Corporate Presentation

February 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.

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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

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Product approvals since IPO exceed other successful, rare disease companies

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

^Approvals for rare disease indications
** Expected based on current pipeline*

Keys to our success:
Experienced team focused on innovation, speed, and execution

Deep scientific understanding increases probability of success

82% demonstrate clinical success

First ever regulatory and **development** approaches

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

Fastest development in the industry

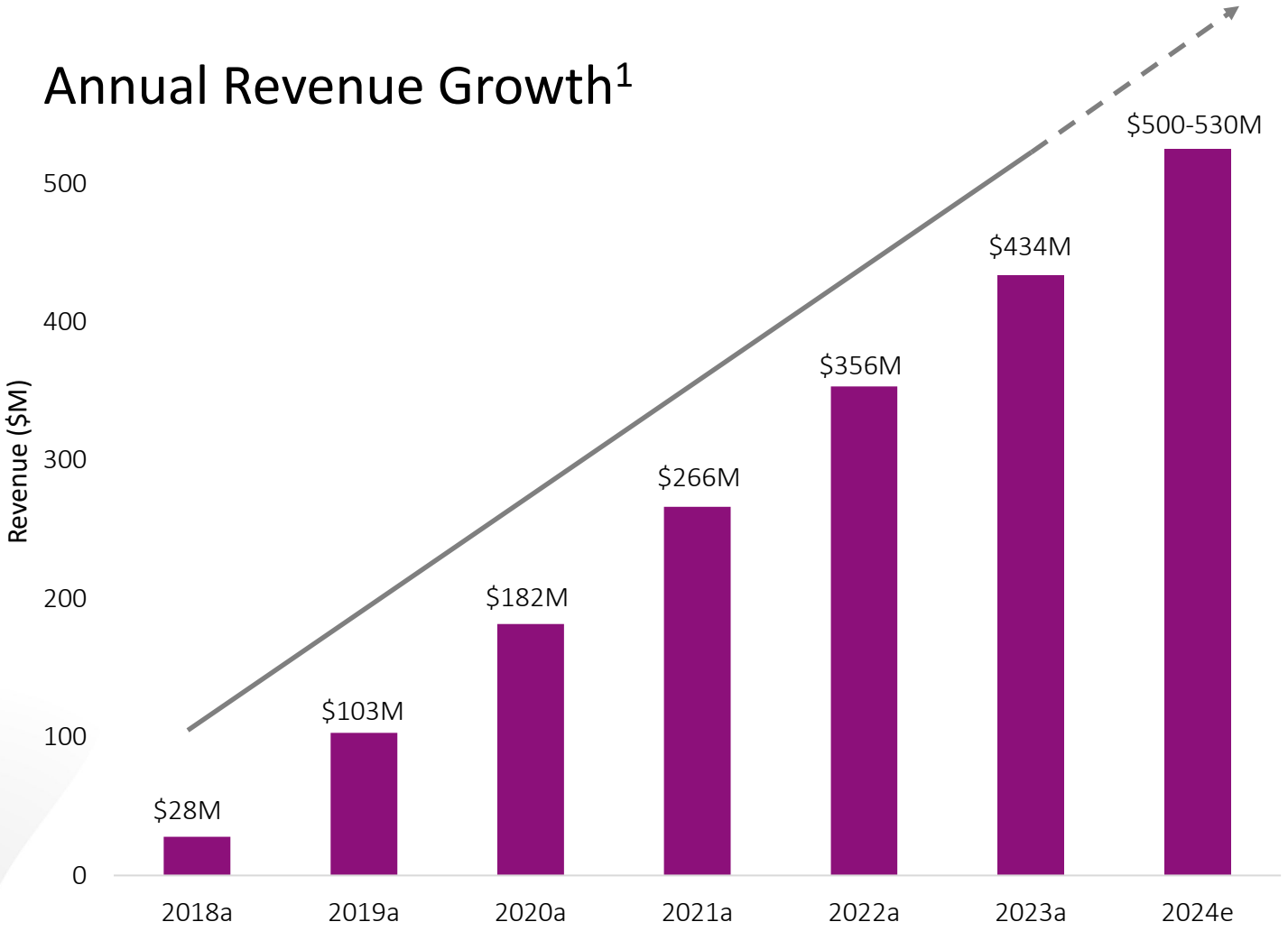
Avg 5.5 yrs from clinic to approval

Global commercial organization

Treating patients in **~34 countries**

2024 revenue growth expected to be ~20% driven by Crysvita in the U.S. and Latin America

Annual Revenue Growth[1]



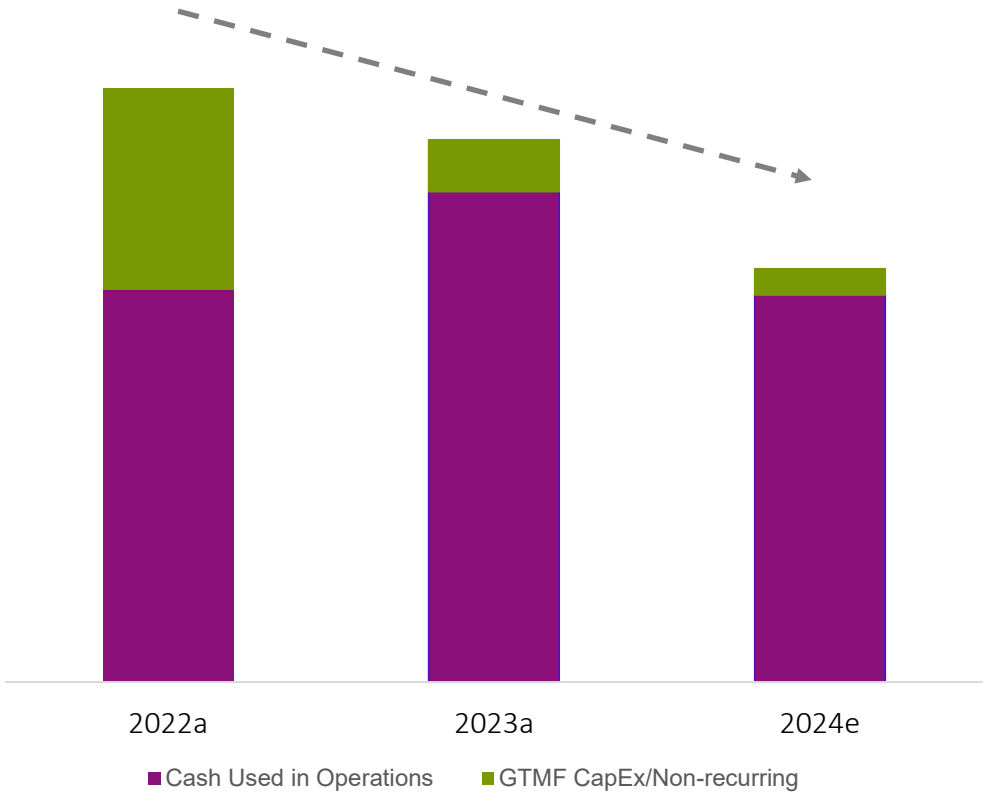
1 Excludes Bayer and Daiichi collaboration revenue and includes preliminary estimates for 2023 and 2024

Product	2023 Actual	2024 Guidance
Crysvita[1]	$328M	$375-400M *14% to 22% growth*
Dojolvi	$71M	$75-80M *6% to 13% growth*
Total Revenue[2]	$434M	$500-530M *15% to 22% growth*

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

Capital allocation focused on key clinical and commercials programs

Uses of Cash[1]



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

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

Cash and equivalents[2] of **$777M** as of December 31, 2023

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 available-for-sale investments as of December 31, 2023

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)

UX810
Duchenne

UX055
CDD

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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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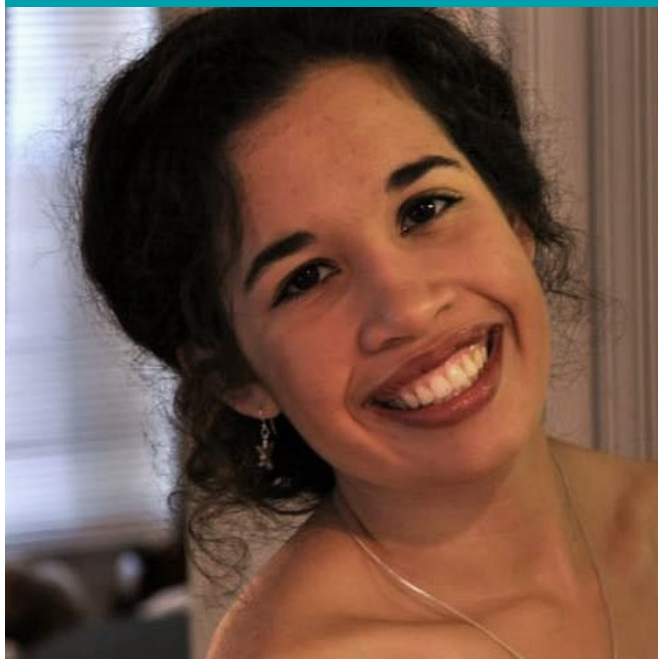
Three opportunities for significant near-term value creation

Osteogenesis Imperfecta

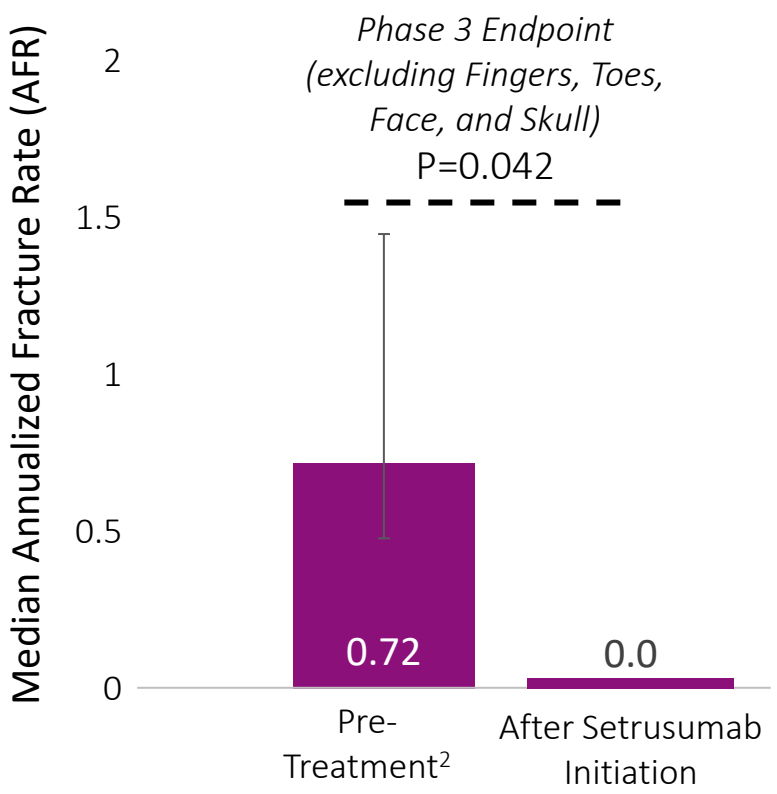
Angelman Syndrome

Wilson Disease



UX143 (setrusumab) for osteogenesis imperfecta: Phase 2 data showed 67% reduction in AFR post-treatment

Radiographically Confirmed Fractures[1]



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

1: Data presented at the company's Analyst Day on October 16, 2023
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

Data presented at the company's Analyst Day on October 16, 2023

GTX-102 for Angelman syndrome: Phase 1/2 showed meaningful and improving changes across multiple domains at longer timepoints[1]

Quantitative improvements in Bayley-4 far exceeding natural history

- Cognition
- Receptive Communication
- Gross Motor

Improvements in ASA show meaningful reductions in severity

- Sleep
- Behavior

Supportive data from

- EEG delta power and sleep spindle

Enrollment in Phase 1/2 complete with 74 patients enrolled across Cohorts 1 to 7 and A through E

1: Data presented at the company's Analyst Day on October 16, 2023

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GTX-102: Multi-Domain Responder Index (MDRI) captures broad clinical benefit across five domains and multiple endpoints

Patient (n=11)	ASA Sleep	ASA Behavior	Bayley-4 Receptive Comm	Bayley-4 Gross Motor	Bayley-4 Cognition	Total Net Responses*
1	**1**	0	1	0	3	**1**
2	**3**	**2**	**7**	3	**17**	**4**
3	**1**	**1**	**16**	**5**	**20**	**5**
4	0	0	**14**	3	**23**	**2**
5	**1**	**2**	**6**	1	4	**3**
6	**4**	0	**7**	1	1	**2**
7	**2**	**1**	**10**	**9**	**16**	**5**
8	**1**	**1**	**15**	**6**	**25**	**5**
9	2	0	**23**	**7**	**11**	**2**
10	n/a	n/a	2	1	**5**	**1**
11	0	0	3	4	**16**	**1**

Improvement Decline

*Day 338

Minimal Important Difference (MID)

- ASA-Sleep +/- 1
- ASA-Behavior +/- 1
- Bayley-4 Receptive Communication +/- 6
- Bayley-4 Gross Motor +/- 5
- Bayley-4 Cognition +/- 5

Median Total Net Responses +2

Net Responses ≠ 0 **p** = 0.001**

** P-value is from a sign test

Data presented at the company's Analyst Day on October 16, 2023

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UX701 for Wilson Disease: Four of five in Cohort 1 tapering SOC, including two completely off chelators and/or zinc therapy[1]

Cohort 1	Weeks on Study	Reduction of SOC [Chelator and/or zinc therapy]	Copper Trafficking
Patient 1	82	0%	Indeterminate
Patient 2	70	100%	Reduced urinary copper and improved trafficking by copper oxidase assay
Patient 3	44	100%	Reduced urinary copper and improved trafficking by copper oxidase assay
Patient 4	20	33%	Reduced urinary copper; pending trafficking assessment
Patient 5	16	50%	Reduced urinary copper; pending trafficking assessment

1: Data presented at the company's Analyst Day on October 16, 2023

Four pivotal gene therapy programs

DTX401 for GSDIa

- HEK293 manufacturing process
- Phase 3 fully enrolled
- Data anticipated in 1H24

UX111 for MPS IIIA

- HEK293 manufacturing process
- Updated pivotal data presented at WORLD*Symposium*™ in February 2024
- Seeking accelerated review path with FDA

DTX301 for OTC

- HEK293 manufacturing process
- Phase 3 FPI in February 2023
- Expect Ph3 to be fully enrolled in 1H24

UX701 for Wilson disease

- Pinnacle PCL™ manufacturing process
- Phase 1/2/3 study ongoing
- Stage 1 enrollment completed in January 2024 & data expected mid-2024

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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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Near-term key clinical catalysts

PROGRAM	OBJECTIVE	Timing
UX143 Osteogenesis Imperfecta	Complete enrollment of Phase 3 *Orbit* study Complete enrollment of Phase 3 *Cosmic* Further Phase 2 data update	1Q 2024 1H 2024 2H 2024
GTX-102 Angelman Syndrome	LPI for Expansion Cohorts Phase 1/2 Expansion data End of Phase 2 Discussion with FDA	✓ 1H 2024 Mid-2024
UX701 Wilson Disease	Stage 1 enrollment completion Stage 1 safety and initial efficacy data Initiation of Stage 2	✓ Mid-2024 2H 2024
DTX401 GSDIa	Phase 3 data	1H 2024
DTX301 OTC deficiency	Phase 3 enrollment completion	1H 2024

We are leading the future of rare disease medicine

Most productive rare disease company in the industry

Near-term catalysts for key clinical programs

Expect multiple blockbuster product approvals in 2-3 years

Revenue growth and expense management support path to profitability

Appendix

Key licenses & intellectual property – Commercial products

Product	License	US Intellectual Property Rights/Royalties
CRYSVITA® (XLH, TIO)	Kyowa Kirin Co. (KKC)	• Anti-FGF23 antibodies and use for treatment of XLH and TIO (2023-2032)[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® (MPS 7)	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)[2] • Mid single-digit royalty
	N/A (IP Owned by Ultragenyx)	• Ultrapure triheptanoin and use in treatment of FAOD (Pending; 2034)

Product	License	EU Intellectual Property Rights/Royalties + Milestones
EVKEEZA® (HOFH)	Regeneron	• Evkeeza antibody and use for treatment of HOFH (2036)[3] • 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 U.S. patent term extension
[3]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)	Sub-License from REGENXBIO of UPENN IP	• AAV8 Capsid (2024) • Low to mid single-digit royalty and development milestones
	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	• AAV8 Capsid (2024) • 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 (2024-2026) • 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

*Includes projected US PTE and EU SPC awards

DOJOLVI® Exclusivity summary

DOJOLVI® TRIHEPTANOIN Oral Liquid

*Includes projected 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

Exemplary additional patent applications pending:

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

Projected expiration dates between 2037-2041

*Includes projected EU SPC award