REGENXBIO®

Delivering the next wave of genetic medicines

Corporate Presentation October 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.

Multiple billion-dollar opportunities, with lead candidate in Duchenne muscular dystrophy.

With the expertise and strong balance sheet, REGENXBIO is leading the future of one-time treatments.

Upcoming catalysts supported by industry-leading end-to-end capabilities.

REGENXBIO®

Late-stage pipeline in multi-billion dollar commercial markets

Retina franchise partnered with AbbVie

Wet AMD: dual route of administration strategy to accelerate and expand access; clinical POC established with sustained vision & safety 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 potential first and only one-time treatment for Hunter syndrome and only treatment to directly address neurocognitive decline; rolling BLA filing 2024

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; expected to advance to pivotal phase in Q4 2024

Strong balance sheet expected to fund operational runway into 2026

*AbbVie milestone payments expected for DR program

REGENXBIO's pipeline

Indication		Description	Phase I	Phase II	Pivotal	Anticipated Milestones
Rare Disease						
Duchenne	**RGX-202**	Novel microdystrophin				Pivotal trial initiation Q4 2024 Functional data 2H 2024
MPS II	**RGX-121**	Iduronate-2-sulfatase enzyme				Submission of a rolling BLA using the accelerated approval pathway to start in Q3 2024
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

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

Driving significant value creation

Diverse pipeline and industry-leading end-to-end capabilities to launch new medicines

R&D Engine
innovating gene therapy through capsid discovery and gene transfer technology

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

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

$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

$7B
Duchenne

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

Pursuing accelerated approval and broad label

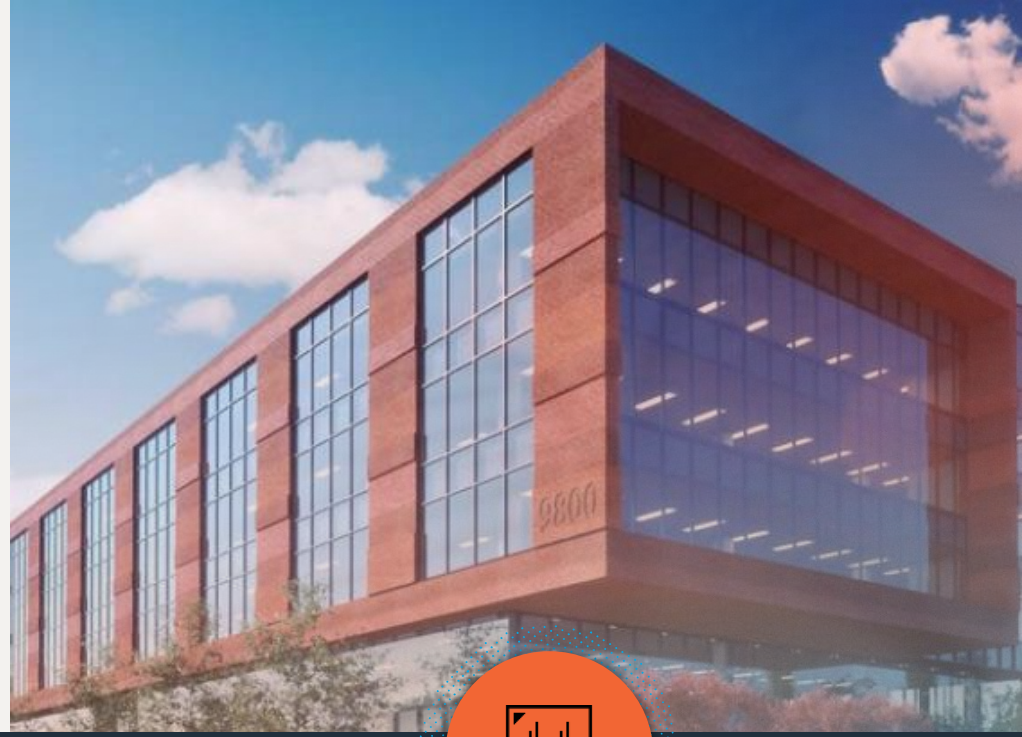
$1B
MPS II

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

Eligible for Priority Review Voucher upon potential accelerated approval

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:

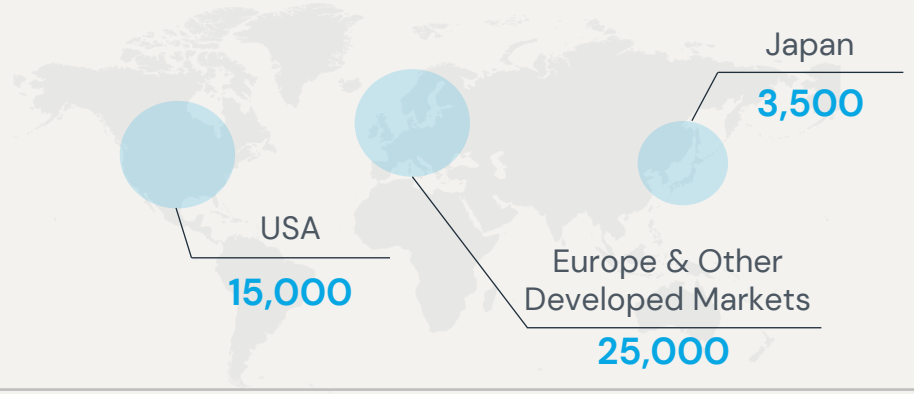
Next generation microdystrophin design for Duchenne Muscular Dystrophy

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]

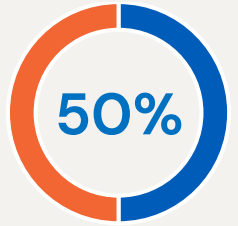


Limited effective treatments to delay loss of ambulation and death[5]

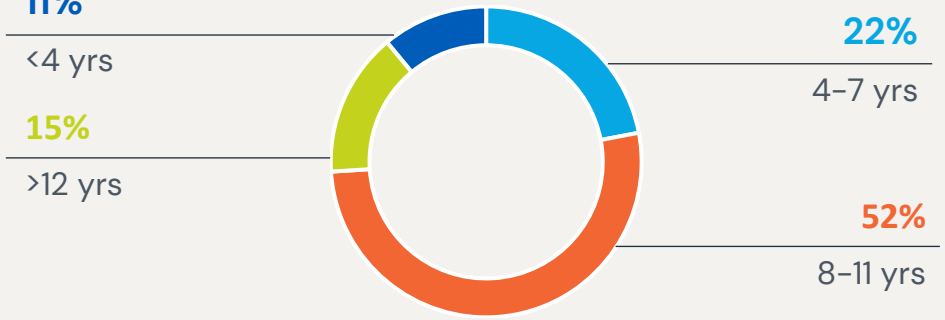
MEDIAN SURVIVAL AGE[4]

Mid to late 20'S

% WITH AMBULATORY LOSS



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

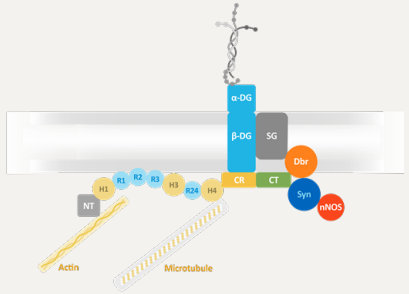


1. Crisafulli et al 2020; 2. Koeks et al 2017; 3. Bello et al 2016; 4.McDonald et al 2018; 5. Health Resources & Services Administration (HRSA) Advisory Committee on Heritable Disorders in Newborns and Children "Nomination and Prioritization Workgroup Report on Duchenne Muscular Dystrophy" Feb 2023;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

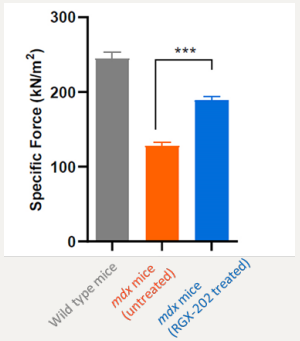
RGX-202 Program Status: Pivotal Initiation in Q4 '24

We Are Here

Optimize Microdystrophin Product



Preclinical Proof-of-Concept



IND-enabling Activities

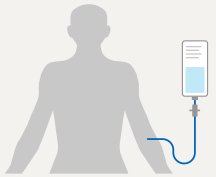
Pharmacology (mdx) and Toxicology (NHP) studies complete

GMP material manufactured

Phase I/II Clinical Study

AFFINITY DUCHENNE™

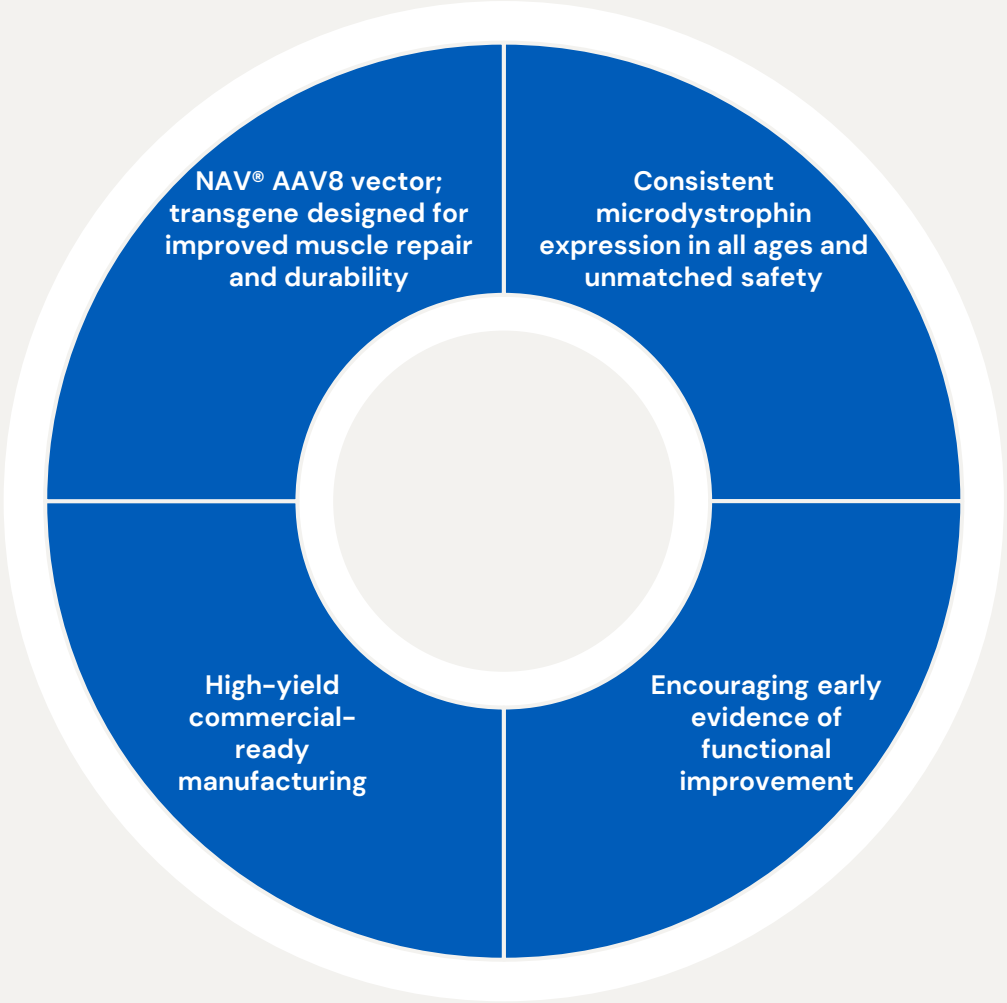
Pivotal Study initiation



Upcoming Milestones

2024	Sharing first functional data (2H)
	Sharing pivotal trial design (early Q4)
	Pivotal trial initiation (Q4)

RGX-202: A potential best-in-class gene therapy



NOVEL CONSTRUCT

- **Only microdystrophin** with CT domain
- **Spc5-12 promoter** for targeted expression in skeletal and heart muscle
- **Codon optimization** for improved expression and translation; CpG content reductions for reduced immunogenicity

POSITIVE DATA PROFILE

- **Highly consistent, double-digit** microdystrophin expression
- **Highest reported levels** of microdystrophin expression in older ambulatory patients
- **Clean safety profile**, no SAEs
- **Early signs of functional improvements** observed through clinic and caregiver videos, showcasing improvements in daily activities

MANUFACTURING

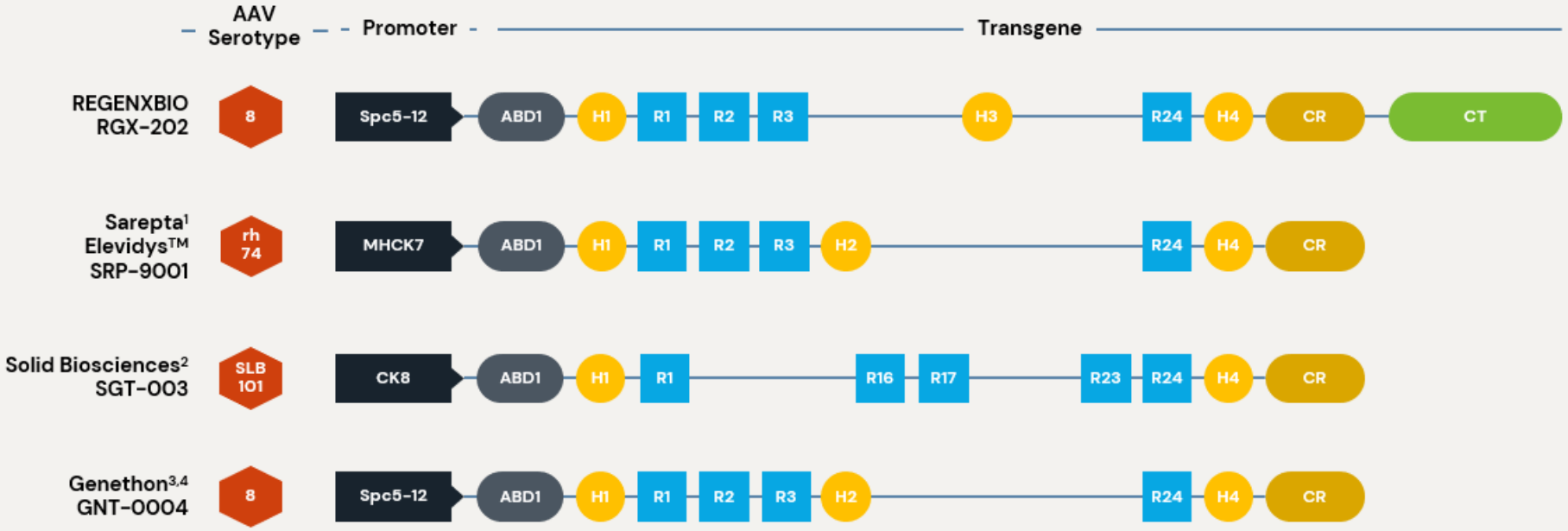
- Highest manufacturing capacity among all companies with approved or investigational DMD products
- Favorable cost of goods

LAUNCH

- **Second-to-market**, positioned to broadly address ongoing unmet need
- **Large market opportunity** includes both incident and prevalent populations at launch
- **Reaching unserved population** as only trial recruiting younger patients in the U.S.

RGX-202: Construct designed for potential improved durability

RGX-202 is the only gene therapy designed to deliver a microdystrophin transgene that incorporates the functional C-Terminal (CT) domain from naturally occurring dystrophin.



Role of the CT Domain

Preclinical studies indicate the CT domain contributes to the long-term durability of RGX-202.

Muscle health

- Plays a crucial role in muscle repair, potentially preventing muscle breakdown and preserving muscle function longer

Prolonged transgene activity

- Supports the half-life of the transgene, allowing RGX-202 to stay in target cells for longer, continuing to preserve muscle.

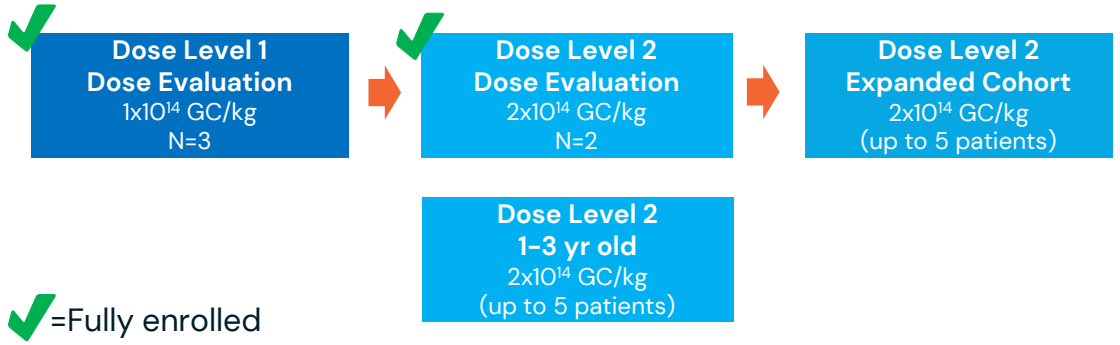
1. Harper (2002) Nat Med
2. https://investors.solidbio.com/Corporate Presentation, January 2024
3. From internal deck on Genethon / Sarepta Collaboration 12.11.2020
4. Le Guiner (2017) Nat Comm

RGX-202 Study overview and program updates

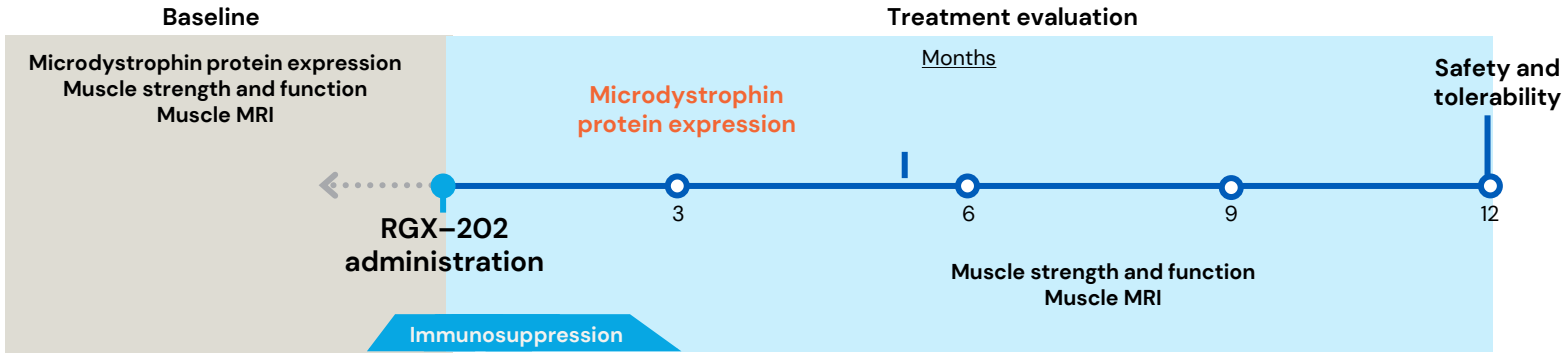
Key Eligibility Criteria

- **Boys aged 1 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



Interim results: microdystrophin expression at 3 months

- Consistent robust RGX-202 microdystrophin expression observed at both dose levels across treated patients
  - Among the highest levels of microdystrophin expression reported in older ambulatory patients
- Observed marked deceases in serum CK levels
- RGX-202 microdystrophin is localized to the sarcolemma at three months

Patient	Age at Dosing	RGX-202 Microdystrophin Western blot (Jess method) (% Normal Control)
Dose Level 1 – $1x10^{14}$ GC/kg		
1	4 yrs 4 mos	38.8
2	10 yrs 5 mos	11.1
3	6 yrs 6 mos	83.4
Dose Level 2 – $2x10^{14}$ GC/kg Pivotal dose		
1	12 yrs 0 mos	75.7
2	8 yrs 1 mos	20.9
3	8 yrs 5 mos	46.5
4	5 yrs 10 mos	77.2

Microdystrophin expression adjusted for muscle content. Muscle biopsies are collected from bicep at baseline and 3 months post RGX-202 administration
Control was level of wild-type (normal) dystrophin in normal muscle. Elevated CK levels are correlated with muscle injury and are uniformly elevated in patients with Duchenne
Data cut date of July 8, 2024; Data not shown for patients with limited follow-up

AFFINITY DUCHENNE: Summary

- RGX-202 has been well-tolerated at both dose levels with no SAEs
- Consistent, robust RGX-202 microdystrophin expression observed in all ages
- Encouraging clinic and caregiver videos show observations of early improvements in daily activities
- Industry-leading, commercial-ready manufacturing capacity using high-yield NAVXpress™ suspension process
- Pivotal trial initiation expected in Q4 2024; confirmed plans to use RGX-202 microdystrophin as a surrogate endpoint likely to predict clinical benefit with FDA

RGX-121:

On track to be the first gene therapy for Hunter Syndrome

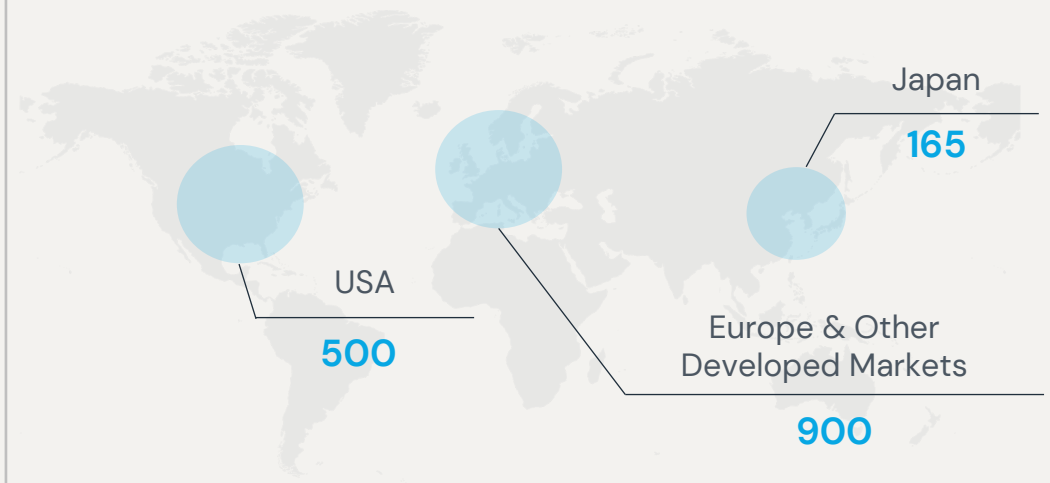
Mucopolysaccharidosis Type II (MPS II) opportunity: an estimated $1B global market within 5 years[9]

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

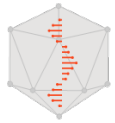
RGX-121 for MPS II: Phase I/II/III CAMPSIITE® study

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

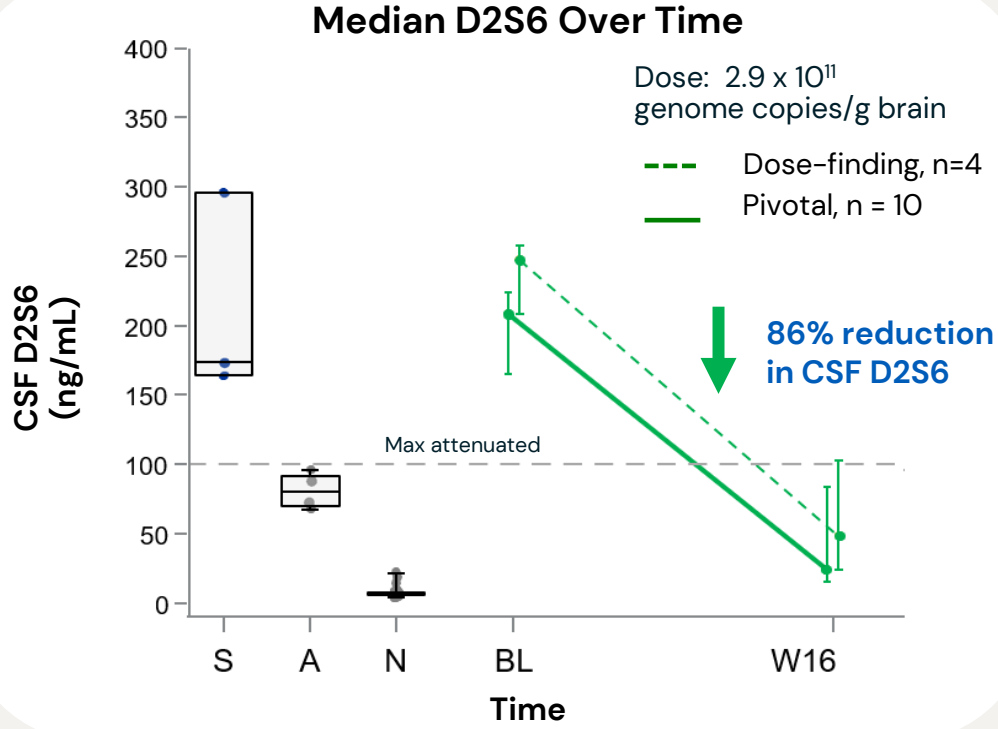
CAMPSIITE Part 2, Pivotal Trial to Support Approval

- 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

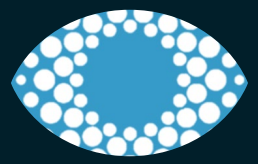
10 participants dosed as of July 31, 2023
Data cut January 3, 2024

* 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: Summary

- RGX-121 was well tolerated in 10 patients at pivotal dose
- Pivotal trial met CSF D2S6 primary endpoint with statistical significance
- Neurodevelopmental and daily activity skill acquisition was observed up to 3 years after RGX-121 administration
- Held positive pre-BLA meeting; rolling BLA submission using the accelerated approval pathway expected to start in Q3 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



Retinal Disease

ABBV-RGX-314

Potential to be the first gene therapy for chronic retinal diseases

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

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	

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
2 Pivotal Doses

Enrolling

Regulatory Submissions

Expected
1H 2026

Phase II Bioreactor Bridging Study

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

Enrolled

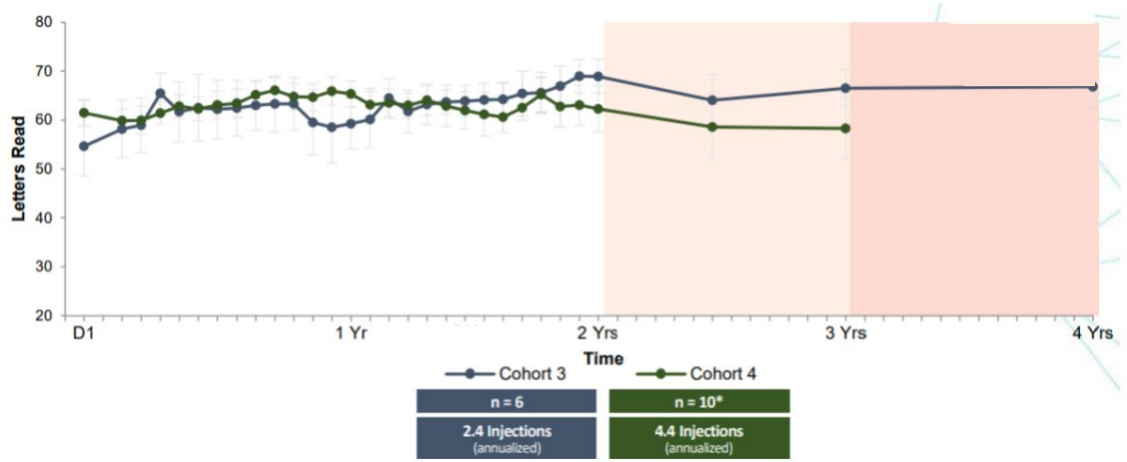
Fellow Eye Study

Up to 20 subjects
Open Label
Pivotal High Dose

Enrolled

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

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*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.

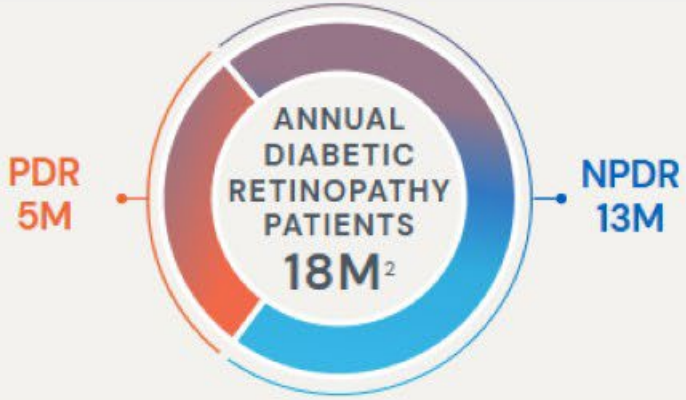
Diabetic retinopathy is a global public health problem

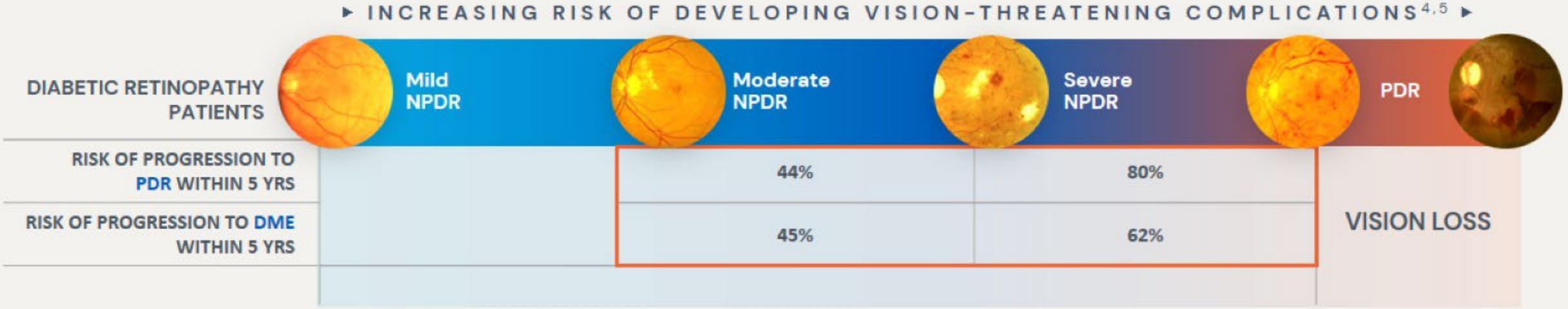
20M is the expected DR patient population in US, EU, JP in the next 5 years[1]

<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





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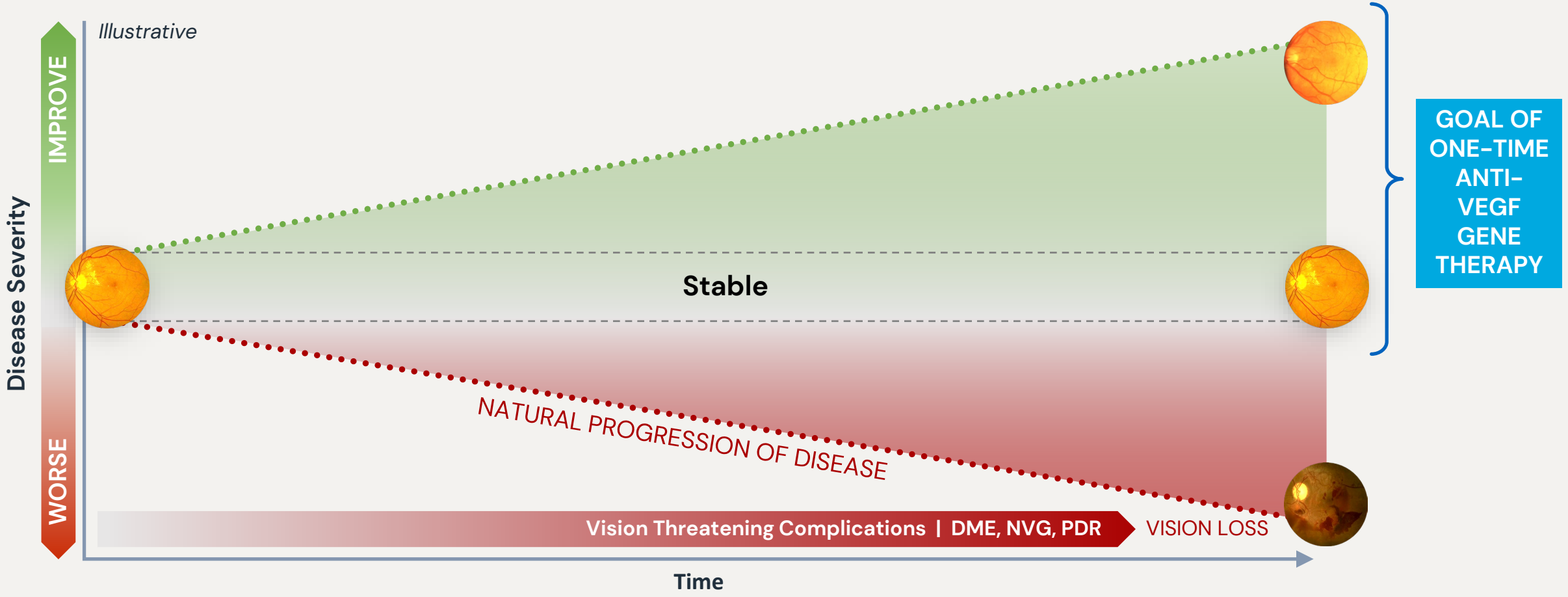
1.DR Market, Reports and Data 2022; 2. Market Scope 2022 Retinal Pharmaceuticals; 3. Market Scope Q4-2022 Retina Quarterly Summary Report; 4. ETDRS Trial Report 12, 1991; 5. Moshfeghi, 2020
DR = diabetic retinopathy; NPDR = non-proliferative diabetic retinopathy; PDR = proliferative diabetic retinopathy;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

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

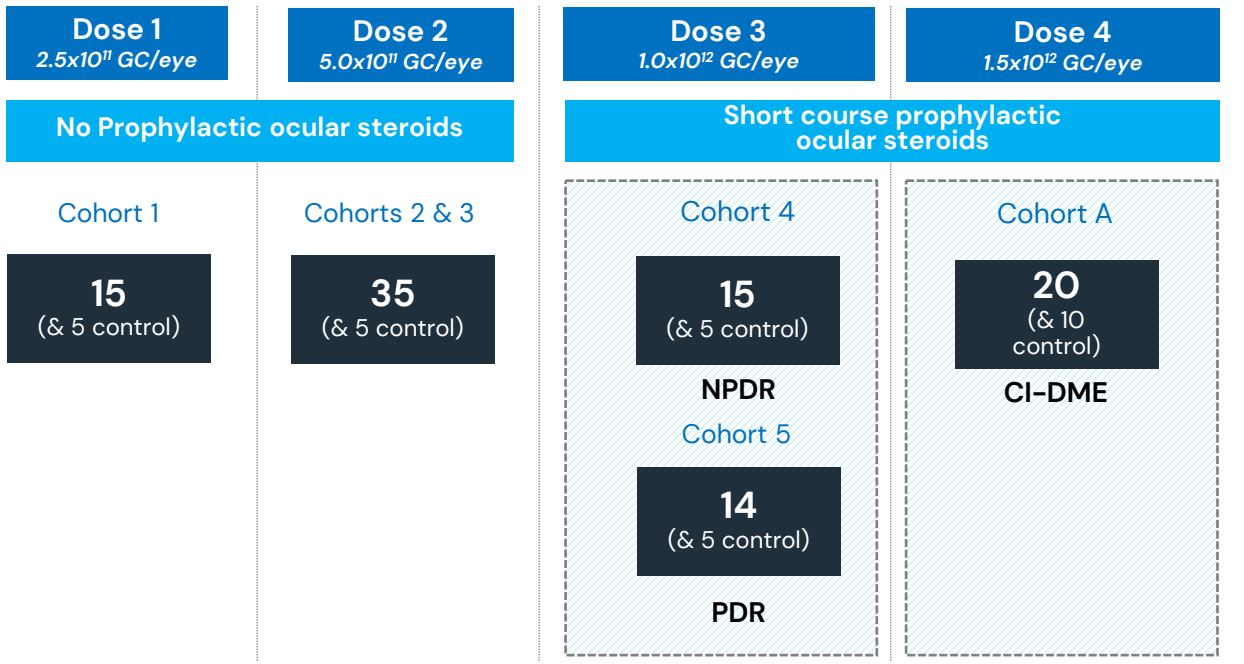
Latest Readouts

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

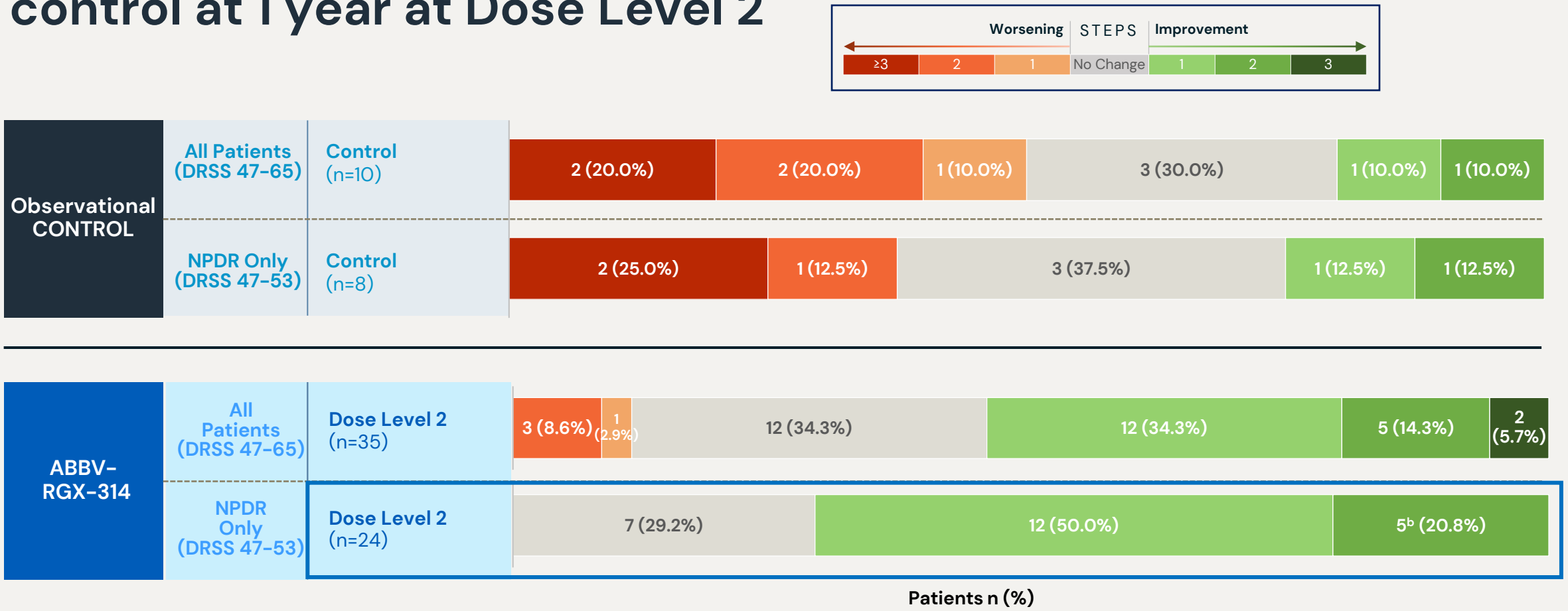
Pivotal Trial Initiation

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

Study Design

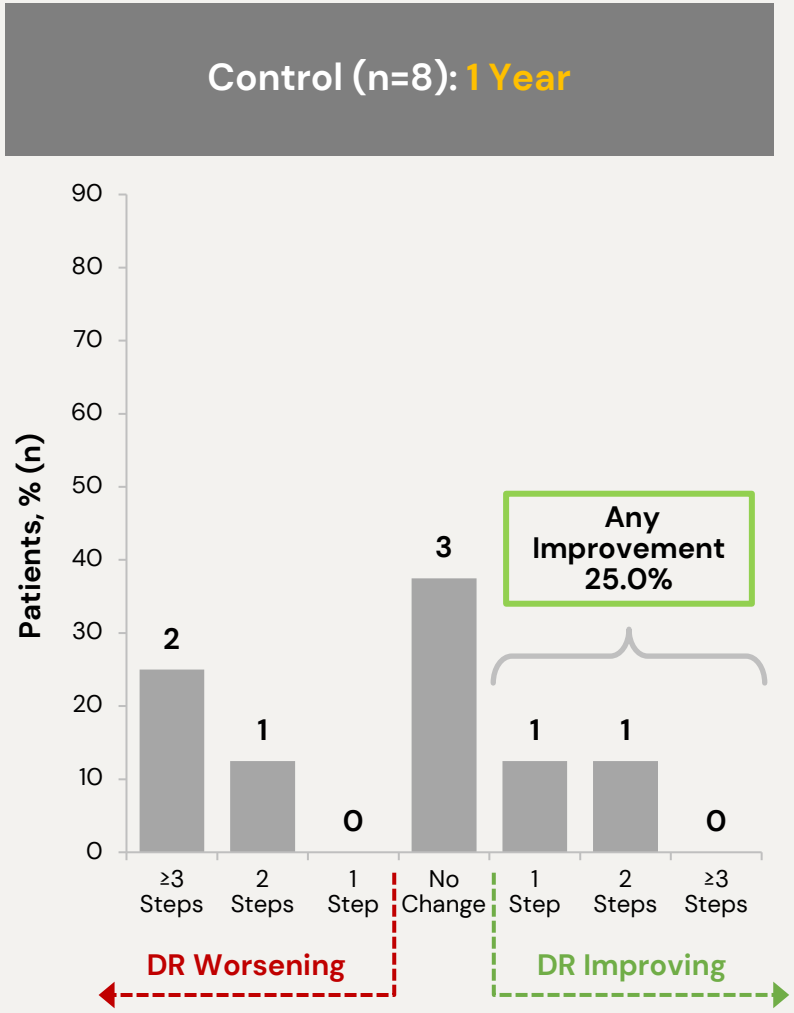


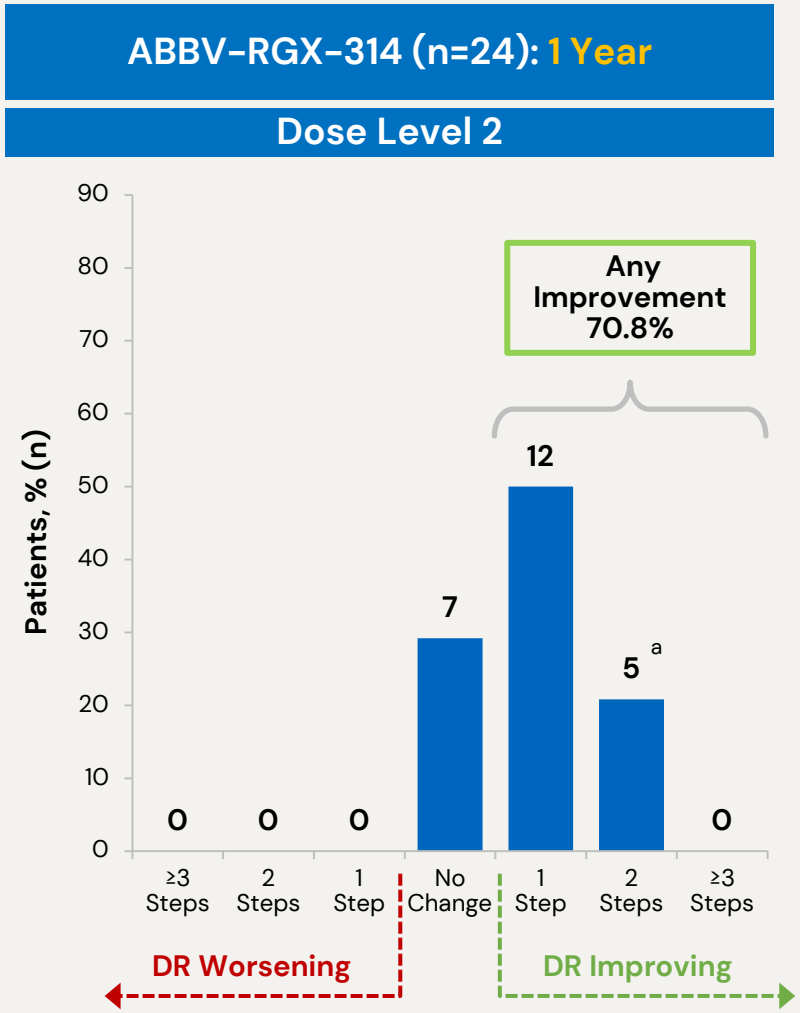
ALTITUDE: Summary of DRSS change compared to control at 1 year at Dose Level 2



Data cut: September 25, 2023.

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

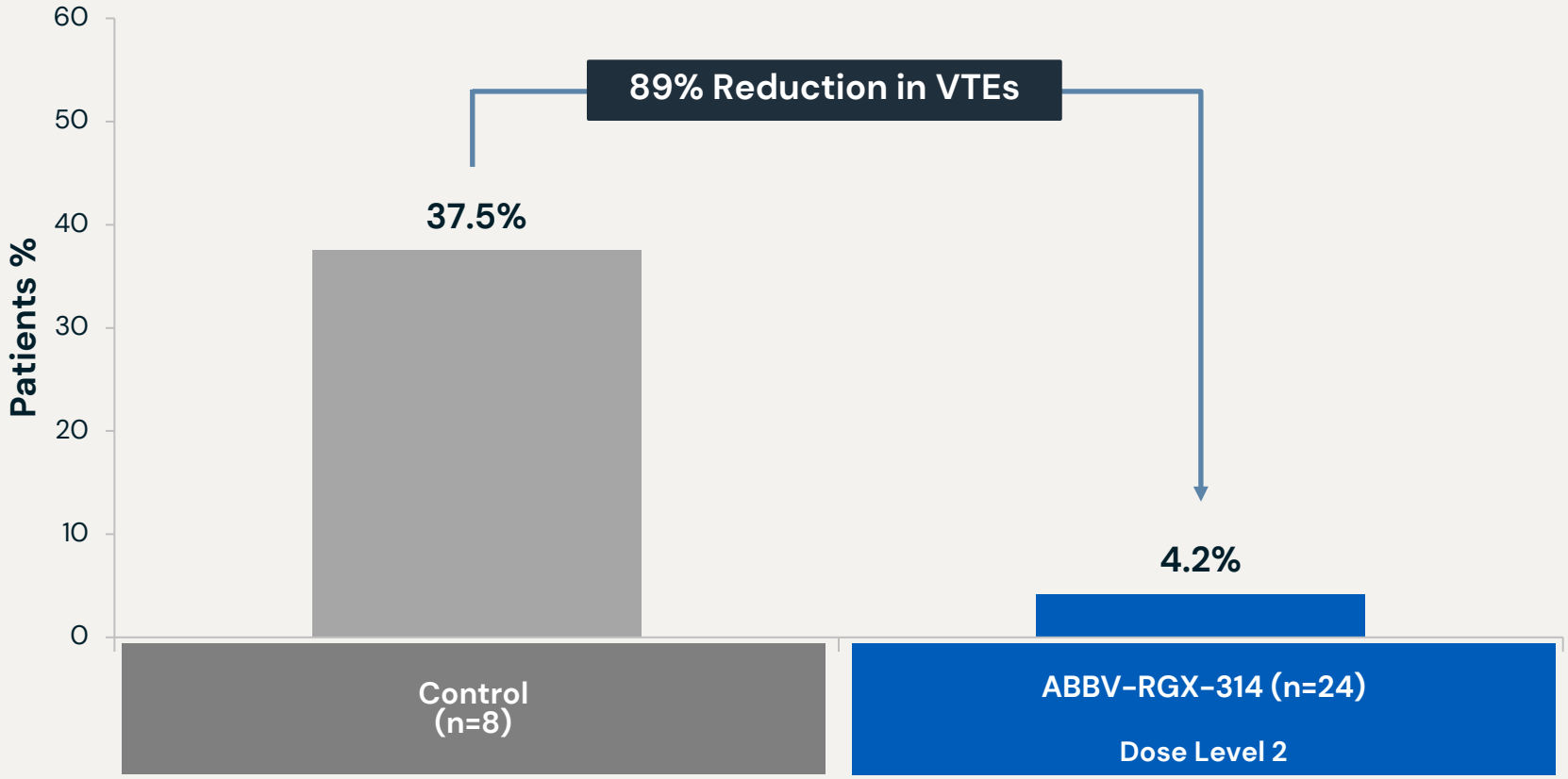




Data cut: September 25, 2023.

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

- **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%.

REGENXBIO

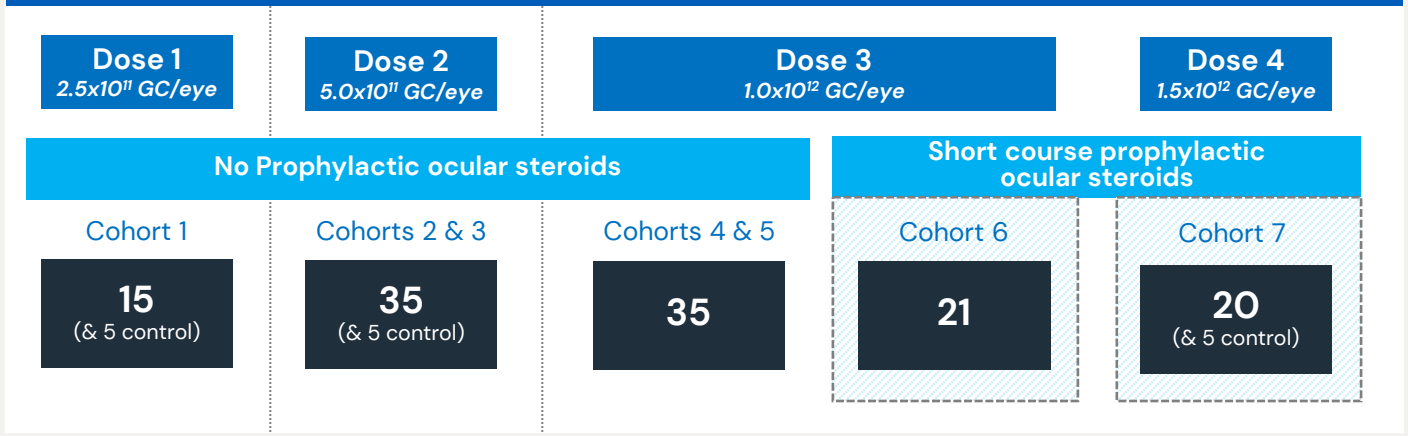
Data cut: September 25, 2023.
No drug-related SAEs. Cases of intraocular inflammation were mild and resolved with topical steroids. There were no cases of chorioretinitis, vasculitis, occlusion or hypotony. Common treatment emergent adverse events included conjunctival hyperemia, conjunctival hemorrhage,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

Study Design

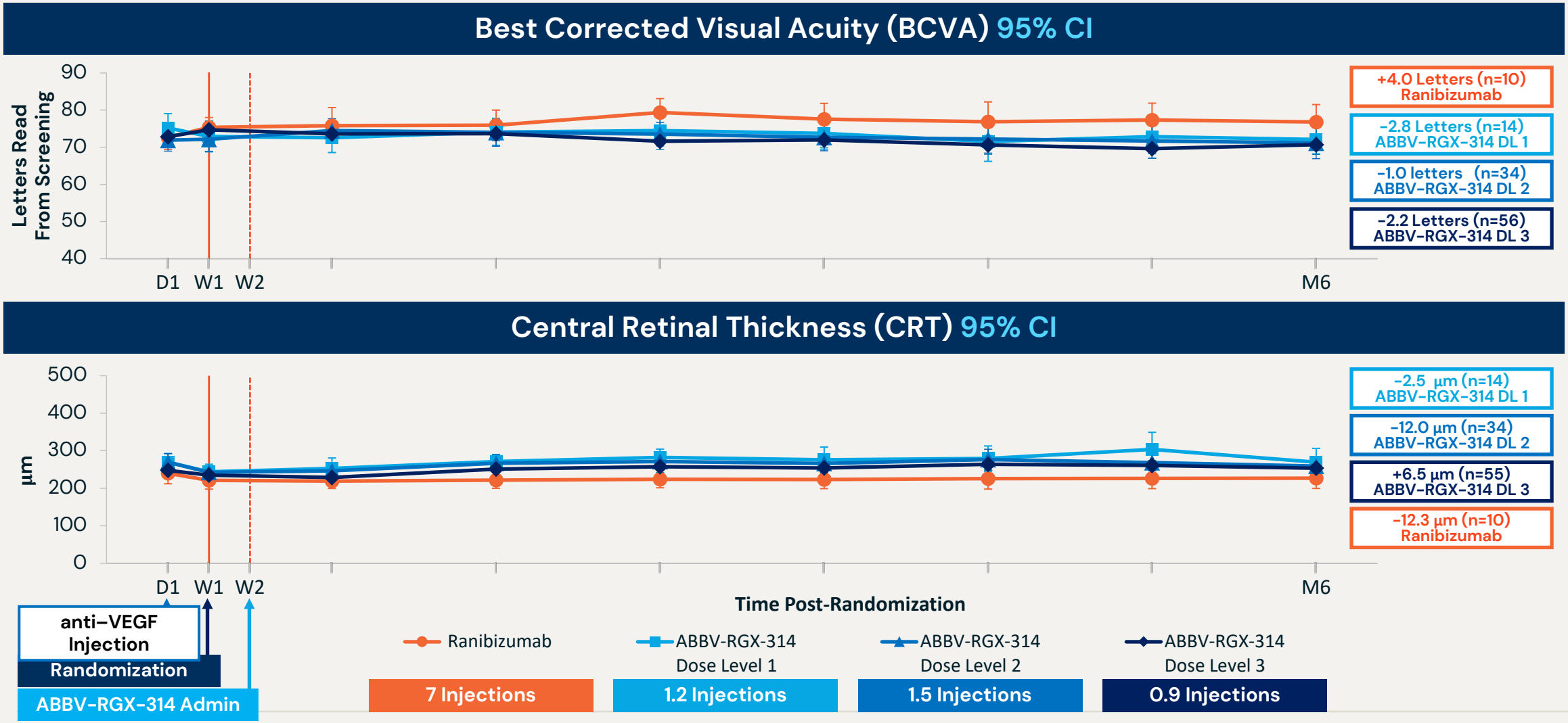


Data Readouts

Latest Readouts

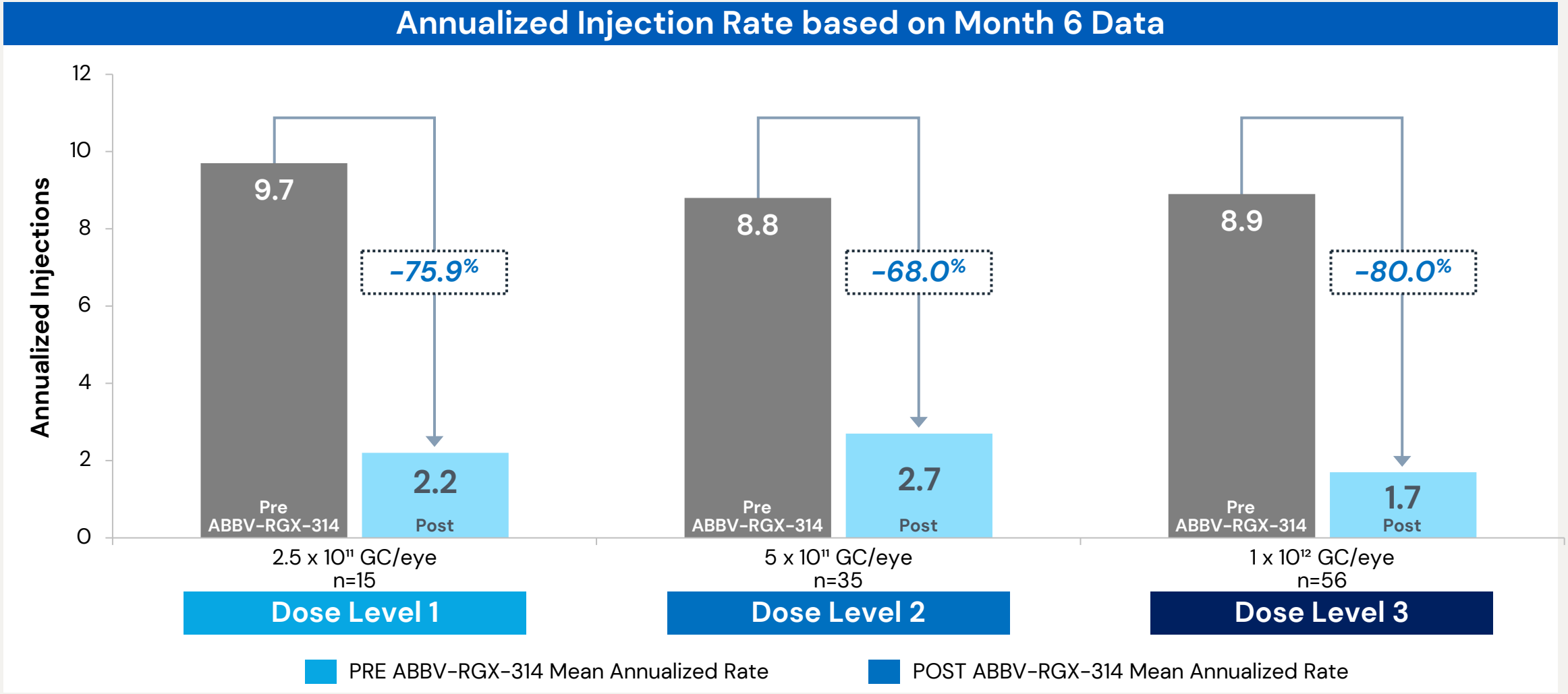
- Cohort 1-4 (DL1-3) at 6 months
- Cohort 1-6 (DL1-3) at 6 months

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



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.

Thank You