



Clinical-Stage CLN-301 · X-Gene Regulation (XGR)

5+ years of human data in CLN3 Batten disease — and genetic medicine's first 'same drug, multiple indications' platform

CLN-301 · CLINICAL-STAGE CATALYST

4 patients dosed · 5+ yrs human data

BLA targeted 2028 · RMAT · Fast Track · ODD · RPDD

+ Expected PRV ~\$150–200M on BLA approval

XGR PLATFORM · LEAD: XGR-201 CDKL5

Wholly-owned XGR-201 CDKL5

Dual human PoC **(Rett + CDKL5)** targeted 2027

A pathway to extend CLN-301 gene therapy to the eye

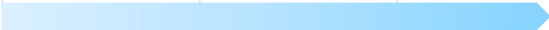
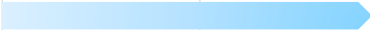
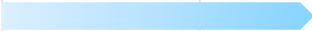
Prepared for Battens Disease Foundation · its Board and its families · August 2026

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Our Pipeline

Wholly-owned XGR-201 CDKL5 registrational lead, a clinical-stage CLN-301 catalyst, and a category-defining XGR platform

Program	Pre-clinical	IND-enabling	Clinical	Registration	Unmet Need	Key Milestones
CLN-301 CLN3 Batten Disease					>610 ³ treatable patients (US, EU, Aus) • Fatal • No approved drugs ✓ RMAT + Fast Track + ODD	BLA targeted 2028 PRV Eligible (\$150M-\$200M)
XGR-201 (CDKL5, lead) + XGR-101 (Rett, upside)					>60K ¹ CDKL5 + Rett Severe • No disease-mod Tx ✓ Preclin + mfg in-process	Rett and CDKL5 Human PoC H2-2027 XGR-201 BLA targeted 2031
XGR Platform Extension: PCDH19 lead; Aicardi + More follow					>45K ² additional across the broader X-linked class (CNS + systemic) Same XGR drug, multi-indication	PCDH19 IND filing targeted 2028 Other X-linked diseases 2028+

CAPITAL EFFICIENCY: One XGR drug, multiple indications. CLN3 clinical-stage anchor. Three value events targeted on \$110M: CLN-301 BLA + PRV, CDKL5 (XGR-201), and Rett/XGR BD optionality. Modality-expansion already in hand: Efralex, a preclinical EphA4 program, extends the CNS portfolio.

BACKED BY

Biogen

Nationwide
Children's

RTW
Investments

UC Davis

UC Riverside

Top-5 Global
Pharma (Confidential)

¹ <https://pubmed.ncbi.nlm.nih.gov/36642718/>, <https://pmc.ncbi.nlm.nih.gov/articles/PMC10801578/>, Ultragenyx public disclosure

² <https://www.nature.com/articles/s41398-024-02753-x>, <https://aicardisyndromefoundation.org/aicardi-syndrome-prevalence-and-survival/>, <https://rarediseases.org/rare-diseases/incontinentia-pigmenti/>, <https://providers2.genedx.com/Resources/TIS-Files/TIS-313-v072021.pdf>, <https://www.ncbi.nlm.nih.gov/books/NBK554742/>, <https://www.nature.com/articles/s41598-023-34053-7>

³ <https://pmc.ncbi.nlm.nih.gov/articles/PMC5505770/pdf/nihms870971.pdf>, <https://pmc.ncbi.nlm.nih.gov/articles/PMC12871039/pdf>, <https://beyondbatten.org/understanding-batten/what-is-batten/>

Leadership — Built and Exited Genetic-Medicines Companies

The scientists who invented the treatments are the ones advancing them

PJ Anand

Founder, President & CEO

25+ yrs building and exiting biotech / medtech. Founded Alcyone (acquired by Biogen). Serial founder — 4 companies, 2 acquisitions.

Kathrin Meyer, PhD

Co-founder, CSO & Head of R&D

Co-inventor of XGR & CLN-301. Led Itvisma (intrathecal Zolgensma, FDA-approved 2025); Zolgensma team at NCH. 2 FDA approvals; 7+ AAV programs to clinic.

Mike Tillinger, MD

SVP, Clinical Development

CNS rare-disease clinical development. Former Wave Life Sciences, Sanofi, Teva, Novo Nordisk.

Deep Singh

Co-founder, EVP Business & Operations

Biotech / medtech R&D, operations, corporate development. Founding exec at Alcyone (acquired), Abbott, Integer, Haemonetics.

Board of Directors & Scientific Advisors

Governance and scientific leadership with proven CNS, gene-therapy, and venture track records

BOARD OF DIRECTORS

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GTCR

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Pres., Abigail Wexner Research Inst.,
Nationwide Children's

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Director, Batten Disease CoE, Rett Center of Excellence
Nationwide Children's

Mihaela MacNair, PhD

Regulatory

Genetic-therapy & CNS rare-disease regulatory leader

Maurizio Pellecchia, PhD

Co-inventor of Efralex

Director, Molecular & Translational Medicine, UC Riverside

Received Guidance on Neela's Programs From:

Regulatory and policy



Dr. Peter Marks, MD, PhD

Current SVP of Molecule Discovery & Head of Infectious Disease, Eli Lilly

Former Director, CBER, U.S. FDA (2016–2025)



Dr. Janet Woodcock, MD

Current Board of Directors, Every Cure & Friends of Cancer Research

Former Principal Deputy Commissioner and Acting Commissioner, U.S. FDA



John F. Crowley

Current President and Chief Executive Officer, Biotechnology Innovation Organization (BIO)

Former Founder and Executive Chairman, Amicus Therapeutics; Chief Executive Officer, 2005–2022



Judy Stecker

Current Council Member, Virginia Rare Disease Advisory Council; Healthcare Strategy Leader, Burson

Former Deputy Chief of Staff and Assistant Secretary for Public Affairs, U.S. Department of Health and Human Services

Received Guidance on Neela's Programs From:

Clinical and scientific



Dr. Emily De Los Reyes, MD

Principal Investigator, CLN-301 trial

Current Director, Batten Disease Center of Excellence and IRSF Center of Excellence; Attending Pediatric Neurologist, Nationwide Children's Hospital



Dr. An N. Dang Do, MD, PhD

CLN3 natural history, NIH

Current Head, Unit on Cellular Stress in Development and Diseases, NICHD, National Institutes of Health

Also Batten Disease Clinical Research Consortium; leads NIH's CLN3 natural history programme



Dr. Timothy Yu, MD, PhD

n-of-1 therapy in Batten disease

Current Attending Physician, Division of Genetics and Genomics, Boston Children's Hospital

Known for Led development of milasen — the first therapy custom-designed for a single patient, in CLN7 Batten disease



Dr. Wendy Chung, MD, PhD

Pediatric and neurodevelopmental genetics

Current Chair of Pediatrics, Boston Children's Hospital; Mary Ellen Avery Professor of Pediatrics, Harvard Medical School

Also Member, National Academy of Medicine; PI, GUARDIAN newborn genomic screening study

Batten Disease Gene Therapy

CLN-301: Disease Modifying Therapy to treat CLN3 Batten Disease

Clinical-Stage: RMAT | ODD | Fast Track | Rare Pediatric Disease

CLN3 Batten Disease – A Devastating Disease That Impacts Children

CLN3 Batten Disease



Disease Etiology

- Autosomal recessive
- Loss of function mutations in the CLN3 gene



Therapeutic Challenges

Standard of Care

- Entirely supportive, focused on managing symptoms

Limitations

- Inevitable progression of disease
- Inability to target CLN3 and slow down neurodegeneration
- Cannot be addressed with enzyme replacement



Market Opportunity

- >610¹ patients in US + EU + Aus alone
- No approved disease therapy

Clinical Manifestations



Age 4-6 – Rapidly Progressing Vision Loss

- Vision loss, night blindness, loss of periphery, loss of visual acuity



Age 4-9 – Cognition and Behavior Problems

- Anxiety, depression
- Angry, aggressive behavior
- Hallucinations
- Sleep problems



Age 10-13 – Seizures, Speech and Movement Challenges

- Seizures start
- Complete blindness
- Speech impairment
- Movement and walking difficulties



Age 14-25 – Memory, Reasoning, Speech, Heart, Movement Challenges

- Memory and reasoning problems
- Loss of speech
- Issues with swallowing and chewing
- Cardiac manifestations
- Loss of independent walking
- Death usually occurs in mid-20s

¹ <https://pmc.ncbi.nlm.nih.gov/articles/PMC5505770/pdf/nihms870971.pdf> , <https://pmc.ncbi.nlm.nih.gov/articles/PMC12871039/pdf> , <https://beyondbatten.org/understanding-batten/what-is-batten/>

CLN-301: Classical AAV9 Gene Replacement with Precedented Components

- Intracellular membrane-bound protein
- Unlike CLN1 and CLN2, CLN3 protein is not secreted
- AAV gene replacement allows making the protein inside the cells



P546 Promoter

- Truncated MeCP2 promoter, used and safe in several clinical trials
- More moderate expression levels compared to the strong CBA promoter, reducing overexpression toxicity risks

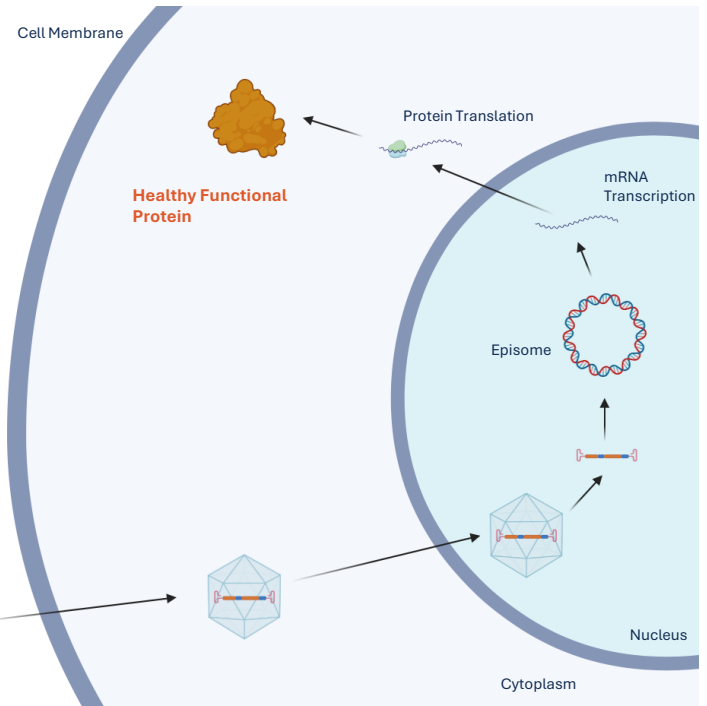
Transgene

- IP protected transgene cassette encoding human CLN3

Same vector backbone and components as ZOLGENSMA®

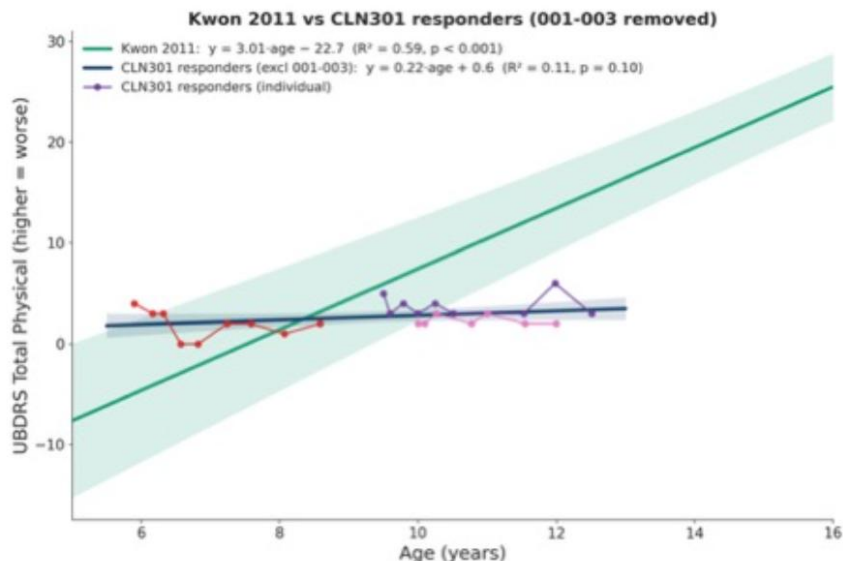
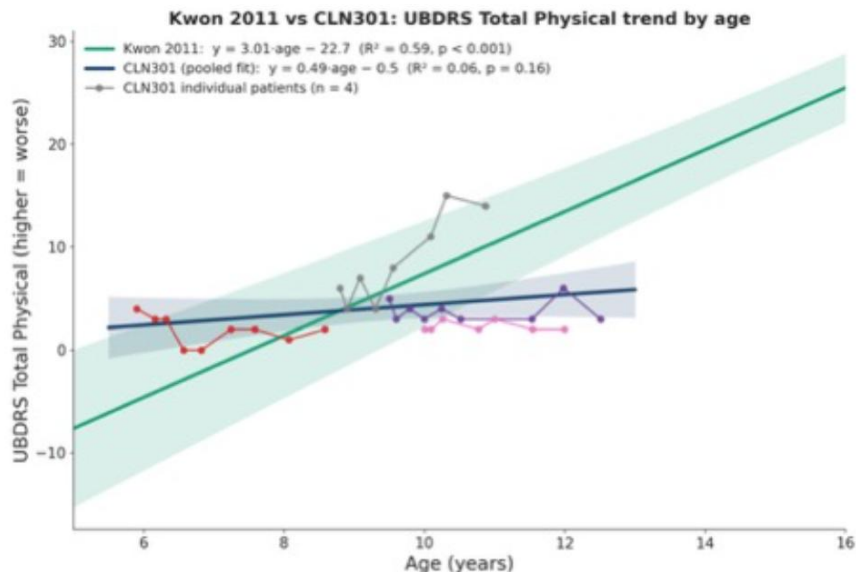
- ITRs, SV40 intron, bGH polyA

**CLN-301 – AAV9
Encapsidated
Gene Replacement Therapy**



(for illustration purposes only)

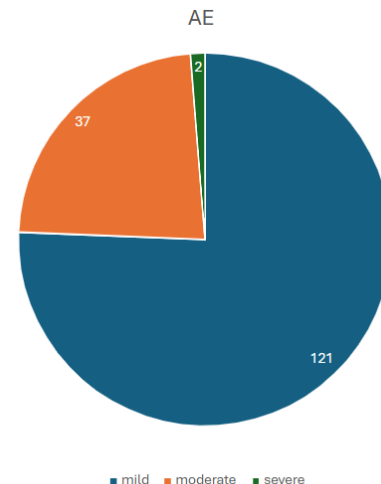
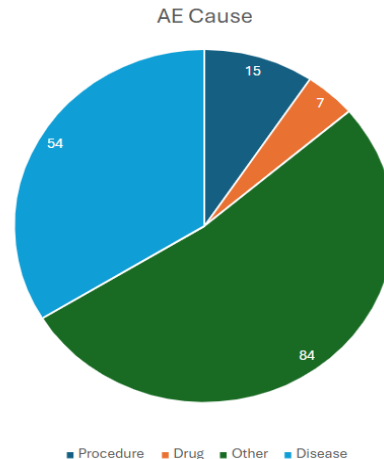
UBDRS PA: CLN-301 Participants vs Natural History of Disease



- Green line (Kwon 2011) shows the well established untreated natural-history of a linear deterioration of $\sim +3$ points/year on the UBDRS physical assessment score (higher score= worse)
- Treated participants: Purple line = participant 001; Red line = participant 002; Grey line = participant 003; Pink line = participant 004
- Pooled treated (n=4) is essentially flat (+0.49 points/ year)
- Responder subset (n=3; excluding participant 003) is essentially flat (+0.22 points/year) and the slopes differ significantly at the patient level ($p = 0.003$)

CLN-301 Safety To Date

- Well tolerated over 5 years of follow up
- 7/160 TEAE in 3 participants considered as drug related
 - All LFT elevations
 - 2 LFT elevations = related SAE
 - Confounding factor: concurrent rhino/enterovirus infection
 - Responsive to steroid treatment



CLN-301: Path to BLA in CLN3 Batten Disease

Inventor-led · Continuous patient follow-up since 2018 · Full BLA pathway (2028) · Plan for PRV monetization

★ **RMAT** · **ORPHAN DRUG** · **FAST TRACK** · **RPDD**

First and only disease-modifying therapy for a 100% fatal pediatric disease

01 · CLINICAL EVIDENCE

5+ Years of Durable Human Efficacy

- **Single intrathecal AAV9 administration** — one-time treatment
- **Cohort I (low dose, n=3)**: quantified UBDRS and J. Hamburg outcomes show disease stabilization through 5+ years vs. natural history progression
- **Cohort II (high dose)**: 1 of 4 dosed; pathway to final cohort dosing post-FDA engagement
- **Continuous follow-up since 2018** — preserved unbroken through NCH → Amicus → Neela

02 · REGULATORY PATHWAY

Plausible Mechanism Framework

- **Full BLA approval pathway** — not accelerated
- **FDA Feb 2026 guidance** provides foundation for ultra-rare BLA with established mechanism + validated natural history
- **Q3 2026 FDA meeting** under RMAT designation; comprehensive briefing book ahead of meeting
- **Endpoints**: UBDRS and J. Hamburg — established CLN3 functional measures
- **Small-cohort BLA** — statistical framework leveraging natural history comparator in development

03 · CATALYST SEQUENCE

Path to ~\$150–200M PRV

- Q3 2026** Type B FDA meeting under RMAT
- Post-mtg** Final cohort dosing pathway initiated
- 2027** Cohort II enrollment complete
- 2028** BLA targeted
- 2029** PRV monetization + commercial launch
- Post-launch** PMR observational study extends RWE

THE PROOF



Cohort I patient

6 years post single dose

Should be wheelchair-bound by natural history, but is active (shown jumping on trampoline).

5+ years of continuous human durability data. Clear regulatory pathway. **Zero approved competitors.**

What CLN3 takes, and in what order

AGE 4–6

Vision

Night blindness, loss of peripheral vision, loss of visual acuity — usually the first sign

AGE 4–9

Cognition and behaviour

Anxiety, depression, aggression, hallucinations, sleep disturbance

AGE 10–13

Seizures, speech, movement

Seizures begin; speech and walking become difficult

TEENS ONWARD

Memory, swallowing, heart

Loss of speech, difficulty swallowing, cardiac involvement, severe movement problems

Vision is the first thing this disease takes. It is also the part our therapy currently reaches least well.

CLN3 Batten disease is autosomal recessive, caused by loss-of-function mutations in the CLN3 gene. It affects roughly 1 in 100,000 children. Death usually occurs in the mid-20s.

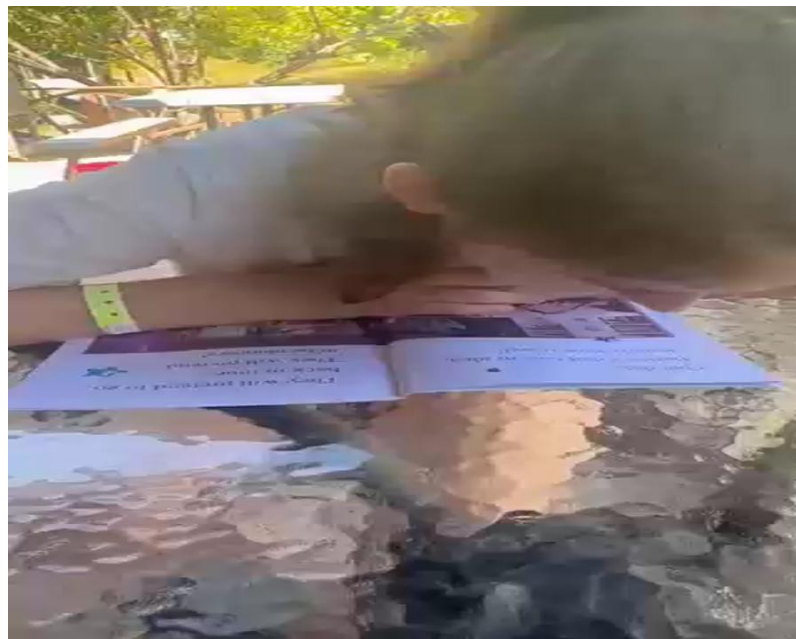
There is no approved therapy that changes the course of this disease.

CLN-301 is the only disease-modifying gene therapy for CLN3 currently in the clinic.

One Family's Account

Gene therapy gave her independence, which gave her happiness. She can do everything she enjoys on her own. Today she can still read books, navigate her iPad and phone, perform gymnastics, play board games, and make crafts — all without any help. Her older brother could no longer do any of these things by the time he turned eight.

— Mother of a treated participant, age 12, five years after treatment. December 2025. Shared with permission.



WHY THIS ACCOUNT MATTERS TO WHAT FOLLOWS

Almost everything this mother describes — reading, a phone screen, a board game, making crafts — depends on sight. It is the part of daily life that CLN3 takes first, and it is the part we most want to protect.

Credit: Nationwide Children's Hospital & The Ohio State University

The pathway — delivering to the eye as well as the spinal fluid

A staged program, beginning in animals and N of few led by an independent clinical investigator

01

A SMALL ANIMAL STUDY FIRST

A short non-human-primate study of intra-ocular delivery, to establish that the approach is safe and reaches the retina before any child is treated.

02

A SEPARATE REGULATORY FILING

An amendment to our existing FDA application, describing the added ocular route and the small study proposed to follow.

03

A STUDY IN A SMALL NUMBER OF CHILDREN

Dual administration — into the eye and into the spinal fluid — in a very small group, to ask whether we can protect sight alongside cognitive and motor function.

WHO WOULD DO THIS WORK

An independent clinical Principal Investigator would lead the study, working with Kathrin Meyer, our Chief Scientific Officer and the inventor of CLN-301, and Tom Mendel, a retinal surgeon at Ohio State.

HOW IT WOULD BE DONE

A novel surgical technique, together with an adjuvant that has shown safety and high AAV9 transduction in animal models. The medicine itself is unchanged — what changes is how it is delivered.

To be clear about what this is: a separate program from CLN-301. Its data would not form part of the CLN-301 approval submission, though it may serve as supporting evidence.

What Partnership Makes Possible

CLN-301 initial clinical data has shown that a single dose can slow what this disease does to the brain.

This pathway asks whether we can also protect what these children lose first.

A short animal study. A regulatory filing. A small number of children. Carefully, and in that order.

FOR THE CHILDREN ALREADY TREATED

Continued follow-up, and a therapy on a real path to approval.

FOR THE CHILDREN DIAGNOSED NEXT

A route that may protect sight as well as thinking.

FOR THE FOUNDATION & FAMILIES

A direct role in the design and progress of the work it supports.

XGR Platform

X-Gene Regulation for X-chromosome Disorders

Pipeline-in-a-Drug to treat Rett Syndrome, CDKL5 & Other X-linked Diseases

IND-Stage: Top-5 global pharma strategic advisory partnership

X-Gene Regulation (XGR): The Foundation of Neela's Long-Term Value Creation

X-Gene Regulation advantages over Gene Replacement

- Reactivate patient's own silenced healthy gene vs. express synthetic micro-gene
- Express endogenous protein and all isoforms as nature intended
- No risk of over-expression

For the first time in genetic medicines:

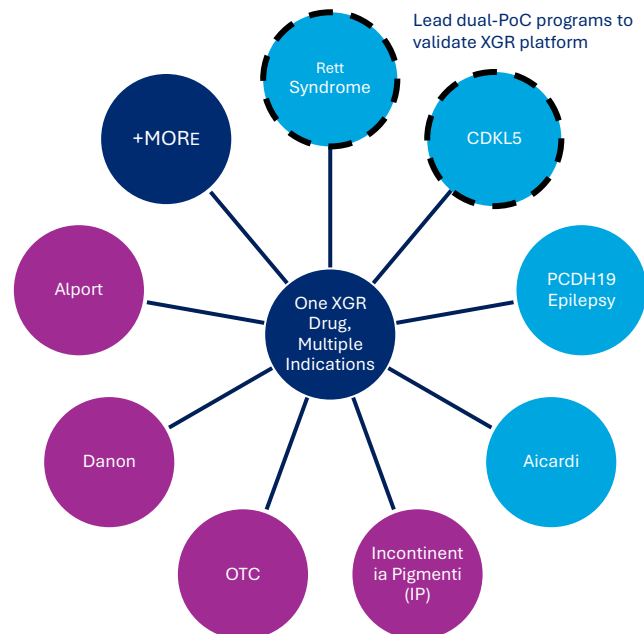
- Same exact drug in multiple X-linked diseases
- One CMC campaign applies to entire pipeline
- Highly capital efficient
- Transform rare diseases into scalable commercial enterprise

Neela is building a category-defining genetic medicines company through a focused, capital-efficient strategy that combines near-term value creation with platform-scale upside.

“If we could leverage a given vector and reuse it... we might be a lot better off.”

Peter Marks, MD, PhD · then-Director, FDA CBER (current Lilly) · BCG interview, 2024

Same Vector, Same Payload, Same CMC, Same Tox Package¹



>105,000 patients with same drug
CNS core NPV @100% >\$5B

CNS

Non-CNS

¹One confirmatory tox study may be needed for non-CNS indications (for systemic administration)

WHY THIS MATTERS FOR CNS

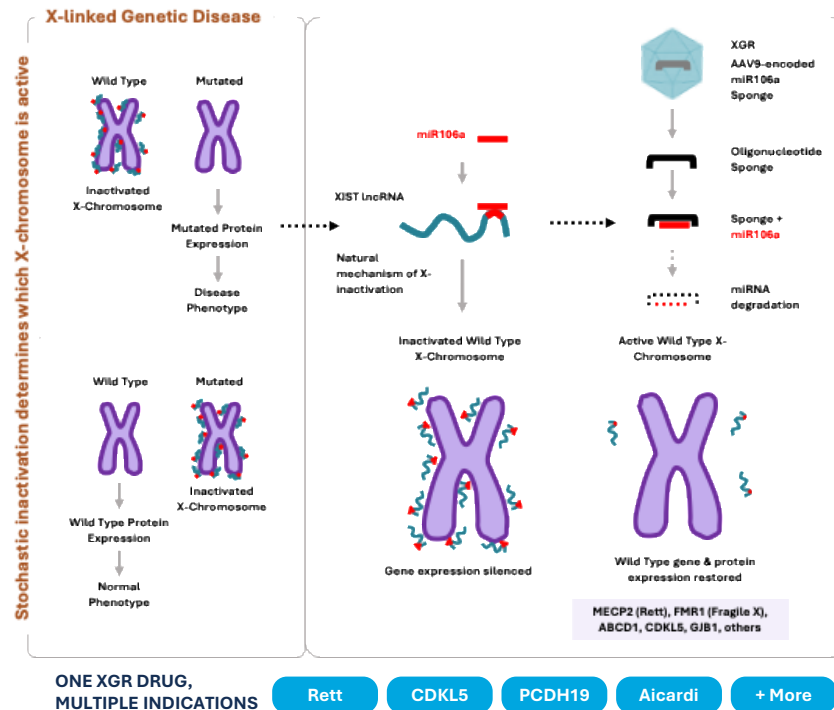
Reactivates the patient's own silenced healthy X chromosome

Native protein, all native isoforms, physiological expression

Cannot overexpress — by design (ceiling set by biology, not engineering)

Single same drug across an entire class of female X-linked CNS disorders

MECHANISM



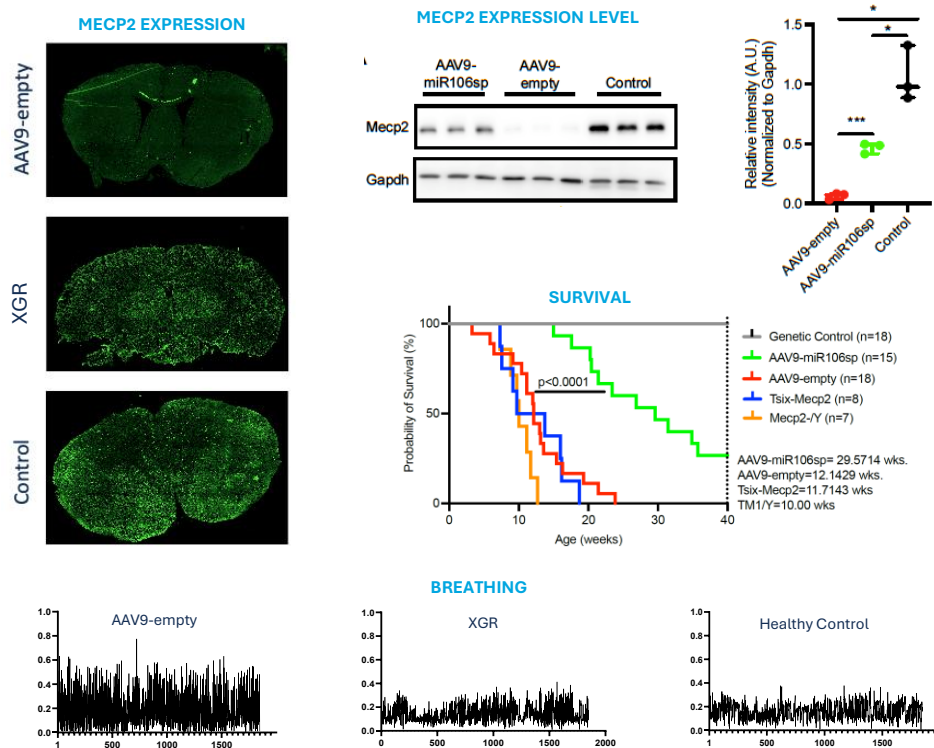
EXTERNAL STRATEGIC VALIDATION

Elite Global Pharma expertise is currently in advising our CMC, regulatory, and clinical execution pathways for XGR development.

XGR Platform IND-stage In Vivo Validation — Foundation for CDKL5 & Rett

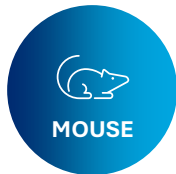
- IND-enabling dose response & GLP Tox complete
- PoCs in patient cell lines demonstrated in multiple X-linked diseases
- Efficacy in Rett Syndrome mouse model
 - Broad MECP2 upregulation and biodistribution in brain
 - Highly improved disease phenotypes even at moderate dose:
 - Motor and cognitive function
 - Improved overall health and tail lesions
 - Improved breathing (reduced apneas and breathing irregularities)
 - Increased brain volume
- CMC process established, clinical mfg. in-process

XGR Efficacy Validated in Rett Syndrome Mouse Model



In Vivo Safety Established with XGR

XGR leverages a naturally occurring MoA and is supported by substantial safety data



Dose Response Expression/Safety Study in Wild Type Mice

- Low, Medium, High dose cohorts evaluated
- Animals sacrificed at 1-, 3- and 6- months post injection
- **No adverse impact on neurobehavior, weight, or survival; no microscopic lesions in neural tissues**

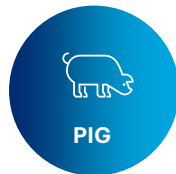
Safety Data Available in miR106a Knockout Mice

- Independent research study using miR106a KO mice¹
- Complete knockout of miR106a (representing maximal inhibition)
- **Does not cause aberrant phenotypes or disease**



Safety Evaluation in Wild Type Non-Human Primates

- CSF delivery
- 4 months in life – no side effects observed
- Good biodistribution
- **No histopathology lesions including liver and neural tissues and DRGs**



IND-enabling GLP Toxicology Safety Study in Wild Type Pigs

- CSF delivery
- 1 and 3 months in life – no side effects observed
- Good biodistribution
- **No histopathology lesions including liver and neural tissues and DRGs, no hematology or serum chemistry findings**



Safety Evaluation in iPSC Derived Rett Syndrome Neurons

- RNA-seq analysis post dosing iPSC derived Rett Syndrome neurons
- XGR-101 only partially reactivates Xi, providing another mechanism for safety
- **XGR-101 did not substantially alter the total expression levels of X-linked genes expressed at normal levels**

XGR-101 was safe and well tolerated in mice, NHPs and Pigs at all doses tested which are expected to scale to therapeutic levels in humans.

Dual-Vanguard Human PoC: CDKL5 Registrational Lead + Rett Syndrome

DUAL PoC



RETT SYNDROME The Established Pathway

N=3-5

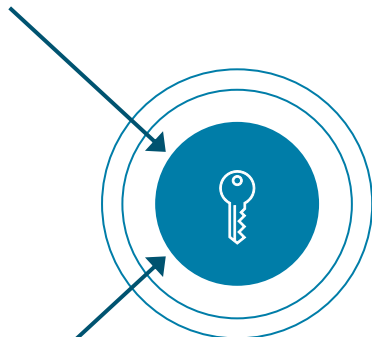
- ✓ High-conviction based on biological rationale
- ✓ Regulatory benchmarks established by conventional gene replacement therapies in clinic
- ✓ Rapid gain of developmental milestones expected within weeks post treatment
- ✓ FIH targeted H1 2027



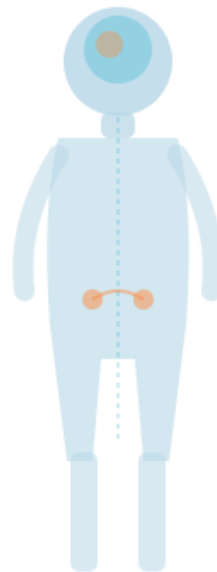
CDKL5 DEFICIENCY Biological Sibling

N=3-5

- ✓ CDKL5 was considered Rett Syndrome until 2018
- ✓ Distinct X-linked locus – proves generalizability of X-reactivation across genes
- ✓ High unmet need – no approved disease-modifying therapy, strong patient advocacy
- ✓ Minimal incremental investment – leverages Rett infrastructure and CMC



PLATFORM
UNLOCK



Brain & Cognition

Severe intellectual disability, loss of acquired skills, regression

Communication

Loss of speech, minimal verbal ability

Hand Stereotypies

Repetitive wringing, loss of purposeful hand use

Seizures

Epilepsy in >80% of Rett; early-onset in CDKL5

Motor Function

Gait abnormalities, ataxia, scoliosis

Breathing Apnea, hyperventilation

Shared Biology: Both Rett and CDKL5 are X-linked, female-predominant, CNS disorders caused by loss-of-function mutations. Both carry a silenced healthy gene copy on the inactive X in every affected cell.

CAPITAL EFFICIENCY: Two parallel N=3-5 studies across distinct X-linked loci. Same drug, same vector, same manufacturing. Together, 6-10 patients across 2 X-linked loci transform XGR into a validated platform.

CDKL5 (XGR-201): Wholly-Owned Path to BLA with a Best-in-Class Mechanism

Dual-vanguard strategy · differentiated mechanism · positioned to be first to registrational-stage in a disorder with no disease-modifying therapy

★ **TARGETED: ODD · RPDD · RMAT · BREAKTHROUGH · FAST TRACK**

Designation strategy — RMAT / Breakthrough with PoC data

01 · CLINICAL FOUNDATION

Trial-ready natural history

ICCRN prospective NH
(NCT05558371) validating CCSA,
CDD-Motor, CDD-Hand

ICDD — decade+ longitudinal;
Marinus used it for ganaxolone

CDKL5 Registry — real-world
caregiver data

Same construct & route as Rett
vanguard — inherits CMC, delivery,
safety

02 · REGULATORY PATHWAY

Seamless, precedent-based

OUS Geography Human PoC (n=3–
4) removes US IND clock

US IND for XGR-201 filed in parallel

Single seamless protocol — each
patient own control vs natural history

Registrational N≈20-30 sized to
TSHA-102 / NGN-401 (~30 total)

03 · PATH TO BLA

Wholly-owned to registration

2027

CDKL5 + Rett FIH +
PoC (OUS)

2028

FDA Meeting
Registrational Design

2029

Registrational enrollment

2030

Pivotal data

2031

BLA targeted

WHY BEST-IN-CLASS MECHANISM

Endogenous CDKL5
all native isoforms

No overexpression
one copy per cell

Lower vector dose
reduced capsid risk

Falcon biodistribution
ML + MRI-optimized IT
delivery

vs. gene replacement
Ultragenyx UX055 (pre-
clin), Elaa Bio ELJ-101
(pre-clinical, earlier
stage)

Validated registries + precedent regulatory design + differentiated mechanism = **a credible, wholly-owned path to the first CDKL5 therapy.**



APPENDIX

Rett Syndrome (XGR-101): Pure Upside — Partner or Retain

	Gene Replacement (Taysha, Neurogene)	X-Gene Regulation (XGR) (Neela)	Why It Matters for Rett Syndrome?
Mechanism	Delivers exogenous MECP2 coding sequence under artificial promoter and artificial regulatory elements	Reactivates patient's own endogenous MECP2 from silenced X	<i>Endogenous expression mirrors healthy biology – the therapeutic goal is restoration, not supplementation</i>
Expression Control	Requires engineered dose control (miRARE, EXACT) to manage supraphysiological expression risk	Native regulatory control — physiological levels with endogenous dynamic regulation	<i>Natural regulation addresses both potential concerns of overexpression as well as the need for dynamic MECP2 level changes that are modulated by miRNA and proteins binding to the UTRs of the MeCP2 transcript.</i>
MECP2 Duplication	Relies on synthetic controls; MECP2 duplication = separate severe disease	Eliminated by design — restores gene without adding transgene copies	<i>No ceiling on transgene-related therapeutic window. Dose-limiting toxicity concern removed from the equation.</i>
Platform Upside	Single-indication specific to the gene of interest (Rett Syndrome only)	Same drug for CDKL5, BPAN, Fragile X, PCDH19, Incontinentia Pigmenti	<i>Rett + CDKL5 investment unlocks an entire X-linked franchise — not a single-product bet.</i>
Clinical Stage	Registrational (BTD, N=12–33, BLA conversations)	Human PoC H2 2027: N=3-5 Rett Syndrome + N=3-5 CDKL5	<i>Later entry with a mechanistically differentiated approach. Gene replacement validates the regulatory pathway; XGR offers a differentiated epigenetic reactivation mechanistically distinct option.</i>

KEY INSIGHT: First-generation gene replacement therapies have set precedence for the Rett regulatory pathway and validated pharma demand. XGR-101 carries three BD-premium attributes into a validated market: mechanistic differentiation (no MeCP2 dosage toxicity), sex-matched preclinical data, and CSO with two FDA gene therapy approvals.

***Mechanistic differentiation → BD premium. Zero additional capital from Neela investors expected.
Rett economics designed to flow back to the cap table.***

XGR Platform – Capital-Efficient Pipeline with Strategic Optionality

ONE DRUG PRODUCT — same vector, payload, CMC & tox · >88K CNS + >17K systemic patients addressable

XGR reactivates the silenced X — it does not target the disease gene — so a single construct applies across the entire female X-linked class.

ADDRESSABLE UNIVERSE · the X-linked class, one drug

Disease	Compartment	Female Patients
CLINICAL PoC – LEAD · Dual Readout 2027		
CDKL5	CNS	>25,000
Rett syndrome	CNS	>35,000
PLATFORM OPTIONALITY – Wholly owned and/or partnered		
PCDH19 epilepsy	CNS	>25,000
Aicardi	CNS	>3,000
Incontinentia Pigmenti	Systemic	>3,000
OTC	Systemic	>5,000
Danon	Systemic	>3,000
Alport	Systemic	>6,000
+ Other CNS / PNS / Systemic X-linked diseases		

Pipeline-in-a-Drug Demonstrated in Pre-clinical Studies

One XGR construct — 3 distinct X-linked genes rescued in patient cells

MECP2 Rett syndrome **CNS**

✓ Patient cells + in-vivo validated

MECP2 restored · neurite health rescued

GJB1 CMTX1 (X-linked CMT) **PNS**

✓ Patient-derived cells

Neurite length & health restored

WDR45 BPAN **CNS**

✓ Patient-derived cells

Neurite health restored

Cellular POC across distinct genes; Rett additionally validated in vivo (Lou et al., Nat Commun 2025).

CAPITAL EFFICIENCY — every indication leverages the same drug, the same CMC, and the same tox / safety package¹

¹One confirmatory tox study may be needed for systemic (non-CNS) indications — low risk. Patient-derived cellular POC shown; Rett additionally validated in vivo.

Female patient epi sources:

<https://pubmed.ncbi.nlm.nih.gov/36642718/>, <https://pmc.ncbi.nlm.nih.gov/articles/PMC10801578/>, Ultragenyx public disclosure, <https://www.nature.com/articles/s41398-024-02753-x>, <https://aicardisyndrome.foundation.org/aicardi-syndrome/incidence-prevalence-and-survival/>, <https://rare-diseases.org/rare-diseases/incontinentia-pigmenti/>, <https://providers2.genedx.com/Resources/TIS-Files/TIS-313-v072021.pdf>, <https://www.ncbi.nlm.nih.gov/books/NBK554742/>, <https://www.nature.com/articles/s41598-023-34053-7>