Delivering the next wave of genetic medicines

Corporate Presentation
December 2024

Forward-Looking Statements

This presentation includes "forward-looking statements," within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as "believe," "may," "will," "estimate," "continue," "anticipate," "assume," "design," "intend," "expect," "could," "plan," "potential," "predict," "seek," "should," "would" or by variations of such words or by similar expressions. The forward-looking statements include statements relating to, among other things, REGENXBIO's future operations, clinical trials, costs and cash flow. REGENXBIO has based these forward-looking statements on its current expectations and assumptions and analyses made by REGENXBIO in light of its experience and its perception of historical trends, current conditions and expected future developments, as well as other factors REGENXBIO believes are appropriate under the circumstances. However, whether actual results and developments will conform with REGENXBIO's expectations and predictions is subject to a number of risks and uncertainties, including the outcome of REGENXBIO's collaboration with AbbVie and other factors, many of which are beyond the control of REGENXBIO. For a summary of certain of these risks and uncertainties, refer to the "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of REGENXBIO's Annual Report on Form 10-K for the year ended December 31, 2023 and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the U.S. Securities and Exchange Commission (SEC) and are available on the SEC's website at www.sec.gov. All of the forward-looking statements made in this presentation are expressly qualified by the cautionary statements contained or referred to herein. The actual results or developments anticipated may not be realized or, even if substantially realized, they may not have the expected consequences to or effects on REGENXBIO or its businesses or operations. Such statements are not guarantees of future performance and actual results or developments may differ materially from those projected in the forward-looking statements. Readers are cautioned not to rely too heavily on the forward-looking statements contained in this presentation. These forward-looking statements speak only as of the date of this presentation. Except as required by law, REGENXBIO does not undertake any obligation, and specifically declines any obligation, to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

Seeking to improve lives through the curative potential of gene therapy

We pioneered the landscape of adeno-associated virus (AAV) gene therapies.

Thousands of patients have been treated with approved and investigational medicines built on our NAV® Technology platform.

We are advancing late-stage, potential first- and best-in-class gene therapies for patients with retinal and rare diseases.

Addressing multiple billion-dollar+ opportunities, with lead candidate in Duchenne muscular dystrophy.

With scientific expertise and end-to-end capabilities, REGENXBIO is leading the future of one-time treatments.

Fully-integrated manufacturing and fill-finish capabilities support multiple potential product launches.

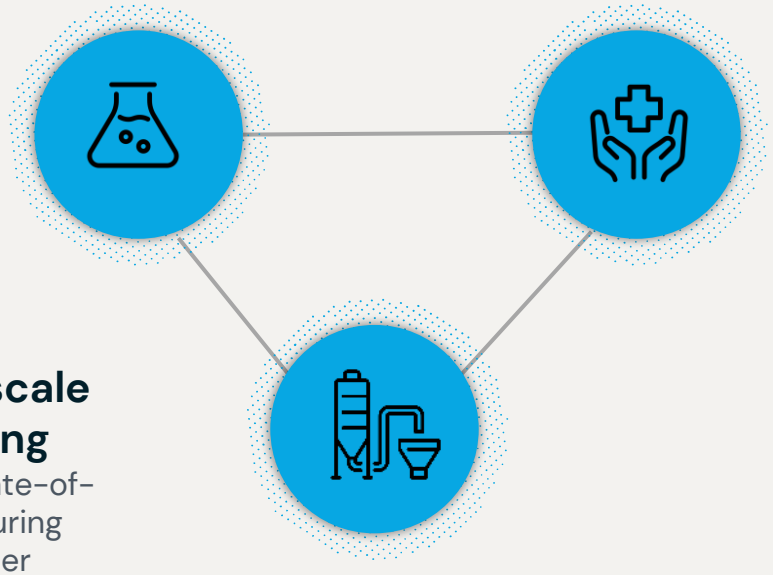
REGENXBIO®

Driving significant value creation

Industry-leading platform to deliver new medicines

R&D Engine
Leveraging capsid discovery and gene transfer technology

Clinical Development
designed to accelerate medicines to patients with no or limited options



Commercial-scale Manufacturing
industry-leading state-of-the-art Manufacturing Innovation Center

$7B^

Duchenne

Blockbuster opportunity as likely second entrant into established infrastructure with a potential best-in-class product for large and underserved patient population

Pursuing accelerated approval and broad label

$1B^

MPS II

Well-positioned to be **first potential one-time treatment** for MPS II

Eligible for Priority Review Voucher upon potential accelerated approval

$17B*

Retinal Disease

Leader in investigational gene therapies for chronic retinal conditions

Positioned to be first approved gene therapy to preserve vision and prevent disease progression in wet AMD and diabetic retinopathy

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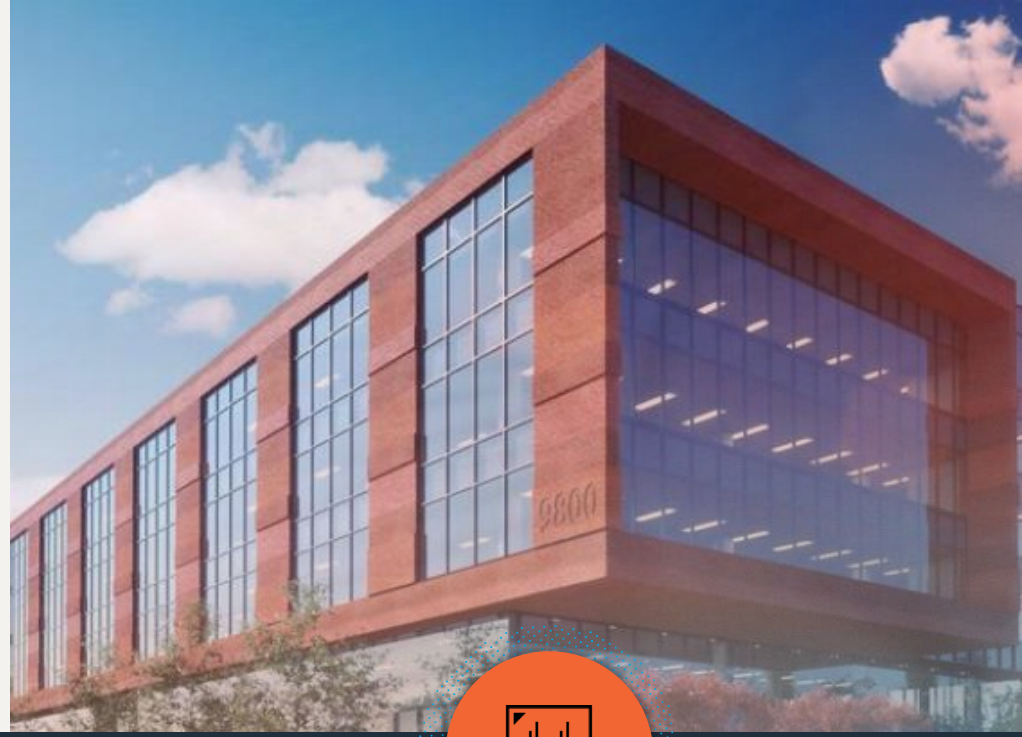
* MarketScope 2022 Retinal Pharmaceuticals Market Report
^ 2024 Evaluate Pharma Consensus Forecast

REGENXBIO's pipeline

Indication		Description	Phase I	Phase II	Pivotal	Anticipated Milestones
Rare Disease						
Duchenne	RGX-202	Novel microdystrophin				BLA using accelerated approval pathway 2026
MPS II	RGX-121	Iduronate-2-sulfatase enzyme				Submission of a rolling BLA using the accelerated approval pathway ongoing, expected to be completed Q1 2025
Retinal Disease						
wet AMD subretinal delivery	ABBV-RGX-314 abbvie eye care collaboration	Anti-VEGF				Global regulatory submissions 1H 2026
Diabetic retinopathy In-office suprachoroidal delivery						Design and evaluation of two pivotal trials is ongoing Pivotal trial initiation 1H 2025
wet AMD In-office suprachoroidal delivery						Initiated dose level 4 cohort with short course prophylactic steroid eye drops

Leaders in gene therapy manufacturing

REGENXBIO Manufacturing Innovation Center ready to serve patients facing rare and retinal diseases.



Capacity & Control

- 2,500 doses/year of RGX-202
- 350,000 doses/year of ABBV-RGX-314
- Internal drug substance and drug product manufacturing enables control of capacity vs. third-party manufacturer

Platform

- Proprietary, high-yielding NAVXpress™ suspension platform process
- Potential for candidate selection to clinical supply in 12 months

Efficiency

- Acceleration of product development and high yields enable lower cost of goods

Rare Disease

RGX-202:

Next generation microdystrophin design for Duchenne Muscular Dystrophy

RGX-202: A next-generation, investigational gene therapy

Four pillars for delivering RGX-202 as next to market for Duchenne

Regulatory and Patients

Aligned with FDA on a path to **Accelerated Approval**; on track to file **BLA in 2026** and are committed to **data transparency with the patient community**

Robust Clinical Biomarkers

Consistent & robust levels of RGX-202 microdystrophin expression and transduction observed in target muscle providing the potential for **long-term, durable clinical outcomes**

Differentiated Safety

Combination of a differentiated construct, proactive immunosuppression regimen and high product purity have enabled a **preferred dose with encouraging safety profile**

Positive Functional Outcomes

RGX-202 demonstrates* functional improvements exceeding natural history benchmarks

RGX-202 NAVXpress™ Process Platform: In-house Manufacturing, High Yield, High Purity, Commercial Ready

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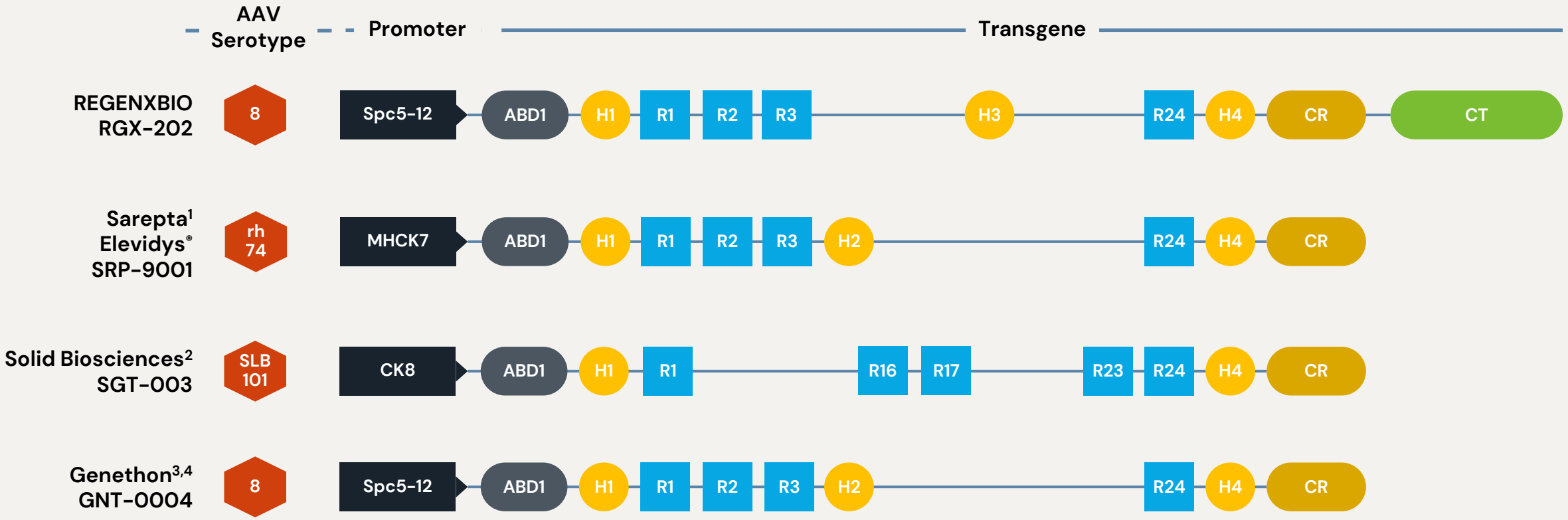
*Data cut date November 1, 2024

RGX-202 is designed for improved function in Duchenne

RGX-202 is the only microdystrophin gene therapy with the functional elements of the C-Terminal (CT) domain found in naturally occurring dystrophin



1. Harper (2002) Nat Med
2. https://investors.solidbio.com/Corporate Presentation, January 2024
3. Mbakam & Tremblay (2023) Expert Rev Neurother
4. Le Guiner (2017) Nat Comm

AFFINITY DUCHENNE® trial design

Pivotal Trial for Accelerated Approval Initiated

- Aligned with FDA on pivotal design and availability of accelerated approval pathway
- **BLA expected 2026** using accelerated approval to include approximately 30 patients, with biomarker+ functional data

Phase I/II

✓ **Dose Evaluation**
(Ages 4–11)

Dose Level 1
$1x10^{14}$ GC/kg
N=3

Dose Level 2
$2x10^{14}$ GC/kg
N=2

✓ **Expansion Cohort**
(Ages 4–11)

Dose Level 2
(N=5)

Younger Patient Cohort
(Ages 1–3)

Dose Level 2
(up to 5 patients)

Pivotal

Dose Level 2 Ambulatory
(patients aged 1+)
N ~ 30

Primary endpoint: Proportion of patients with ≥10% microdystrophin levels

Secondary endpoints: Timed function tests, including time to stand velocity, SV95C ages 1–3

Exploratory endpoint: NSAA, SV95C for 4+

Interim safety

RGX-202 Treatment-Emergent Adverse Events		Dose Level 1 Dose Evaluation ($1x10^{14}$ GC/kg)	Dose Level 2 Dose Evaluation / Expansion ($2x10^{14}$ GC/kg)	Dose Level 2 Younger Boys ($2x10^{14}$ GC/kg)	Total n = 11
Age Range (number dosed)		4–11 (n = 3)	4–11 (n = 7)	1–3 (n = 1)	All Age Ranges
SAE		0	0	0	0
AESI	Central Or Peripheral Neurotoxicity	0	0	0	0
	Drug-Induced Liver Injury	0	0	0	0
	Thrombocytopenia*	0	0	0	0
Myocarditis*		0	0	0	0
Myositis*		0	0	0	0
The most common drug-related AEs reported are anticipated with gene therapy: nausea (n=3), vomiting (n=6), and fatigue (n=5), all resolved					

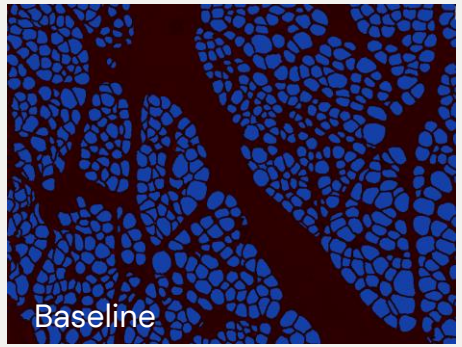
RGX-202 has been well-tolerated in all patients at both dose levels with no SAEs or AESIs

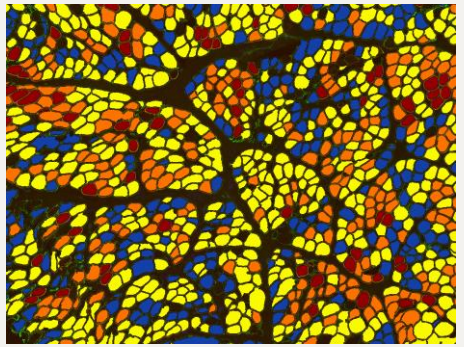
Data cut data November 1, 2024
SAE – Serious Adverse Event; AESI – Adverse Events of Special Interest
*based on Common Terminology Criteria for Adverse Events (CTCAE) version 5

Biomarkers support consistent robust expression, transduction, and sarcolemmal localization of RGX-202 microdystrophin

Mean at 12 Weeks (min, max)	Dose Level 1 $1x10^{14}$ GC/kg		Dose Level 2 $2x10^{14}$ GC/kg	
Age range at screening (number with data)	**4–7** (2)	**8–11** (1)	**4–7** (1)	**8–11** (5)
RGX-202 Microdystrophin* % (Western Blot)	**60.6** (38.8, 83.4)	**11.1** (n/a)	**77.2** (n/a)	**39.7** (20.9, 75.7)
VCN copies/nucleus (qPCR)	**9.8** (7.4, 12.1)	**5.4** (n/a)	**55.4** (n/a)	**17.8** (12.0, 30.7)
Positive Fibers** % (Immunofluorescence)	**79.3**** (n/a)	**34.6** (n/a)	**71.1** (n/a)	**45.7** (21.3, 70.6)

Fiber Intensity †

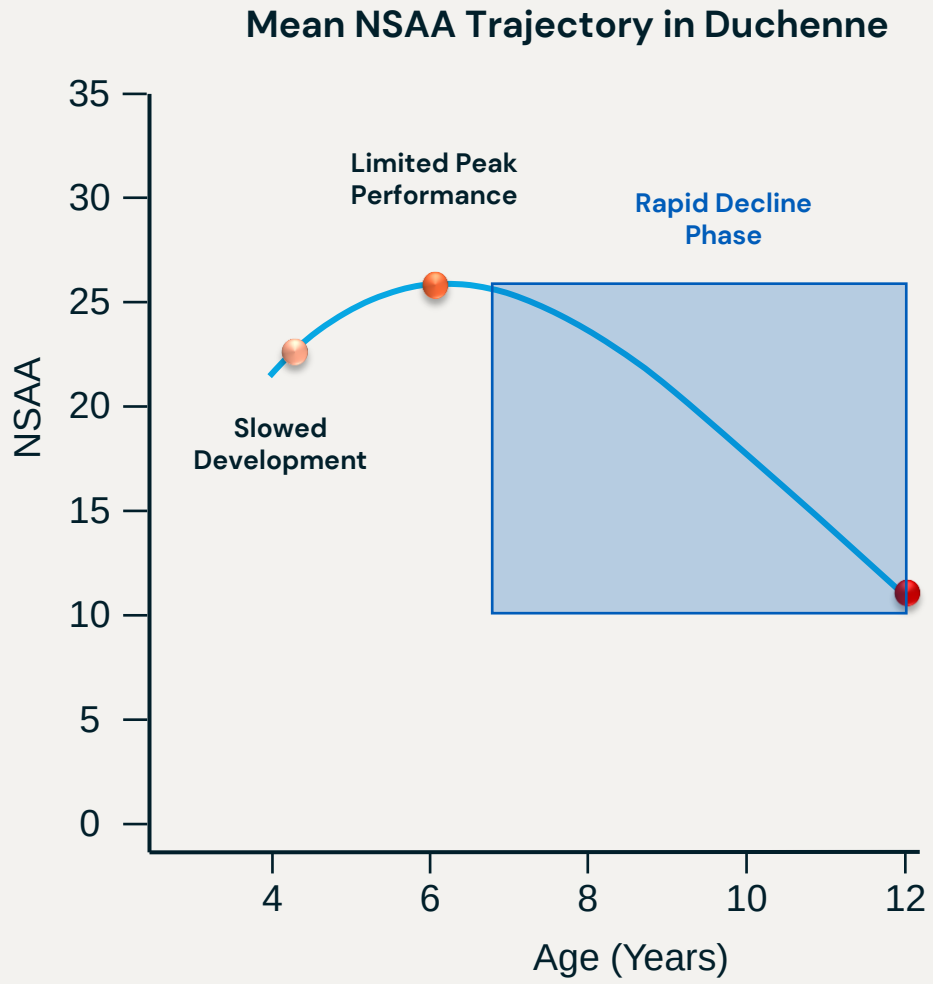




RGX-202 DL2, 12 weeks

Data cut date November 1, 2024
* Microdystrophin expression adjusted for muscle content; % normal control
**Positive Fibers defined as change from baseline of RGX-202 microdystrophin & dystrophin positive fibers
*** One sample could not be evaluated
† Microdystrophin Fiber Intensity: Blue = Negative; Yellow = +1 (low) intensity; Orange = +2 (medium) intensity; Red = +3 (strong) intensity

RGX-202 functional data: natural history control methodology



Functional Data at Clinically Meaningful Timepoints

- Dose level 1
  - N=3 at 12 months post-RGX-202 administration
- Dose level 2
  - N=2 at 9 months post-RGX-202 administration

Method for External Controls

Heterogeneity is present in baseline disease stage, rate of disease progression, and anticipated efficacy response

Matched controls from Natural History Dataset* enable comparison to RGX-202 for rate of disease progression and anticipated efficacy response.

Natural history control matching criteria:**

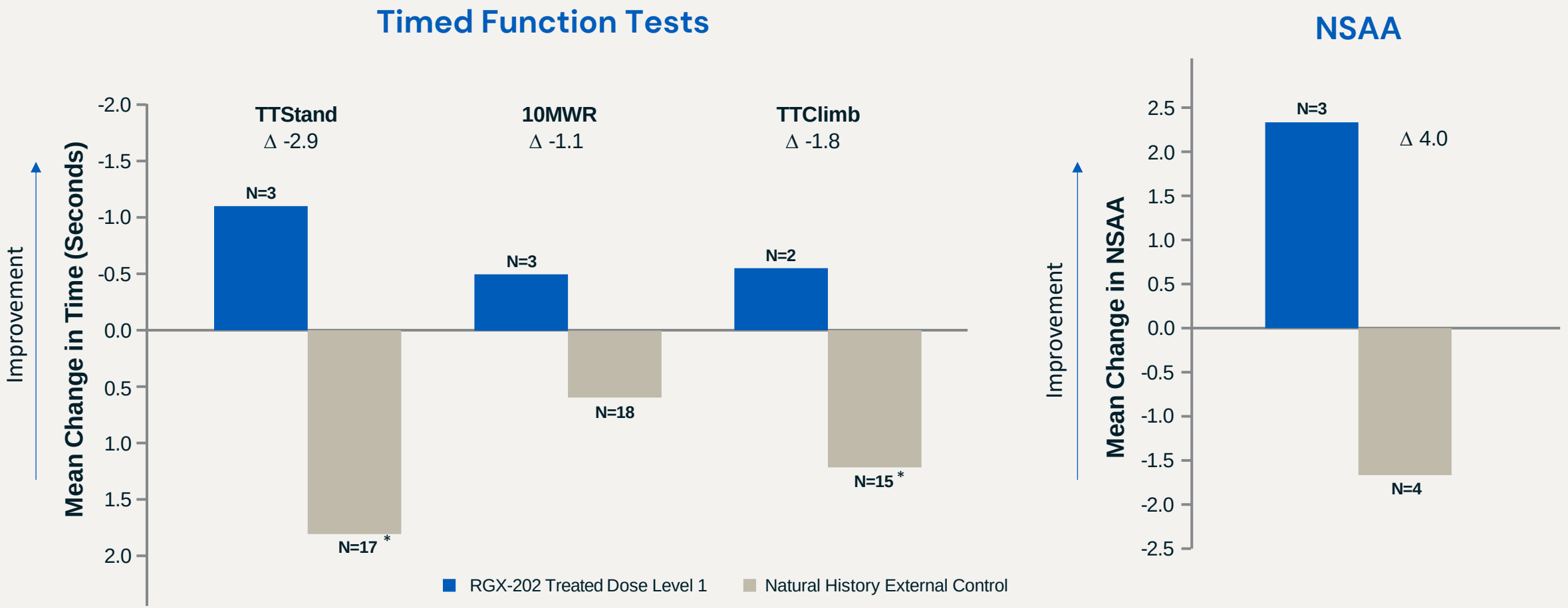
- Age
- Baseline function

Graph adapted from Muntoni 2019

* Natural history datasets included 420 patients with steroid exposure from CINRG and the D-RSC Data Platform. 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).

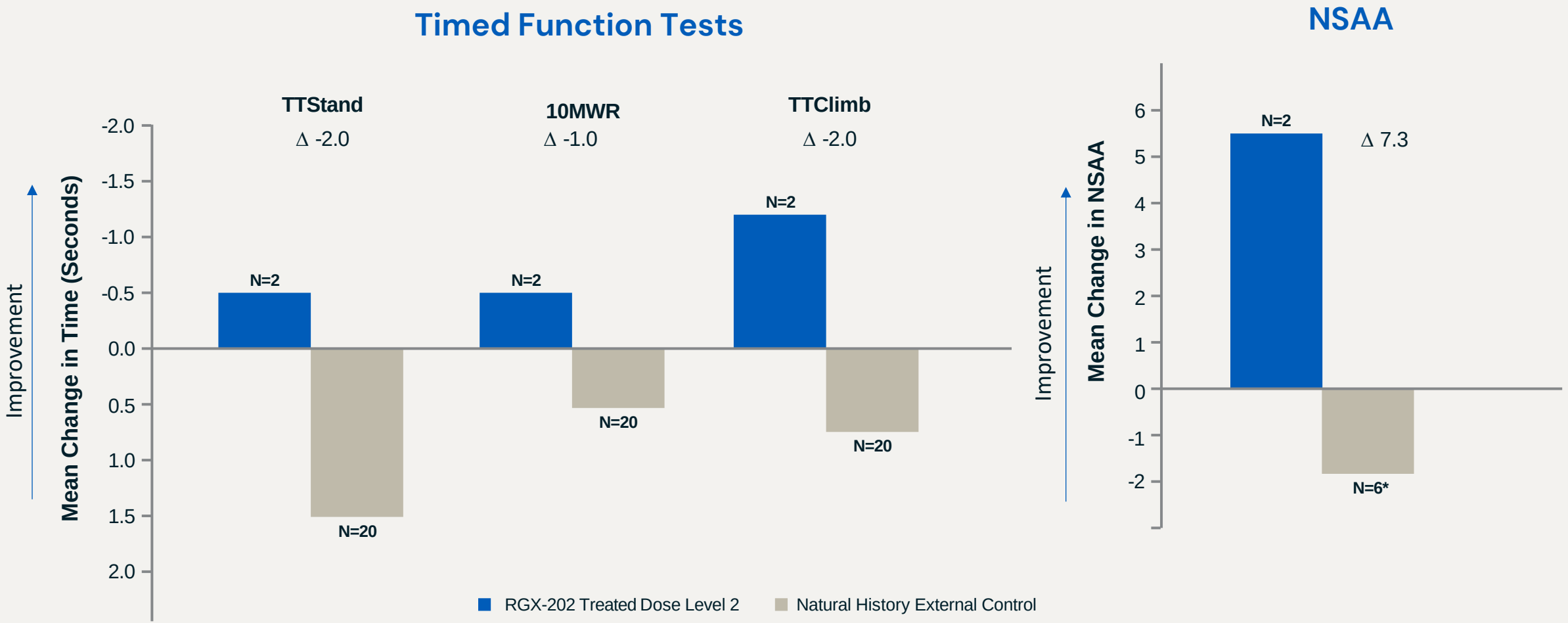
** Criteria for matching:, TTSTAND, TTRW, and TTCLIMB . Group mean for external controls were weighted by the number of matched NH patient per each RGX- 202 treated participants.

Dose level 1 participants demonstrate improvement in function and exceed external controls at 12 months



Data cut date November 1, 2024
Time to Stand (TTStand); 10M Walk Run (10MWR); Time to Climb (TTClimb)
*One NH EC patient did not have a TTStand and one RGX-202 patient did not have TTClimb so associated NH EC also removed

Pivotal dose participants demonstrate improvement in function at 9 months



Data cut date November 1, 2024
Time to Stand (TTStand); 10M Walk Run (10MWR); Time to Climb (TTClimb)
* For NSAA, one patient did not have matching natural history external controls

Caregivers reported improved function

Caregivers reported improvements in the home and community environments as measured by PODCI

Improved skills included:

- Running
- Riding a bicycle/tricycle
- Climbing stairs
- Walking in the community
- Participating in recreational activities and sports with peers

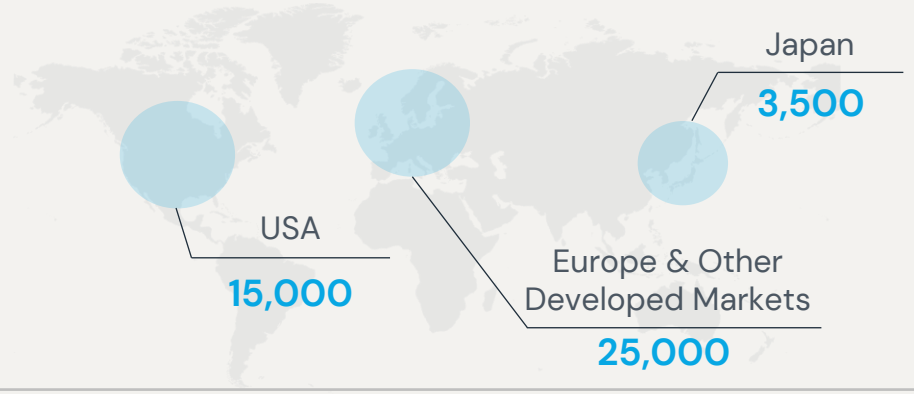
Data cut date November 1, 2024
Pediatric Outcomes Data Collection Instrument (PODCI)
* All 5 participants had improved PODCI scores on at least one of the measures at 12 months (dose level 1) or 9 months (dose level 2) change from baseline

Duchenne Muscular Dystrophy (DMD) opportunity: an estimated $7B global market with ongoing unmet need

DUCHENNE MARKET IN THE NEXT 5 YEARS[6]

$7B

PATIENTS IN MAJOR GLOBAL MARKETS[1-5]

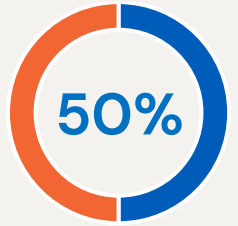


Limited effective treatments to delay loss of ambulation and death[5]

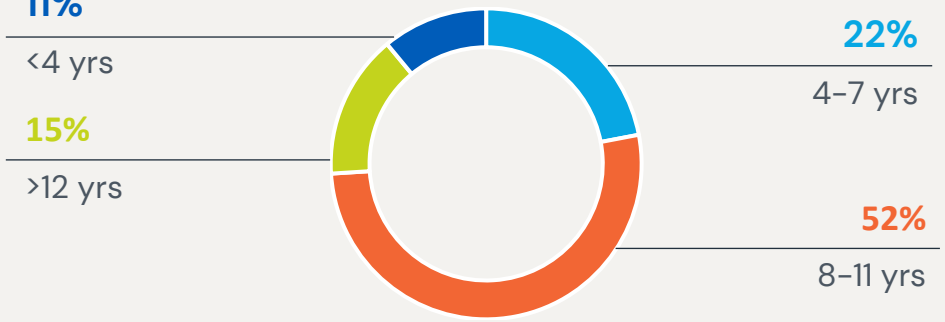
MEDIAN SURVIVAL AGE[4]

Mid to late 20s

% WITH AMBULATORY LOSS



TOTAL DIAGNOSED AMBULATORY POPULATION SPLIT BY AGE RANGE[2,3]



1. Crisafulli et al 2020; 2. Koeks et al 2017; 3. Bello et al 2016; 4.McDonald et al 2018; 5. Health Resources & Services Administration (HRSA) Advisory Committee on Heritable Disorders in Newborns and Children "Nomination and Prioritization Workgroup Report on Duchenne Muscular Dystrophy" Feb 2023; 6. Evaluate Pharma

Phase I/II AFFINITY DUCHENNE: Interim Summary

Positive safety, biomarker and functional data demonstrate the potential of RGX-202 to be a differentiated, best-in-class gene therapy

RGX-202 has been well-tolerated in 11 patients across both dose levels with no SAEs or AESIs

Biomarkers support consistent robust expression, transduction, and sarcolemmal localization of RGX-202 microdystrophin

Participants treated with RGX-202:

- Demonstrated clinically meaningful improvement in functional outcomes at both dose levels
- Exceeded comparisons using NH external controls and MCID*

Evidence of altering the trajectory of disease

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Data cut date November 1, 2024

* MCID (minimal clinically important difference) (Duong, 2021) compared to dose level 1 at 12 months

RGX-121:

On track to be the first gene therapy for Hunter Syndrome

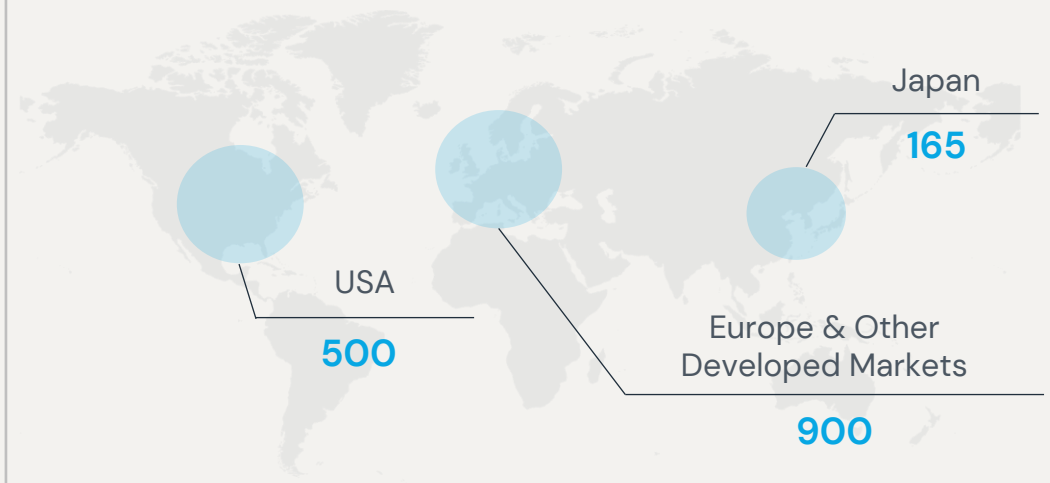
Mucopolysaccharidosis Type II (MPS II) opportunity: an estimated $1B global market within 5 years[9]

RGX-121 is the only product in late-stage development with the potential to address neurocognitive development in patients diagnosed under age 2 years[5]

MPS II MARKET IN THE NEXT 5 YEARS[9]

$1B

PATIENTS IN MAJOR GLOBAL MARKETS[1-3,8]



~70%

Neuronopathic form accounts for the majority of patients[1-4]

CHRONIC ENZYME REPLACEMENT THERAPY IS INADEQUATE

Current standard of care is **weekly IV ERT** infusion to treat **somatic** symptoms only[1]

No approved treatment to prevent neurocognitive loss^

BROAD ACCESS TO NEWBORN SCREENING EXPECTED TO INCREASE EARLY DIAGNOSIS AND TREATMENT BY 2025

Newborns Screened for MPS II [6,7]
~60%

Average Age of Diagnosis with Newborn Screening: **0.2 yrs**[1]

^ERT approved in select Asian markets 1. Mucopolysaccharidosis Type II External Evidence Review by HRSA 2022; 2. Martin et al 2008; 3. Shapiro E and Eisengart J 2021; 4.Burton B WORLD 2022 5.Clinicaltrials.gov; 6. EveryLife Foundation; 7. National MPS Society; 8. Khan et al 2017; 9. Evaluate Pharma (Jun'23); ERT – enzyme replacement therapy

RGX-121 for MPS II: Phase I/II/III CAMPSIITE® study

The Disease

- Reduced ability to process glycosaminoglycans (GAGs), leading to neurodegeneration, and early death
- X-linked recessive disease
- Available treatment is inadequate to treat neurodegeneration
- More than 500 patients born annually worldwide

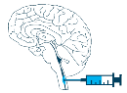
RGX-121 PRODUCT CANDIDATE

Vector:
AAV9

Gene:
IDS Gene Replacement

FDA Designations:
- Orphan Drug Designation
- Rare Pediatric Disease Designation
- Fast Track Designation
- Regenerative Medicine Advanced Therapy Designation

Route of administration:
Intracisternal delivery



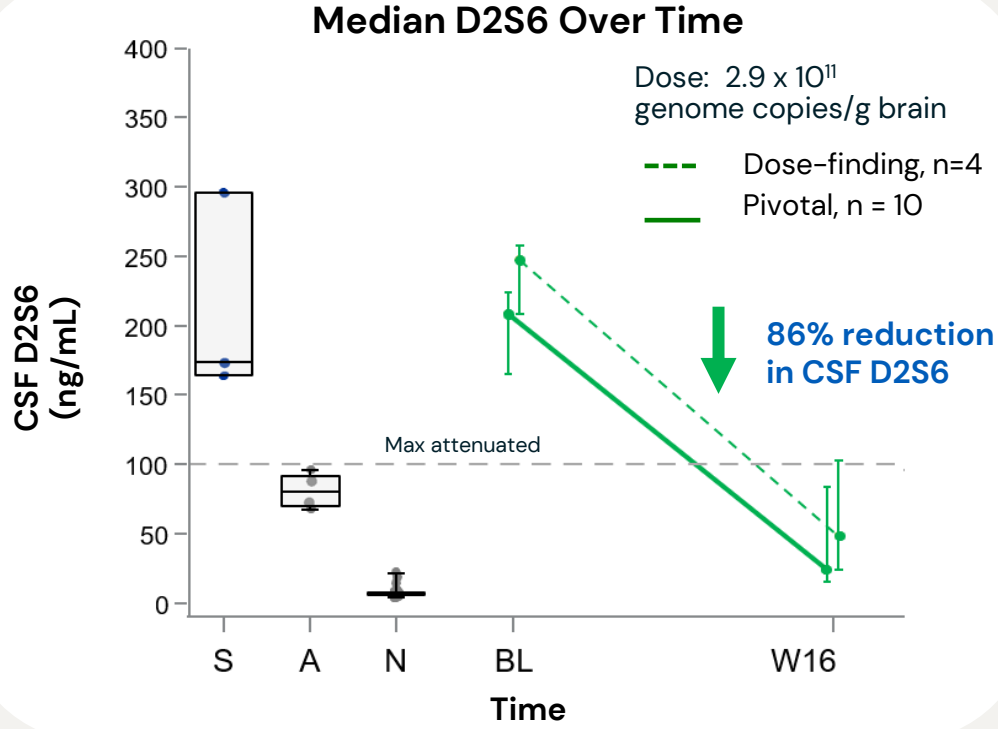
CAMPSIITE Part 2, Pivotal Trial to Support Approval

- Completed enrollment of 10 boys with neuronopathic MPS II, aged 4 months up to five years to support the BLA filing utilizing the accelerated approval pathway
- Pivotal dose: 2.9 x 10^{11} GC/g of brain mass, using commercial-scale cGMP material from REGENXBIO's proprietary, high-yielding NAVXpress™ platform process
- Trial collecting GAGs in CSF and neurodevelopmental data, and caregiver reported outcomes

CAMPSIITE Part 2: Pivotal trial primary endpoint achieved

Primary Endpoint:
Proportion of Patients with CSF D2S6 below maximum attenuated level at W16

- Primary endpoint reached with statistical significance (p value of 0.00016)*
  - 8 of 10 pivotal patients demonstrated reductions in CSF D2S6 to below maximum attenuated levels
  - Other 2 pivotal patients also exhibited robust reductions in CSF D2S6 (55%, 85%)



Meaningful reductions in CSF D2S6, approaching normal levels

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10 participants dosed as of July 31, 2023
Data cut date January 3, 2024

* Response rate was compared to the margin of 20% (two-sided p value of 0.00016)
Median CSF D2S6 concentration +/- Q1 and Q3 per cohort.
(S) Severe (A) Attenuated (N) Normative

CAMPSIITE and RGX–121 Summary

RGX-121 was well tolerated across 25 patients in all phases of CAMPSIITE

Pivotal trial met CSF D2S6 primary endpoint with statistical significance

Neurodevelopmental and daily activity skill acquisition was observed up to 4 years after RGX-121 administration

Held positive pre-BLA meeting; submission of a rolling BLA using the accelerated approval pathway initiated, expected to be completed in Q1 2025

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 June 20, 2023): No SAE considered related to RGX-121

Retinal Disease

ABBV-RGX-314

Potential to be the first gene therapy for chronic retinal diseases

Potential to Address the Real-World Unmet Need for Vision Preservation

ABBV-RGX-314 continues to demonstrate stable disease control in wet AMD and prevention of vision-threatening events in diabetic retinopathy

A single treatment with ABBV-RGX-314 could close the gap in outcomes between randomized clinical trials and real-world outcomes

ABBV-RGX-314 has the potential to be transformative by delivering an anti-VEGF clinical benefit with a one-time injection

Advancing dual routes of administration strategy for expanded access

AMD, age-related macular degeneration;; VEGF, vascular endothelial growth factor.

Global eye-care alliance with AbbVie to develop and commercialize ABBV-RGX-314 retina franchise

Leadership and expertise in AAV and retinal gene therapy

Strong in-house capabilities of AAV manufacturing

Leading eye care company

Established commercial Infrastructure in 170+ countries

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 the U.S.**
- **REGENXBIO will lead the manufacturing of ABBV-RGX-314** for clinical development and U.S. commercial supply

ABBV-RGX-314 clinical studies summary

Suprachoroidal (SCS) wAMD & DR

Clinical Study		Status	Region	Estimated Size	Description	Planned Readout
wAMD	*Phase II* AAVIATE	Enrolling	US	140	Dose finding	2024
DR & DME	*Phase II* ALTITUDE	Enrolling	US	130	Dose finding	2024

Subretinal (SR) wAMD

Clinical Study	Status	Region	Estimated Size	Description	Planned Readout
Pivotal ATMOSPHERE	Enrolling	US	540	Pivotal, 2 dose levels	2025
Pivotal ASCENT	Enrolling	Global	660	Pivotal, 2 dose levels	2025
Phase II Bioreactor bridging	Enrolled	US	60	Open label, 2 Pivotal doses	2024
Fellow Eye	Enrolled	US	20	Open label, bilateral safety	2024
Long Term Follow Up	Enrolling	Global	-	Supports Durability	2024
Phase I/IIa	Enrolled	US	42	Dose finding	

Wet AMD: Global launch readiness for subretinal ABBV-RGX-314

Robust clinical data

Positive vision and safety profile established in Phase I/IIa study with up to 4 years of durability

Results consistent in bridging and fellow eye studies to support launch strategy

Pivotal Program to Evaluate Differentiated Treatment

Pivotal trial program designed for potential global regulatory submissions, expected 1H 2026

ATMOSPHERE

ASCENT

Global Commercial Launch Execution

Global eye-care collaboration with AbbVie to leverage commercial launch teams with proven record of product launch success in global retina markets

AbbVie's commercial footprint includes operations in more than 70 countries across five world regions

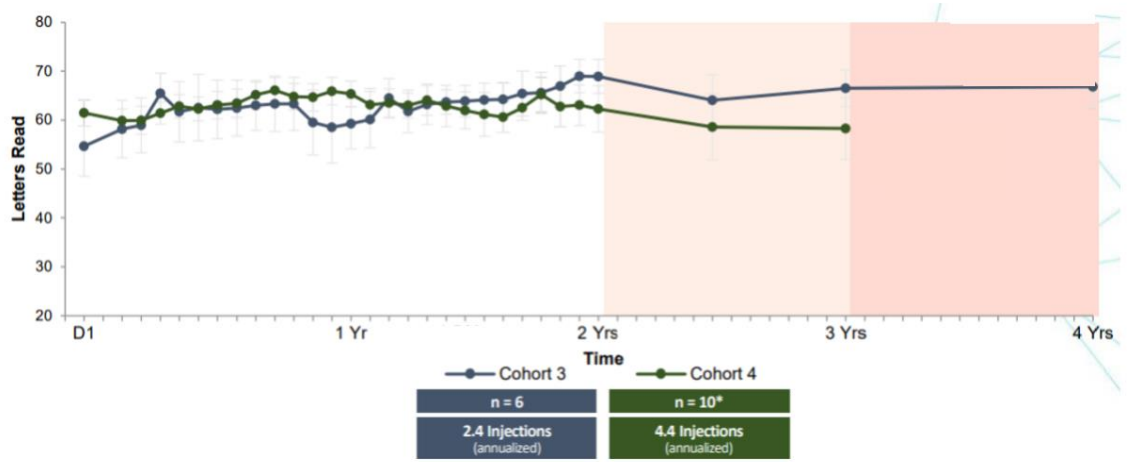
Positioning ABBV-RGX-314 to prevent vision loss in millions of patients worldwide

AMD, age-related macular degeneration; DME, diabetic macular edema; DR, diabetic retinopathy; NPDR, non-proliferative diabetic retinopathy; PDR, proliferative diabetic retinopathy; VEGF, vascular endothelial growth factor.

1. 2023 Retinal Pharmaceuticals Market Report, Market Scope.
2. Ciulla TA, et al. *Ophthalmol Retina.* 2022;6(9):796-806.

SR wet AMD: Leading ocular gene therapy clinical data

Phase I/IIa LTFU (BCVA)



A single ABBV-RGX-314 treatment has the potential to become a *new standard-of-care option* by sustaining vision health and reducing treatment burden.

Overall Safety

- ABBV-RGX-314 has been well tolerated across Phase I/II (up to 4 years)* and Phase II Bioreactor Bridging^ studies (at 6 months) at doses similar to pivotal study
  - No drug-related SAEs
  - Common AEs[1] including post-op conjunctival hemorrhage and post-op inflammation[2] resolving within days to weeks, eye irritation, eye pain, retinal degeneration, IOP increase, post-operative visual acuity reduction and retina hemorrhage; retinal pigmentary changes classified as mild to moderate

Efficacy Endpoints

- With a single injection of ABBV-RGX-314 at dose levels similar to the pivotal trial, patients demonstrate a long-term, durable treatment effect up to 4 years
  - Stable to improved visual acuity
  - Meaningful reductions in anti-VEGF injection burden

REGENXBIO

*Data cut August 29, 2022; ^ Data cut November 20, 2023
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.

The majority of patients with DR are lost to follow-up

Patients with diabetic retinopathy experience greater obstacles to care due to inherent differences in disease symptoms onset and risk factors, which can be alleviated by a one-time treatment

Majority of patients with diabetes will **develop diabetic retinopathy**[1]

Leading causes **of blindness and vision loss** in working age adults (45-50)

Affects **both eyes at the same time** in majority of patients

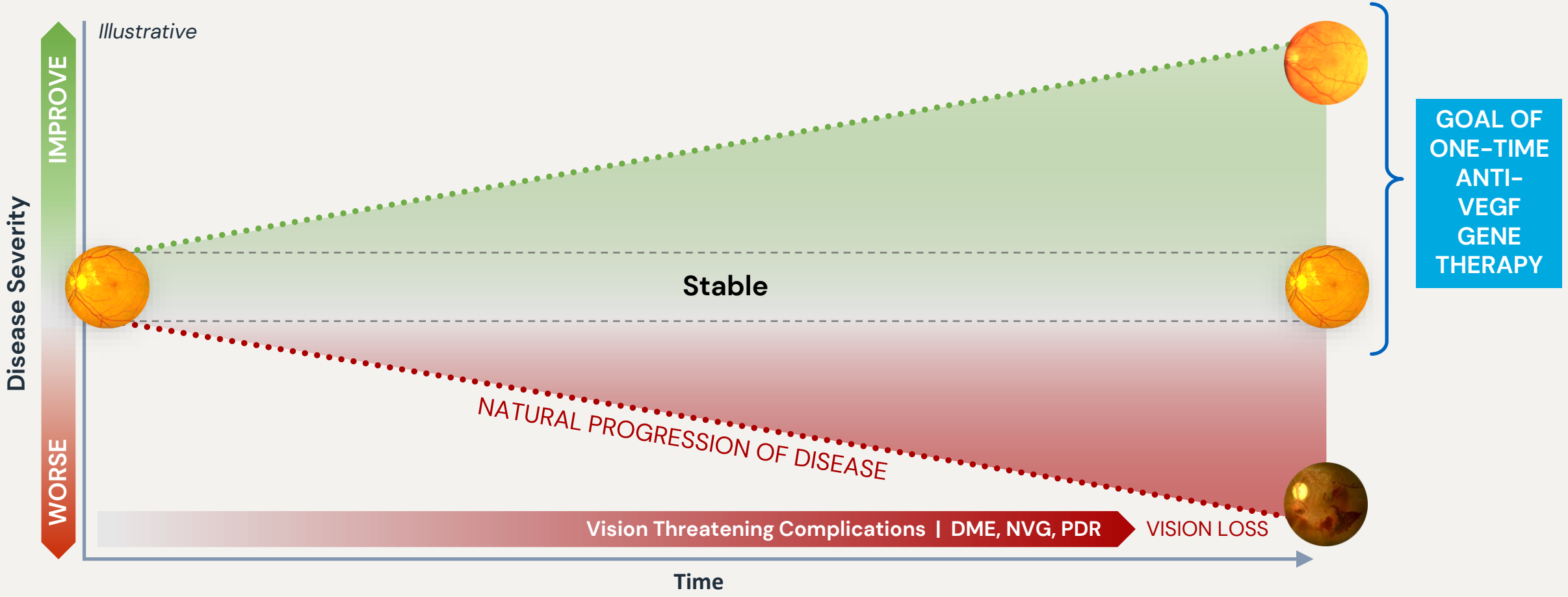
Over **50% of patients** with Diabetic retinopathy are **lost to follow up**[4]

A one-time, in office treatment represents an ideal treatment strategy for patients with DR

REGENXBIO

[1]2019 AAO Preferred Practice Patterns for Diabetic Retinopathy; [2]Vijan S, et al. The Impact of Diabetes on Workforce Participation: Results from a National Household Sample. *Health Serv Res.* December 2004; [3]Health and Human Services Healthy People 2030 Initiative https://health.gov/healthypeople/objectives-and-data/browse-objectives/diabetes/increase-proportion-adults-diabetes-who-have-yearly-eye-exam-d-04. Accessed June 27, 2023; [4]Green M, Tien T, Ness S. Predictors of loss to follow up in patients being treated for proliferative diabetic retinopathy. *Am J Ophthalmol.* March 31, 2020.

One time, in-office injection of gene therapy could potentially provide long-lasting improvement in DR severity and reduce risk of vision-threatening complications



DME = Diabetic Macular Edema. NVG = Neovascular Glaucoma. DR = Proliferative Diabetic Retinopathy.

ABBV-RGX-314 SC DR & DME: Phase II ALTITUDE® trial

Study Overview

- ~130 subjects
- Key Outcome measures:
  - Change in DRSS (Diabetic Retinopathy Severity Scale)
  - Safety and tolerability of ABBV-RGX-314
  - Development of DR-related ocular complications

Data Readouts

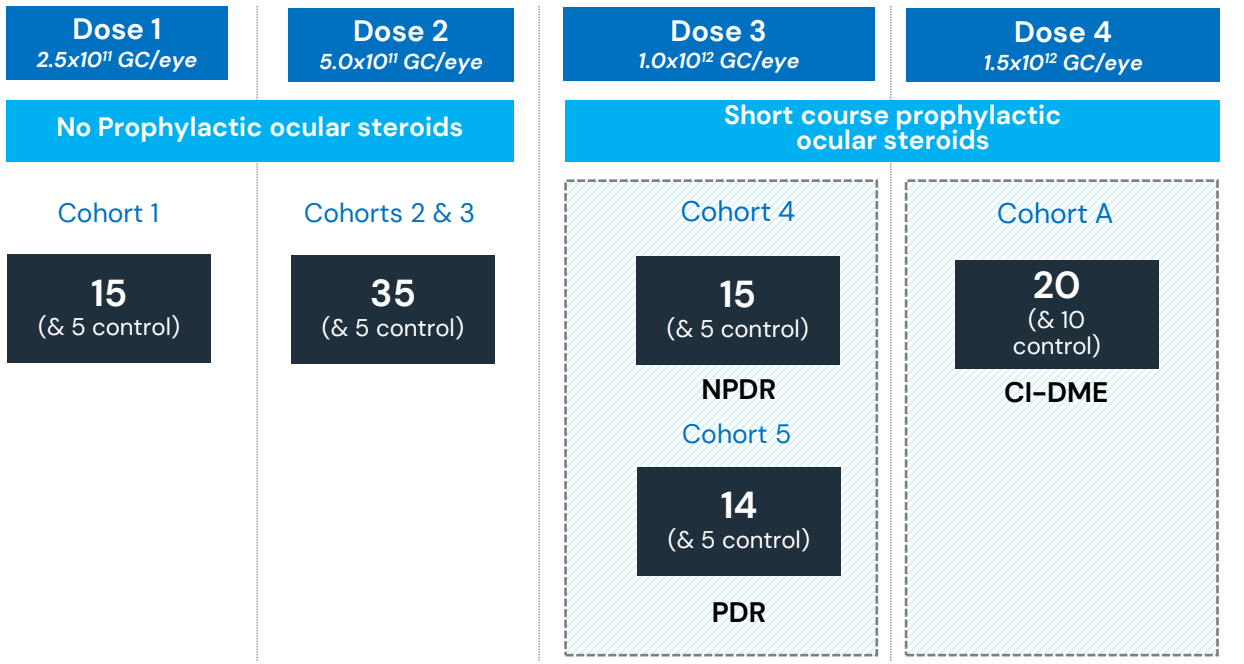
Latest Readouts

- Cohorts 1-3 (DL1-2) at 1 year
- Cohort 4-5 (DL3) at 11 -24 weeks safety, with prophylactic topical steroids

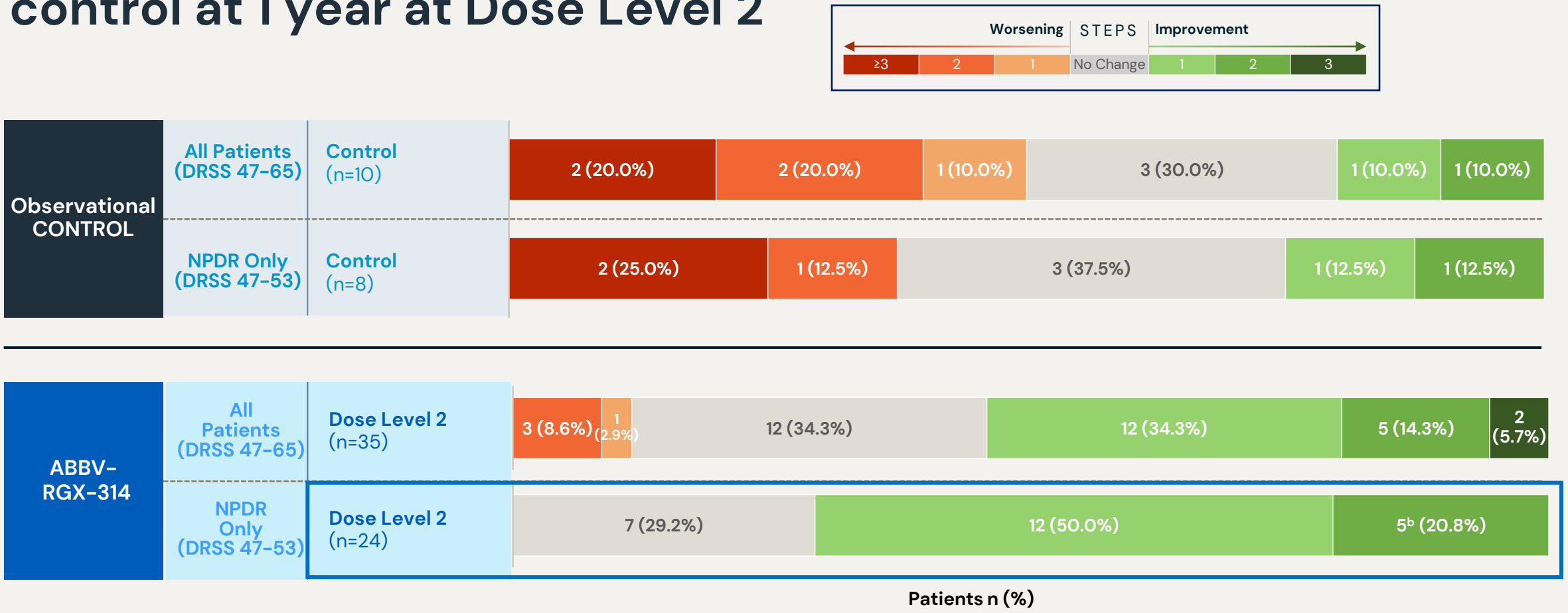
Pivotal Trial Initiation

- Design and evaluation of two pivotal trials is ongoing; initiation of pivotal trial expected 1H 2025

Study Design

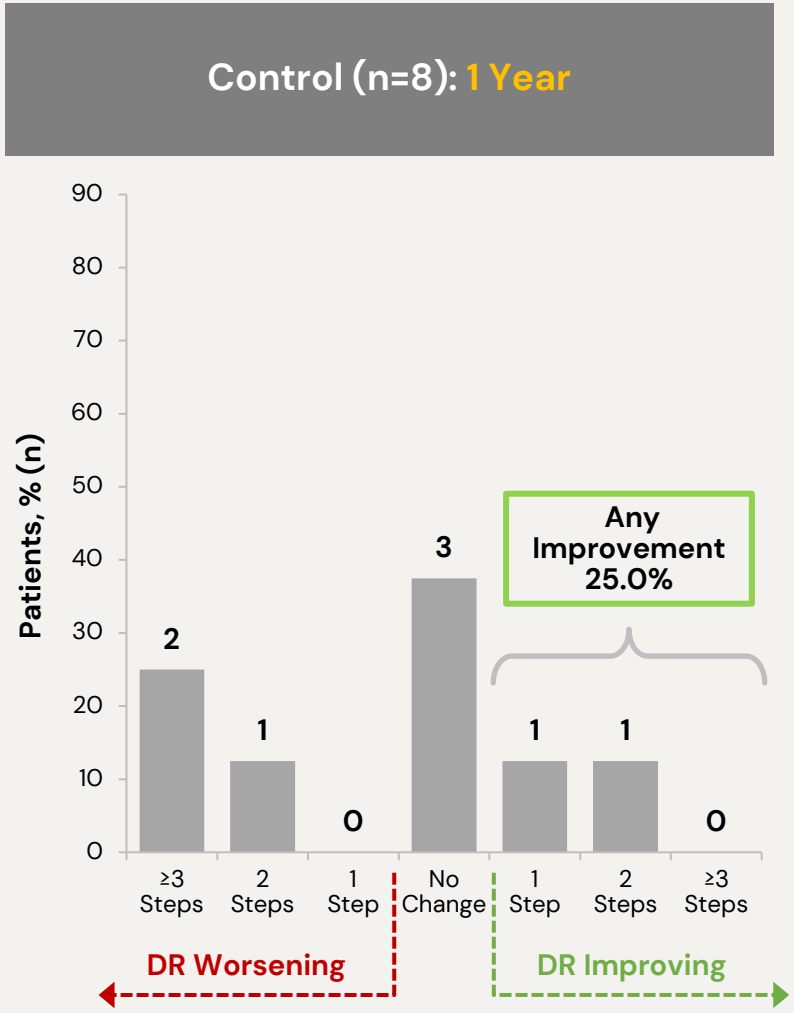


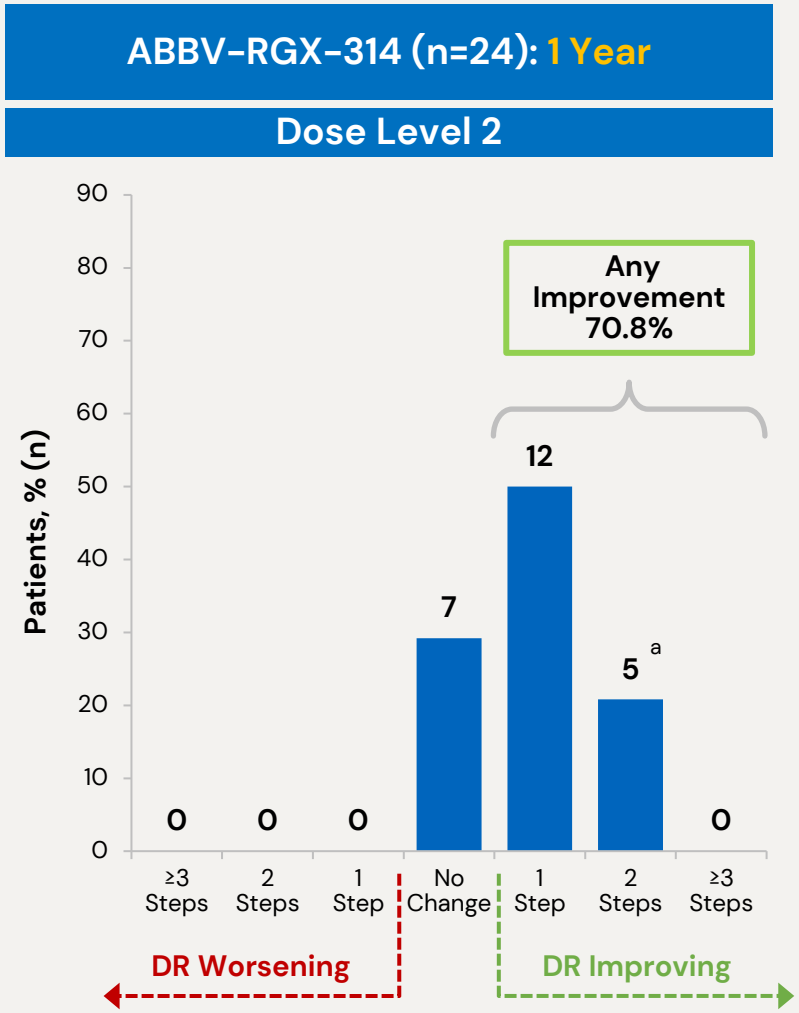
ALTITUDE: Summary of DRSS change compared to control at 1 year at Dose Level 2



Data cut: September 25, 2023.

ALTITUDE: Change in DRSS at 1 year at Dose Level 2– NPDR only (DRSS 47–53)

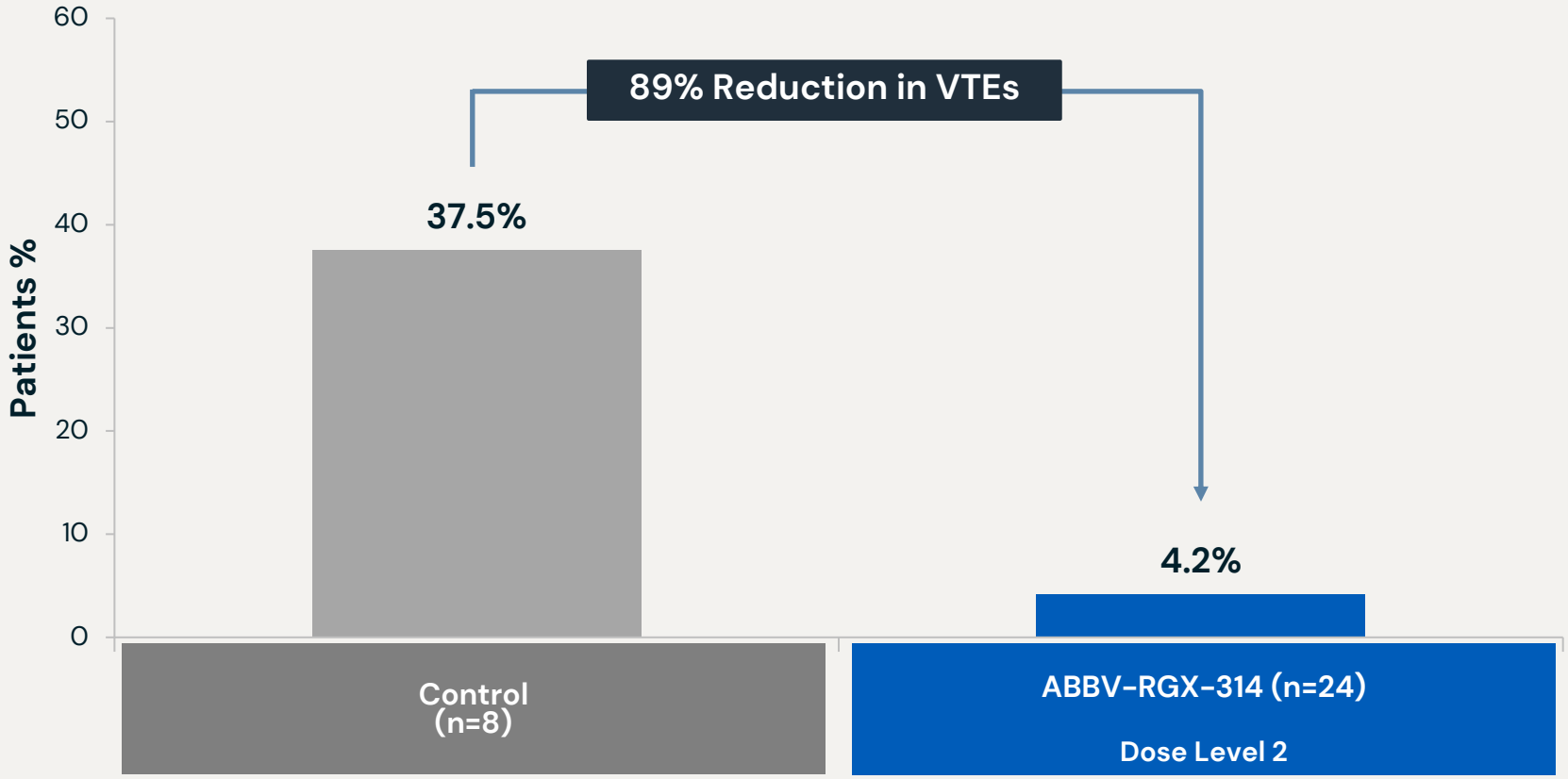




Data cut: September 25, 2023.

ALTITUDE: Vision-threatening events (VTEs) through year 1 at Dose Level 2– NPDR only (DRSS 47-53)

ABBV-RGX-314 treatment reduced VTEs compared to control group through 1 year



Data cut: September 25, 2023.
CI-DME: Center-Involved Diabetic Macular Edema; PDR: Proliferative Diabetic Retinopathy; ASNV: Anterior Segment Neovascularization; VTCs: Vision-Threatening Complications; VTEs: Vision-Threatening Events ; VTEs = VTCs + CI-DME; VTCs could include PDR or ASNV. No cases of ASNV were reported.

ALTITUDE: Interim results summary

Safety

- Suprachoroidal ABBV-RGX-314 continues to be **well-tolerated in dose levels 1 - 3**

Efficacy Endpoints: 1 Year Results for Dose Levels 1 and 2

- **One-time in-office injection** of investigational ABBV-RGX-314 demonstrated clinically meaningful improvements in disease severity and reduction of VTEs in NPDR patients
- **In Dose Level 2 patients with baseline NPDR (n=24):**
  - **100%** demonstrated stable to improved disease severity
    - 70.8% achieved any disease improvement vs. 25.0 % in Control
    - 0% worsened ≥2 steps vs. 37.5 % in Control
  - 4.2% developed VTEs vs. 37.5% in Control

Dose Level 2 prevented disease progression in all NPDR patients and reduced vision-threatening events by 89%.

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Data cut: September 25, 2023.
No drug-related SAEs. Cases of intraocular inflammation were mild and resolved with topical steroids. There were no cases of chorioretinitis, vasculitis, occlusion or hypotony. Common treatment emergent adverse events included conjunctival hyperemia, conjunctival hemorrhage, and episcleritis.

ABBV-RGX-314 SCS wAMD: Phase II AAVIATE® trial

Study Overview

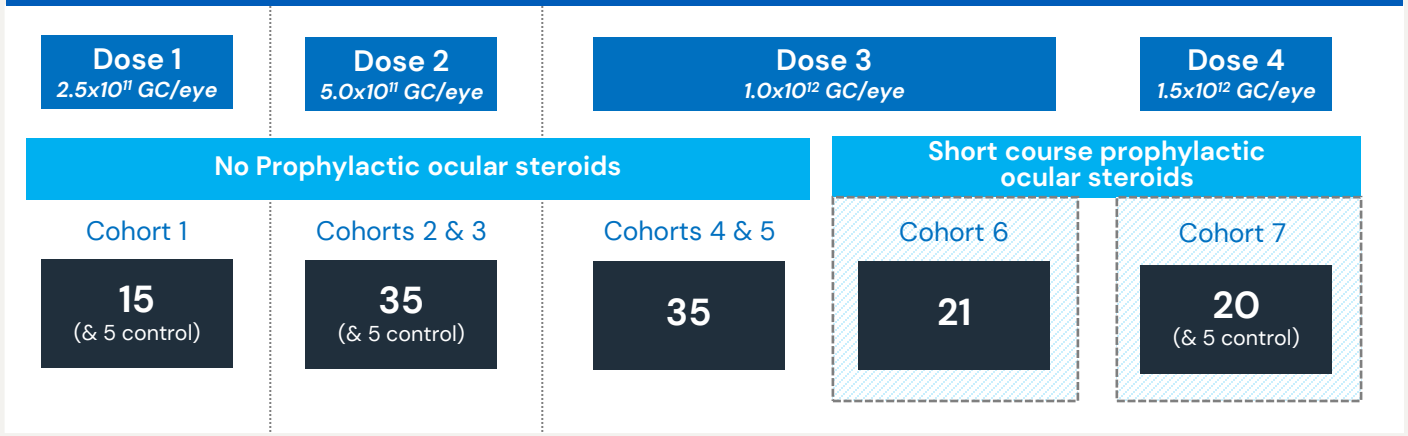
- ~140 subjects
- Key Outcome measures:
  - Visual acuity
  - Safety and tolerability
  - Retinal anatomy
  - Additional anti-VEGF injections post ABBV-RGX-314

Data Readouts

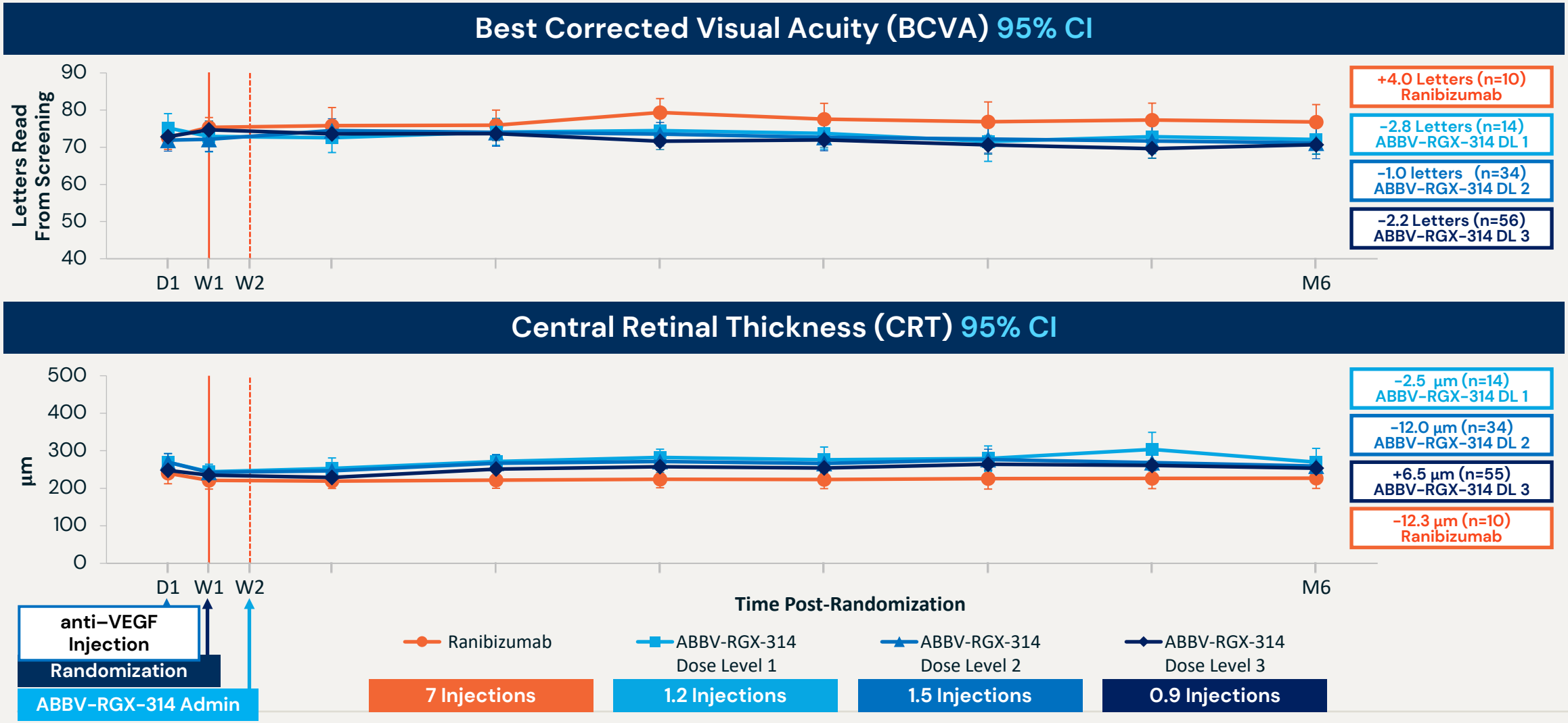
Latest Readouts

- Cohort 1-4 (DL1-3) at 6 months
- Cohort 1-6 (DL1-3) at 6 months

Study Design



AAVIATE: Dose Levels 1–3: Mean BCVA and CRT from Day 1 Through Month 6



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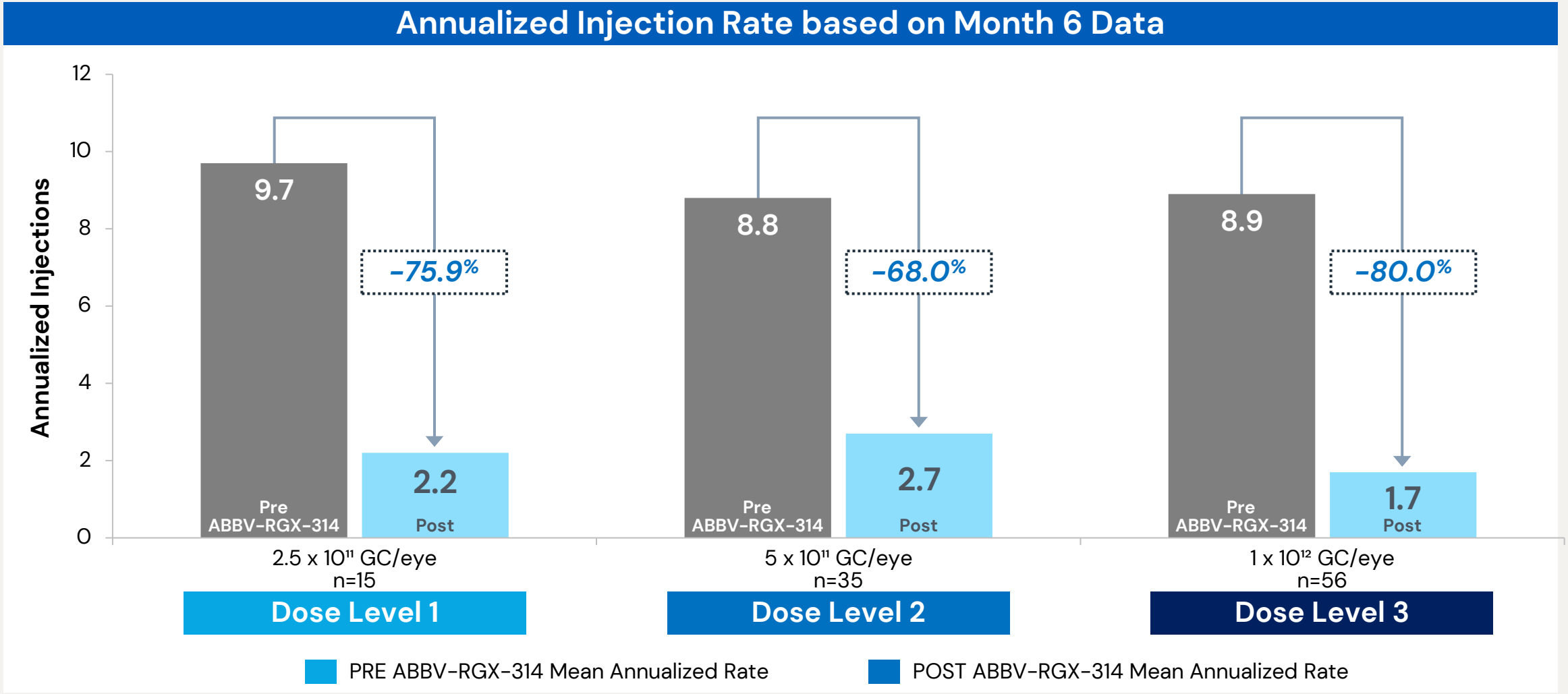
Data cut: November 06, 2023.
Cohort 6 (DL3) patients were randomized at D1 and received additional anti–VEGF run-in injections at W-4 and W4.

AAVIATE: Mean change in annualized injection rate pre- and post- ABBV-RGX-314 by dose level



Data cut: November 06, 2023.
Cohort 6 (DL3) patients were randomized at D1 and received additional anti-VEGF run-in injections at W-4 and W4.

AAVIATE: Interim results summary

ABBV-RGX-314 Dose Levels 1-3 (n=106): 6 Month Results

- Suprachoroidal ABBV-RGX-314 has been well-tolerated
- Zero cases of IOI in subset of Dose Level 3 with short-course prophylactic topical steroids
- ABBV-RGX-314 continues to demonstrate stable vision and retinal thickness, with a meaningful reduction in treatment burden with the highest reduction seen in Dose Level 3:
  - 80% reduction in annualized injection rate
  - 50% injection-free

Dose Level 3 continues to show encouraging interim results with a well-tolerated profile, including zero cases of IOI with short-course prophylactic topical steroids

Data cut: November 06, 2023.
No drug-related SAEs. Common treatment emergent adverse events included episcleritis, conjunctival hyperemia, intraocular inflammation (IOI), conjunctival hemorrhage and increased intraocular pressure.
Cases of IOI were mild to moderate and resolved with topical steroids. There were no cases of chorioretinitis, vasculitis, occlusion or hypotony.

REGENXBIO executive team

Curran Simpson
President and CEO

Steve Pakola, M.D.
EVP, Chief Medical Officer

Olivier Danos, Ph.D.
EVP, Chief Scientific Officer

Mitchell Chan
EVP, Chief Financial Officer

Shiva Fritsch
EVP, Chief Communications & People Officer

Patrick Christmas
EVP, Chief Legal Officer

Ram Palanki, Pharm.D.
EVP, Commercial Strategy & Operations

Late-stage pipeline in multi-billion dollar commercial markets

Retina franchise partnered with AbbVie

Wet AMD: dual route of administration strategy to expand access; clinical POC established with sustained vision & safety up to 4 years post-dosing

Diabetic retinopathy: pivotal trial initiation* expected 1H 2025 for significant untapped market

Expecting to commercialize the first gene therapy for MPS II in 2026

RGX-121 represents the first potential one-time treatment for Hunter syndrome and only treatment to directly address neurocognitive decline; rolling BLA submission expected to be completed in Q1 2025

Potential best-in-class treatment for Duchenne Muscular Dystrophy (DMD)

RGX-202 delivers a microdystrophin that is closest in length and functional capabilities to full-length dystrophin of commercial or investigational gene therapies; BLA using the accelerated approval pathway expected in 2026

Strong balance sheet expected to fund operational runway into 2026

*AbbVie milestone payments expected for DR program

Thank You