Delivering the promise of gene therapy

REGENXBIO Corporate Presentation

May 2026

REGENXBIO®

Forward-looking statements

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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

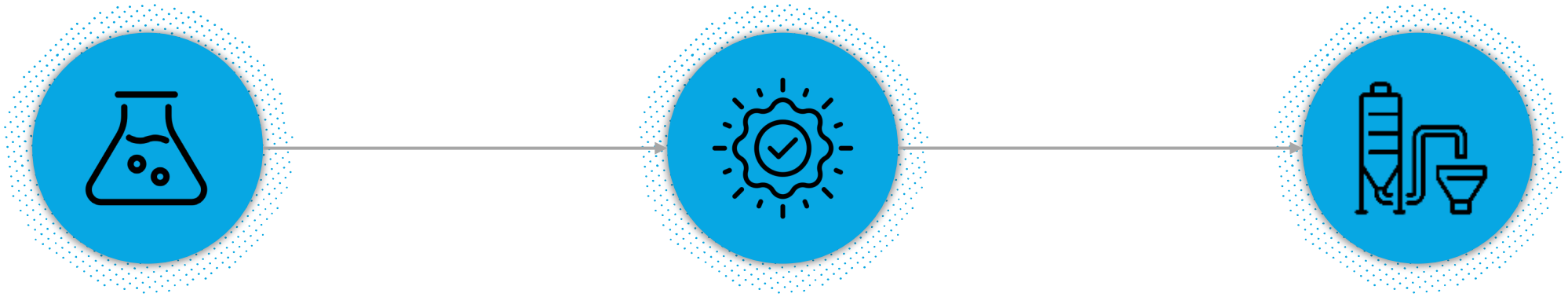
FDA-inspected cGMP® facility capable of production at commercial scale

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. 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/III interim results: met pivotal primary endpoint, favorable safety profile, functional improvement*
- Only gene therapy with CT domain
- Capacity to supply virtually entire available market at planned launch in 2027
- 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: April 16, 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

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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

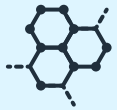
- **Unique market opportunity:** On track to initiate rolling BLA submission in Q3 2026 and potentially launch in 2H 2027 when prevalent market still available
- **Encouraging interim data:** Favorable safety and efficacy profile, consistent and robust microdystrophin levels, encouraging functional and 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 (>80% full capsids) in Duchenne gene therapy enables maximum therapeutic dose ($2x10^{14}$ GC/kg) with lower capsid load
- FDA-inspected, commercial-ready facility

Pivotal Phase III Portion

AFFINITY DUCHENNE®

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

Topline Pivotal Data Support Potential Best-in-Class Profile

AFFINITY DUCHENNE® Pivotal Study of RGX-202 in Ambulatory Patients Aged 1+: Results Summary

Primary endpoint[1] met with high statistical significance (p<0.0001)

- **28 of 30[2] participants (93%)** achieved microdystrophin expression above 10%[1]
- **80%** of participants achieved **>40% microdystrophin expression**
- Robust microdystrophin expression **averaged 71.1% across all participants**, and **41.6% in older boys** (aged 8+)

Participants exceeded expected disease trajectory at 1 year

- **Demonstrated statistically significant correlation between RGX-202 microdystrophin expression and functional improvement (NSAA),** a landmark distinction in Duchenne gene therapy
- **Participants exceeded expected disease trajectory on NSAA and all timed function tests,** including older boys (aged 8+)

Favorable, differentiated safety profile

- **RGX-202 was well-tolerated**

PIVOTAL TOPLINE DATASET

Biomarker:
N=30[2]

Interim Functional Data:
N=9 participants aged 4+ who reached 12 months post-treatment

Interim Safety:
N=31

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[1] Primary endpoint is the proportion of participants who achieve a RGX-202 microdystrophin expression level of >10% at Week 12, measured via muscle biopsy
[2] 30 of 31 total participants have Week 12 biopsy available for evaluation; one participant refused muscle biopsy

Biomarkers Support Consistent Robust Expression, Transduction, and Sarcolemmal Localization of RGX-202 Microdystrophin

WEEK 12 BIOPSY mean (±SD)	RGX-202 Microdystrophin[1] by western blot (% of normal control)	VCN copies/nucleus (qPCR)	Positive Fibers[2] by immunofluorescence (%)
Aged 1 to <4 (N=10)	115.2 (±81.0)	19.6 (±10.7)	79.3 (±17.4)
Aged 4 to 7 (N=9)	58.0 (±37.2)	21.9 (±16.4)	45.7 (±31.5)
Aged ≥8 (N=11)	41.6 (±24.5)	17.7 (±7.0)	55.0 (±20.7)
Overall (N=30)	71.1 (±60.6)	19.6 (±11.4)	60.6 (±26.7)

Data cut date: April 16, 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. Data available for 23 participants (N=8 for aged 1-4, N=7 for aged 4-7, N=8 for aged 8+) as of data cut date.

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

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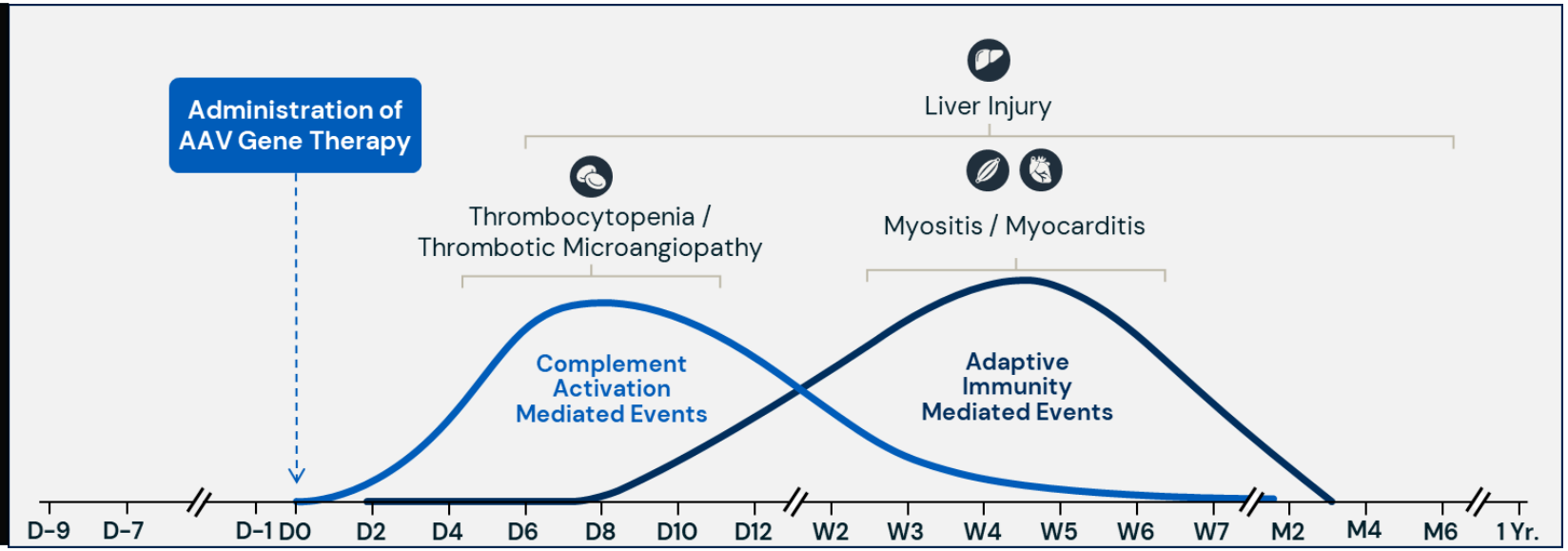
Proactive Immune Suppression Regimen Designed to Mitigate AEs Observed in Other Duchenne Gene Therapy Programs

PREVIOUSLY OBSERVED IMMUNE RESPONSE WITH EXISTING GENE THERAPIES



REGENXBIO NOVEL IMMUNE SUPPRESSION REGIMEN

Eculizumab (Inhibits Complement) — TOTAL OF 5 DOSES

Sirolimus (Inhibits B and T cells via mTOR) — W8 W9–12

Additional Steroids (prednisone) (General immune suppression) — W8 W9–12

Adapted from Servais, Neuromuscular Disorders (2023) 33:884-896.

Duvuru, *Ped Neurol* (2024) 163:1-3. Laforet, *Hum Gene Ther* (2025) 36:64-76. Oskoui, *Neurology* (2025) 104:e213604. Salabarria, *J Clin Invest* (2024) 134(1):e173510. *Silver, Int J Card* (2024) 398:131617. Zaidman, *J Neuromusc Dis* (2024) 11:687-699.

RGX-202 Continues to Demonstrate Favorable Safety Profile

- **RGX-202 was well-tolerated**
- **2 TR-SAEs, both easily managed and resolved without sequelae**
- **All other TR-TEAEs mild to moderate and resolved without sequelae**

TR-SAEs	Aged <4 Years N = 11, n (%)	Aged ≥4 Years N = 20, n (%)	Overall N = 31, n (%)
Subacute myocarditis[1]	0	1 (5.0)	1 (3.2)
Liver injury[2]	0	1 (5.0)	1 (3.2)
TR-TEAEs *	**5** (45.5)	**19** (95.0)	**24** (77.4)
Vomiting	**4** (36.4)	**15** (75.0)	**19** (61.3)
Fatigue	**3** (27.3)	**8** (40.0)	**11** (35.5)
Nausea	**1** (9.1)	**9** (45.0)	**10** (32.3)
Abdominal pain	**0**	**7** (35.0)	**7** (22.6)
Pyrexia	**1** (9.1)	**3** (15.0)	**4** (12.9)

1. 8-year-old participant (23kg weight at dosing) with premature stop codon in exon 60 presented with subacute myocarditis onset 33 days after dosing presenting with normal troponin I and mild elevation of high-sensitivity troponin I (<2x ULN), mild chest and abdominal pain, and no evidence of fibrosis on cardiac MRI. Fully resolved with no sequelae 49 days after onset, and most recent follow up cardiac MRI confirms no heart muscle fibrosis and no change in Ejection Fraction (65%).
2. 10-year-old participant (34kg weight at dosing) with exon 3-7 duplication presented with asymptomatic liver injury diagnosed based on laboratory assessment 43 days after dosing, with GGT peak elevation 123 U/L (2x ULN by local lab, 5x ULN by central lab). Abdominal ultrasound and bilirubin levels were normal. Fully resolved with no sequelae 46 days after onset.

No drug-related thrombocytopenia, myositis, or neurotoxicity reported

Data cut date: April 16, 2026

AE: Adverse event; TR-SAE: Treatment-related serious adverse event; TR-TEAE: Treatment-related treatment-emergent adverse event; GGT: Gamma glutamyl transferase; MRI: Magnetic resonance imaging; ULN: Upper limit of normal

*TR-TEAEs listed are those affecting >= 10% of participants

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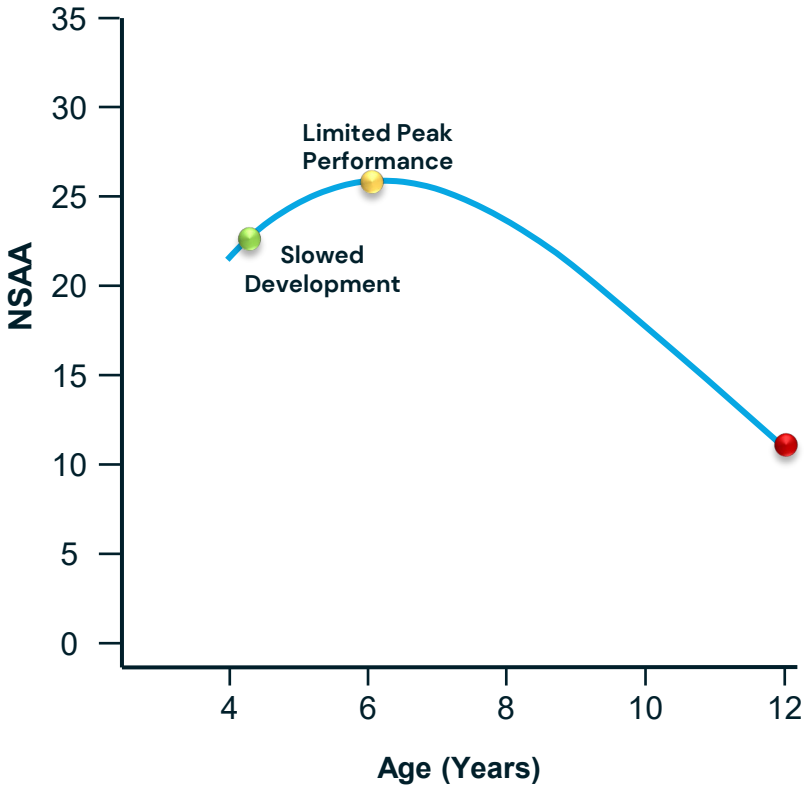
AFFINITY DUCHENNE® External Control Methodology



External Data Sources

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

STEP 1

Filter EC Participants by Key Entry Criteria[1]

- Stable dose of corticosteroid for 12 weeks
- Aged ≥ 4 and ≤1 + the maximum age of treated group
- TTSTAND >3 and <7 Seconds
- TTRW within +/- 1 sec of the treated group

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)[2]

- 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

*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)

[1] Age and functional outcomes criteria based on N=9 participants who received Month 12 as of the April 16, 2026 data cut.

[2] 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.

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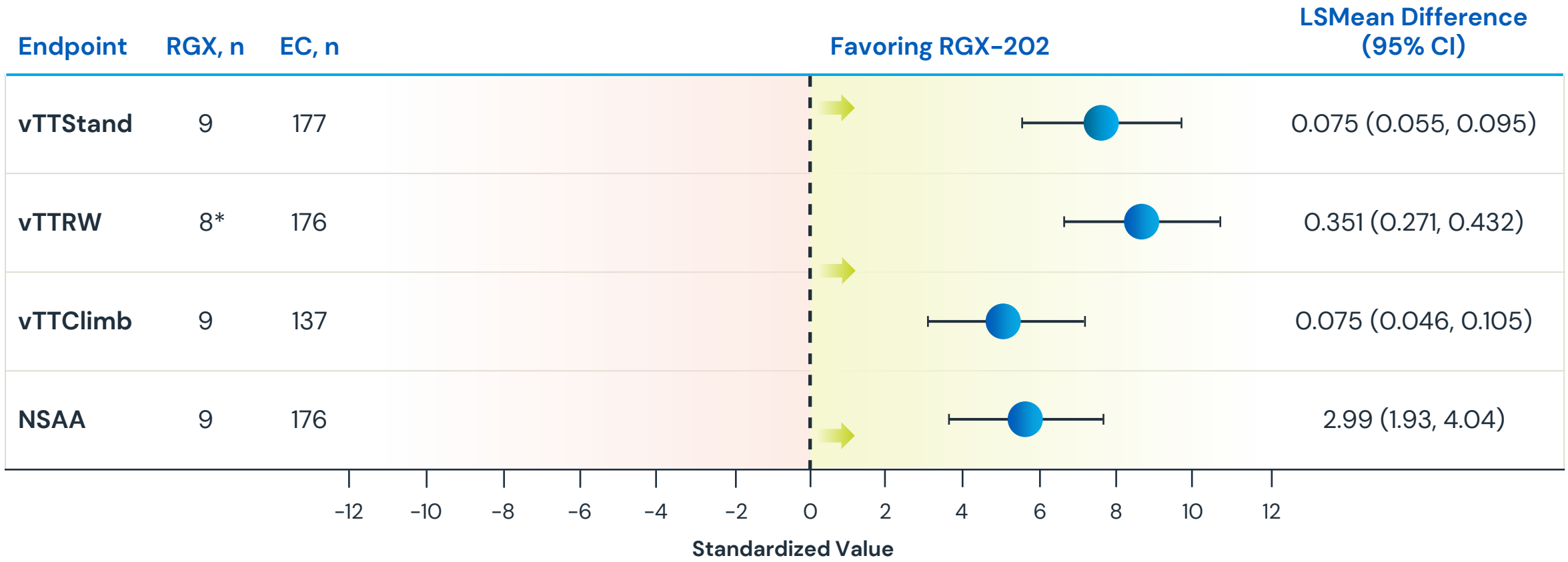
Participants Exceeded External Controls on All Functional Measures at 1 Year

Functional improvements vs. external controls using propensity score weighting

Endpoint	RGX, n	EC, n	LSMean Difference (95% CI)
vTTStand	9	177	0.075 (0.055, 0.095)
vTTRW	8*	176	0.351 (0.271, 0.432)
vTTClimb	9	137	0.075 (0.046, 0.105)
NSAA	9	176	2.99 (1.93, 4.04)



Data cut date: April 16, 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).

*TTRW data for one (n=1) participant had missing data at Week 52, result was determined to be invalid due to participant behavior.

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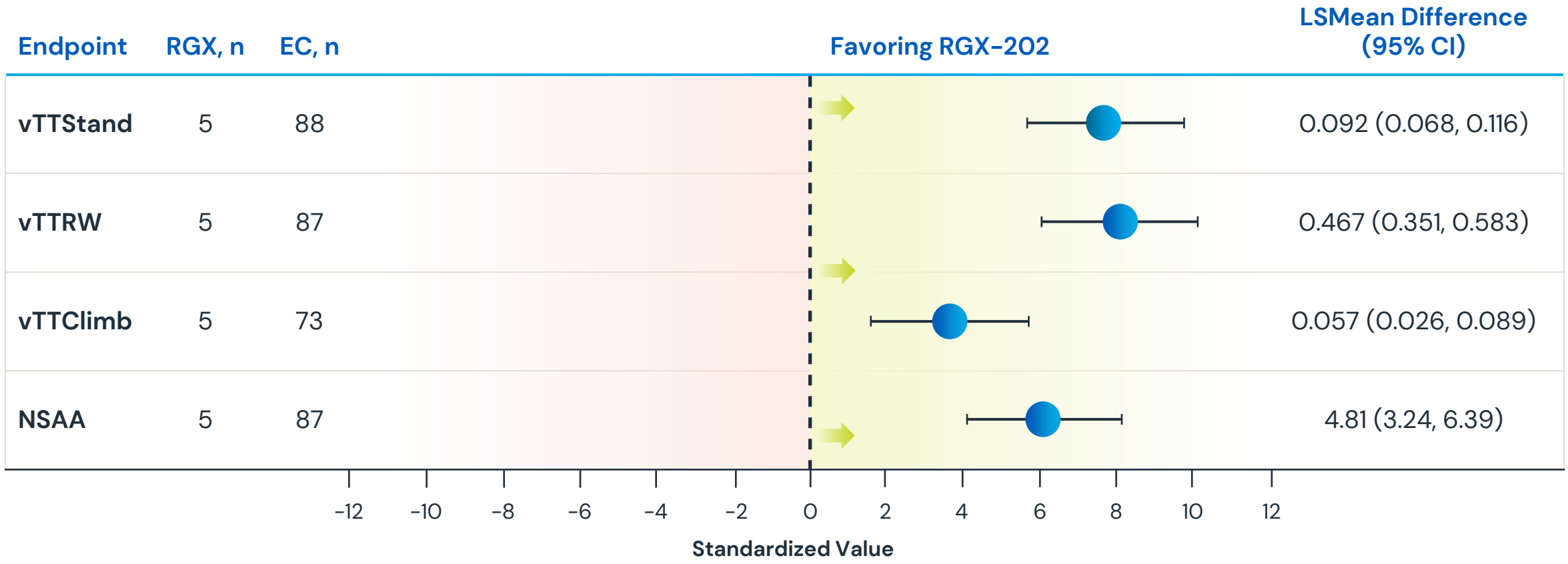
Participants Aged 8+ (N=5) Exceeded External Controls on All Functional Measures at 1 Year

Functional improvements vs. external controls using propensity score weighting

Endpoint	RGX, n	EC, n	LSMean Difference (95% CI)
vTTStand	5	88	0.092 (0.068, 0.116)
vTTRW	5	87	0.467 (0.351, 0.583)
vTTClimb	5	73	0.057 (0.026, 0.089)
NSAA	5	87	4.81 (3.24, 6.39)



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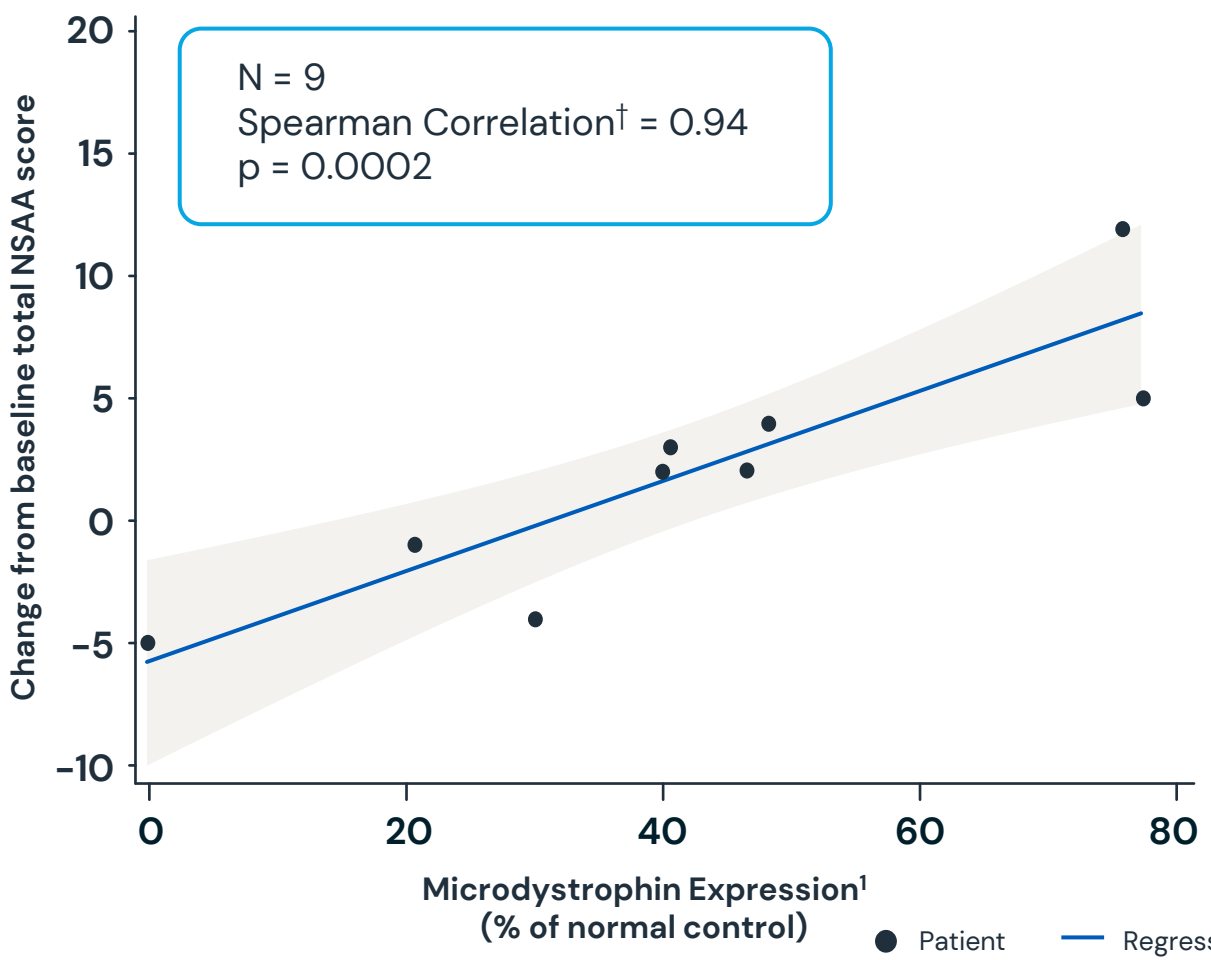
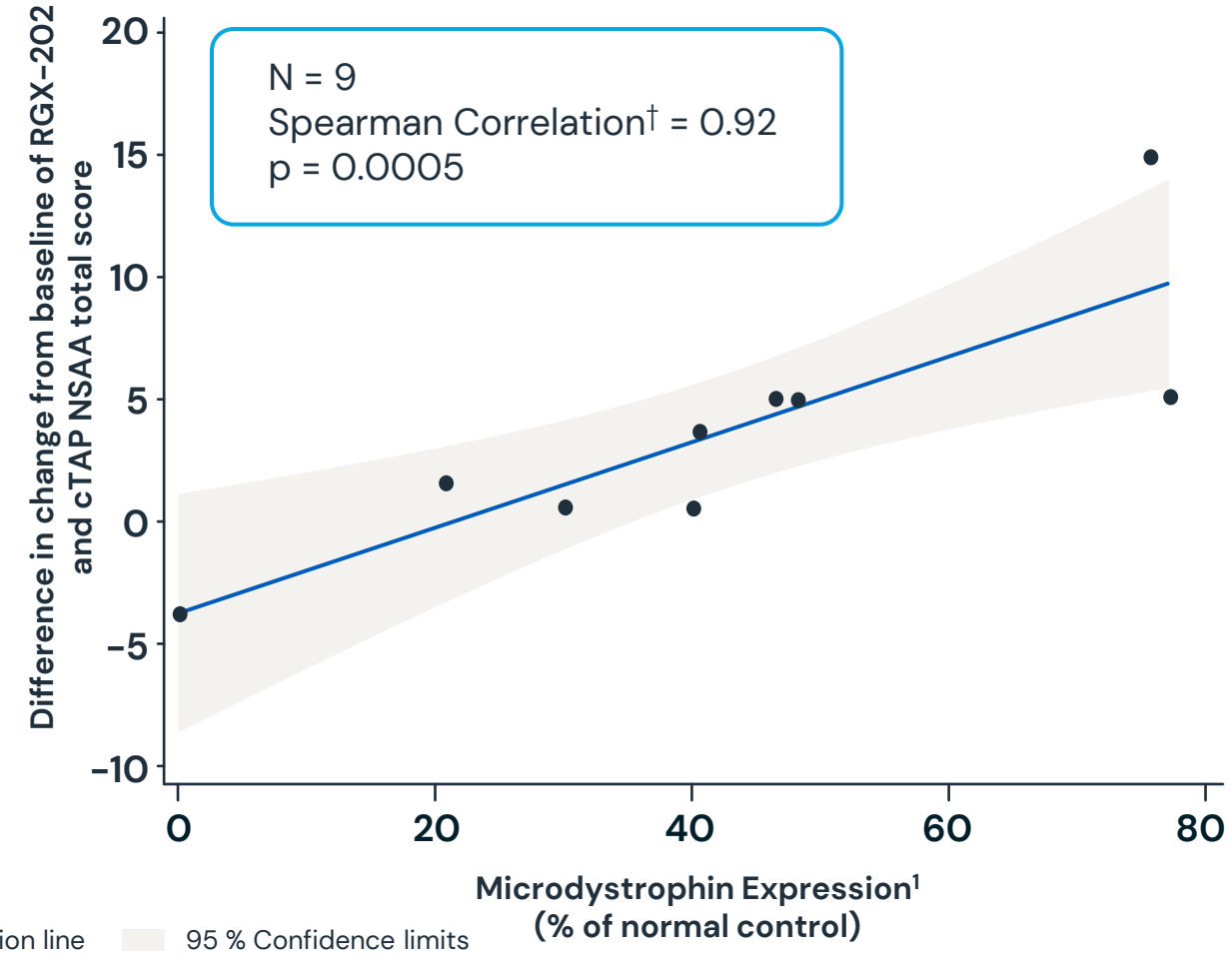
RGX-202 Microdystrophin Expression Correlated with Functional Improvement

Statistically significant correlation with NSAA change from baseline and from cTAP predicted value at 1 year





Patient — Regression line — 95 % Confidence limits

NSAA: North Star Ambulatory Assessment

†Additionally, regression model was used to visualize the linear relationship between microdystrophin expression and change from baseline in NSAA function. To support linear relationship of figure results, Pearson correlation coefficient was also calculated (r=0.88[p= 0.0016] for change from baseline, r=0.84[p= 0.0048] for difference from cTAP), p-values accounted for potential confounding effect of age at dosing and baseline function are 0.0132 (Left) and 0.0027 (Right).

¹Microdystrophin expression assessed at Week 12 visit; adjusted for muscle content; % normal control

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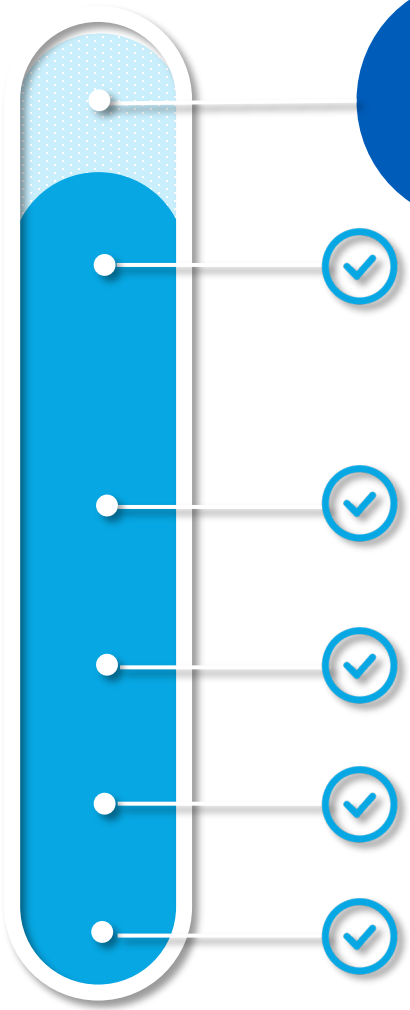
RGX-202 momentum and commercial readiness



Initiate rolling BLA submission Q3 2026 under accelerated approval pathway to support potential approval and launch in 2H 2027

May '26: Positive topline pivotal data; primary endpoint met with statistical significance + landmark correlation between microdystrophin and functional improvement supporting potential accelerated approval

October/November '25: Completed pivotal enrollment, initiated confirmatory trial (add'l 30 patients)

September '25: Began manufacturing batches intended for commercial supply

May/June '25: Passed 50% enrolled in pivotal, presented positive interim phase 1/2 data

November '24: Initiated pivotal trial, began ~30 ambulatory patients aged 1+

Preparing to meet demand for improved options

- Planning for broad ambulatory access
- Only sponsor with full control of drug supply
- Building commercial infrastructure
- 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

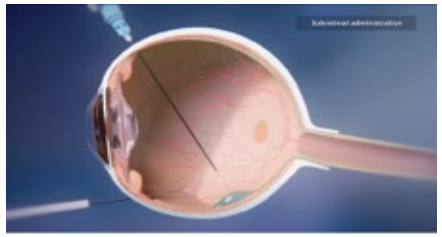
Uses the NAV® AAV8 vector to encode an antibody fragment designed to inhibit vascular endothelial growth factor (VEGF) and fluid accumulation in the retina

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



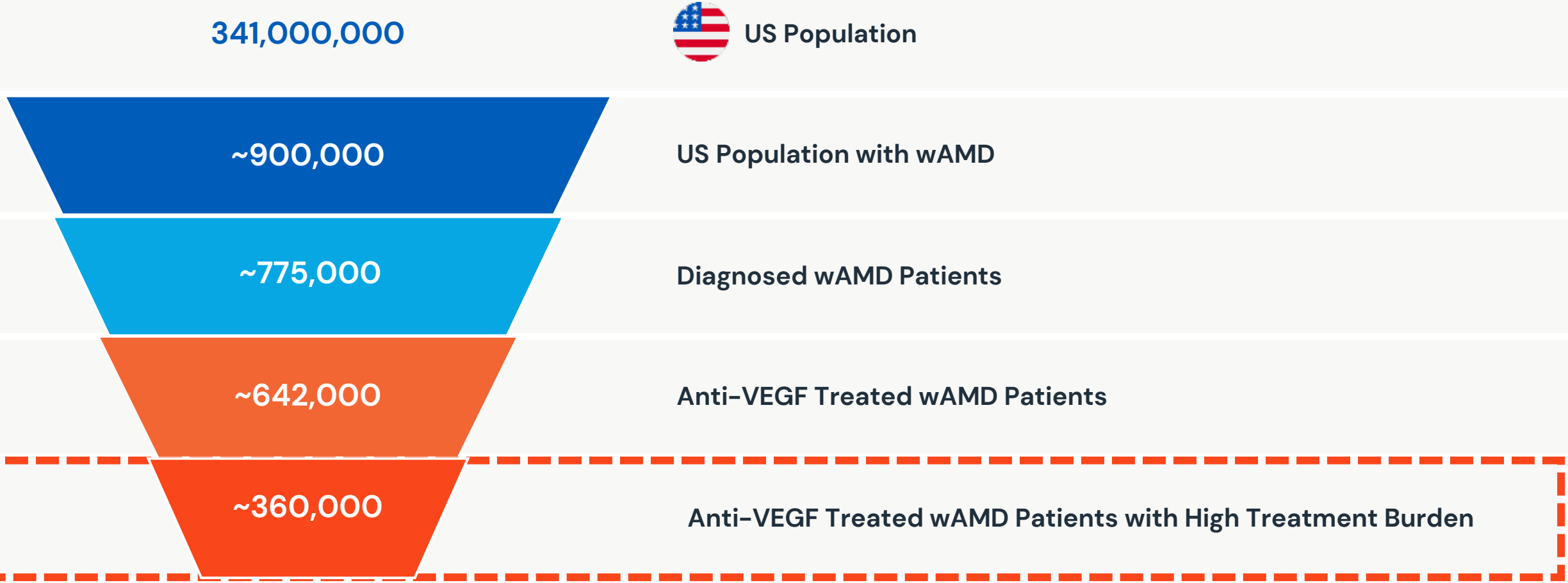
- SR space most immune-privileged space for ocular gene therapy
- No prophylactic steroids in SR

SUPRACHOROIDAL

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

REGENXBIO

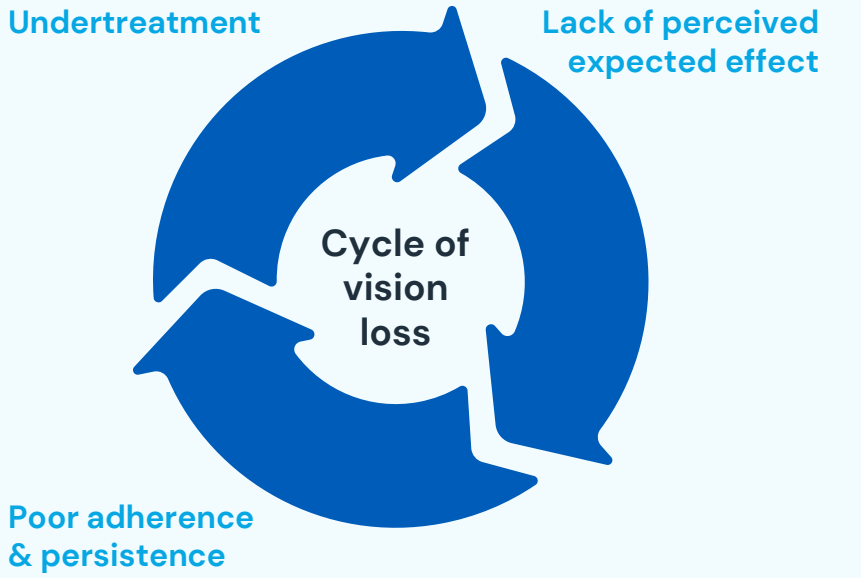
Majority of anti-VEGF-treated patients face high burden + poor compliance, leading to vision loss over time



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®)

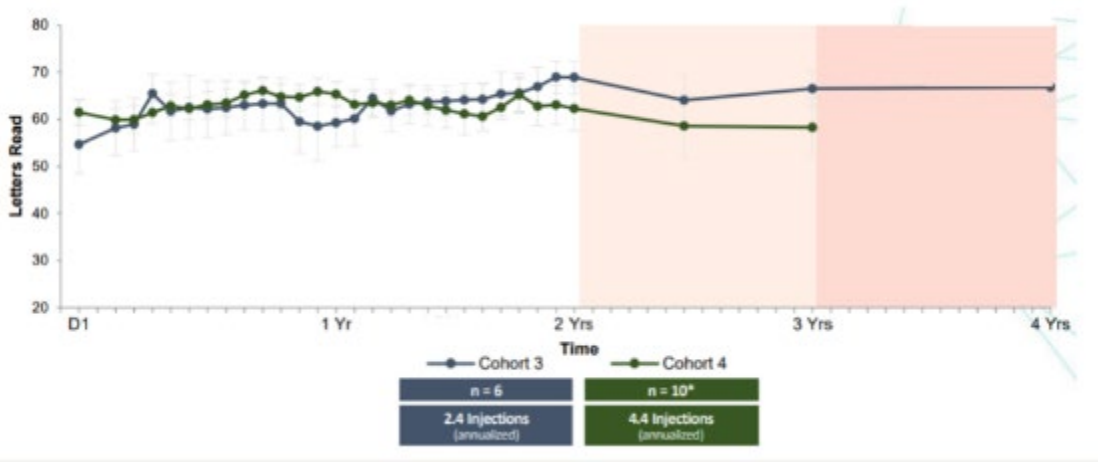
Two pivotal trials evaluating subretinal delivery for wet AMD, with Phase I/IIa data demonstrating long-term safety and tolerability, stable to improved vision and retinal thickness

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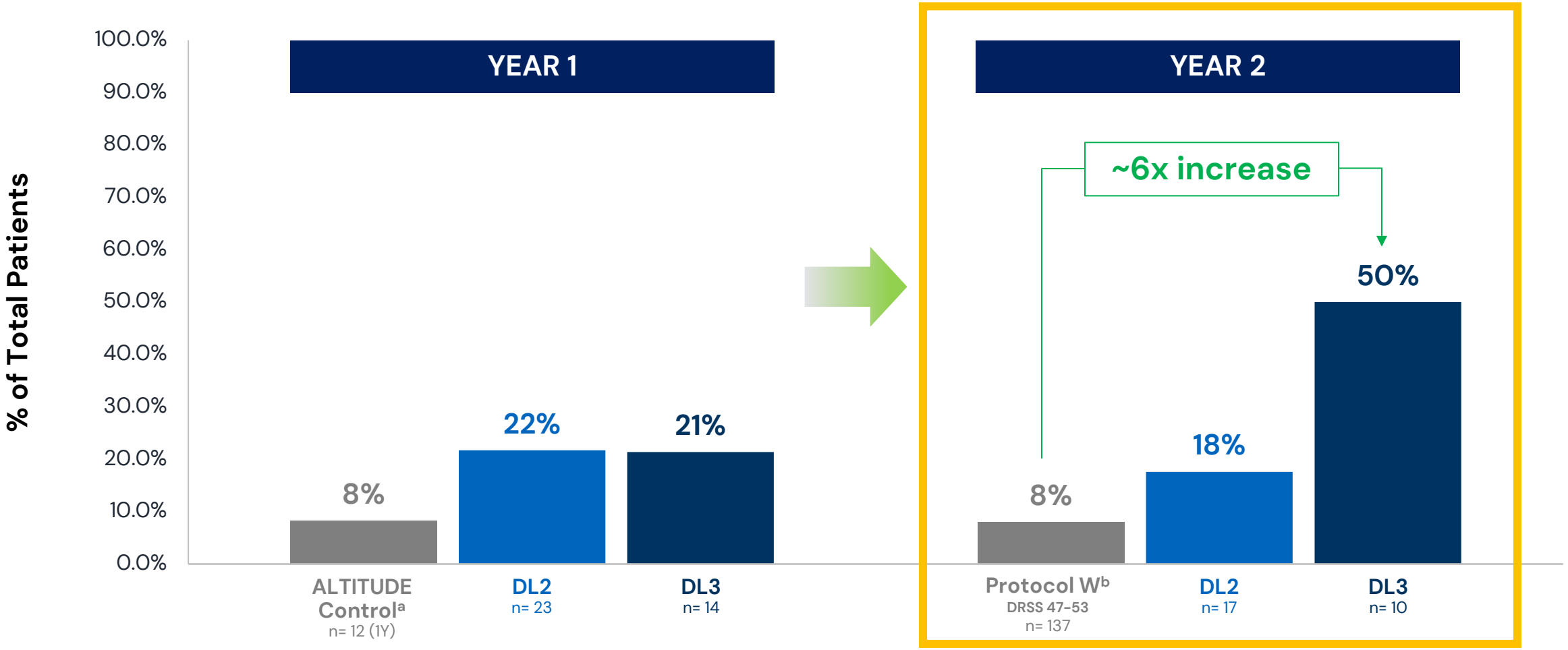
*Dose Level 1 at 2.5x1011 GC/eye; Dose Level 2 at 5x1011 GC/eye; Dose Level 3 at 1x1012 GC/eye; Dose Level 4 at 1.5x1012 GC/eye
Data cut: June 9, 2025.

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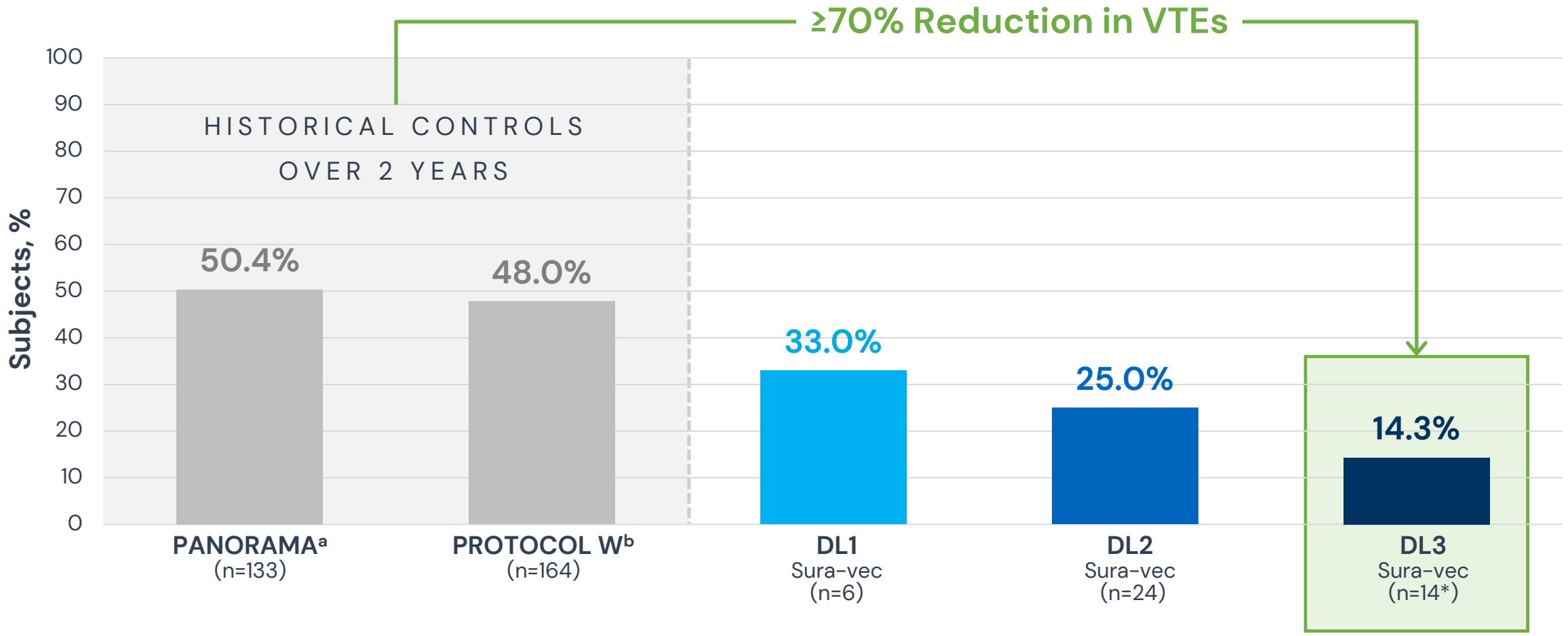
≥ 70% risk reduction in vision threatening events over 2 years observed in DL3 subjects treated



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.

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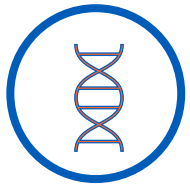
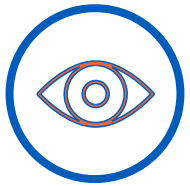
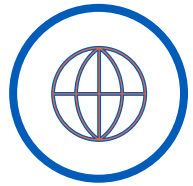
Global eye-care alliance with AbbVie validates retina franchise and enables world-class commercialization capabilities



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 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

REGENXBIO

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 %**

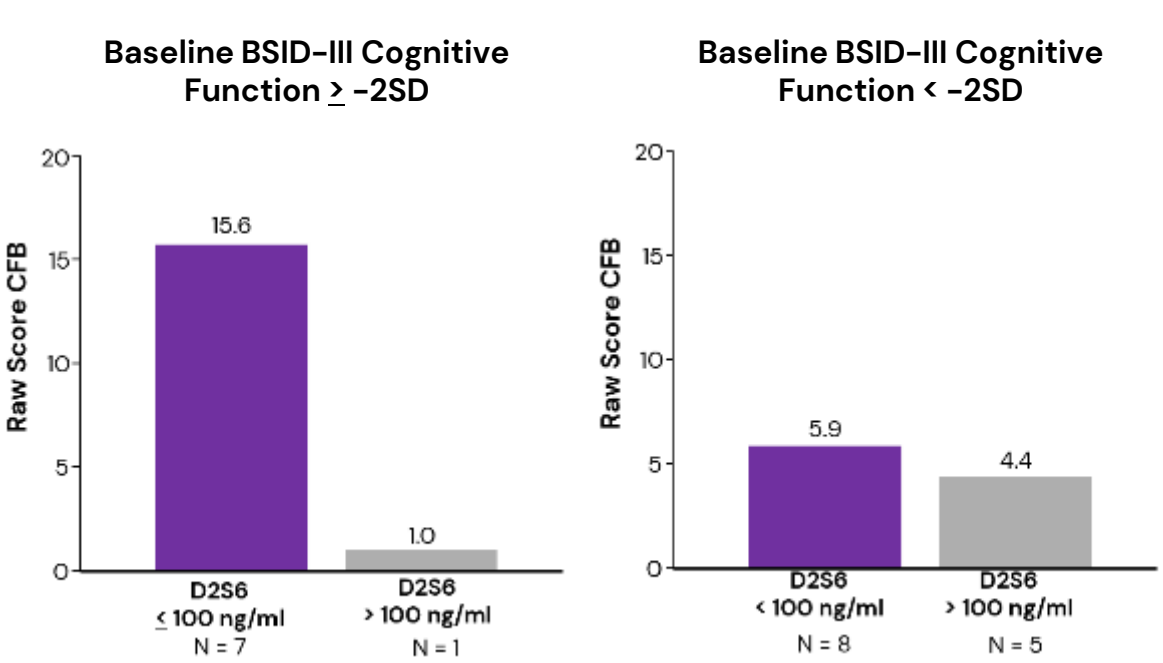
Pivotal: Neurodevelopmental Skill Acquisition or Stability on all BSID-III Subscales in Participants at 1 Year

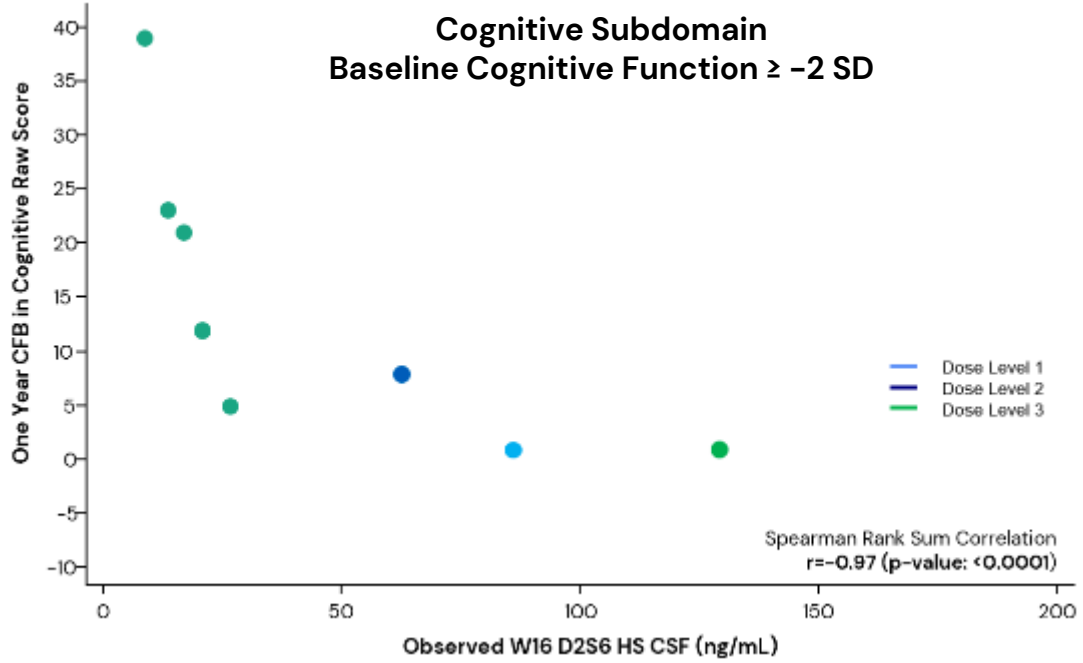
	Above/Equal to -2SD AEq (SE) N = 5			Below -2SD AEq (SE) N = 8		
BSID-III* Subscale**	Baseline	Year 1	**Change from Baseline**	Baseline	Year 1	**Change from Baseline**
Cognitive	15.7 (6.0)	24.2 (4.4)	**+ 8.5** (3.3)	13.9 (3.1)	16.6 (2.9)	**+ 2.7** (1.5)
Fine motor	16.1 (6.6)	22.6 (4.3)	**+ 6.5** (3.5)	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)

REGENXBIO

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





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

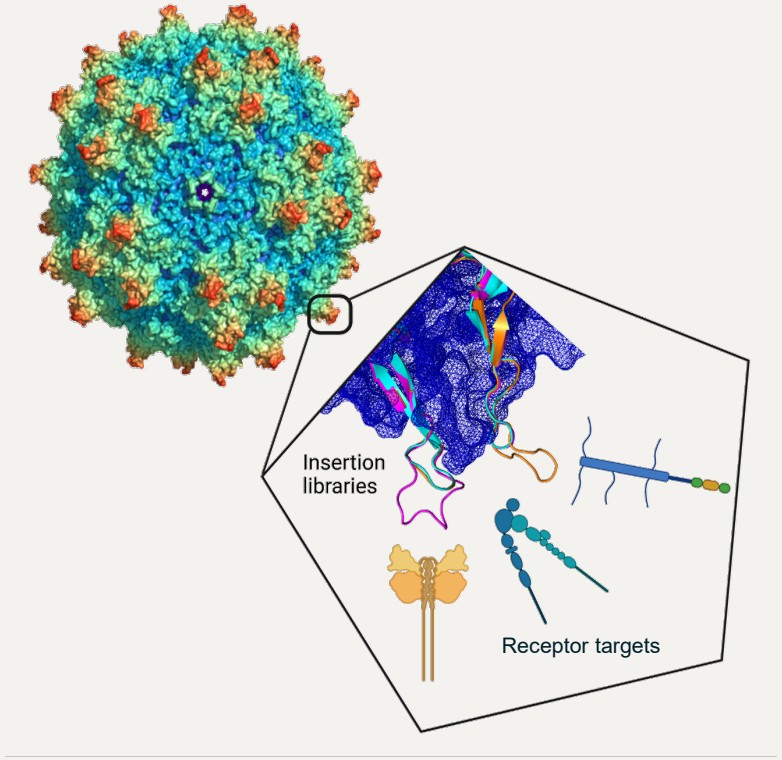
Discovering the next wave of gene therapies

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

REGENXBIO

Seeking to improve lives through the curative potential of gene therapy

REGENXBIO