AFFINITY DUCHENNE®
Pivotal Trial of RGX-202

Topline Data

May 14, 2026

Forward-looking statements

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Agenda

- **Welcome**
- **AFFINITY DUCHENNE® Pivotal Trial**
  - RGX-202 therapeutic approach
  - Topline Data
- **Q&A**

Curran Simpson
President and CEO
REGENXBIO Inc.

Steve Pakola, M.D.
Chief Medical Officer
REGENXBIO Inc.

Aravindhan Veerapandiyan, M.D.
Arkansas Children's Hospital
AFFINITY DUCHENNE® Principal Investigator

Carolina Tesi-Rocha, M.D.
Stanford University
AFFINITY DUCHENNE® Principal Investigator

Diana Castro, M.D.
Founder and Director Neurology and Neuromuscular Care Center and Founder and Director Neurology Rare Disease Center

REGENXBIO Q1 2026 Highlights

Advancing a late-stage pipeline of potential first- and best-in-class gene therapies

RGX-202

Designed for improved outcomes in Duchenne

- Positive pivotal topline results: primary endpoint met, meaningful interim functional improvements at one year (n=9), favorable interim safety profile
- Over 20 patients enrolled in the ongoing confirmatory trial (n=30)
- Data supports regulatory filing strategy for potential accelerated approval in 2027

Surabgene lomparvovec

(sura-vec, ABBV-RGX-314)

Potential 1st gene therapy for chronic retinal disease

- Sites activated in Phase IIb/III study for diabetic retinopathy (DR); first patient dosed expected Q2 2026
- REGENXBIO will receive a $100 million payment from AbbVie upon first patient dosed in the Phase IIb
- Topline data with wet AMD pivotal trials (ATMOSPHERE® and ASCENT®) expected in Q4 2026

RGX-121

(clemidsogene lanparvovec)

Only potential gene therapy for MPS II

- Partial clinical hold lifted
- REGENXBIO met with FDA leadership and filed an appeal of the CRL

Cash, cash equivalents and marketable securities were $150.5 million as of March 31, 2026.
$100 million diabetic retinopathy milestone expected upon first patient dosed.

RGX-202 Therapeutic Approach

RGX-202: A differentiated, potential best-in-class gene therapy for Duchenne muscular dystrophy

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Boys with Duchenne Continue to Face High Unmet Need

Current options are limited by variable efficacy, safety concerns, and restricted access

THE CHALLENGE
Progressive, degenerative disease

Devastating disease

Caused by mutations in the DMD gene that prevent production of dystrophin, leading to progressive muscle degeneration

Persistent decline

Boys gain functional skills until ~6 years, then experience loss of ambulation and rapid decline.*

GROWING POPULATION

- Affects 1 in 3,500 to 5,000 male births worldwide*
- Estimated US population = ~15,000**
- Significant ongoing need for a gene therapy to serve prevalent and incident markets with the potential to deliver a favorable safety and efficacy profile

Child has not participated in clinical trials of RGX-202

OUR VISION for RGX-202

Deliver a one-time microdystrophin gene therapy designed for greater safety and efficacy potential, and durable benefit to improve lives.

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* Koeks et al 2017
**Crisafulli et al 2020

RGX-202: A Differentiated Therapeutic Approach

Designed to maximize the potential for durable benefit

NOVEL CONSTRUCT

- NAV® AAV8 vector
- Muscle-specific promoter
- Only microdystrophin construct encoding C-Terminal domain, shown to protect and preserve muscle in preclinical studies

IMMUNE SUPPRESSION

- Comprehensive, proactive immune suppression regimen
- Implemented at start of the program and has remained unchanged

IN-HOUSE MANUFACTURING

- Leading purity levels (>80% full capsids) in Duchenne gene therapy enables maximum therapeutic dose (2x10)[14] with lower capsid load
- FDA-inspected, commercial-ready facility

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AFFINITY DUCHENNE® Pivotal Trial

RGX-202: A differentiated, potential best-in-class gene therapy for Duchenne muscular dystrophy

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Positive Topline 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

AFFINITY DUCHENNE® Pivotal Study

Key Eligibility Criteria

- **Ambulatory boys aged ≥1 year** at screening
- **Genetically confirmed DMD:** except those with deletions or point mutations in exons 8, 9, and/or 10
- **No pre-existing antibodies** to the gene therapy (AAV8 capsid)

Pivotal Dose: $2x10^{14}$ GC/kg

1 to <4 years

- 10-meter walk without assistance
- Stable dose on or off corticosteroids for prior 12 weeks
- Weight >10 kg
- Perform supine to stand without assistance

≥4 years

- 100-meter walk without assistance
- Stable dose of corticosteroids for prior 12 weeks
- NSAA ≥ 16
- Time to stand ≥3 and <7 seconds

PROACTIVE IMMUNE SUPPRESSION REGIMEN

Pivotal Trial Endpoints

- **Primary Endpoint:**
  Proportion of patients with > 10% RGX-202 microdystrophin expression at Week 12
- **Secondary Endpoints:**
  - **≥4 years:**
    Velocity and time of Timed Function Tests; NSAA; Safety
  - **1 to <4 years:**
    PDMS-3 & SV95C for 1 to <4 years; Safety
- **Exploratory Endpoints:**
  - **≥4 years:** SV95C & MRIs

TTStand: Time to Stand; 10MWR: 10M Walk Run; TTClimb: Time to Climb; NSAA: North Star Ambulatory Assessment PDMS-3: Peabody Developmental Motor Scale, Third Edition; SV95C: Stride velocity 95th centile; MRI: Magnetic resonance imaging

Key Baseline Demographics

Treated patients: Aged 1-3 (N=11), Aged 4-7 (N=9), Aged 8+ (N=11)

	AGE RANGE AT SCREENING (NUMBER DOSED)		
	1 to <4 (N = 11)	≥4 (N = 20)	Overall (N = 31)
VARIABLE MEAN (range)			
Age at Dosing (yrs)	**3.3** (1.8-4.1)	**8.4** (5.0-13.4)	**6.6** (1.8-13.4)
Mean age at last assessment (yrs)	**4.0** (2.3-5.2)	**9.4** (5.5-14.1)	**7.5** (2.3-14.1)
Time from Dosing (months)	**10.7** (4.1-19.3)	**12.6** (4.4-24.3)	**11.9** (4.1-24.3)
Weight (kg) **at dosing**	**14.6** (10.3-17.9)	**26.6** (17.3 – 47.7)	**22.4** (10.3 – 47.7)
BMI (kg/m2)	**17.7** (14.5-20.6)	**18.8** (14.6-26.6)	**18.4** (14.5-26.6)
BASELINE FUNCTION			
NSAA	n/a†	**24.2** (17.0-30.0)	**24.2** (17.0-30.0)
Time to Stand (sec)	n/a†	**4.4** (2.9-6.2)	**4.4** (2.9-6.2)
10 Meter Walk Run (sec)	n/a†	**5.0** (3.7-7.0)	**5.0** (3.7-7.0)
Time to Climb (sec)	n/a†	**3.2** (1.6-5.3)	**3.2** (1.6-5.3)

Data cut date: April 16, 2026

† Boys 1-3 years old complete the Peabody Developmental Motor Scale, Third Edition (PDMS-3) at baseline; NSAA collected but not applicable Pakola (2025) World Muscle Society, Vienna. Austria

GC: Genome copies; BMI: Body mass index; NSAA: North Star Ambulatory Assessment

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Primary Endpoint

Proportion of participants with microdystrophin expression >10% at Week 12

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Primary Endpoint Met with High Statistical Significance

Primary endpoint to support accelerated approval: proportion of participants with microdystrophin expression >10% at Week 12

- 28 of 30* participants (**93%**) achieved >10% microdystrophin expression (p<0.0001)
  - **80%** of participants achieved **>40% microdystrophin expression**
- **71.1%** average microdystrophin expression among all participants
  - **41.6%** average microdystrophin expression in participants aged 8+; highest expression reported in older age group across gene therapy programs

Robust expression achieved across ages, supporting potential for improved functional outcomes

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Data cut date: April 16, 2026

Microdystrophin expression adjusted for muscle content; % normal control

*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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Topline Interim Safety Results

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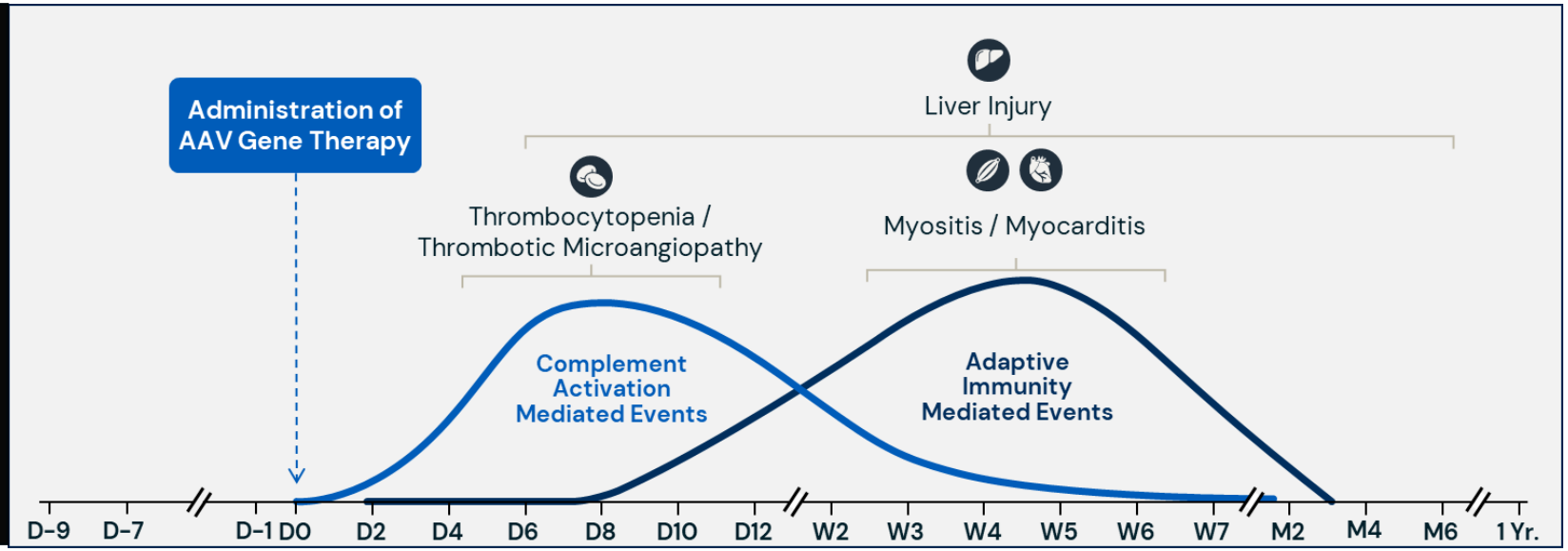
Proactive Immune Suppression Regimen Designed to Mitigate AEs Observed in Other Duchenne Gene Therapy Programs

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

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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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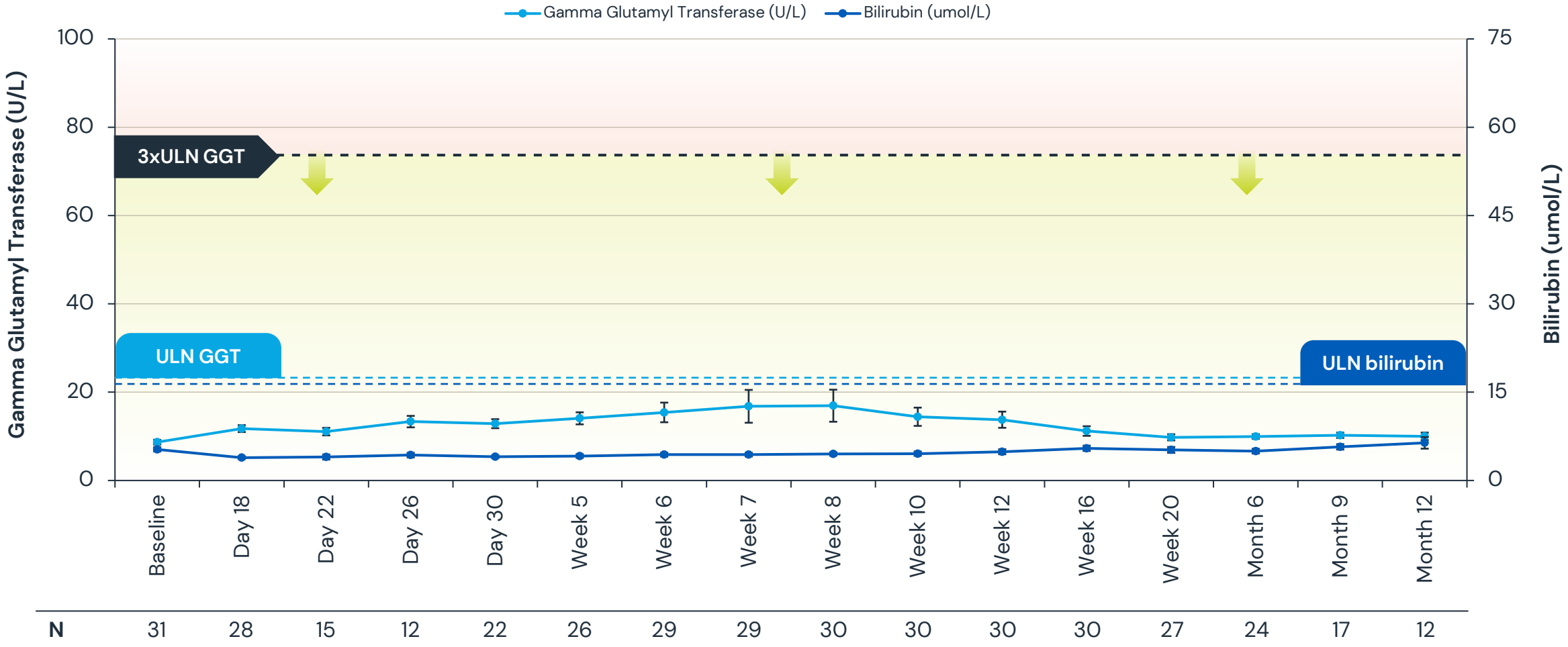
RGX-202 Demonstrates Positive, Differentiated Liver Safety Profile

Mean GGT and total bilirubin not exceeding ULN up to 12 months



Data cut date: April 16, 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

Topline Interim Functional Results

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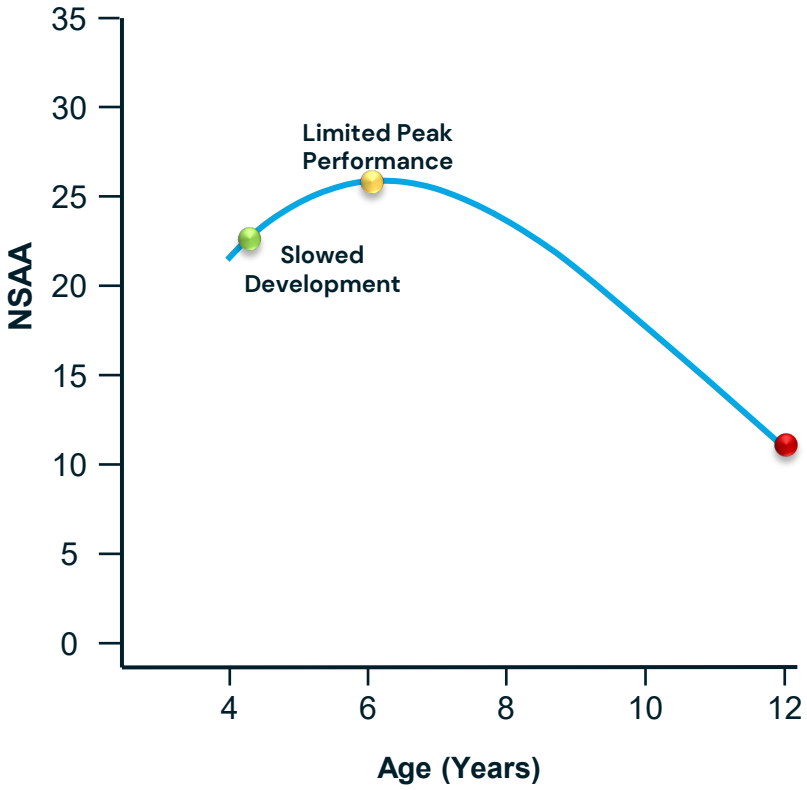
AFFINITY DUCHENNE® External Control Methodology



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)

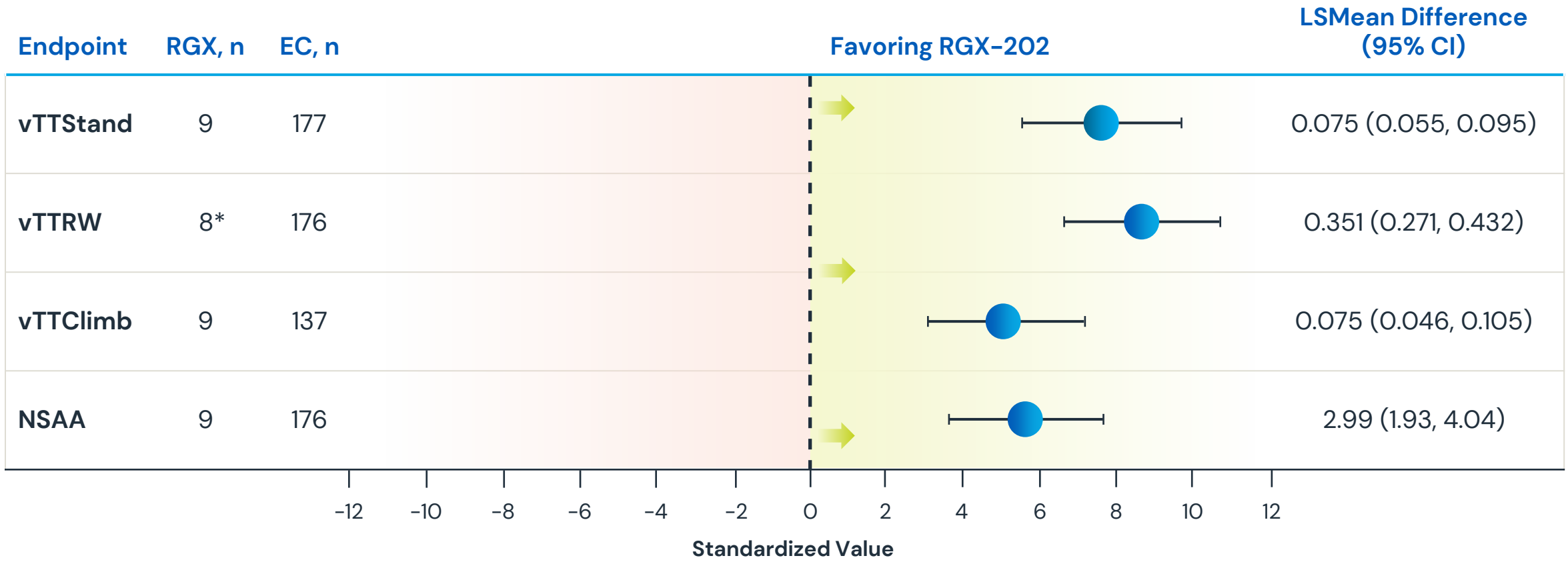
[1] Age and functional outcomes criteria based on N=9 participants who received Month 12 as of the April 16, 2026 data cut.

[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.

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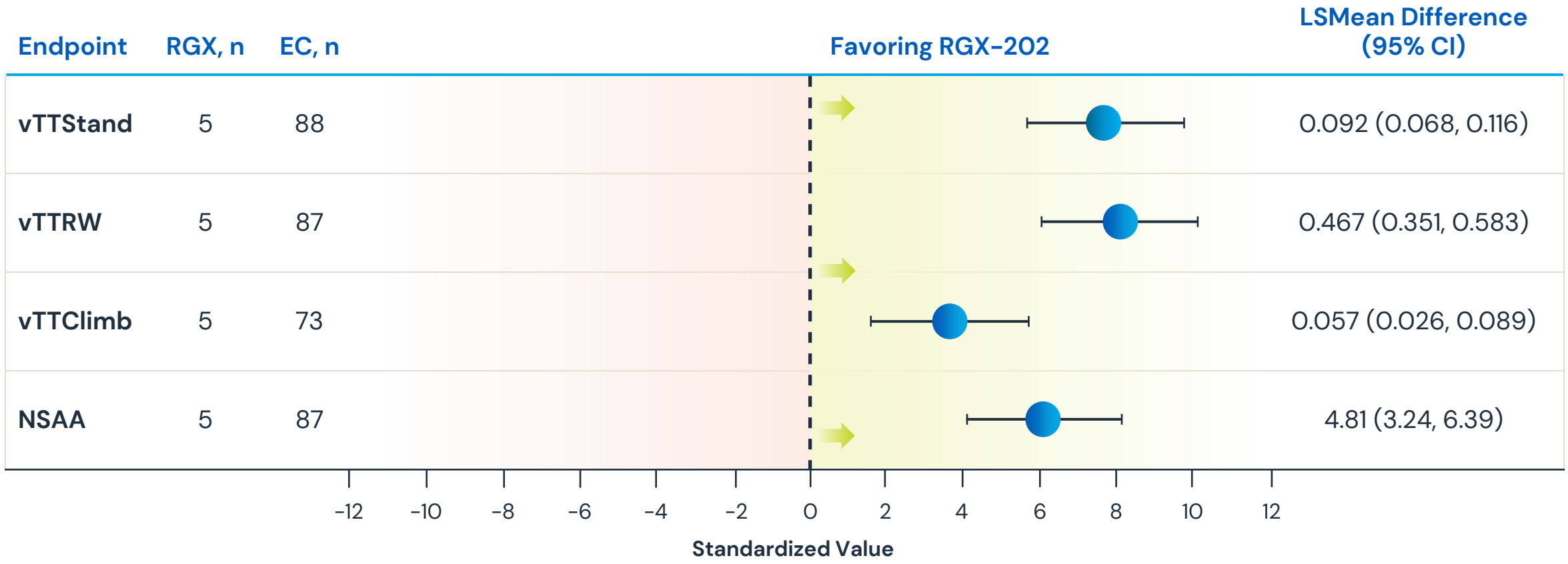
Participants Aged 8+ (N=5) Exceeded External Controls on All Functional Measures at 1 Year

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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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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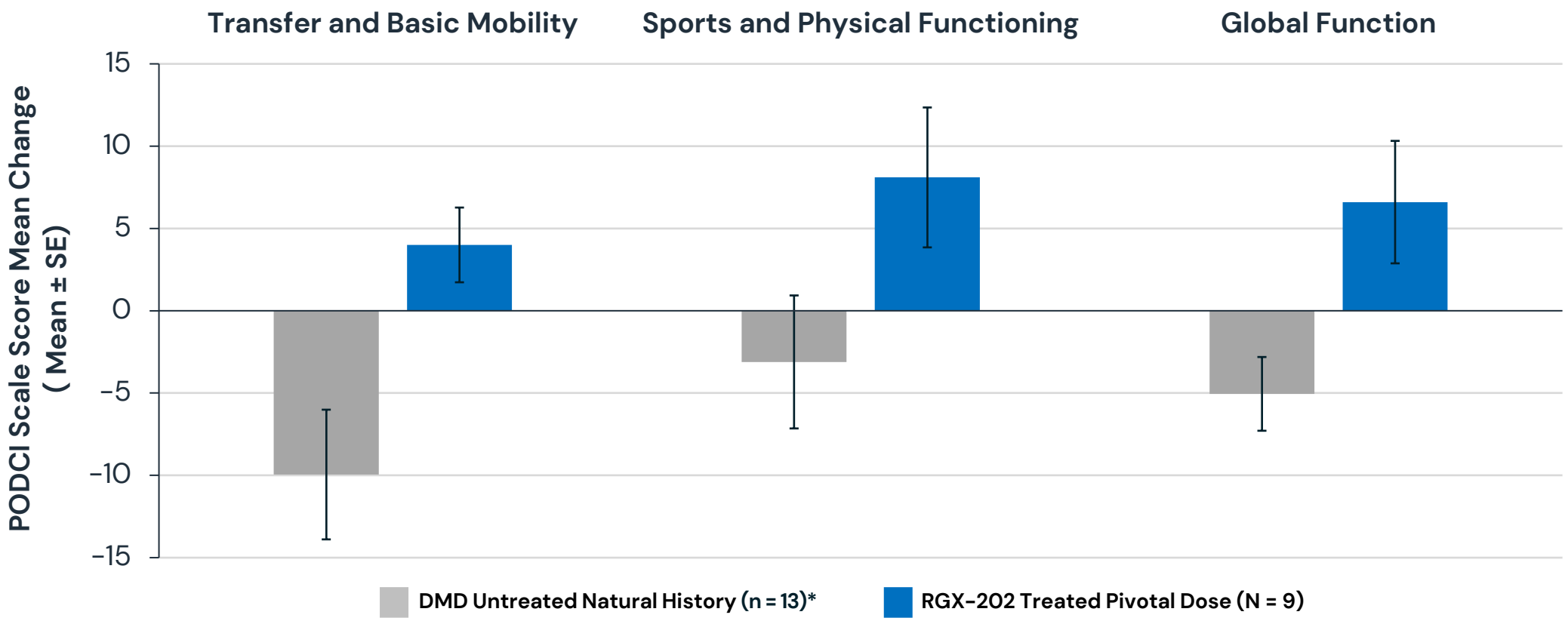
Caregivers Reported Improved Function at 12 Months



Caregivers reported improved function in the home and community as measured by key dimensions of the PODCI at 12 months

Date cut date: April 16, 2026

PODCI: Pediatric Outcomes Data Collection Instrument

Mean change from baseline

PODCI Scale Scores range from 0-100, with 100 being highest function

*Henricson (2013) PLoS: DMD Untreated

PODCI subtests selected due to ability to differentiate between ambulatory boys with DMD and controls and sensitive to one-year changes (Henricson, 2013)

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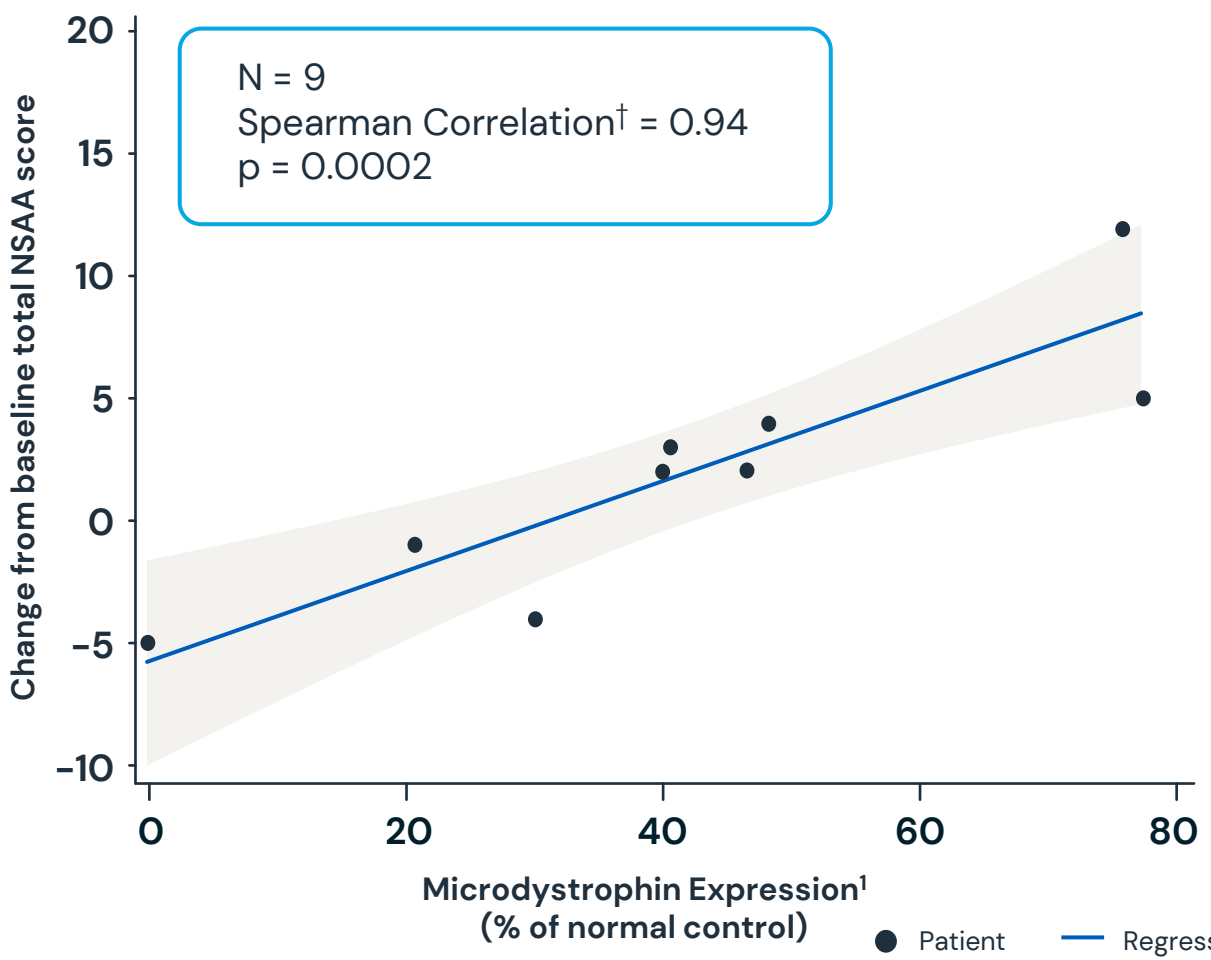
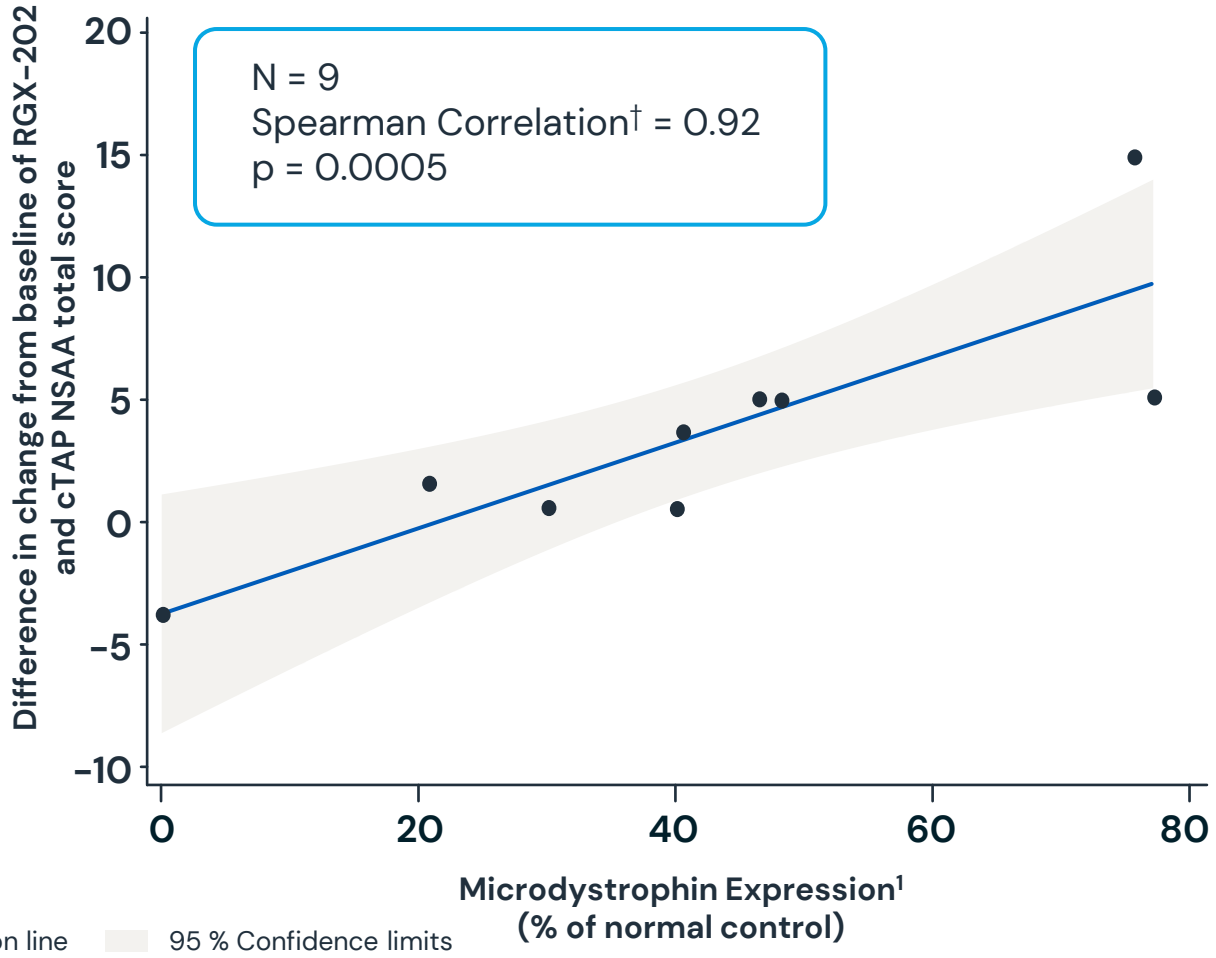
RGX-202 Microdystrophin Expression Correlated with Functional Improvement

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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Discussion

Steve Pakola, M.D.
Chief Medical Officer
REGENXBIO Inc.

Aravindhan Veerapandiyan, M.D.
Arkansas Children's Hospital
AFFINITY DUCHENNE® Principal Investigator

Carolina Tesi Rocha, M.D.
Stanford University
AFFINITY DUCHENNE® Investigator

Diana Castro, M.D.
Founder and Director Neurology and Neuromuscular Care Center and Founder and Director Neurology Rare Disease Center

AFFINITY DUCHENNE® Pivotal Study of RGX-202

Topline Interim Results Summary

- **Primary endpoint met with high statistical significance (p<0.0001)**
  - **28 of 30* participants (93%)** achieved microdystrophin expression above 10%
  - Robust microdystrophin expression **averaged 71.1% across all participants**, and **41.6% in older boys** (aged 8+)
  - **80%** of participants achieved **>40% microdystrophin expression**

- **Interim safety:**
  - RGX-202 was well-tolerated and continued to demonstrate a favorable, differentiated safety profile

- **Interim functional data at 1 year:**
  - Participants **exceeded expected disease trajectory on NSAA and all timed function tests**, including older boys (aged 8+)

- **Strong statistically significant correlation between RGX-202 microdystrophin expression and functional improvement** (NSAA, n=9), a landmark distinction in Duchenne gene therapy

RGX-202 demonstrates an encouraging safety profile and evidence of positive functional outcomes, supporting potential FDA approval via accelerated approval pathway in 2027

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Data cut date: April 16, 2026

NSAA: North Star Ambulatory Assessment

*30 of 31 total participants have Week 12 biopsy available for evaluation; one participant refused muscle biopsy at Week 12

THANK YOU

to the boys, families,
clinicians, and study site staff
who participated in our study

REGENXBIO®