Corporate Presentation

May 2026

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, including the company's expectations related to benefits and savings from the strategic restructuring plan, 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, the company's ability to provide the requested documentation and address the comments in the CRL for UX111 to the satisfaction of the FDA, the timing of FDA review of the company's BLA submissions, the timing and outcome of any FDA inspections related to UX111 or other clinical product candidates, 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, our ability to successfully manage the expansion of our company, delays or unexpected costs related to the strategic restructuring plan, 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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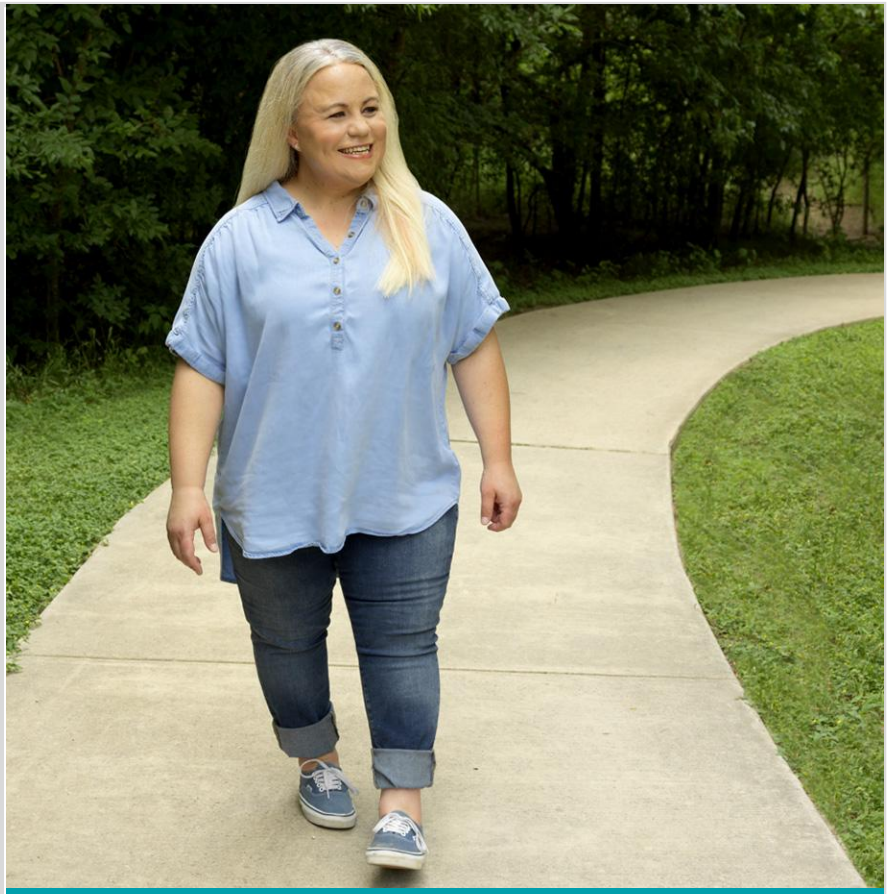
Ultragenyx founded to develop first-ever treatments

Leading the future of rare disease



Aly lives with
X-Linked Hypophosphatemia

Our differentiated approach to rare diseases

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

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

Mason lives with Angelman syndrome

4 commercial products

CRYSVITA® burosumab

DOJOLVI®

Evkeeza®

Mepsevii®

Diverse clinical pipeline

5 Phase 2/3 programs → 2 Potential approvals in 2026

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Focused on three therapeutic areas

Late-stage pipeline will leverage successful global commercial organization

	BONE-ENDOCRINE	INBORN ERRORS OF METABOLISM	NEUROLOGY
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] CLINICAL 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 Ph 1/2: UX016 for GNEM

1: Clinical pipeline available in Appendix

Diverse late-stage clinical pipeline

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

Candidate	Description	Phase 1 / Phase 2 / Phase 3	Route of Admin	Prevalence[1]
GTX-102	ASO activating paternal expression of UBE3A	Angelman Syndrome (AS)	Intrathecal (IT) Infusion	**~60,000**
UX016[2]	Small molecule prodrug	GNE Myopathy	Oral substrate replacement	**~10,000**
UX111	AAV9 SGSH gene therapy	Sanfilippo Syndrome (MPS IIIA)	Intravenous (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**
UX143 (setrusumab)	Anti-Sclerostin monoclonal antibody	Osteogenesis Imperfecta (OI)	IV Infusion	**~60,000**

1: Prevalence in commercially accessible geographies
2: Externally funded by a venture philanthropy agreement through clinical proof-of-concept

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Clinical Programs

UX111 for Sanfilippo syndrome (MPS IIIA)

AAV9 gene therapy; PDUFA action date set for September 19, 2026

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

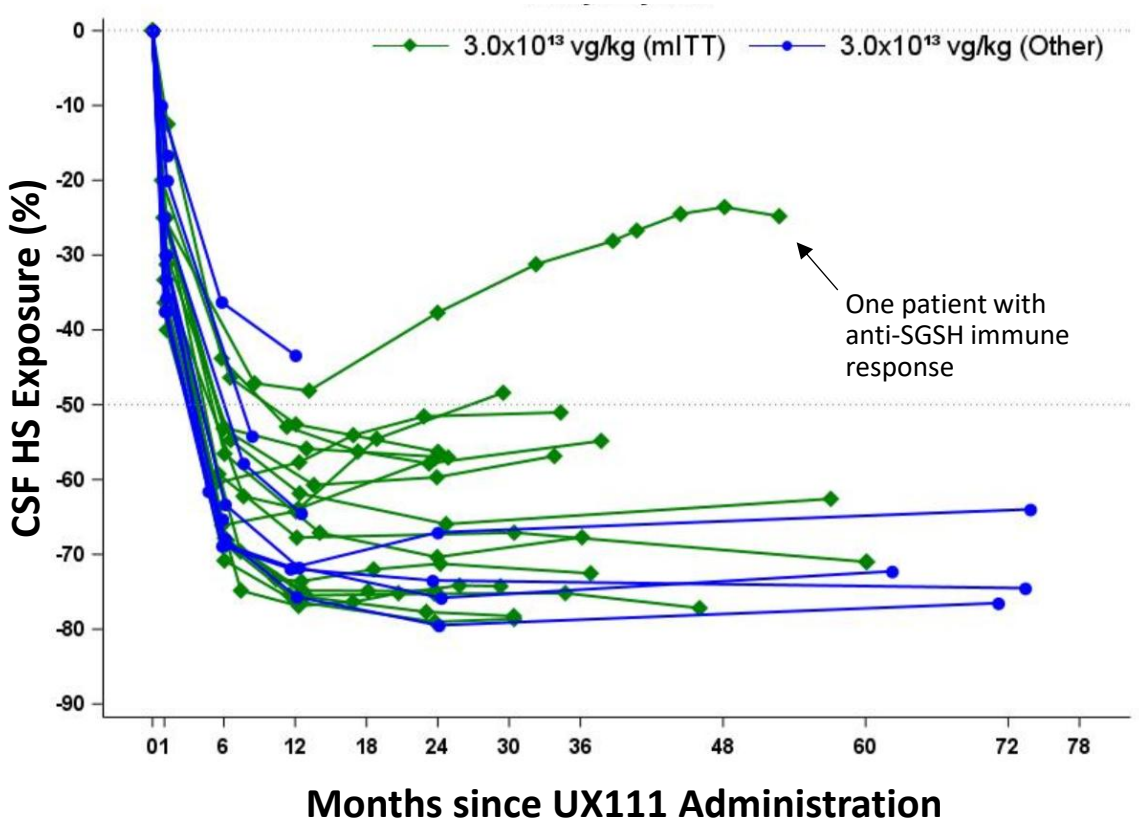
- PDUFA action date: September 19, 2026
- Investing in commercial supply
- Leverage existing inborn errors of metabolism field team
- PRV eligible

"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: Substantial and durable reduction in CSF HS[1] exposure regardless of age or stage of disease

Rapid and sustained reduction in CSF HS[1] up to 7.76 years



Key Takeaways

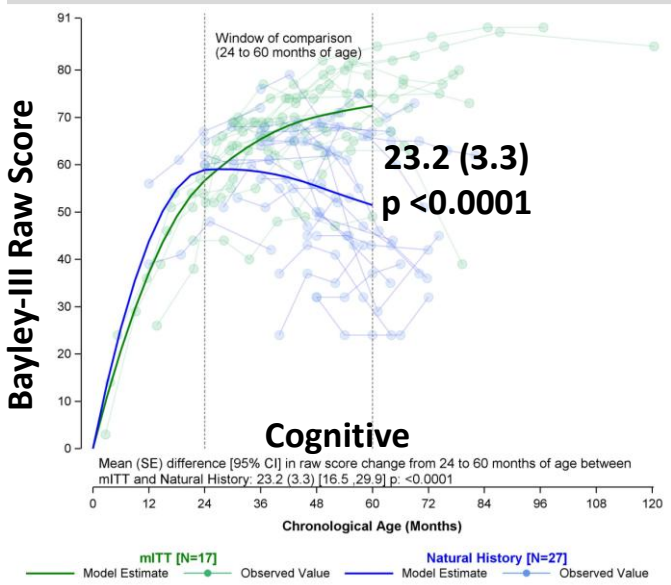
- > 80% of participants reduced CSF HS by 50% in efficacy set
- Median CSF HS exposure
  - Efficacy set (N=27): -63.98% (p<0.001)
  - mITT* set (N=17): -62.58% (p<0.0001)
  - Maximum reduction: ~79%

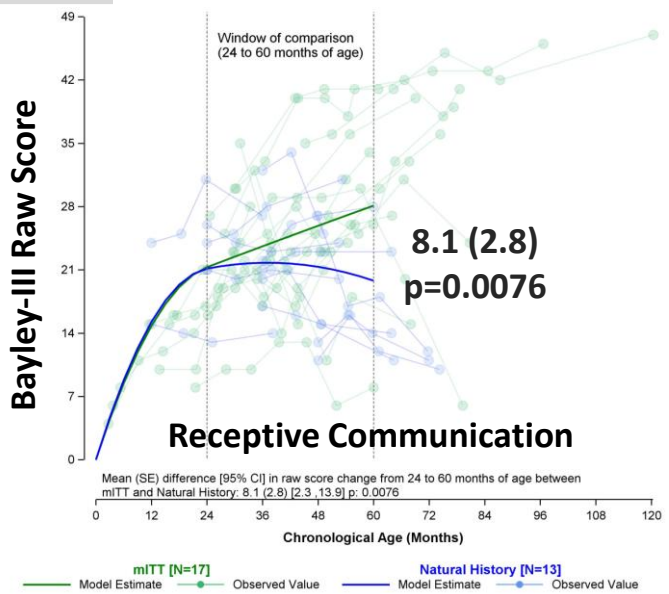
*mITT: modified intention-to-treat

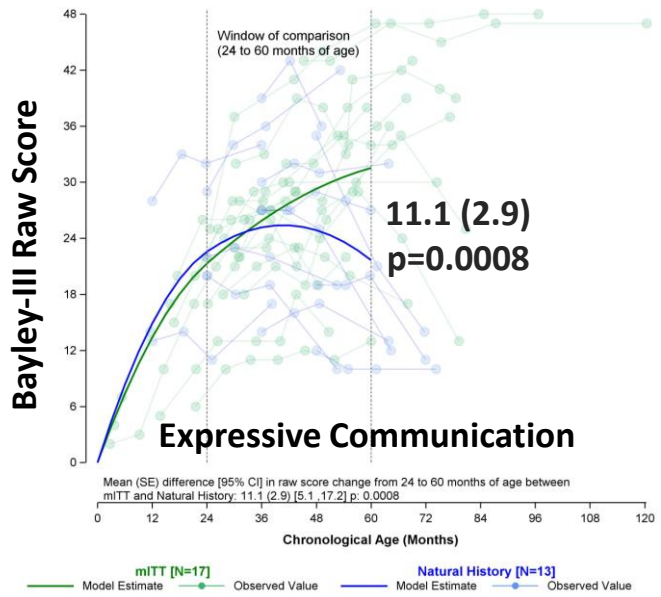
1: cerebral spinal fluid (CSF) heparan sulfate (HS)

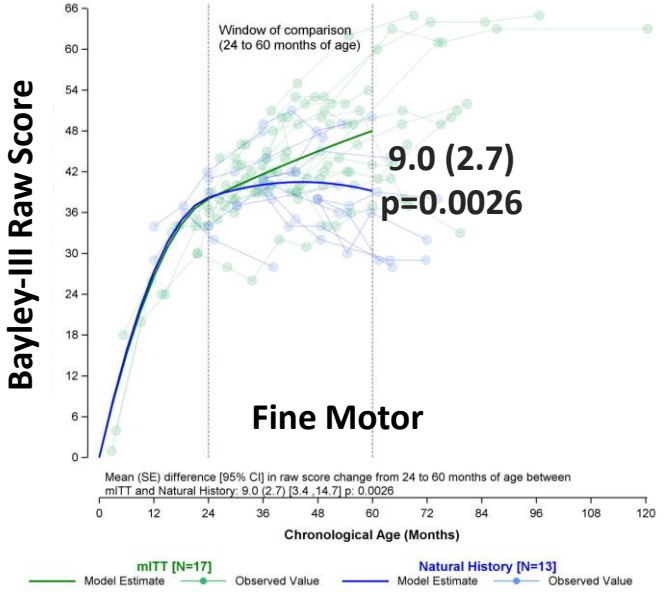
Data presented at WORLD*Symposium* in Feb 2026

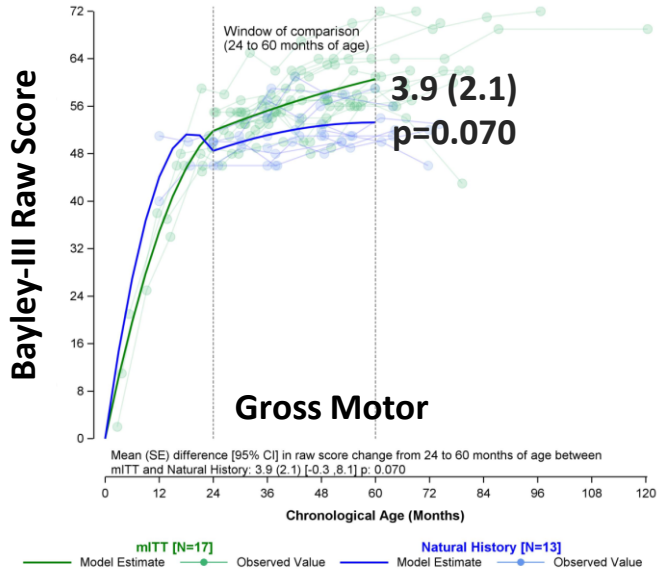
UX111 for MPS IIIA: Bayley raw scores on all 5 subtests were improved or stabilized in mITT set compared to natural history











- The caregiver-reported Vineland data was consistent in the communication, motor, and personal subdomains
- 8 children in the mITT set reached a cognitive level of 36 months developmental age, that allowed testing with the Kaufman
  - No children in the natural history set achieved this cognitive developmental level

Data presented at WORLD*Symposium* in Feb 2026

UX111 for MPS IIIA: Conclusions / Next Steps

- Substantial and durable reductions in CSF HS exposure
  - Regardless of dose, age or stage of disease at time of intervention
- Developmental skills durably improved or stabilized relative to declining natural history
  - Meaningful differences with persuasive statistical significance
  - Early-stage disease treatments improves 5 subtests of the BSID-III
  - Older children with later-stage disease were able to retain key functional skills of communication, ambulation, and eating/self-feeding
- 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

PDUFA action date: September 19, 2026

Data presented at WORLD*Symposium* in Feb 2026

DTX401 for glycogen storage disease type Ia (GSDIa)

AAV8 gene therapy; PDUFA action date set for August 23, 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 granted priority review, PDUFA date August 23, 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

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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)	0.131

Week 48 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

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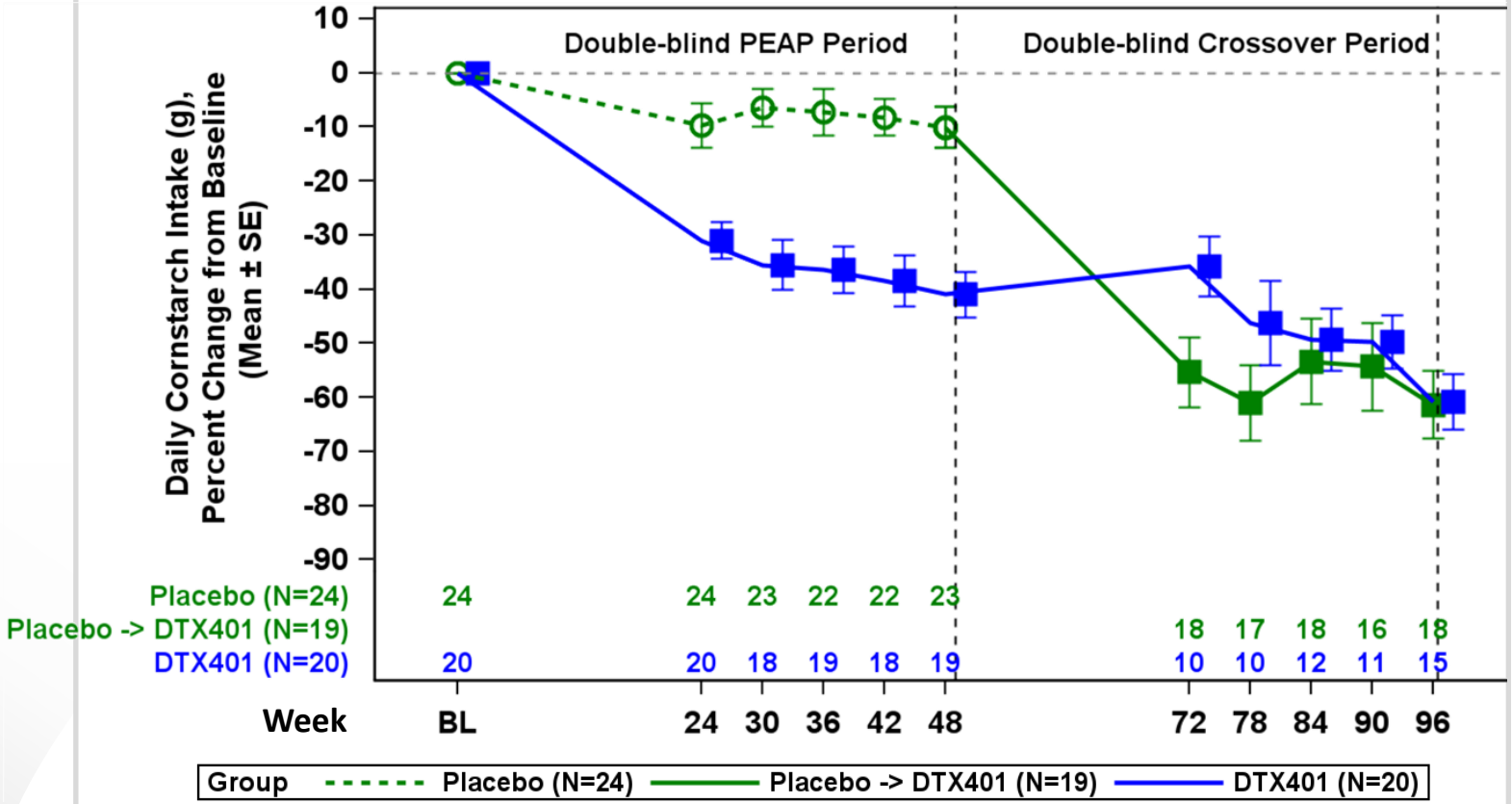
DTX401 for GSDIa: Phase 3 crossover and original treatment arm continued reducing daily cornstarch through Week 96

Statistically significant and clinically meaningful cornstarch reductions continued in crossover period



Week 96
Daily cornstarch (CS) reductions

- **61% reduction** in daily CS at Week 96 in the original DTX401 group and Crossover patients
- **Titrate CS much more rapidly** once treatment confirmed and timely, direct access to their glucose levels
- **Consistent and acceptable safety** profile as of the data cut-off

Data presented at ICIEM September 2025

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DTX401 for GSDIa: Statistically significant reductions in **frequency and quantity** of day and nighttime CS vs placebo at Week 48 that continued to Week 96

Total Daily Cornstarch (CS) Doses

Total Daily CS Doses (n)	DTX401[1]	Placebo / Crossover[2]	p-value
Baseline Mean (SE)	5.8 (0.3)	5.1 (0.3)	
Δ BL to Week 48 Mean (SE)	**-1.1 (0.2)**	**-0.1 (0.1)**	**0.0011**
Δ BL to Week 96 Mean (SE)	**-1.9 (0.4)**	**-1.6 (0.5)**	

1: N = 24; 2: N=20 for patients initially treated with placebo and N=19 for patients who crossed over to DTX401. Crossover group was re-baselined at Week 48 and change in Crossover group reflects change from Week 48.

"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)	DTX401[1]	Placebo / Crossover[2]	p-value
Baseline Mean (SE)	1.7 (0.3)	1.7 (0.2)	
Δ BL to W48 Mean (SE)	-0.4 (0.2)	+0.5 (0.4)	0.0105
Δ BL to Week 96 Mean (SE)	-0.8 (0.2)	-1.1 (0.5)	

Changes from baseline for patients who required nighttime CS at baseline

Nighttime CS Intake (g)	DTX401[1]	Placebo / Crossover[2]	p-value
Baseline Mean (SE)	85.8 (9.2)	100.4 (20.6)	
%Δ BL to W48 Mean (SE)	-42.5 (7.8)	+15.2 (27.7)	0.0132
%Δ BL to W96 Mean (SE)	-70.2 (10.0)	-75.1 (7.6)	

Changes from baseline for patients who required nighttime CS at baseline
1: N = 15; 2: N=15 for patients initially treated with placebo and N=13 for patients who crossed over to DTX401. Crossover group was re-baselined at Week 48 and change in Crossover group reflects change from Week 48.

Data presented at ICIEM September 2025

DTX401 for GSDIa: Patients reported meaningful improvements in disease condition at Week 48 and Week 96

Patient Global Impression of Change (PGIC)

- Week 48: 79% (15/19) reported improved GSDIa in DTX401-treated group versus 52% (12/23) Placebo
- Week 96: (end of Double-blind Crossover Period) improved GSDIa reported in:
  - 83% (10/12) DTX401 group
  - 95% (18/19) Crossover DTX401 group

Patient interviews

- Week 48: 39% reported improved ability to self-regulate blood sugar levels (e.g., feeling of a “safety net”) in DTX401 versus 18% Placebo
- DTX401-treated participants most frequently reported:
  - Reductions in cornstarch intake, hypoglycemia, tiredness
  - Improvements in physical function, social, and diet/daily regimen impacts

Data presented at ICIEM September 2025

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DTX401 for GSDIa: Safety / Next Steps

- Acceptable safety profile in Phase 3, consistent with Phase 1/2 study
  - Anticipated hepatic reactions managed with a prophy corticosteroids
  - No AAV8 class effects of dorsal root ganglion toxicity, malignancy or thrombotic microangiopathy were observed through Week 96
  - Hypertriglyceridemia observed in all study groups more frequently following DTX401

BLA granted priority review, PDUFA date August 23, 2026

Data presented at ICIEM September 2025

DTX301 for ornithine transcarbamylase (OTC) deficiency

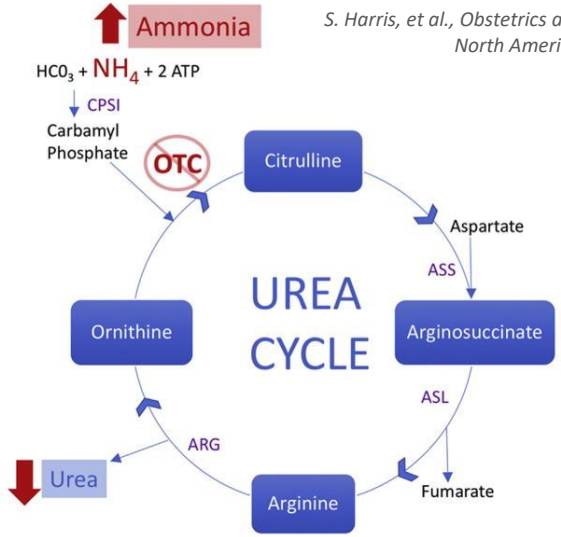
AAV8 gene therapy; Phase 3 responder data expected in 1H-2027

OTC Deficiency: X-linked urea cycle disorder, genetic defect in ammonia detoxification

- Acute hyperammonemic episodes
- Adverse cognitive, neurological effects, and death

- Treatment limited: Primarily ammonia scavenger medications and protein restricted diet
- Prevalence*: ~10,000; 80% late-onset

**Prevalence in commercially accessible geographies*



S. Harris, et al., Obstetrics and Gynecology Clinics of North America (2018)

DTX301: Gene therapy to express OTC

- Phase 3 complete responder data in 1H-2027
- Manufacturing in-house at our Bedford, MA plant
- Leverage existing inborn errors of metabolism field team
- Orphan Drug Designation in both the U.S. and EU and Fast Track Designation in the U.S.

"We are extremely encouraged by the phase 3 findings given the significant medical needs faced by patients with OTC deficiency, who remain at risk for unpredictable and potentially life threatening hyperammonemic crises."

Eric Crombez

Chief Medical Officer, Ultragenyx

Data disclosed in press release on March 12, 2026

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DTX301 for OTC:
Positive 36-week data from Phase 3 *Enh3ance* clinical study

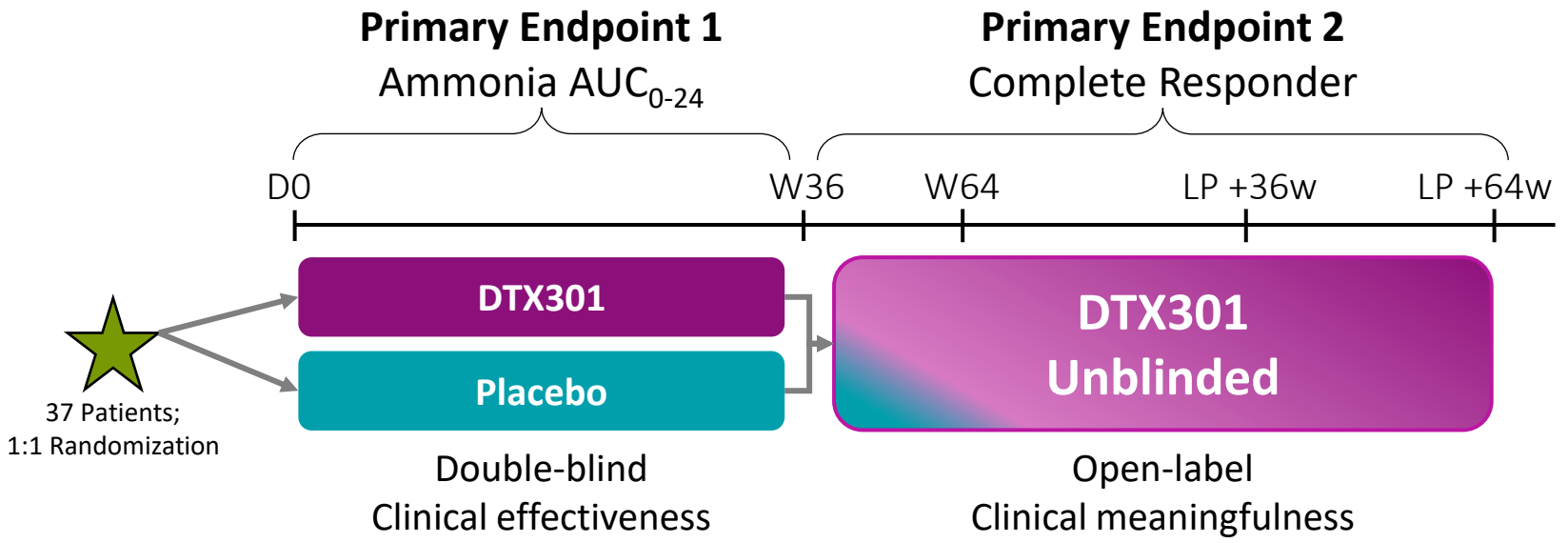
DTX301 showed statistically significant and clinically meaningful 18% reduction in plasma ammonia AUC_{0-24} vs placebo at Week 36 (p=0.018)

Reduction in ammonia sustained with concurrent reductions of scavenger medication and liberalization of protein restricted diet

Improvements in OTCD-related symptoms in feeling and functioning are being reported in DTX301 arm on PGIC

Data disclosed in press release on March 12, 2026

DTX301 for OTC: Phase 3 study schematic



LP = Last Patient receiving unblinded DTX301 after crossover

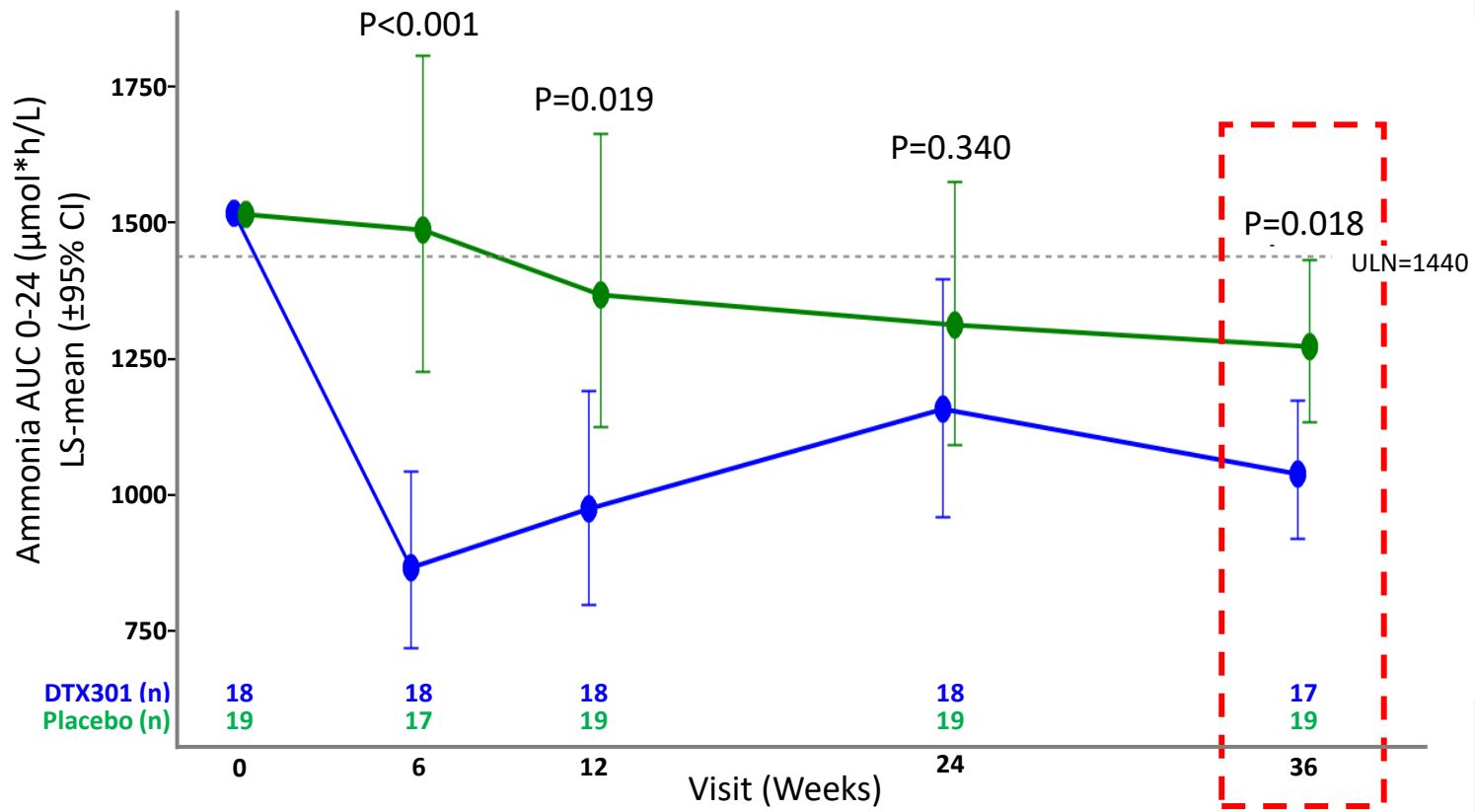
About Phase 3 *Enh3ance*

- Enrollment: 37 patients; ages 12 years of age or older
- Global sites: 16 sites across 10 countries
- DTX301 dose: 1.7 x 10^13 GC/kg
- Primary Endpoint 1: plasma ammonia as measured by 24-hour ammonia (AUC0-24) at Week 36
- Primary Endpoint 2: Complete Responder rate at the final study visit after DTX301 exposure, with patients from both the crossover and treatment groups followed through 64 Weeks of DTX301 exposure

Data disclosed in press release on March 12, 2026

DTX301 for OTC: Primary endpoint of ammonia control showed statistically significant and clinically meaningful improvements versus placebo

At Week 36, statistically significant and clinically meaningful 18% (p=0.018) reduction in ammonia compared to placebo



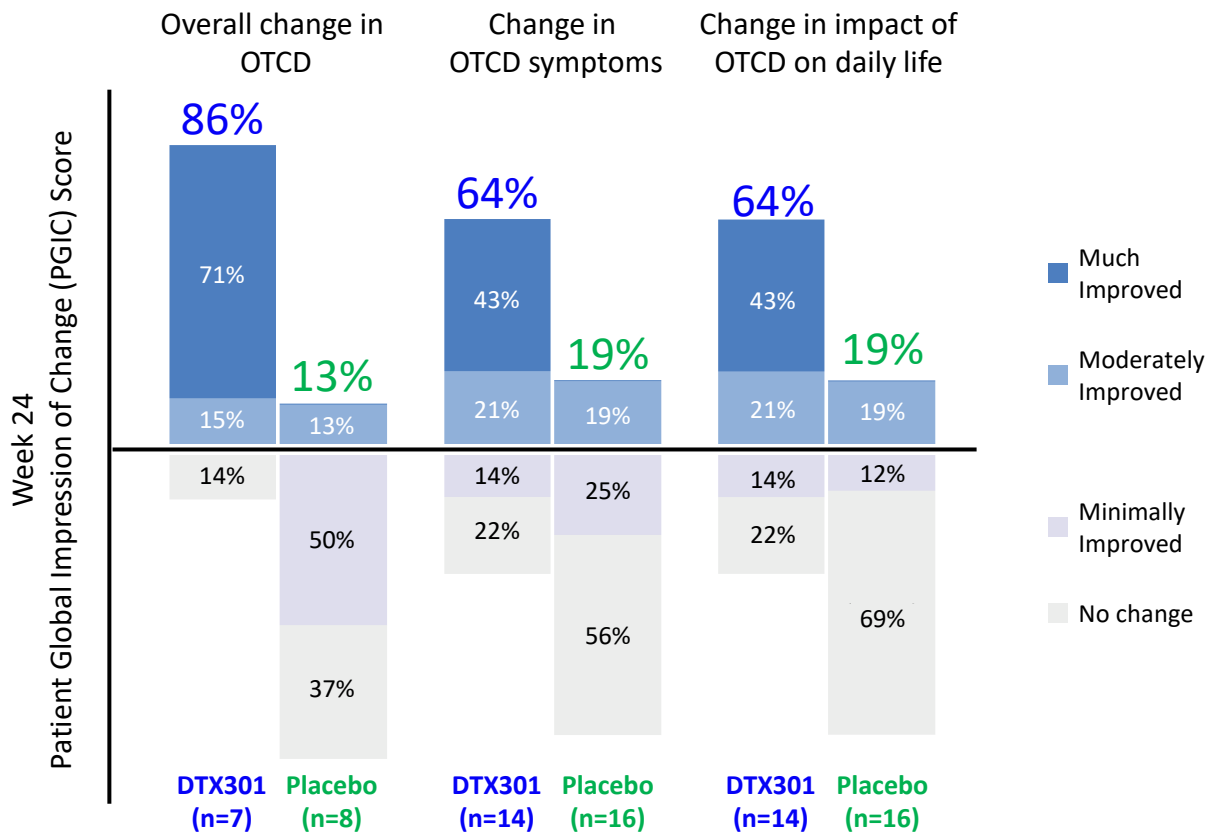
Impact on scavenger medications & protein restricted diet at Wk36

- At baseline, despite being managed by ammonia scavenger medications and diet, 50% of DTX301 patients and 68% of placebo patients had normal ammonia
- At Week 36, in DTX301 patients (n=18), ammonia was generally maintained in normal range despite a mean 27% reduction in scavenger medications and a ~13% increase in protein
- At Week 36, in placebo patients (n=19), no change to baseline scavenger medication or diet

Data disclosed in press release on March 12, 2026

DTX301 for OTC: At Week 24 patients treated with DTX301 showed improvements in feeling and functioning compared to placebo

At Week 24, 86% of DTX301 patients showed clinically important improvement in OTCD vs 13% placebo patients



Clinically important change is moderate improvement (+2) or much improved change (+3)

Change from baseline in Week 24 PGIC scores

DTX301 treated patients

- 86% demonstrated clinically important change in overall OTCD, with 71% much improved

Placebo treated patients

- 87% did not demonstrate clinically important changes in overall OTCD
- Majority reported “no change” on impact to disease, symptoms, or daily life

Data disclosed in press release on March 12, 2026

DTX301 for OTC: Safety / Next steps

- In Phase 3, DTX301 was well tolerated with acceptable safety profile, consistent with Phase 1/2
- Most common treatment-emergent AE: mild to moderate hepatic reactions managed with steroids
- One SAE of acute hepatitis assessed as treatment-related and resolved with steroids
- Hyperammonemic crises were primarily in placebo groups; 6 events (5 placebo and 1 DTX301)
  - 1 patient randomized to placebo died after presenting with grade 5 hyperammonemia with rapid decompensation with rising ammonia levels that were refractory to intensive medical intervention
- No SAEs or AEs related to thrombotic microangiopathy, dorsal root ganglion toxicity, malignancies, or other complex immune reactions

Evaluating Phase 3 complete responder rate, data expected in 1H-2027

Data disclosed in press release on March 12, 2026

GTX-102 for Angelman syndrome (AS)

Antisense oligonucleotide; Phase 3 Aspire data expected in 2H-2026

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

- Enrollment completed in Phase 3 *Aspire* Study in deletion patients
- Enrollment completion in Phase 2/3 *Aurora* study in other genotypes and ages expected in 2H-2026
- Phase 3 *Aspire* data expected in 2H-2026

"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)

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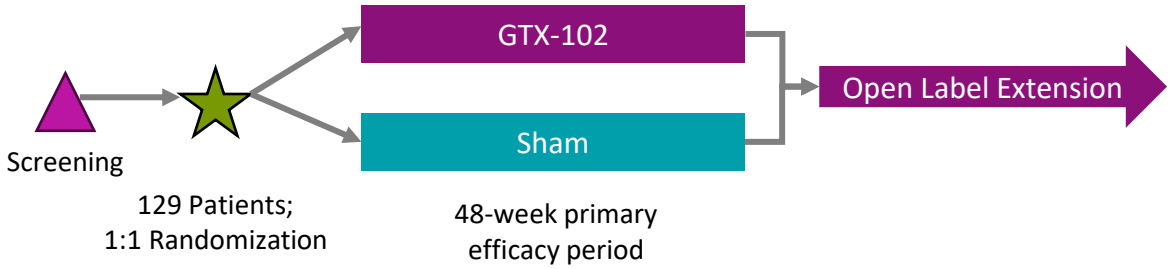
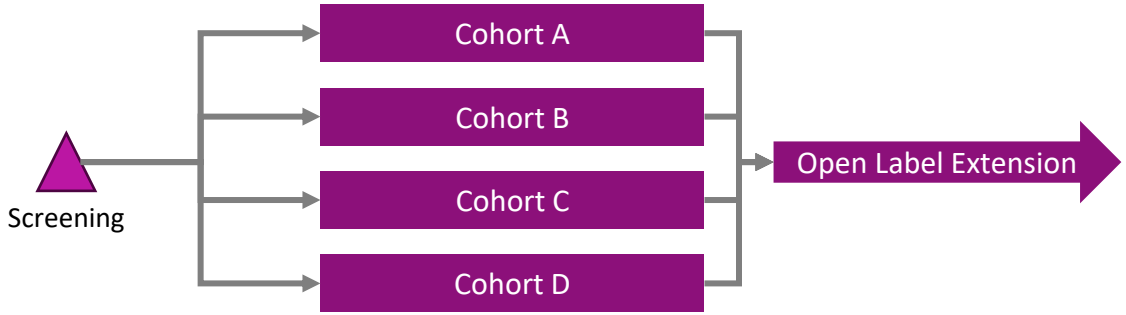
GTX-102 for AS:
Two global studies enrolling broad age range and genotypes

Aspire Phase 3 Sham-Controlled Study



- Randomized, controlled study in deletion patients
- Enrollment: 129 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 Open Label Study



Cohort	Age (years)	Genotype	Primary Endpoint
A	≥1 to <4	Deletion	Bayley-4 Cognitive raw score
B	≥4 to <18	UPD/ICD	MDRI
C	≥18 to <65	All	MDRI
D	≥4 to < 18	Mutation	MDRI

- Global study will enroll ~60 participants
- Open label, 48-week primary efficacy period

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UX701 for Wilson disease (WD)

AAV9 gene therapy; Stage 1, dose finding data expected in 2026

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 completed in August 2025
- Stage 1, dosing finding data expected in 2026
- Manufacturing in-house at our Bedford, MA plant

GT manufacturing facility in Bedford, MA

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

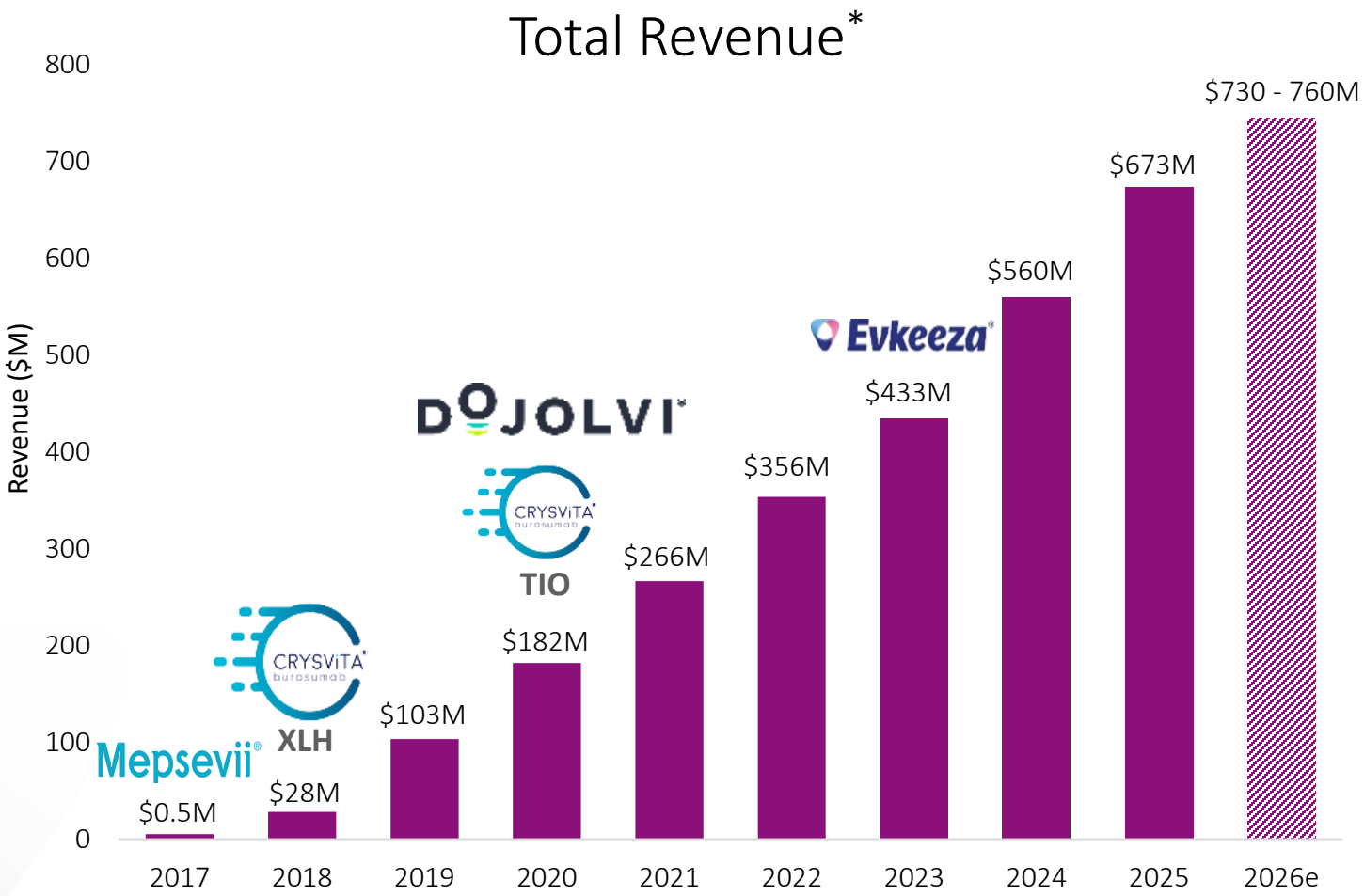
- Clinical activity observed across all three dose cohorts in Stage 1
  - 6 of 15 patients completely off chelators and/or zinc therapy
  - 1 additional patient tapering standard of care
  - In responders, non-ceruloplasmin bound copper (NCC) stabilized to normal, healthy levels
  - Some patients demonstrated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

Enrollment completed in fourth cohort with moderately increased dose and with optimized immunomodulation

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

Financials and 2026 Catalysts

Total revenue grew approximately 20% in 2025



* Excluding Bayer and Daiichi collaboration revenue and potential future launches. Estimate for 2026.

Product	2025 Actuals	2026 Guidance
Total revenue[1]	$673M *20%*	$730-760M *8-13%*
Crysvita[2]	$481M *17%*	$500-520M *4-8%*
Dojolvi	$96M *9%*	$100-110M *4-14%*

1 Total Revenue for 2025 and 2026 includes Crysvita, Dojolvi, Mepsevii, and Evkeeza, and excludes growth from potential new product launches
2 Total Crysvita revenue, including North America, Latin America, and Europe

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Strategic restructuring plan supports path to profitability in 2027

Profitability assumptions

- **Revenue:** Continued growth from current products, plus contribution from upcoming launches
- **Expected combined R&D and SG&A expenses* compared to 2025:**
  - 2026: flat to down low-single digits
  - 2027: decrease at least 15%
    - R&D decrease by ~38%, partially offset by increase in SG&A to support launches and existing approved products
- **Cash:**
  - Planned monetization of PRVs from UX111 and DTX401
  - $534M in cash and investments as of March 31, 2026

*Assumptions reflect net impact from the strategic restructuring plan, severance and other one-time non-recurring restructuring costs, and targeted launch investments in UX111 and DTX401

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Key clinical and regulatory catalysts

PROGRAM	OBJECTIVE	ANTICIPATED TIMING
DTX401 GSDIa	BLA accepted PDUFA decision date	✓ August 23, 2026
UX111 Sanfilippo syndrome	BLA accepted PDUFA decision date	✓ September 19, 2026
GTX-102 Angelman syndrome	Phase 3 *Aspire* data Phase 2/3 *Aurora* enrollment completion	2H-2026 2H-2026
UX701 Wilson disease	Stage 1 dose finding data (Cohorts 1-4)	2026
DTX301 OTC deficiency	Phase 3 ammonia analysis Phase 3 complete responder analysis	✓ 1H-2027
UX016 GNE Myopathy	Phase 1/2 IND clearance Phase 1/2 FPI	✓ 2H-2026

We are leading the future of rare disease with first ever treatments

History of consistent and ongoing revenue growth from base business

Near-term catalysts from 5 Phase 2/3 programs and 2 potential approvals

Revenue growth and new launches plus expense management to reach 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 (2029)[1] • Mid single-digit royalty
	N/A (IP Owned by Ultragenyx)	• Ultrapure triheptanoin and use in treatment of FAOD (2034)

Product	License	Europe Intellectual Property Rights/Royalties + Milestones
EVKEEZA® (HOFH)	Regeneron	• Evkeeza antibody and use for treatment of HOFH (2036)[2] • Treatment of HOFH-associated atherosclerosis and use in combination with LDL-C lowering regimen (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 granted extensions via supplementary protection certificates (SPCs)

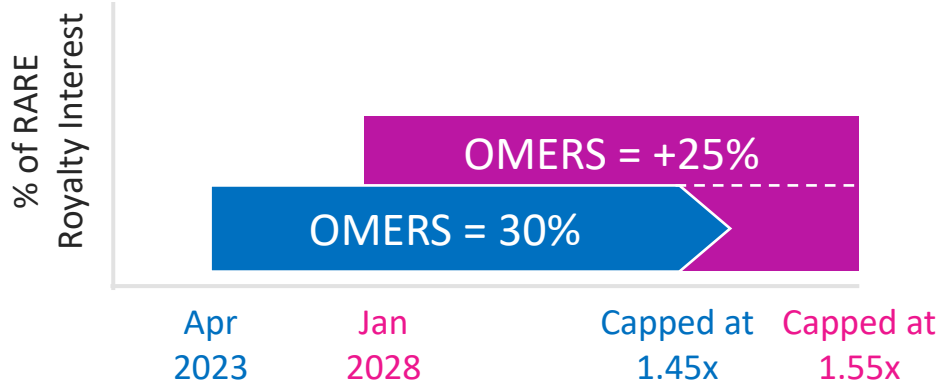
Key licenses & intellectual property – clinical programs

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 for treatment of MPS IIIA (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)	UPENN	• Recombinant vectors comprising certain regulatory and coding sequences packaged in UX701 (2039) • Development milestones 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 (2042)
GTX-102 (Angelman Syndrome)	Texas A&M University	• UBE3A-ATS antisense oligonucleotides including GTX-102 and their use in 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
UX016 (GNE Myopathy)	N/A (IP Owned by Ultragenyx)	• Sialic acid prodrugs including UX016 and their use in treatment of GNE Myopathy (2032) • Crystalline forms of sialic acid prodrugs including UX016 (Pending; 2044)

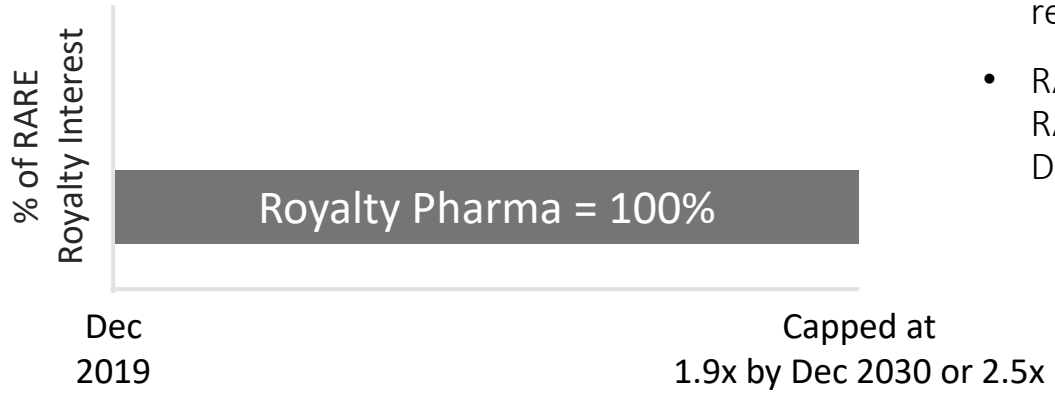
Crysvita partnership revenue recognition

	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

Illustration of Crysvita Royalty Monetization



- RARE receives annual tiered royalty up to 30% on KKC's U.S./Canada revenue
- RARE received $500M from OMERS for 30% of RARE royalties up to 1.45x
  - Payments began April 2023
- RARE received $400M from OMERS for addtl 25% of RARE royalties and an extension of prior deal after its cap is hit up to a total of 1.55x
  - Payments to OMERS begin in January 2028



- RARE receives 10% royalty on KKC's European revenue
- RARE received $320M cash from RP for 100% of RARE royalties up to 1.9x by December 2030 or 2.5x
  - Payments began January 2020

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CRYSVITA® exclusivity summary

XLH Orphan Exclusivity: 2025

TIO Orphan Exclusivity: 2027

Biologics Exclusivity: 2030

Crysvita CoM Patent: 2032*

Q2W Dosing Patent: 2035

2018 2020 2022 2024 2026 2028 2030 2032 2034 2036

2028: XLH Orphan + D&M Exclusivity

2033*: Crysvita CoM Patent

2035: Q2W Dosing Patent

*Includes US PTE and EU SPC awards — Regulatory Exclusivity — Issued Patents

DOJOLVI® exclusivity summary

NCE Exclusivity: 2025

LC-FAOD Orphan Exclusivity: 2027

Dojolvi CoM Patent: 2029*

Ultrapure Dojolvi Patent: 2034

2018 2020 2022 2024 2026 2028 2030 2032 2034 2036

2034

Ultrapure Dojolvi (Pending)

*Includes US PTE award — Regulatory Exclusivity — Issued Patents — Pending Patent Applications

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

Regulatory Exclusivity Issued Patents

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EVKEEZA® exclusivity summary

Evkeeza® (evinacumab-dgnb) Injection

*Includes EU SPC award — Regulatory Exclusivity — Issued Patents — Pending Patent Applications

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UX143 (setrusumab) for Osteogenesis Imperfecta

Data presented at JP Morgan Healthcare Conference 2026

UX143 for osteogenesis imperfecta Phase 3 results

Neither study achieved primary endpoint of reduction in AFR[1] compared to placebo (*Orbit*) or bisphosphonates (*Cosmic*)

Both studies demonstrated statistically significant increases in bone mineral density (BMD)

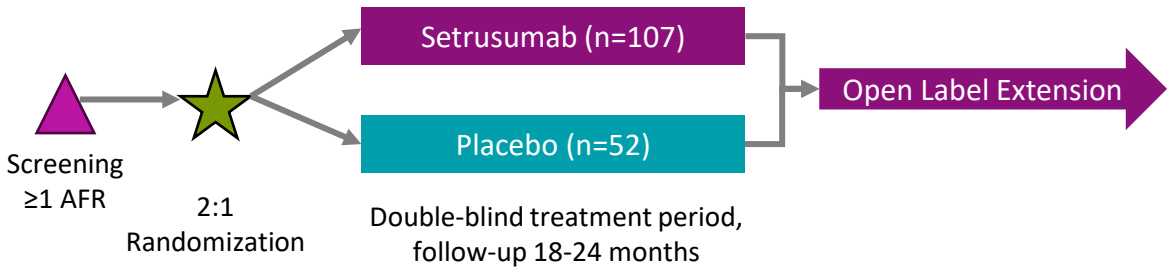
Additional data shows reduction in vertebral fractures and improvements in patient reported outcomes of disease severity, pain/comfort, and daily activities

Further understanding will help determine if there is a potential path forward

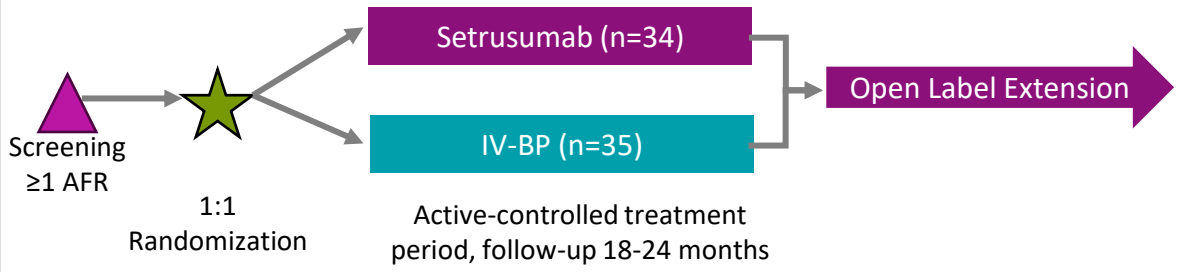
1: Annualized fracture rate

Two randomized Phase 3 studies provide large data set controlled with placebo or active

Orbit Phase 3 Placebo-Controlled Study Design



Cosmic Phase 3 Active-Controlled Study Design



Orbit Phase 3 Enrollment (2:1)

	Setrusumab (%)	Placebo (%)
Total n	**107 (67.3)**	**52 (32.7)**
Type 1	43 (40.2)	21 (40.4)
Type 3	43 (40.2)	10 (19.2)
Type 4	21 (19.6)	21 (40.4)
Peds 5 to <12 yo	44 (41.1)	23 (44.2)
Teens 12 to <18 yo	47 (43.9)	21 (40.4)
Adults 18 to 26 yo	16 (15.0)	8 (15.4)

Cosmic Enrollment (1:1)

	Setrusumab (%)	IV-BP (%)
Total n	**34 (49.3)**	**35 (50.7)**
Type 1	12 (35.5)	16 (45.7)
Type 3	15 (44.1)	13 (37.1)
Type 4	7 (20.6)	6 (17.1)
Peds 2 to 7 yo	34 (49.3)	35 (50.7)

Baseline fractures are comparable between groups in both studies
Orbit: more severe type III/IV patients exited placebo via rescue criteria

Orbit Phase 3 Baseline Historical Fractures[1]		
	Setrusumab	**Placebo**
Mean / Median Number of fractures	3.2 / 2.0	3.3 / 2.0
Fracture ≤3 Patients Number (%)	71 (66.4)	35 (67.3)
Fracture >3 Patients Number (%)	36 (33.6)	17 (32.7)

1: All suspected and radiographically confirmed fractures over prior 2 years

Cosmic Phase 3 Baseline Historical Fractures[1]		
	Setrusumab	**IV-Bisphos.**
Mean / Median Number of fractures	4.1 / 4.0	4.3 / 3.0
Fracture ≤4 and no FTH Patients Number (%)	4 (11.8)	4 (11.4)
Fracture >4 or ≥1 FTH Patients Number (%)	30 (88.2)	31 (88.6)

1: All suspected and radiographically confirmed fractures over prior 2 years
FTH = Femur, Tibia or Humerus

In Orbit, 31 (19.5%) patients met rescue criteria at 12 months primarily due to fractures

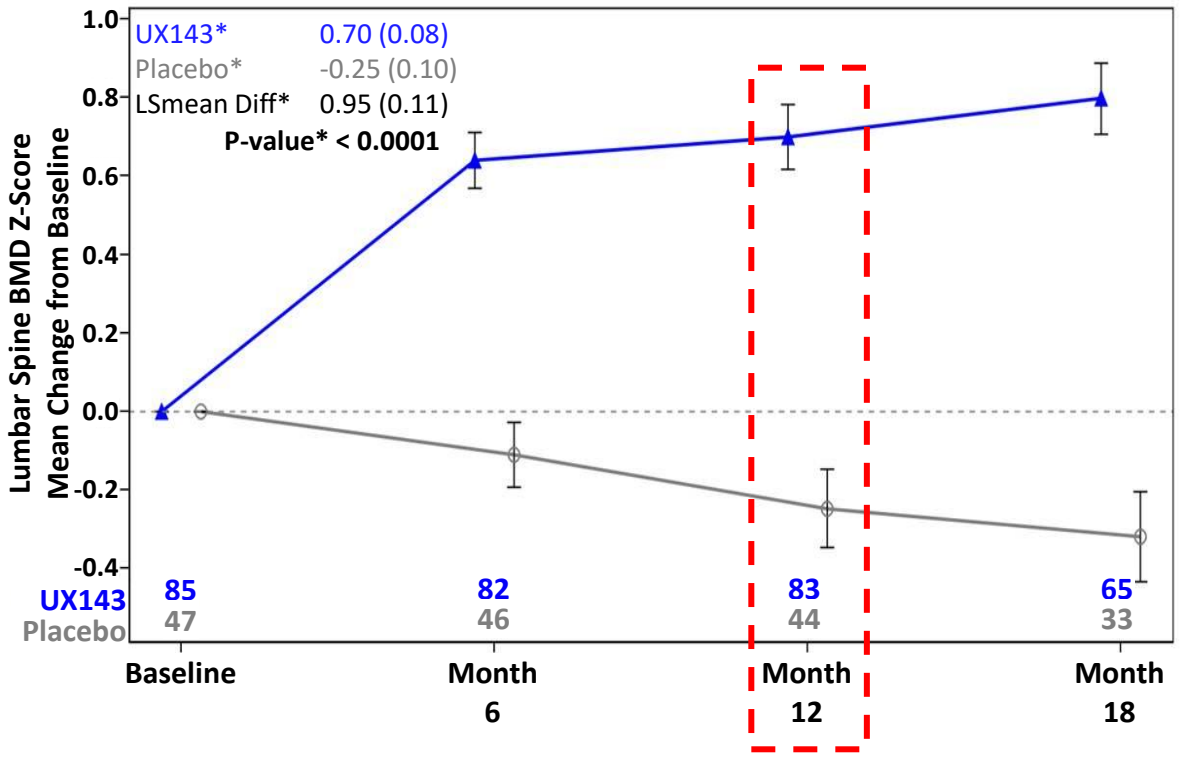
- 28 of 31 were more severe Type 3/4 patients
  - UX143 15/64 (23%)
  - **Placebo 13/31 (42%)**

A substantially larger number of Placebo patients exited Orbit

Cosmic had no rescue criteria since it was active treatment controlled

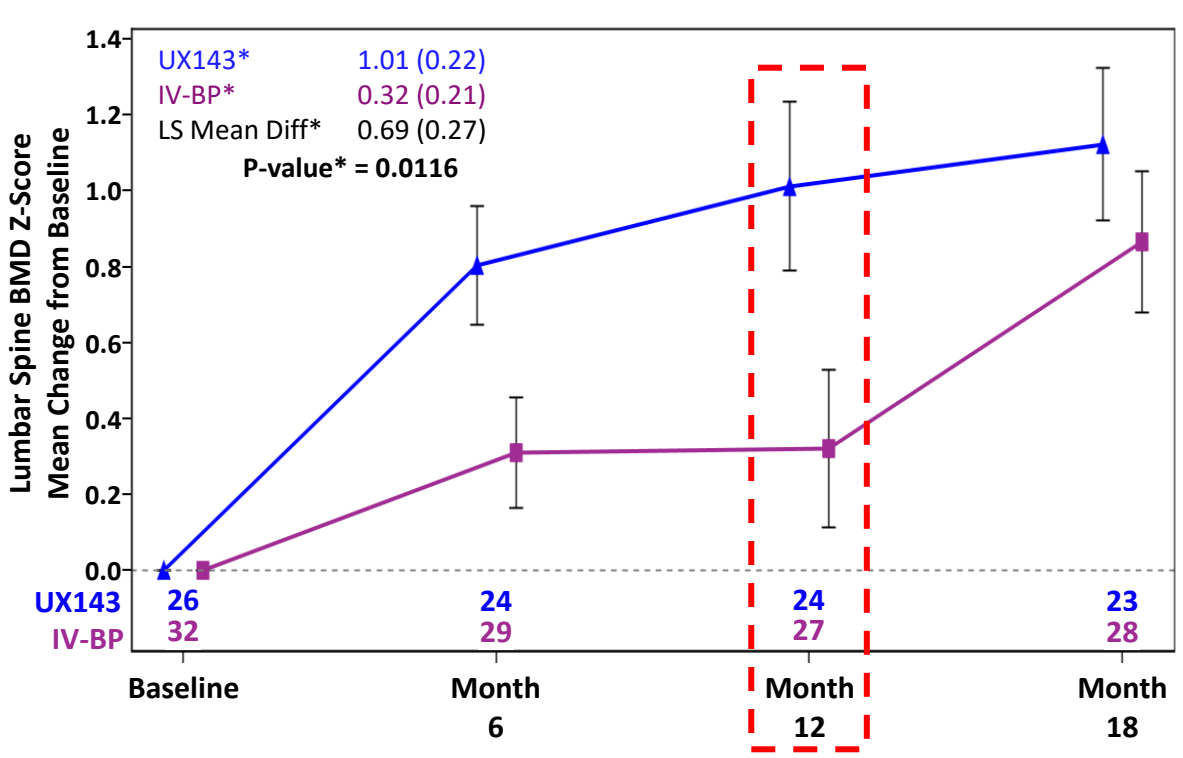
Setrusumab is substantially more effective in increasing BMD

Orbit: UX143 demonstrated clinically and statistically significant increases in BMD vs placebo



*Based on Month 12 timepoint

Cosmic: UX143 demonstrated clinically and statistically significant increases in BMD vs active comparator

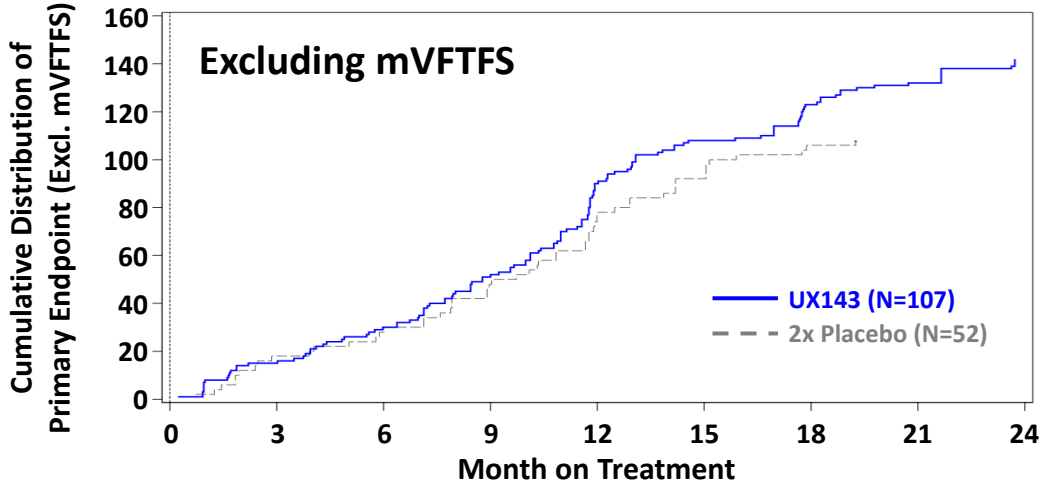


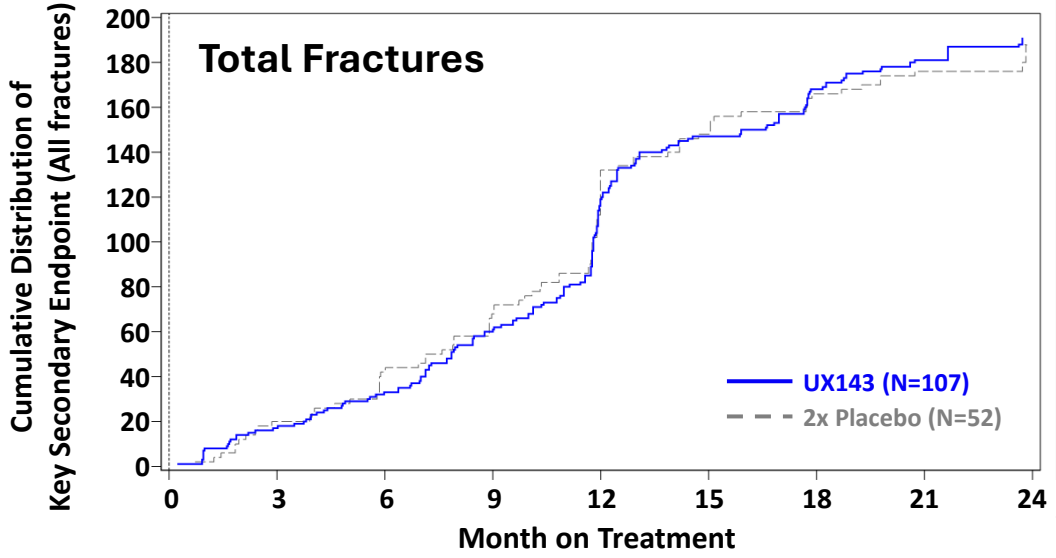
*Based on Month 12 timepoint

Orbit: UX143 patients showed an increase in fractures over a low placebo rate, but were the same as placebo when all fractures were considered (p=ns)

Confirmed fractures by x-ray and skeletal survey		**Primary Endpoint**[1] Excl. mVFTFS	**Key Secondary** All Fractures
UX143 AFR (n=107)	# of fractures	142	191
	Mean (SD, SE)	0.92 (1.16, 0.11)	1.22 (1.29, 0.12)
	Median (Q1, Q3)	**0.58** (0.00, 1.53)	0.68 (0.00, 1.82)
Placebo AFR (n=52)	# of fractures	54	94
	Mean (SD, SE)	0.80 (1.48, 0.21)	1.27 (1.96, 0.27)
	Median (Q1, Q3)	**0.00** (0.00, 0.93)	0.61 (0.00, 2.02)
Estimated[2] UX143 AFR (95% CI)		**0.71** (0.50, 0.99)	1.16 (0.90, 1.50)
Estimated[2] Placebo AFR (95% CI)		**0.55** (0.35, 0.86)	1.12 (0.80, 1.57)
Rate Ratio[2] UX143/Placebo (95% CI)		1.28 (0.80, 2.06)	1.03 (0.71, 1.52)
Rate Change[2] UX143 Placebo (95% CI)		**28.14** (-20.21, 105.79)	**3.38** (-29.48, 51.54)
P-value[2]		0.305	0.865

[1] Radiographically confirmed fractures, excluding morphometric vertebral fractures and fingers, toes, face, and skull
[2] Negative Binomial model

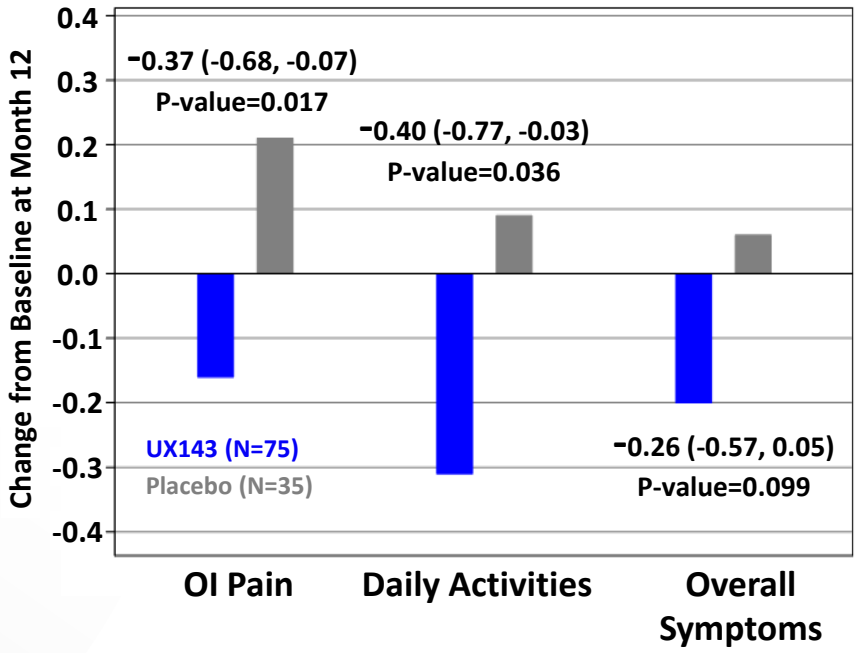




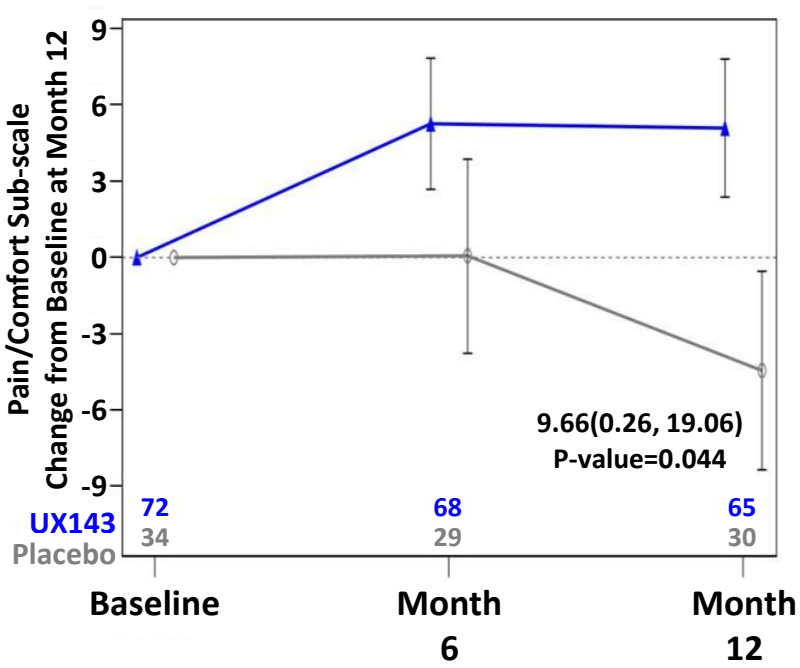
Orbit: In setrusumab patients, disease severity (PGIS) in peds/teens **reduced** and pain/comfort & sports/activity **improved**

Peds/Teens patients constitute 85% of subjects in Orbit Ph3 study (135/159)

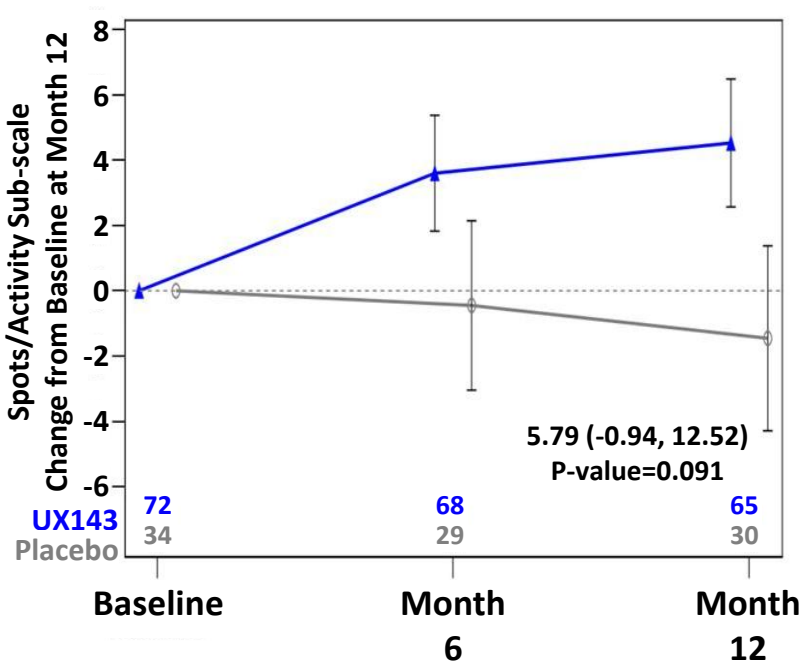
Patient Global Impression Scale of Severity (PGIS)



Pain/Comfort
POSNA-PODCI



Sports/Activity
POSNA-PODCI



12 month assessment is as randomized and most important as no patients had exited due to rescue criteria

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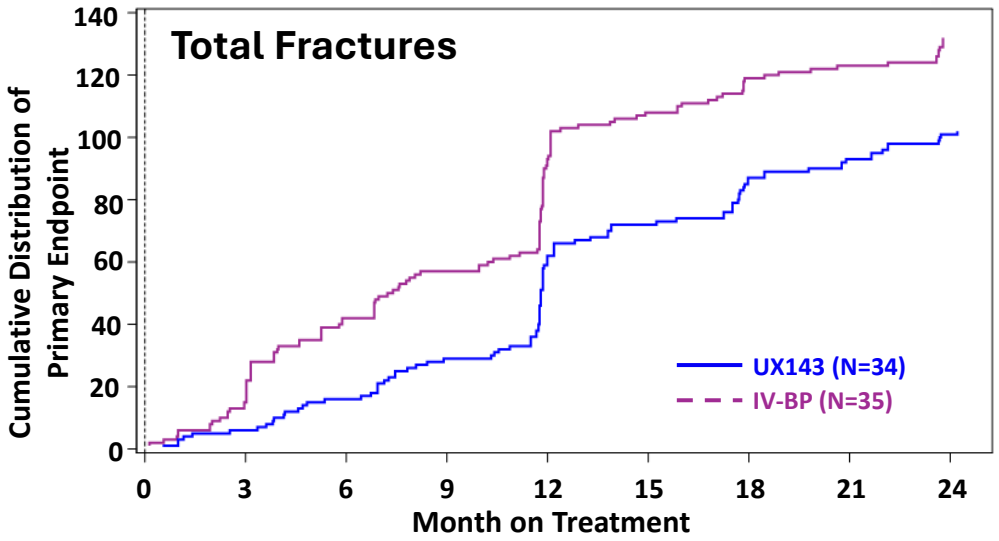
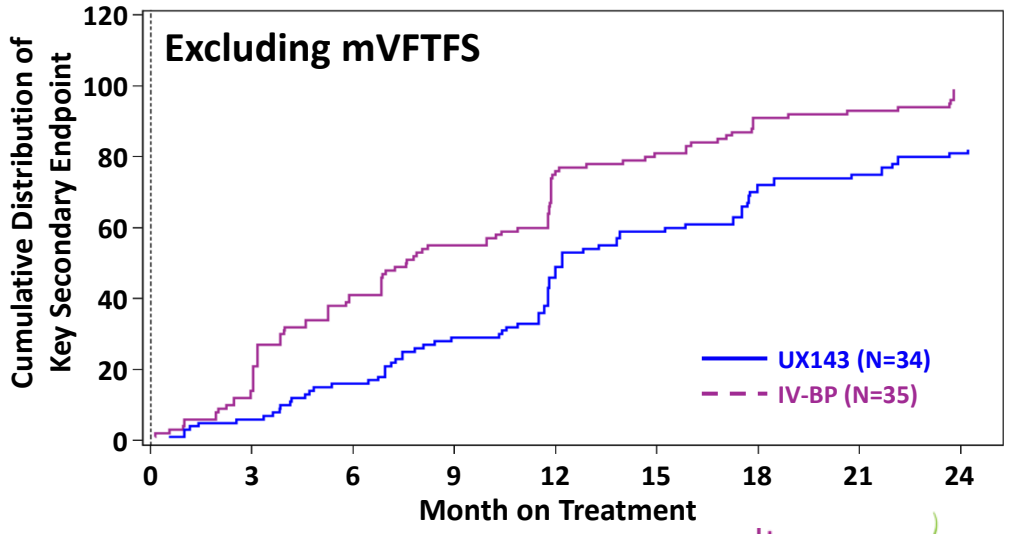
Cosmic: Setrusumab treatment shows reduced fractures over IV-BP (p=ns)

Confirmed fractures by x-ray and skeletal survey		Primary Endpoint Total fractures	Key Secondary[1] Excl. mVFTFS
UX143 AFR (n=34)	# of fractures	102	82
	Mean (SD, SE)	1.87 (1.69, 0.29)	1.53 (1.53, 0.26)
	Median (Q1, Q3)	2.02 (0.00, 3.04)	1.42 (0.00, 2.53)
IV-BP AFR (n=35)	# of fractures	132	99
	Mean (SD, SE)	2.6 (3.19, 0.54)	1.97 (2.90, 0.49)
	Median (Q1, Q3)	1.38 (0.55, 4.06)	0.67 (0.00, 3.04)
Estimated[2] UX143 AFR (95% CI)		**0.91** (0.51, 1.60)	0.68 (0.34, 1.35)
Estimated[2] IV-BP AFR (95% CI)		**1.15** (0.65, 2.04)	0.79 (0.39, 1.61)
Rate Ratio[2] UX143/IV-BP (95% CI)		0.79 (0.48, 1.28)	0.86 (0.47, 1.57)
Rate Change[2] favoring UX143 (95% CI)		**-21.27** (-51.75, 28.47)	**-14.27** (-53.07, 56.61)
P-value[2]		0.338	0.616

[1] Radiographically confirmed fractures, excluding morphometric vertebral fractures and fingers, toes, face, and skull
[2] Negative Binomial model





Cosmic: Large reduction in vertebral fractures on setrusumab (p-value 0.081)

Despite MORE severe type III/IV patients on UX143 (65% UX143 vs 54% to IV-BP)

Cosmic Phase 3	Radiographically confirmed fractures			
	Total fractures All types		Vertebral Fractures	
	UX143	IV-BP	UX143	IV-BP
All fractures	102	132	19	46
All fractures (Excluding mV[1])	84	104	1	18
All fractures (Excluding mVFTFS[2])	82	99	1	18
mVertebral fractures (Tertiary endpoint)	18	28	18	28

1: Morphometric vertebral fractures 2: Morphometric vertebral fractures and fingers, toes, face, and skull

Radiographically Confirmed Fracture Type (comparing 19 vs 46 vertebral fractures*)		All Vertebral Fractures
Negative Binomial Model (95% CI)	Est UX143 AFR (95% CI)	0.14 (0.04, 0.51)
	Est IV-BP AFR (95% CI)	0.33 (0.10, 1.12)
	Ratio UX143/IV-BP (95% CI)	0.44 (0.18, 1.11)
	Rate Change favoring UX143 (%) (95% CI)	-56.00 (-82.48, 10.53)
	P-value	0.081

Radiographically Confirmed Fracture Type (Tertiary endpoint)		Morphometric Vertebral Fractures, Only
Negative Binomial Model (95% CI)	Est UX143 AFR (95% CI)	0.15 (0.04, 0.51)
	Est IV-BP AFR (95% CI)	0.24 (0.07, 0.79)
	Ratio UX143/IV-BP (95% CI)	0.64 (0.26, 1.61)
	Rate Change favoring UX143 (%) (95% CI)	-35.87 (-74.43, 60.86)
	P-value	0.344

* Post hoc analysis

Setrusumab 59% fewer vertebral fractures of all types

Setrusumab 94% fewer non-morphometric vertebral fractures

Data presented at JP Morgan Healthcare Conference 2026

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No new safety concerns identified, reported TEAEs are consistent with the anticipated safety profile for setrusumab

Orbit*

Treatment emergent adverse events (TEAE)

- No serious-related TEAE's
- Low incidence (<2%) severe-related TEAE's
- Low incidence (<3%) TEAE's leading to treatment or study discontinuation

Adverse events of special interest (AESI)

- No ischemic CV Events
- No hypersensitivity reactions related to UX143
- 1 TEAE in neurologic effect due to bony overgrowth
  - Radial nerve injury following a surgical procedure

No Deaths

*Data presented above is representative of setrusumab arm only

Cosmic*

Treatment emergent adverse events (TEAE)

- No serious related TEAE's
- Low incidence (<3%) severe related TEAE
- No TEAE's leading to treatment discontinuation or study discontinuation

Adverse events of special interest (AESI)

- No ischemic CV events
- No hypersensitivity reactions related to UX143
- No neurologic sequalae due to bony overgrowth

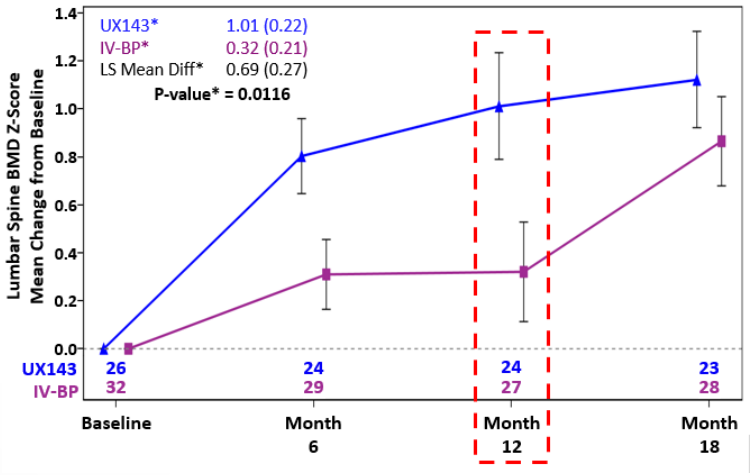
No Deaths

*Data presented above is representative of setrusumab arm only

Overall data suggest an impact of setrusumab on OI disease but missed primary AFR endpoints are a challenge

The largest BMD improvements found in the lumbar spine BMD are associated with reduced vertebral fractures and improved pain and functional outcomes in pediatric patients

Improved Lumbar Spine BMD



Reduced Vertebral fractures

	UX143	IV-BP
All fractures	19	46
All fractures (Excluding mV[1])	1	18

1: Morphometric vertebral fractures

Improved functional outcomes

- Decreased bone pain
- Improved functional ability
- Improved walking ability

Further understanding will help determine if there is a potential path forward