Corporate Presentation

November 2022

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, the effects from the COVID-19 pandemic on our clinical trial activities, business and operating results, 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, the uncertainties inherent in the clinical drug development process, such as the regulatory approval process, 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, 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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Leading the Future of Rare Disease Medicine

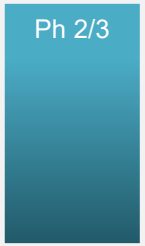
Growing Commercial Revenue	▪ Crysvita, Dojolvi, Mepsevii ▪ Evkeeza launching in Europe ▪ Anticipate ~30% revenue growth[1]
Strong Clinical Pipeline	▪ 7 clinical programs ▪ 5 ongoing pivotal studies ▪ Supporting future revenue growth
More than Ultra-Rare	▪ Four large value programs ▪ Multiple ways to reach multi-billion value creation

1: Anticipated product revenue in 2022 compared to 2021

Three Rare Disease Franchises and Five Commercial Programs

COMMERCIAL DEVELOPMENT

BONE ENDOCRINE

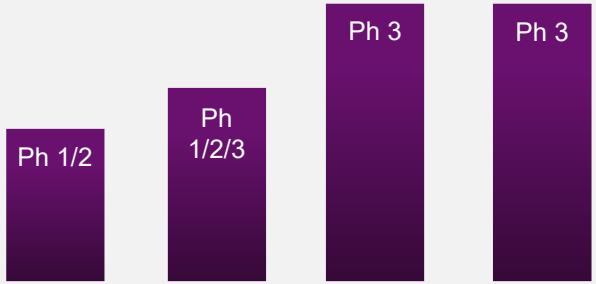


Setrusumab
for OI

CRYSVITA® burosumab — **XLH**

CRYSVITA® burosumab — **TIO**

METABOLICS



UX053 for GSDIII

UX701 for Wilson

DTX401 for GSDIa

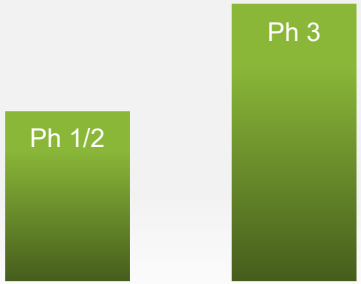
DTX301 for OTC

Mepsevii™ (vestronidase alfa-vjbk) injection — **MPS VII**

DOJOLVI® TRIHEPTANOIN Oral Liquid — **LC-FAOD**

Evkeeza® (evinacumab-dgnb) Injection — **HoFH**

CNS / MUSCLE



GTX-102 for Angelman

UX111 for MPS IIIA

Preclinical
(Disclosed)

UX055 for CDKL5

UX810 for Duchenne

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Three Independent Paths to Create Meaningful Value Based on Four Large Value Programs

Gene Therapy Programs

Wilson Disease Large Value Opportunity

- **Clinical proof of concept for GSDIa & OTC to Ph3**
- **Commercial scale manufacturing platform**

Angelman Syndrome (GTX-102)

Advancement of GTX-102 to Phase 3 Trials

- **Neuro-developmental disorder**
- **Promising, interim Ph 1/2 data**

Osteogenesis Imperfecta (UX143)

Phase 3 Transition in Mid-2023

- **Traditional MAb Biologic**
- **Clear dose response and proven MOA**

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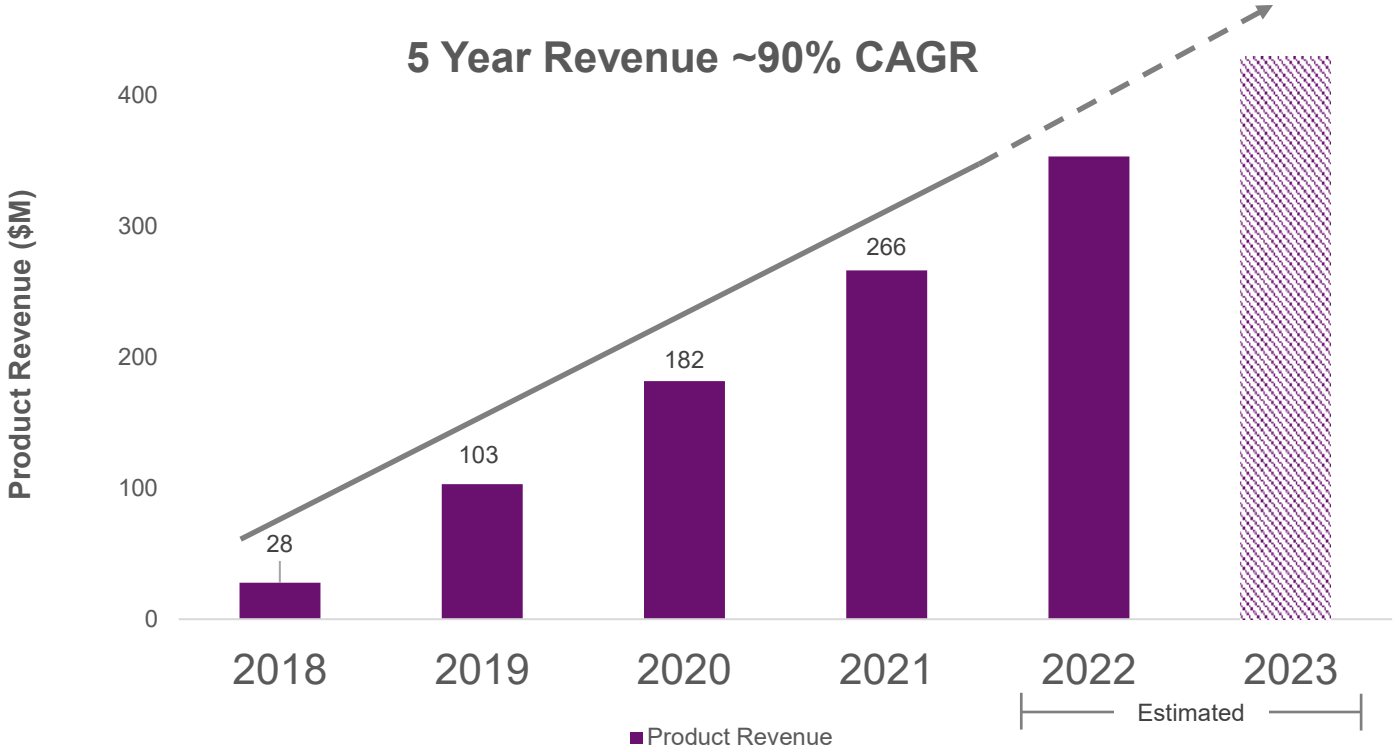
Solid Base of Revenue Growth with Significant Opportunity from Larger Clinical Programs



Growth Driven by:

Commercial products provide growing base

Pipeline assets further accelerate growth trajectory (Osteogenesis Imperfecta, Angelman, Wilson, Duchenne)

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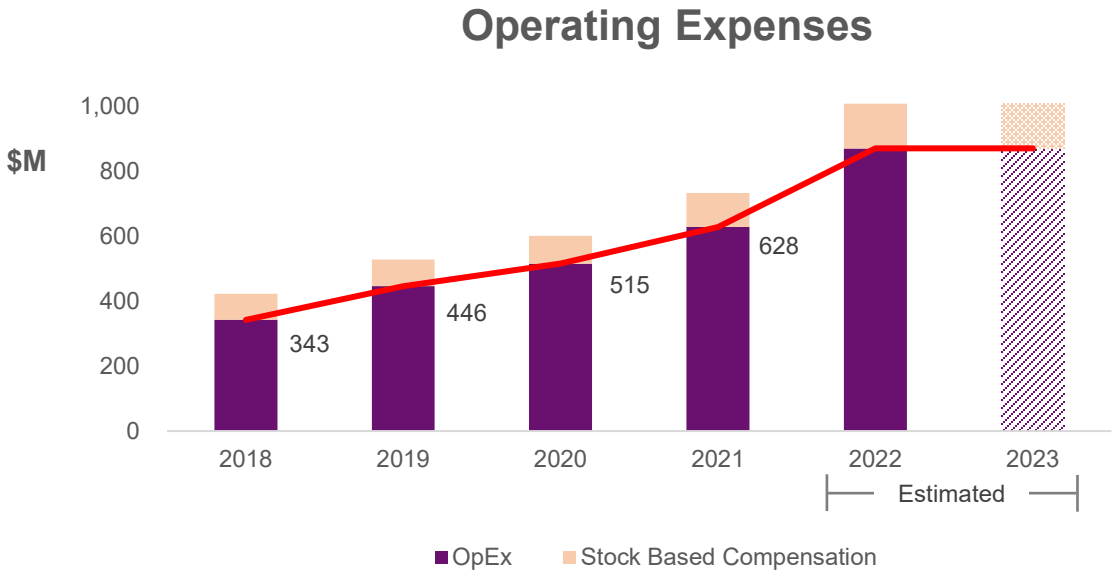
Prioritization of High Value Programs Expected to Limit Operating Expenses and Decrease Net Cash Burn in 2023



- Strong capital position: ~$1.0 billion in cash and equivalents[1]
- One-time expenses refilled pipeline and established the teams needed to deliver second generation pipeline
- Prioritizing high value programs
- Staging early-stage programs
- Managing spend and FTEs tightly

1: Cash, cash equivalents, and available-for-sale investments as of September 30, 2022

Key Upcoming Milestones in 2022 and 2023

PROGRAM	OBJECTIVE	TIMING
DTX401 GSDIa	Ph 3 LPI Ph 3 data readout	Around the end of 2022 ~1 year from LPI
UX143 Osteogenesis Imperfecta	Ph 2 LPI Ph 2 data readout and Ph 3 transition Initiate young pediatric study	Early 2023 Mid-2023 1H23
GTX-102 Angelman syndrome	FPI for Expansion Cohorts Ph 1/2 data readout	1H23 2023
UX701 Wilson disease	Stage 1 enrollment completion Stage 1 safety and initial efficacy	Mid-2023 End of 2023 or early 2024
DTX301 OTC deficiency	Ph 3 FPI	Around the end of 2022
UX053 GSDIII	Ph 1/2 Single ascending dose data	1H23

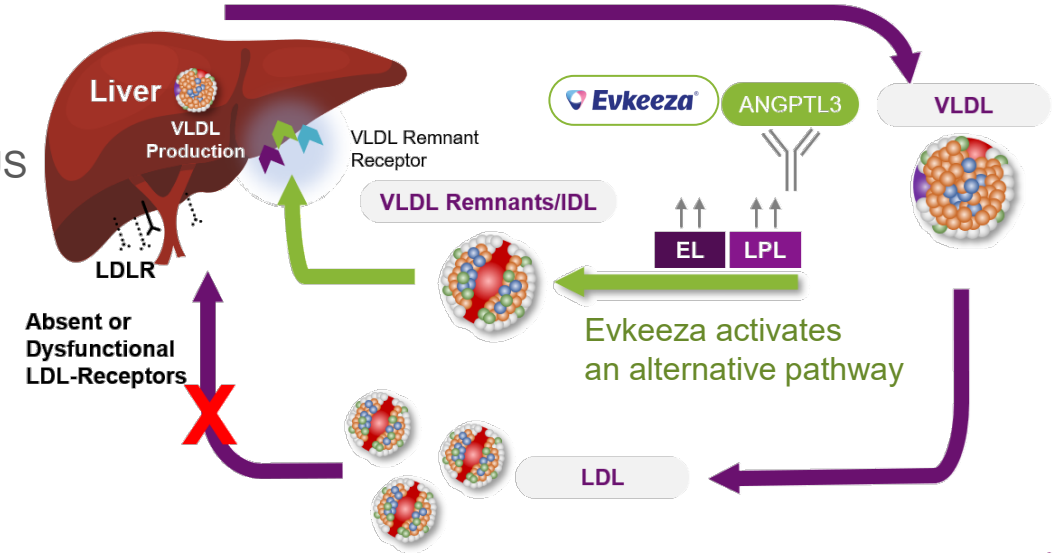
Evkeeza (evinacumab) for Homozygous Familial Hypercholesterolemia (HoFH)

Approved and Launching in Europe

Evkeeza®
(evinacumab-dgnb)
Injection

Evkeeza Lowers Cholesterol and Triglycerides by Enhancing an Alternative Short Cut Lipid Path to the Liver

- **HoFH:** Form of familial hypercholesterolemia with dangerously high levels of LDL-C (>400 mg/dL)
  - Premature atherosclerosis & cardiac events
  - Most treatments inadequate for HoFH
- **Prevalence** 3,000 to 5,000 in key markets ex-US
  - Approximately 1,600 in EU where approved
- **Evkeeza:** fully human MAb given IV or SC
  - **BLOCKS** anti-ANGPTL3
  - **ENHANCES** an alternative path to the liver
  - **Optimal mechanism** for bypassing the defective LDL-Receptor and restoring lipid delivery to the Liver
  - **Reduces LDL by 50%** on top of all other meds



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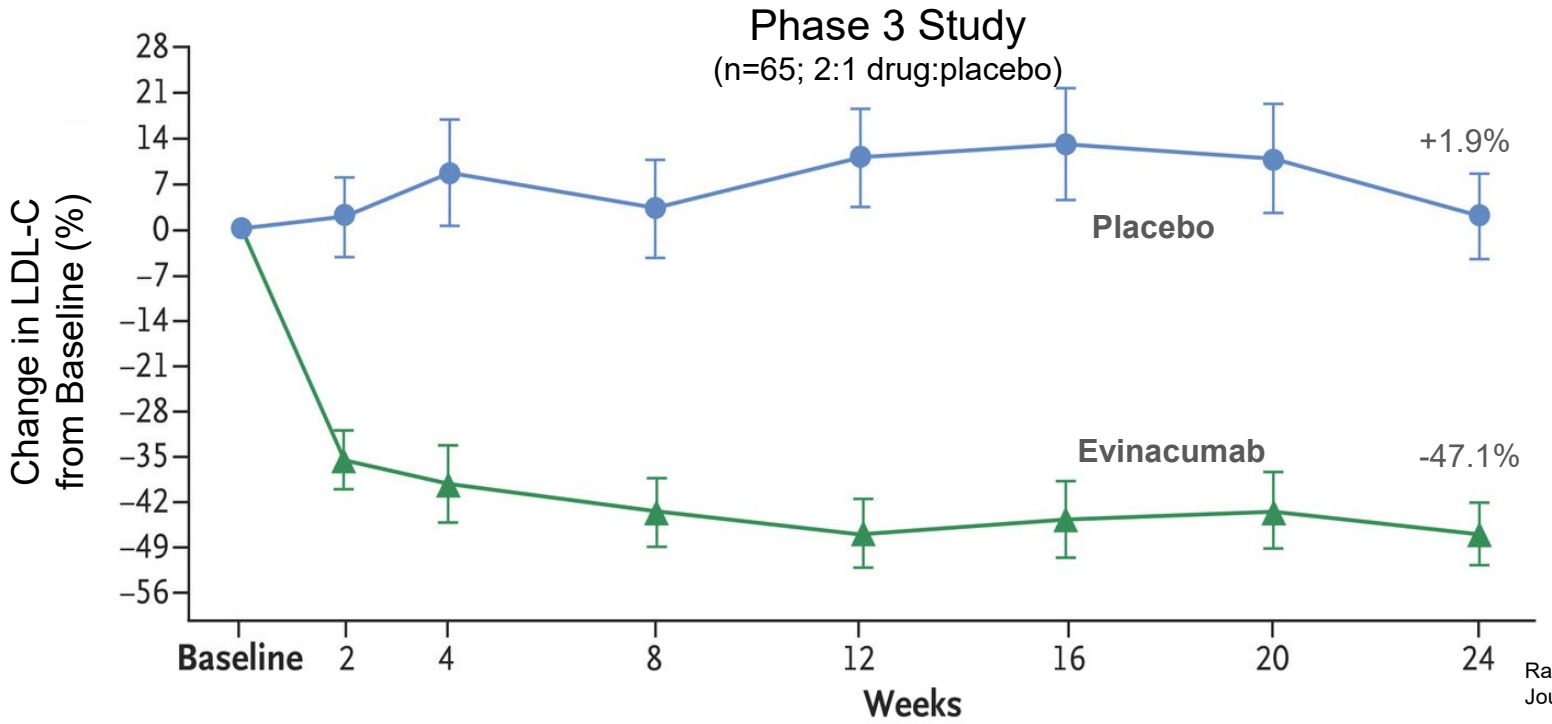
Patients on Evinacumab Had Substantial LDL-C Reductions *on Top of All Ongoing Lipid-lowering Therapy Regardless of Genotype*



Raal *et al.* New England Journal of Medicine, Aug 2020

LDL-C reduced 72% relative to placebo in patients with <2% LDLR activity

Triglycerides also reduced by 50% across study participants

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Evkeeza Well-Positioned in High Unmet Need HoFH

Evkeeza high potency and monthly dosing compares well

- **Competition** for HoFH with low LDLR activity
  - Juxtapid/Lojuxta (lomitapide) Poor tolerability issues with persistence/compliance issues
  - Apheresis to remove LDL with frequent and long sessions, short efficacy period
- **Market research** with 10 top KOLs indicate strong interest in Evkeeza profile
- **Reimbursement dossiers** filed or being filed across the EU
- **Expect revenue steady build** as reimbursement achieved in each EU country
- **Canada and Japan next filings** with strong KOL interest

"I'm extraordinarily excited about evinacumab for HoFH. It's going to be way more convenient than current interventions and its big advantage is that it works independently of LDLR"

– Italian KOL

"I will try to get all of my HoFH patients on evinacumab. It's got to be first line."

– Canadian KOL

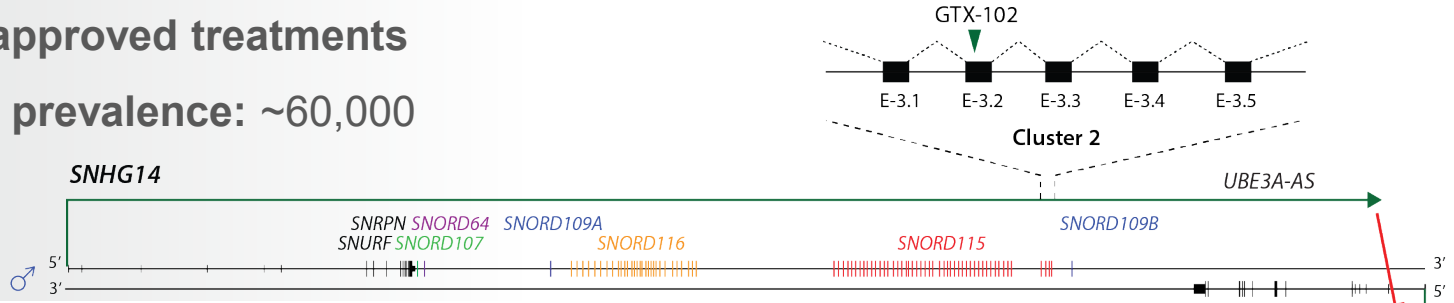
GTX-102 Program for Angelman Syndrome

Positive interim Phase 1/2 efficacy data from ASO program in Angelman syndrome

GTX-102 for Angelman Syndrome

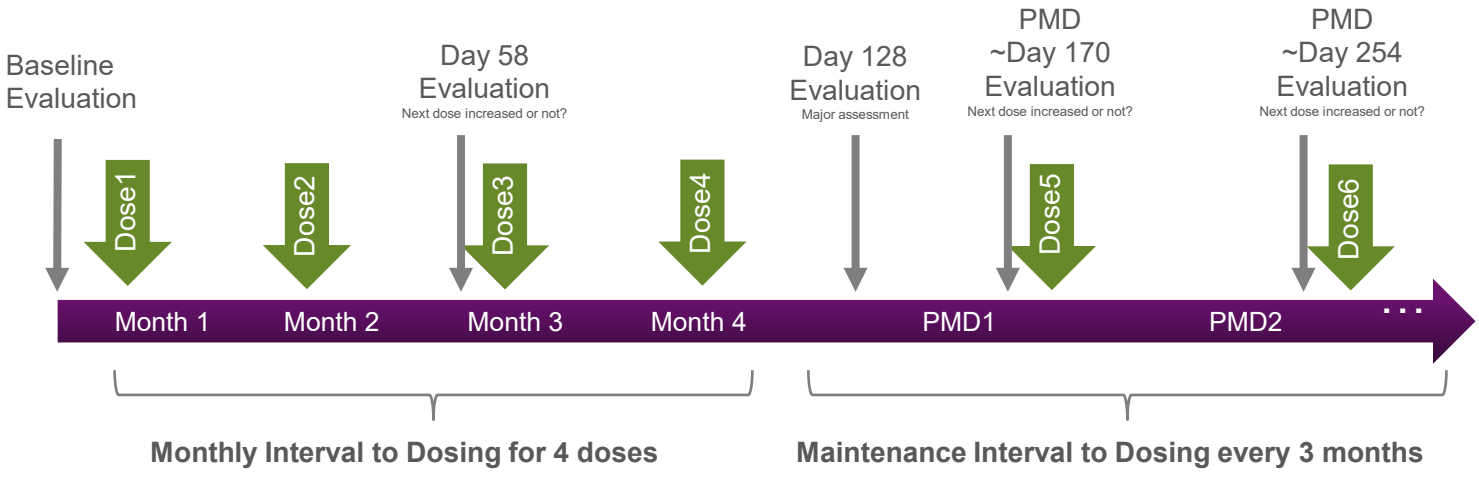
Large neurodevelopmental disorder

- **Angelman syndrome:** Neurogenetic disorder caused by loss of expression of UBE3A gene
- **Devastating Neuro Disease:** Motor dysfunction, lack of speech, cognitive impairment, sleep disorder
- **GTX-102**: Antisense oligonucleotide (ASO) that can unlock expression of missing enzyme UBE3A from paternal chromosome
- **No approved treatments**
- **WW prevalence:** ~60,000



Study Dosing Schematic for GTX-102 in U.K. and Canada

Dose of 10-12mg tolerated well in young and older cohorts



Dose in mg

Cohort	Doses #1-2	Doses #3-4	Doses #5-6
4-8 years old			
4	3.3	5	7.5, 10
6	7.5	10	10-12
8-17 years old			
5	5	7.5	10-12
7	10	12	12-14

Day 58 assessment: CGI-C-AS to guide dose escalation for 3rd/4th doses
Baseline, Day 128, PMD evals: CGI-C-AS; CGI-S-AS; Bayley 4; Vineland 3; ORCA; EEG; Seizure Sleep Diary; Functional Domain Interviews
Day 128 CGI-C-AS and future evaluation guide additional dose escalation during maintenance to maximum of 14 mg

CGI-C-AS=clinical global impression of change (improvement), Angelman syndrome; CGI-S-AS=clinical global impression of severity, Angelman syndrome; ORCA=observer-reported communication assessment; PMD=pre-maintenance dose

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Increases in Receptive and Expressive Communication

Exceeds Threshold to be Significant; Comparable to first 5 patients

Receptive Comm Bayley-4 GSV[1] Latest Assessment[2] Change from Baseline	
Original 5 patients	**Canada / UK**
-1	6*
-4	7*
3	3
5	4
9*	12*
	2
	8*
	21*
	25*

Expressive Comm Bayley-4 GSV[1] Latest Assessment[2] Change from Baseline	
Original 5 patients	**Canada / UK**
15*	3
6	4
-6*	4
0	0
0	2
	8*
	5
	12*
	-7*

* Statistically significant values : Improvement in green ■ Impairment in red ■

- Psychologist administered
- A score of +7 or higher is statistically larger than variation observed
- Current Canada/UK patients have a higher frequency of significant changes

In Natural History studies[3] scores on these measures do not meaningfully change

1 Bayley-4 Growth Scale Values. Threshold for statistically significant difference ($p < .05$): RC and EC = 6
2 Latest assessment is Day 128, except for Patients 103-001, 103-002, and 103-003 where it is Day 170
3 Keute, M et al, *Mol Psych*, 2020 https://doi.org/10.1038/s41380-020-0858-6

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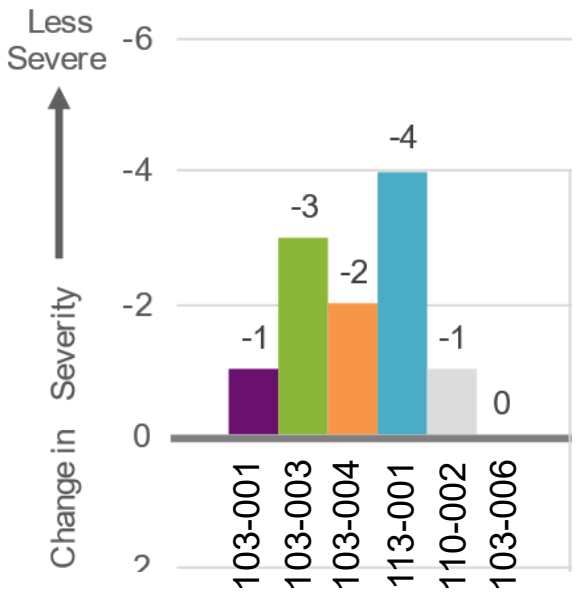
Sleep Domain: Substantial, clinically meaningful changes

Cohort 4 : AS Sleep Change in Severity



Comments from Caregivers on Sleep

- **103-003:** "Her sleep has improved which is humongous, and very helpful for me and our whole family. She's more rested, we're all more rested. So everyone's able to function better and she's able to do more."
- **113-001:** "Before [the trial], she would wake up like three, four times in the night. Now, she doesn't wake up at all, and she'll sleep for a good 12 hours. And before, she'll sleep for two, three hours max."
- **(cohort 5) 103-005: (-2 improvement in AS Sleep score)** "…before the trial, I would hear her more often in the middle of the night. I wouldn't necessarily go to her, but I could hear her wrestling and reading a book or shaking her water bottle and I can say that since mid-trial, I think she's sleeping much more soundly. I often don't hear a peep out of her…..So I think – her sleep has improved."

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GTX-102 Safety Profile Under Amended Protocol
Improved administration procedure

- No adverse events of leg weakness
- Patients treated for over 1 year
- Starting doses range from 2 mg to 10 mg with escalation up to 14 mg
- Total of 71 doses administered
- Cumulative doses up to 61 mg

- 2 patients rechallenged with no resulting lower extremity weakness
  - Both patients in Cohort 1 have been re-dosed with 2 or 4 doses

Next program update based on substantive data on a larger number of patients in the program

Data as of 25 Oct 2022

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UX143 (setrusumab) for Osteogenesis Imperfecta

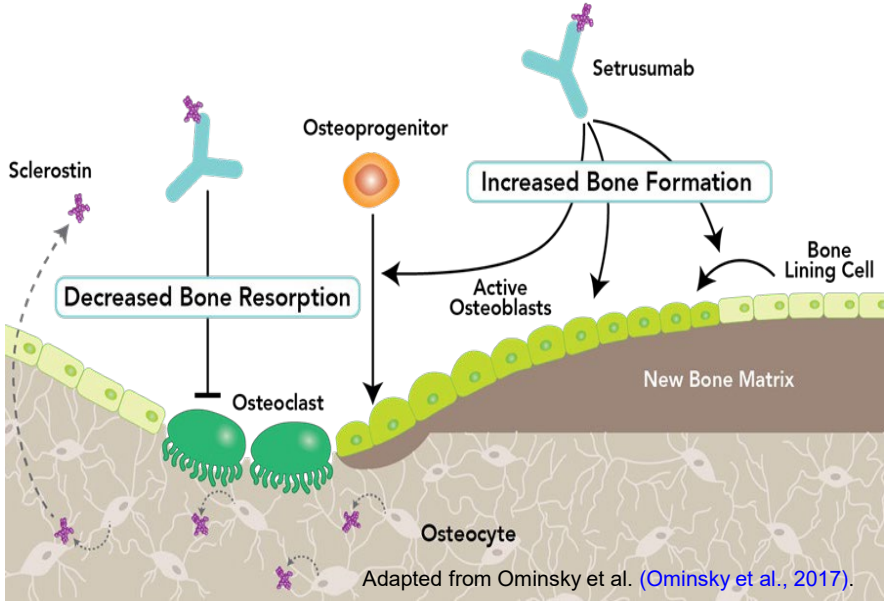
Enrolling pivotal Phase 2/3

UX143 for Osteogenesis Imperfecta (OI)

Large genetic bone disorder with positive Phase 2b adult data

- **OI:** Reduced or abnormal collagen causes increased bone resorption and inadequate bone production
  - Leads to decreased bone mass, strength & fractures
- **No approved treatments;** bisphosphonates anti-resorptive treatments are off-label
- **UX143 (setrusumab):** Fully human anti-sclerostin antibody that increases bone formation and density
- **WW prevalence:** ~60,000 (targeting types I/III/IV)
- **Status:** Enrolling pivotal, Phase 2/3 study

Mechanism of Anti-Sclerostin Antibody



Adapted from Ominsky et al. (Ominsky et al., 2017).

Setrusumab reverses the abnormal bone biology by repressing excess resorption and increases bone production where it is needed

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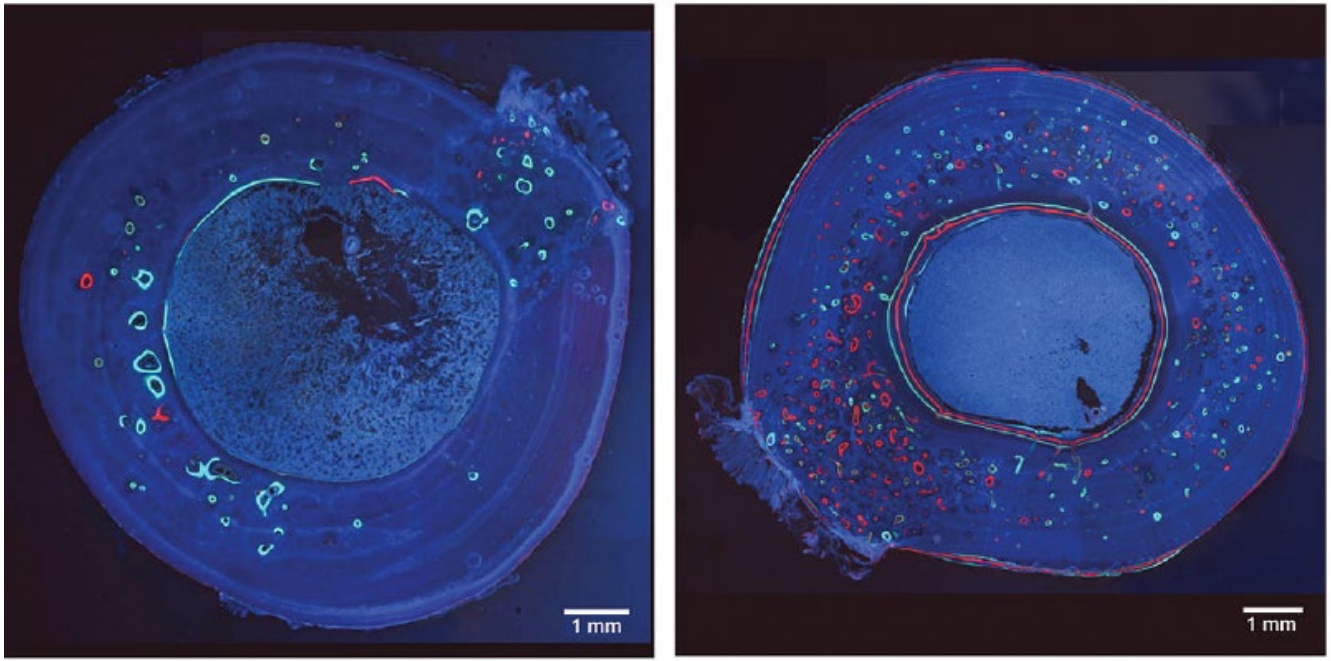
Anti-Sclerostin Antibody Increases Bone Formation on All Bone Surfaces

OVX Monkeys (12mo Tx)

Vehicle

Scl-Ab

Femur Diaphysis



Ominsky et al 2017

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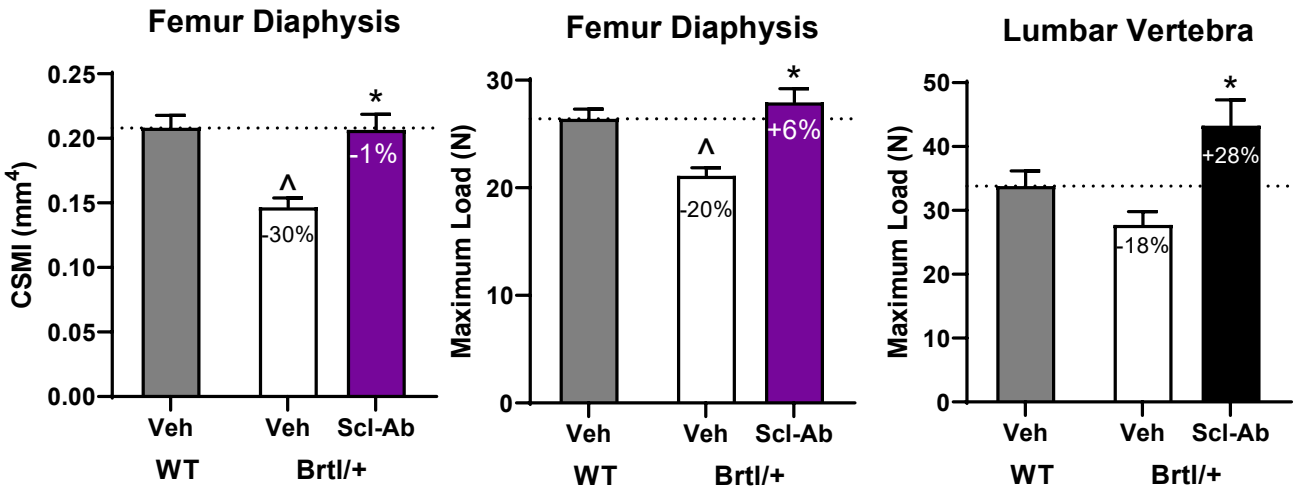
Setrusumab can normalize bone mass and strength in Brittle OI mice *even if collagen still mutated*

- Setrusumab (5wks) restored cortical bone geometry & strength in Brtl/+ mice to WT levels
- These changes were well correlated, demonstrating that increased bone quantity was sufficient to restore bone strength
- Anti-sclerostin is restoring normal bone physiology of production and resorption



Stephan ASBMR 2021; Mean ± SE, n=19-22/gp
^p<0.05 vs WT+Veh; *p<0.05 vs Brtl + Veh

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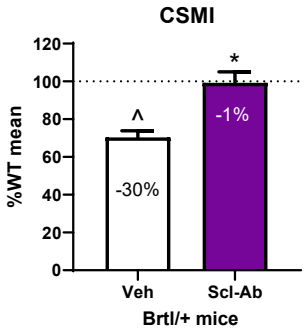
Setrusumab makes better bone than bisphosphonates

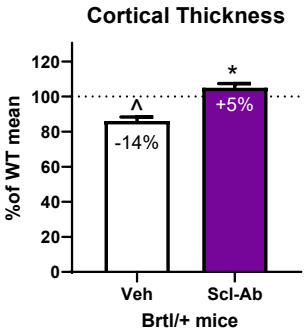
5wk Setrusumab vs 12wk Alendronate

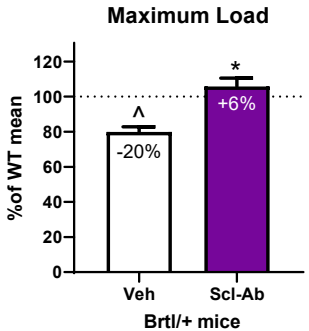
BONE MASS INDICES

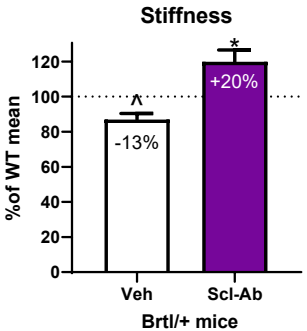
BONE STRENGTH INDICES

SETRUSUMAB

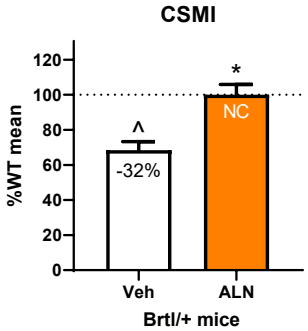


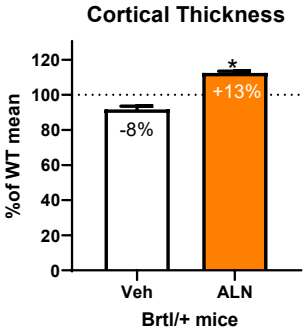


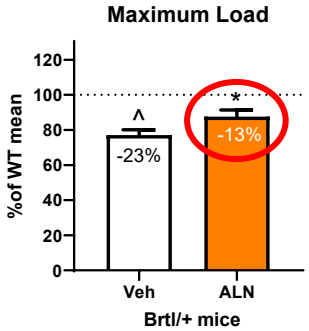


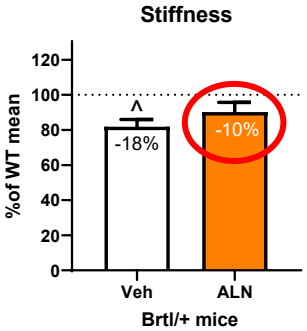


ALENDRONATE









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Phase 2b Adult OI study: Large Effects of Setrusumab

BMD and bone strength were dose-dependently improved

Mean % Δ from BL at 12 mos	Setrusumab 20 mg/kg	Setrusumab 8 mg/kg	Setrusumab 2 mg/kg
Lumbar spine aBMD	8.97 (p<0.001)	6.65 (p<0.001)	2.35 (p<0.05)
Total hip aBMD	2.48 (p<0.01)	2.69 (p<0.01)	1.99 (p<0.05)
Radius Total vBMD	1.88 (p<0.01)	0.86	0.04
Radius FE Failure Load	3.17 (p<0.01)	2.33	0.57

BMD Increases Consistent across Site and OI Type

Peripheral Bone Strength Indices Improved

Setrusumab was well tolerated in adults;
data will be available in children with OI as clinical trials progress

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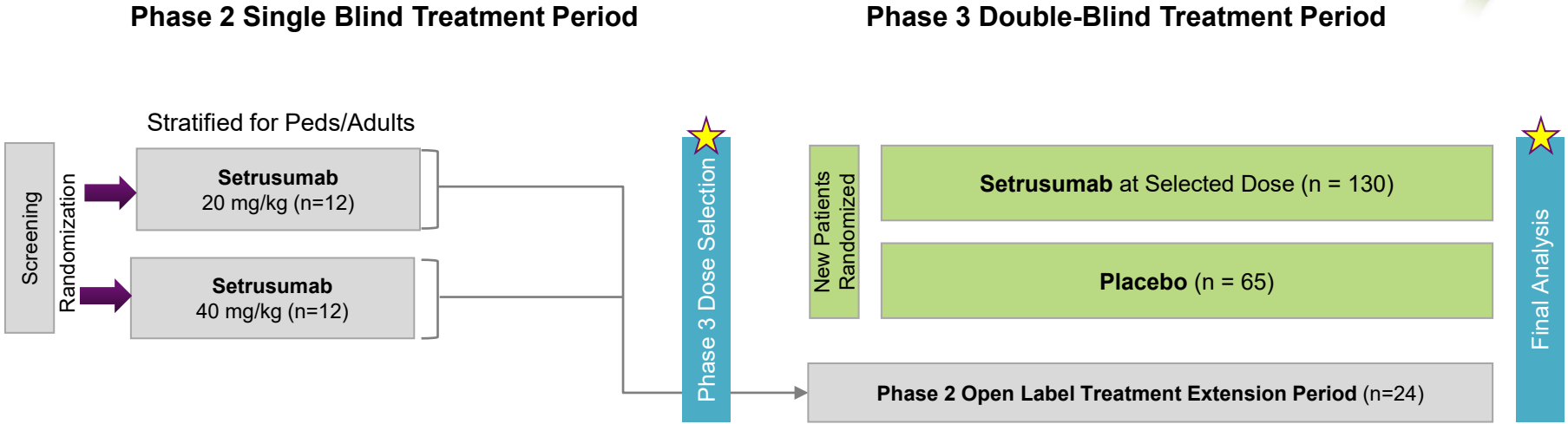
ORBIT Phase 2/3 Study Schema
Phase 2 biomarker data expected in mid-2023



- **Change from baseline in serum P1NP at Month 1**
- Safety
- Serum setrusumab concentration
- Change from BL in serum P1NP, CTx, BSAP, and OCN
- Change from BL in DXA lumbar spine BMD
- Anti-setrusumab Abs

- **Annualized fracture rate, excl morphometric vertebral fractures**
- Safety
- Annualized fracture rate, incl morphometric vertebral fractures
- Change from baseline in DXA lumbar spine BMD
- QoL: POSNA-PODCI (<18y); SF-36 (≥18 y): Physical Function / Pain
- Anti-setrusumab Abs

UX143: Next Steps

- Phase 2/3 *Orbit* study in patients 5-25 years old initiated in April 2022
  - Phase 2 portion: identify optimal dose strategy based on increases in collagen production using serum P1NP levels
  - Phase 3 transition: expected to initiate in mid-2023; evaluate fractures over 15-24 months
- Additional randomized bisphosphonate controlled study in patients <5 years old expected to be initiated in first half of 2023

Development program to be led by development organization that achieved rapid approval for Crysvita in XLH and TIO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Gene Therapy Platform

Diverse and Late-Stage Gene Therapy Pipeline

Candidate	Description	Pre-Clinical	IND	Phase 1	Phase 2	Phase 3	Approved	Est'd Patients in Dev. World
UX111 (ABO-102)	AAV9 Gene Therapy	Mucopolysaccharidosis Type IIIA (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

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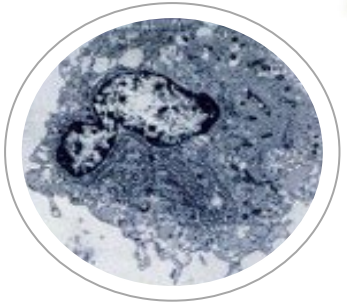
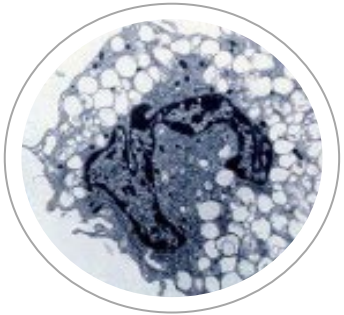
Sanfilippo Syndrome Type A (MPS IIIA)

Lysosomal storage disease leading to early neurocognitive decline & death

- **MPS IIIA:** Severe lysosomal storage disease due to an enzyme deficiency, causing abnormal accumulation of heparan sulfate (HS)
  - Progressive cognitive decline begin at age 1-2 to devastated state by early teens; severe neurological problems
  - Death by teens or twenties
  - No current therapies
- **UX111 is a self-complementary AAV9** intravenous gene therapy that provides the cross-correcting enzyme that can treat the brain
  - similar to the Zolgensma strategy
- **WW prevalence:** ~3,000 - 5,000
- **Status:** Evaluating biomarker and other data and are finalizing an approach to FDA regarding filing for approval



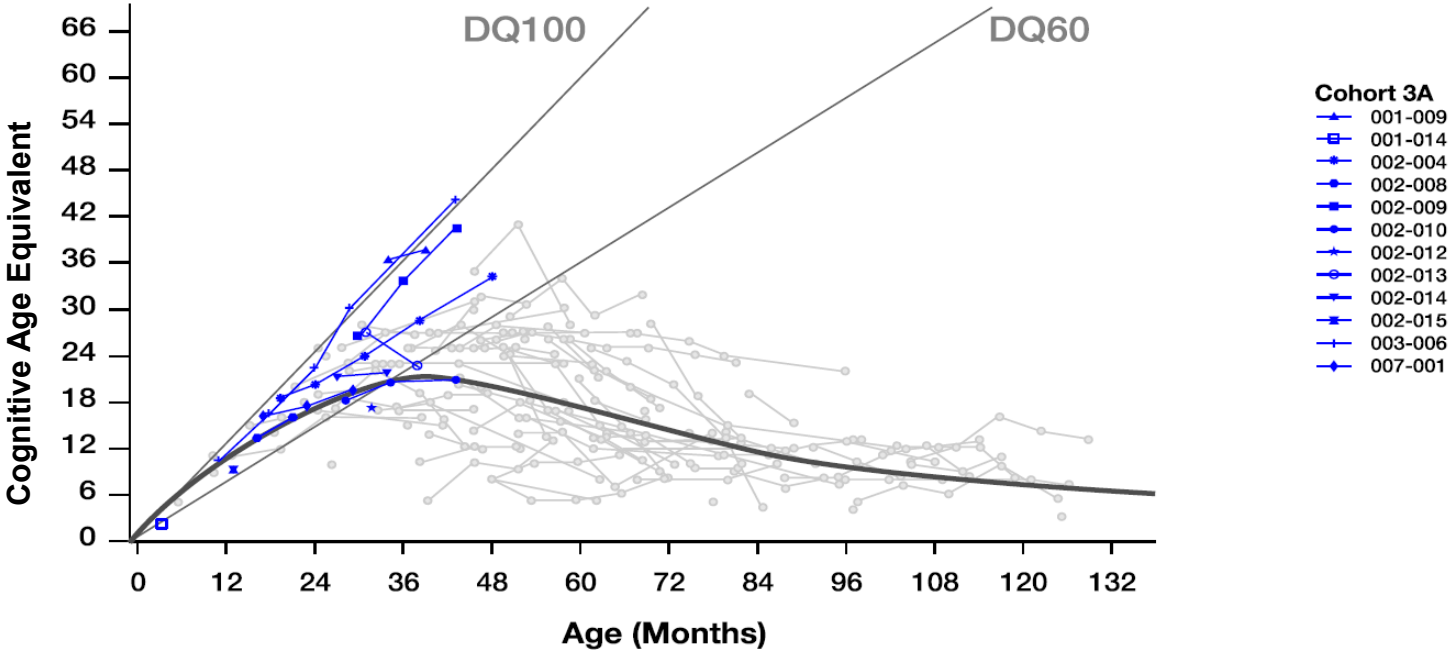
Normal cell



Cell with lysosome deficiency showing vacuolization

Mullen Cognitive Age-Equivalent Data

Patients treated at an early age track along normal development range



- **Black solid line and gray data points:** Typical developmental pattern for children with MPS IIIA per natural history data
- **DQ60 and DQ100 lines:** Expected development for children without disease. Development Quotient (DQ): ratio between age equivalent and actual age (chronological)
- **Cognitive age equivalent:** Functional age of the child, calculated by comparison with the age at which a child in the normal population develops similar skills

Flanigan *et al.* American Society of Gene & Cell Therapy (ASGCT), May 2022

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Summary of Transpher A Results and Next Steps

Pivotal study data with UX111

- Patients track along normal development range, showing continuous improvements
- Cohort 3 (highest dose, n=10), stabilization or increase in cortical gray matter, total cerebral, and amygdala volumes
- Statistically significant reduction in liver volume
- Sustained, and statistically significant reductions in biomarkers

UX111 was well tolerated

- Drug-related AEs have been grade 1 or 2 (mostly mild, grade 1) and all resolved within 2 months
- Subclinical ALT and AST elevations, low and transient AAV9-positive responses (8 patients), and mild thrombocytopenia (5 patients)

Meeting with FDA to discuss earlier filing path anticipated in 1H23

DTX401: AAV8 for Glycogen Storage Disease Type Ia

- **GSDIa:** Defect in liver's ability to release glucose to the circulation due to G6Pase deficiency
  - Severe life-threatening hypoglycemia
  - Long-term liver and renal disease
  - Severe long-term complications (70-80% patients)
- **Treatment:** Diet and cornstarch only
  - Keeps patients alive but not normal
  - Only curative approach is liver transplantation
- **WW prevalence:** 6,000
- **Status:** Last patient dosed in Phase 3 around the end of the year

Patient 3 Cornstarch when Travelling

Before After

"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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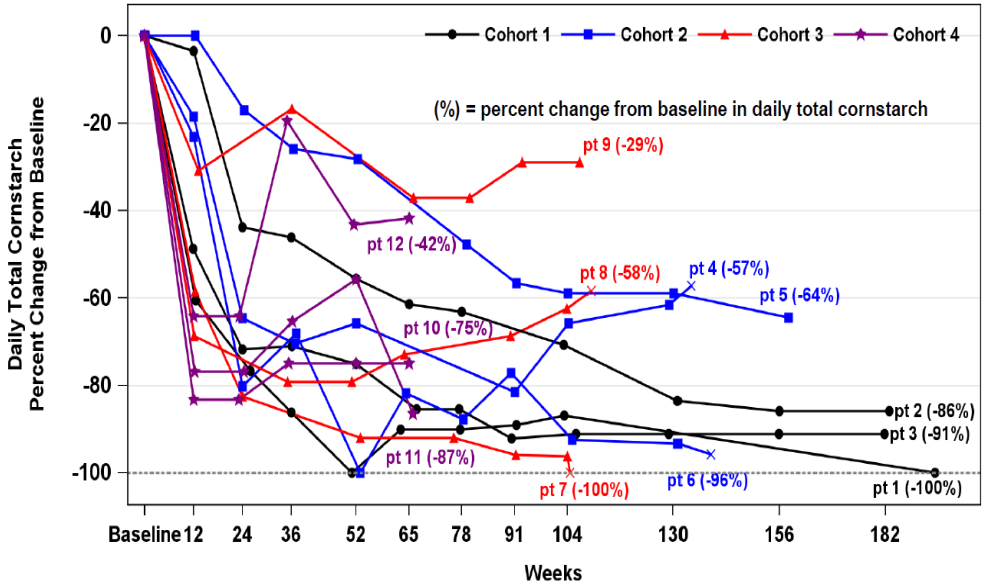
All Patients Sustained Reduction in Cornstarch Therapy While Maintaining or Improving Time in Euglycemia



Cornstarch Rx is a critical repetitive oral glucose infusion required to keep patients from hypoglycemia crashes and consequences.

It's a "gun to the head" every day and night their entire lives, patient and family

Overall mean reduction at last visit = 73.8%
(p<0.0001)

Cohorts 3/4: Cornstarch reduced 51-65%, while time in euglycemia increased or remained flat

Riba-Wolman *et al.* American Society of Gene & Cell Therapy (ASGCT), May 2022

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Enrollment and Dosing Ongoing in Phase 3 Study

Expect to complete enrollment around end of 2022

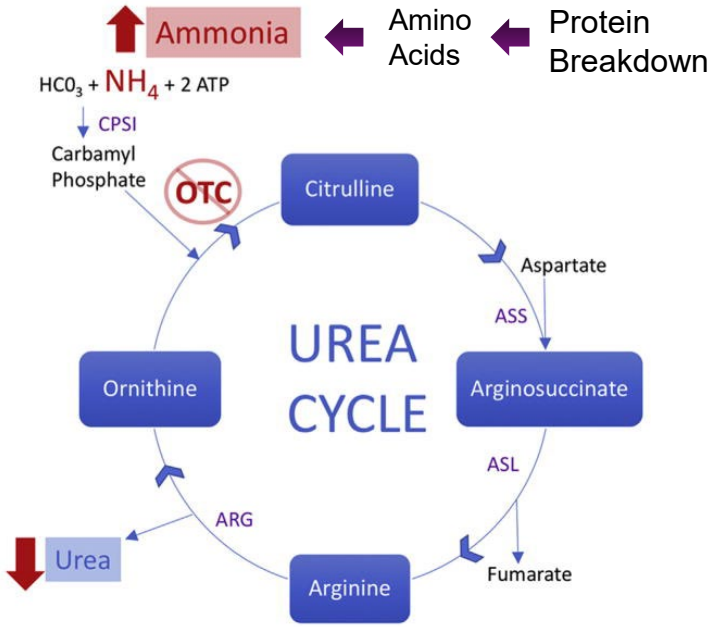
Phase 3 Study Design

- 50 patients, randomized 1:1 DTX401 (1.0 x 10^{13} GC/kg) to placebo
  - Patients on placebo will cross over to gene therapy arm
- 48-week duration
  - Longer-term Phase 1/2 data to be included in submission to support durability
- Primary endpoint: reduction in oral glucose replacement therapy (cornstarch) while maintaining or improving glucose control as measured by Continuous Glucose Monitoring (CGM)
- Secondary endpoints include time to hypoglycemia during fasting challenge, GSD Functional Assessment Diary (FAD)

DTX301: AAV8 for OTC Deficiency

AAV8 gene therapy for stable expression of OTC

- **OTC Deficiency:** X-linked urea cycle disorder, genetic defect in ammonia detoxification
- **Key symptoms/prognosis:**
  - Acute hyperammonemic episodes
  - Adverse cognitive & neurological effects
- **Treatment limited:** Liver transplantation only curative, ammonia scavengers, protein restricted diet
- **WW prevalence:** ~10,000, 80% late-onset
- **Status:** Ph3 FPI around the end of the year



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

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Seven of Eleven Patients Show Durable Metabolic Control and Sustained Responses up to 4.5 years

Dose Cohort	Patient # (Gender); Follow-Up	Ammonia Levels (baseline → after treatment)	Current Response Status
1	1 (M); 234 wks	Normal levels maintained	**Complete responder**
2	4 (M); 182 wks	Normal levels maintained	**Complete responder**
2	6 (F); 182 wks	91% decrease from Baseline	Responder
3	7 (F); 130 wks	Normal levels maintained	**Complete responder**
3	8 (F); 104 wks	56% decrease from Baseline	Responder
3	9 (M); 104 wks	Normal levels maintained	Responder
4	11 (F); 52 wks	58% decrease from Baseline	**Complete responder**

- No treatment-related serious AEs, infusion-associated reactions, or dose-limiting toxicities have been reported to date
  - Eight patients had mild, asymptomatic ALT increases which resolved with a protocol-specified tapering regimen of oral corticosteroids

Harding *et al.* American Society of Gene & Cell Therapy (ASGCT), May 2022

DTX301: Phase 3 Design

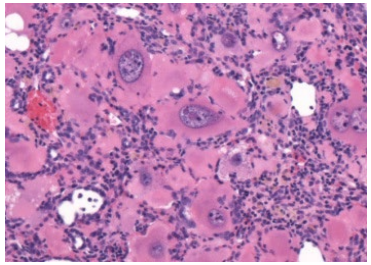
Phase 3 Study Design

- 50 patients, randomized 1:1 DTX301 (1.7 x 10^{13} GC/kg) to SOC/placebo
  - Patients on SOC/placebo will cross over to gene therapy arm
- 64-week duration
  - Longer-term Phase 1/2 data to be included in submission to support durability
- Primary endpoints
  - Complete response as measured by discontinuation of baseline disease management
  - Ammonia control as measured by 24-hour ammonia levels
- Enrollment beginning in around the end of the year

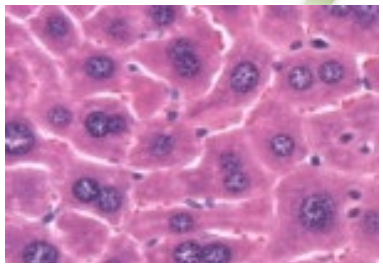
UX701: AAV9 for Wilson Disease

Second clinical program to utilize PCL manufacturing system

- **Wilson Disease:** Causes copper to accumulate in liver, brain and other vital organs
- **Key symptoms/prognosis:** Liver failure, neurological deterioration, death
- **Standard of Care:** Chelation therapy and dietary restriction
  - Many patients still experience liver and neurological deterioration
- **WW prevalence: >50,000**
- **Status:** Stage 1 (dose finding) enrollment completion mid-2023

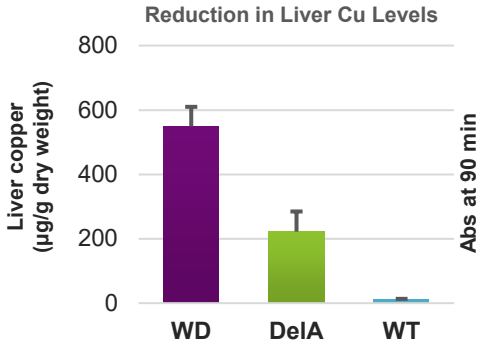


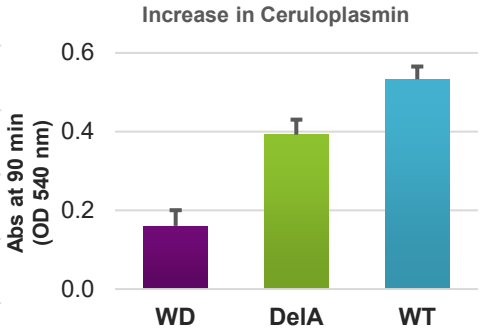
Untreated KO Mice



1x10^{11} GC Treated Mice

Reduced Liver Copper Accumulation Leading to Improved Liver Pathology in Preclinical Models





* Copper metabolism measures include 24-hr urinary CU, ceruloplasmin concentration, ceruloplasmin activity, non-ceruloplasmin bound copper, and total serum copper

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Broad and Diverse Clinical Pipeline to Build on Strong Commercial Foundation

Stable & Growing Revenue Base

>$300M revenue in 2022 from current commercial portfolio

Significant Clinical Catalysts over Next Few Years

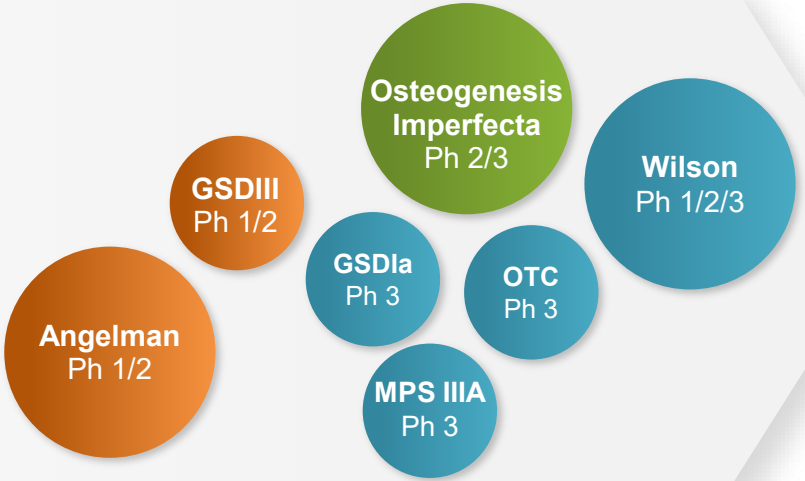
Diversified across modalities and smaller and larger indications

CRYSVITA® burosumab — XLH & TIO

DOJOLVI® TRIHEPTANOIN Oral Liquid — LC-FAOD

Evkeeza® (evinacumab-dgnb) Injection — HoFH (ex-US)

Mepsevii™ (vestronidase alfa-vjbk) injection — MPS VII



Protein Biologic | Small Molecule | Gene Therapy | ASO / mRNA

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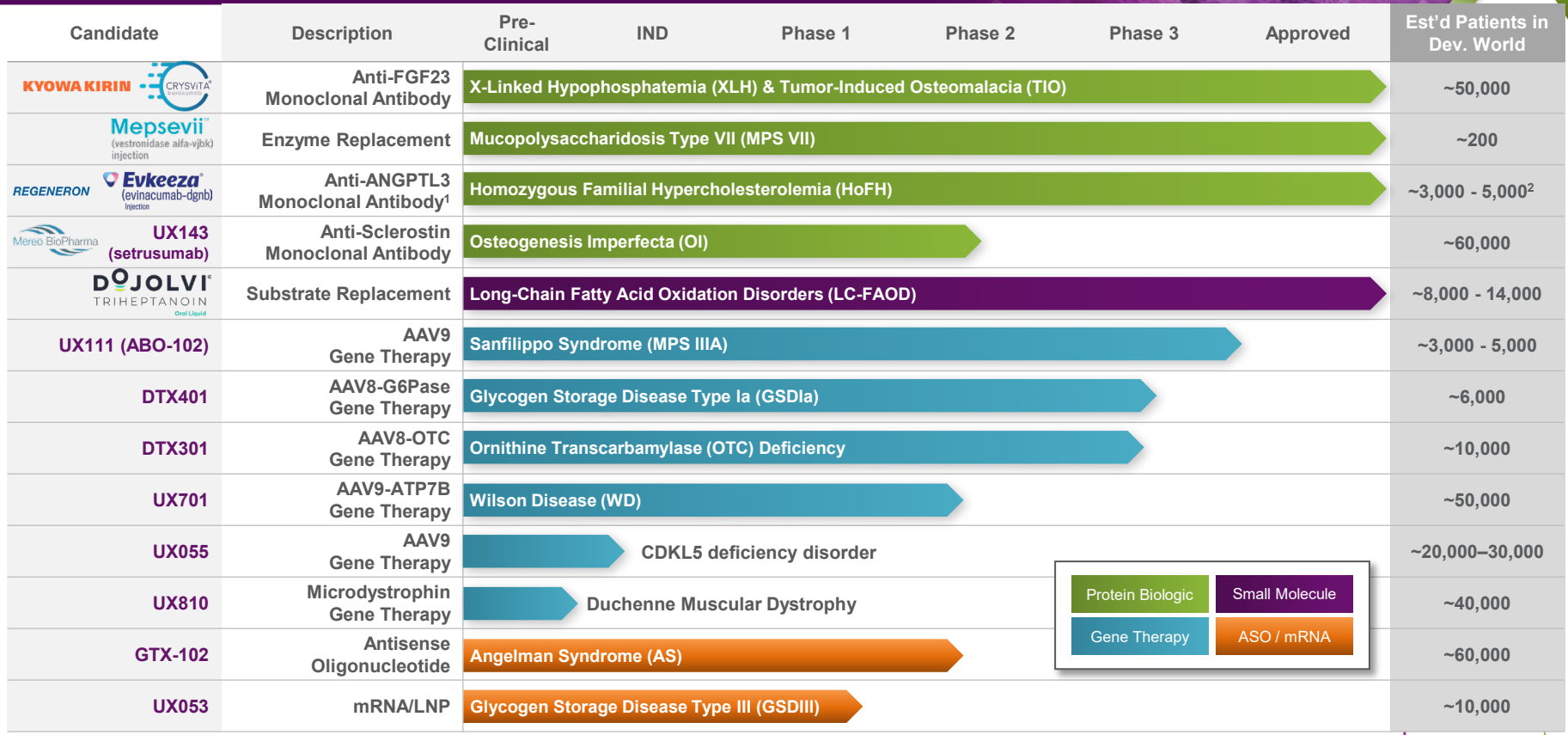
Appendix

Diverse Clinical Pipeline with Larger Indications to Drive Long-Term Growth



1: Ultragenyx licensed ex-US rights to Evkeeza from Regeneron
2: Excludes the US, where Regeneron has rights

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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 (2022-2032)[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)

[1]Includes granted U.S. patent term extension
[2]Includes projected U.S. patent term extension

Product	License	EU Intellectual Property Rights/Royalties
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

[3]Includes projected extension via supplementary protection certificates (SPCs)

Key Licenses & Intellectual Property – Clinical Programs

Product	License	US Intellectual Property Rights/Royalties
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
DTX401 (GSDIa)	Sub-License from REGENXBIO of UPENN IP	▪ AAV8 Capsid (2022-2024) ▪ Low to mid single-digit royalty
	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 and commercial milestones plus royalties
	Abeona Therapeutics	▪ Development and commercial milestones plus royalties
DTX301 (OTC Deficiency)	Sub-License from REGENXBIO of UPENN IP	▪ AAV8 Capsid (2022-2024) ▪ Recombinant vectors comprising codon-optimized OTC gene (2035) ▪ Low to mid single-digit royalty
DTX201 (Hemophilia A)	Sub-License from REGENXBIO of UPENN IP	▪ Hu37 Capsid (2024) ▪ Recombinant vectors comprising codon-optimized Factor VIII gene (2037) ▪ Low to mid single-digit royalty
UX701 (Wilson Disease)	Sub-License from REGENXBIO of UPENN IP	▪ AAV9 Capsid (2024-2026) ▪ Mid to high single-digit royalty
	UPENN	▪ Recombinant vectors comprising certain regulatory and coding sequences packaged in UX701 (2039) ▪ Development and commercial milestones 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 royalties
UX053 (GSDIII)	Arcturus Therapeutics	▪ Various cationic lipids including the lipid used in UX053 (2034-2038) ▪ Various codon-optimized mRNA sequences encoding AGL including the codon-optimized version expressed by UX053 (2039) ▪ Development and commercial milestones plus low to mid single-digit royalty

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 projected 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

2034 — Ultrapure Dojolvi (Pending)

*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

Evkeeza® (evinacumab-dgnb) Injection

Data & Marketing Exclusivity

2031

Evkeeza Ab Patent

2036[1]

Exemplary additional patent applications pending:

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

Projected expiration dates between **2037-2041**

2022 2024 2026 2028 2030 2032 2034 2036 2038 2040

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