REGENXBIO®

Corporate Presentation

Leader in AAV Gene Therapy

April 2024

Forward-looking Statements

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

Leading pipeline of AAV Therapeutics: differentiated product candidates addressing large commercial opportunities and value generation.

Prioritized pipeline expected to advance into pivotal stage and first BLA filing in 2024.

Strategic, cost-sharing partnership with AbbVie to develop and commercialize AAV Therapeutics for retinal diseases.

Strong balance sheet expected to fund operational runway into 2026.

REGENXBIO's internal pipeline

Indication		Description	Phase I	Phase II	Pivotal	Approval
Retinal Disease						
wet AMD subretinal delivery	ABBV-RGX-314 abbvie eye care collaboration	Anti-VEGF				Global regulatory submissions late 2025 – H1 2026
wet AMD suprachoroidal delivery						
Diabetic retinopathy suprachoroidal delivery						
Neuromuscular Disease						
Duchenne	RGX-202	Novel microdystrophin				
Neurodegenerative Disease						
MPS II	RGX-121	Iduronate-2-sulfatase enzyme				BLA filing in 2024

Strategic, cost-sharing partnership with AbbVie to develop and commercialize ABBV-RGX-314 for wet AMD and diabetic retinopathy

Leadership and expertise in AAV and retinal gene therapy

Strong in-house capabilities of AAV manufacturing

Leading eye care company

Global development and commercial infrastructure

Details of Partnership

- **$370 million upfront payment** with up to **$1.38 billion in additional development, regulatory and commercial milestones**
- Collaboration for the development and commercialization of ABBV-RGX-314 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

Retinal Disease: An estimated $17B global market within 5 years[1]

- wAMD patient population expected to **increase to 5.7M** in US, EU, JP in the next 5 years[1]
- Most wAMD patients are required to receive **anti-VEGF injections every 4–16 weeks** for the duration of their disease
- In real world, **high treatment burden leads to undertreatment** and vision loss over time

RETINAL DISEASE MARKET IN NEXT 5 YEARS[1]

ANNUAL US RETINA ANTI-VEGF wAMD MARKET[2-4]

$4.5B	800K	4M
Branded Anti-VEGF Market	wAMD Patients Receiving Treatment	Anti-VEGF Injections

2.5K US RETINA SPECIALISTS[5]

ANNUAL US RETINA SURGICAL LANDSCAPE[6-7]

90%	4K	400K
of Retina Specialists Are Surgically Trained	Retina Surgical Sites	Vitrectomy Surgeries

1. Market Scope 2022 Retina Pharmaceuticals Report; 2. Evaluate Ltd 2023; 3. 2022 IQVIA Data; 4. Market Scope Q4-2022 Retina Quarterly Summary; 5. Ibis World 2021; 6. ASRS PAT Survey 2022; 7. 2022 IQVIA Data; 8. Market Scope 2023 Retinal Device Report
EU = Western Europe; wAMD = wet age-related macular degeneration
aVEGF US market comprised of ~50% wAMD injections; retinal disease market includes branded ocular aVEGFs, off-label cancer drugs, biosimilars, gene/cellular therapies, sustained delivery, other

Diabetic retinopathy is a global public health problem

20M

is the expected DR patient population in US, EU, JP in the next 5 years[1]

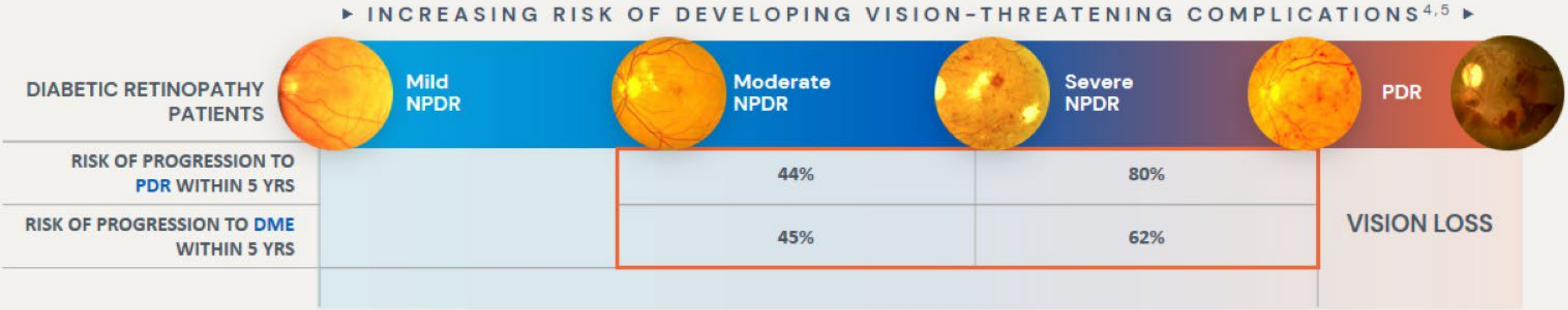
<1%

of patients with early DR are treated due to high treatment burden[3]

45–50 YRS

Median age of disease onset

Early treatment with longer lasting therapy can potentially modify and prevent disease progression



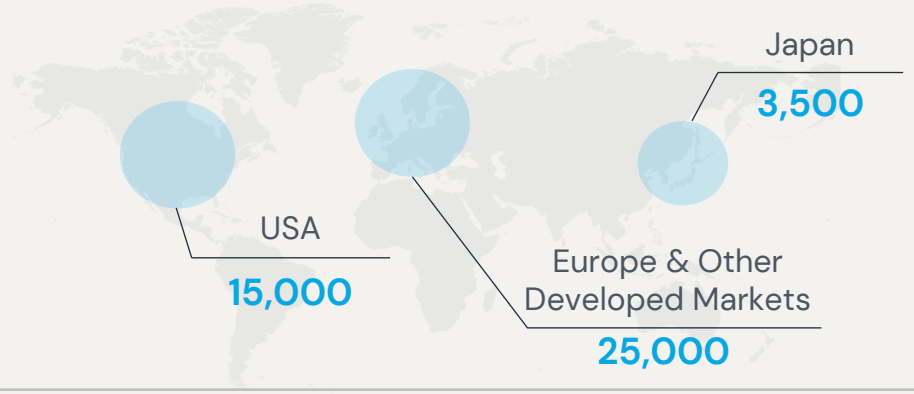
1.DR Market, Reports and Data 2022; 2. Market Scope 2022 Retinal Pharmaceuticals; 3. Market Scope Q4-2022 Retina Quarterly Summary Report; 4. ETDRS Trial Report 12, 1991; 5. Moshfeghi, 2020
DR = diabetic retinopathy; NPDR = non-proliferative diabetic retinopathy; PDR = proliferative diabetic retinopathy; DME = diabetic macular edema

Duchenne Muscular Dystrophy (DMD) opportunity: an estimated $7B global market within 5 years[6]

DUCHENNE MARKET IN THE NEXT 5 YEARS[6]

$7B

PATIENTS IN MAJOR GLOBAL MARKETS[1-5]

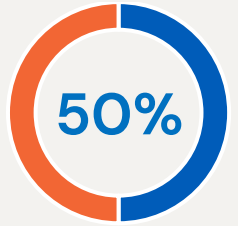


Limited effective treatments to delay loss of ambulation and death[5]

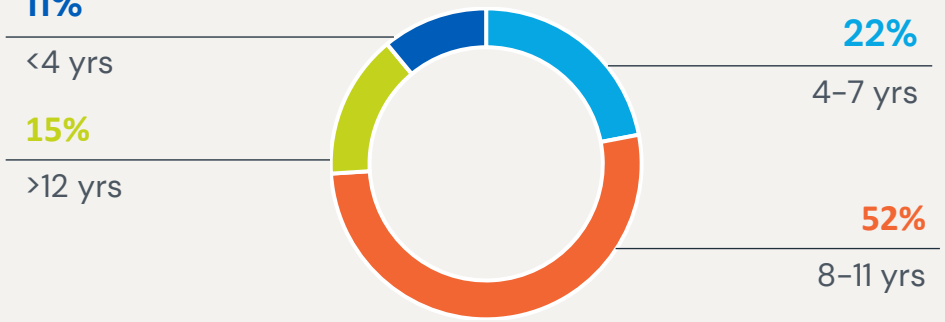
MEDIAN SURVIVAL AGE[4]

Mid to late 20'S

% 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

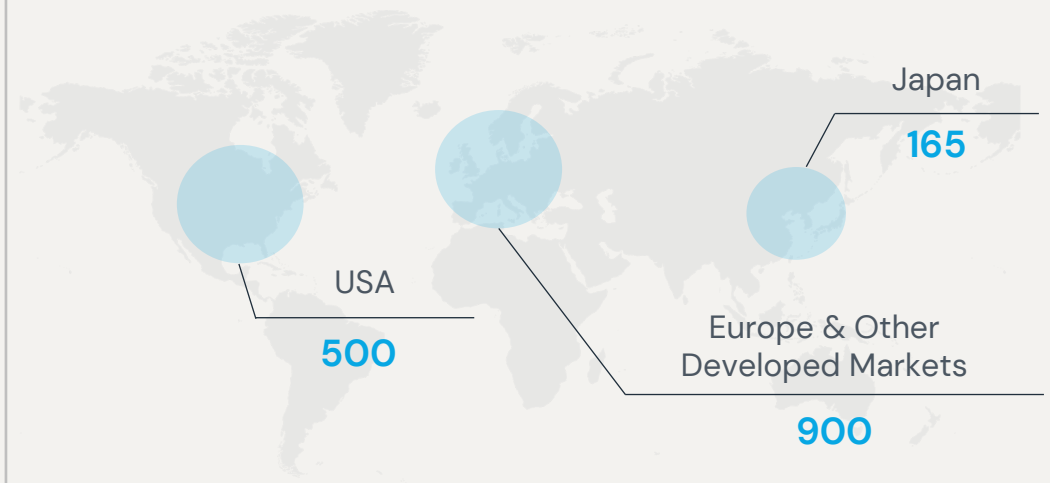
Mucopolysaccharidosis Type II (MPS II) opportunity: an estimated $1B global market within 5 years[9]

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]

REGENXBIO

^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

ABBV-RGX-314

For wet-AMD and Diabetic Retinopathy

ABBV-RGX-314 clinical studies summary

Suprachoroidal (SCS) wAMD & DR

Clinical Study		Status	Region	Size	Description	Planned Readout
wAMD	*Phase II* AAVIATE	Enrolled	US	116	Dose finding	2024
DR	*Phase II* ALTITUDE	Enrolled	US	115	Dose finding	2024

Subretinal (SR) wAMD

Clinical Study	Status	Region	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	Enrolling	US	20	Open label, bilateral safety	2024
Long Term Follow Up	Enrolling	Global	-	Supports Durability	2024
Phase I/IIa	Enrolled	US	42	Dose finding	

ABBV-RGX-314 SR wAMD: Clinical program overview

Phase I/IIa Study

42 subjects
5 yr LTFU
supports pivotal program

Enrolled

2 Pivotal Studies

ATMOSPHERE
ASCENT

1,200 subjects,
Randomized Controlled
Global: US, Europe, Japan, Israel
2 Pivotal Doses

Enrolling

Regulatory Submissions

Late 2025
through
1H 2026

Phase II Bioreactor Bridging Study

60 subjects, Open Label
2 Pivotal Doses
Data supports pivotal dose levels and platform process

Enrolled

Fellow Eye Study

Up to 20 subjects
Open Label
Pivotal High Dose

Enrolling

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

Study Overview

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

Data Readouts

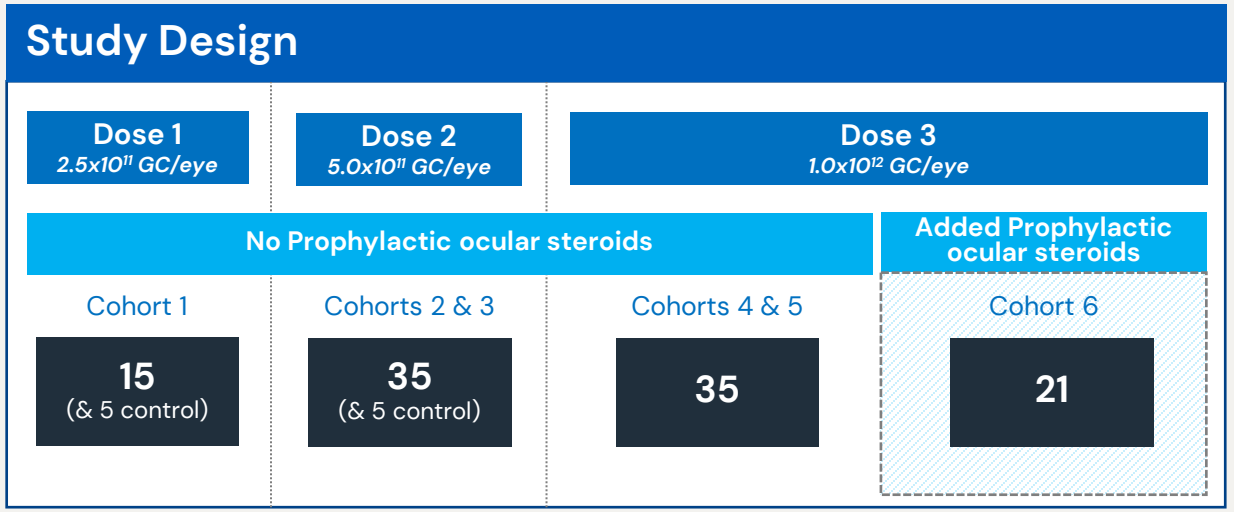
Latest Readouts

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

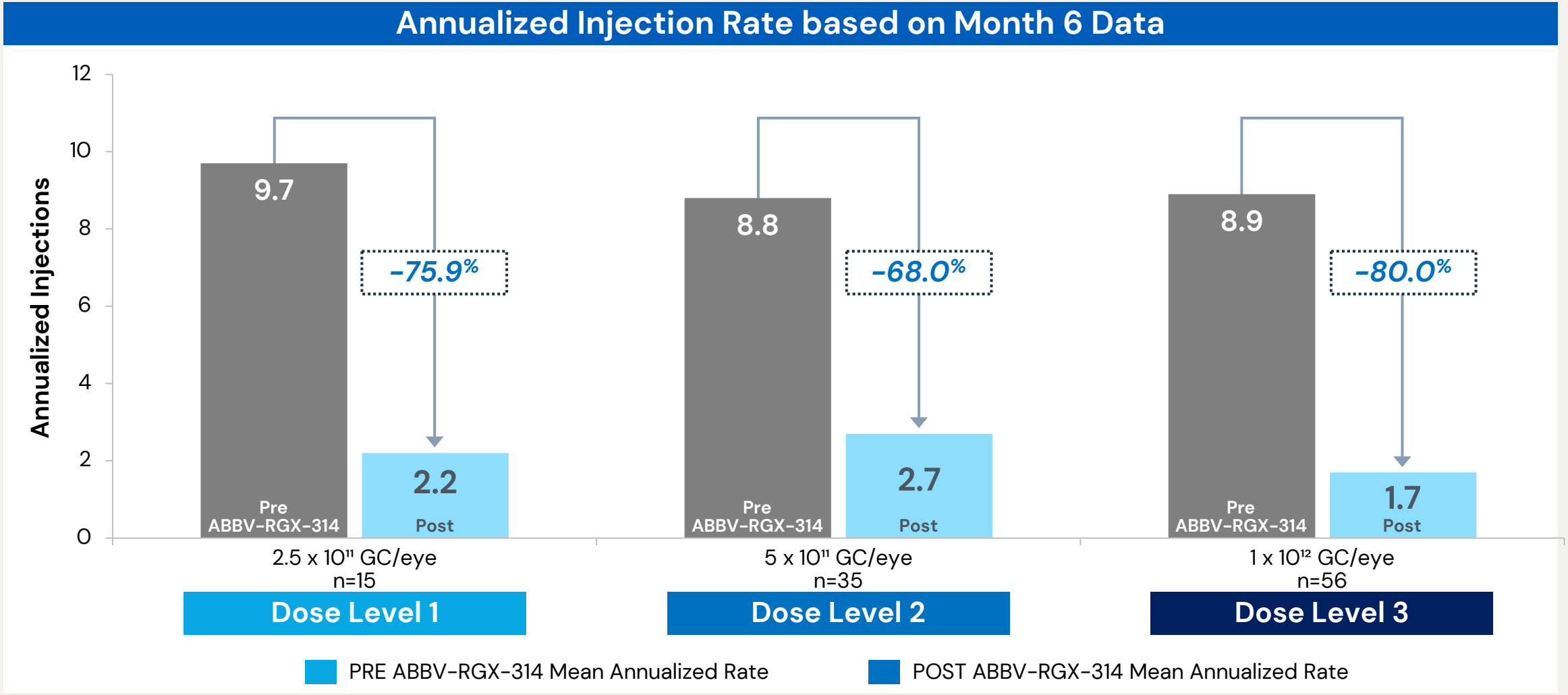
Next Planned Update

- 2024

Study Design



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



Data cut: November 06, 2023.

Summary of interim results from the Phase II AAVIATE® nAMD study

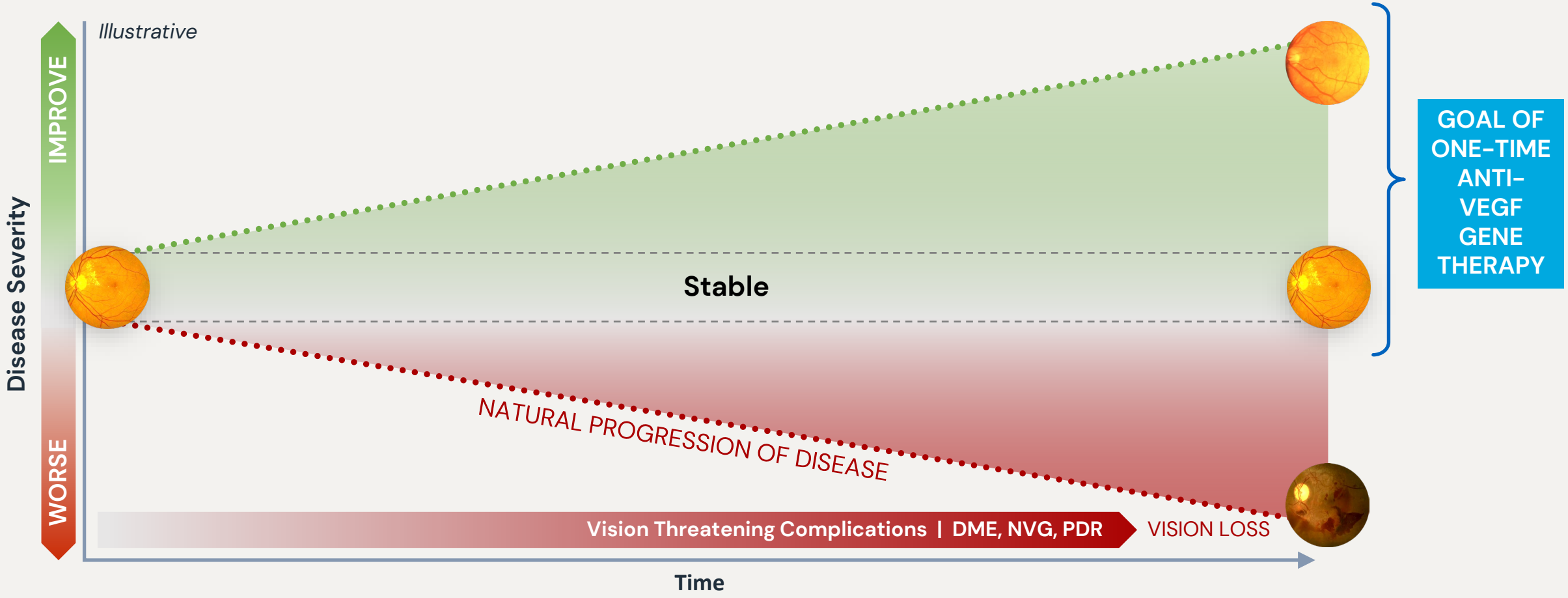
ABBV-RGX-314 Dose Levels 1-3 (n=106): 6 Month Results

- 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.

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.
PDR = Proliferative Diabetic Retinopathy.

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

Study Overview

- 99 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

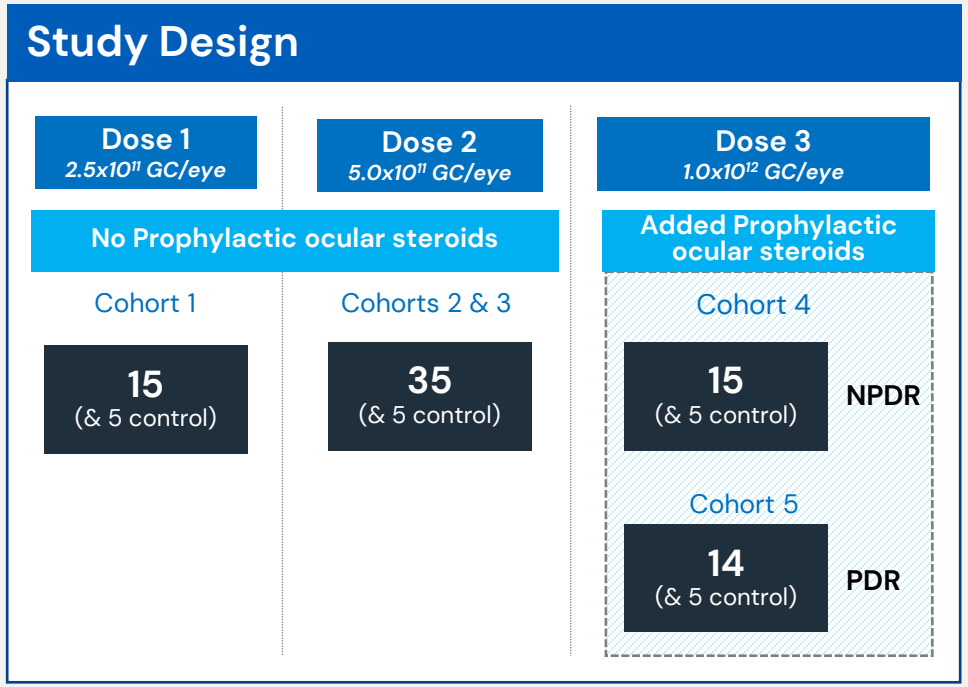
Latest Readouts

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

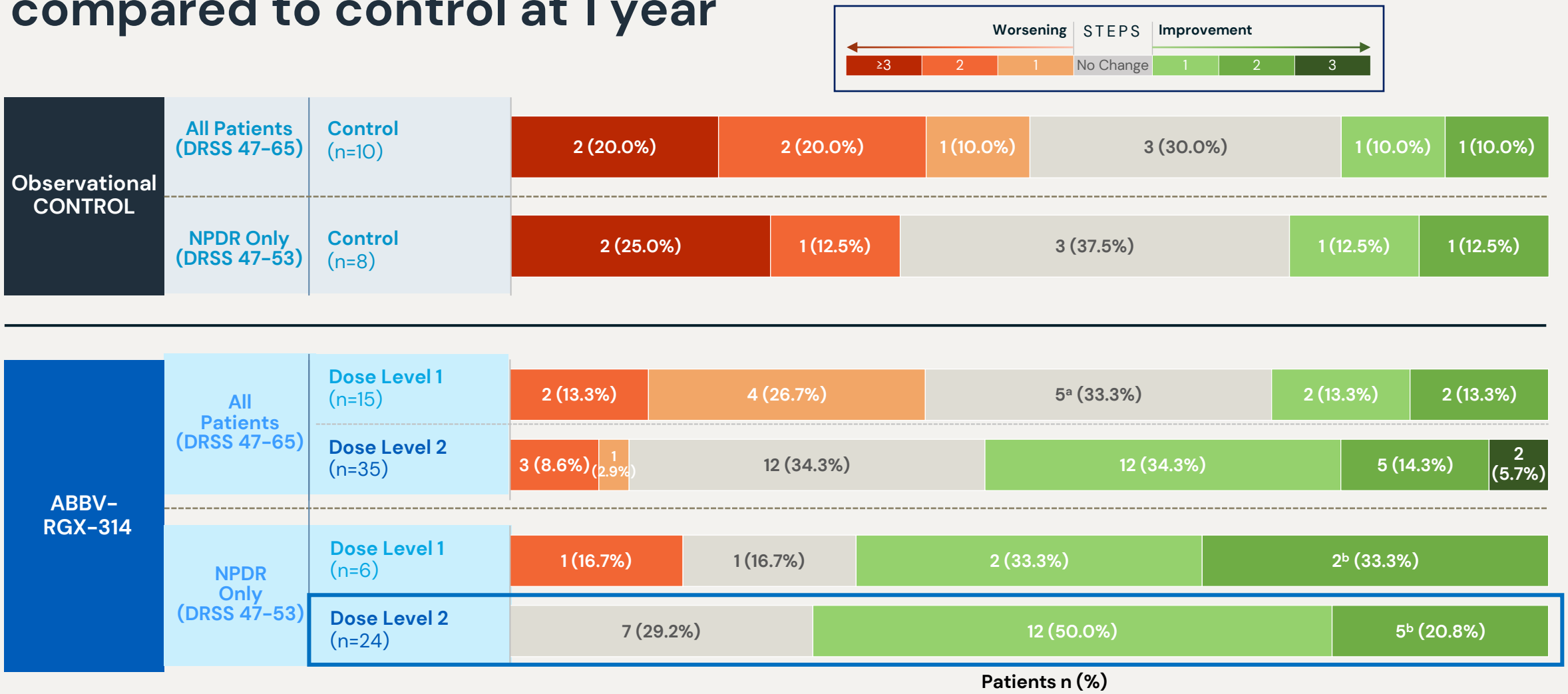
Next Planned Update

- 2024

Study Design

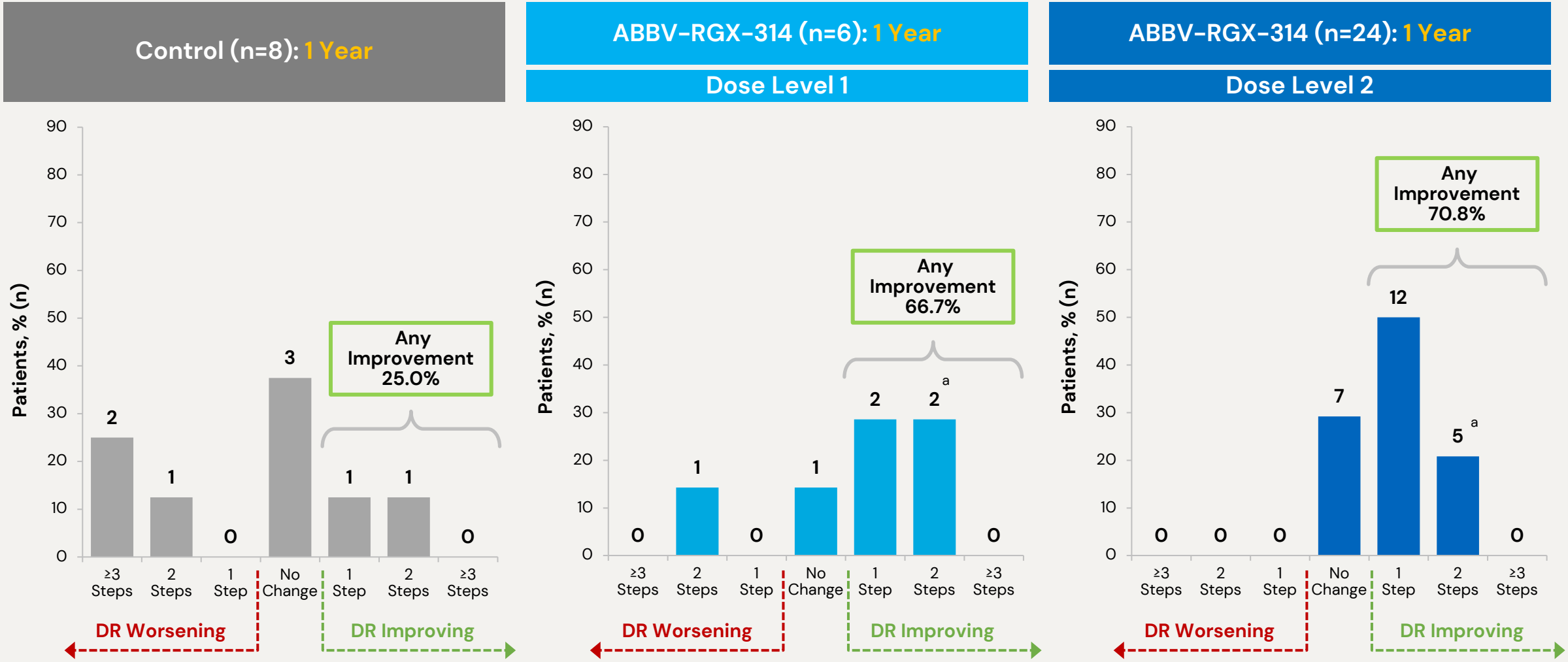


Summary of DRSS change with dose levels 1 and 2 compared to control at 1 year



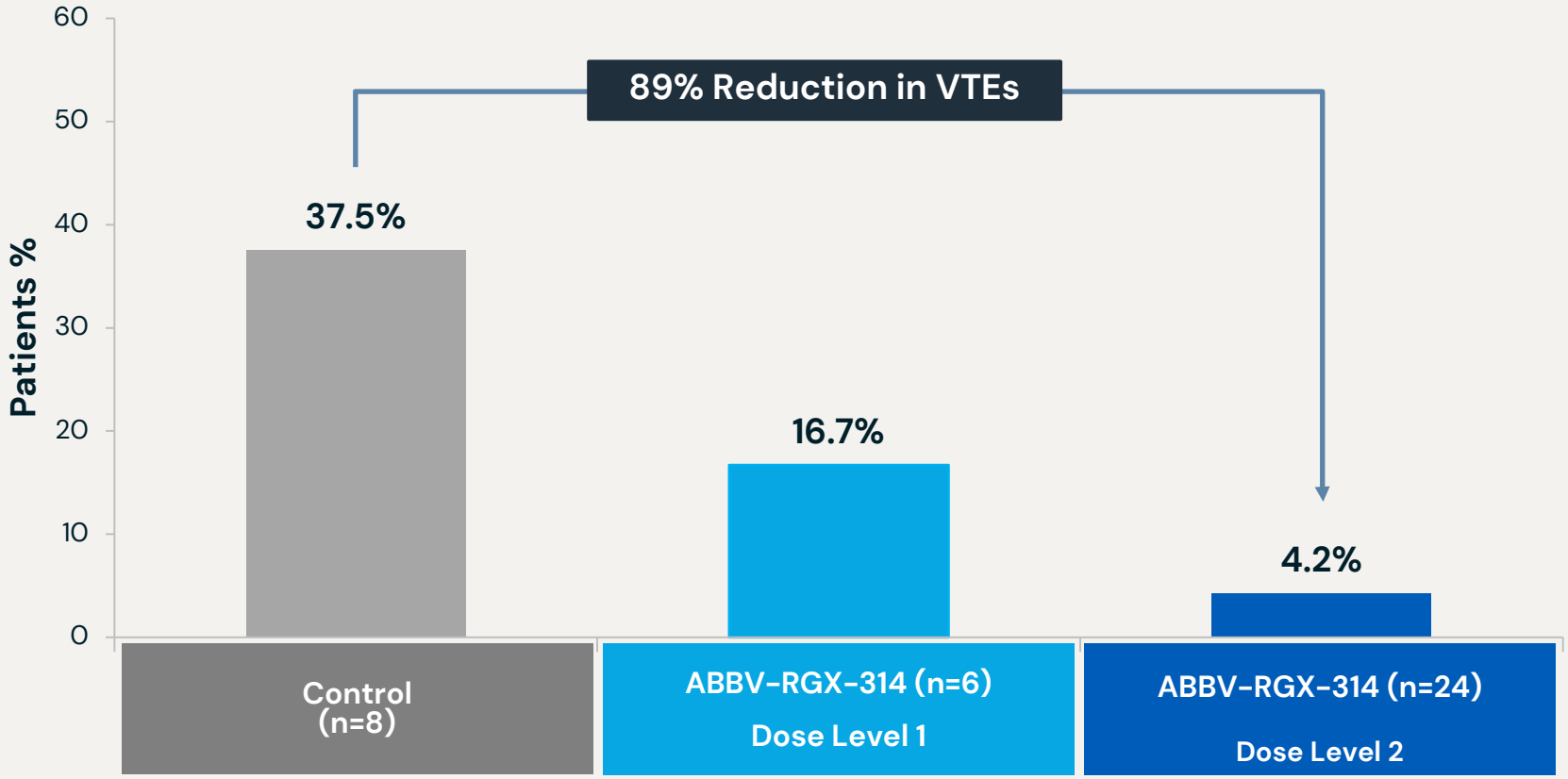
a. Data cut: September 25, 2023.

Change in DRSS at 1 year by dose level – NPDR only (DRSS 47–53)



Vision-threatening events (VTEs) through year 1 by dose level – 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 results summary

Safety

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

Efficacy Endpoints

- **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%

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.

RGX-202

For Duchenne Muscular Dystrophy

Duchenne muscular dystrophy: disease overview

Duchenne muscular dystrophy is a neuromuscular degenerative disease of high unmet medical needs

1

Presentation

- Progressive degenerative muscle disease
  - Muscle weakness, atrophy, fatigue and respiratory failure
  - Cardiomyopathy
- Symptoms present around 4 years and worsen rapidly
- Diagnosis: Genetic testing, muscle biopsy for dystrophin
- SOC: Corticosteroids, physiotherapy, orthopedic appliances, respiratory support; Exondys for subset

2

Pathophysiology

- Partial or complete lack of dystrophin
- Muscle cell death associated with progressive tissue degeneration
- Affects skeletal muscles used for movement, diaphragm and cardiac muscle
- Loss of ambulation in the early teens
- Premature death by 20s-30s

3

Etiology

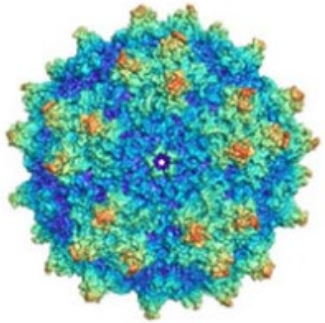
- X-linked recessive mutations in DMD gene
  - ~60% of mutations are insertions or deletions that lead to frameshift errors downstream
  - ~40% are point mutations or small frameshift rearrangements
  - Mutations that lead to abnormal version of dystrophin that retain some function cause Becker muscular dystrophy

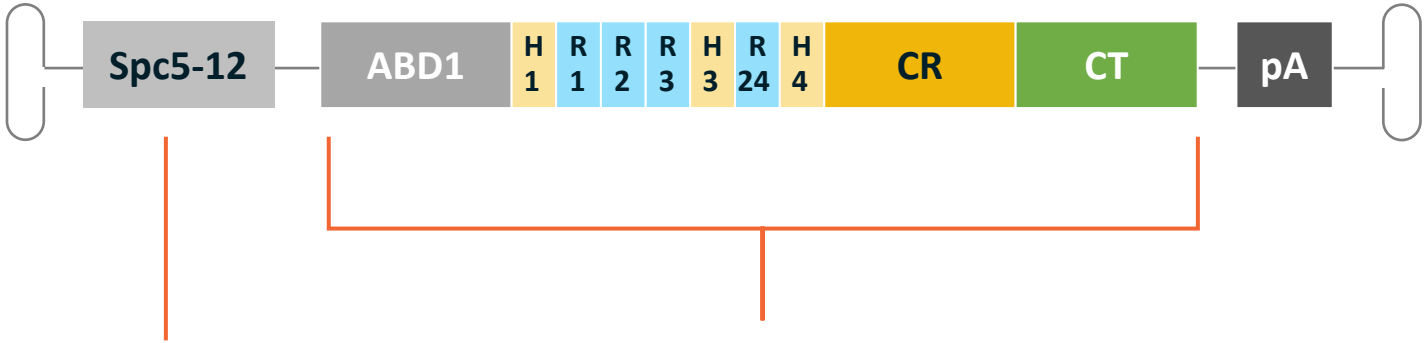
4

Epidemiology

- 1 in 3,500 to 5,000 newborn males
- 300,000 boys living with DMD worldwide
  - ~32,000 boys living with DMD in G7 countries
  - ~10,000-15,000 boys living with DMD in US

RGX-202 is an investigational gene therapy using a novel microdystrophin construct for the treatment of Duchenne





AAV8 vector

- Efficiently transfers genes to skeletal and heart muscle[1, 2, 3 ,4]
- Utilized in numerous clinical trials
- 2000-liter commercial process available at REGENXBIO

Spc5-12 promoter

- Targets expression in skeletal and heart muscle[1,2]

Microdystrophin Transgene

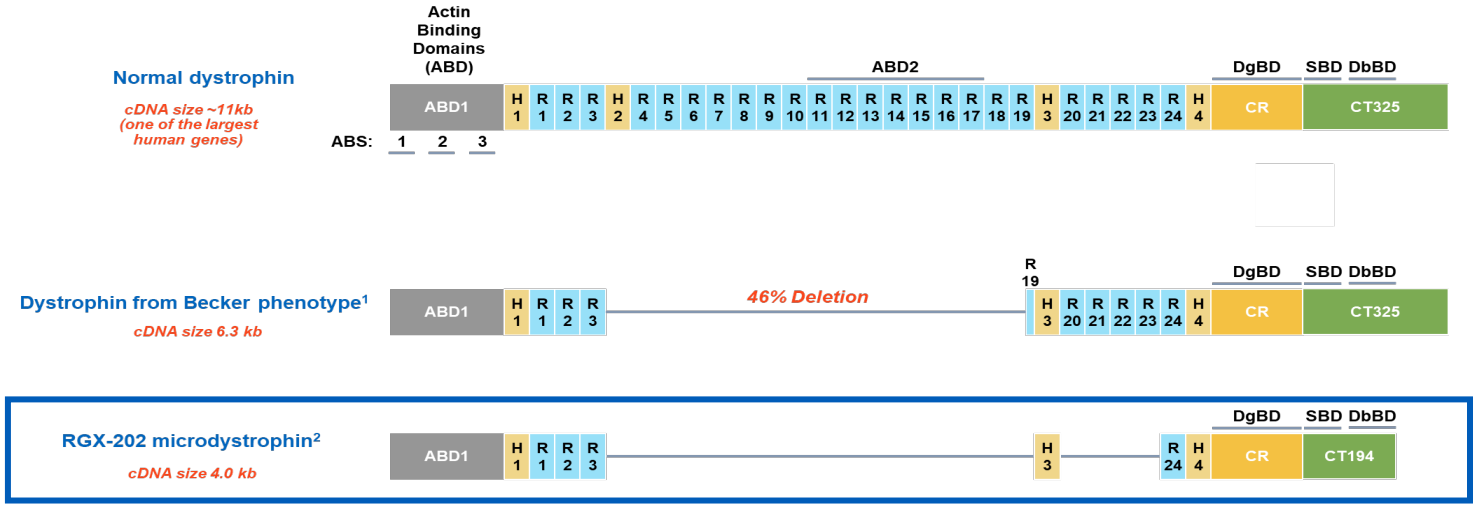
- Designed to encode a protein that retains key elements of full-length dystrophin including the extended coding region of the C-Terminal (CT) domain
- Reduced CpG content to potentially minimize immunogenicity[5]
- Codon optimization to potentially increase protein expression

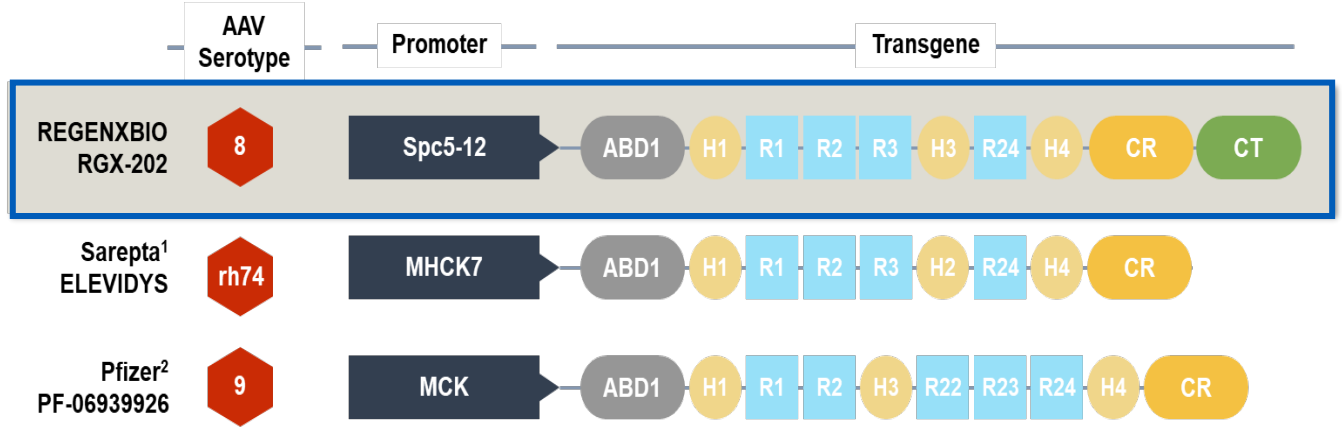
1. Le Guiner (2017) Nat Commun 2017
2. Li X (1999) Nat Biotechnol
3. Mack (2017) Mol Ther
4. Shieh (2019) ASGCT Virtual
5. Faust (2013) J Clin Invest

Abbreviations: ABD1: Actin binding domains; CR: Cystein rich domain; CT: carboxyl terminus; pA: polyA signal; H: hinge; R: rod

RGX-202 is novel among current class of AAV- microdystrophins

RGX-202 expresses a new, differentiated microdystrophin with important biology that is the most similar to a natural shortened dystrophin found in boys and men that protects muscles from degenerating





RGX-202 is the only microdystrophin designed to deliver a transgene that includes the functional elements of the C-Terminal (CT) domain found in naturally occurring dystrophin.

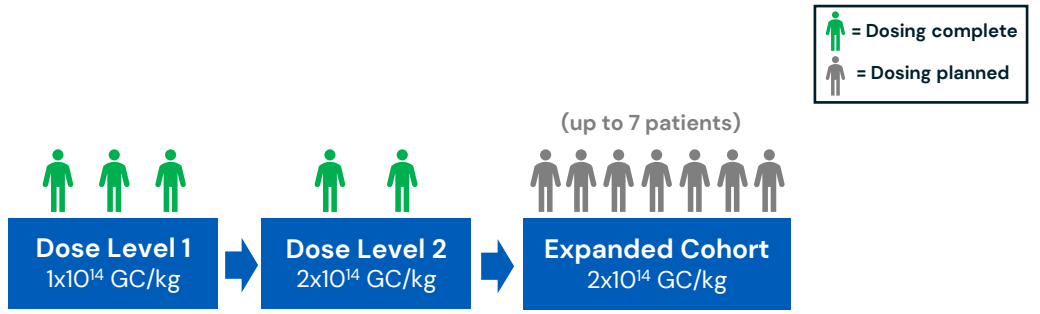
1. Harper (2002) Nat Med
2. Wang (2000) PNAS

RGX-202 Study Overview

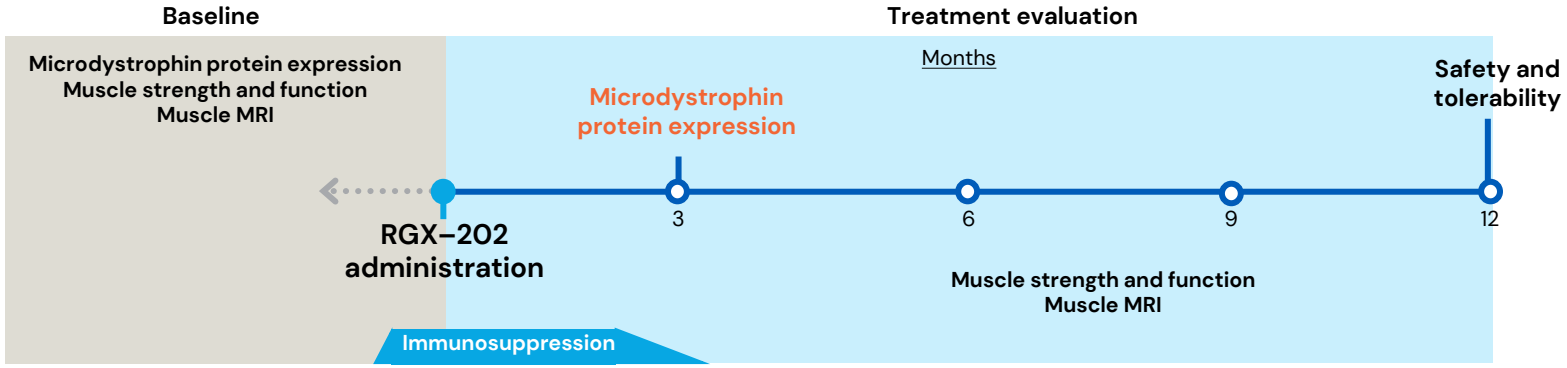
Key Eligibility Criteria

- **Boys aged 4 to 11 years** at screening
- **Genetically confirmed DMD** (mutations in exons 18 and above)
- **100-meter walk:** able to perform without assistive devices
- **No pre-existing antibodies** to the gene therapy (AAV8 capsid)

Study Plan



Administration and Assessments Timeline



RGX-202 has been well tolerated in 5 patients at both dose levels with no serious adverse events.
Age at dosing: 4 years 4 months – 12 years; post-administration follow up: 1 – 11 months

Interim data: Dose level 1

Dose Level 1

- Robust RGX-202 microdystrophin expression observed
- Serum CK levels meaningfully decreased, representative of improvement in muscle disease

Patient	Age at Dosing	RGX-202 Microdystrophin Western blot (Jess method) (% Normal Control)	CK Levels, week 10 (% reduction from baseline)
Dose Level 1 (1x1014 GC/kg)			
1	4 yrs 4 mos	38.8	-43
2	10 yrs 5 mos	11.1	-44
3	6 yrs 6 mos	83.4	-93

Microdystrophin expression adjusted for muscle content
Control was level of wild-type (normal) dystrophin in normal muscle
Muscle biopsies are collected from bicep at baseline and 3 months post RGX-202 administration
Elevated CK levels are correlated with muscle injury and are uniformly elevated in patients with Duchenne
Data cut date of February 23, 2024

Interim data: Dose level 2, 1st patient

Robust RGX-202 microdystrophin expression was observed at three months in 12-year-old, with comparable results obtained via Western Blot and LC-MS

RGX-202 Microdystrophin Expression

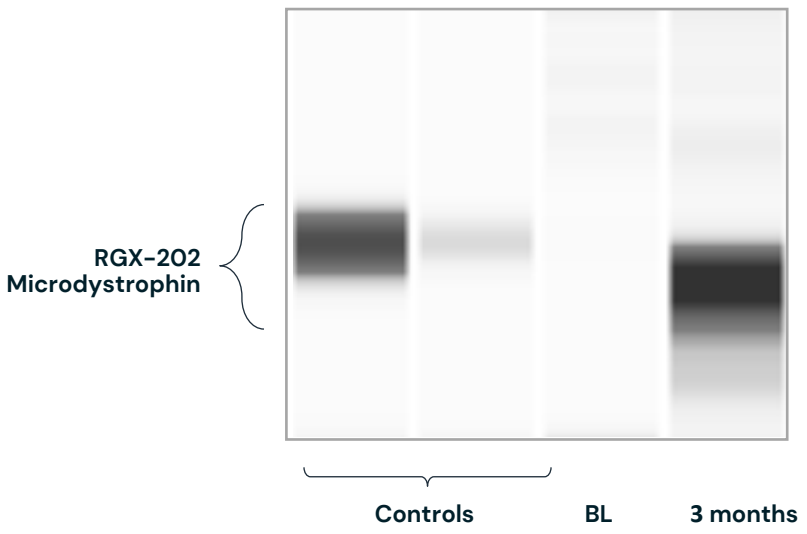
RGX-202 Microdystrophin (% Normal Control)	Patient 4 (12 yrs 0 mo 24.3 kg)
Western blot (Jess method)	75.7

CK levels

	Avg Baseline	Week 10
CK Levels (U/L)	13,131	2,983
% Reduction	-77	

Elevated CK levels are associated with muscle injury and are uniformly elevated in patients with Duchenne

Western Blot (Jess)



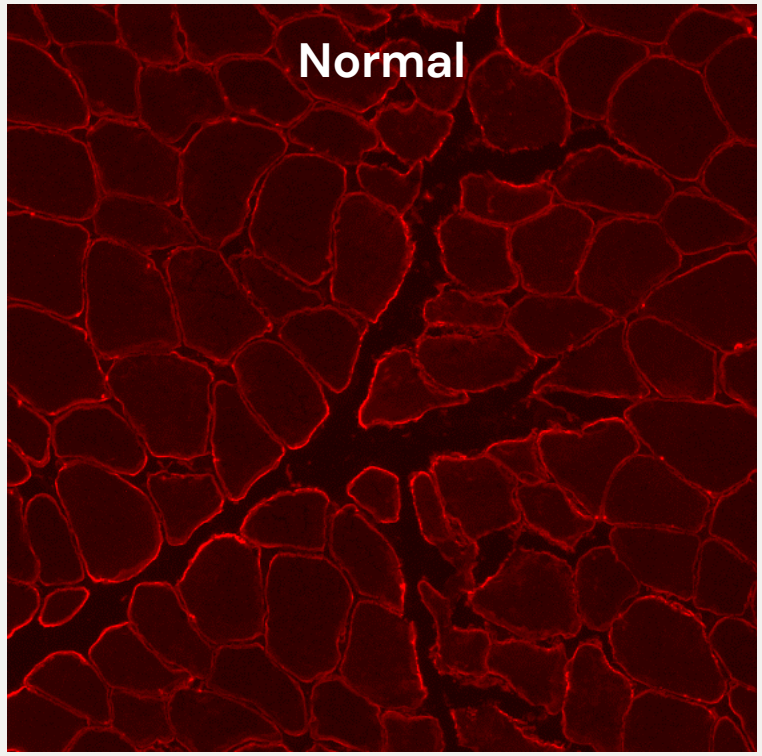
LC-MS

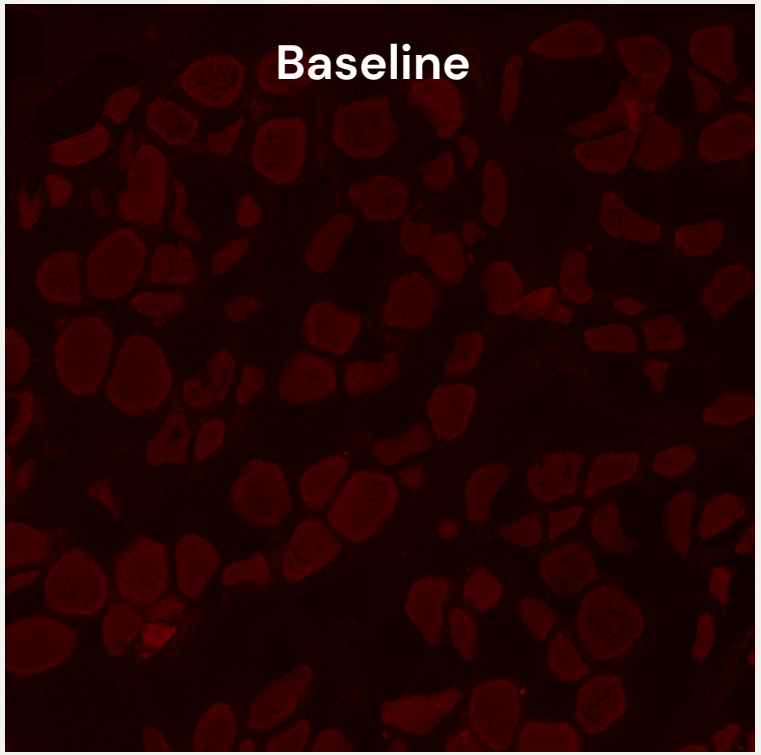
RGX-202 microdystrophin expression at 3 months

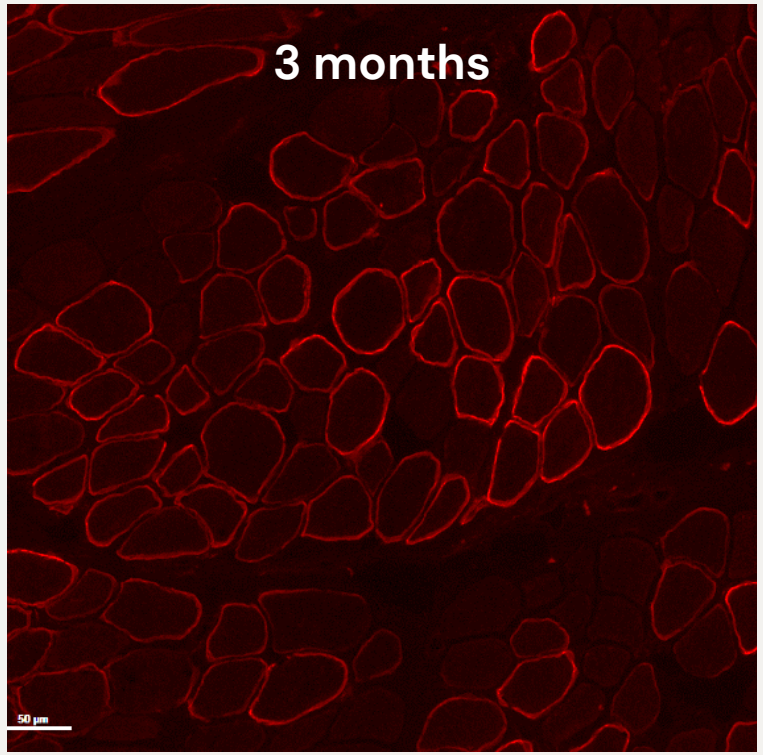
Robust RGX-202 microdystrophin expression was demonstrated at both dose levels

Age range at screening	Dose Level 1 (n = 3)	Dose Level 2 (n = 2)
	% RGX-202 microdystrophin	
4 to 5 years	38.8	
6 to 7 years	83.4	
8 to 11 years	11.1	**75.7**

RGX-202 microdystrophin is localized to the sarcolemma at three months







Muscle biopsies are collected from biceps at baseline and 3 months post RGX-202 administration (images from patient 1)

AFFINITY DUCHENNE: Summary

RGX-202 has been well tolerated at both dose levels with no SAEs

Robust RGX-202 microdystrophin expression was observed at both dose levels in all ages

Encouraging observations of early improvements in daily activities associated with strength and function provided by caregivers

REGENXBIO to initiate pivotal trial in second half of 2024 using RGX-202 microdystrophin expression as a surrogate endpoint likely to predict clinical benefit

RGX-121

For MPS II

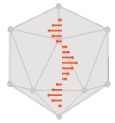
RGX-121 for MPS II: Phase I/II/III CAMPSIITE® study

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

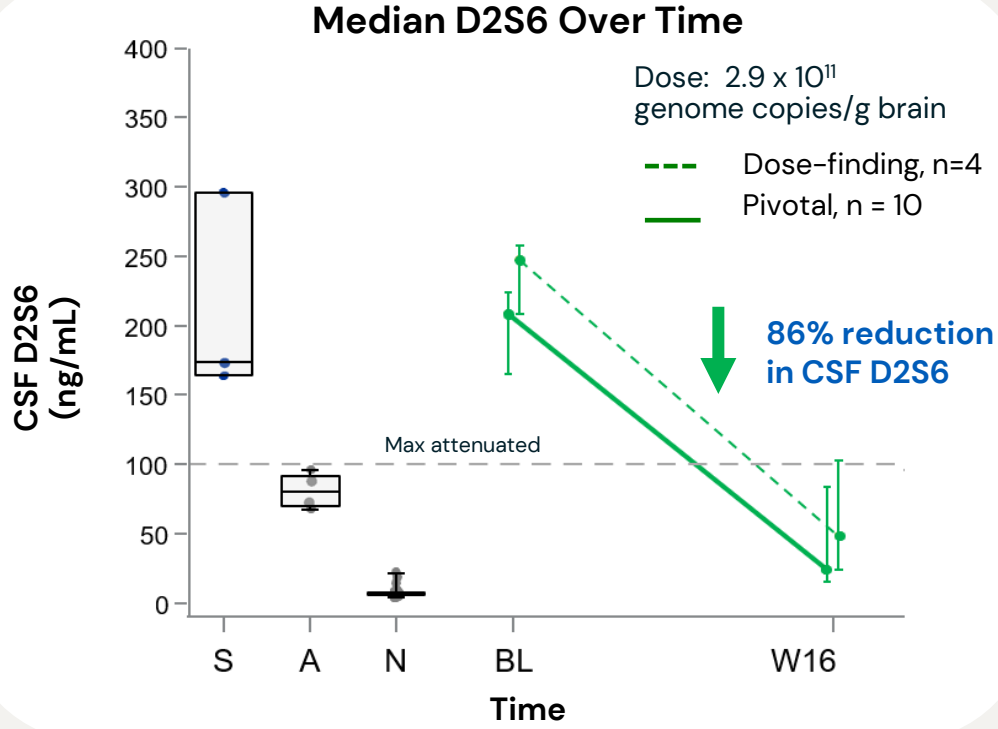
CAMPSIITE Part 2, Pivotal Trial to Support Full Approval

- 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

10 participants dosed as of July 31, 2023
Data cut 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 pivotal – Summary and next steps

RGX-121 was well tolerated in 10 patients at pivotal dose

Pivotal trial met CSF D2S6 primary endpoint with statistical significance

CSF D2S6 is surrogate endpoint reasonably likely to predict clinical benefit for CNS disease

Results support plans to file BLA in H2 2024 utilizing the Accelerated Approval pathway

Data cut as of January 3, 2024
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

Manufacturing Innovation Center Leadership and Financials

REGENXBIO Manufacturing Innovation Center

REGENXBIO has built a new world-class gene therapy manufacturing facility

Everything from developing the manufacturing process to vector production, product quality testing, and filling into sterile vials is done in the facility.

UP TO 2,000L BIOREACTOR SCALE • MULTI-PRODUCT • SINGLE-USE TECHNOLOGY • DIGITALLY-ENABLED FACILITY • ON-SITE WAREHOUSE

CONTROL OF GMP MANUFACTURING

Quality | Schedule | Cost

CLINICAL MANUFACTURING & FUTURE COMMERCIAL SUPPLY

Ability to produce

350,000 doses / year of ABBV-RGX-314

1,600 doses / year of RGX-202

CO-LOCATED WITH R&D

Rapid technology transfer into manufacturing

Potential for candidate selection to clinical supply in 12 months

The REGENXBIO team

Ken Mills
Co-Founder, President and CEO

Olivier Danos
EVP, Chief Scientific Officer

Vit Vasista
EVP, Chief Financial Officer

Steve Pakola
EVP, Chief Medical Officer

Curran Simpson
EVP, Chief Operating Officer

Patrick Christmas
EVP, Chief Legal Officer

Laura Coruzzi
EVP, Intellectual Property

Ram Palanki
EVP, Commercial Strategy & Operations

Shiva Fritsch
EVP, Chief Communications & People Officer

Financial results and guidance

Full year 2023 financial results (millions)	
Revenue	$90
R&D expense	$232
G&A expense	$88
Net loss	$263
Basic share count (12/31/23)	44.0

Q4 2023 financial highlights

Ended 2023 with **$314 million** in cash, cash equivalents and marketable securities. Excludes proceeds from public offering of common stock and pre-funded warrants in March 2024.

ZOLGENSMA® royalty revenues of **$85 million** for the full year 2023. Novartis reported global sales of ZOLGENSMA® of **$1.21 billion** in 2023.

Financial guidance:
We ended 2023 with $314 million in cash, cash equivalents and marketable securities. In the first quarter of 2024, the Company conducted an equity offering in which we received approximately $131 million of net proceeds. With the completion of the offering, we believe we have cash, cash equivalents and marketable securities to fund operations into 2026. This cash runway guidance is based on the Company's current operational plans and excludes the impact of any payments that may be received from AbbVie upon the achievement of development or commercial milestones under our ABBV-RGX-314 collaboration.

On track to advance all programs to pivotal phase or BLA filing in 2024

wet AMD Diabetic retinopathy	ABBV-RGX-314	**Subretinal wet AMD:** Regulatory submissions in US and Europe expected in late 2025 through the first half of 2026 **Suprachoroidal wet AMD:** Program and data updates expected in 2024 **Suprachoroidal DR:** Program and data updates expected in 2024
Duchenne	RGX-202	**Phase I/II AFFINITY DUCHENNE® Trial:** Pivotal dose determination and initiation of pivotal program expected in 2024
MPS II	RGX-121	**Phase I/II/II CAMPSIITE® Trial:** Expected to file BLA in 2024 using accelerated approval pathway

Thank You