# Subretinal Delivery of Investigational ABBV-RGX-314* as a Gene Therapy for nAMD

## One-year Results of a Fellow Eye Bilateral Dosing Study

ABBV-RGX-314 is also known as surabgene lomparvovec (sura-vec)

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Clinical Trials at the Summit

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*ABBV-RGX-314 is an investigational product that has not been approved by any regulatory authority. No conclusions regarding the safety and efficacy of the product can be made.

# Disclosures

Alcon - C
Alimera Sciences - C
Allergan - C, R
Apellis - S
Astellas- S
BVI - C
Coherus - S
EyePoint - C, R
Genentech - C
Neurotech - C
Outlook Therapeutics - C
RecensMedical - C
Regeneron - C, S
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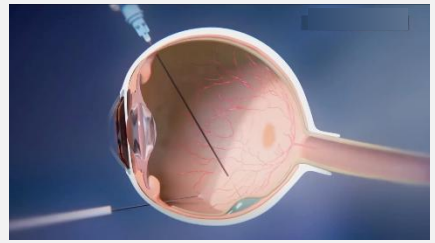
This is a preliminary analysis performed by REGENXBIO for an ongoing trial.

# Sura-vec for Treatment of Neovascular Age-related Macular Degeneration (nAMD)

NAV® VECTOR **AAV8**

GENE **Anti-VEGF fab**

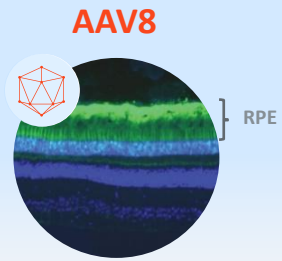
## SUBRETINAL ADMINISTRATION



MECHANISM OF ACTION

Reducing leaky blood vessel formation by giving ocular cells the ability to produce an anti-VEGF fab

Improved AAV vector technology




Efficient gene delivery to the RPE[1]

+

Leveraging current standard of care in transgene

FDA-approved mAbs and mAb fragments that inhibit VEGF are the current standard of care for treatment of nAMD

**Sura-vec transgene encodes an anti-VEGF mAb fragment (fab)**

=

**Sura-vec:** AAV8 encoding anti-VEGF fab

**Potential for long-term therapeutic anti-VEGF expression**

1. Vandenberghe et al. 2011 Science Translational Medicine.

AAV: adeno-associated virus; RPE: retinal pigment epithelium VEGF: vascular endothelial growth factor.

# Current Program Status for Sura-vec

## Subretinal

**Phase 1/2a trial** is complete

**Phase 2 pharmacodynamic trial** is ongoing

**Phase 2b/3 pivotal trials** are ongoing

ATMOSPHERE ASCENT

LONG-TERM FOLLOW-UP STUDY

**Subretinal** sura-vec for **nAMD**
in participants from parent trials

**FELLOW EYE SUB-STUDY**

**Investigating bilateral nAMD treatment**

## Suprachoroidal

**Phase 2 trial** is ongoing

AAVIATE

LONG-TERM FOLLOW-UP STUDY

**Suprachoroidal** sura-vec for **nAMD**

**Phase 2 trial** is ongoing

ALTITUDE

LONG-TERM FOLLOW-UP STUDY

**Suprachoroidal** sura-vec for **DR**

# First Ever Bilateral Gene Therapy Clinical Trial for Treatment of nAMD

## Fellow Eye Sub-Study of Subretinal Sura-vec

### 1st Eye Treated:

**PHASE 1/2a Study**
**n=3**

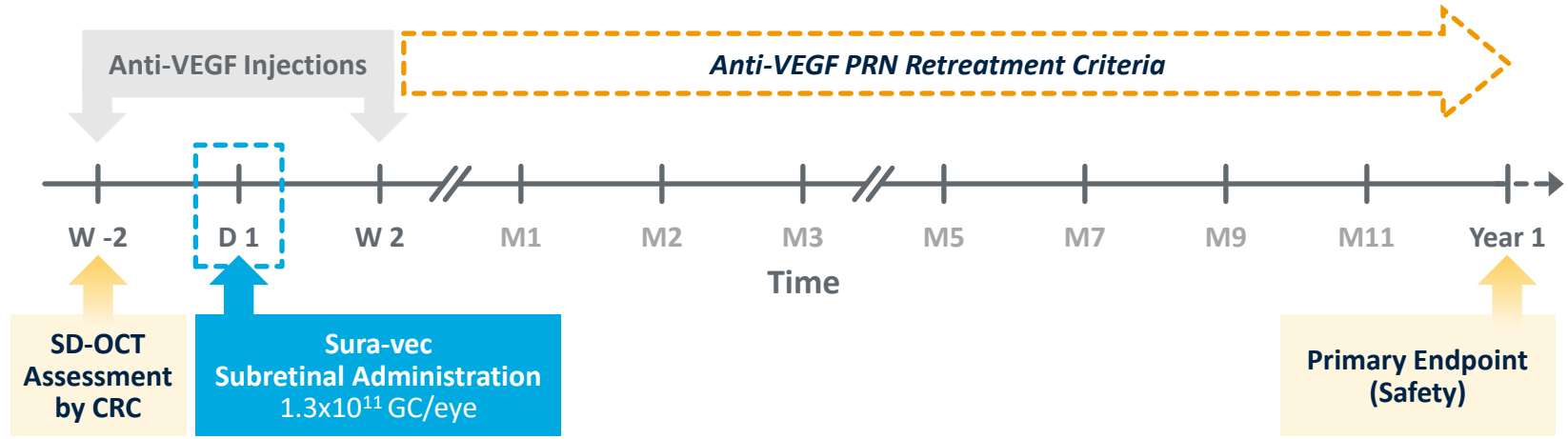
**PHASE 2 Pharmacodynamic Study**
**n=8***

ATMOSPHERE

ASCENT

*Masked n=10*

### 2nd Eye Treatment Evaluation (n=21*)




**No prophylactic steroids given beyond routine vitrectomy SOC**

**Study is designed to monitor safety, immune responses, and efficacy of sura-vec treatment in the fellow eye**

*One participant withdrew after their Month 2 visit, so one additional participant was enrolled.
nAMD: Neovascular age-related macular degeneration; VEGF: Vascular endothelial growth factor; SOC: Standard of care; CRC: Central reading center; PRN: pro re nata; GC: genome copies; M: month.
This is a preliminary analysis performed by REGENXBIO for an ongoing trial.

# The Potential of Bilateral Dosing of Gene Therapy in nAMD

## nAMD is frequently a bilateral disease and the ability to treat both eyes with gene therapy is important

- Two other subretinal AAV gene therapies have treated IRD participants bilaterally[1,2]
- After exposure to AAVs, antibodies are formed, which could impact the potential to dose a 2nd eye[2]
- Outcomes of bilateral dosing may vary, depending on factors such as **route of administration**[2,3]
- For subretinal delivery of sura-vec, presence of pre-existing antibodies to AAV8 did not impact safety, protein production or efficacy outcomes in the 1st eye[4]

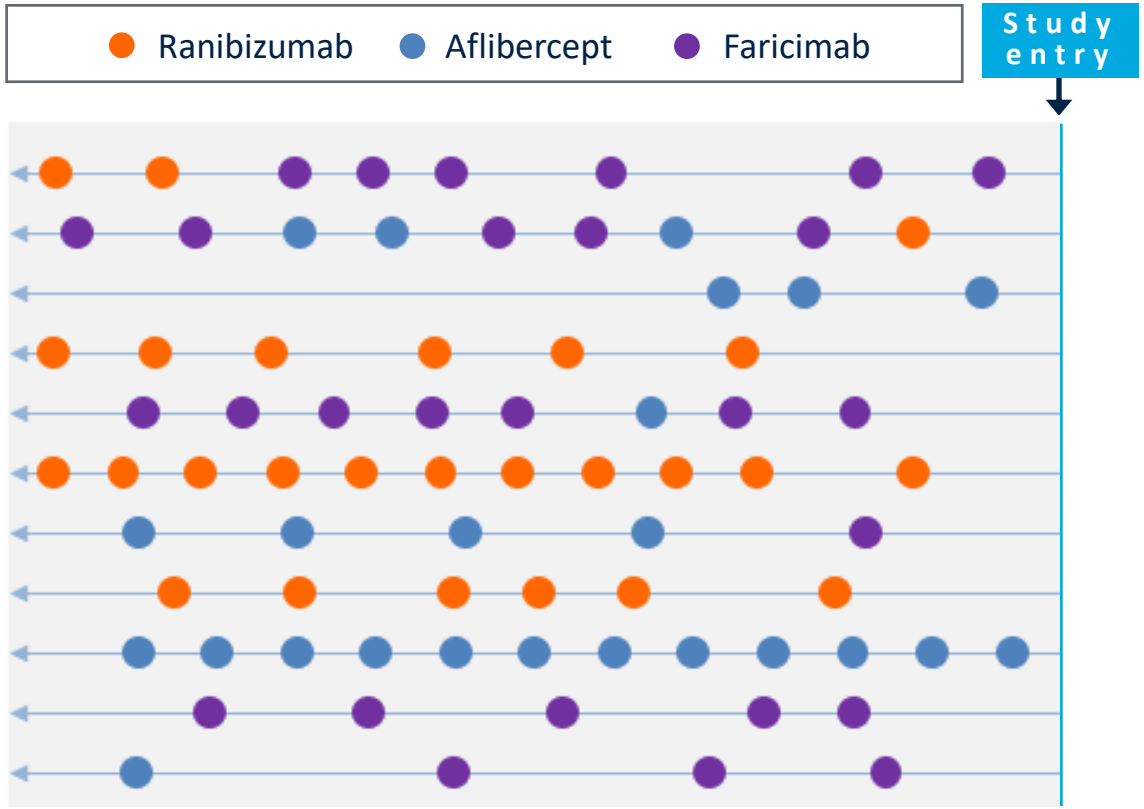
1. LUXTURNA [package insert]. Philadelphia, PA: Spark Therapeutics, Inc. Revised April 2022. Accessed August 22, 2024. 2. MacLaren RE, et al. *Hum Gene Ther*. 2024;35(15-16):564-575. 3. Yiu G, et al. *Mol Ther Methods Clin Dev*. 2020;16:179-191. 4. Campochiaro, PA et al. *The Lancet*, Volume 403, Issue 10436, 1563–1573.
AAV: adeno-associated virus: IRD: inherited retinal disease; nAMD: neovascular age-related macular degeneration.

# Fellow Eye Sub-Study (FESS): Open Label Participant Population

## FESS BASELINE DEMOGRAPHICS
(Open Label Participants Only)

| | 2nd Study Eye (n=11) |
|---|---|
| **Mean age** (years) | 77.8 |
| **FE BCVA** (letters) | 72.8 (~20/40) |
| **FE OCT** (µm) | 249.5 |
| **FE Injections in the past year** (#)* | **8.0** |
| **Average annualized injections in the past year in FE** (#)* | 8.8 |

## FE INJECTIONS IN YEAR PRIOR TO TREATMENT




**2nd eyes had a high treatment burden, despite use of more durable agents**

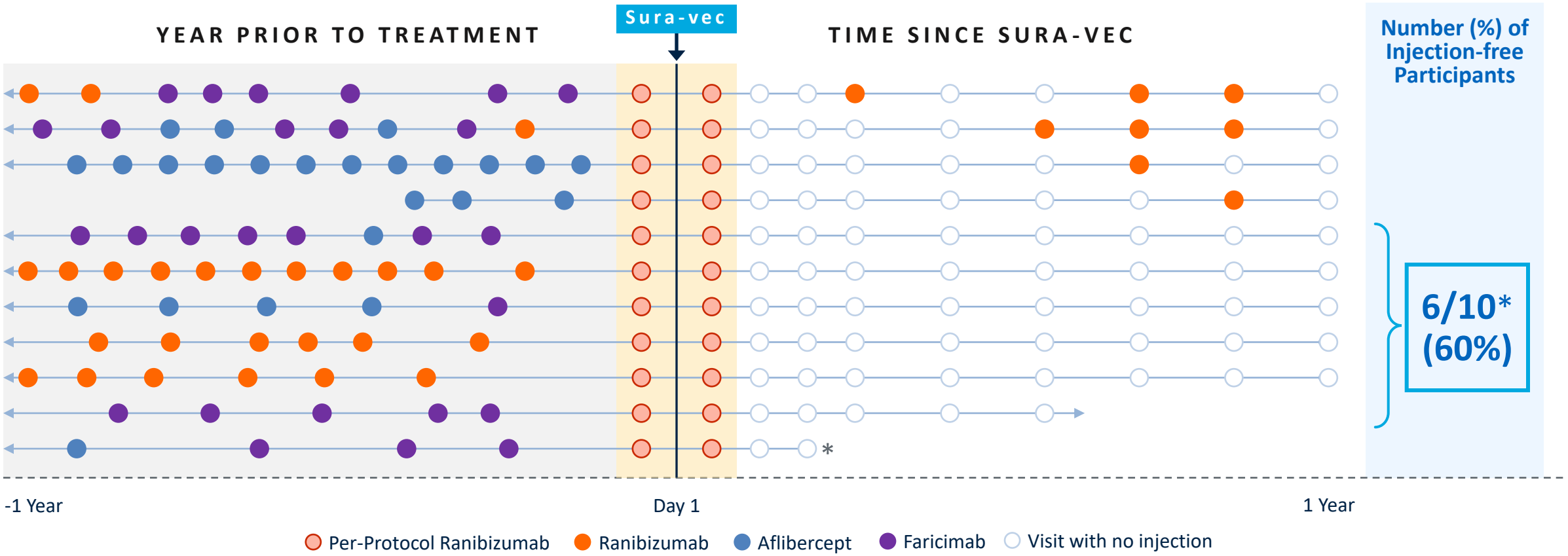
Data cut: February 25, 2025.
2nd eyes dosed approximately 1 year or more post-administration in 1st eye. Ocular variables refer to study eye only.
Average annualized injections in the past year is: (Total # of prior injections)/(minimum (366 days, Duration between first injection and Week -2)/365.25). *Includes anti-VEGF injection at baseline visit.
This is a preliminary analysis performed by REGENXBIO for an ongoing trial.

# Safety Summary for 2nd Eyes through 1 Year for Open-label Participants

- **Sura-vec was well-tolerated by all open-label participants (n=11*)**
- **No drug-related SAEs**
- No cases of chorioretinitis, vasculitis, occlusion, or hypotony were observed
- Common AEs** in the treated fellow eye included:
  - Retinal pigmentary changes all occurring in periphery as assessed by CRC (91%) – all mild
  - Post-operative conjunctival hemorrhage (27.3%) – all resolved within days to weeks

Data cut: May 26, 2025.
2nd eyes dosed approximately 1 year or more post-administration in 1st eye.
*One participant withdrew after their Month 2 visit, so one additional participant was enrolled.
** Includes AEs for total open-label group ≥15% with onset up to W54 visit. Participants are counted once for each Preferred Term regardless of the number of events.
SAE: Serious Adverse Event; AE: Adverse event; CRC: Central Reading Center.
This is a preliminary analysis performed by REGENXBIO for an ongoing trial.

# Majority of Participants Were Injection-free Through 1 Year

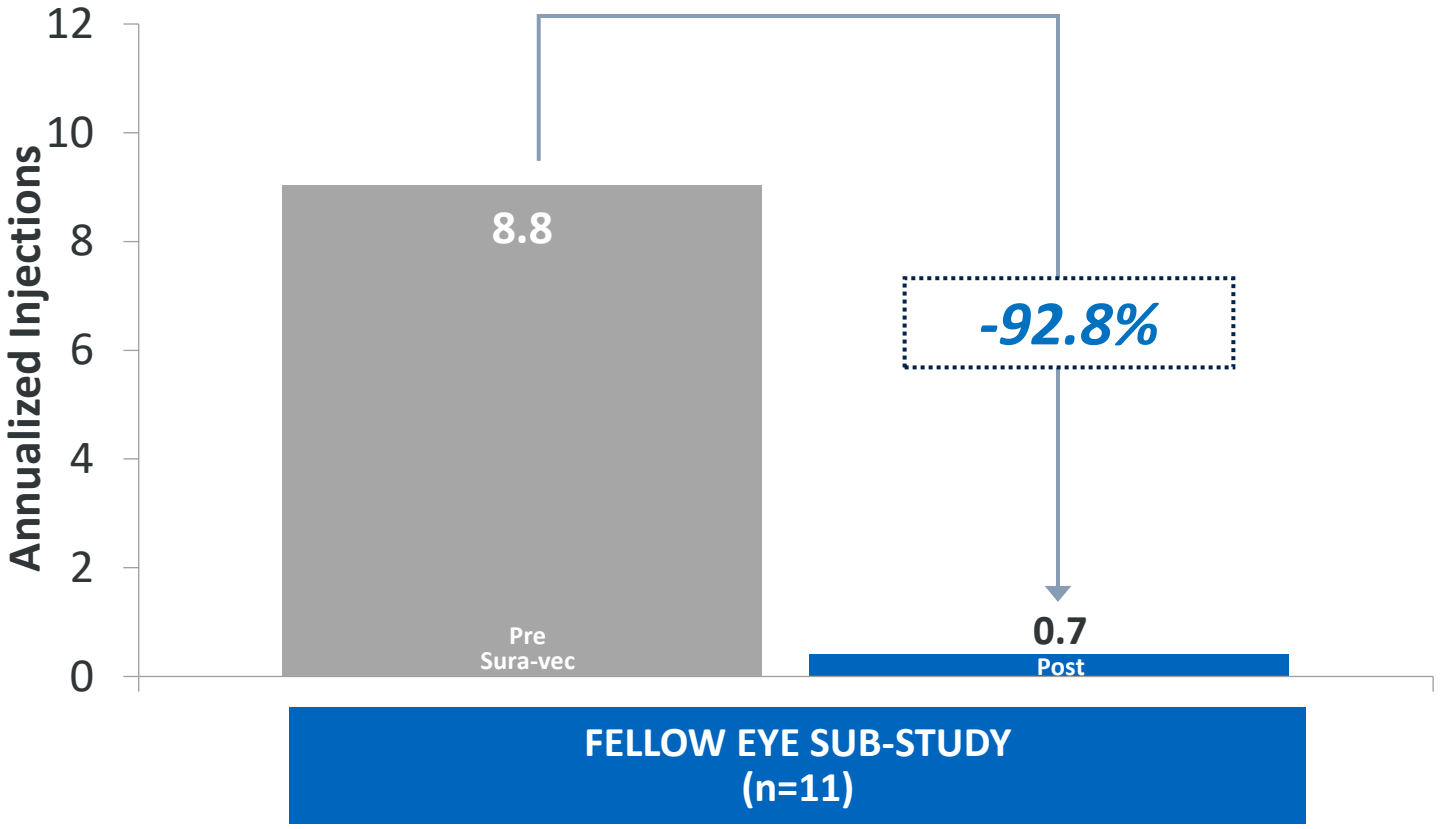



## 80% of Treated 2nd Eyes Required Either 0 or 1 Supplemental Injection Post-sura-vec Administration

Data cut: May 26, 2025.
*One participant withdrew after their Month 2 visit and is not included. One additional participant was enrolled and has only completed visits through Week 30.
This is a preliminary analysis performed by REGENXBIO for an ongoing trial.

# Participants Had Meaningful Reduction in Anti-VEGF Injection Burden in 2nd Eyes Treated

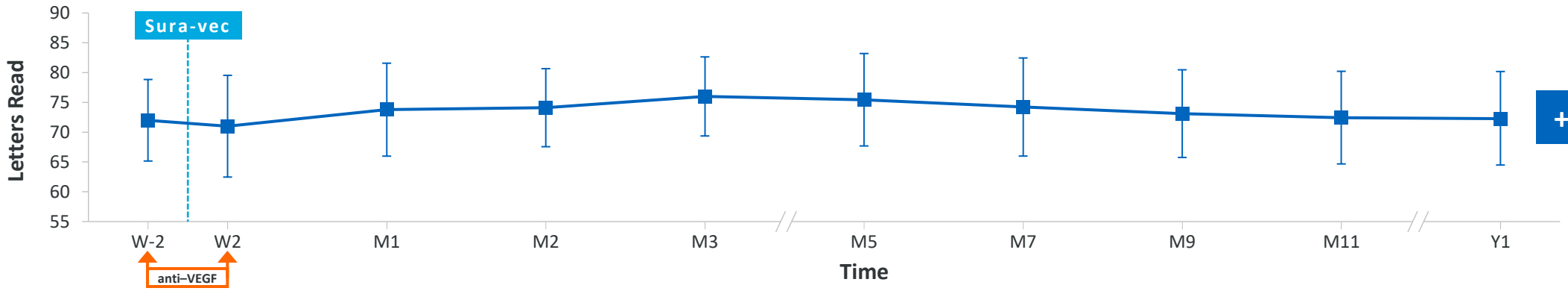
## Annualized Injection Rate Based on 1 Year Data




Data cut: May 26, 2025.
Average annualized injections in the past year is: (Total # of prior injections)/(minimum(366 days, Duration between first injection and Week -2)/365.25) and includes anti-VEGF injection at baseline visit 1 (Week -2). Prior Year injections were capped at 13. Post-dosing annualized rate is calculated based on supplemental injections through year
One participant withdrew after their Month 2 visit, so one additional participant was enrolled and has only completed visits through Week 30; this analysis includes data from both of those participants.
VEGF: vascular endothelial growth factor.
This is a preliminary analysis performed by REGENXBIO for an ongoing trial.

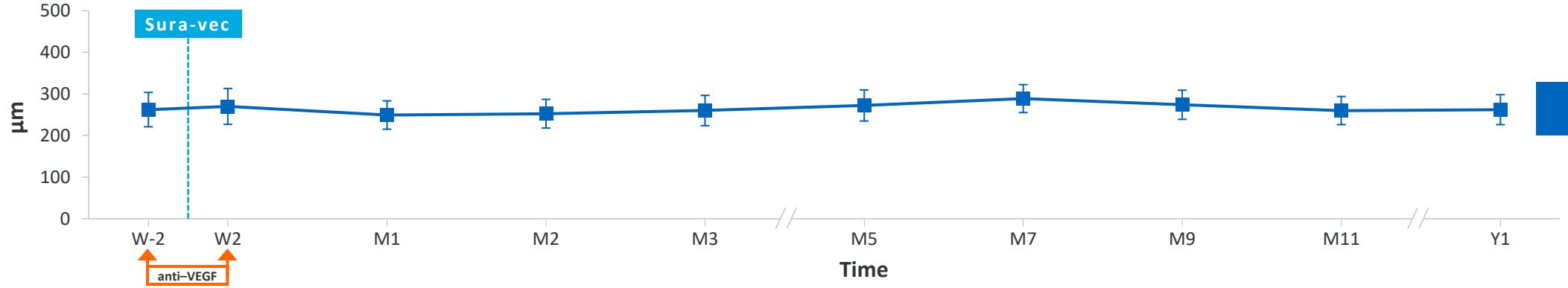
# Treated 2nd Eyes Demonstrated Sustained BCVA and CRT through 1 Year

## MEAN BEST CORRECTED VISUAL ACUITY (BCVA) 95% CI




**+0.3 Letters**

## MEAN CENTRAL RETINAL THICKNESS (CRT) 95% CI




**-0.1 µm**

$1.3x10^{11}$ GC/eye (n=9)

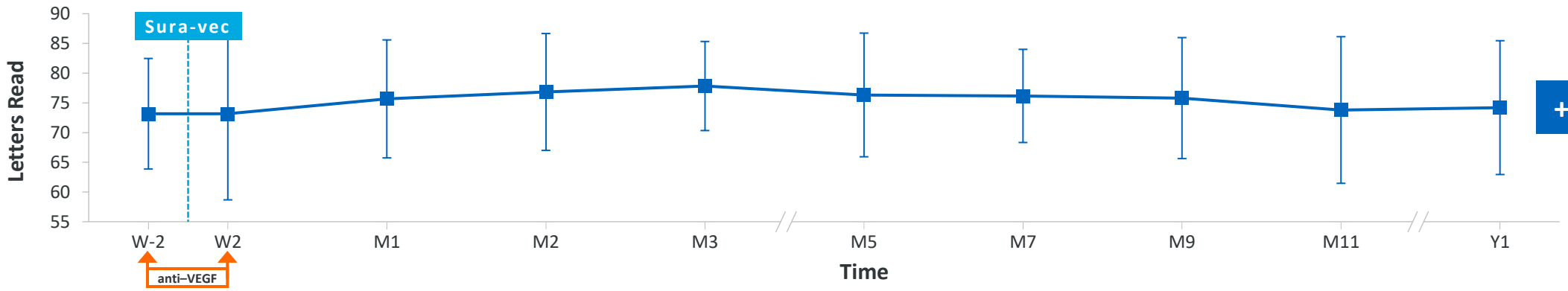
**0.9** Mean on-study Supplemental Injections through 1 Year

Data cut: May 26, 2025.
One participant withdrew after their Month 2 visit, so one additional participant was enrolled and has only completed visits through Week 30; this analysis does not include data from these participants.
GC: genome copies.
This is a preliminary analysis performed by REGENXBIO for an ongoing trial.

# Participants with No Supplemental Anti-VEGF Injections over 1 Year

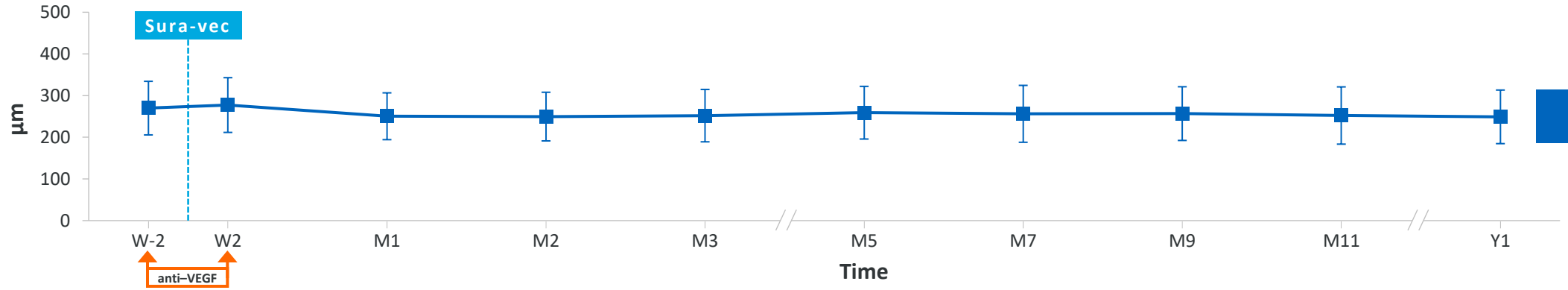
## Treated 2nd Eyes Demonstrated Sustained BCVA and CRT

### MEAN BEST CORRECTED VISUAL ACUITY (BCVA) 95% CI




**+2.4 Letters**

### MEAN CENTRAL RETINAL THICKNESS (CRT) 95% CI




**-34.8 µm**

$1.3x10^{11}$ GC/eye (n=5*)

**7.8** Mean Historical Injections in Prior Year

Data cut: May 26, 2025.
One participant withdrew after their Month 2 visit, so one additional participant was enrolled and has only completed visits through Week 30; this analysis does not include data from these participants.
GC: genome copies.
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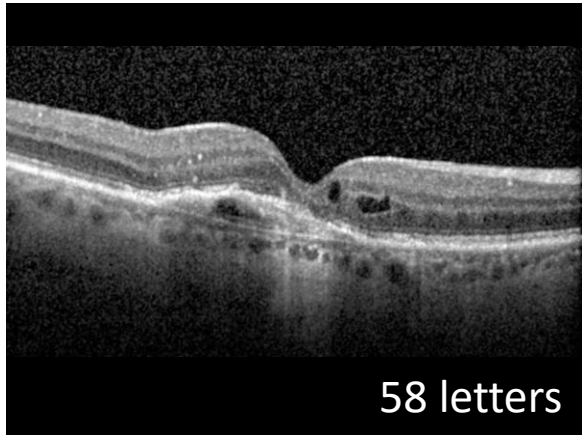
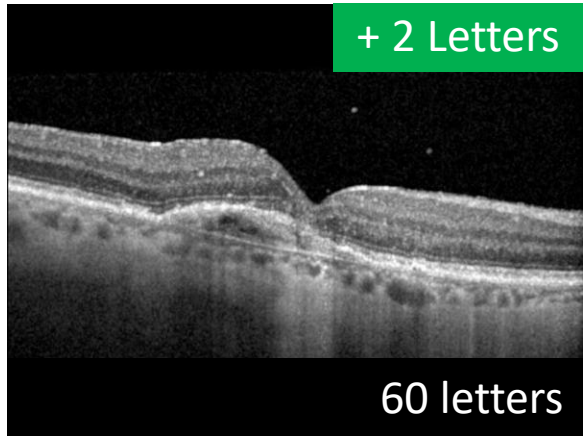
# Participant Case: Pivotal Doses Administered in Both Eyes

## Injection-free in Both Eyes

### 1st Eye: OD
**Treated in Phase 2 PD Study**
**Low Dose**
**$6.4x10^{10}$ GC/eye**



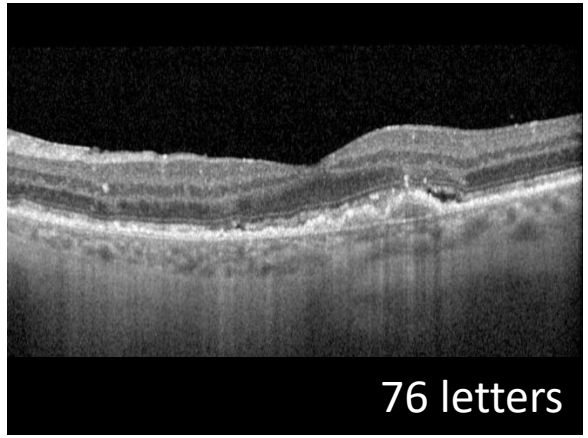

**Baseline**



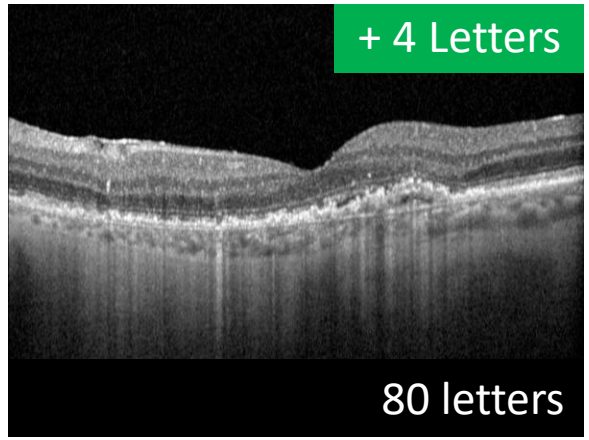

**1 Year**

**Received 10 injections in the prior year**

### 2nd Eye: OS
**High Dose**
**$1.3x10^{11}$ GC/eye**




**Baseline**




**1 Year**

**Received 6 injections in the prior year**

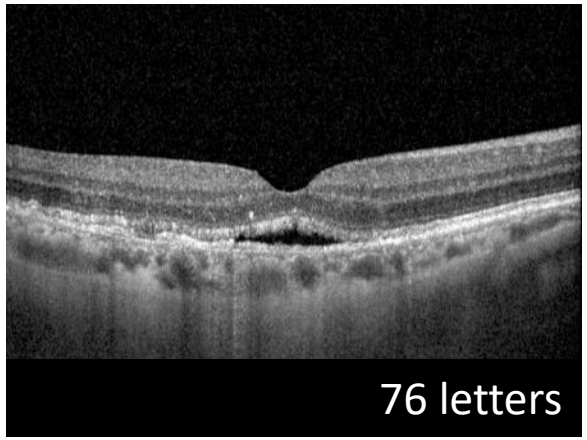
Data cut: May 26, 2025.
This slide presents results from an individual participant and is not indicative of outcomes experienced by all participants in this trial.
GC: genome copies; PD: pharmacodynamic.
This is a preliminary analysis performed by REGENXBIO for an ongoing trial.


Case courtesy of Ashkan Abbey, MD

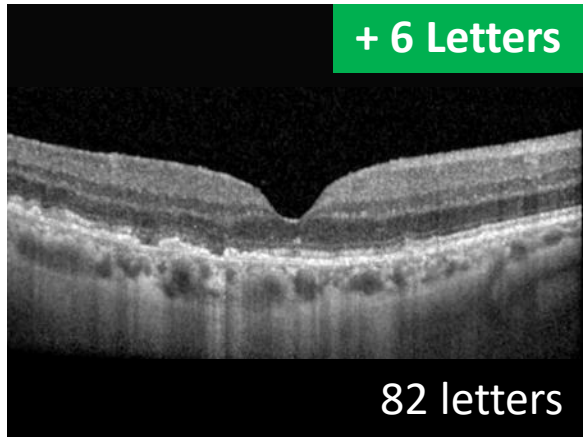
# Participant Case: Pivotal Doses Administered in Both Eyes

## Injection-free in Both Eyes

### 1st Eye: OD
Treated in Phase 2 PD Study
Low Dose
$6.4 \times 10^{10}$ GC/eye



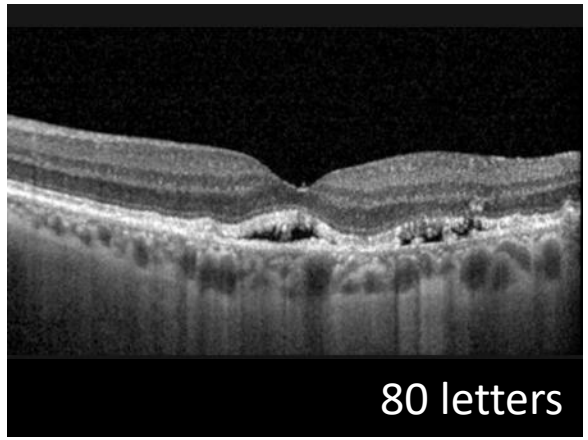

Baseline



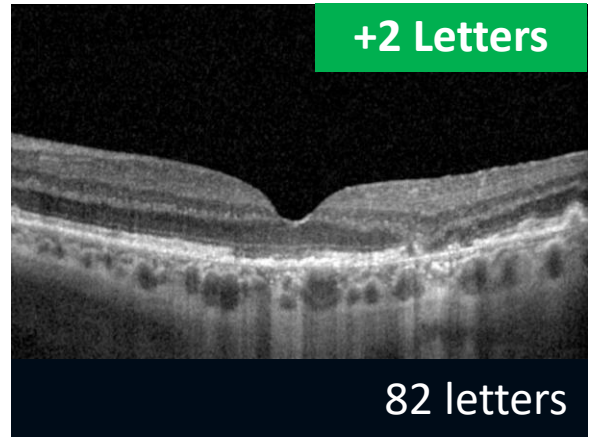

1 Year

Received 9 injections in the prior year

### 2nd Eye: OS
High Dose
$1.3 \times 10^{11}$ GC/eye




Baseline




1 Year

Received 7 injections in the prior year

Data cut for first eye: September 11, 2024; second eye: May 26, 2025.
This slide presents results from an individual participant and is not indicative of outcomes experienced by all participants in this trial.
GC: genome copies; PD: pharmacodynamic.
This is a preliminary analysis performed by REGENXBIO for an ongoing trial.


Case courtesy of Stephen Huddleston, MD

# 2nd Eyes Treated Achieved Similar Safety and Efficacy Outcomes to 1st Eyes Treated with Sura-vec

## 1 Year Results of Sura-vec Subretinal 2nd Eye Treatment

**Well-tolerated**

**Sustained vision and anatomy**

**Meaningful reduction in anti-VEGF injections in participants with high treatment burden**

- 60% Injection-free
- 80% Required 0 or 1 Supplemental injection
- 93% Reduction in anti-VEGF treatment burden

Data cut: May 26, 2025.
Results from Open label participants only.
Average annualized injections in the year prior to treatment with sura-vec: 9.0 injections.
This is a preliminary analysis performed by REGENXBIO for an ongoing trial.

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Thank You