scores show ample power for Phase 3 trial

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^ (α = 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^ (α = 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: Multi-domain responder index shows ~80% of participants with clinically meaningful net improvement in ≥ 1 domain at Day 338

Cohorts A&B Day 338 Net Response: +2; p-value: <0.0001

Cohort A & B (N=28) responder analysis

ASA **Sleep**	ASA **Gross Motor**	ABC-C **Hyperact/Noncomp**	Bayley-4 **Rec Comm**	Bayley-4 **Cognition**	Total Net Response
					+5
					+5
					+4
					+4
					+4
					+4
					+3
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					+2
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					+2
					+1
					+1
					+1
					+1
					0
					0
					0
					0
					0
					-1

Key Takeaways

- MDRI is a key secondary endpoint in Phase 3: Sleep, Gross Motor, Behavior (Hyperact./ Noncompl.), Rec. Comm. and Cognition
- Ph1/2 data: persuasive statistically significance, capturing meaningful responses
- Bayley-4 GSV score to be used for MDRI
  - MID exists for GSV scores

Minimal important difference (MID):

ASA: Sleep = ± 1; Gross Motor = ± 1

ABC-C: Hyperactivity/Noncompliance RAW = ± 6

Bayley-4: Receptive Comm GSV = ± 6; Cognition GSV = ± 5

Green color code indicates an improvement: ≥ +1 MID

Pink color code indicates a decline: ≤ -1 MID

White indicates minimal to no change

Last observation used for imputing missing post-baseline data

Data shared via press release on November 9, 2024

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GTX-102 for AS: Safety summary

- 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

Data shared via press release on November 9, 2024

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

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UX111 for Sanfilippo syndrome (MPS IIIA) *AAV9 gene therapy*
Priority Review Received; PDUFA action date August 18, 2025; AdCom not likely

MPS IIIA: Fatal lysosomal storage disease of CNS

- Early childhood onset
- Rapid neurodegeneration

- Treatment: No approved therapies
- Prevalence*: ~3,000 to 5,000

** Prevalence in commercially accessible geographies*

Sadie lives with MPSIIIA

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

- PDUFA date August 18, 2025; potential 2H-2025 launch
- Priority review granted, PRV eligible
- Investing in commercial supply
- Leverage existing inborn errors of metabolism field team

"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

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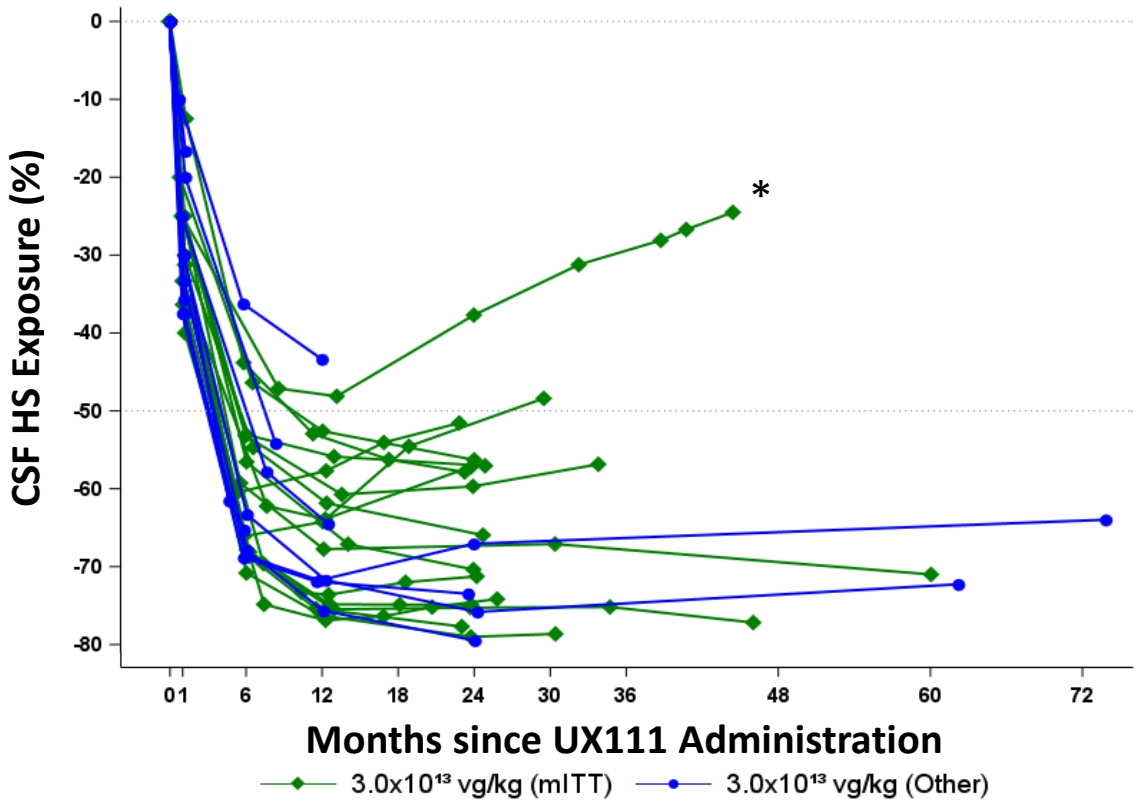
UX111 for MPS IIIA: Substantial reduction in CSF HS[1] exposure regardless of age or stage of disease

Rapid reduction in CSF HS[1] over 7 to 77 months



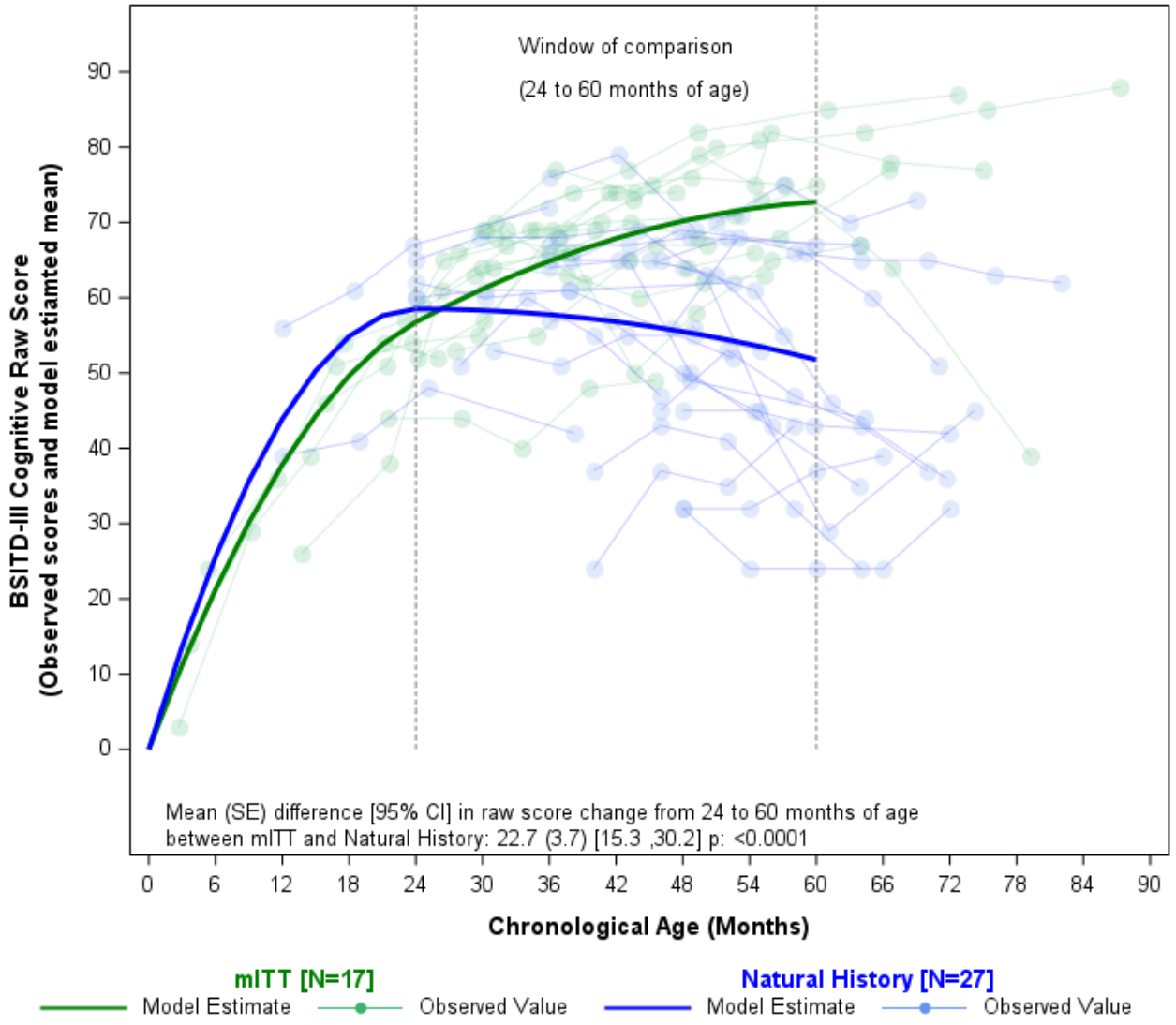
Key Takeaways

- > 80% of participants reduced CSF HS by 50% in efficacy set
- Median CSF HS exposure
  - Efficacy set (N=27): −64.51% (p<0.0001)
  - mITT set (N=17): −65.96% (p<0.0001)
  - Maximum reduction: ~79%
- * One patient with immune response lost expression and cognitive function

1: cerebral spinal fluid (CSF) heparan sulfate (HS)

UX111 for MPS IIIA: Treatment with UX111 led to improved Bayley-III raw scores compared to natural history

- Model-based est'd LS mean (SE) of change from ages 24 to 60 mths in BSITD-III Cognitive Raw Score
  - mITT participants improved by **+16.0 (2.9) points**
  - Untreated Natural History patients declined by **-6.8 (2.3) points**
  - **Treatment effect: +22.7 points (p <0.0001)**
- **Statistically significant improvement in receptive & expressive communication raw scores** (not shown)
- Numerical improvement in fine motor and gross motor scores (not shown)
  - Gross motor function is generally lost later in the disease process, and longer-term follow-up may be needed to see statistically significant changes



Data presented at WORLD*Symposium* 2025

UX111 for MPS IIIA: Conclusions

- UX111 led to substantial and sustained reductions in CSF HS exposure over time, irrespective of age or stage of disease progression at the time of treatment
- Reduction in CSF HS exposure correlated with improved Bayley-III Scores
  - Statistically significant correlations between CSF HS exposure and estimated yearly change were seen for all 5 Bayley-III subdomains
- Younger participants treated early in disease progression showed gains in cognitive skills, expressive and receptive communication, and fine motor skills compared to natural history
- Older participants treated at more advanced stages of disease showed retention of key functions of communication, feeding, and ambulation
- UX111 was generally well tolerated across all doses, including the highest dose of 3.0×10^{13} vg/kg, and observed adverse reactions were manageable

PDFUA action date: August 18, 2025

Data presented at WORLD*Symposium* 2025

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

- Treatment: Modified diet, cornstarch slurries every few hours around the clock, or liver transplantation
- Prevalence*: ~6,000

**Prevalence in commercially accessible geographies*

DTX401: Gene therapy to express 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

"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

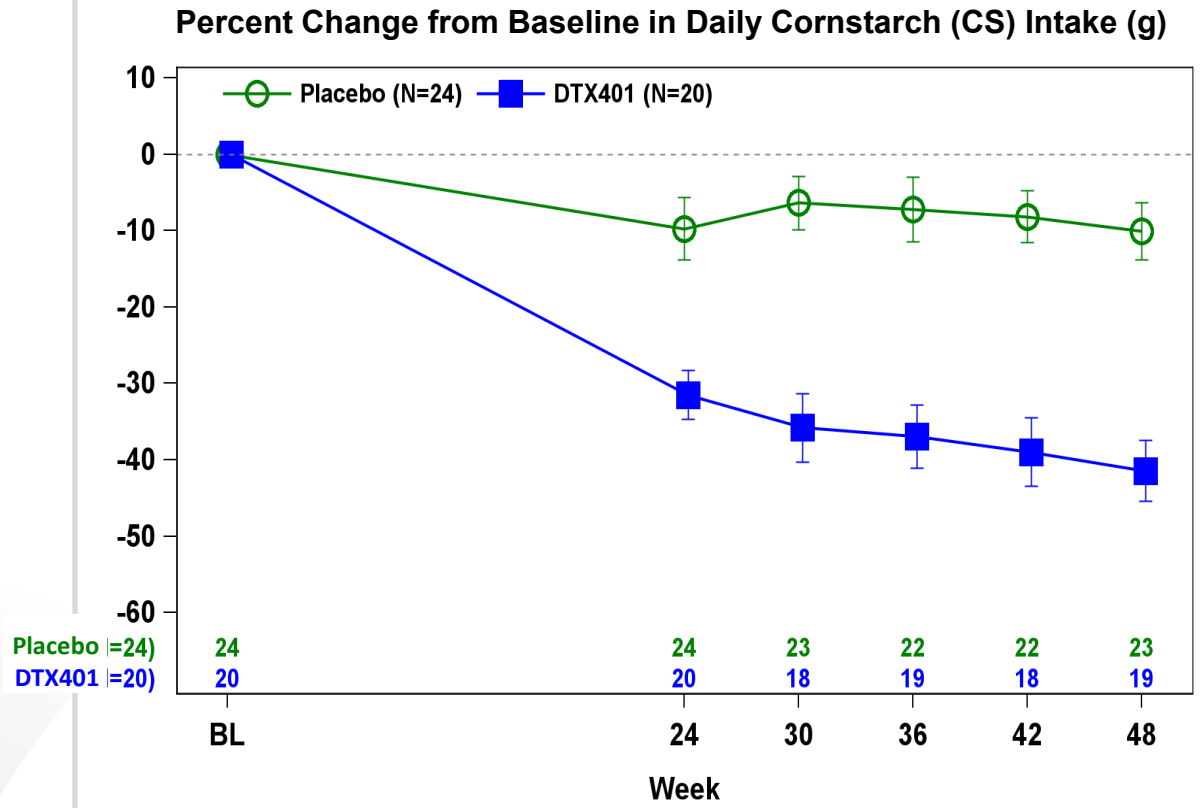
Key Takeaways

- 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 dependance on cornstarch, while maintaining glucose control
- Substantial unmet need and we have extensive experience commercializing rare disease medicines

Data presented via conference call on May 30, 2024

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

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



Week 120: Crossover and original treatment arms continued reducing daily cornstarch (CS)

- Crossover patients, previously treated with placebo, demonstrated a **64% reduction** in daily CS at their last visit
  - 69 week mean follow-up post-DTX401 treatment
  - Patients able to titrate CS much more rapidly once treatment confirmed with DTX401 and with timely, direct access to their glucose levels
- Patients in the original DTX401 group demonstrated a **60% reduction** in daily CS at their last visit
  - 120 week mean follow-up
- DTX401 demonstrated a consistent and acceptable safety profile as of the data cut-off

Week 120 data presented via press release on May 6, 2025

DTX401 for GSDIa: Patients treated showed significant reduction in **frequency and quantity** of day and nighttime cornstarch vs placebo at Week 48

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

Data presented via conference call on May 30, 2024

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

- Treatment: Modified diet, chelation therapy, or liver transplantation
- Prevalence*: ~50,000

**Prevalence in commercially accessible geographies*

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

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

GT manufacturing facility in Bedford, MA

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

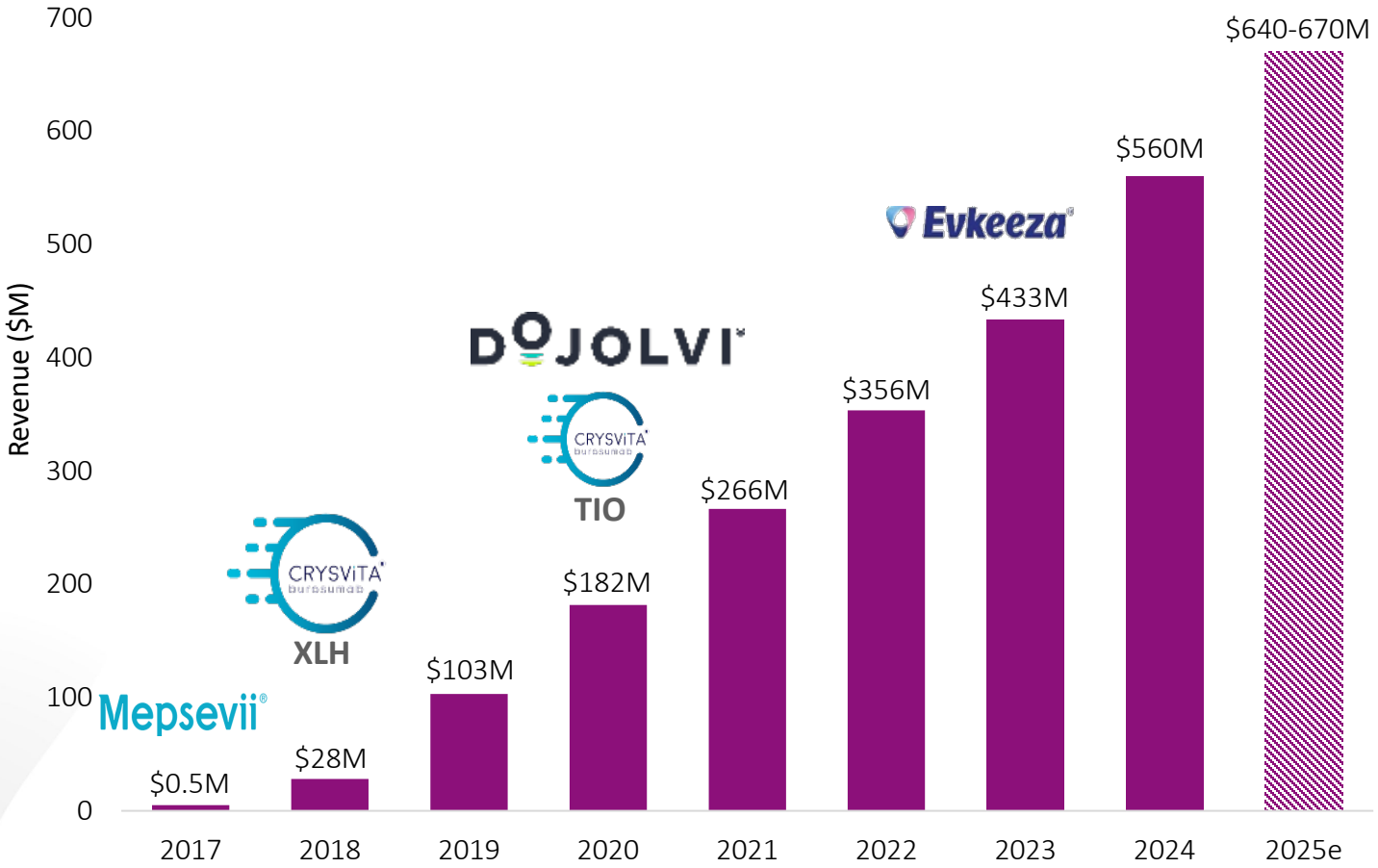
- Clinical activity observed across all three dose cohorts in Stage 1
  - 6 of 15 patients completely off chelators and/or zinc therapy
  - 1 additional patient tapering standard of care
  - In responders, non-ceruloplasmin bound copper (NCC) stabilized to normal, healthy levels
  - Some patients demonstrated 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

2025 total revenue expected to grow 14-20%

Annual Revenue Growth[1]



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

Product	2024 Actuals	2025 Guidance
Crysvita[1]	$410M	$460-480M *12-17%*
Dojolvi	$88M	$90-100M *2-13%*
Total Revenue[2]	$560M	$640-670M *14-20%*

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

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
  - $563M in cash, cash equivalents, and marketable debt securities as of March 31, 2025

We expect 2025 to be a transformative year:
Key clinical and regulatory catalysts

PROGRAM	OBJECTIVE	ANTICIPATED TIMING
UX143 Osteogenesis imperfecta	Phase 3 *Orbit* interim analysis 2 (threshold: p<0.01) Phase 3 *Orbit* final analysis (threshold: p<0.04)	Mid-2025 4Q-2025
GTX-102 Angelman syndrome	Phase 3 *Aspire* study initiation Phase 3 *Aspire* enrollment completion Phase 2/3 *Aurora* study initiation	✓ 2H-2025 2025
UX111 Sanfilippo syndrome	BLA submission PDUFA date Potential commercial launch	✓ August 18, 2025 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	✓

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

History of outstanding clinical ***and*** outstanding commercial execution

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

Revenue growth and new launches plus expense management to achieve expected full-year GAAP profitability in 2027 and beyond

Appendix

Key licenses & intellectual property – commercial products

Product	License	United States Intellectual Property Rights/Royalties
CRYSVITA® (XLH, TIO)	Kyowa Kirin Co. (KKC)	• 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)	• Low single-digit royalty until expiration of orphan drug exclusivity
	N/A (IP Owned by Ultragenyx)	• Recombinant human GUS (rhGUS) and use for treatment of MPS7 (2035)
DOJOLVI® (LC-FAOD)	Baylor Research Institute (BRI)	• Compositions comprising triheptanoin (2025-2029)[1] • Mid single-digit royalty
	N/A (IP Owned by Ultragenyx)	• Ultrapure triheptanoin and use in treatment of FAOD (Pending; 2034)

Product	License	Europe Intellectual Property Rights/Royalties + Milestones
EVKEEZA® (HOFH)	Regeneron	• 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

[1]Includes granted U.S. patent term extension
[2]Includes 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	• 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)	• Recombinant vectors comprising codon-optimized G6Pase gene (2034) • Low single-digit royalty
UX111 / ABO-102 (MPS IIIA)	Nationwide Children's Hospital (NCH)	• Recombinant vectors comprising SGSH gene (Pending; 2032) • Development milestones up to $1M plus low single-digit royalty
	Abeona Therapeutics	• Commercial milestones up to $30M plus tiered royalty up to 10%
DTX301 (OTC Deficiency)	Sub-License from REGENXBIO of UPENN IP	• 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	• AAV9 Capsid (2026) • Mid to high single-digit royalty and up to $9M in development milestones
	UPENN	• 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)	• Recombinant vectors expressing a novel truncated version of ATP7B protein produced by UX701 (Pending; 2040)
GTX-102 (Angelman Syndrome)	Texas A&M University	• Use of UBE3A-ATS antisense oligonucleotides including GTX-102 for treatment of AS (2038) • Development and commercial milestones plus mid single-digit royalty
	GeneTx	• Development, regulatory, and commercial milestones up to $190M plus mid to high single-digit royalty

Crysvita partnership revenue recognition

	Product Sales: Latin America & Türkiye	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 on Latin America revenue	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

*Includes US PTE and EU SPC awards

DOJOLVI® exclusivity summary

DOJOLVI® TRIHEPTANOIN Oral Liquid

NCE Exclusivity — 2025

LC-FAOD Orphan Exclusivity — 2027

Dojolvi CoM Patent — 2029*

Ultrapure Dojolvi (Pending) — 2034

2018 2020 2022 2024 2026 2028 2030 2032 2034 2036

Ultrapure Dojolvi (Pending) — 2034

*Includes US PTE award

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MEPSEVII® exclusivity summary

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

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

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EVKEEZA® exclusivity summary

Evkeeza® (evinacumab-dgnb) Injection

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

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