Delivering the promise of gene therapy

REGENXBIO Corporate Presentation

March 2026

REGENXBIO®

Forward-Looking Statements

This presentation includes "forward-looking statements," within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as "believe," "may," "will," "estimate," "continue," "anticipate," "assume," "design," "intend," "expect," "could," "plan," "potential," "predict," "seek," "should," "would" or by variations of such words or by similar expressions. The forward-looking statements include statements relating to, among other things, REGENXBIO's future operations, clinical trials, costs and cash flow. REGENXBIO has based these forward-looking statements on its current expectations and assumptions and analyses made by REGENXBIO in light of its experience and its perception of historical trends, current conditions and expected future developments, as well as other factors REGENXBIO believes are appropriate under the circumstances. However, whether actual results and developments will conform with REGENXBIO's expectations and predictions is subject to a number of risks and uncertainties, including the timing of enrollment, commencement and completion and the success of clinical trials conducted by REGENXBIO, its licensees and its partners, the timing of commencement and completion and the success of preclinical studies conducted by REGENXBIO and its development partners, the timing or likelihood of payments from AbbVie or Nippon Shinyaku, the monetization of any priority review voucher, the timely development and launch of new products, the ability to obtain and maintain regulatory approval of product candidates, the ability to obtain and maintain intellectual property protection for product candidates and technology, trends and challenges in the business and markets in which REGENXBIO operates, the size and growth of potential markets for product candidates and the ability to serve those markets, the rate and degree of acceptance of product candidates, and other factors, many of which are beyond the control of REGENXBIO. Refer to the "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of REGENXBIO's Annual Report on Form 10-K for the year ended December 31, 2025, which will be filed with the U.S. Securities and Exchange Commission (SEC). and comparable “risk factors” sections of REGENXBIO’s Quarterly Reports on Form 10-Q and other filings, which are available on the SEC’s website at www.sec.gov. All of the forward-looking statements made in this press release are expressly qualified by the cautionary statements contained or referred to herein. The actual results or developments anticipated may not be realized or, even if substantially realized, they may not have the expected consequences to or effects on REGENXBIO or its businesses or operations. Such statements are not guarantees of future performance and actual results or developments may differ materially from those projected in the forward-looking statements. Readers are cautioned not to rely too heavily on the forward-looking statements contained in this press release. These forward-looking statements speak only as of the date of this press release. Except as required by law, REGENXBIO does not undertake any obligation, and specifically declines any obligation, to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

Our Vision

A world in which debilitating diseases can be treated with a one-time therapy, resulting in lasting benefits

Our Mission

Seeking to improve lives through the curative potential of gene therapy

About REGENXBIO

Industry-Leading AAV Platform

100+ NAV® vectors,
5 licensees,
5,000+ patients dosed

Late-Stage Rare and Retinal Programs

Multiple potential first- or best-in-class candidates in or entering pivotal study

Commercial Readiness

Near-term catalysts and in-house, U.S.-based manufacturing drive transition to commercial company

Innovative Next-Gen Capsids

Expanding pipeline with capsids designed for improved tropism and transduction

abbvie

NIPPON SHINYAKU CO., LTD.

Global Partnerships

Global eyecare collaboration with AbbVie and US + Asia partnership with Nippon Shinyaku for MPS II and MPS I

Industry-leading Manufacturing

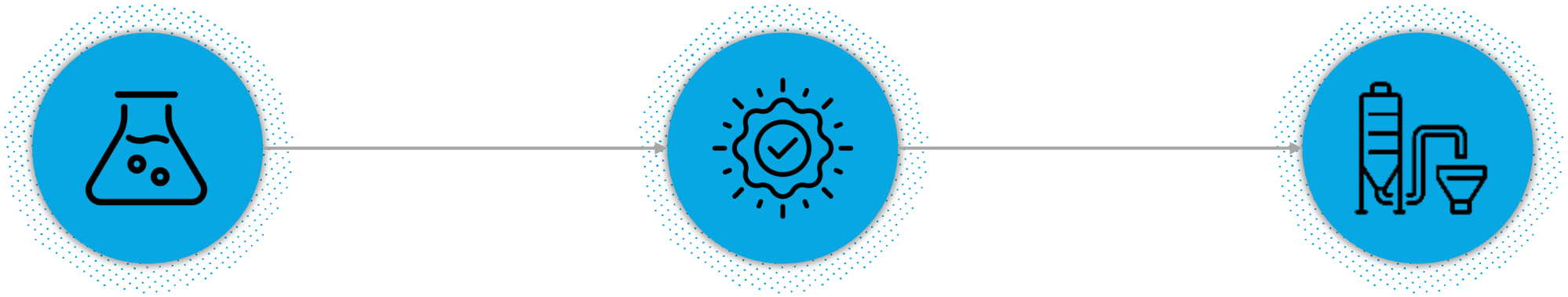
FDA-inspected cGMP® facility capable of production at commercial scale

FDA

Orphan Drug, RMAT & Fast Track Designations

Six designations provided across programs to date

Leveraging in-house, end-to-end capabilities to deliver potential first- or best-in-class therapies



Capsid Discovery & Engineering

Innovating new capsids engineered for improved expression, on-target tissue specificity, safety, manufacturability, and increased transduction, leveraging our strong foundation established with NAV®AAV8 and AAV9

Clinical Development Engine

Advancing gene therapy candidates designed to maximize therapeutic benefit through innovative constructs, delivery methods and proactive safety approaches

Industry-Leading Manufacturing

Optimizing purity, productivity, and manufacturability at commercial scale at our FDA-inspected, U.S. facility

Our gene therapy franchise for rare and retinal diseases

Disease Area	Indication	Product Candidate	Phase 1	Phase 2	Phase 3	Commercial Rights
Rare Disease	Duchenne Muscular Dystrophy (DMD)	Novel microdystrophin *NAV® AAV8*	RGX-202			*WHOLLY OWNED*
	Hunter Syndrome (MPS II)*	Direct delivery of IDS to CNS *NAV® AAV9*	RGX-121			NIPPON SHINYAKU CO., LTD. REGENXBIO *U.S. & Asia: Double-Digit Royalties* *ROW: RGNX-Owned*
	Hurler Syndrome (Severe MPS I)*	Direct delivery of IDUA to CNS *NAV® AAV9*	RGX-111			
Retinal Disease	Wet AMD	Anti-VEGF Subretinal delivery *NAV® AAV8*	Sura-vec (ABBV-RGX-314)			abbvie REGENXBIO *U.S. 50/50 Profit Share* *Ex-U.S.: Double-Digit Royalties*
			Sura-vec (ABBV-RGX-314)			
		Anti-VEGF Suprachoroidal delivery *NAV® AAV8*	Sura-vec (ABBV-RGX-314)			
	Diabetic Retinopathy		Sura-vec (ABBV-RGX-314)			
	Geographic Atrophy	C5 inhibitor	Two preclinical ocular programs utilizing **next-generation capsids** for suprachoroidal delivery			–
	Undisclosed	Anti-VEGF				–

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**The FDA placed RGX-111 and RGX-121 on clinical hold in January 2026. The FDA issued a Complete Response Letter for the RGX-121 BLA in February 2026.*

Late-stage investigational gene therapies

RGX-202

Designed for improved outcomes in Duchenne

- Phase I/II interim results: favorable safety profile, functional improvement and high transduction*
- Only gene therapy with CT domain
- Capacity to supply virtually entire available market at planned launch
- Market continues to grow with increased newborn screening

Surabgene lomparvovec

(sura-vec, ABBV-RGX-314)

Potential first gene therapy for chronic retinal disease

- Potential to preserve vision and prevent disease progression in wet AMD and diabetic retinopathy (DR)
- High treatment burden with SOC (life-long, frequent injections) drives undertreatment and vision loss
- Patients showing 4+ years of sustained vision in wet AMD**

RGX-121

(clemidsogene lanparvovec)

Only potential AAV gene therapy for MPS II

- ~500 patients in the U.S., vast majority with severe disease
- No approved treatments that address CNS decline (current SOC is weekly IV ERT)
- Potential to be first gene therapy and one-time treatment

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* Data cut date: January 5, 2026
**Data cut August 29, 2022

Our experienced leadership team is committed to delivering the curative potential of gene therapy in 2026

Curran Simpson

President and Chief Executive Officer

GSK · Human Genome Sciences

Steve Pakola, M.D.

EVP, Chief Medical Officer

aerpio pharmaceuticals · amakem therapeutics

Mitchell Chan

EVP, Chief Financial Officer

CATALIO CAPITAL MANAGEMENT · VIELABIO

Olivier Danos, Ph.D.

EVP, Chief Scientific Officer

Biogen · Kadmon

Shiva G. Fritsch

EVP, Chief Communications and People Officer

hhmi Howard Hughes Medical Institute · Human Genome Sciences

Patrick Christmas, J.D.

EVP, Chief Strategy and Legal Officer

Lumara Health · BioVeris

Ram Palanki, Pharm.D.

EVP, Chief Commercial Officer

Santen · Genentech

Craig Malzahn

EVP, Product Development, Chief Technology Officer

GSK · Human Genome Sciences

Nina Hunter, Ph.D.

SVP, Global Regulatory Strategy and Quality

FDA

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REGENXBIO Manufacturing Innovation Center

Fully In-House in Rockville, MD

REGENXBIO

We built next-generation manufacturing, delivering biologics-level scalability and industry-leading vector purity

REGENXBIO Manufacturing Innovation Center in Rockville, MD

- Full control of product quality, clinical, and commercial supply
- **Capacity to supply market**
  - 2,500 RGX-202 doses/year
  - 350,000 sura-vec doses/year
- NAVXpress® platform accelerates drug development, reduces risk and cost
- FDA PLI inspection successfully completed with no observations

Product Purity

- 80%+ full capsids in Duchenne
- Supports high-dose delivery
- Enables lower total viral load

Productivity

- Efficient purification and high-yield
- Rapid path from candidate to clinical supply (<12 months)
- Robust scalability, with consistent batch-to-batch product profile

Manufacturing excellence driving clinical, regulatory, and commercial readiness

2017 - 2019

- First analytical and development labs operational
- First GMP batches (at CMO)
- NAVXpress® suspension-based process developed

2020

- Broke ground on manufacturing facility

2021

2022

- REGENXBIO Manufacturing Innovation Center (RMIC) grand opening

2023

- Hosted first FDA facility training event
- RGX-121 PPQ series

2024

- Sura-vec PPQ series

2025

- Successful FDA Pre-Licensure Inspection*
- RGX-202 PPQ series

2026 and beyond**

- Build supply intended for potential RGX-202 and sura-vec commercial launches

* No 483 observations
BDS (bulk drug substance); FDP (final drug product); PPQ (process performance qualification)

Our commercial-ready NAVXpress® manufacturing platform accelerates drug development and reduces regulatory risk

Consistent, High-Quality Product

- High-yield suspension process delivers reproducible, high-purity product across programs
- Standardized manufacturing approach ensures consistent product profiles and stronger IND/BLA packages

Faster Development and Scale-Up

- Ready, plug-and-play manufacturing eliminates bespoke process development
- Shared characterization and validation speed tech transfer and scale-up across programs

Reliable, Risk-Reduced Supply

- Standardized materials and training improve compliance and batch reliability
- Unified processes reduce supply-chain, quality, regulatory, and cost risks at clinical and commercial scale

NAVXpress® enables consistent, rapid, and reliable manufacturing across programs—addressing key challenges in advancing gene therapies to market and at scale

U.S.-based, in-house, cGMP facility offers key advantages

	RGX-202	Sura-vec Surabgene lomparvovec	RGX-121* Clemidsogene lanparvovec
Partner	***Wholly-owned***	abbvie	NIPPON SHINYAKU CO., LTD.
Indication/s	**Duchenne Muscular Dystrophy**	**Wet AMD and Diabetic Retinopathy**	**MPS II**
Process	NAVXpress	NAVXpress	NAVXpress
Manufacturing facility	RMIC	RMIC	RMIC
Capacity to supply global market	✔	✔	✔
Status	✔ Process characterization ✔ PPQ series ○ PLI inspection	✔ Process characterization ✔ PPQ series ○ PLI inspection	✔ Process characterization ✔ PPQ series ✔ PLI inspection

*The FDA placed RGX-121 on clinical hold in January 2026. The FDA issued a Complete Response Letter for the RGX-121 BLA in February 2026.

RGX-202:

Potential next and best-in-class opportunity in Duchenne Muscular Dystrophy (DMD)

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RGX-202: Designed to strengthen and preserve muscle long-term

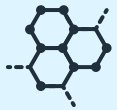
- **Unique market opportunity:** On track to submit BLA in 2026 and potentially launch in 2027 when prevalent market still available
- **Encouraging interim data:** Favorable safety and efficacy profile, consistent and robust microdystrophin levels, encouraging functional and cardiac data with potential for long-term, durable benefit
- **Commercial-ready manufacturing:** Proprietary, high-yielding manufacturing consistently delivering 80%+ product purity levels, enabling higher therapeutic dose and lower total viral load

Duchenne child has not participated in a REGENXBIO clinical trial

Multiple factors may contribute to better outcomes for patients

RGX-202 proactive, comprehensive therapeutic approach

Novel Construct



- NAV® AAV8 vector
- Muscle-specific promoter
- C-Terminal domain

Immune Suppression

- Comprehensive, proactive immune suppression regimen implemented from the outset of the program and designed to improve safety outcomes

Manufacturing

- Leading purity levels in Duchenne gene therapy means fewer empty capsids and lower vector load

Phase I/II/III AFFINITY DUCHENNE® Trial of RGX-202

Phase I/II: RGX-202 has demonstrated positive interim efficacy and safety outcomes

- RGX-202 participants have demonstrated microdystrophin expression levels above 10% (pivotal trial endpoint) at Week 12
- Functional improvements observed at both dose levels
- Pivotal dose showed highly consistent functional improvements at 1 year, exceeding disease trajectory on NSAA and timed function tests
- Favorable safety profile, no SAEs or AESIs observed**
- Pivotal dose caregivers reported improvements in home and community environments, including running, riding a bicycle/tricycle, climbing stairs, walking in the community, and participating in recreational activities and sports with peers*

Pivotal Phase III Portion

- Evaluating RGX-202 in ~30 ambulatory boys aged 1+
- Primary endpoint: proportion of participants with a microdystrophin expression level of ≥10% at Week 12

Pivotal enrollment completed, confirmatory trial enrolling

*Data cut date: May 7, 2025
** Data cut date: January 5, 2026

Phase I/II Interim Safety

RGX-202 was well tolerated with no SAEs or AESIs up to 24 months

RGX-202 TREATMENT Emergent adverse events	DOSE LEVEL 1 1X10^{14} GC/kg	DOSE LEVEL 2 (PIVOTAL DOSE) 2X10^{14} GC/kg		TOTAL N = 13
Age Range (number dosed)	**4–11** **Dose Evaluation** (n = 3)	**1–3** **Younger Boys** (n = 3)	**4–11** **Dose Evaluation/ Expansion** (n = 7)	**All Age Ranges**
SAE	0	0	0	0
AESI				
Central Or Peripheral Neurotoxicity	0	0	0	0
Drug-Induced Liver Injury	0	0	0	0
Thrombocytopenia	0	0	0	0
Myocarditis	0	0	0	0
Myositis	0	0	0	0

The most common drug-related AEs reported were: vomiting (n=7), fatigue (n=6), and nausea (n=4)

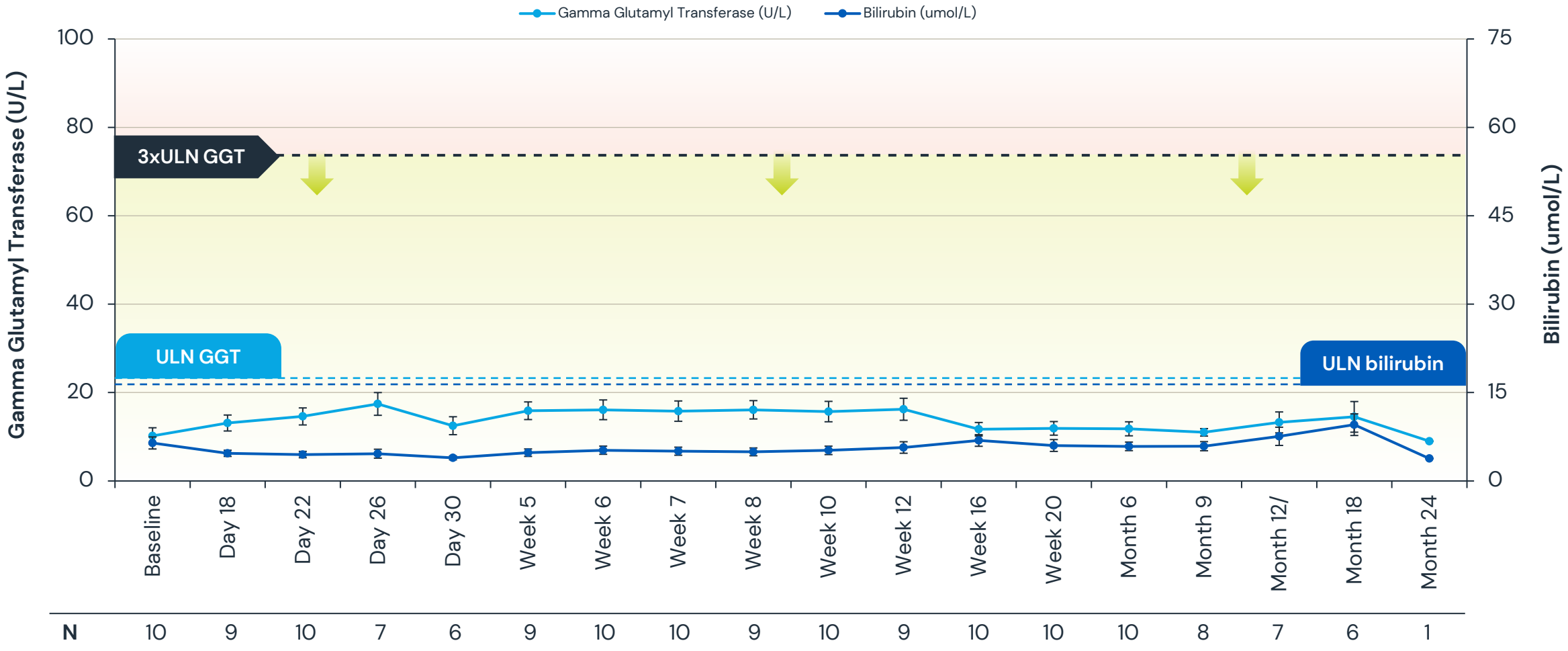
Data cut date: January 5, 2026

AESI criteria prospectively defined per protocol.
Drug-Induced Liver Injury: GGT > 3 × ULN, or GLDH > 2.5 × ULN, or ALT > 3 × baseline

SAE: Serious adverse event; AESI: Adverse event of special interest; GC: Genome copies

Pivotal Dose: Liver Safety Findings Up to 24 Months

Mean GGT and total bilirubin not exceeding ULN



Data cut date: January 5, 2026

Each point represents the mean +/- SEM of all subjects with an assessment windowed into that particular visit.
Due to constraints, not all counts are displayed beneath the figure.
Bilirubin ULN is 17.1 umol/L; GGT ULN is 24 U/L

GGT: Gamma glutamyl transferase; ULN: Upper limit of normal

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Phase I/II Biomarkers Supported Consistent Robust Expression, Transduction, and Sarcolemmal Localization of RGX-202 Microdystrophin

WEEK 12 BIOPSY		RGX-202 Microdystrophin[1] % (Western Blot)	VCN copies/nucleus (qPCR)	Positive Fibers[2] % (Immunofluorescence)
DOSE LEVEL 1 $1X10^{14}$ GC/kg	4–7 (2)	60.6 (37.8, 83.4)	9.8 (7.4, 12.1)	79.3[3]
	8–11 (1)	10.4	5.4	34.6
DOSE LEVEL 2 (PIVOTAL DOSE) $2X10^{14}$ GC/kg	1–3 (3)	97.3 (51.2, 122.3)	24.8 (20.4, 29.1)	76.7 (50.8, 97.1)
	4–7 (2)	54.3 (31.5, 77.2)	30.1 (4.9, 55.4)	50.3 (29.4, 71.1)
	8–11 (5)	39.7 (20.8, 75.7)	17.8 (12.0, 30.7)	45.7 (21.3, 70.6)

Data cut date: January 5, 2026

[1] Microdystrophin expression adjusted for muscle content; % normal control
[2] Positive Fibers defined as change from baseline of RGX-202 microdystrophin & dystrophin positive fibers
[3] One sample could not be evaluated

VCN: Vector copy number; qPCR: Quantitative polymerase chain reaction; GC: Genome copies

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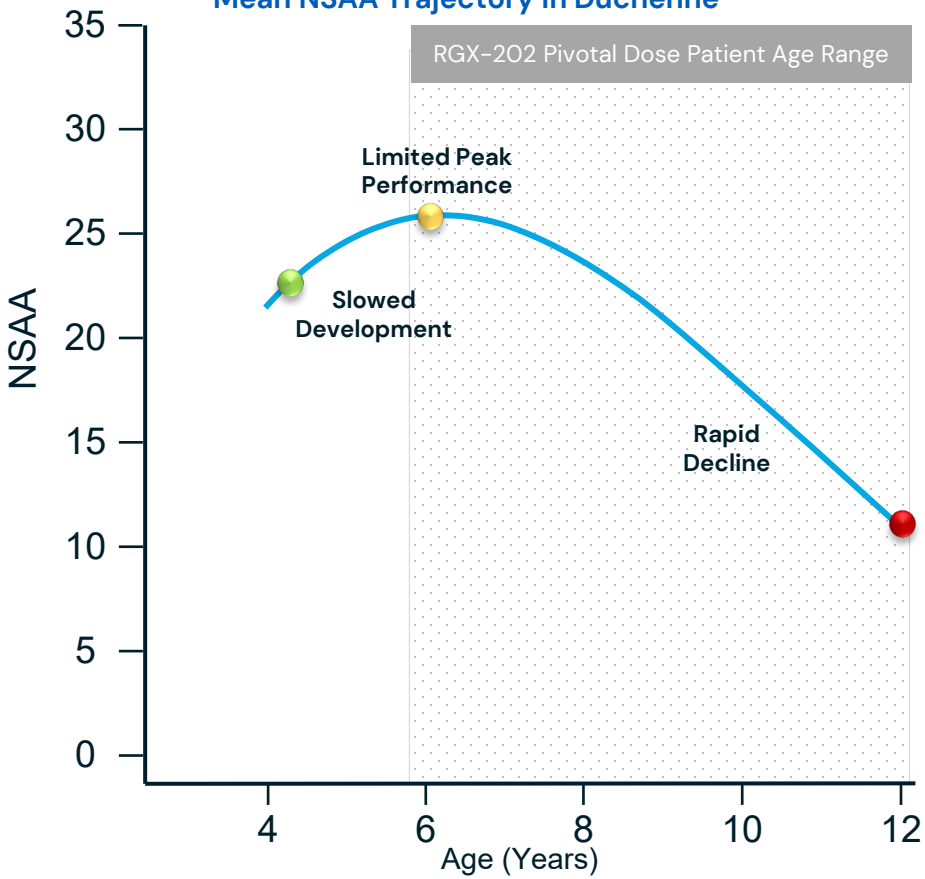
AFFINITY DUCHENNE® External Control Methodology

Mean NSAA Trajectory in Duchenne[1]



STEP 1

Filter EC Participants by Key Entry Criteria

- Stable dose of corticosteroid for 12 weeks
- Aged ≥ 4 and ≤ 1 + the maximum age of treated group (13)
- TTSTAND >3 and < 9 Seconds
- TTRW within +/- 1 sec of the treated group (3.2-7.0 seconds)

External Data Sources

- FOR-DMD
- BioMarin PRO-DMD-01 (CureDuchenne)
- CINRG DNHS
- cPATH / D-RSC

STEP 2

Further Balance Baseline Covariates of RGX and EC Group at Individual Patient Level

SAP Primary Method: *Propensity-Score Weighting

- Age
- NSAA
- TTSTAND
- TTRW

cTAP (Collaborative Trajectory Analysis Project)

- A cross-validated, longitudinal prognostic model that uses baseline age and motor function measures to predict up to 5-year NSAA trajectories in ambulatory steroid-treated boys with DMD.

MULTIPLE, VALIDATED METHODS TO DETERMINE EXPECTED TRAJECTORY

Graph adapted from Muntoni 2019; *Propensity-score weighting method mimics a randomization setting for RGX-202-1101 study by taking an EC group with similar entry criteria and balancing baseline age and function. It assigned higher weights to patients in the EC group with greater similarity to RGX-202 treated patients. FOR-DMD, Finding the Optimum Regimen for Duchenne Muscular Dystrophy; NSAA, North Star Ambulatory Assessment; TTSTAND, Time to stand; TTRW, time to run/walk 10 meters.

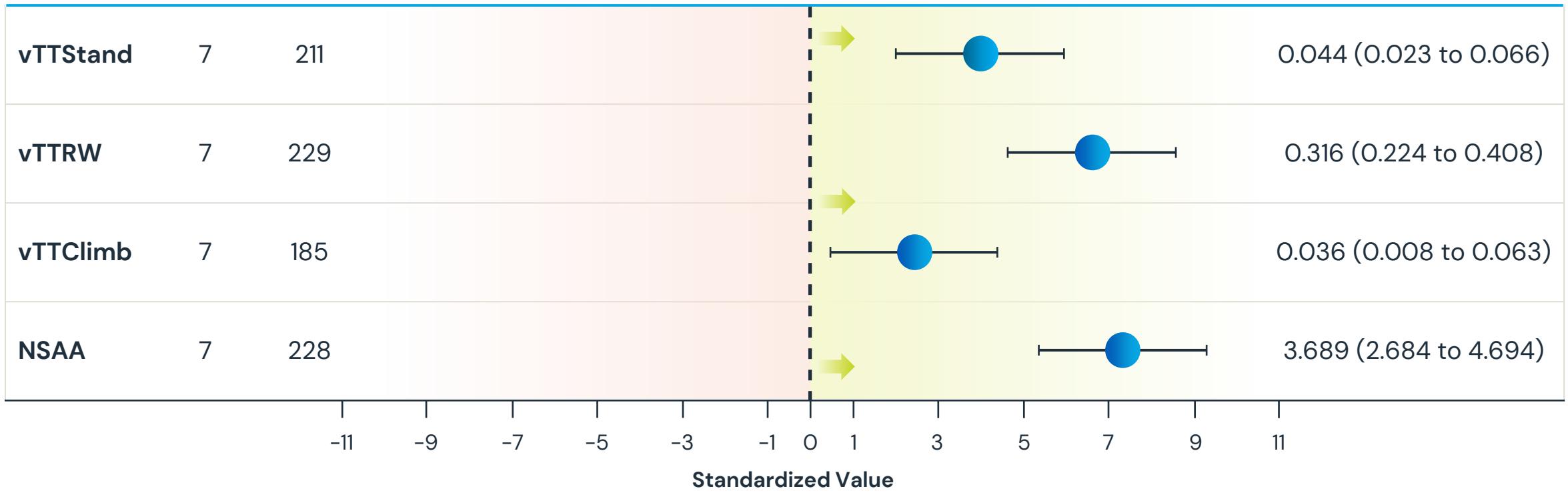
The D-RSC Data Platform initiative is a public/private partnership funded by the Parent Project Muscular Dystrophy (PPMD) and launched in August of 2015 by Critical Path Institute (cPath)

Muntoni F, Signorovitch J, Goemans N, Manzur AY, Done N, Sajeev G, Li J, Akbarnejad H, Sharma A, Ward SJ, Niks EH, Servais L, Mercuri E, Guglieri M, Straub V, de Groot I, Ridout D; PRO-DMD-01 study investigators; Association Française contre les Myopathies; NorthStar Clinical Network; McDonald C. Predicting trajectories of the north star ambulatory assessment total score in Duchenne muscular dystrophy. PLoS One. 2025 Jun 27;20(6):e0325736. doi: 10.1371/journal.pone.0325736. PMID: 40577272; PMCID: PMC12204569.

Pivotal Dose: Functional Improvements at 1 Year Compared with External Controls

Exceeded external controls using propensity score weighting

Endpoint	RGX, n	EC, n	LSMean Difference (95% CI)
vTTStand	7	211	0.044 (0.023 to 0.066)
vTTRW	7	229	0.316 (0.224 to 0.408)
vTTClimb	7	185	0.036 (0.008 to 0.063)
NSAA	7	228	3.689 (2.684 to 4.694)



Data cut date: January 5, 2026

V: velocity; TTStand: Time to Stand; TTRW: Time to Run and Walk; TTClimb: Time to Climb; NSAA: North Star Ambulatory Assessment; EC: External controls

Least Square Mean (LSMean) differences were estimated using a mixed model for repeated measures (MMRM), comparing the change from baseline for RGX versus external controls (EC), adjusting for age at dosing and baseline functional test score. To ensure that a favorable RGX effect appears to the right side of zero in the forest plot, data transformations were applied. Specifically, the values of timed functional tests were multiplied by -1. The plot also standardized the values of different parameters with different units by graphing the standardized effect size (LSM and 95% CI divided by standard error).

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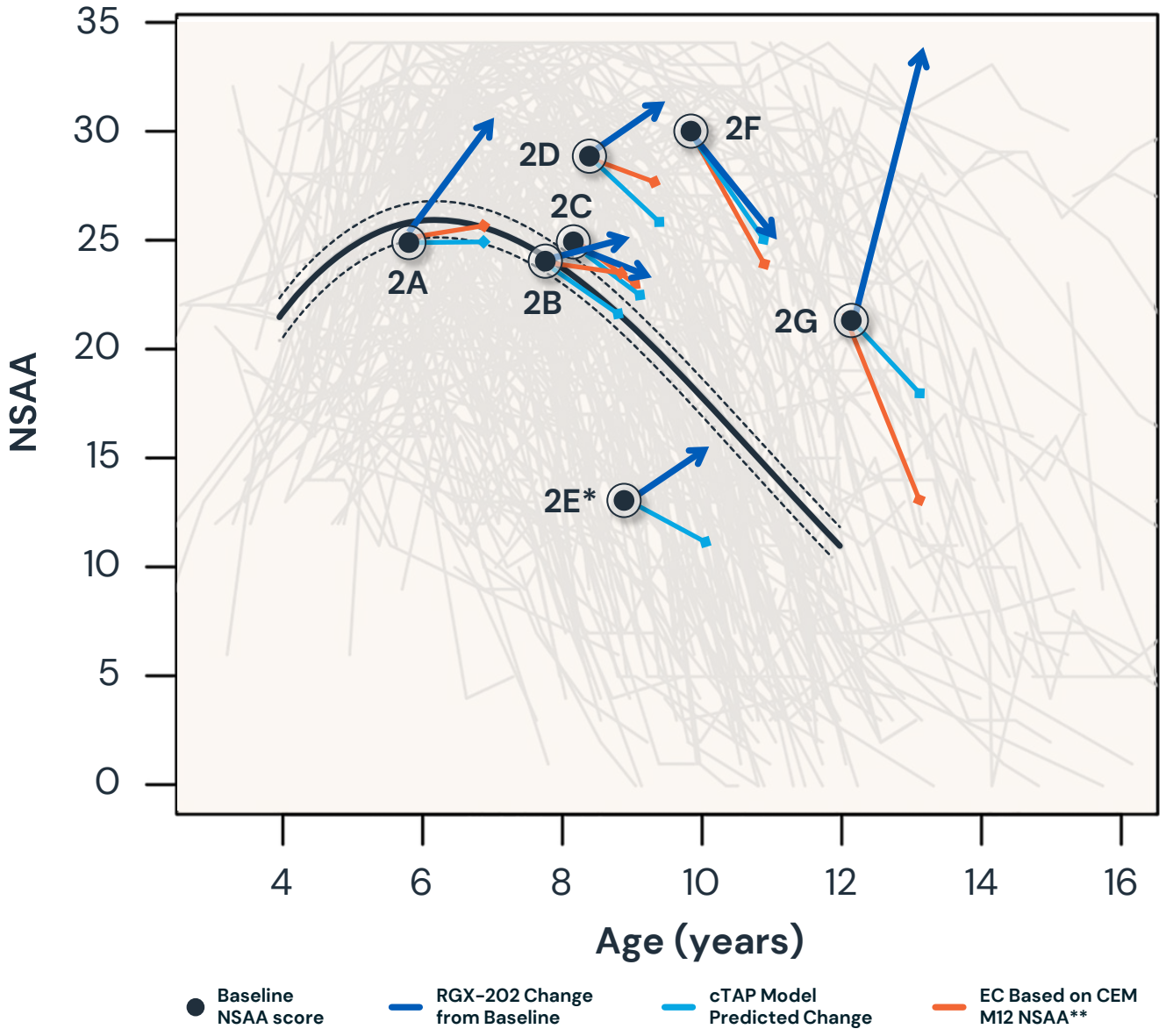
Pivotal Dose: NSAA Outcomes at 1 Year

NSAA performance exceeded external controls and expected disease trajectory



Data cut date: January 5, 2026

Fig derived from Muntoni et al. NSAA total score trajectories for individual patients by age (in grey) and the fitted mean and 95% confidence interval (in black). Each grey line represents NSAA total scores from an individual patient plotted versus age; the population mean and its 95% confidence bands are shown in black.

NSAA, North Star Ambulatory Assessment; CEM: Coarsened exact matching; M12: Month 12

Muntoni F et al. (2019) Categorising trajectories and individual item changes of the North Star Ambulatory Assessment in patients with Duchenne muscular dystrophy. PLoS ONE 14(9): e0221097. https://doi.org/10.1371/journal.pone.0221097

*2E Month 12 collected approximately Month 10.8

**Coarsened exact matching based on: Age +/- 1 year, NSAA +/-2, TTSTAND +/- -.5 seconds; TTRW +/- 1 sec.

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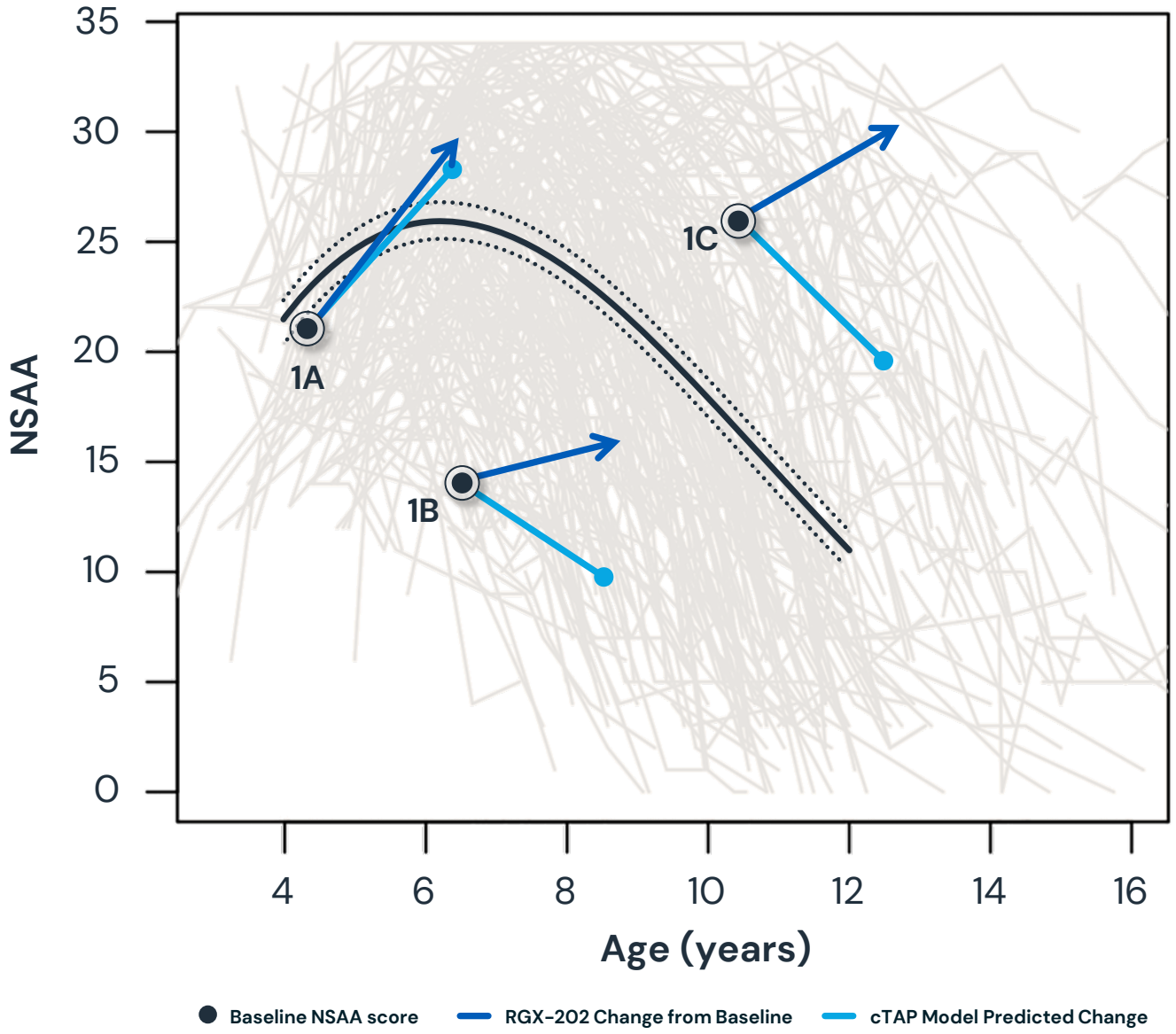
Dose level 1: NSAA Outcomes at 2 Years

NSAA performance exceeded expected disease trajectory



Data cut date: January 5, 2026

Fig 1. NSAA total score trajectories for individual patients by age (in grey) and the fitted mean and 95% confidence interval (in black).
Each grey line represents NSAA total scores from an individual patient plotted versus age; the population mean and its 95% confidence bands are shown in black.

NSAA, North Star Ambulatory Assessment.

Muntoni F et al. (2019) Categorising trajectories and individual item changes of the North Star Ambulatory Assessment in patients with Duchenne muscular dystrophy. PLoS ONE 14(9): e0221097. https://doi.org/10.1371/journal.pone.0221097.g001

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Pivotal Dose: Cardiac Function Remained Stable Through 1 Year

	BASELINE	12 MONTHS
Subjects (N)	**7**	**7**
Age Mean (range)	**8.7*** (5.8–12.1)	**9.7** (6.8–13.1)
Left Ventricular Ejection Fraction Mean (range) Median	**61.7 %** (54–72**) 60%	**61.6%** (57–74) 60%
Global Circumferential Strain*** Mean (range) Median	**-20.4 %** (-22% to -19%) -20.4 %	**-20.9 %** (-23% to -17%) -21.5%
Late Gadolinium Enhancement (LGE)	***1 participant with fibrosis***	***No change from baseline***

Data cut date: January 5, 2026

*Age at dosing
**One participant met LVEF criteria at baseline by ECHO of >55%; later cMRI had measure of 54
*** More negative strain values are better.

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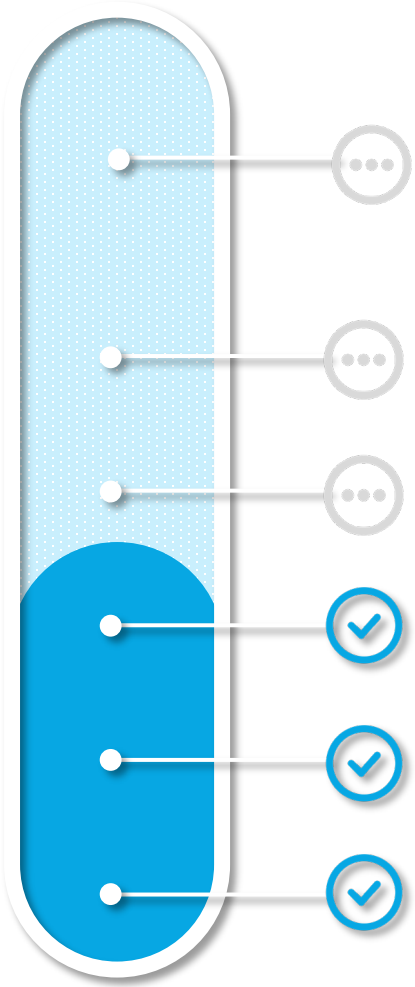
RGX-202 planned milestones and commercial readiness



1H '26: FDA & EMA interactions
- Request pre-BLA meeting mid-'26
  - *Expect confirmatory trial to be near final at time of submission*

Early Q2 '26: Topline pivotal data

March '26: Additional Ph I/II data update at MDA

November '25: Initiated confirmatory trial, enrolling ~30 additional patients

October '25: Completed pivotal enrollment, 30 patients dosed

September '25: Manufactured batches intended for commercial supply

Preparing to meet demand for improved options

- Planning for broad ambulatory access
- Only sponsor with full control of drug supply
- Building commercial infrastructure
- Activating additional confirmatory trial sites
- Engaging potential commercial treatment centers
- Expanding AFFINITY DUCHENNE® trial globally

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Surabgene lomparvovec (sura-vec, ABBV-RGX-314):

Potential to be the first gene therapy for chronic retinal diseases

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Sura-vec: Potential first gene therapy for chronic retinal diseases

- **High treatment burden:** Chronic, VEGF-driven diseases see high treatment burden (frequent intraocular or intravitreal injections) that leads to undertreatment and ultimately vision loss over time
  - Today's predominant approach halts degeneration temporarily but does not address underlying cause; compliance with multiple injections poses major limitation
- **Potential SOC:** Sura-vec has potential to prevent disease progression and preserve vision long term, as well as reduce treatment burden for wet AMD and diabetic retinopathy (DR)
- **Partnership with AbbVie:** Strategic collaboration reinforces commercial strength and validates global potential

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Patient has not participated in clinical trials of ABBV-RGX-314

Sura-vec is a one-time investigational gene therapy designed to deliver sustained treatment effect in wet AMD and DR

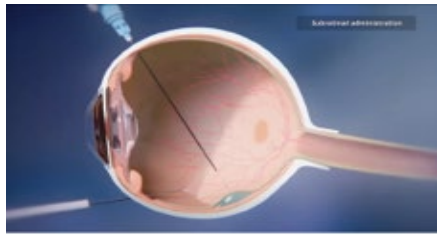
Uses the NAV® AAV8 vector to encode an antibody fragment designed to inhibit vascular endothelial growth factor (VEGF) and fluid accumulation in the retina

NAV® VECTOR
AAV8

GENE
Anti-VEGF Fab

MECHANISM OF ACTION
Reducing leaky blood vessel formation by giving retinal cells the ability to produce an anti-VEGF Fab

SUBRETINAL



- SR space most immune-privileged space for ocular gene therapy
- No prophylactic steroids in SR

SUPRACHOROIDAL

- SCS allows for in-office delivery of ocular gene therapy with minimized inflammation risk
- Minimal, 7-week prophylactic steroids in SCS

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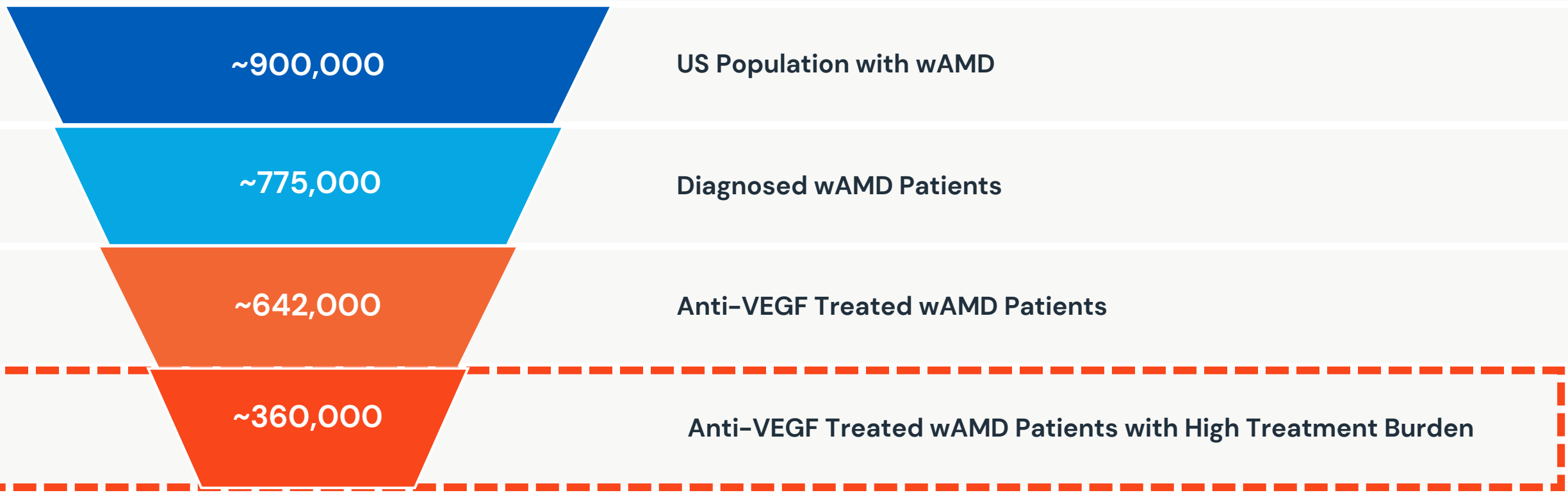
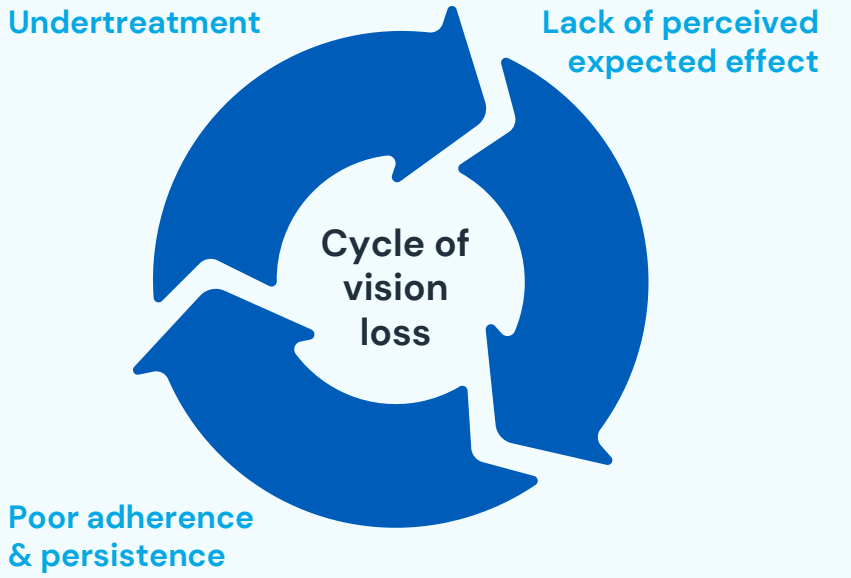
Majority of anti-VEGF-treated patients face high burden + poor compliance, leading to vision loss over time



US Census Bureau, 2024 Clarivate DRG AMD Report; 2024 Vestrum EMR w AMD Analysis;
High treatment burden defined aa ≤ 8 w k injection intervals

Sura-vec designed to disrupt cycle of undertreatment

Burdensome dosing regimens are main driver of current anti-VEGF treatment discontinuation



Subretinal sura-vec anticipated to integrate into existing retina practice infrastructure: Retina specialists routinely perform vitrectomy procedures as part of wet AMD management, supporting the feasibility of a subretinal surgical approach.

2.5K
US Retina Specialists

90%
of Retina Specialists are Surgically Trained

4K
Retina Surgical Sites

400K
Vitrectomy Surgeries

Ibis World 2021; 2022 ASRS PAT Survey; 2022 IQVIA LAAD data; Market Scope 2023 Retinal Device Report

Subretinal sura-vec is in late-stage clinical trials and is on track to be the first gene therapy for a large indication (wet AMD)

ATMOSPHERE

Phase III evaluating subretinal sura-vec in ~540 wet AMD patients at 2 dose levels* vs. ranibizumab (LUCENTIS®)

ASCENT

Phase III evaluating subretinal sura-vec in ~660 wet AMD patients at 2 dose levels* vs. aflibercept (EYLEA®)

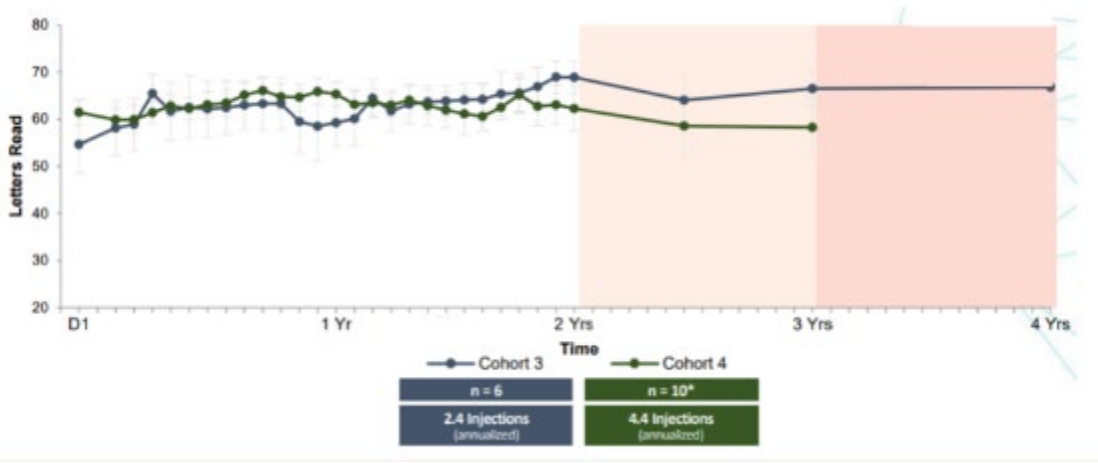
Two pivotal trials evaluating subretinal delivery for wet AMD, with Phase I/IIa data demonstrating long-term safety and tolerability, stable to improved vision and retinal thickness

- Long-term follow-up study showed durable treatment effect up to 4 years at doses similar to pivotal trials
- Data from robust clinical strategy (Phase I/IIa long-term follow up, pharmacodynamic, and fellow eye studies) support potential pivotal outcomes and commercial opportunity
- Top-line data expected Q4 2026

Data cut August 29, 2022

Data seen to date in Phase I/II subretinal studies of sura-vec supportive of potential pivotal outcomes for wet AMD

Phase I/IIa LTFU (BCVA)



Overall Safety

- Sura-vec has been well tolerated across Phase I/II (up to 4 years)* and Phase II Bioreactor Bridging^ studies (at 1 year) at doses similar to pivotal study
  - No drug-related SAEs
  - Common AEs[1] including post-op conjunctival hemorrhage and post-op inflammation[2] resolving within days to weeks, peripheral retinal pigmentary changes as measured by central reading center

Efficacy Endpoints

- With one-time treatment of sura-vec at dose levels similar to the pivotal trial, patients demonstrate a long-term, durable treatment effect up to 4 years
  - Stable to improved visual acuity
  - Meaningful reductions in anti-VEGF injection burden

Fellow Eye Sub-study

- Positive data from Phase II Fellow Eye presented June 2025
  - 93% reduction in treatment burden at 12 months
  - No drug-related SAEs+

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*Data cut August 29, 2022; ^ Data cut October 21, 2024; +Data cut: May 26, 2025
1. Common ocular AEs across cohorts in the study eye defined by ≥ 15% of patients for Phase I/II and ≥ 10% of patients for the Phase II Bioreactor Bridging Study; 2. Post-operative inflammation is defined as inflammation AEs which occurred within 30 days of subretinal procedure.

Positive safety and efficacy in Phase II ALTITUDE® trial support initiation of pivotal Phase IIb/III NAAVIGATE study

Phase IIb NAAVIGATE

- Multicenter, randomized, masked, sham-controlled study
- ~136 subjects with NPDR without center-involved diabetic macular edema (CI-DME)
- Primary endpoint: ≥2-step DRSS improvement
- All subjects will receive sura-vec at $1.0x10^{12}$ (GC)/eye (ALTITUDE Dose Level 3) and short-course topical prophylactic steroids.

ALTITUDE evaluating suprachoroidal delivery of sura-vec in ~100 DR patients across 3 dose levels and 30 DME participants at DL4*

Phase II ALTITUDE interim results in non-proliferative DR show suprachoroidal sura-vec was well-tolerated across dose levels 1 – 3

- No IOI in NPDR subjects at dose level 3 with short-course prophylactic topical steroids
- One-time in-office injection at dose level 3 demonstrated durable efficacy profile with 50% of participants achieving > 2-step improvement without additional DR treatment
- Dose Level 3 prevented disease progression in NPDR participants and reduced vision-threatening events by >70% over 2 years compared to historical controls

NAAVIGATE first patient dosed expected in Q2 2026

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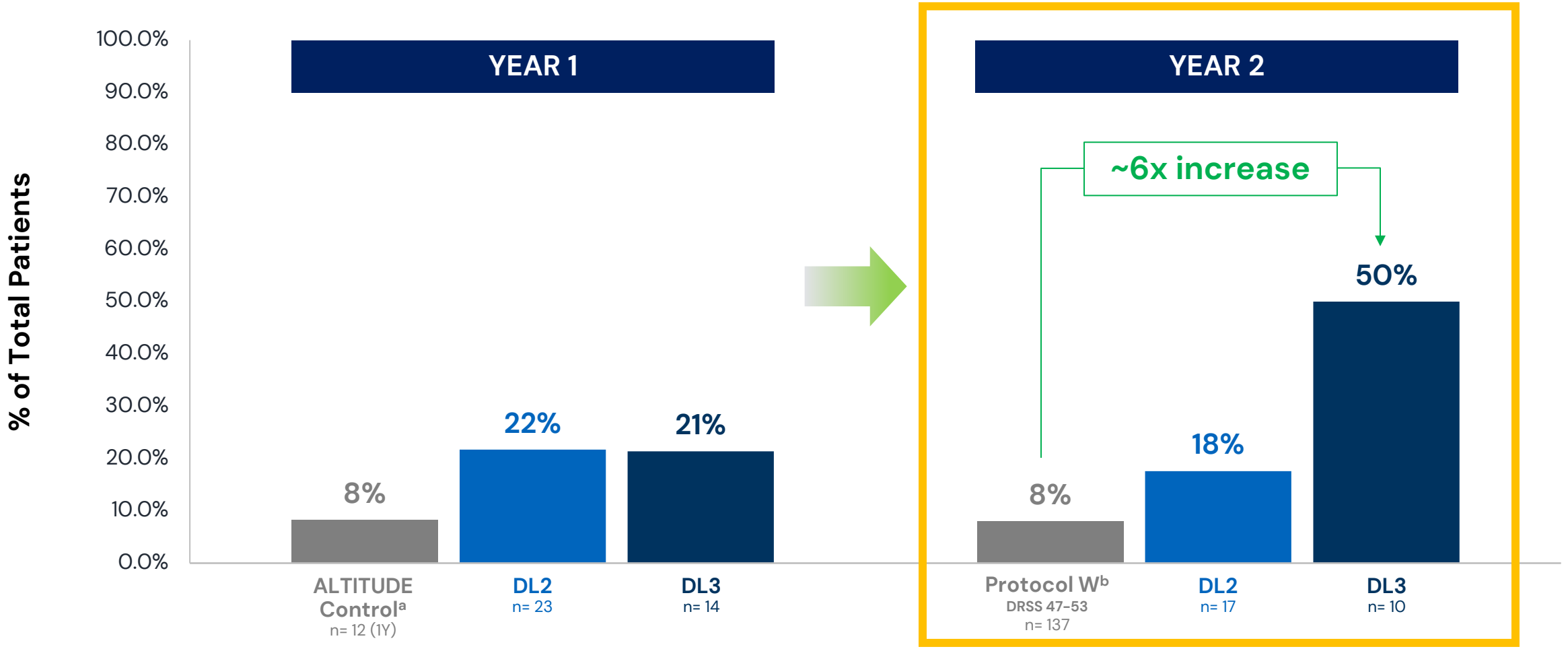
*Dose Level 1 at 2.5x1011 GC/eye; Dose Level 2 at 5x1011 GC/eye; Dose Level 3 at 1x1012 GC/eye; Dose Level 4 at 1.5x1012 GC/eye
Data cut: June 9, 2025.

≥2-Step DRSS improvement without additional DR treatment at 2 years; DL3 sura-vec treated subjects outperformed all other groups and controls



Data cut: June 09, 2025.
a. Control subjects crossed over to receive sura-vec at Year 1.
b. Maturi RK, et al. *JAMA Ophthalmology*. 2021;139(7):701-712. Protocol W results based on subgroup analysis of subjects with Baseline DRSS 47 and 53.
One subject in Dose Level 2 missed their 1-Year visit. One subject in Dose Level 3 was found to have confounding disease at baseline and their data was excluded.
DL: Dose Level; DRSS: Diabetic Retinopathy Severity Scale

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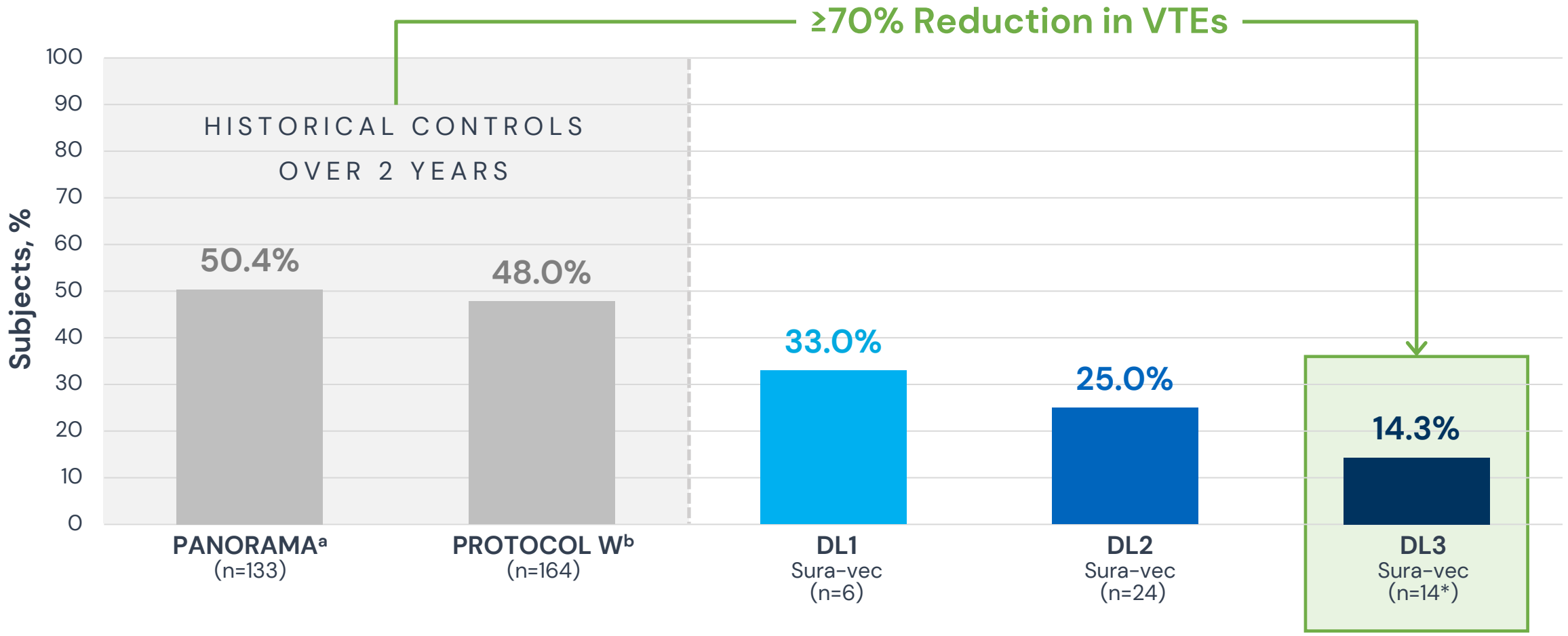
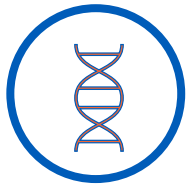
≥ 70% risk reduction in vision threatening events over 2 years observed in DL3 subjects treated

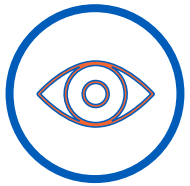


Data cut: June 09, 2025.
Data shown is using LOCF. VTEs = VTCs + CI-DME; VTCs could include PDR or ASNV. Historical controls include VTC+CI-DME.
*One subject in Dose Level 3 was found to have confounding disease at baseline and their data was excluded.
a. Brown DM, et al. *JAMA Ophthalmology*. 2021;139(9):946-955. b. Maturi RK, et al. *JAMA Ophthalmology*. 2021;139(7):701-712. Protocol W results are based on the 2-year cumulative probability for development of PDR and CI-DME applied to the sub-population with Baseline DRSS 47 and 53.

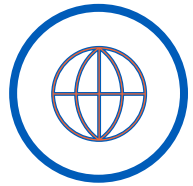
Global eye-care alliance with AbbVie validates retina franchise and enables world-class commercialization capabilities



REGENXBIO brings leadership and expertise in AAV and retinal gene therapy, with strong in-house capabilities of AAV manufacturing



Sura-vec global pivotal trials on track to deliver highly differentiated treatment option with global commercial launch teams in place



AbbVie brings 75+ years of commitment in eye care market, with commercial footprint of 10+ marketed eye care products in 175+ countries across five world regions

Details of Strategic Partnership

- **$370 million upfront payment** with up to **$1.38 billion in additional development, regulatory and commercial milestones**
- AbbVie supports majority of development with **equal share of profits in U.S., and REGENXBIO to receive royalties outside U.S.**
- **REGENXBIO will lead the manufacturing of sura-vec** for clinical development and U.S. commercial supply

RGX-121 (clemidsogene lanparvovec):

Potential first gene therapy for Hunter Syndrome (MPS II)

REGENXBIO

RGX-121: Potential to move MPS II treatment paradigm beyond ERT

- **No cure:** Ultra-rare, rapidly progressive, life-threatening genetic disease; most do not live past the age of 20
- **High treatment burden:** Current SOC is weekly IV enzyme replacement therapy (ERT)
- **Urgent need:** RGX-121 has the potential to be first one-time treatment for MPS II; REGENXBIO is continuing to engage with FDA with the goal of resubmitting the BLA
- **Commercial-ready:** Strategic partnership with Nippon Shinyaku
- **Strategic value:** If RGX-121 is approved, REGENXBIO expects to receive a Priority Review Voucher

MPS II child has not participated in clinical trials of RGX-121.

The FDA placed RGX-121 on clinical hold in January 2026. The FDA issued a Complete Response Letter for the RGX-121 BLA in February 2026.

RGX-121 designed to address genetic cause of MPS II

What is RGX-121?

One-time gene therapy using NAV® AAV9 vector to deliver a working copy of the gene missing or malfunctioning in boys with MPS II; administered directly to the central nervous system

CAMPSIITE™ STUDY

Pivotal phase evaluating RGX-121 for safety, key biomarker activity, and neurodevelopment in 13 boys aged 4 months up to 5 years with neuronopathic MPS II

RGX-121 has shown strong signs of efficacy and favorable safety profile across all phases of Phase I/II/III trial

- Phase III pivotal trial met primary endpoint
- Consistently demonstrated significant, sustained reductions in surrogate endpoint through one year at the pivotal dose
- Strong correlation between measured CSF HS D2S6 levels at Week 16 and neurodevelopmental outcomes at one year
- Neurodevelopmental and daily activity skill acquisition observed up to 4 years after administration of RGX-121
- Well tolerated, and program includes careful monitoring to manage risks

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Data cut date: August 20, 2024
Pivotal dose: 2.9 x 1011 GC/g of brain mass
CAMPSIITE Part 2, Pivotal: One SAE possibly related to RGX-121, elevated liver enzymes, resolved with steroid treatment.
CAMPSIITE Part 1, Dose Finding Study (as of January 5,2024): No SAE considered related to RGX-121
Developmental skill acquisition data as of June 20, 2023

>80% reduction in CSF HS D2S6, key biomarker likely to predict clinical benefit in MPS II brain disease, sustained through 1 year

Pivotal: Primary Endpoint Achieved with Sustained Reduction in CSF D2S6 through 1 Year			
	Week 16	**Week 24**	**1 Year**
Proportion of participants with CSF HS D2S6 at or below maximum attenuated level	**9/13 Primary Endpoint (p < 0.0001)***	**10/13**	**8/11****
% Median reduction of CSF HS D2S6	**-81 %**	**-82 %**	**-82 %**

Pivotal: Neurodevelopmental Skill Acquisition or Stability on all BSID-III Subscales in Participants at 1 Year

	Above/Equal to -2SD AEq (SE) N = 5			Below -2SD AEq (SE) N = 8		
BSID-III* Subscale**	Baseline	Year 1	**Change from Baseline**	Baseline	Year 1	**Change from Baseline**
Cognitive	15.7 (6.0)	24.2 (4.4)	**+ 8.5** (3.3)	13.9 (3.1)	16.6 (2.9)	**+ 2.7** (1.5)
Fine motor	16.1 (6.6)	22.6 (4.3)	**+ 6.5** (3.5)	14.2 (2.4)	16.5 (2.9)	**+ 2.3** (2.5)
Gross Motor	13.6 (5.3)	18.8 (2.8)	**+ 5.2** (2.8)	12.3 (1.9)	15.5 (1.2)	**+ 3.2** (1.5)
Receptive Language	14.7 (5.4)	19.8 (3.7)	**+ 5.1** (1.9)	9.7 (3.3)	11.2 (2.7)	**+ 1.5** (1.9)
Expressive Language	14.3 (5.2)	19.0 (3.6)	**+ 4.7** (2.1)	12.3 (3.6)	12.5 (2.6)	**+ 0.2** (1.6)

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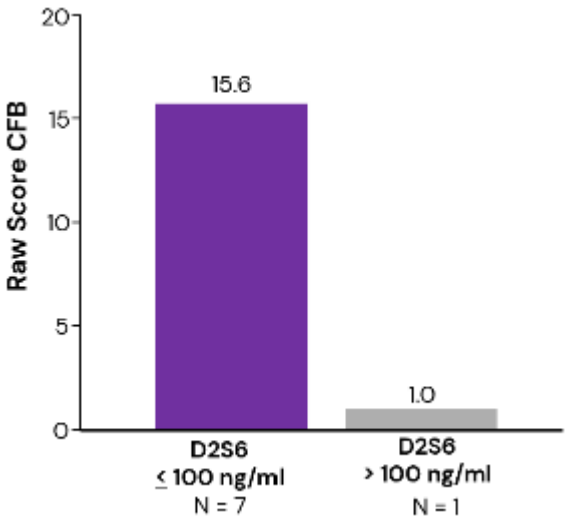
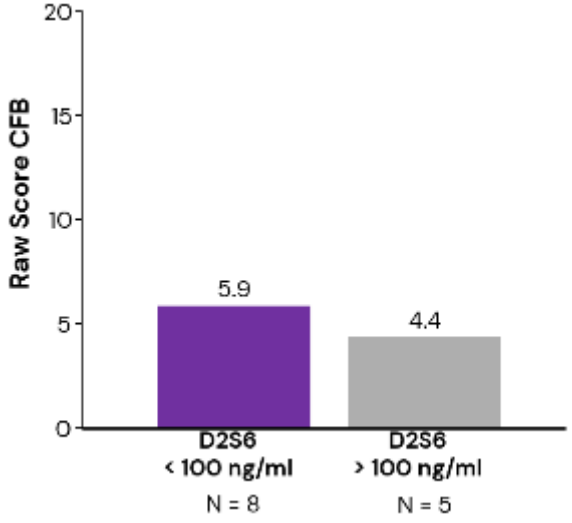
Data cut date: August 20, 2024
MPS II attenuated reference point < 100 ng/mL based on analysis presented in Boulos N, World Symposium, 2022
* Primary endpoint was compared to the margin of 20%
** Samples could not be obtained from 2 participants at 1 year
*** BSID-III, Bayley Scale of Infant and Toddler Development, 3rd Edition

RGX-121 data demonstrate correlation between measured CSF HS D2S6 level at Week 16 and cognitive outcomes at 1 year

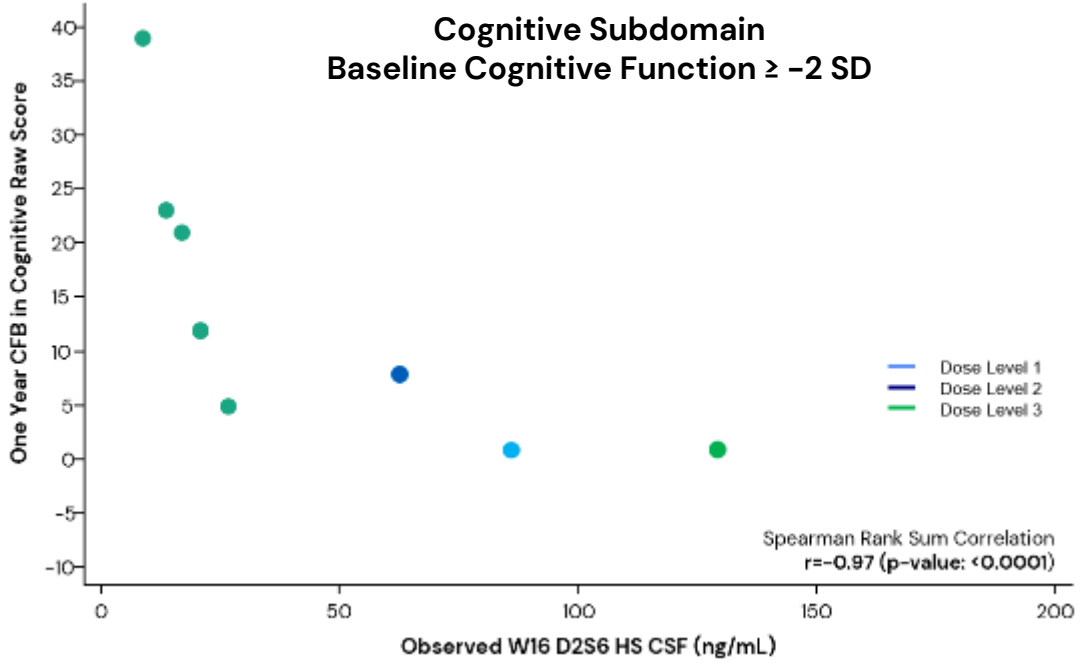
Baseline BSID-III Cognitive Function ≥ -2SD



Baseline BSID-III Cognitive Function < -2SD



Cognitive Subdomain Baseline Cognitive Function ≥ -2 SD



Dose-finding & Interim Pivotal
CFB, Change from Baseline
Analysis includes participants from dose-finding (all doses) and pivotal
2 Dose Level 1 participants did not have a week 16 value
Max. attenuated D2S6 level: ≤ 100ng/ml

Dose-finding & Interim Pivotal
CFB, Change from Baseline
Participants from dose-finding and pivotal with baseline BSID cognitive subscale score ≥ -2SD at baseline
n = 8; 2 Dose Level 1 participants did not have a week 16 value

Data cut date August 20, 2024

Strategic partnership with Nippon Shinyaku bolsters commercialization capabilities

NIPPON SHINYAKU CO., LTD. REGENXBIO

Nippon Shinyaku leads commercialization of RGX-121 and RGX-111 in U.S. and Asia

REGENXBIO leads manufacturing

NS Pharma prepared to commercialize RGX-121 upon potential approval in US, focused on qualified treatment centers

Maximizes collective strengths to accelerate access for MPS patients, brings value to shareholders

- **REGENXBIO received $110 million** at closing and rights to developmental milestones and royalties on net sales
- **REGENXBIO retains rights to RGX-121 Priority Review Voucher**
- **REGENXBIO reserves the right to develop and commercialize these products in countries outside of the Licensed Territory**

REGENXBIO *Rights to select development milestones and royalties were included in the May 2025 strategic financing with HCRx

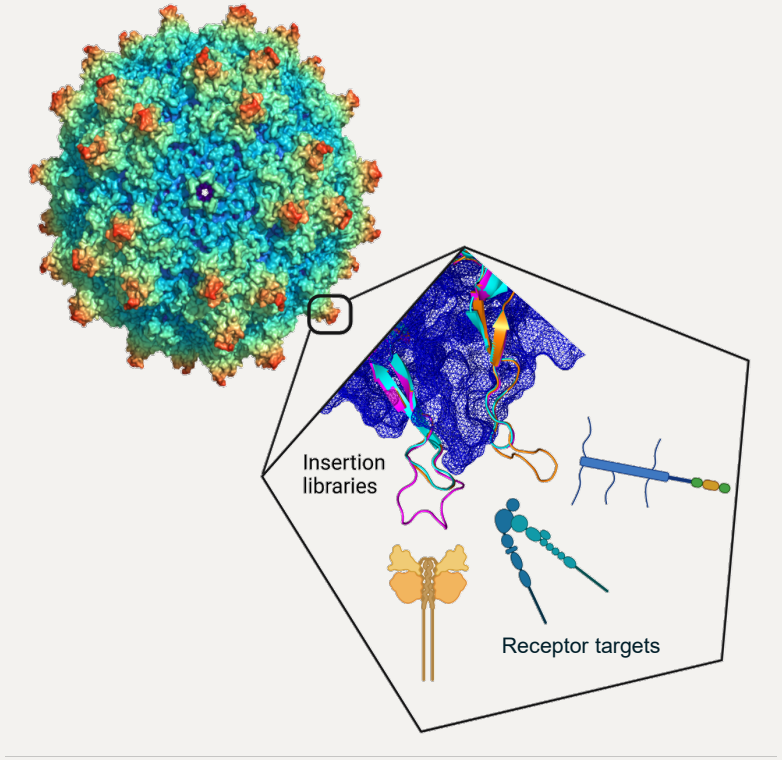
Discovering the next wave of gene therapies

Preclinical pipeline driven by new, efficient capsids

REGENXBIO

Expanding the therapeutic potential of AAV gene delivery

AI-powered engineering platform generates new capsids that can improve efficacy at lower doses



Developing new capsids that can:

- Improve tissue tropism and cell specificity
- De-target the liver
- Increase transduction efficiency

Approaching IND readiness for capsid that has demonstrated higher transgene expression via suprachoroidal delivery in the eye

Enabling novel gene therapy modalities using in vitro and in vivo models for improved clinical translatability

Applying machine learning for high-throughput screening of AAV libraries with ligand-specific binders and peptide insertions

Seeking to improve lives through the curative potential of gene therapy

REGENXBIO