Corporate Presentation

October 2026

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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 our February 2026 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, the commercial launch, availability and market acceptance of GENGLYCOS™ and FAYUVI™, 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, risks and uncertainties related to the commercial launch and market acceptance of GENGLYCOS and FAYUVI, 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 including risks associated with AAV gene therapy, 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, manufacturing risks, including our limited experience operating our own manufacturing facility and the ability to manufacture and supply our approved products in sufficient quantities and in compliance with regulatory requirements, 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.

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

6 commercial products

CRYSVITA® burosumab

genglycos™ pariglasgene brecaparvovec-opnr suspension for IV infusion

Evkeeza®

FAYUVI™ rebisufligene etisparvovec-hopf suspension for IV infusion

DOJOLVI®

Mepsevii®

Diverse clinical pipeline

4 Ongoing clinical programs → 3 Phase 2/3 programs

12 years from IPO

↓

Heading to $1 Billion in Revenue

↓

Path to GAAP Profitability in 2027

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Large portfolio of commercial and clinical stage programs

	Product/ Candidate	Description	Disease / Therapeutic Area: Bone/Endo · Inborn Errors of Metabolism · Neurology	Prevalence[1]
Commercial	CRYSVITA burosumab	Anti-FGF23 monoclonal antibody	X-linked hypophosphatemia and tumor induced osteomalacia	51,000
	DOJOLVI TRIHEPTANOIN Oral Liquid	Substrate replacement	Long-chain fatty acid oxidation disorders (LC-FAOD)	8,000 – 14,000
	genglycos pariglasgene brecaparvovec-opnr suspension for IV infusion	AAV8 G6Pase gene therapy	Glycogen storage disease type Ia (GSDIa)	6,000 – 8,000
	Evkeeza (evinacumab-dgnb) Injection	Anti-ANGPTL3 monoclonal antibody	Homozygous familial hypercholesterolemia (HoFH)	3,000 – 5,000
	FAYUVI rebisufligene etisparvovec-hopf suspension for IV infusion	AAV9 SGSH gene therapy	Sanfilippo syndrome (MPS IIIA)	3,000 – 5,000
	Mepsevii (vestronidase alfa-vjbk) injection	Enzyme replacement	Sly syndrome (MPS VII)	200
Clinical Development	**UX143** (setrusumab)	Anti-Sclerostin monoclonal antibody	Osteogenesis Imperfecta (OI) in Phase 3	60,000
	DTX301	AAV8-OTC gene therapy	Ornithine Transcarbamylase (OTC) in Phase 3	10,000
	DTX701	AAV9-ATP7B gene therapy	Wilson Disease (WD) in Phase 2	50,000
	UX016	Small molecule prodrug	GNE Myopathy in Phase 1/2	10,000

1: Approximate prevalence in commercially accessible geographies

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FAYUVI (UX111) and GENGLYCOS (DTX401) **Now Approved**

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FAYUVI for Sanfilippo syndrome (MPS IIIA)

Standard full approval granted on September 17, 2026

MPS IIIA: Fatal lysosomal storage disease of CNS

- Early childhood onset, rapid neurodegeneration

- Treatment: No approved therapies
- High urgency to treat as young as possible
- Prevalence*: ~3,000 to 5,000

** Prevalence in commercially accessible geographies*

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

- Full approval on neuro endpoints, not accelerated approval
- Received a Priority Review Voucher (PRV)
- Sales to leverage existing inborn errors of metabolism field
- U.S. based manufacturing at Andelyn and Ultragenyx's GTMF

Sadie lives with MPSIIIA

"This gene therapy addresses a pressing unmet clinical need and offers families a promising therapeutic option. It is additionally gratifying in that this vector was first developed at Nationwide Children's more than a decade ago, and its approval highlights our commitment to developing therapies that meaningfully impact children's health."

Kevin M. Flanigan, M.D.
Director of the Center for Gene Therapy at Nationwide Children's Hospital

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FAYUVI for MPS IIIA: *Transpher A* clinical trial and long-term follow-up demonstrated clinical benefit and durable treatment effect

Biochemical efficacy demonstrated by a reduction in accumulated cerebral spinal fluid (CSF) heparan sulfate (HS) levels throughout the study and all age groups.

Clinical efficacy based on patients' mean change in Bayley-III Cognitive raw score from 24 to 60 months of age and demonstrated a 23.5 point higher ($p<0.0001$) cognitive score versus natural history.

Safety: Generally well tolerated with a maintained, favorable profile

MPS IIIA is extremely devastating disease for which patients and doctors will have *high urgency to treat* and FAYUVI is a single IV infusion.

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Discussed on September 17, 2026 conference call

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GENGLYCOS for glycogen storage disease type Ia (GSDIa)

Accelerated Approval Granted on August 19, 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 – 8,000

**Worldwide in commercially accessible geographies*

GENGLYCOS: Approved on August 19, 2026

- Indicated to reduce daily cornstarch intake in adult and pediatric patients 8 years of age and older with GSDIa
- Received a PRV
- Manufacturing in-house at Bedford, MA plant
- Leverages existing inborn errors of metabolism field team

"Day-to-day management of GSDIa requires a relentless regimen of raw cornstarch and strict dietary management that can be extraordinarily demanding for patients and families. Even with meticulous adherence to this regimen, patients must be perfect. Any missed cornstarch puts patients at risk of severe hypoglycemia, seizures, and even death"

David Weinstein, M.D., MMSc
Former Director-Glycogen Storage Disease Program
Connecticut Children's Medical Center

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GENGLYCOS for GSDIA:
Robust and durable effect seen throughout clinical development

Phase 1/2 and Phase 3: 52 treated patients, over 8 years of follow-up

Phase 3: Significant reductions in daily cornstarch requirements, compared to placebo, while maintaining glycemic control

Safey: Well-tolerated with an acceptable safety profile

GSDIa has a *high urgency to treat* due to the significant burden and creates fear for any error that might cause severe hypoglycemia or death

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Discussed on August 19, 2026 conference call

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Patients, providers and payers at the forefront of gene therapy launches

Dedicated Gene Therapy Guides added to UltraCare® to support patients and caregivers

Delivered through Qualified Treatment Centers (QTCs) with specialized expertise and training to safely administer gene therapy

GENGLYCOS enhanced Disease Monitoring Program (DMP) will collect 2 years of safety and efficacy data and FAYUVI DMP will follow patients up to 10 years

Extensive engagement with state Medicaid and national payers has reinforced recognition of disease severity

Discussed on August 19, 2026 and September 17, 2026 conference calls

Clinical Programs

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UX143 for osteogenesis imperfecta (OI)
Fully human monoclonal antibody; discussing potential path forward with FDA

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*

UX143 Setrusumab:

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

- Phase 3 *ORBIT* and *COSMIC* did not achieve primary endpoints, but achieved the secondary endpoint of improvements in BMD with strong statistical significance[1]
- Discussing potential path forward with FDA, targeting alignment by EOY

Matthew lives with osteogenesis imperfecta

1: Data presented at JP Morgan Healthcare Conference 2026

UX143 for OI: Phase 3 status

- Neither study achieved primary endpoint of reduction in AFR[1] compared to placebo (*ORBIT*) or bisphosphonates (*COMIC*)
- 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
- Post-hoc analyses were conducted in patients <18 years of age with >3 fractures at baseline over the previous 2 years

1: Annualized fracture rate

UX143 for OI: Additional analyses support meaningful reductions in AFR in patients with >3 fractures at baseline

Control AFR lower than the anticipated rates assumed for planning

Annualized Fracture Rate	Anticipated	Estimated
ORBIT Placebo	0.75	0.55
COSMIC IV-BP	2.0	1.14

Assumptions per SAP v9.0; earlier public descriptions were approximations

Post-hoc analyses revealed that fracture rates may have been impacted by:

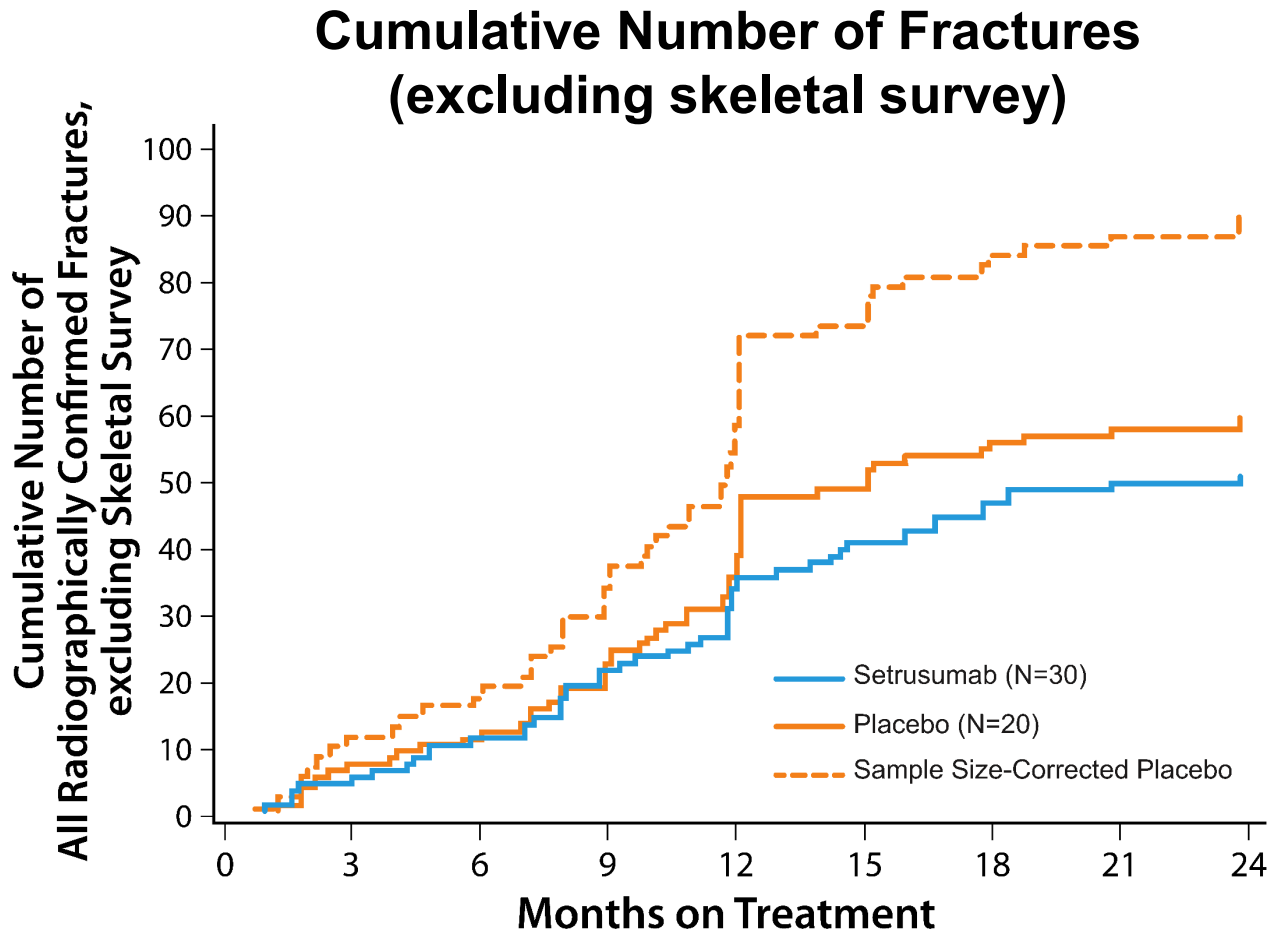
- More frequent OI Type III in UX143-treated groups
- Increased activity in the *ORBIT* setrusumab group
- Pre-treatment with IV-BP therapy

Post-hoc analyses conducted in patients <18 years with >3 fractures at baseline over the previous 2 years

ORBIT **post-hoc** analysis shows numerically lower AFR on UX143 vs placebo (p=0.063)

Confirmed fractures by Baseline and 12-mo intervening "For-Cause" x-rays		**Clinical fractures, excluding survey**
UX143 AFR (n=30)	# of fractures	51
	Mean (SD)	1.20 (1.29)
Placebo AFR (n=20)	# of fractures	60
	Mean (SD)	2.15 (2.66)
Estimated[1] UX143 AFR (95% CI)		**1.15** (0.74, 1.79)
Estimated[1] Placebo AFR (95% CI)		**2.15** (1.32, 3.50)
Rate Ratio[1] UX143/Placebo (95% CI)		0.54 (0.28, 1.04)
Rate Change[1] UX143/Placebo (95% CI)		**-46.30** (-72.14, 3.53)
P-value[1]		**0.063**

[1] Negative Binomial model



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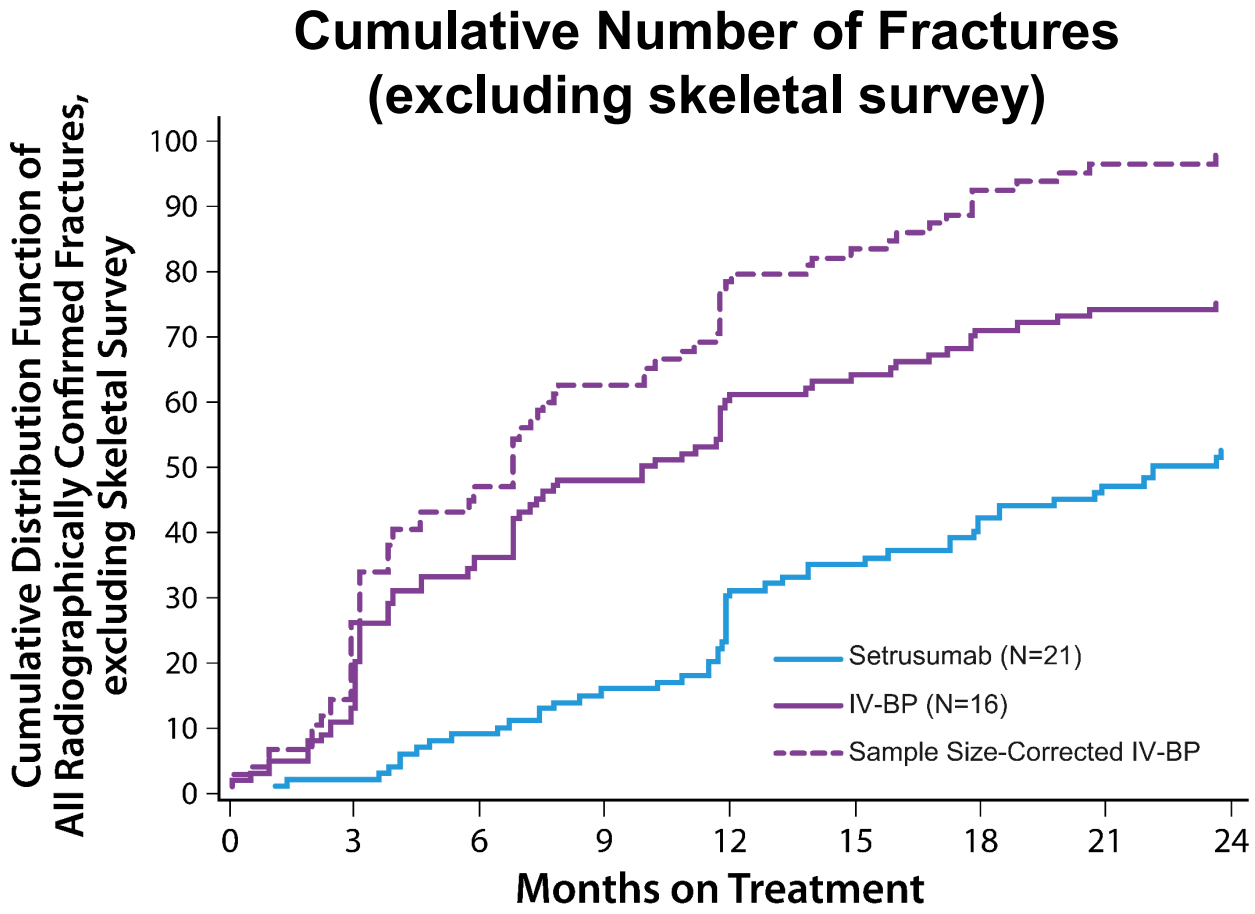
Data presented at ASBMR 2026 Annual Meeting

COSMIC **post-hoc** analysis shows significantly lower AFR in patients treated with UX143 vs IV-BP (p=0.009)

Confirmed fractures by Baseline and 12-mo intervening "For-Cause" x-rays		**Clinical fractures, excluding survey**
UX143 AFR (n=21)	# of fractures	52
	Mean (SD)	1.41 (1.31)
IV-BP AFR (n=16)	# of fractures	75
	Mean (SD)	3.24 (2.89)
Estimated[1] UX143 AFR (95% CI)		**1.44** (0.97, 2.13)
Estimated[1] IV-BP AFR (95% CI)		**3.04** (2.04, 4.51)
Rate Ratio[1] UX143/IV-BP (95% CI)		0.47 (0.27, 0.83)
Rate Change[1] UX143/IV-BP (95% CI)		**-52.58** (-72.87, -17.11)
P-value[1]		**0.009**



[1] Negative Binomial model

Exploratory post-hoc analysis (not prespecified): rate ratio 0.47 (95% CI 0.27–0.83); nominal p=0.009, not adjusted for multiplicity; hypothesis-generating

Data presented at ASBMR 2026 Annual Meeting

UX143 for OI: Conclusions and next steps

- Neither study met the primary endpoint of reducing annualized fracture rate (AFR)
- Significant improvements occurred in a number of key endpoints including aBMD Z-score, vertebral fractures and, relative to placebo, patient-important outcomes
- Setrusumab was well tolerated, with no new safety concerns
- Post hoc analyses suggest greater fracture benefit in pediatric patients with >3 fractures in the previous 2 years, supported by biomarker outcomes:
  - *ORBIT*: (5 to <18 years): numerically lower AFR versus placebo
  - *COSMIC*: (2 to <7 years): significantly lower AFR versus IV-BP

Working with FDA to determine what prospective clinical data would be required to support a BLA

Data presented at ASBMR 2026 Annual Meeting

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DTX301 for ornithine transcarbamylase (OTC) deficiency

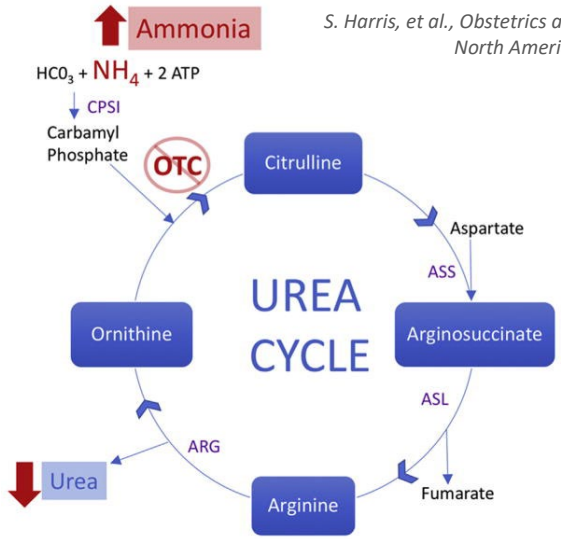
AAV8 gene therapy; Phase 3 responder data expected in 1H-2027

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 reduction in plasma ammonia AUC_{0-24} vs placebo at Week 36 (p=0.018)

Reduction in ammonia into normal range sustained despite reductions of scavenger medication and liberalization of protein restricted diet

Strong differentiated improvements in OTCD-related disease and symptoms in PGIC: DTX301: +2/+3 in 86% vs only 13% Placebo

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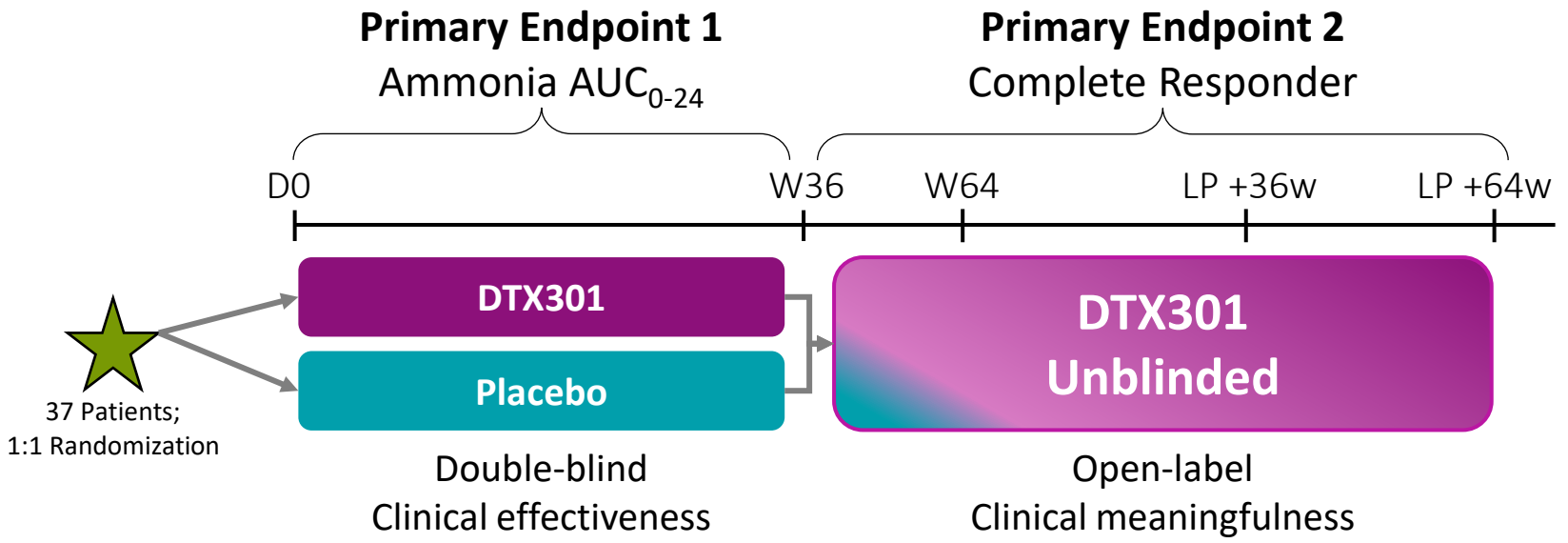
Data disclosed in press release on March 12, 2026

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

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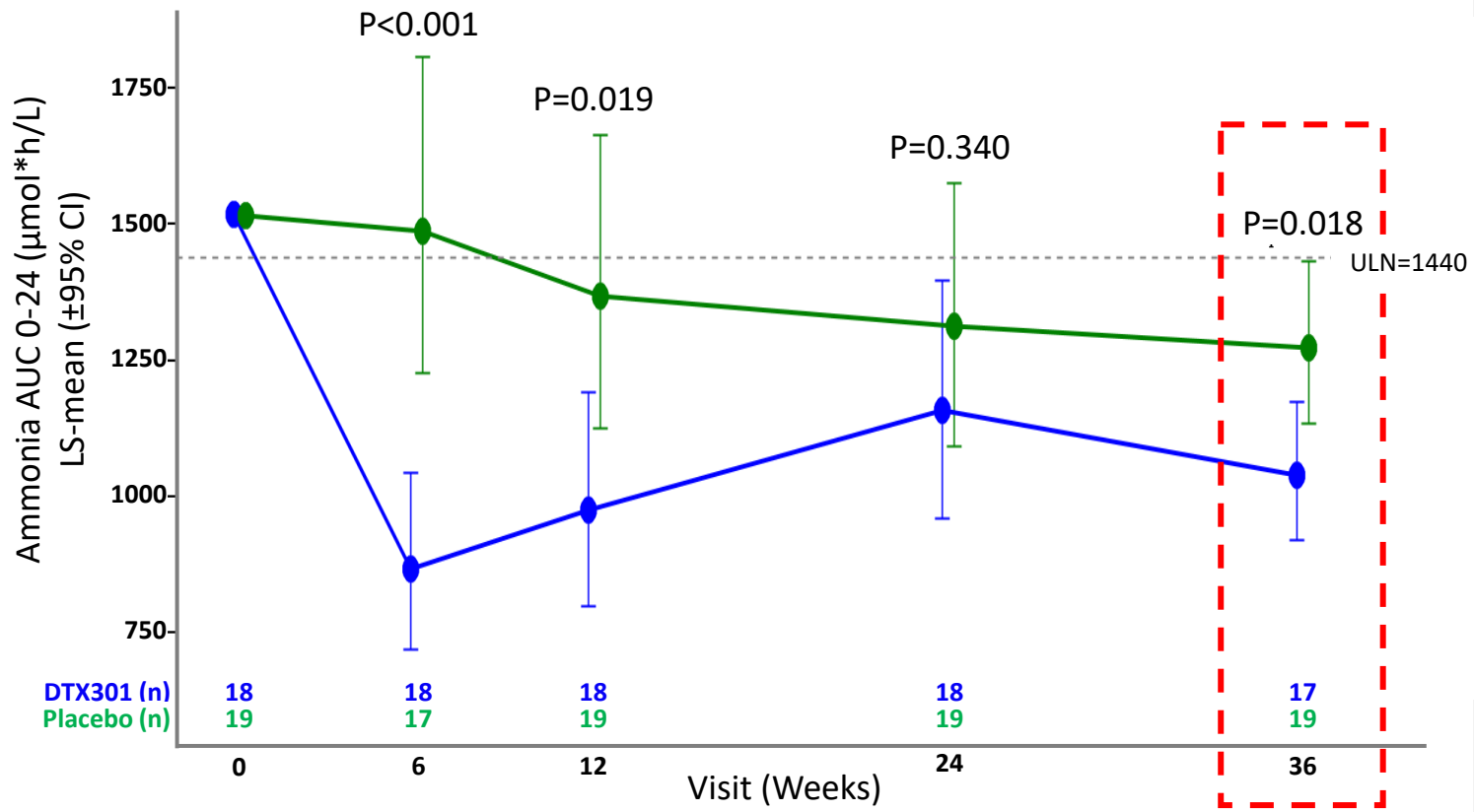
Data disclosed in press release on March 12, 2026

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



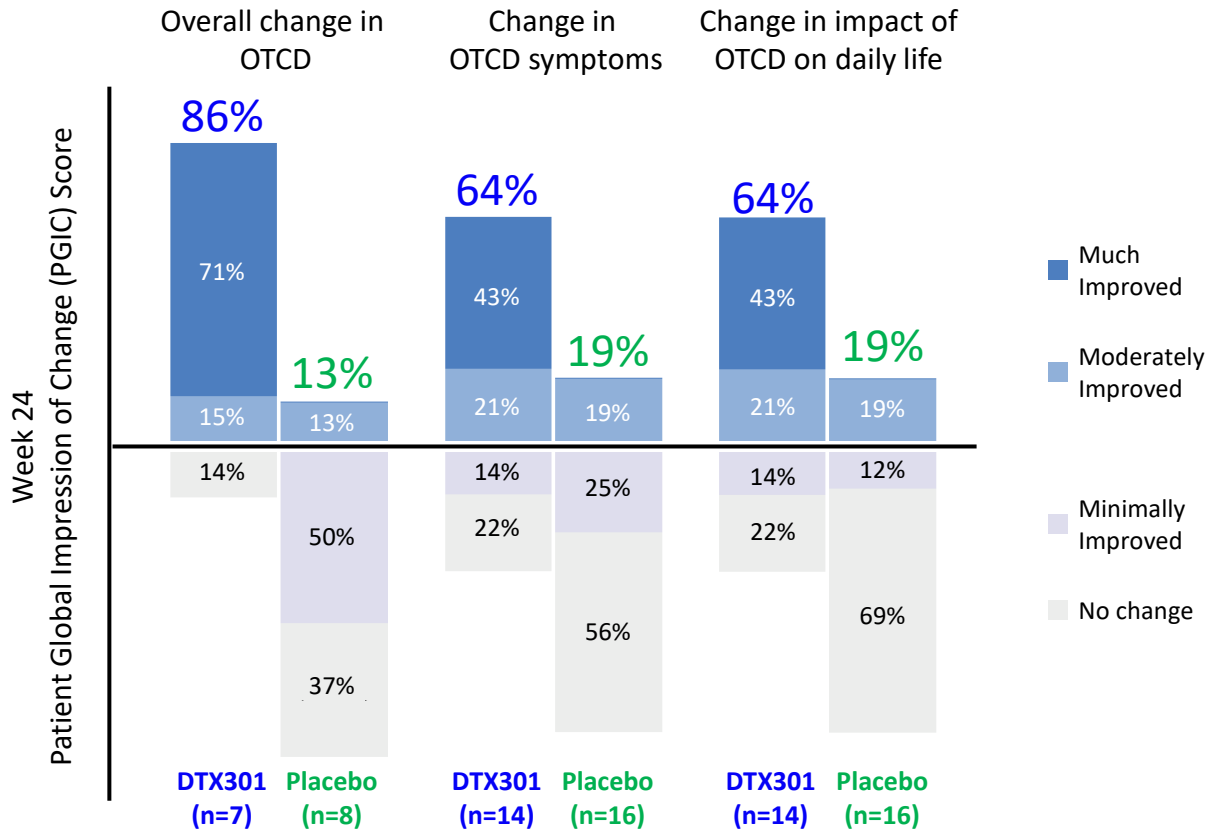
Impact on scavenger medications & protein restricted diet at Wk36

- 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

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UX701 for Wilson disease (WD)

AAV9 gene therapy; Stage 1, dose finding data expected in 4Q-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, dose finding data expected in 4Q-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
- Cohort 4 higher dose patients treated and pending data in 4Q-2026
- 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

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Financials and 2026 Catalysts

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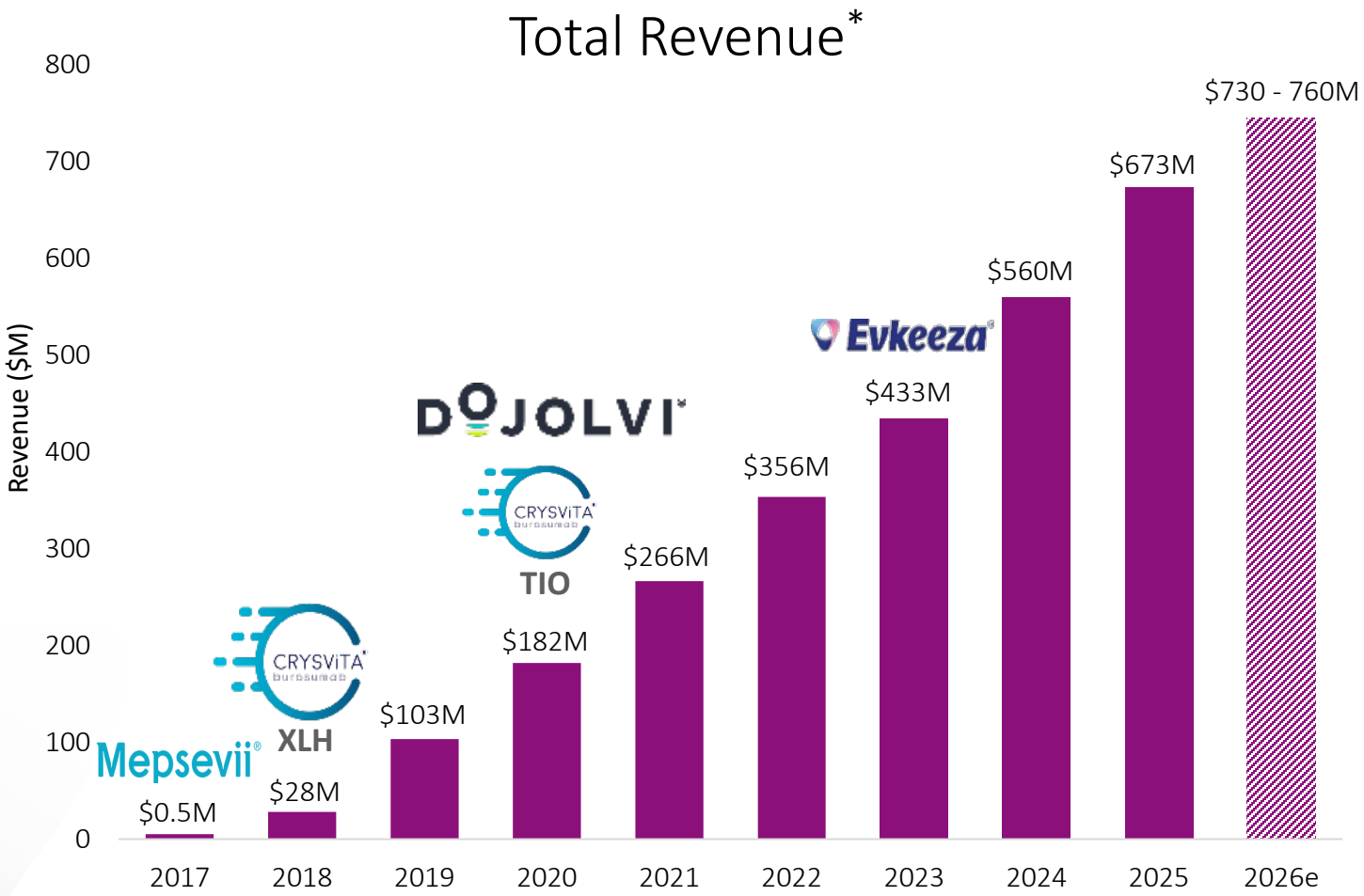
Consistent year over year revenue growth with 20% in 2025



* Excluding Bayer and Daiichi collaboration revenue and potential future launches including GENGLYCOS and FAYUVI. 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 including GENGLYCOS and FAYUVI
2 Total CRYSVITA revenue, including North America, Latin America, and Europe

Planned PRV monetization and new product launches support 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[1] compared to 2025:**
  - **2026:** flat to down low-single digits *with multiple launches & Ph3 studies*
  - **2027:** decrease at least 15%
    - R&D decrease by ~38%, partially offset by increase in SG&A to support launches and existing approved products
- **Additional expense reductions to be discussed on 3Q26 quarterly results conference call**
- **Cash: $436M in cash and investments as of June 30, 2026**
- GENGLYCOS PRV monetized for $210M[2]; expect to also monetize FAYUVI PRV

1: Assumptions reflect net impact from the February strategic restructuring plan, severance and other one-time non-recurring restructuring costs, and targeted launch investments in GENGLYCOS and FAYUVI
2: Gross proceeds. Upon closing of the transaction, 20% will be payable by the Company to the NIH.

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Key clinical and regulatory catalysts

PROGRAM	OBJECTIVE	ANTICIPATED TIMING
DTX401 GSDIa	BLA accepted PDUFA decision	✓ Approved
UX111 Sanfilippo syndrome	BLA accepted PDUFA decision	✓ Approved
UX701 Wilson disease	Stage 1 dose finding data (Cohorts 1-4)	4Q-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 launching two, first-ever gene therapy treatments

History of consistent and ongoing revenue growth from an expanding global commercial base business

Near-term catalysts from GENGLYCOS and FAYUVI commercial launches

Revenue growth and new launches plus expense management to reach profitability in 2027 and beyond

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Appendix

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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)	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)
GENGLYCOS™ (GSDIa)	NIH (Non-Exclusive)	• Recombinant vectors comprising codon-optimized G6Pase gene (2034) • Low single-digit royalty
FAYUVI™ (MPSIIIa)	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 mid to high single-digit royalty

Product	License	Europe Intellectual Property Rights/Royalties + Milestones
EVKEEZA® (HOFH)	Regeneron	• EVKEEZA antibody and use for treatment of HOFH (2036)[2]; Stabilized formulations of EVKEEZA (Pending; 2041) • Treatment of HOFH-associated atherosclerosis and use in combination with LDL-C lowering regimen (2037) • 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)

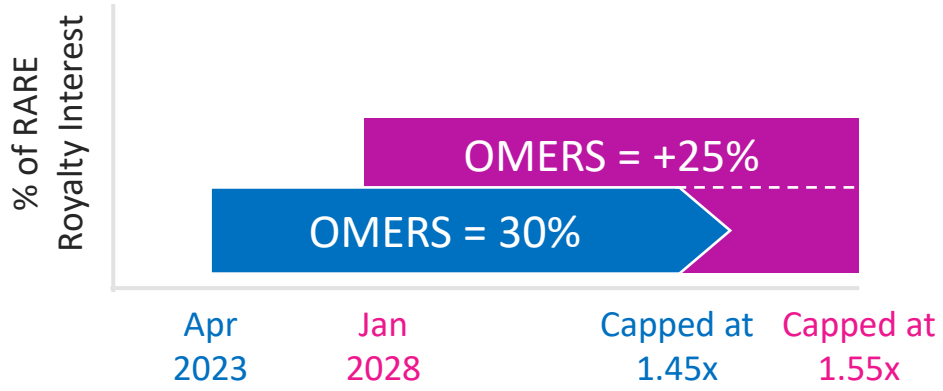
Key licenses & intellectual property – clinical programs

Product	License	United States 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
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)
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)

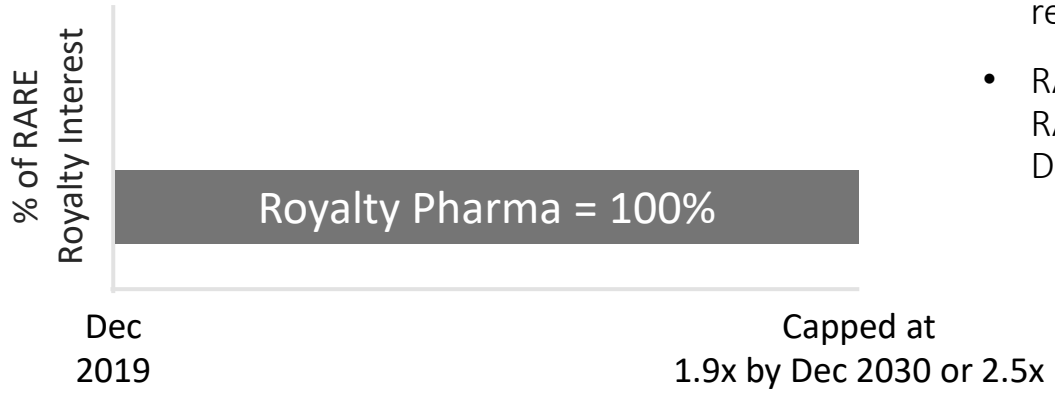
CRYSVITA partnership revenue recognition

	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

ultragenyx

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

Confidential and Proprietary

GENGLYCOS™ exclusivity summary

genglycos™
pariglasgene
brecaparvovec-opnr
suspension for IV infusion

GSDIa Orphan Exclusivity

2033

GENGLYCOS CoM Patent

2034

Biologics Exclusivity

2038

2022 2024 2026 2028 2030 2032 2034 2036 2038 2040

2034

GENGLYCOS CoM Patent

Regulatory Exclusivity Issued Patents

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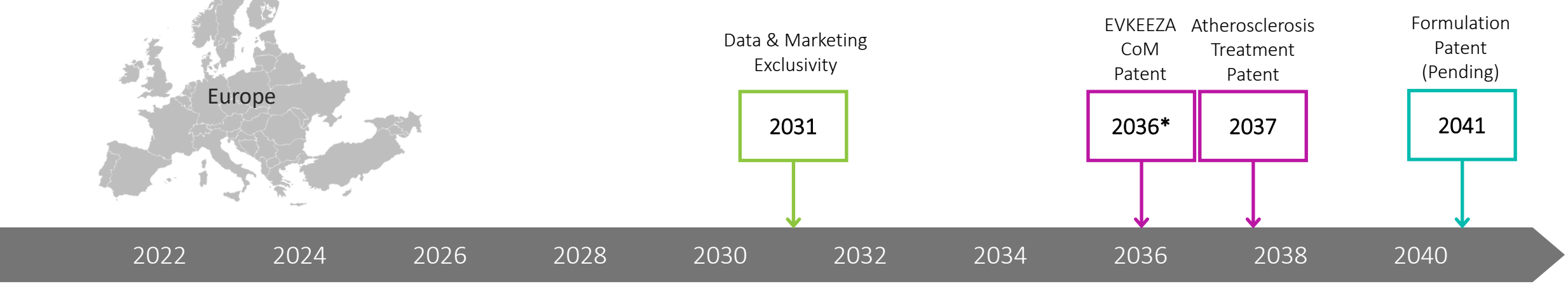
EVKEEZA® exclusivity summary

Evkeeza® (evinacumab-dgnb) Injection



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

Confidential and Proprietary

ultragenyx

FAYUVI™ exclusivity summary

FAYUVI™
rebisufligene
etisparvovec-hopf
suspension for IV infusion

FAYUVI for MPSIIIa Patent — 2032

MPSIIIa Orphan Exclusivity — 2033

Biologics Exclusivity — 2038

2022 2024 2026 2028 2030 2032 2034 2036 2038 2040

Regulatory Exclusivity Issued Patents

Confidential and Proprietary

ultragenyx

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

Confidential and Proprietary

ultragenyx