Delivering the promise of gene therapy

REGENXBIO Corporate Presentation

November 2026

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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

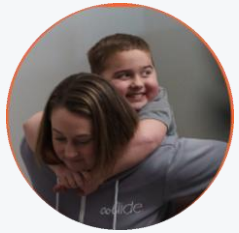
FDA

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

AMBULATORY PATIENT POPULATION

~6K–8K [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

Patients have not participated in a REGENXBIO clinical trial

1. Crisafulli et al., 2020; US Census Bureau, Landfeldt et al., 2014; 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				–

**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

	2026	2027
	Recent & anticipated milestones	
RGX-202 Duchenne Muscular Dystrophy	✓ Topline pivotal data (2Q) ✓ Complete confirmatory enrollment	○ U.S. BLA submission (1Q) ○ Initiate AFFINITY® RISE[1] (1H) ○ Potential FDA approval (2H)
Sura-vec Wet AMD	✓ 5-year Ph I/II follow up data (July) ○ Topline pivotal data (4Q)	○ Global regulatory submissions (2027)[2]
Sura-vec Diabetic Retinopathy	✓ Ph IIb/III first patient dosed (1H) ✓ 3-year Ph II follow-up data (Sept)	
RGX-121 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

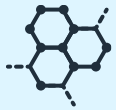
Duchenne child has not participated in a REGENXBIO clinical trial
Pivotal topline interim data cut date: April 16, 2026

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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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Pivotal topline interim data cut date: April 16, 2026

[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)

Pivotal topline interim 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.

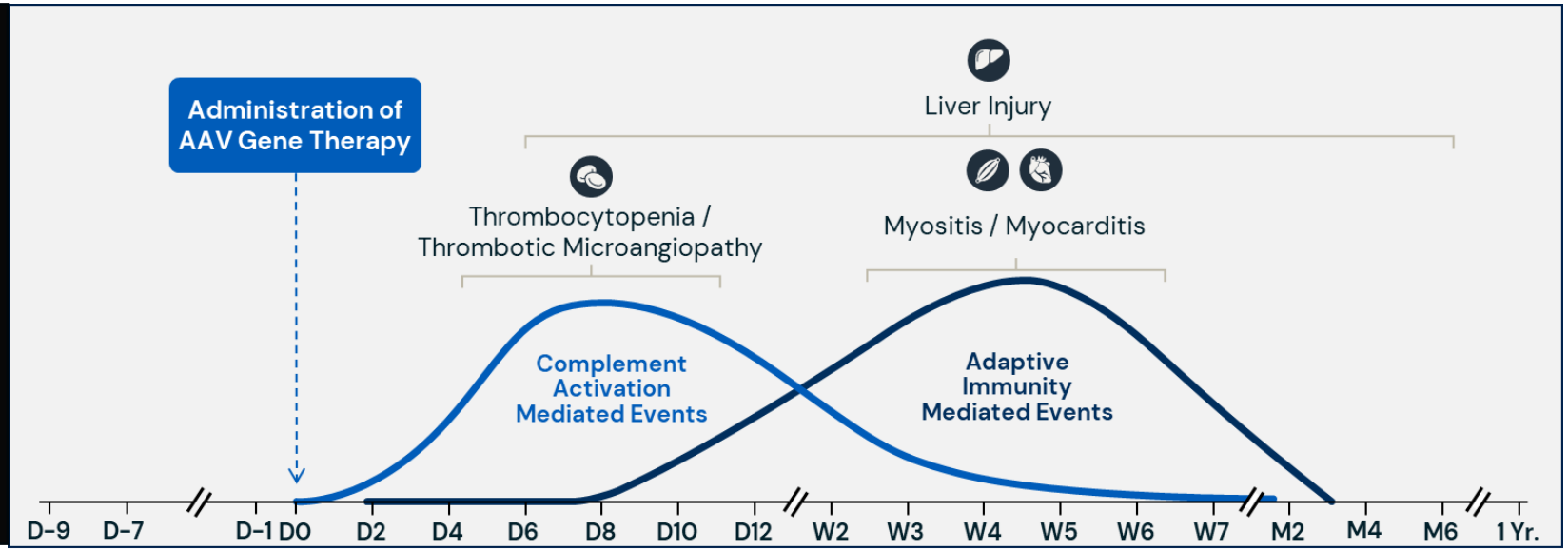
SD: Standard deviation; VCN: Vector copy number; qPCR: Quantitative polymerase chain reaction; GC: Genome copies

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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**

	Aged <4 Years N = 11, n (%)	Aged ≥4 Years N = 20, n (%)	Overall N = 31, n (%)
TR-SAEs			
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.

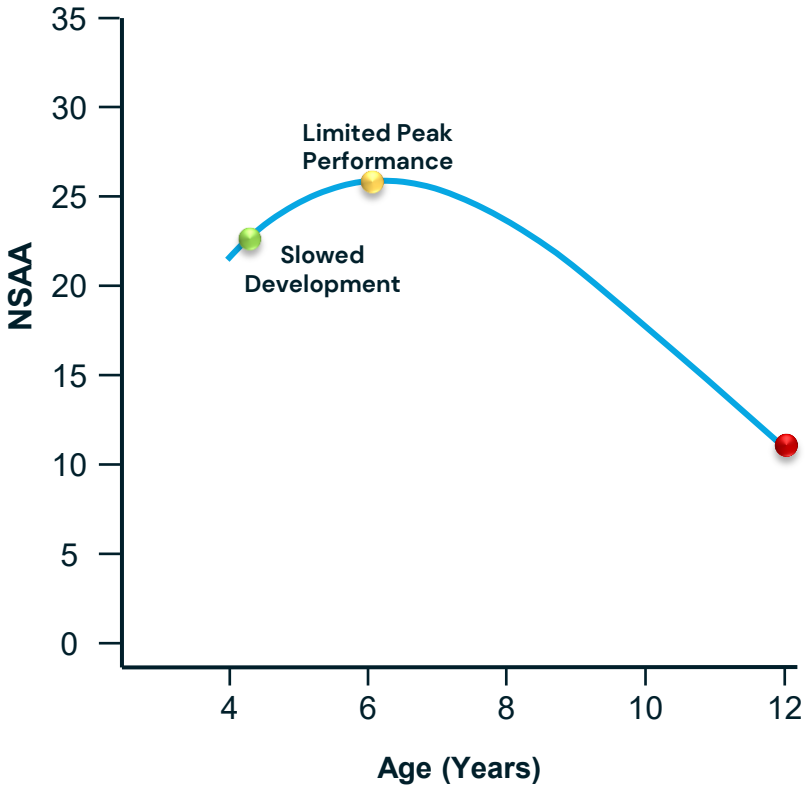
No drug-related thrombocytopenia, myositis, or neurotoxicity reported

Pivotal topline interim 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.

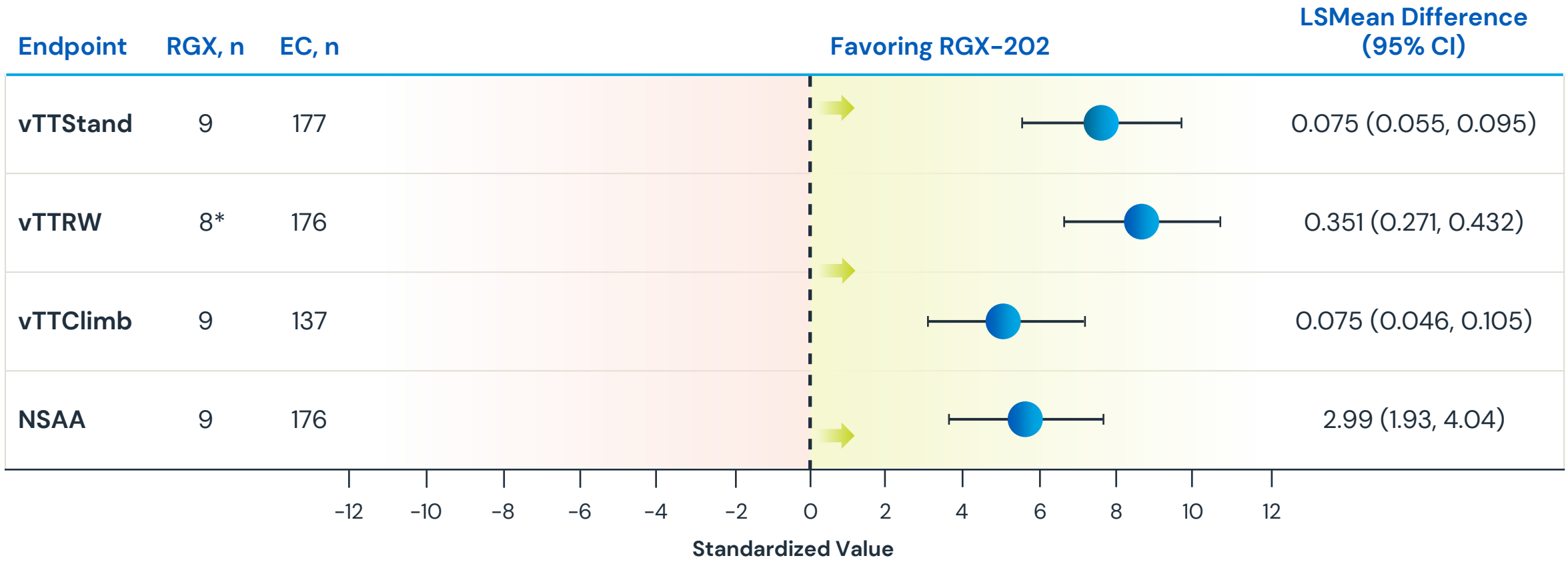
[2] Muntoni F, Signorovitch J, Goemans N, Manzur AY, Done N, Sajeev G, Li J, Akbarnejad H, Sharma A, Ward SJ, Niks EH, Servais L, Mercuri E, Guglieri M, Straub V, de Groot I, Ridout D; PRO-DMD-01 study investigators; Association Française contre les Myopathies; NorthStar Clinical Network; McDonald C.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



Pivotal topline interim 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.

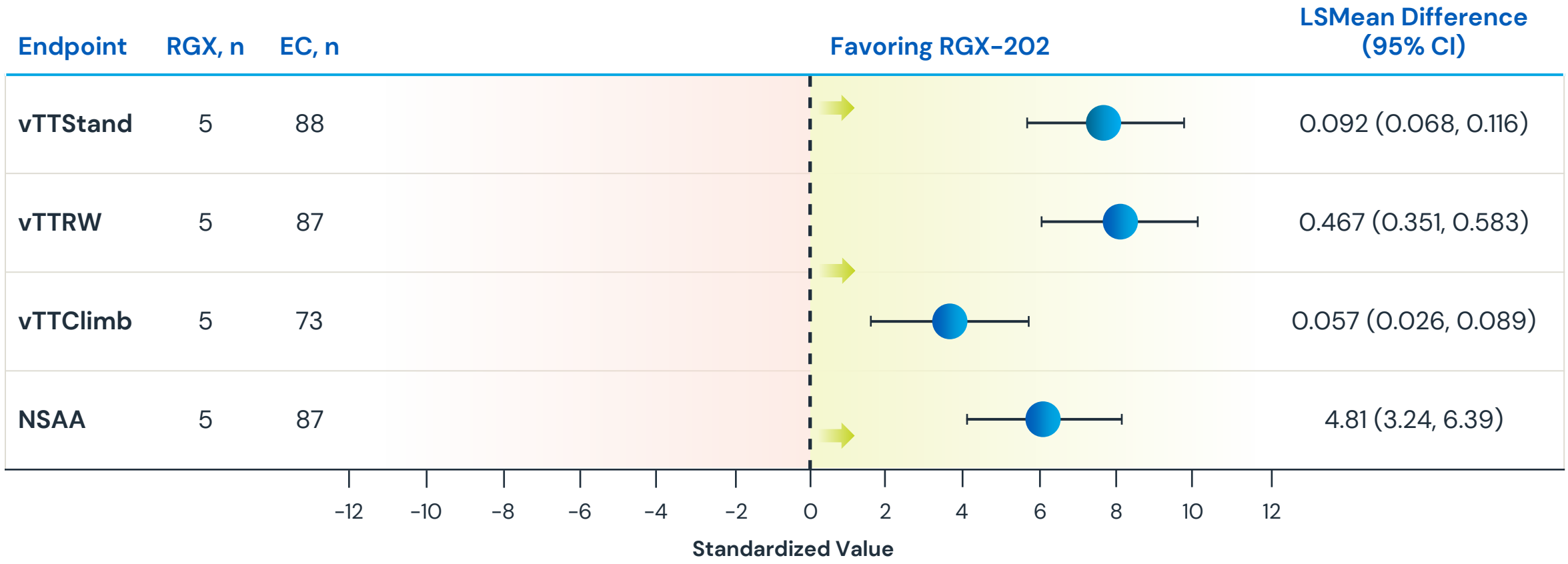
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Participants aged 8+ (N=5) exceeded external controls on all functional measures at 1 year

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)



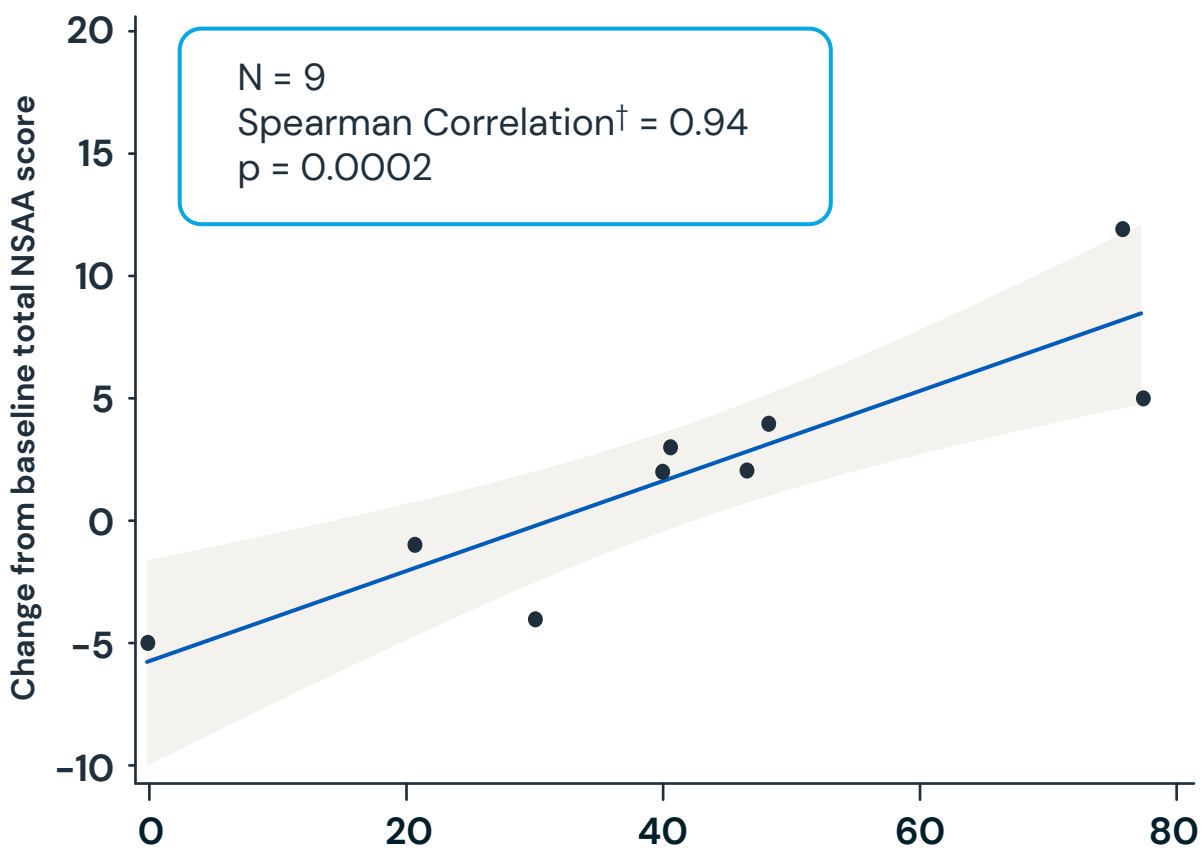
Pivotal topline interim data cut date: April 16, 2026
V: velocity; TTStand: Time to Stand; TTRW: Time to Run and Walk; TTClimb: Time to Climb; NSAA: North Star Ambulatory Assessment; EC: External controls
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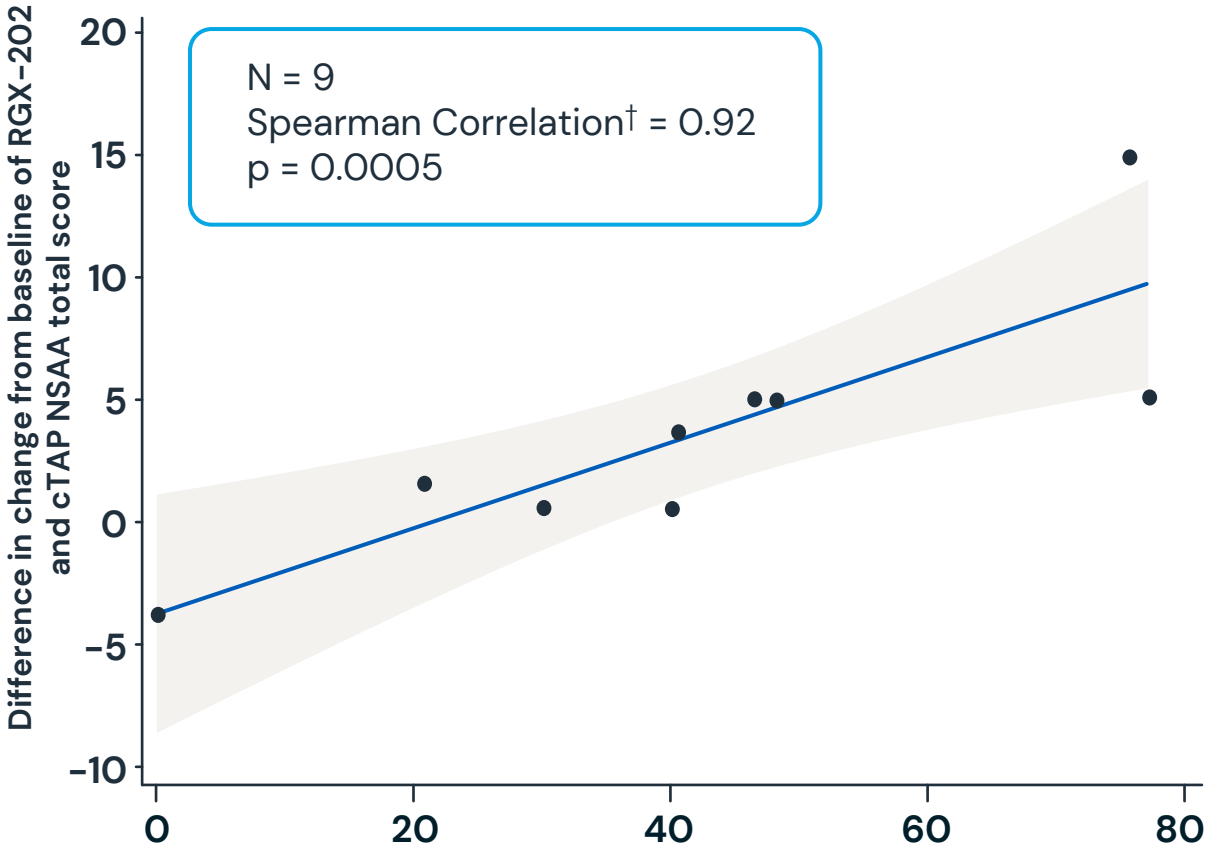
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RGX-202 microdystrophin expression correlated with functional improvement

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





Patient — Regression line — 95 % Confidence limits

Pivotal topline interim data cut date: April 16, 2026

NSAA: North Star Ambulatory Assessment

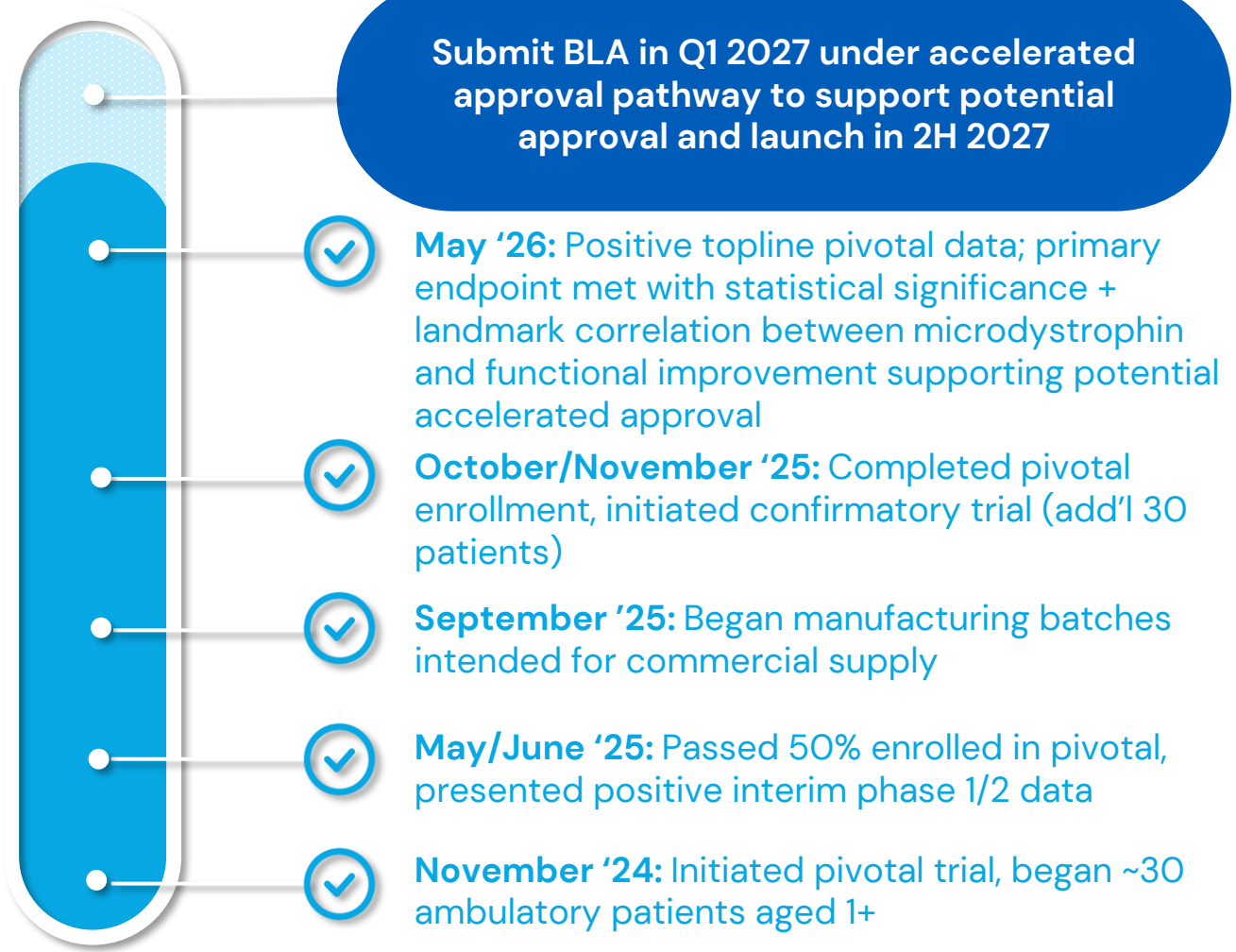
†Additionally, regression model was used to visualize the linear relationship between microdystrophin expression and change from baseline in NSAA function. To support linear relationship of figure results, Pearson correlation coefficient was also calculated (r=0.88[p= 0.0016] for change from baseline,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



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)



- 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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Sura-vec is positioned to transform wet AMD care

Five pillars of clinical and commercial success

1

Transform the treatment paradigm

First-in-class position to move beyond chronic injections to a foundational, one-time gene therapy

2

Address the Spectrum of Unmet Need

Clinical development includes patients across a broad range of disease activity, including those with the highest treatment burden

3

Differentiation by Design

Combines sustained anti-VEGF expression with targeted delivery to support potential for durable disease control and a favorable tolerability profile

4

Clinical Evidence Built for Global Success

Comprehensive clinical program designed to support regulatory success and broad commercial adoption

5

Commercialization Built on an Established Foundation

Built for integration into existing retinal practices, with the global commercial strength to support broad patient access

Subretinal sura-vec brings together the clinical profile, breadth of evidence and commercial infrastructure needed to establish a potential new treatment paradigm in wet AMD

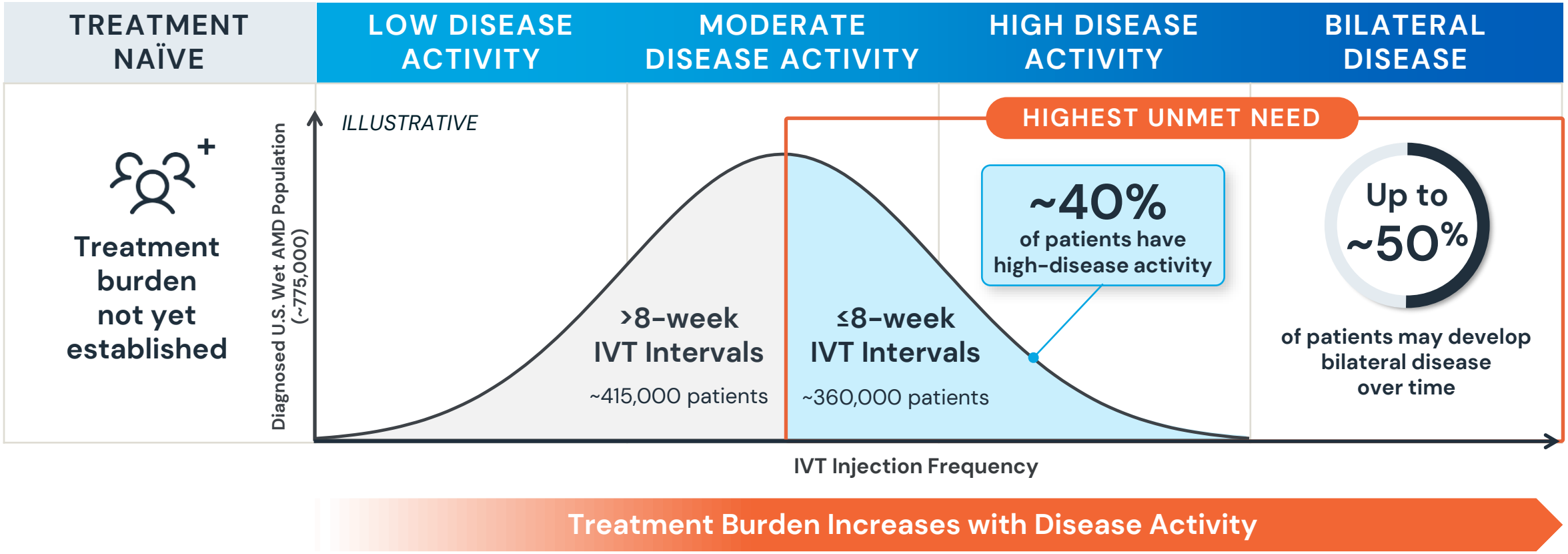
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Sura-vec is investigational and has not been approved by any regulatory authority.

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The problem: wet AMD patients span a spectrum of disease activity and associated treatment burden

Patients with high disease activity and bilateral disease represent highest unmet need



US Census Bureau, 2024 Clarivate DRG AMD Report; 2024 Vestrum EMR w AMD Analysis; High treatment burden defined as ≤ 8-week injection intervals; Joachim N, et al. "Five-year progression of unilateral age-related macular degeneration to bilateral involvement: the Three Continent AMD Consortium report." *British Journal of Ophthalmology*. 2017;101:1185–1192.

A patient can develop bilateral disease at any point after diagnosis

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Sura-vec approach is differentiated

Clinical development includes patients across a broad range of disease activity, including those with the high disease activity and bilateral disease

		SURA-VEC	IVT Gene Therapy	TKIs
Modality		AAV8, subretinal	Modified AAV2, intravitreal	Intravitreal
Stage		Phase 3	Phase 3	Phase 3
Dosing Frequency		One-time administration	One-time administration	Recurring dosing
Pivotal Program	**Steroids**	None beyond routine vitrectomy care	20+ week taper	None
	Sample Size	n=1,300+ subjects across 2 studies	Varies	Varies
	Comparator	Both SoC comparators (Q4w ranibizumab & Q8w aflibercept)	Q8w aflibercept	Single dose aflibercept or Q8w aflibercept
	Population	Treatment experienced (up to 4 years) including patients with high disease activity	Treatment naïve and experienced	Treatment naïve and experienced
Bilateral Evidence		Confirmed with 1 year data	In development	n/a
Long-Term Evidence		Up to 5 years	2 to 5 years	1 year

Sources: company presentations (4DMT corporate deck 2026; Ocular Investor Day, June 17, 2026, EyePoint Investor Presentation April 2026); Wykoff CC. Ixo-vec Gene Therapy for nAMD: Clinical Progress from the LUNA Phase 2 Trial, FLORetina Dec. 2025, No head-to-head data.

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Sura-vec in wet AMD: A comprehensive, global clinical program

Clinical development includes patients across a broad range of disease activity, including those with the high disease activity and bilateral disease

1,400+
Patients enrolled

200+
Clinical sites across 10+ countries

Up to
5 years
of reported clinical follow up

500+
Surgeons trained

Broad Populations
Including patients with high disease activity

Bilateral Treatment
Clinical effect treating the second eye

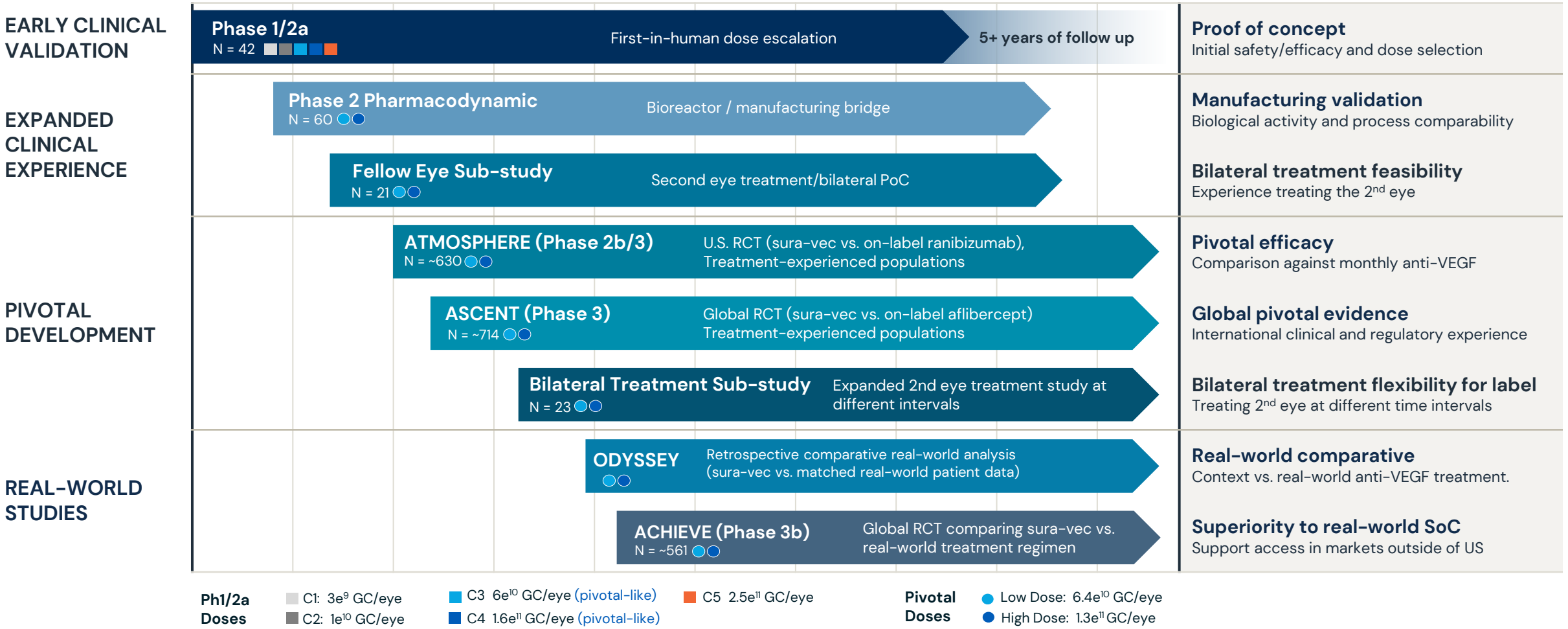
Real-World Comparisons
Comparison studies against real-world anti-VEGF

1. REGENXBIO press release, October 6, 2025. ATMOSPHERE and ASCENT enrolled more than 1,200 participants across more than 200 global clinical sites.
2. AbbVie/REGENXBIO clinical development and surgical training records. Enrollment and surgeon-training totals subject to confirmation.
3. Vajzovic L, et al. EURETINA; Vienna, Austria; Oct 2, 2026.
4. Pitcher JD. Retina Society; Los Angeles, CA; Sep 26, 2026.
5. ATMOSPHERE (NCT04704921) and ASCENT (NCT05407636) protocols;
6. ODYSSEY Phase 2 Pharmacodynamic Study, ASRS 2026.
Total patients enrolled 1400+ based on Ph1/2a (n=42) + Phase 2 Pharmacodynamic (n=60) + Fellow Eye Sub-study (n=21) + ATMOSPHERE (n=~630) + ASCENT (n=~714)

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Sura-vec wet AMD global clinical program overview

Clinical strategy supports global regulatory, payer, and commercial needs



Illustrative study timing
RCT: Randomized control trial, PoC: Proof-of-concept, SoC: Standard of care

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Consistent clinical outcomes across multiple studies

Including patients with high disease activity and bilateral disease

	Phase 1/2a (Cohorts 3 and 4) N = 18	Phase 2 PHARMACODYNAMIC (BIOREACTOR) N = 60	FELLOW EYE SUB-STUDY N = 11
Stable to improved vision	✓	✓	✓
Stable to improved anatomy	✓	✓	✓
Reduced treatment burden	✓	✓	✓

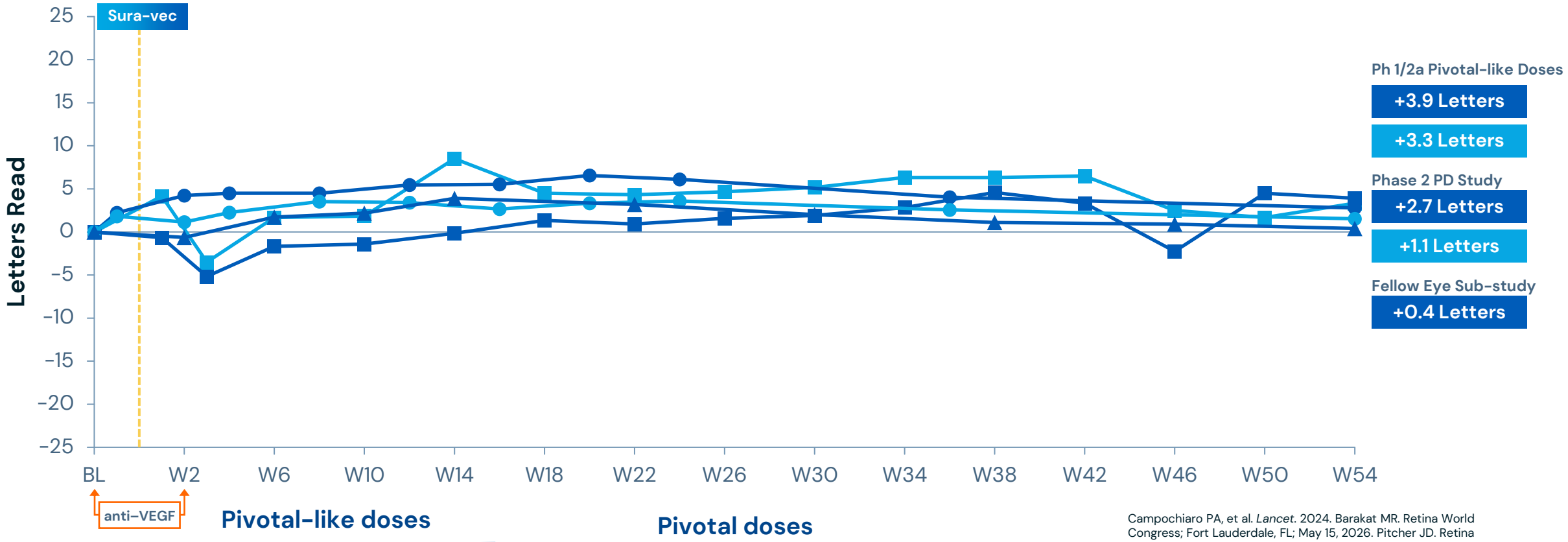
Campochiaro PA, et al. *Lancet*. 2024. Barakat MR. Retina World Congress; Fort Lauderdale, FL; May 15, 2026. Pitcher JD. Retina Society; Los Angeles, CA; Sep 26, 2026.

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Vision

Stable to improved across studies

BCVA ACROSS STUDIES (BASELINE TO 1 YEAR)



Pivotal-like doses

- Ph 1/2a, C3 ($6e^{10}$ GC/eye) (n=6)
- Ph 1/2a, C4 ($1.6e^{11}$ GC/eye) (n=12)

Pivotal doses

- Ph2 PD, Low Dose ($6.4e^{10}$ GC/eye) (n=30)
- Ph2 PD, High Dose ($1.3e^{11}$ GC/eye) (n=30[a])
- Fellow Eye Sub-study, High Dose ($1.3e^{11}$ GC/eye) (n=10[b])

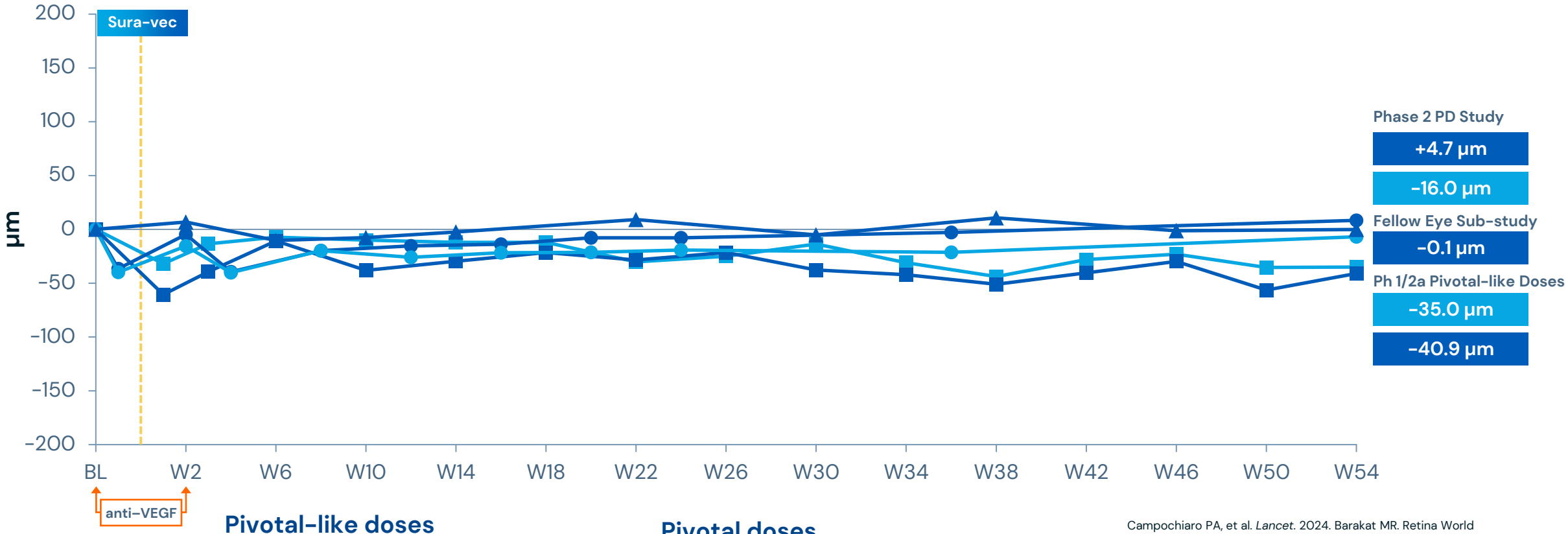
Campochiaro PA, et al. *Lancet*. 2024. Barakat MR. Retina World Congress; Fort Lauderdale, FL; May 15, 2026. Pitcher JD. Retina Society; Los Angeles, CA; Sep 26, 2026. BCVA: best-corrected visual acuity; BL: baseline; HD: high dose; Ph: phase; LD: low dose; VEGF: vascular endothelial growth factor. W: week.
a. Two participants in the HD group withdrew from the study (one after their 6mo visit, one after their 3mo visit), so their data are not included in the 1Y change from baseline. b. One participant withdrew after their Month 2 visit and is not included. One additional participant was enrolled and has completed the study.

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Anatomy

Stable to improved across studies

CRT ACROSS STUDIES (BASELINE TO 1 YEAR)



Pivotal-like doses

- Ph 1/2a, C3 ($6e^{10}$ GC/eye) (n=6)
- Ph 1/2a, C4 ($1.6e^{11}$ GC/eye) (n=12)

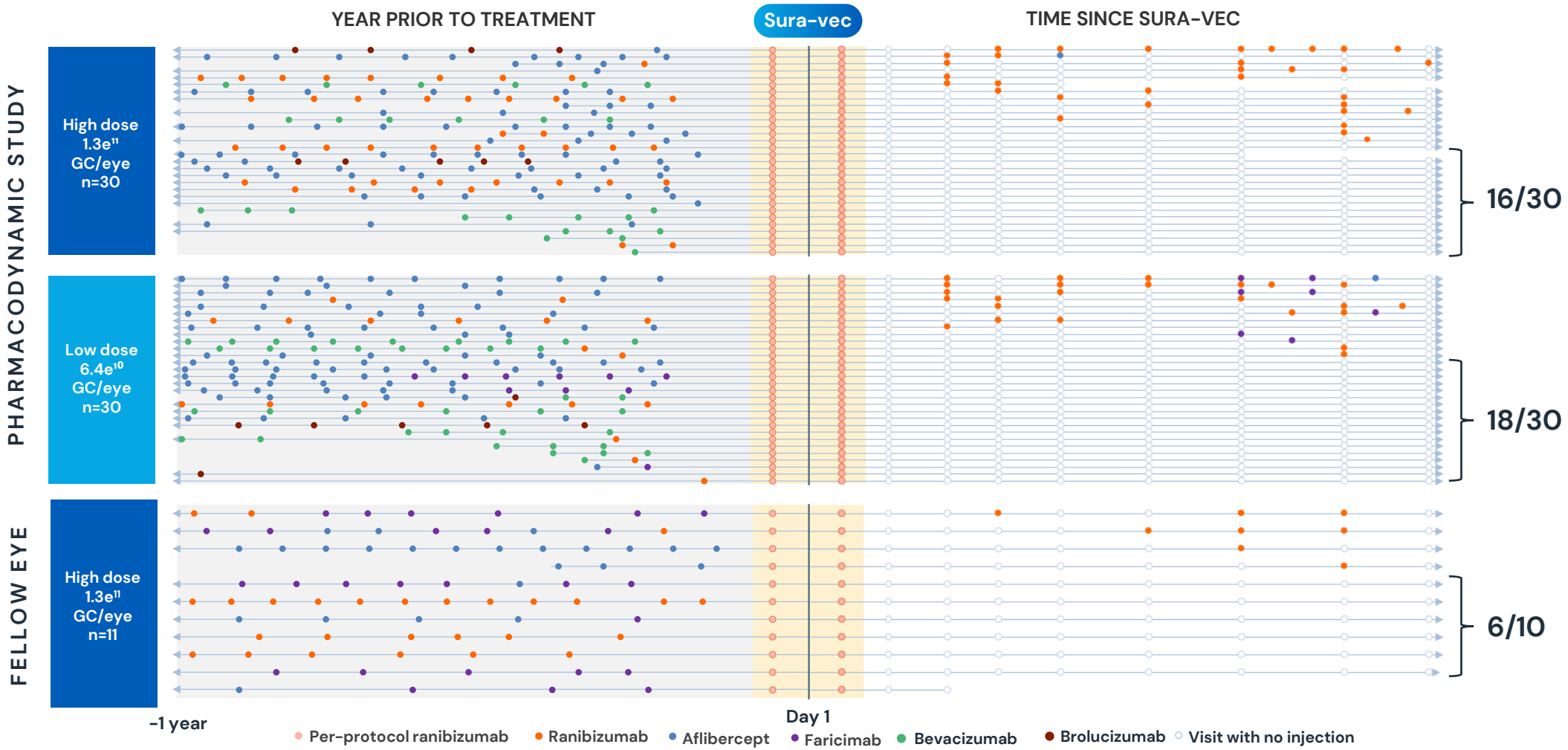
Pivotal doses

- Ph2 PD, Low Dose ($6.4e^{10}$ GC/eye) (n=30)
- Ph2 PD, High Dose ($1.3e^{11}$ GC/eye) (n=30[a])
- Fellow Eye Sub-study, High Dose ($1.3e^{11}$ GC/eye) (n=10[b])

Campochiaro PA, et al. *Lancet*. 2024. Barakat MR. Retina World Congress; Fort Lauderdale, FL; May 15, 2026. Pitcher JD. Retina Society; Los Angeles, CA; Sep 26, 2026. BCVA: best-corrected visual acuity; BL: baseline; HD: high dose; Ph: phase; LD: low dose; VEGF: vascular endothelial growth factor. W: week
a. Two participants in the HD group withdrew from the study (one after their 6mo visit, one after their 3mo visit), so their data are not included in the 1Y change from baseline. b. One participant withdrew after their Month 2 visit and is not included. One additional participant was enrolled and has completed the study.

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Clinically meaningful treatment burden reduction at pivotal doses at 1 year across studies



53% Injection-free Participants

60% Injection-free Participants

60% Injection-free Participants

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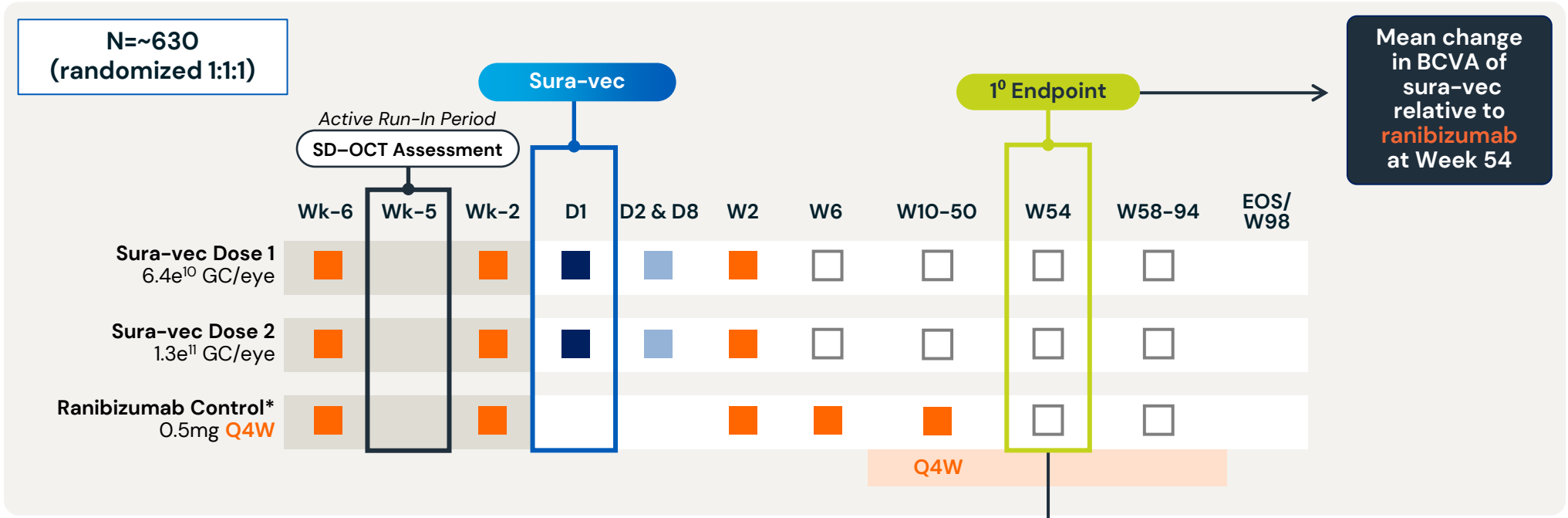
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Campochiaro PA, et al. *Lancet*. 2024. Barakat MR. Retina World Congress; Fort Lauderdale, FL; May 15, 2026. Pitcher JD. Retina Society; Los Angeles, CA; Sep 26, 2026.

Pivotal #1: ATMOSPHERE® study design

Randomized, Controlled, Partially Masked Study

ATMOSPHERE



Inclusion of Broad wAMD Population:

- Treatment experienced and anti-VEGF responsive patients
- No maximum on anti-VEGF injections in prior year
- No maximum CRT thickness screening visit 1
- Documented response to anti-VEGF (1 week after screening injection)

Eligible to treat 2nd eye as part of Bilateral Treatment Sub-study

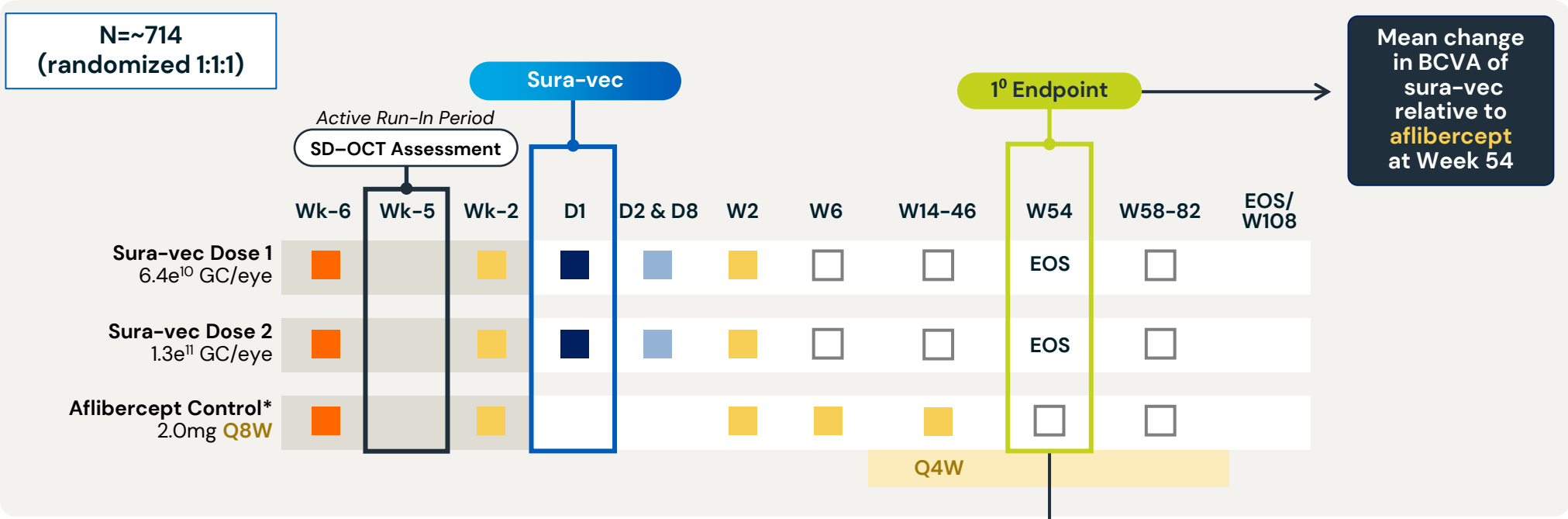
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Pivotal #2: ASCENT® study design

Randomized, Controlled, Partially Masked Study

ASCENT



Inclusion of Broad wAMD Population:

- Treatment experienced and anti-VEGF responsive patients
- No maximum on anti-VEGF injections in prior year
- No maximum CRT thickness screening visit 1
- Documented response to anti-VEGF (1 week after screening injection)

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Potential advantages of sura-vec subretinal delivery

Targeted administration designed to support a favorable efficacy and safety profile without the need for extended steroid prophylaxis



One-Time Targeted Delivery

Compartmentalized

Immune-privileged



Well-tolerated

- No drug-related SAEs across multiple studies at pivotal or pivotal-like doses



No Prophylactic Steroid Burden

- No steroids required beyond routine vitrectomy



Bilateral Treatment Optionality

- Clinical evidence supports treating both eyes

Sura-vec is investigational. Potential benefits reflect delivery rationale and available clinical evidence; no head-to-head comparison is implied.

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Established coding and payment precedents support potential sura-vec commercialization

Subretinal administration reimbursement

DRUG PAYMENT
Product reimbursement

ASP-based, like other retina drugs we use

HCPCS C/J-code
Product specific

Medicare **ASP + 6%**

Commercial **ASP + 8–9%**

◷ Assigned ~4–6 mo. post-PDUFA

PHYSICIAN PAYMENT
Subretinal procedure

Physician payment to the retina surgeon

CPT 0810T
Subretinal Category III code

~$1,000–$1,230
Physician payment per procedure

✓ Code in place today

FACILITY PAYMENT
ASC / hospital outpatient (HOPD)

Facility payment: ASC or hospital outpatient

CPT 0810T
Subretinal Category III code

ASC **$2,284**

HOPD **$4,251**

✓ Code in place today

Physician and facility codes already exist; the product code follows the standard CMS path after approval

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ASC: ambulatory surgical center; ASP: average sales price; CPT: Current Procedural Terminology; HCPCS: Healthcare Common Procedure Coding System; HOPD: hospital outpatient department; MAC: Medicare Administrative Contractor. *0810T replaced C9770 – HCPCS code C97X1 and maps to APC 1562, previously 1561 (Status Indicator T; New Technology – Level 24) (Federal Register 2021-24011; CMS 2023 OPPS Final Rule). 1. 2026 MAC published rates for First Coast Service Options, Inc. and Novitas Solutions. 2. Product-specific coding follows applicable CMS processes; timing depends on approval and application cycle Source: 2026 MPFS and MAC published rates, 2026 ASC payment files, 2026 OPPS payment files.

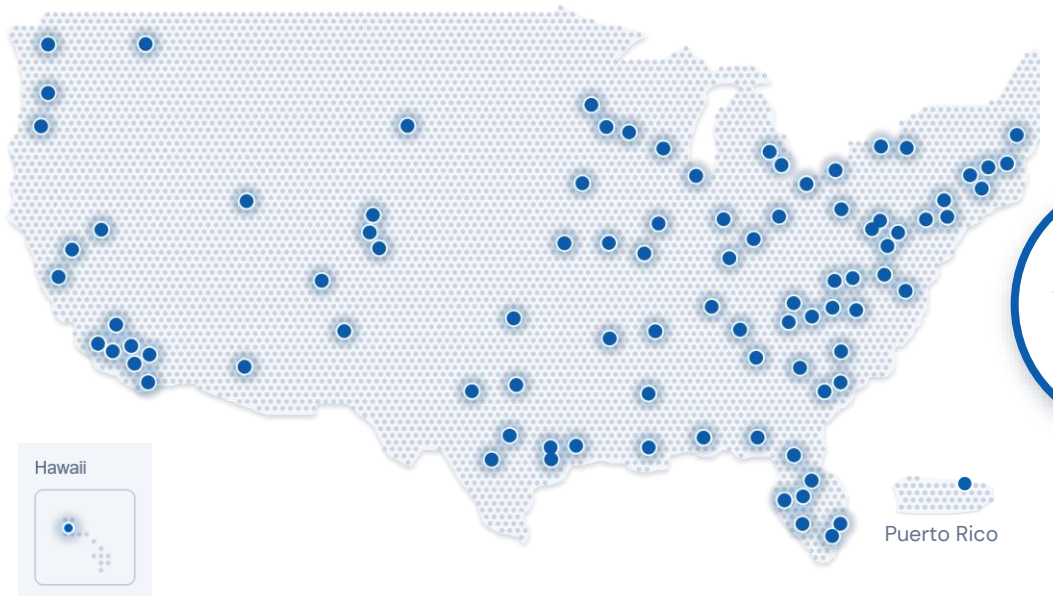
Sura-vec coverage and payment will depend on approval, payer policies, and applicable coding

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Sura-vec pivotal program footprint provides a foundation for potential commercialization

Approximately 80% overlap of clinical sites with top 1–3 tier centers

SURA-VEC CLINICAL SITES IN US

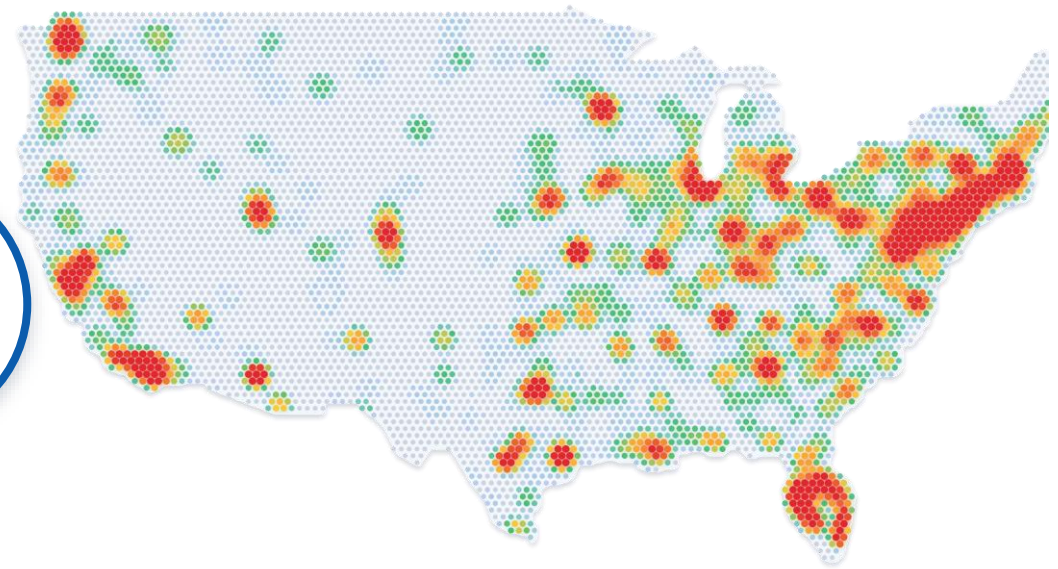


ATMOSPHERE

>300
Retinal surgeons trained in U.S.[1]

~80%
overlap

TOP 1–3 TIER US CENTERS



Low Tier 5 — High Tier 1*

Pivotal sites already concentrate patients, surgeons, and infrastructure

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1. Over 300 retinal surgeons in the United States were trained on the sura-vec procedure, over 500 globally.
Note: U.S. estimates. Tier 1–3 sites based on internal analysis. Share of offices/sites derived from figures shown (150 of ~2K; 120 of ~1K);
~80% overlap of top 3 tier retina offices and surgical centers per internal analysis. aVEGF = anti-VEGF; ppV = pars plana vitrectomy. Sources: Internal analyses; Market Scope 2024; AAO IRIS® Registry 2023; published literature.

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 Phase IIb subjects with NPDR without center-involved diabetic macular edema (CI-DME)
- All treated 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 60% (6/10) of participants achieving ≥ 2-step improvement without additional DR treatment at 3 years
- These participants did not experience any vision-threatening events.

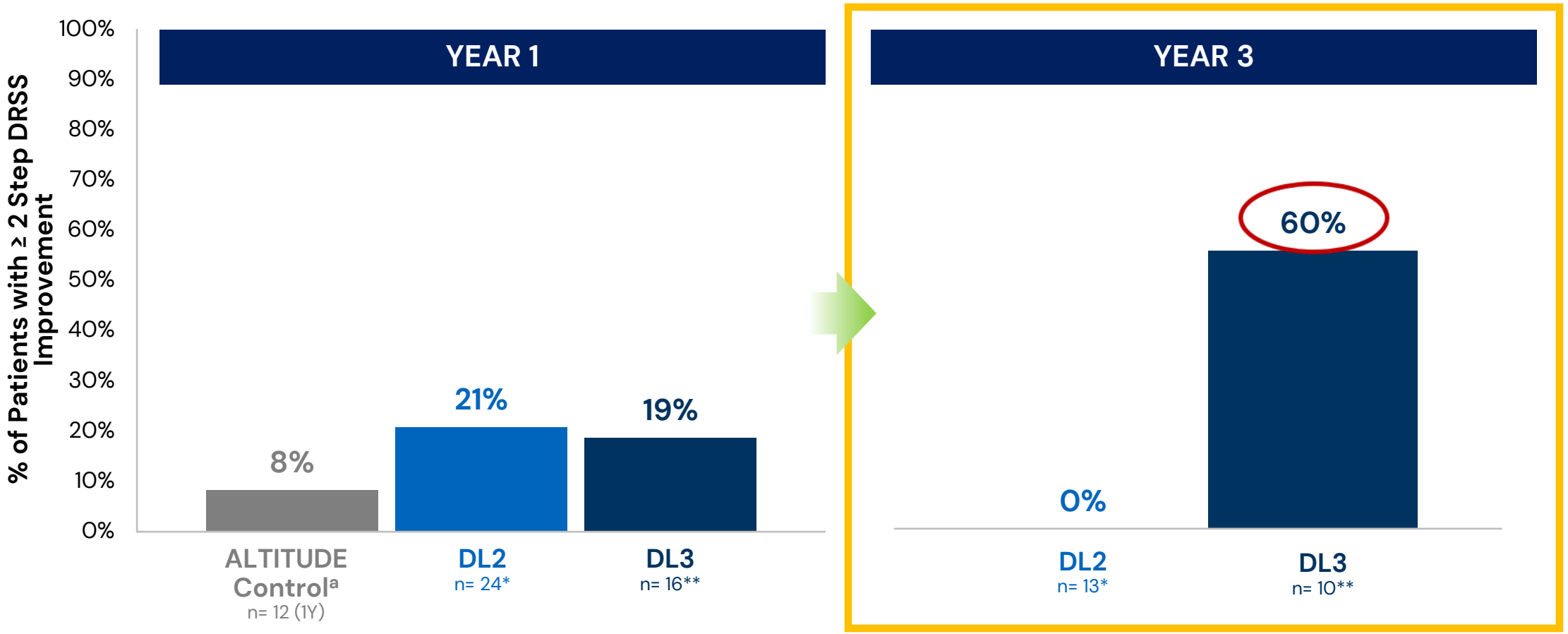
First patient in NAAVIGATE study was dosed in Q2 2026

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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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Single injection of sura-vec at pivotal dose shows continued DRSS improvement through 3 years



Data cut: Aug 17, 2026.
*Includes 1 subject who crossed over from control; **Includes 2 subjects who crossed over from control.
a. Control arm ended at 1 year. 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.
DL: Dose Level; DR: diabetic retinopathy; DRSS: Diabetic Retinopathy Severity Scale; Sura-vec: surabgene lomparvovec; Y: year.
This is an interim analysis performed by REGENXBIO for an ongoing trial.
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60% (6/10) achieved ≥2-step improvement without vision-threatening events or additional DR treatment at 3 years



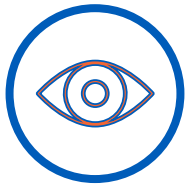
Data cut: Aug 17, 2026. *Control subjects were eligible to cross over to receive sura-vec at Year 1. Subjects who were DRSS 47 and 53 at crossover are included in the treatment arm analyses (n=2). One subject in Dose Level 3 was found to have confounding disease at baseline and their data was excluded. a. One subject enrolled in long-term follow-up and had no return visits;. b. Patient received 2 IVT after Y2 for DME, but OCT showed dry retina at the time of injection. Subject was receiving IVT for DME in FE. 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; LTFU: long-term follow-up; Sura-vec: surabgene lomparvovec; VTC: Vision Threatening Complication; VTE: Vision Threatening Event. This is an interim analysis performed by REGENXBIO for an ongoing trial.

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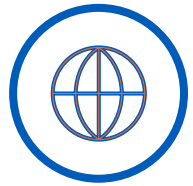
Global eye-care alliance with AbbVie validates retina franchise and enables world-class commercialization capabilities



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)

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

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

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

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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

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

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

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

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

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

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

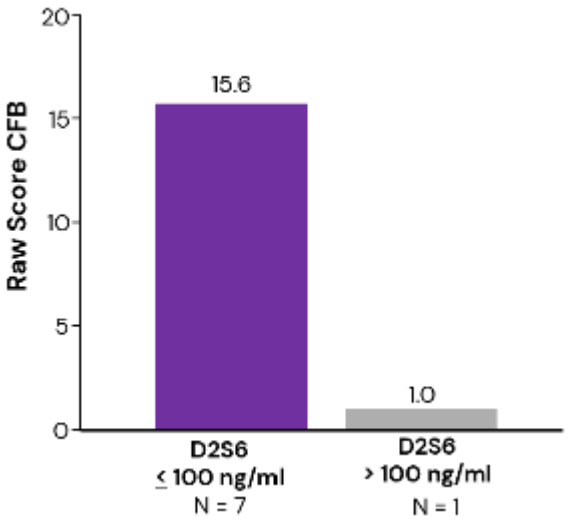
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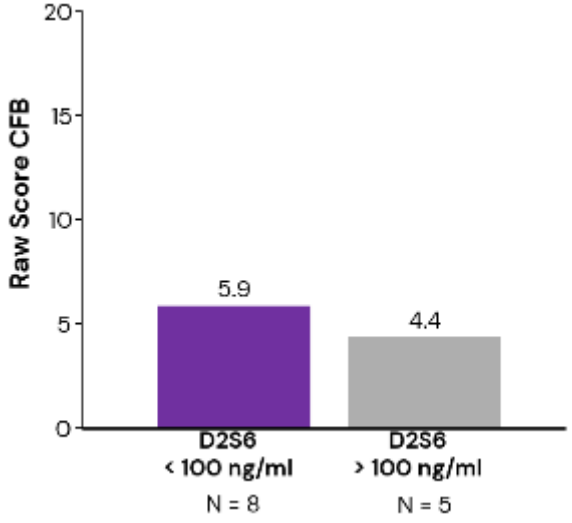
Data cut date: August 20, 2024
MPS II attenuated reference point < 100 ng/mL based on analysis presented in Boulos N, World Symposium, 2022
* Primary endpoint was compared to the margin of 20%
** Samples could not be obtained from 2 participants at 1 year
*** BSID-III, Bayley Scale of Infant and Toddler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

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

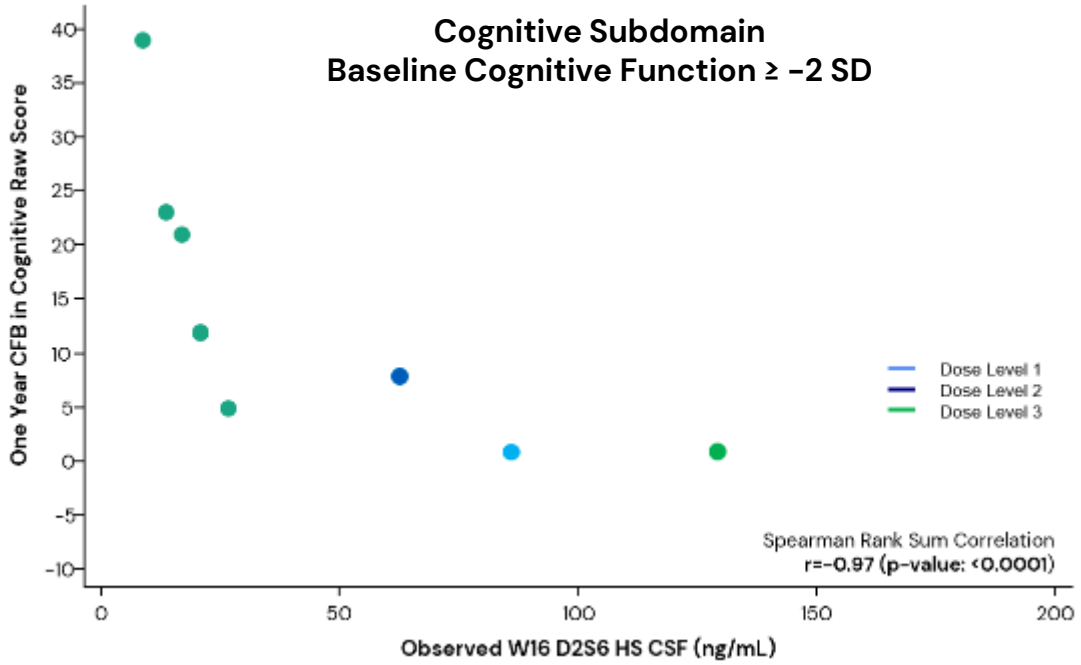
Baseline BSID-III Cognitive Function ≥ -2SD



Baseline BSID-III Cognitive Function < -2SD



Cognitive Subdomain Baseline Cognitive Function ≥ -2 SD



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

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

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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

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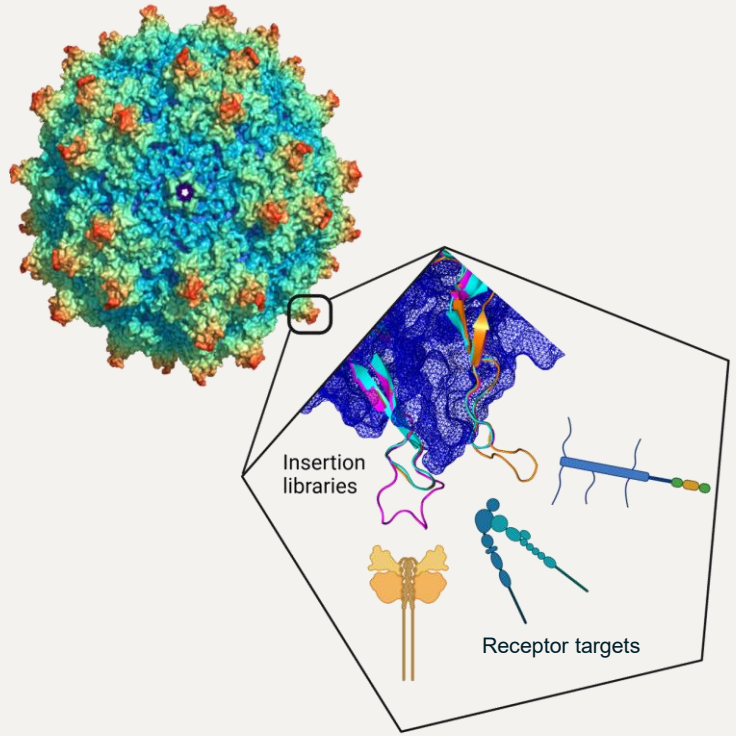
Discovering the next wave of gene therapies

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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