# Delivering the promise of gene therapy

REGENXBIO Corporate Presentation

September 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





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# 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

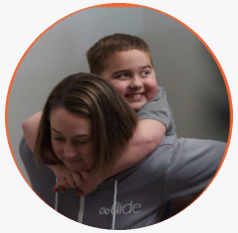
FDA

## Orphan Drug, RMAT & Fast Track Designations

Six designations provided across programs to date

# Two lead programs approaching regulatory approvals

## *Differentiated gene therapies addressing substantial patient populations*



**Andy**
*Living with Duchenne*

### RGX-202

**DUCHENNE MUSCULAR DYSTROPHY**

**PATIENT POPULATION**

**~15K**[1]

U.S. patients

**DIFFERENTIATION**

**Potential best-in-class**

gene therapy for DMD

**ANTICIPATED REGULATORY MILESTONE**

**2027**

BLA submission (1Q) and Potential U.S. approval (2H)



**Gerie**
*Living with wet AMD*

### Sura-vec

**WET AMD**

**PATIENT POPULATION**

**~5.7M**[2]

Expected patients in US, EU and Japan by 2027

**DIFFERENTIATION**

**Potential first**

gene therapy for wet AMD

**ANTICIPATED REGULATORY MILESTONE**

**2027**

Global regulatory submissions

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Patients have not participated in a REGENXBIO clinical trial
1. Crisafulli et al 2020; US Census Bureau, 2. Market Scope 2022 Retina Pharmaceuticals Report

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# 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 on a clinical hold in January 2026 and RGX-121 on clinical hold in August 2026.*

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# Our future is now

## *Recent & upcoming milestones demonstrate momentum toward commercial stage*

**Recent & anticipated milestones**

| | 2026 | 2027 |
|---|---|---|
| **RGX-202**<br>Duchenne Muscular Dystrophy | ✓ Topline pivotal data (2Q)<br>✓ Complete confirmatory enrollment | • U.S. BLA submission (1Q)<br>• Initiate AFFINITY RISE®[1] (1H)<br>• Potential FDA approval (2H) |
| **Sura-vec**<br>Wet AMD | ✓ 5-year Ph I/II follow up data (July)<br>• Topline pivotal data (4Q) | • Global regulatory submissions (2027)[2] |
| **Sura-vec**<br>Diabetic Retinopathy | ✓ Ph IIb/III first patient dosed (1H)<br>✓ 2.5-year Ph II follow-up data (July) | |
| **RGX-121**<br>Hunter Syndrome (MPS II) | ✓ Type A Meeting (July) | |

*[1] AFFINITY RISE is an ex-US, randomized, placebo-controlled study designed to support future global regulatory submissions*

*[2] $100M in milestone payments to REGENXBIO between sura-vec BLA acceptance and first commercial sale of sura-vec in wet AMD*

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# 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

# 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:** Potential for approval in 2H 2027 to serve as the next approved gene therapy in Duchenne
- **Encouraging clinical data:** Only gene therapy program to demonstrate the combination of robust microdystrophin expression, functional improvement across timed function tests, statistically significant, strong correlation between microdystrophin expression and NSAA and a favorable safety profile.
- **Leading Purity:** 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

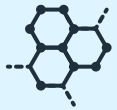


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# RGX-202's proactive, comprehensive therapeutic approach

## Multiple factors may contribute to better outcomes for patients

### 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

### Purity



- Leading purity levels (>80% full capsids) in Duchenne gene therapy enables maximum therapeutic dose ($2x10^{14}$ GC/kg) with lower capsid load

## RGX-202 Pivotal Program

AFFINITY DUCHENNE®

- Evaluating RGX-202 ambulatory boys aged 1+
- 60+ patient dosed between pivotal and confirmatory studies
- Pivotal primary endpoint: proportion of participants with a microdystrophin expression level of >10% at Week 12



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# 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



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# 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.

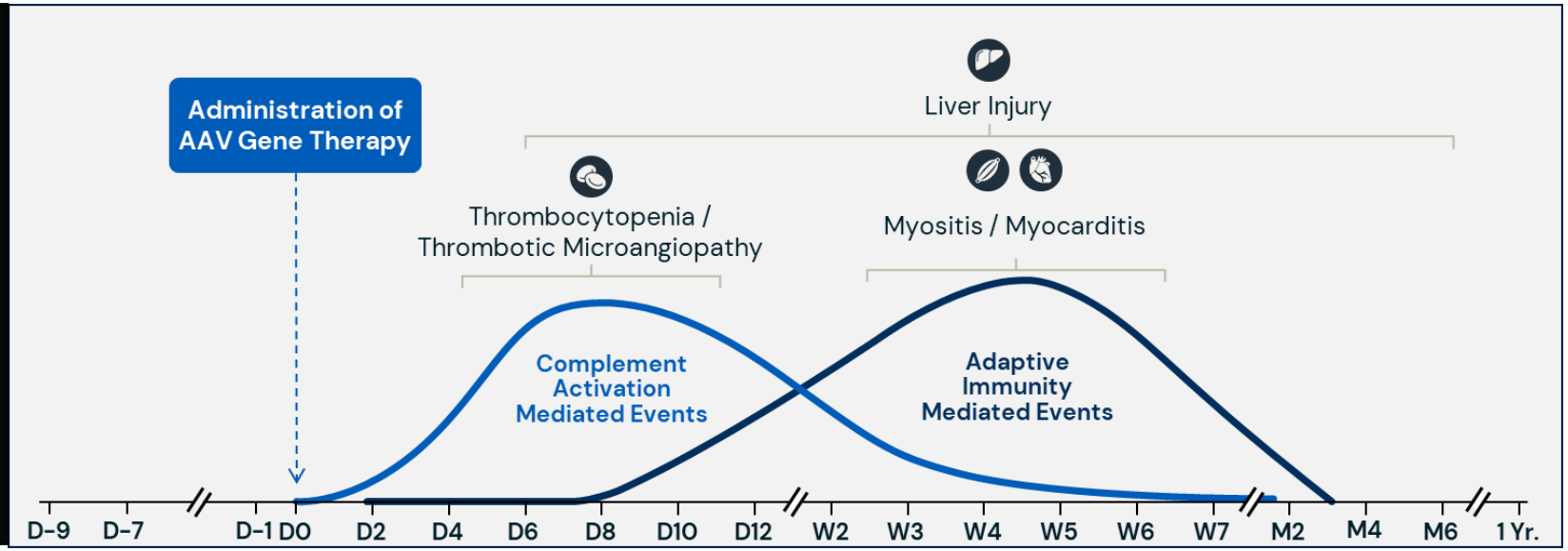
SD: Standard deviation; VCN: Vector copy number; qPCR: Quantitative polymerase chain reaction; GC: Genome copies



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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.

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# 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

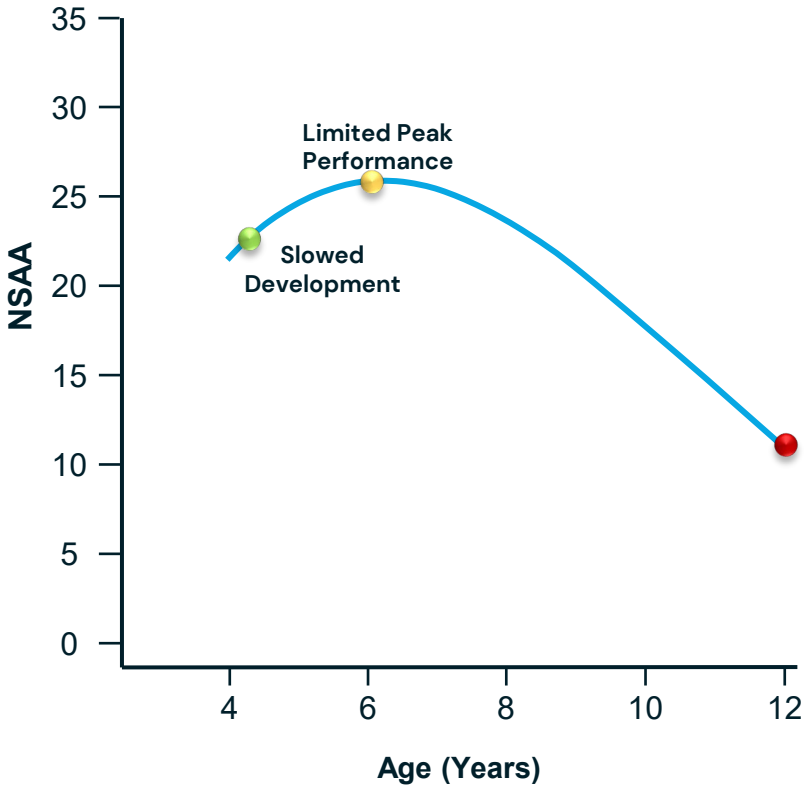
AE: Adverse event; TR-SAE: Treatment-related serious adverse event; TR-TEAE: Treatment-related treatment-emergent adverse event; GGT: Gamma glutamyl transferase; MRI: Magnetic resonance imaging; ULN: Upper limit of normal

*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.

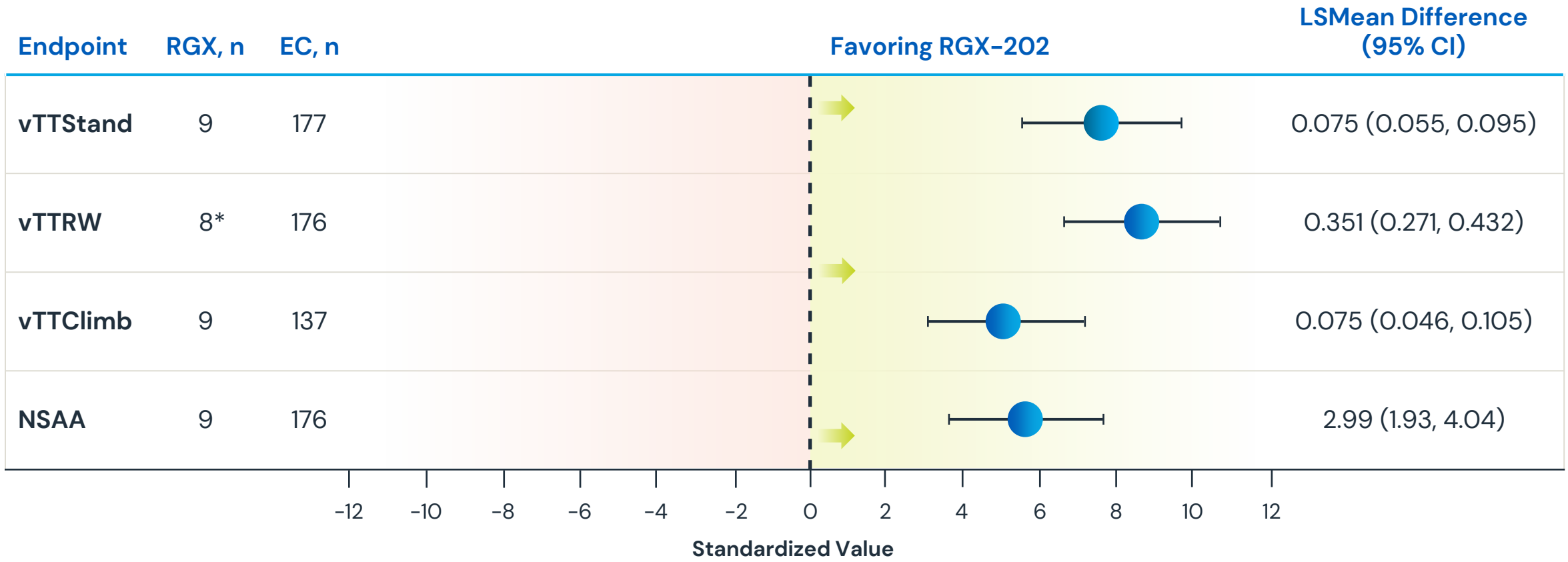
[2] Muntoni F, Signorovitch J, Goemans N, Manzur AY, Done N, Sajeev G, Li J, Akbarnejad H, Sharma A, Ward SJ, Niks EH, Servais L, Mercuri E, Guglieri M, Straub V, de Groot I, Ridout D; PRO-DMD-01 study investigators; Association Française contre les Myopathies; NorthStar Clinical Network; McDonald C. 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




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.

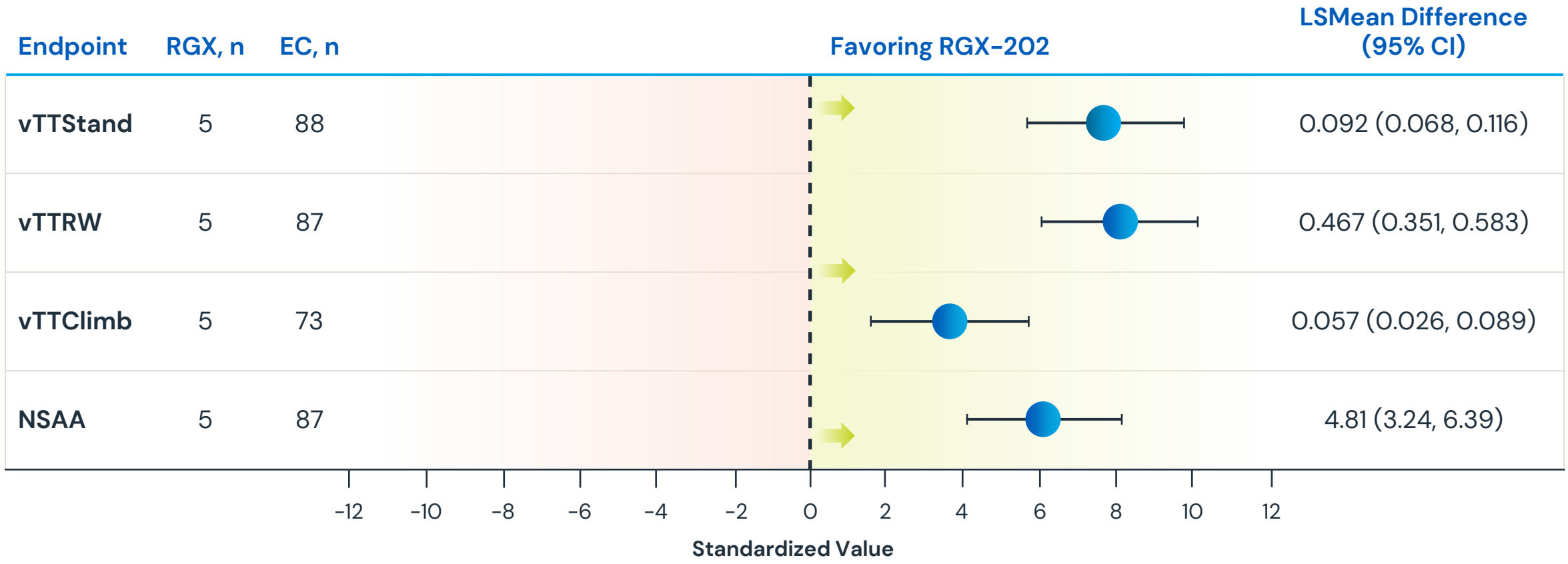
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# Participants aged 8+ (N=5) exceeded external controls on all functional measures at 1 year

## 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) |




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

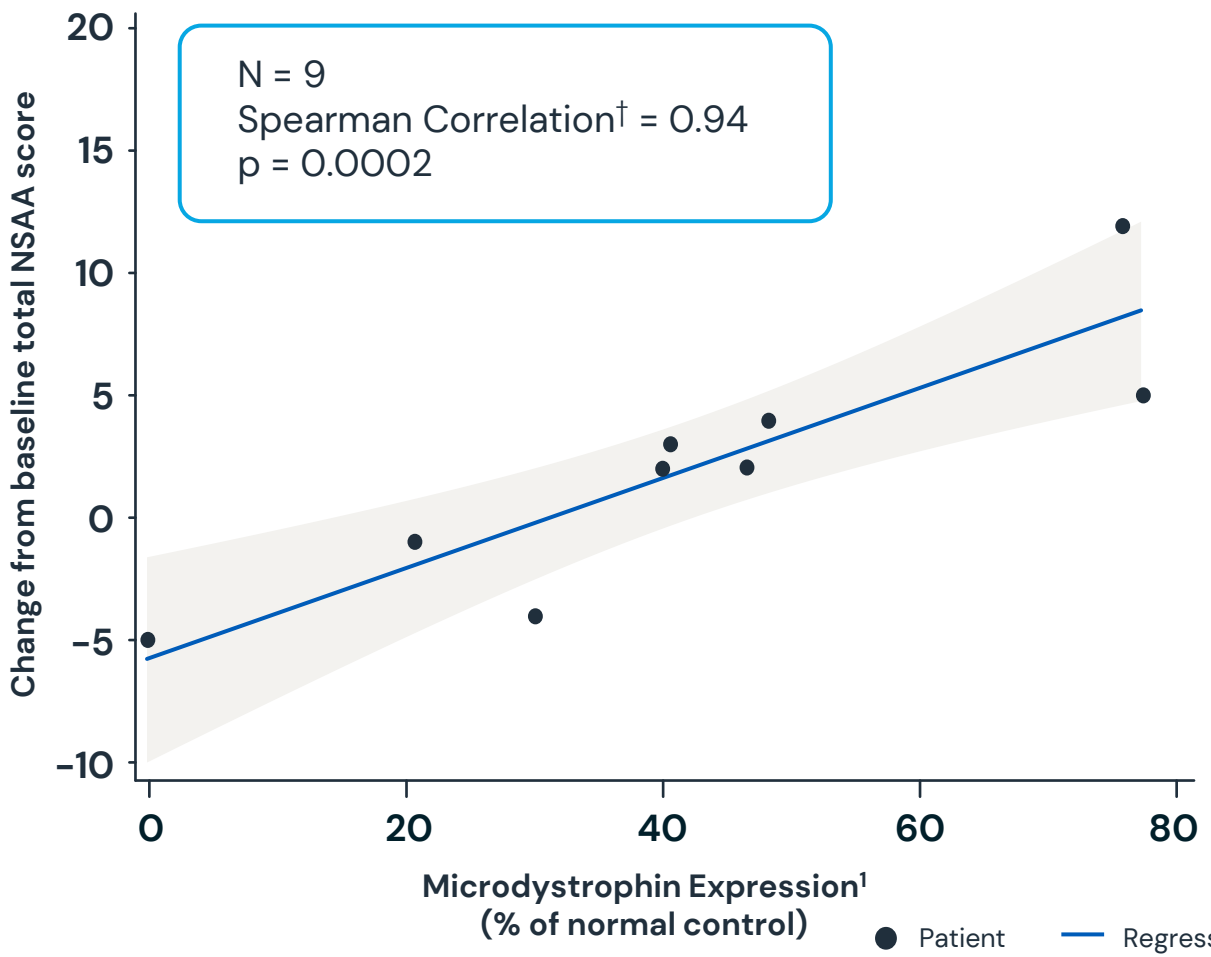
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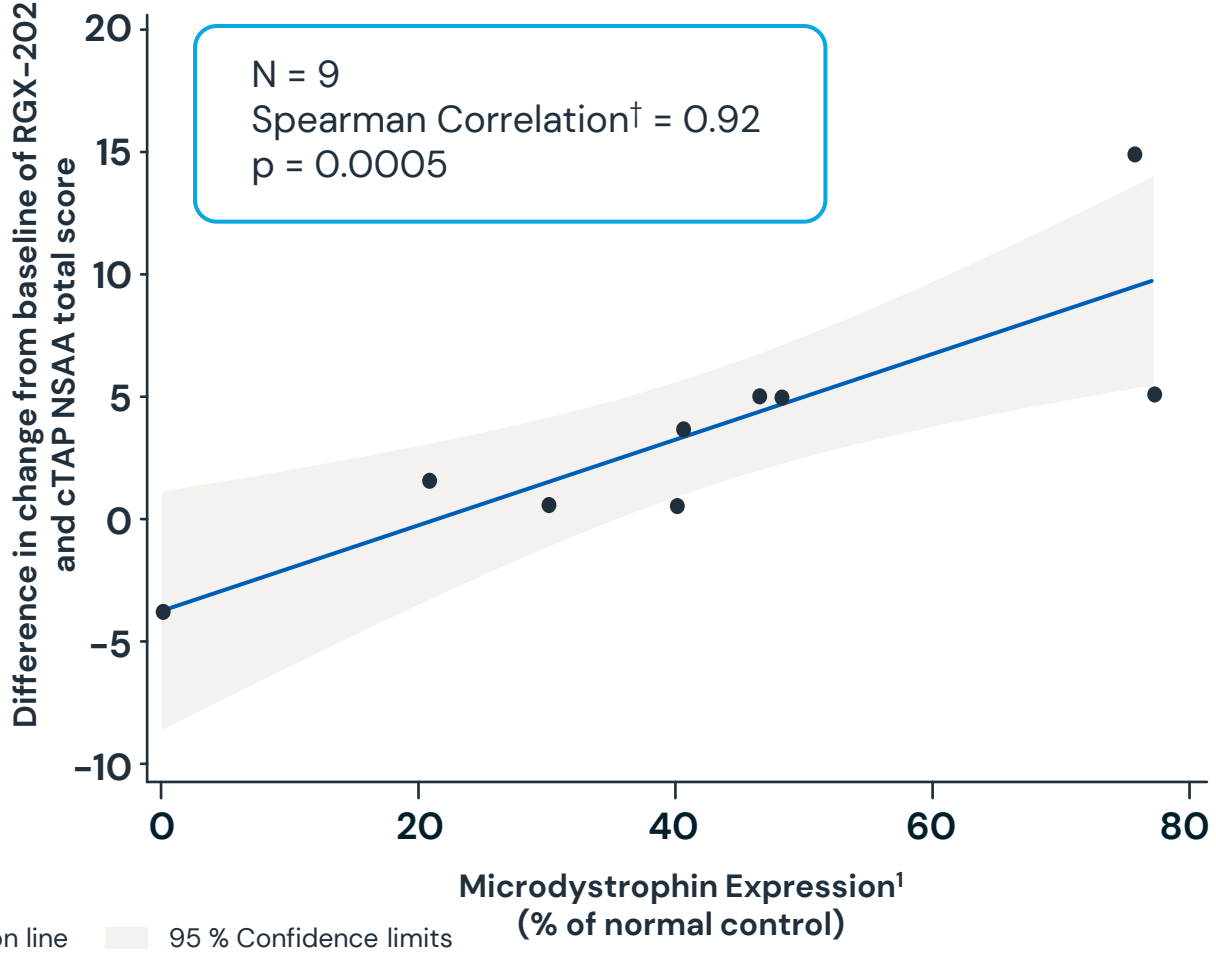
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# RGX-202 microdystrophin expression correlated with functional improvement

## 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

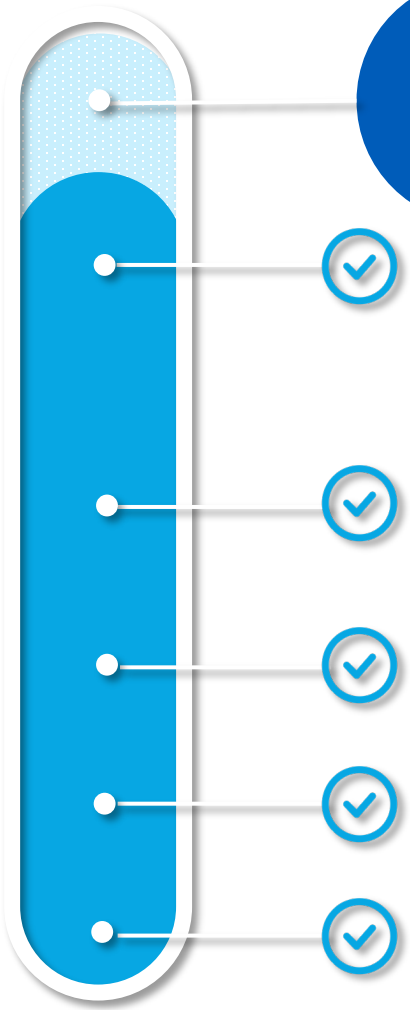
†Additionally, regression model was used to visualize the linear relationship between microdystrophin expression and change from baseline in NSAA function. To support linear relationship of figure results, Pearson correlation coefficient was also calculated (r=0.88[p= 0.0016] for change from baseline, 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).

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

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# RGX-202 momentum and commercial readiness



**Submit BLA in Q1 2027 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
- On track to initiate AFFINITY® RISE ex-U.S. randomized, placebo-controlled study in 1H 2027 to support global regulatory submissions

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

## Potential to be the first gene therapy for Wet AMD and Diabetic Retinopathy

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# Sura-vec: Potential first gene therapy for wet AMD and diabetic retinopathy

- **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



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# Sura-vec is a one-time investigational gene therapy designed to deliver sustained treatment effect

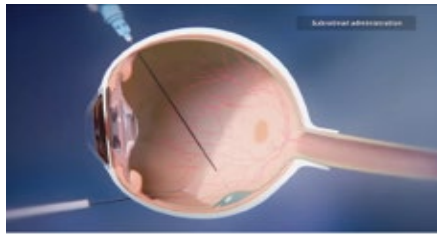
Uses the NAV® AAV8 vector to encode an antibody fragment designed to inhibit vascular endothelial growth factor (VEGF) and fluid accumulation in the retina

**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**



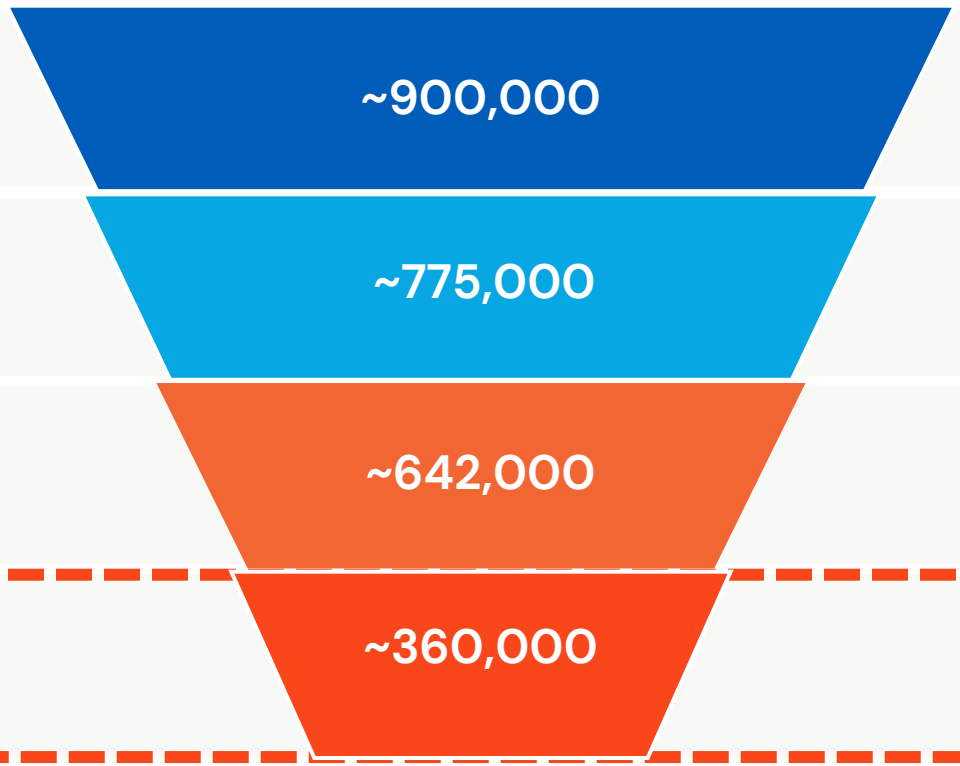
- SCS allows for in-office delivery of ocular gene therapy with minimized inflammation risk
- Minimal, 7-week prophylactic steroids in SCS

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

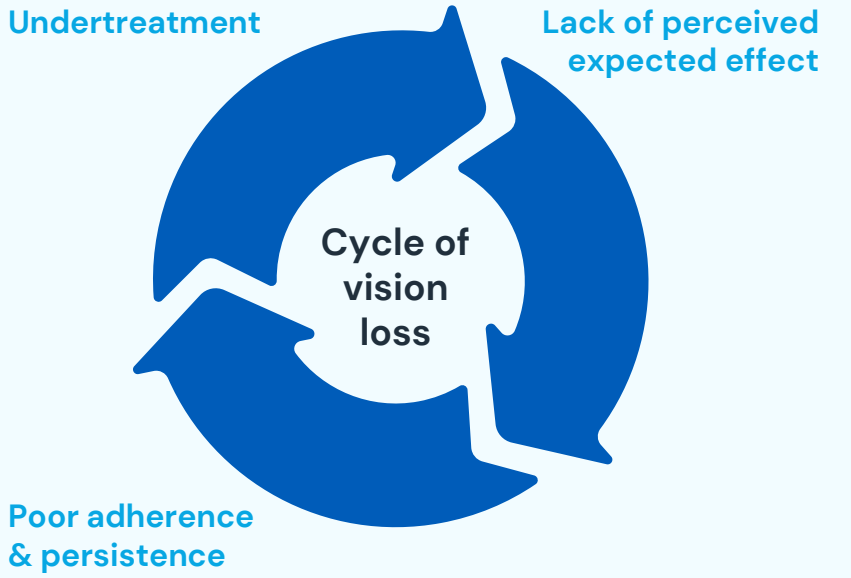



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*US Census Bureau, 2024 Clarivate DRG AMD Report; 2024 Vestrum EMR w AMD Analysis;*
*High treatment burden defined as ≤ 8-week injection intervals*

# 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



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

# Wet AMD: Subretinal sura-vec is in late-stage clinical trials and is on track to be the first gene therapy

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 5 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 pivotal data expected Q4 2026

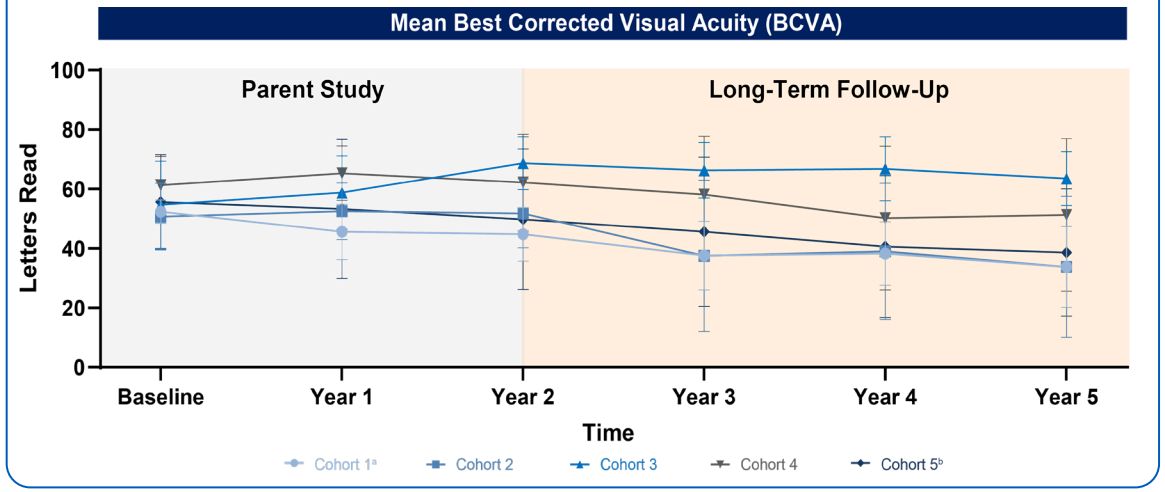
Data cut August 29, 2022



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

## Phase I/IIa LTFU (BCVA)




## Overall Safety

- Sura-vec has been well tolerated across Phase I/II (up to 5 years)* and Phase II Bioreactor Bridging^ studies (at 1 year) at doses similar to pivotal study
  - No post-operative intraocular inflammation (IOI) events deemed related to sura-vec
  - 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 5 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 November 21, 2025; ^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.

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

## Phase IIb/III NAAVIGATE

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

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 55% of participants achieving > 2-step improvement without additional DR treatment at 2.5 years
- Dose Level 3 prevented disease progression in NPDR participants and reduced vision-threatening events by >70% over 2 years compared to historical controls

**First patient in NAAVIGATE study was dosed in Q2 2026**

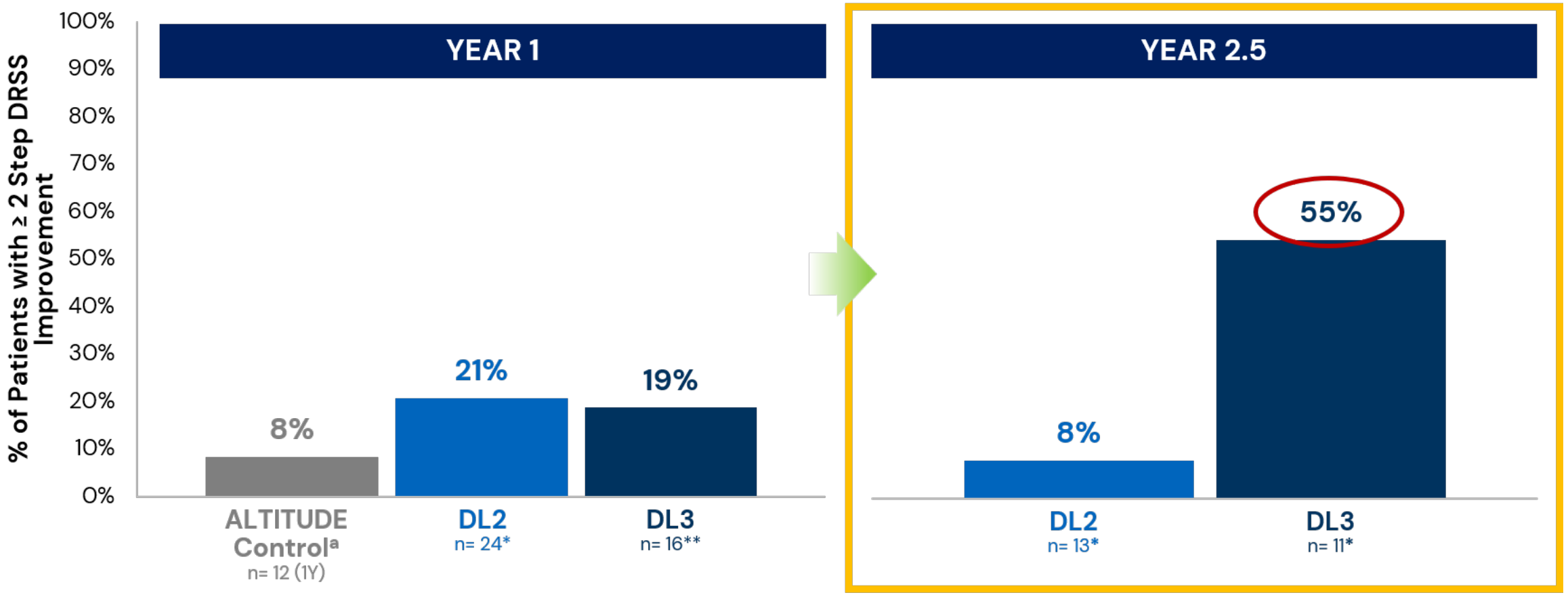
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*Dose Level 1 at 2.5x1011 GC/eye; Dose Level 2 at 5x1011 GC/eye; Dose Level 3 at 1x1012 GC/eye; Dose Level 4 at 1.5x1012 GC/eye
Data cut: June 9, 2025.



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# ≥2-Step DRSS improvement without additional DR treatment at 2.5 years; DL3 sura-vec treated subjects outperformed all other groups and controls

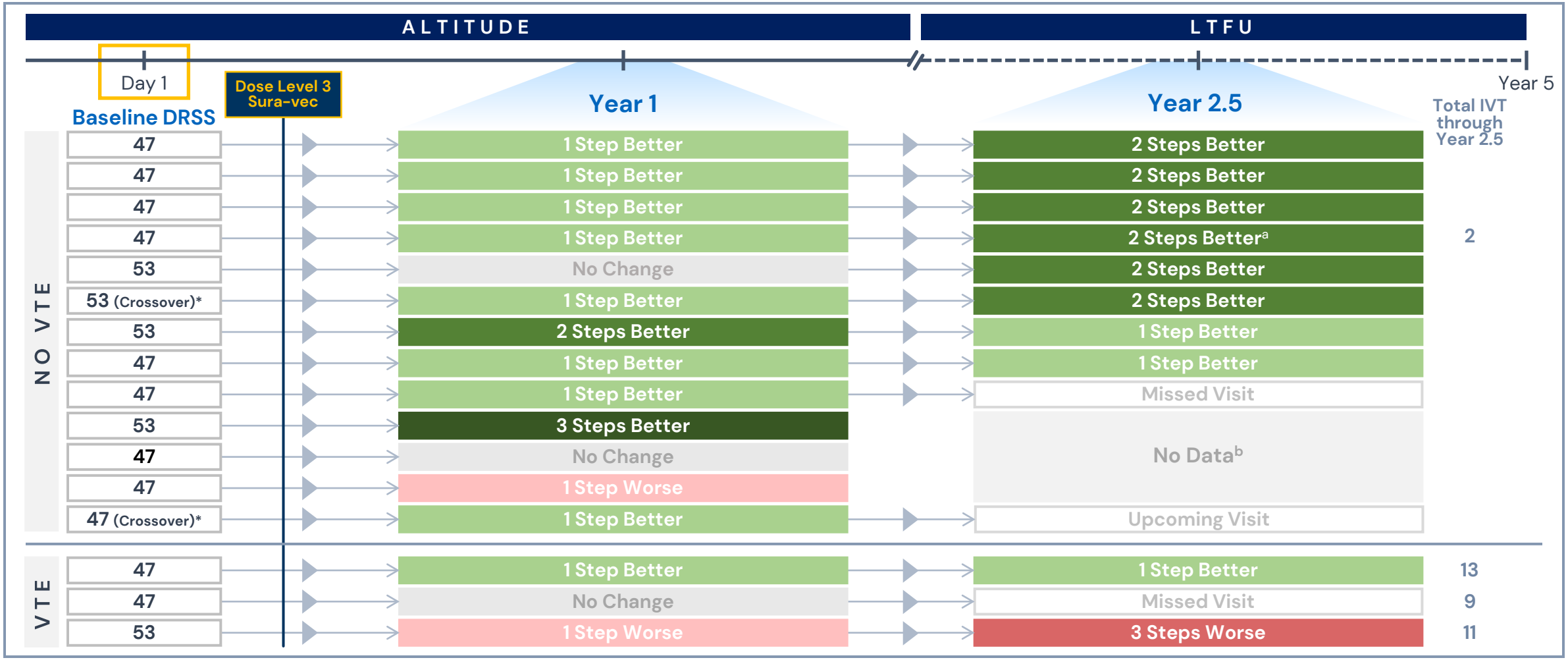



Data cut: May 25, 2026.
*Includes 1 subject who crossed over from control; **Includes 2 subjects who crossed over from control.
Control subjects were eligible to cross over to receive sura-vec at Year 1. Subjects who were DRSS 47 and 53 at crossover are included in the treatment arm analyses.
One subject 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. One subject In Dose Level 3 without IVT missed their 2Y visit but had a 2-Step DRSS improvement at their 2.5Y visit. DL: Dose Level; DR: diabetic retinopathy; DRSS: Diabetic Retinopathy Severity Scale; IVT: intra-vitreal therapy; Sura-Vec: surabgene lomparvovec; Y: year.

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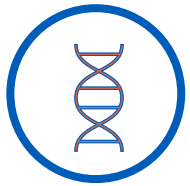
# 55% Achieved ≥2-Step Improvement without Additional DR Treatment at 2.5 Years

## › 70% of Pivotal Dose Subjects Did Not Experience a VTE

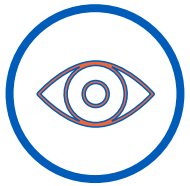



Data cut: May 25, 2026. *Control subjects were eligible to cross over to receive sura-vec at Year 1. Subjects who were DRSS 47 and 53 at crossover are included in the treatment arm analyses (n=2).
a. Subject received 2 IVT after Y2 for DME, but OCT showed dry retina at the time of injection. Subject was receiving IVT for FE DME. b. One subject enrolled in long-term follow-up and had no return visits. One subject in Dose Level 3 was found to have confounding disease at baseline and their data was excluded.
VTEs = VTCs + CI-DME; VTCs could include PDR or ASNV ; DR: diabetic Retinopathy; DRSS: Diabetic Retinopathy Severity Scale; FE: Fellow eye; IVT: intra-vitreal therapy: Sura-Vec: surabgene lomparvovec.
This is an interim analysis performed by REGENXBIO for an ongoing trial.
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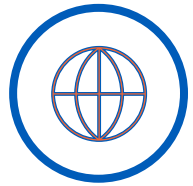
# Global eye-care alliance with AbbVie validates retina franchise and enables world-class commercialization capabilities



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



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# **RGX-121** (clemidsogene lanparvovec):

## Potential first gene therapy for Hunter Syndrome (MPS II)

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# 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
- **Commercial-ready:** Strategic partnership with Nippon Shinyaku
- **Strategic value:** If RGX-121 is approved, REGENXBIO expects to receive a Priority Review Voucher

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

The FDA placed the program on clinical hold in August 2026.

# RGX-121 is 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) |

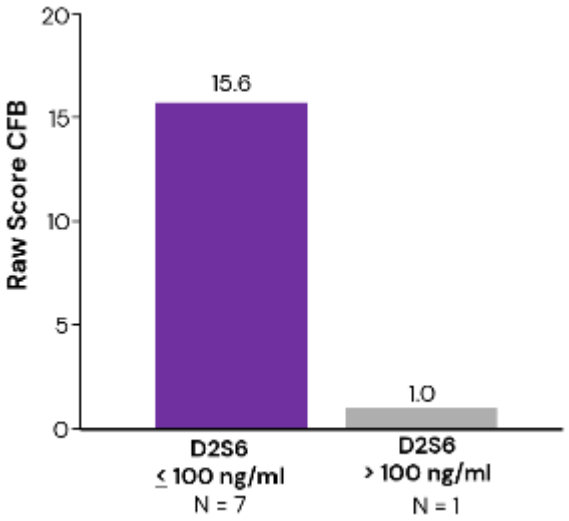
Data cut date: August 20, 2024
MPS II attenuated reference point < 100 ng/mL based on analysis presented in Boulos N, World Symposium, 2022
* Primary endpoint was compared to the margin of 20%
** Samples could not be obtained from 2 participants at 1 year
*** BSID-III, Bayley Scale of Infant and Toddler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

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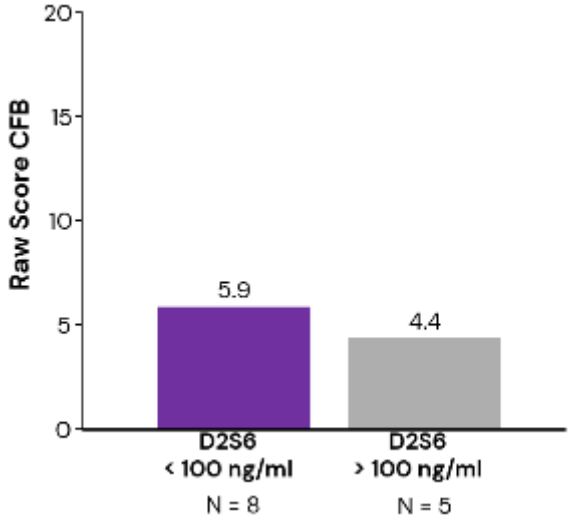
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# RGX-121 data demonstrate correlation between measured CSF HS D2S6 level at Week 16 and cognitive outcomes at 1 year

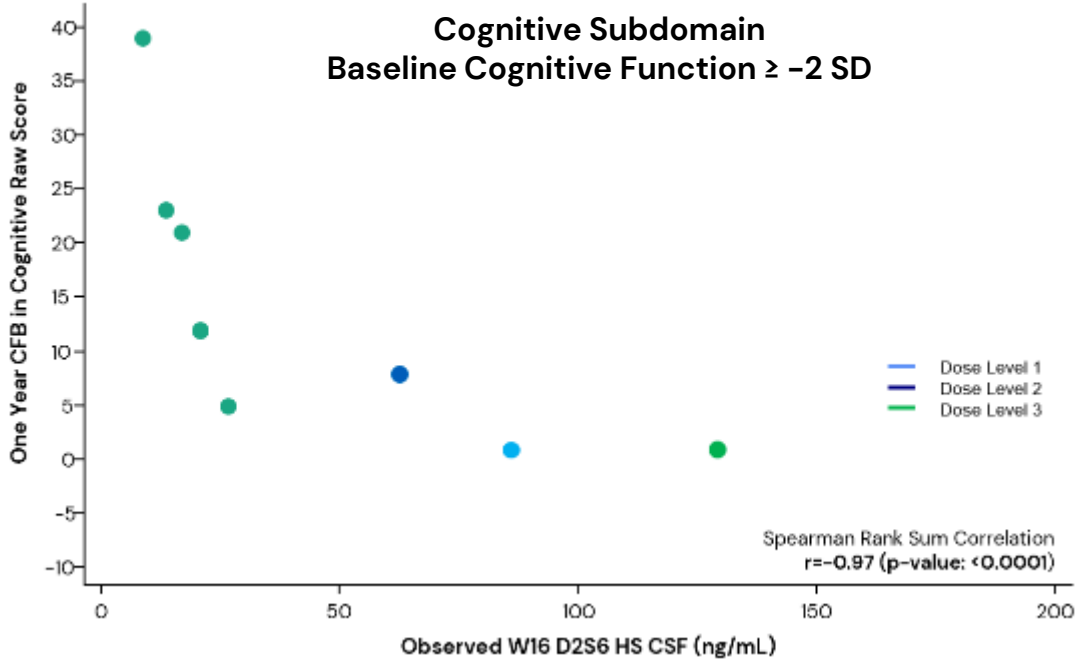
**Baseline BSID-III Cognitive Function ≥ -2SD**




**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

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# 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



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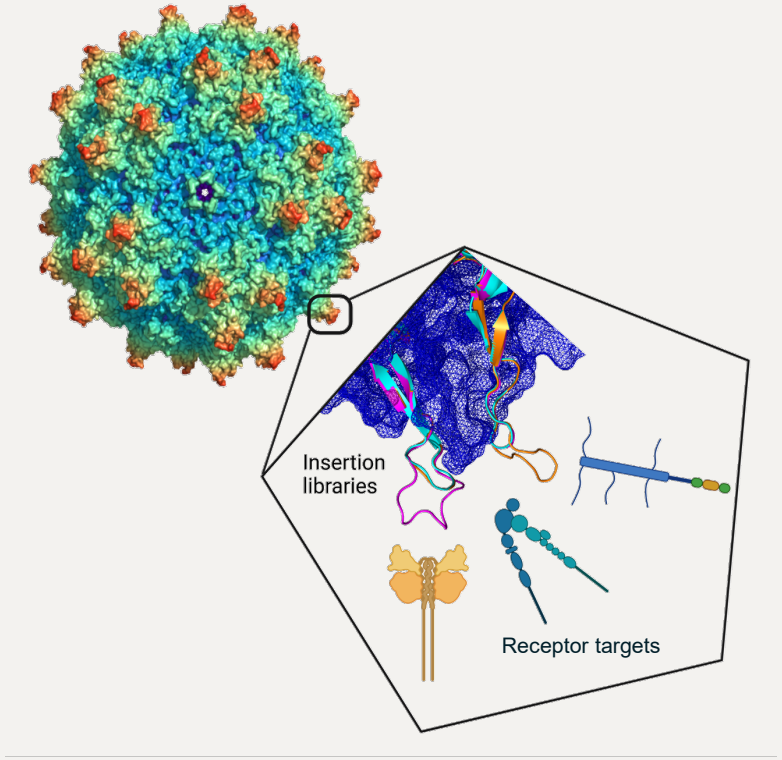
# Discovering the next wave of gene therapies

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


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# Seeking to improve lives through the curative potential of gene therapy

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