Corporate Presentation

November 2024

Forward Looking Statements

Cautionary note regarding forward-looking statements: This presentation contains forward-looking statements, including, but not limited to, statements regarding our expectations and projections regarding our future operating results and financial performance, anticipated cost or expense reductions, plans with respect to commercializing our product and product candidates, our translational research program, expectations regarding our manufacturing capabilities, the expected timing of release of additional data for our product candidates, plans to initiate additional studies for product candidates and timing and design of these studies, plans regarding ongoing studies for existing programs, our liquidity position as of the most recent fiscal quarter end, expectations regarding the adequacy of clinical data to support marketing applications and approvals of product candidates, our intent to file, and potential timing and success of, marketing applications and other regulatory approvals, expectations regarding timing of receiving potential approval of product candidates, expectations regarding prevalence of patients, future regulatory interactions, and the value to be generated by our pipeline. Such forward-looking statements involve substantial risks and uncertainties that could cause our clinical development programs, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, our reliance on our third party partner, Kyowa Kirin Co., Ltd., for the supply of Crysvita, fluctuations in buying or distribution patterns from distributors and specialty pharmacies, the transition back to Kyowa Kirin of our exclusive rights to promote Crysvita 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, our actual results may materially differ from our current expectations, estimates, and projections. Given these uncertainties, you should not place undue reliance on these forward-looking statements.

Any forward-looking statements made by us in this presentation speak only as of the date of this presentation and represent our estimates and assumptions 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.

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.

Most Productive Rare Disease Company in the Industry

4 products across **5** indications approved in **10** years

CRYSVITA® burosumab

DOJOLVI®

Evkeeza®

Mepsevii®

Largest clinical pipeline in rare disease

6 late-stage studies

4 modes targeting cause of disease

ultragenyx

Product Approvals Since IPO Exceed Other Successful, Rare Disease Companies

	Years from IPO to 1st approval[1]	# of approvals[1] 10y post-IPO	# of approvals[1] 15y post-IPO
ultragenyx	**3**	**5**	**Up to 8-12***
BioMarin	4	3	5
Genzyme	5	2	3
Alexion	11	0	2
Alnylam	14	0	2
Vertex	21	0	0

1 *Approvals for rare disease indications*
* *Potential based on current pipeline*

Keys to Our Success:
Experienced team focused on innovation, speed, and execution

Deep scientific understanding increases probability of success

82% demonstrated clinical success[1]

Utilize **innovative regulatory** and **development** approaches

Approved products based on **novel trial designs** and **endpoints**

Among the fastest development in the industry

Avg 5.5 yrs from clinic to approval[1]

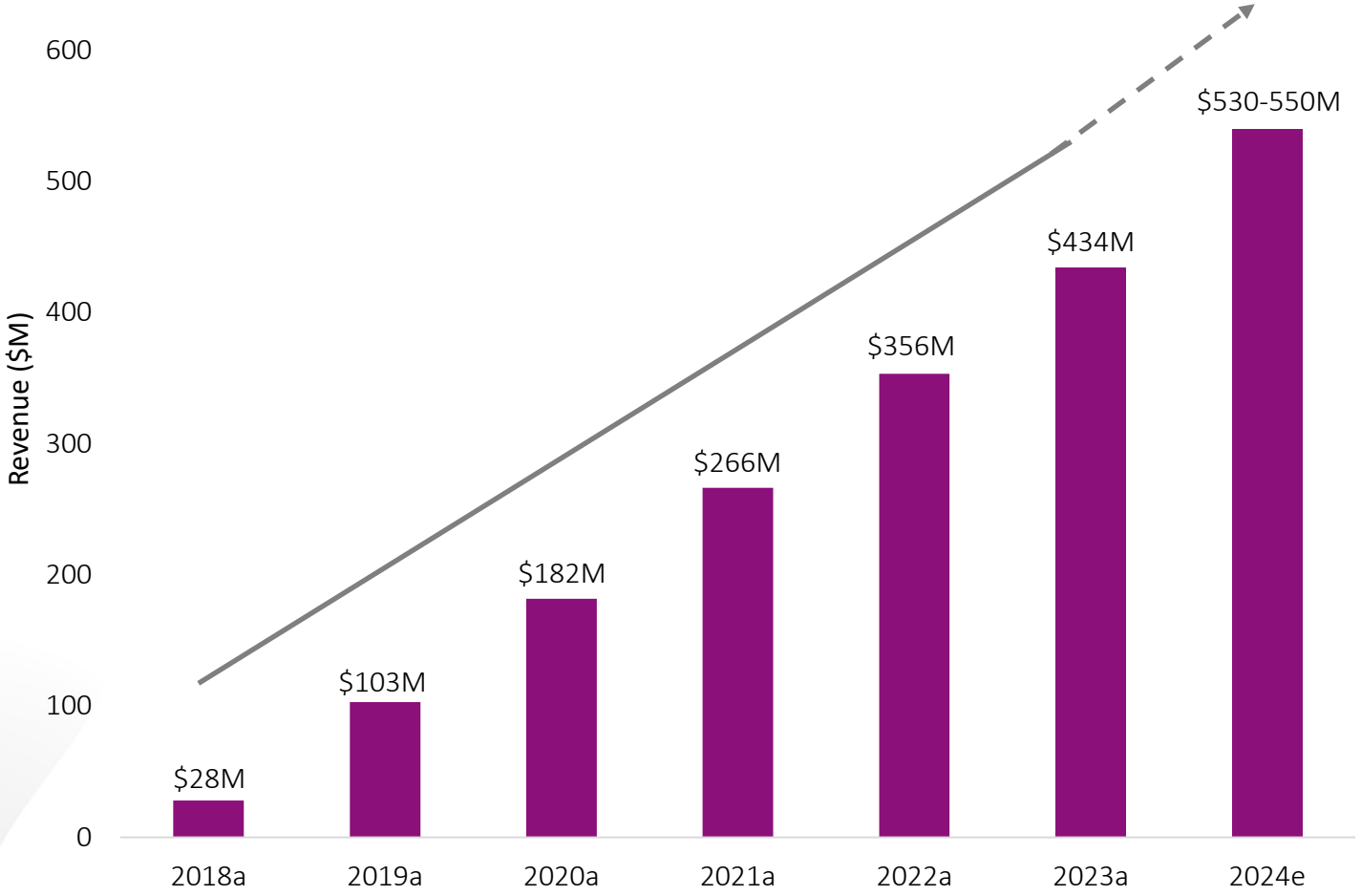
Global commercial organization

Treating patients in **~34 countries**

1 Clinical success and approvals to date (June 2024)

2024 Revenue Now Expected to Grow 22-27% driven by Crysvita in the U.S. and Latin America

Annual Revenue Growth[1]



1 Excludes Bayer and Daiichi collaboration revenue and includes estimate for 2024

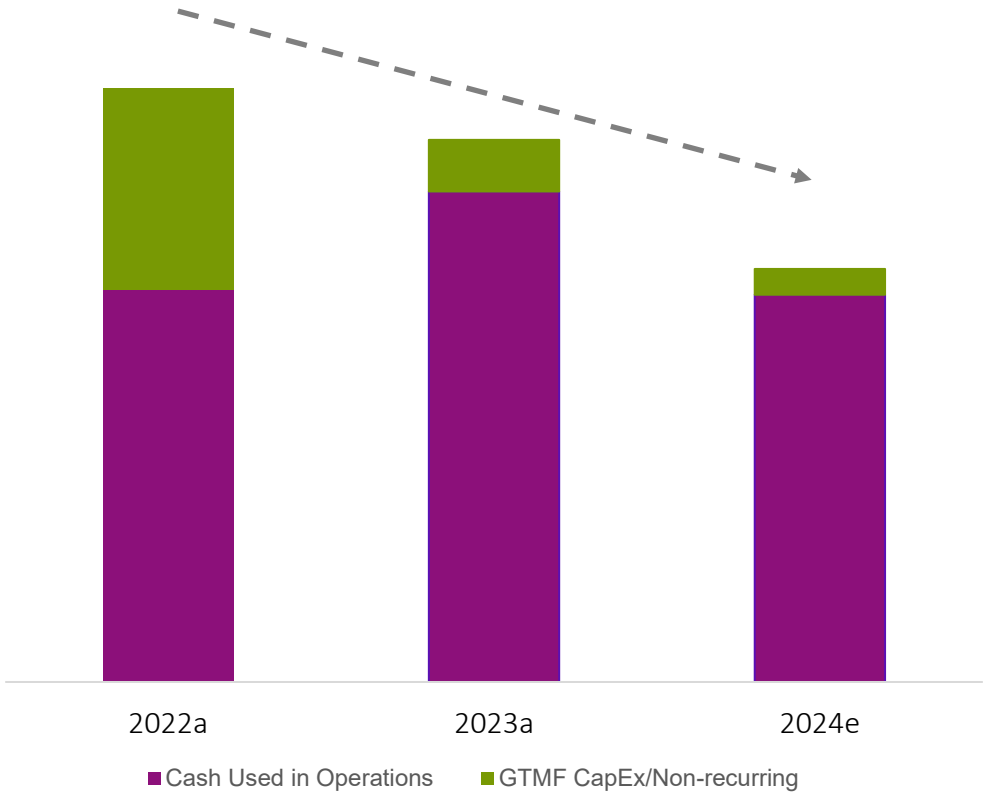
Product	2023 Actual	2024 Guidance[3]
Crysvita[1]	$328M	$375-400M *14% to 22% growth*
Dojolvi	$71M	$75-80M *6% to 13% growth*
Total Revenue[2]	$434M	$530-550M *22% to 27% growth*

1 Total Crysvita revenue, including North America, Latin America, and Europe
2 Total Revenue includes Crysvita, Dojolvi, Mepsevii, and Evkeeza
3 As provided on November 5, 2024

Capital Allocation Focused on Key Clinical and Commercials Programs

Net cash used in operations is declining as revenue grows

Uses of Cash[1]



Declining YoY Cash Used in Operations expected to be **around $400M in 2024**

Ongoing revenue growth and continued expense management create **path to profitability in 2026**

Cash and equivalents[2] of **$825M** as of September 30, 2024

1 Cash used in operations, Gene Therapy Manufacturing Facility (GTMF) Capital Expenses and select non-recurring uses of cash; estimated values for 2024
2 Cash, cash equivalents, and marketable debt securities as of September 30, 2024

Focused on Three Therapeutic Areas

DEVELOPMENT

COMMERCIAL

BONE ENDOCRINE

Ph 3

UX143
Osteogenesis Imperfecta

CRYSVITA® burosumab

XLH

CRYSVITA® burosumab

TIO

INBORN ERRORS OF METABOLISM

Ph 2

UX701
Wilson

Ph 3

DTX301
OTC

Ph 3

DTX401
GSDIa

Ph 3

UX111
MPS IIIA

Mepsevii™ (vestronidase alfa-vjbk) injection

MPS VII

DOJOLVI® TRIHEPTANOIN Oral Liquid

LC-FAOD

Evkeeza® (evinacumab-dgnb) Injection

HoFH

CNS / MUSCLE

Ph 2

GTX-102
Angelman Syndrome

Preclinical
(Disclosed)

UX055
CDKL5

UX810
Duchenne

ultragenyx

Diverse Commercial and Clinical Pipeline

Candidate	Description	Pre-Clinical / IND / Phase 1 / Phase 2 / Phase 3 / Approved	Prevalence[1]
Kyowa Kirin CRYSVITA®	Anti-FGF23 Monoclonal Antibody	X-Linked Hypophosphatemia (XLH) & Tumor-Induced Osteomalacia (TIO)	~50,000
Mepsevii	Enzyme Replacement	Mucopolysaccharidosis Type VII (MPS VII)	~200
Regeneron Evkeeza® [2]	Anti-ANGPTL3 Monoclonal Antibody[2]	Homozygous Familial Hypercholesterolemia (HoFH)	~3,000 – 5,000[3]
Mereo Biopharma **UX143 (setrusumab)**	Anti-Sclerostin Monoclonal Antibody	Osteogenesis Imperfecta (OI)	~60,000
DOJOLVI®	Substrate Replacement	Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD)	~8,000 – 14,000
UX111 (ABO-102)	AAV9 Gene Therapy	Sanfilippo Syndrome (MPS IIIA)	~3,000 – 5,000
DTX401	AAV8-G6Pase Gene Therapy	Glycogen Storage Disease Type Ia (GSDIa)	~6,000
DTX301	AAV8-OTC Gene Therapy	Ornithine Transcarbamylase (OTC) Deficiency	~10,000
UX701	AAV9-ATP7B Gene Therapy	Wilson Disease (WD)	~50,000
UX055	AAV9 Gene Therapy	CDKL5 Deficiency Disorder	~20,000 – 30,000
UX810	Microdystrophin Gene Therapy	Duchenne Muscular Dystrophy	~40,000
GTX-102	Antisense Oligonucleotide	Angelman Syndrome (AS)	~60,000

1: Prevalence in commercially accessible geographies
2: Ultragenyx licensed ex-US rights to Evkeeza from Regeneron
3: Excludes the US, where Regeneron has rights

Key: Protein Biologic | Small Molecule | Gene Therapy | Nucleic Acid

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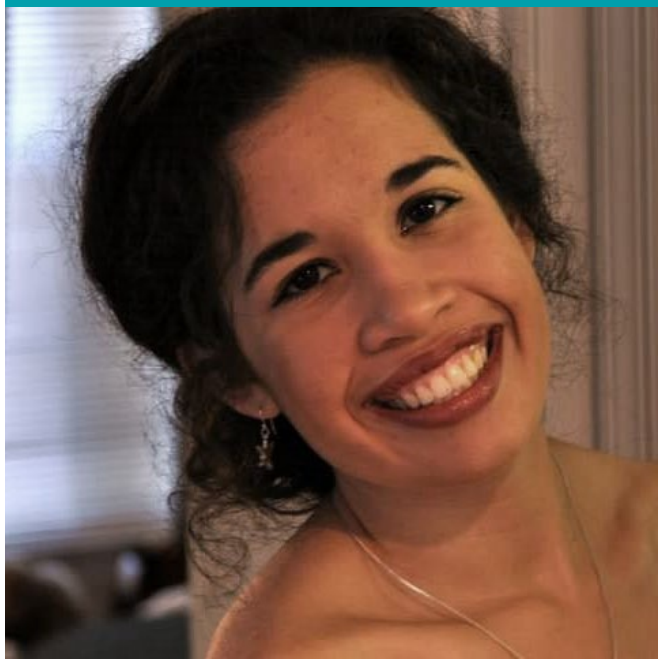
Three Opportunities for Significant Near-term Value Creation

Osteogenesis Imperfecta

Angelman Syndrome

Wilson Disease



UX143 for Osteogenesis Imperfecta (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

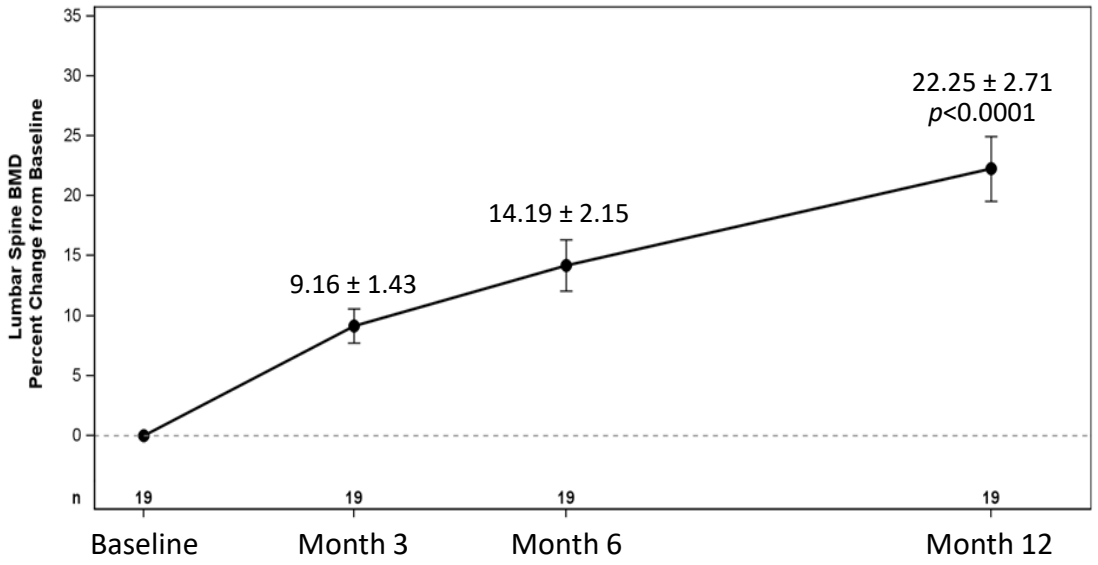
- Purpose: Replicate strong Ph2 data in a larger, placebo controlled study to support BLA filing
- Enrollment: 5-25 y/o; N=159; OI Type 1, 3, 4; randomized 2:1 UX143 to placebo

- Primary Endpoint: Annualized Fracture Rate[2]
- Phase 3 Readout:
  - Interim Analysis (1 or 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

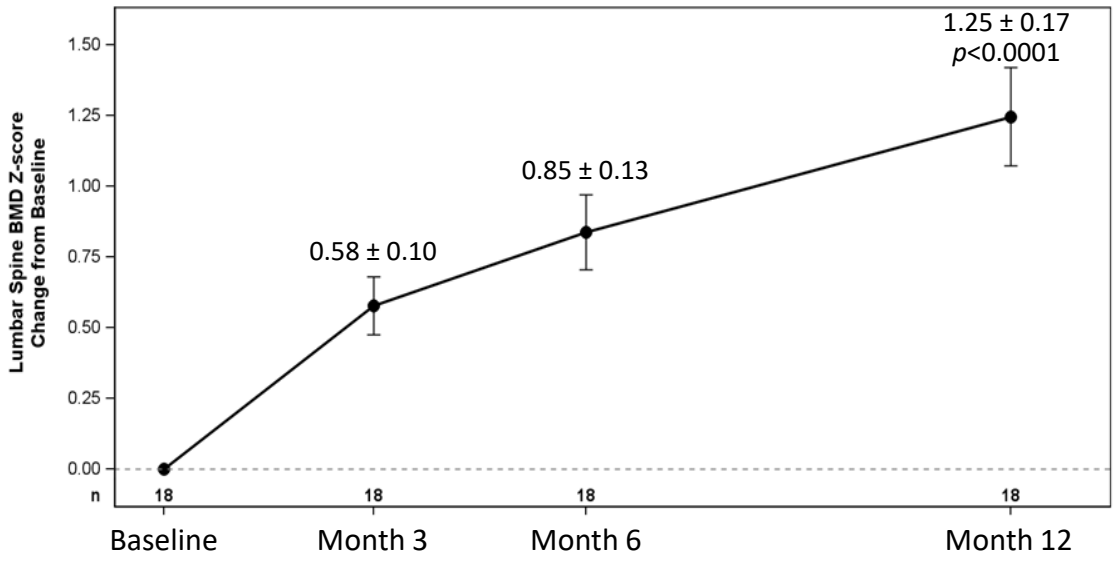
UX143 for OI: Phase 2 Data Demonstrated Increase in Lumbar Spine BMD and Z-score Observed at >14 Months

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

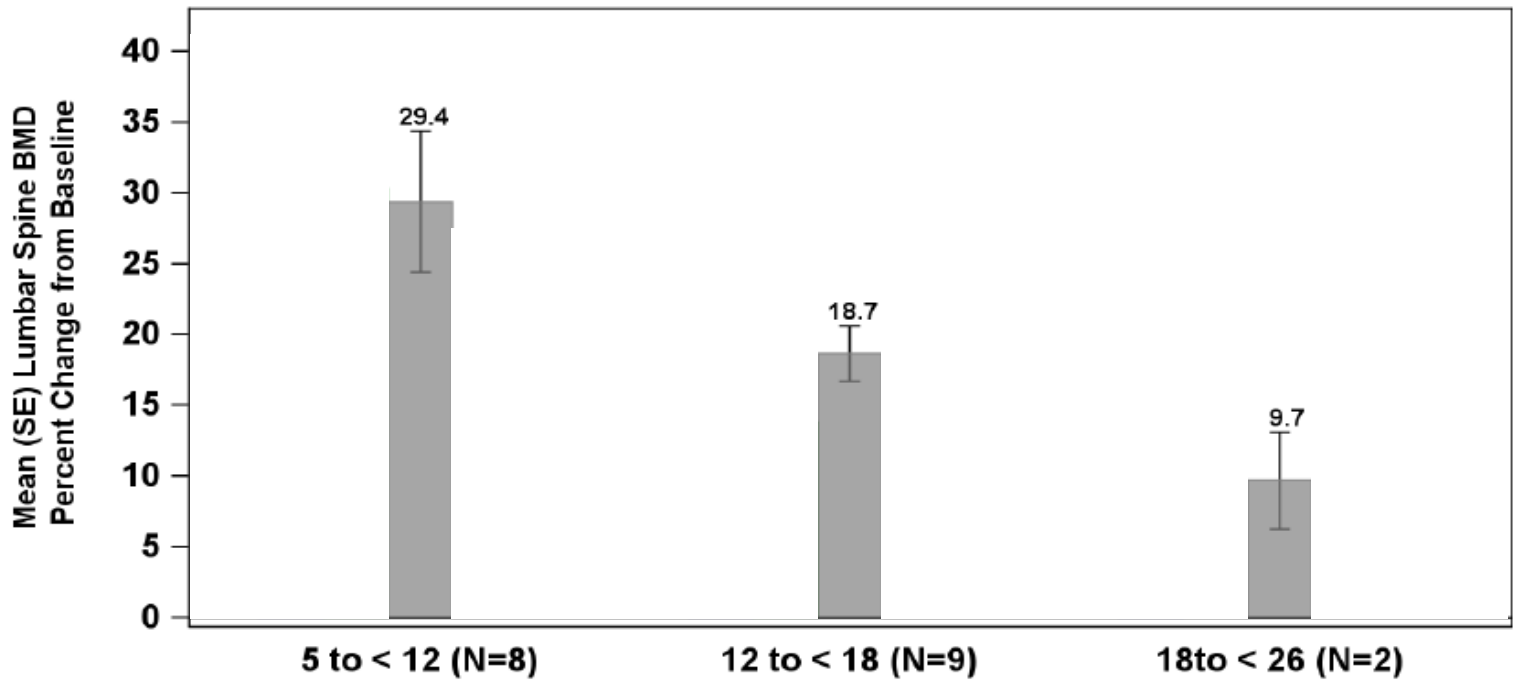


Mean Baseline Z-score of -1.73 goes to -0.49 at 12 months; P-values represent change from Baseline

1 Interim data as of May 24, 2024

UX143 for OI: Younger Patients in Phase 2 Showed Very large BMD Improvement at Month 12

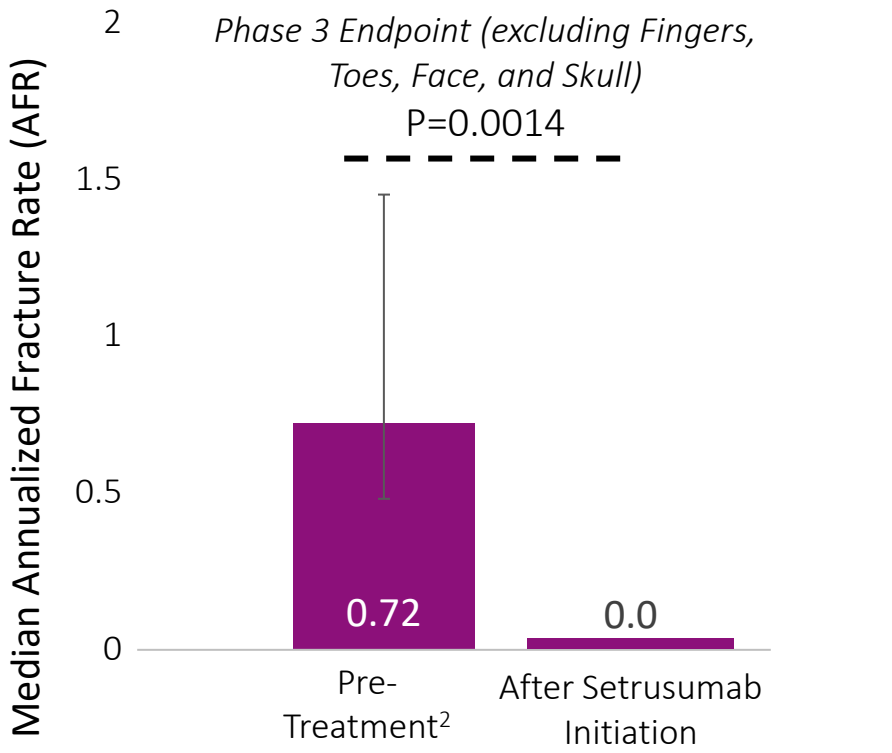
Lumbar Spine BMD by Age Group[1]
% Change from Baseline at Month 12



1 Interim data as of May 24, 2024

UX143 for OI: Updated Phase 2 Data Showed 67% Reduction[1] in Annualized Fracture Rate (AFR) Post-Treatment

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

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: Summary of Potential Timing for Phase 3 Readouts

Potential Readout	Expected Timing	Threshold
Interim Analysis 1 (IA1)#	End of 2024 or early in 2025	p<0.001
Interim Analysis 2 (IA2)#	Mid-2025	p<0.01
Final Analysis	Fourth Quarter 2025	p<0.04

#: Only if an interim analysis clears our stringent threshold, would we share that the readout was successful. Topline clinical results would be announced several months later as the study requires patients complete final visits over a couple months and time to collect and prepare the data for a complete analysis

Potential successful readout scenarios driven by baseline fracture variability, accumulated fracture events, and p-value stringency

GTX-102 for Angelman Syndrome (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

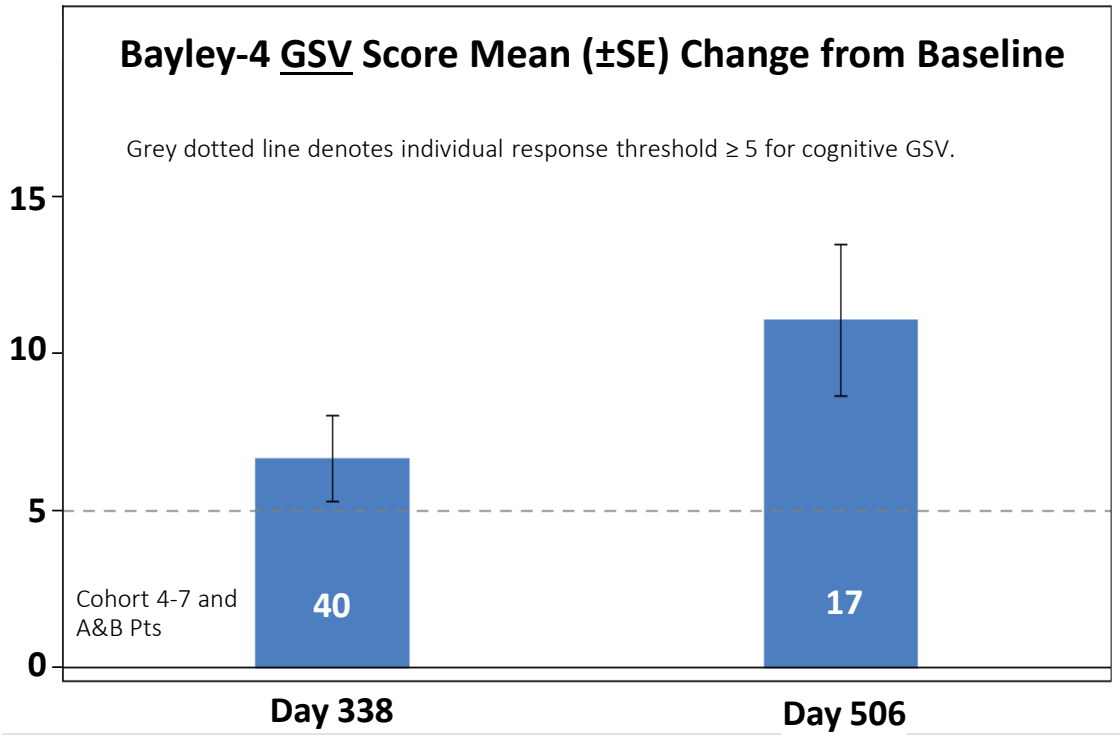
No additional cases of lower extremity weakness; safety profile is understood and remains consistent

Phase 3 study *Aspire* is expected to initiate by the end of 2024

Data shared via press release on November 9, 2024

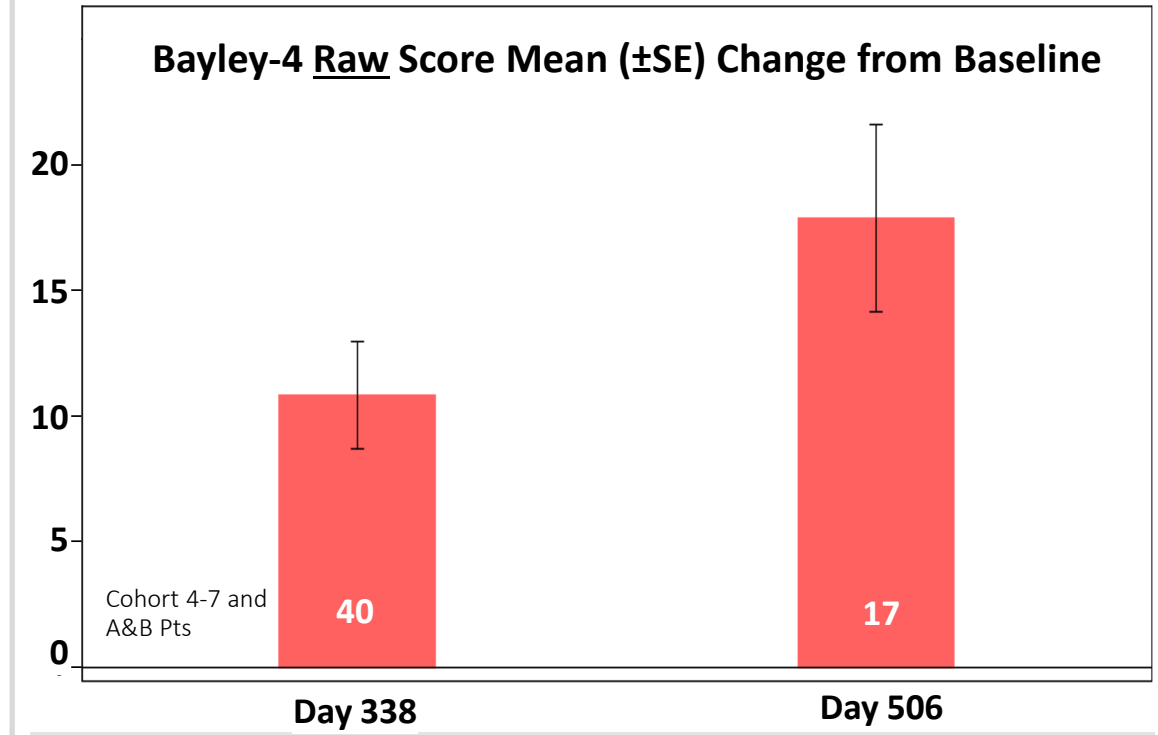
GTX-102 for AS: Cognition by Bayley-4 GSV and Raw Scores Show Ample Power for Phase 3 Trial

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. *Most 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
					+3
					+3
					+3
					+3
					+2
					+2
					+2
					+2
					+2
					+2
					+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:
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

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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

Expect to initiate *Aspire* by end of 2024; *Aurora* in 2025

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

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UX701 for Wilson Disease: 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) has 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

UX111 for MPS IIIA: Data Showed Meaningful Accumulated Substrate Reduction and Improved Clinical Function in Pediatric Patients

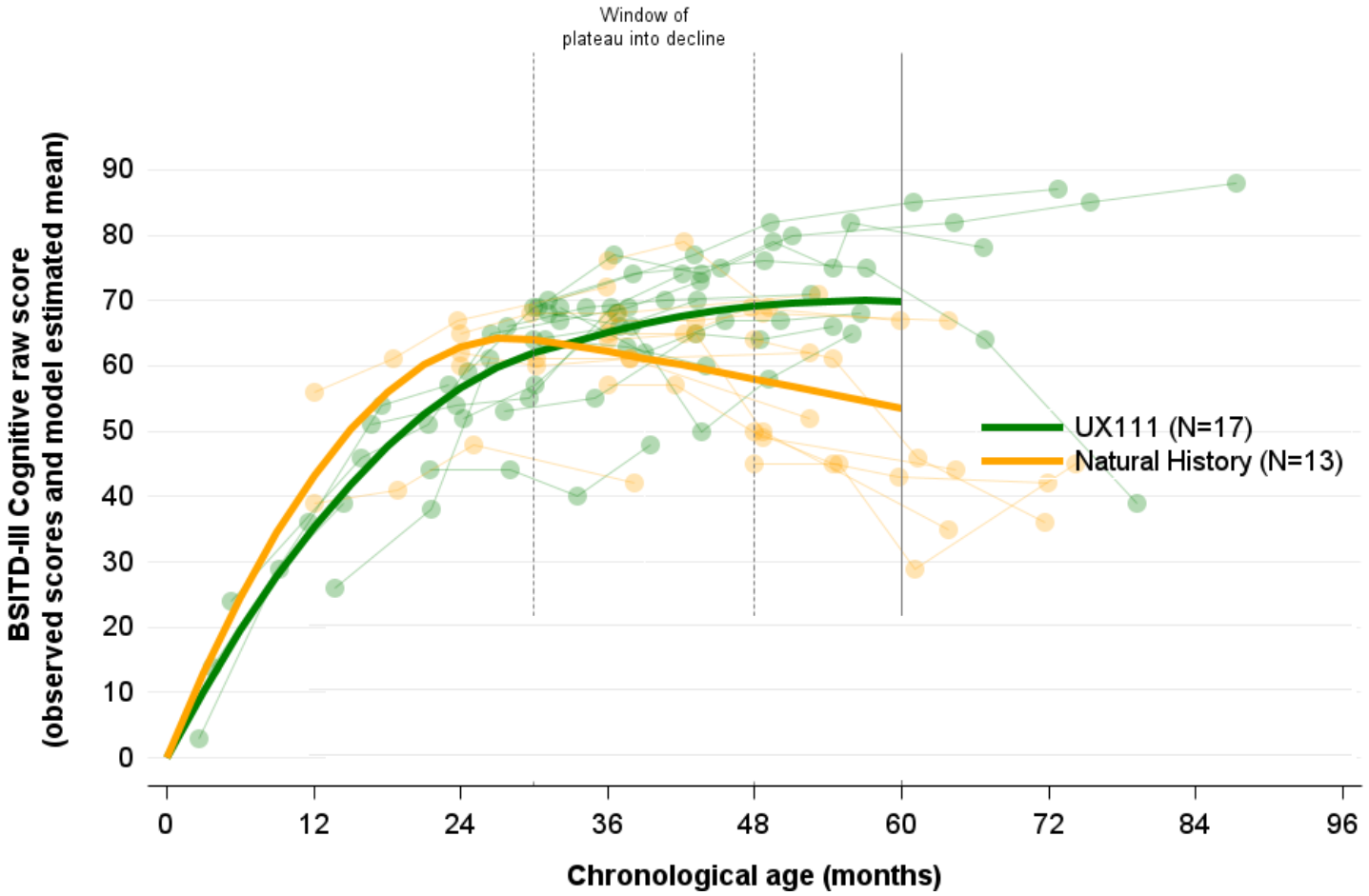
- Reduction in CSF HS[1] exposure predicts increase in BSITD-III Cognitive raw scores
  - Rapid and sustained reduction (≥ 50%) in toxic CSF HS exposure over a median follow-up period of approximately 2 years (23.9 months) after treatment with UX111
  - Gain or stability in BSITD-III Cognitive raw scores 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 expected to be filed around the end of 2024

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

Data presented at WORLD*Symposium* 2024

UX111 for Sanfilippo Syndrome (MPS IIIA): Divergence Emerging at 48 Month Using Estimated Mean BSITD-III Cognitive Raw Scores



The mean (95% CI) difference between the UX111-treated and control group in BSITD-III Cognitive raw score change was 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

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

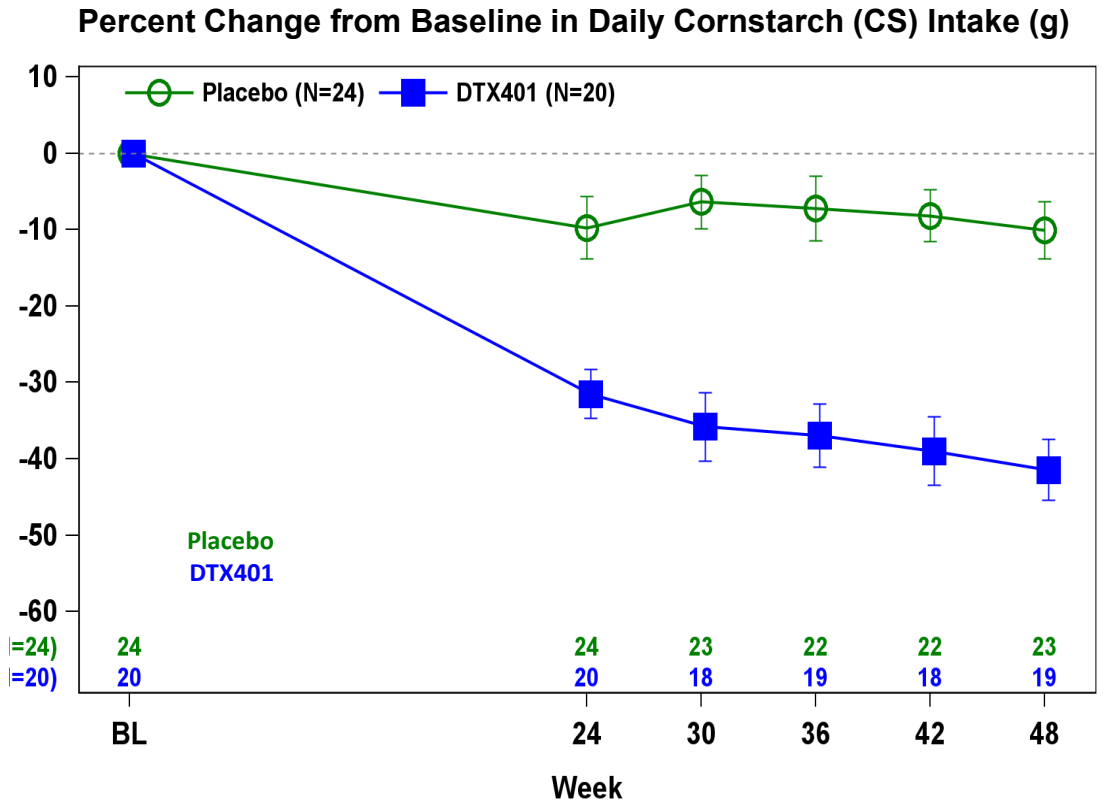
Key Takeaways

- 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: Statistically Significant Reduction (41%) in Daily Cornstarch Intake at Week 48 ($p < 0.0001$) with Maintenance of Glucose Control

Persuasive statistically significant cornstarch reduction continued through Week 48



-10.1 value skewed by one spurious patient (note Std Dev=18.0)

% Change BL to W48	Placebo N=24	DTX401 N=20	p-value
Mean (SD)	-10.1# (18.0)	-41.4 (17.5)	
Median	-2.9	-36.9	
LS Mean (SE)	-10.3 (4.1)	-41.3 (4.5)	<0.0001

Mean Baseline CS (g): Placebo was 269g and DTX401 was 296g

Responder Analysis at Week 48

≥ 30% reduction in cornstarch

- 13/19 (68%) in DTX401 arm compared to 3/23 (13%) in placebo ($p = 0.0003$)

≥ 50% reduction in cornstarch

- 7/19 (37%) in DTX401 compared to 1/23 (4%) in placebo ($p = 0.0038$)

Data presented via conference call on May 30, 2024

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

Data presented via conference call on May 30, 2024

DTX401 for GSDIa: Phase 3 Data in Crossover Patients Demonstrated 62% Mean Reduction in Cornstarch at Week 30

- Twelve crossover patients have reached Week 30 post-treatment and have had a substantial 62% mean reduction of daily cornstarch
  - 2x the rate of decrease compared to patients in the original DTX401 treatment arm
  - Patients able to titrate cornstarch much more rapidly once confirmed to have been treated with DTX401 and with timely, direct access to their glucose levels
- Patients from the original DTX401 treatment arm are continuing to reduce their daily cornstarch intake, while maintaining glycemic control
- DTX401 demonstrated a consistent and acceptable safety profile with no new safety concerns as of the data cut-off

Phase 3 data will be discussed with regulatory authorities to support a BLA submission in mid-2025

Data presented via press release on November 5, 2024

Gene Therapy Platform
Built on Best-in-Class Manufacturing Capabilities

Manufacturing facility in Bedford, MA

Pinnacle PCL™ platform

- Efficient, reliable production of AAV
- Improved product quality and yield
- Lower cost and increased speed of production
- Potentially improved safety of AAV therapy at higher doses

Facility capable of running both HEK and Pinnacle PCL

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We are Hitting our 2024 Clinical Catalysts with More to Come

PROGRAM	OBJECTIVE	Anticipated Timing
UX143 Osteogenesis Imperfecta	Complete enrollment of Phase 3 *Orbit* and *Cosmic* studies Further Phase 2 data update	✓ ✓
GTX-102 Angelman Syndrome	Phase 1/2 Expansion LPI and data End of Phase 2 Discussion with FDA Phase 3 *Aspire* study initiation	✓ ✓ By end of 2024
UX701 Wilson Disease	Stage 1 enrollment completion Stage 1 safety and initial efficacy data	✓ ✓
UX111 Sanfilippo Syndrome	Updated pivotal data at WORLDSymposium™ Path for accelerated review with FDA BLA Filing	✓ ✓ Around end of 2024
DTX401 GSDIa	Topline Phase 3 data	✓
DTX301 OTC deficiency	Phase 3 enrollment completion	Around end of 2024

We are Leading the Future of Rare Disease Medicine

History of strong clinical and commercial execution

Near-term catalysts for key clinical programs

Expect multiple significant product approvals

Revenue growth and expense management support path to profitability

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® Exclusivity Summary

	XLH Orphan Exclusivity	TIO Orphan Exclusivity	Biologics Exclusivity	Crysvita CoM Patent	Q2W Dosing Patent
United States	2025	2027	2030	2032*	2035
Europe	2028 XLH Orphan + D&M Exclusivity			2033* Crysvita CoM Patent	2035 Q2W Dosing Patent

2018 2020 2022 2024 2026 2028 2030 2032 2034 2036

*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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