Suprachoroidal Delivery of Investigational ABBV-RGX-314 for Neovascular AMD: Results from the Phase II AAVIATE® Study

January 16, 2024

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Agenda

- **Welcome**
- **Suprachoroidal Delivery of Investigational ABBV-RGX-314 for Neovascular AMD: Results from the Phase II AAVIATE® Study**
  - Data review
  - Data discussion with retina specialists
- **Q&A**

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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]

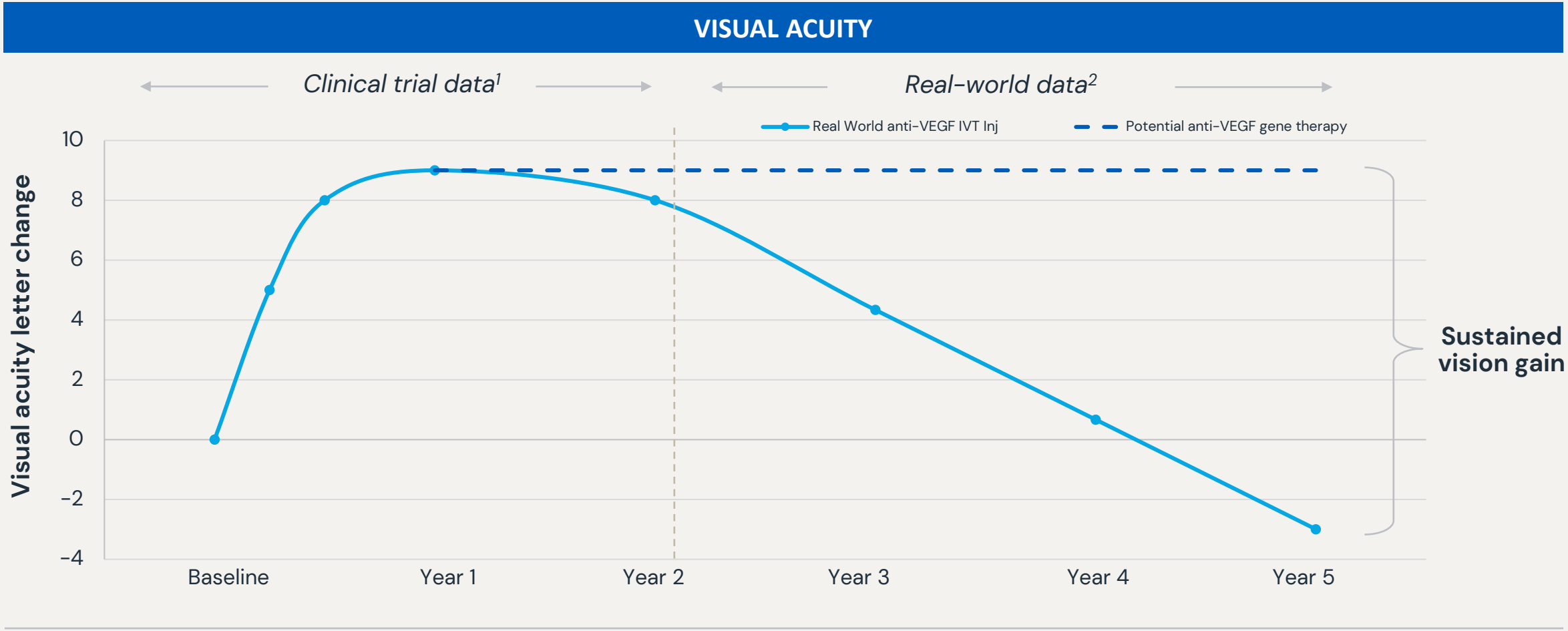
90%	4K	400K
of Retina Specialists Are Surgically Trained	Retina Surgical Sites	Vitrectomy Surgeries

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

Unlike Real World experience, a single treatment with ABBV-RGX-314 has the potential to close the gap between randomized clinical trials and real-world outcomes



1. HARBOR (n = 1098) and CATT data (n = 1208); 2. CATT data; Potential anti-VEGF gene therapy curve hypothesized

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

STUDY OVERVIEW

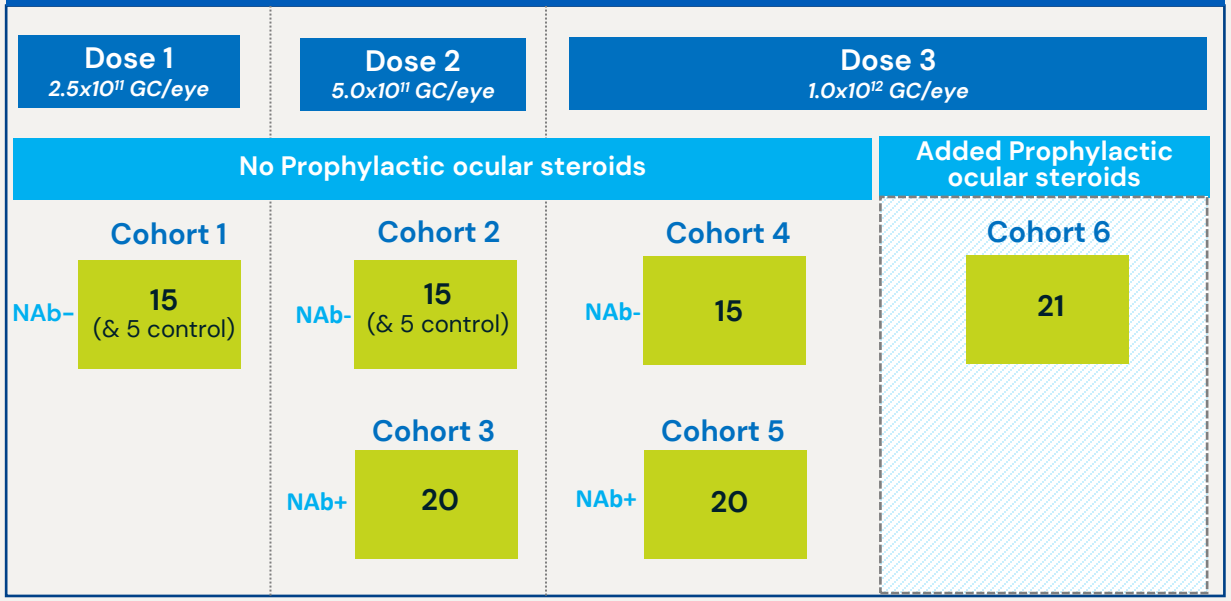
- Phase II, dose escalation study
- Region: US
- 116 Subjects
- Key Outcome measures:
  - BCVA
  - Safety and tolerability of ABBV-RGX-314
  - CRT*
  - Additional anti-VEGF injections post ABBV-RGX-314

DATA READOUTS

Latest Readouts

- Cohort 1-4 (DL1-3) at 6 months
- Cohort 6 (DL3) at 6-26 weeks safety, with prophylactic topical steroids

STUDY DESIGN



*CRT: central retinal thickness as measured by Spectral Domain Optical Coherence Tomography (SD-OCT)

ABBV-RGX–314 for Treatment of Neovascular Age-related Macular Degeneration (nAMD)

ABBV-RGX–314 PRODUCT CANDIDATE

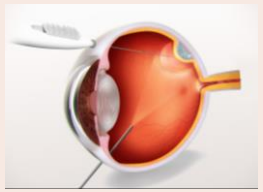
Vector: AAV8

Gene: anti-VEGF fab

Route of administration:

Subretinal (nAMD) or

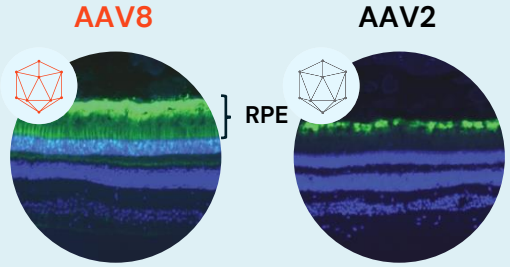
Suprachoroidal (nAMD/DR)



Mechanism of action:

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

Improved AAV vector technology



More efficient gene delivery to the RPE[1]

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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
- **ABBV-RGX–314 gene encodes an anti-VEGF mAb fragment (fab)**

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ABBV-RGX–314: 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

AAVIATE® Baseline Characteristics – All Patients (Dose Levels 1–3)

	Variable	Control Ranibizumab (N=10)	Dose Level 1 2.5×10^{11} (N=15)	Dose Level 2 5.0×10^{11} (N=35)	Dose Level 3 1.0×10^{12} (N=56)	Total (N=116)
BASELINE	**Mean Age** (Years)	75.9	74.0	74.9	77.3	76.0
	Screening BCVA (Letters)	72.7	75.1	71.9	72.8	72.8
	Screening OCT (Microns)	240.3	269.2	270.0	247.9	256.7
	Phakic n (%)	3 (30.0%)	6 (40.0%)	17 (48.6%)	29 (51.8%)	55 (47.4%)
PRIOR THERAPY	**Months Since nAMD Diagnosis** (Mean)	27.1	30.2	19.0	20.9	22.1
	# Injections Since nAMD Diagnosis (Mean)	13.4	20.6	10.3	12.4	12.9
	# Injections in the Past Year (includes 1 protocol mandated)	6.8	7.2	6.1	6.3	6.4
	Average Annualized Injections in the Past Year ((includes 1 protocol mandated)	8.8	9.7	8.8	8.9	9.0

Average annualized injections in the past year is: (Total # of prior injections)/(minimum(366 days, Duration between first injection and Day 1)/365.25). One patient in DL3 is missing number of historical injections.

AAVIATE® Dose Level 1–3 Interim Safety Summary through 6 Months

ABBV-RGX-314 has been well-tolerated in Dose Level 1–3 for AAVIATE (n=106)

- No study drug-related SAEs
- No cases of chorioretinitis, vasculitis, occlusion, or hypotony

Common Ocular TEAEs[1] in the Study Eye through Month 6	Dose Level 1 **No** PPX (N=15)	Dose Level 2 **No** PPX (N=35)	Dose Level 3 (N=56)		
			No PPX (N=35)	**With** PPX One-time Subtenon Steroid (N=11)	**With** PPX Topical Steroid (N=10)
Episcleritis[2]	0	5 (14.3%)	13 (37.1%)	2 (18.2%)	3 (30.0%)
Conjunctival Hyperemia	2 (13.3%)	2 (5.7%)	14 (40%)	2 (18.2%)	1 (10.0%)
Intraocular Inflammation (IOI)[3]	4 (26.7%)	6 (17.1%)	7 (20.0%)	2 (18.2%)	0
Conjunctival Hemorrhage	5 (33.3%)	5 (14.3%)	3 (8.6%)	2 (18.2%)	0
Intraocular Pressure Increased[4]	1 (6.7%)	5 (14.3%)	5 (14.3%)	3 (27.3%)	0

Zero cases of IOI with short-course prophylactic topical steroids

Data cut: November 06, 2023.
1. Includes AEs ≥10% of the total groups.
2. All mild to moderate (grade 1 and 2), presented within 1 week to 26 weeks post injection and resolved or are tapering off topical corticosteroids.
3. All cases were mild to moderate (range +0.5 to 2+), most presented 2-6 weeks post injection, predominantly as anterior cells on slit lamp examination. Resolved on topical corticosteroids
4. Intraocular pressure increased and ocular hypertension have been combined into one group. All mild to moderate and all controlled.

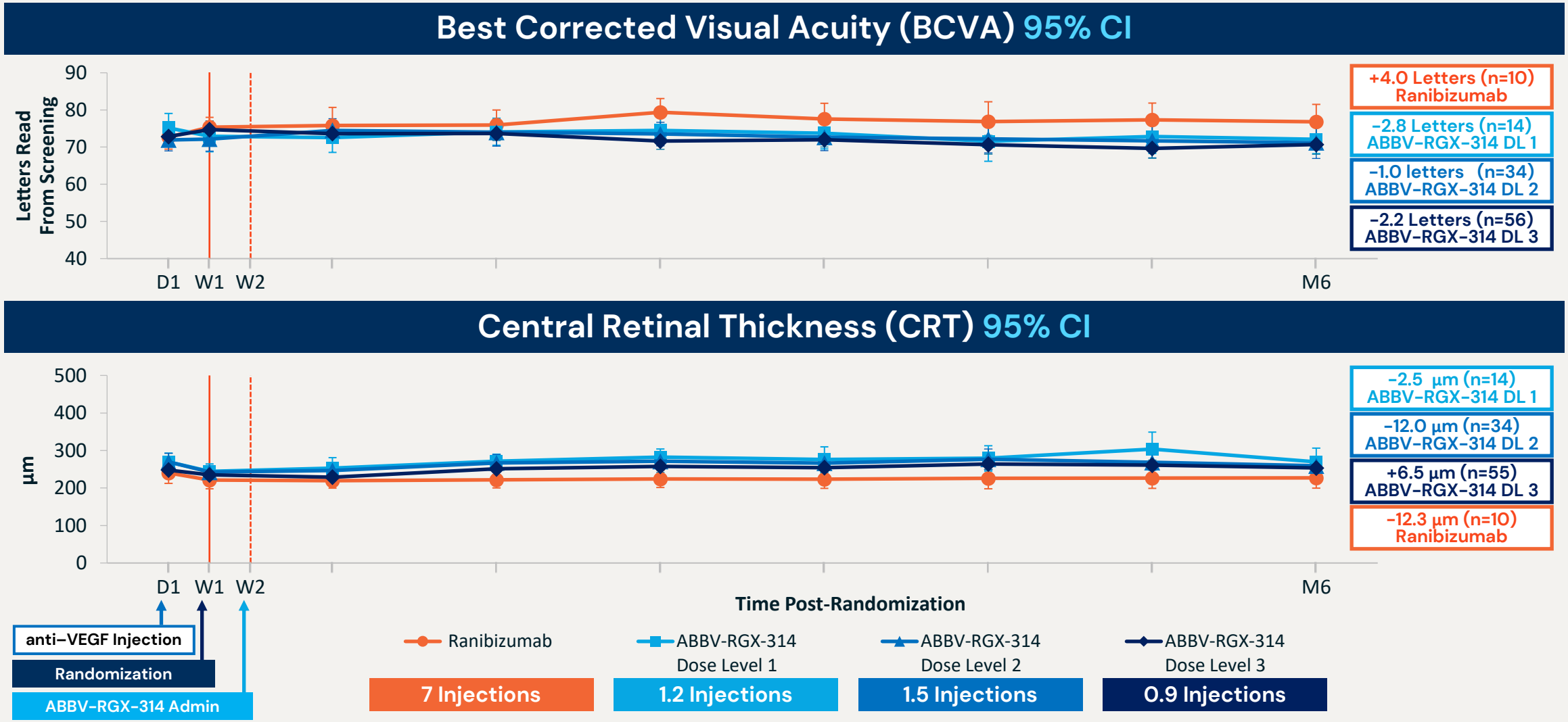
AAVIATE® Cohort 6 with Short-course Prophylactic Ocular Steroids: Zero cases of IOI with Topical Steroids

ABBV-RGX-314

	SUBJECT	Dosing	D2	W1	W2	W4	W6	W8	W10	W12	W14	W16	W18	W20	W22	W24	W26
Periocular Steroid	COHORT 6		Periocular Steroid														
	Patient 1			0	0	0	0		0		–		0		0		0
	Patient 2			0	0	0	0		0		0		0		0		0
	Patient 3			0	0	0	0		0		0		0		0		0
	Patient 4			0	–	0	0		0		0.5* AC		0		0		0
	Patient 5			0	0	0	0		0		0		0		0		0
	Patient 6			0	0	0	0		0		0		0		0		0
	Patient 7			0	0	0	0		0		0		0		0		0
	Patient 8			0	1* AC	0	0		0		0		0		0		0
	Patient 9			0	0	0	0		0		0		0		0		0
	Patient 10			0	0	0	0		0		0		0		0		0
	Patient 11[1]			0	0	0	0		0		0		0		0		0
Topical Steroid			Topical Steroid Drops (7 weeks)														
	Patient 1			0	0	0	0		0		0		0		0		0
	Patient 2			0	0	0	0		0		0		0		0		0
	Patient 3			0	0	0	0		0		0		0		0		0
	Patient 4			0	0	–	0		0		0		0		0		0
	Patient 5			0	0	0	0		0		0		0		0		0
	Patient 6			–	0	0	0		0		0		0		0		0
	Patient 7			0	0	0	0		0		0		0		0		0
	Patient 8			0	0	0	0		0		0		0		0		0
	Patient 9			0	0	0	0		0		0		0		0		0
	Patient 10			0	0	0	0		0		0		0		0		0

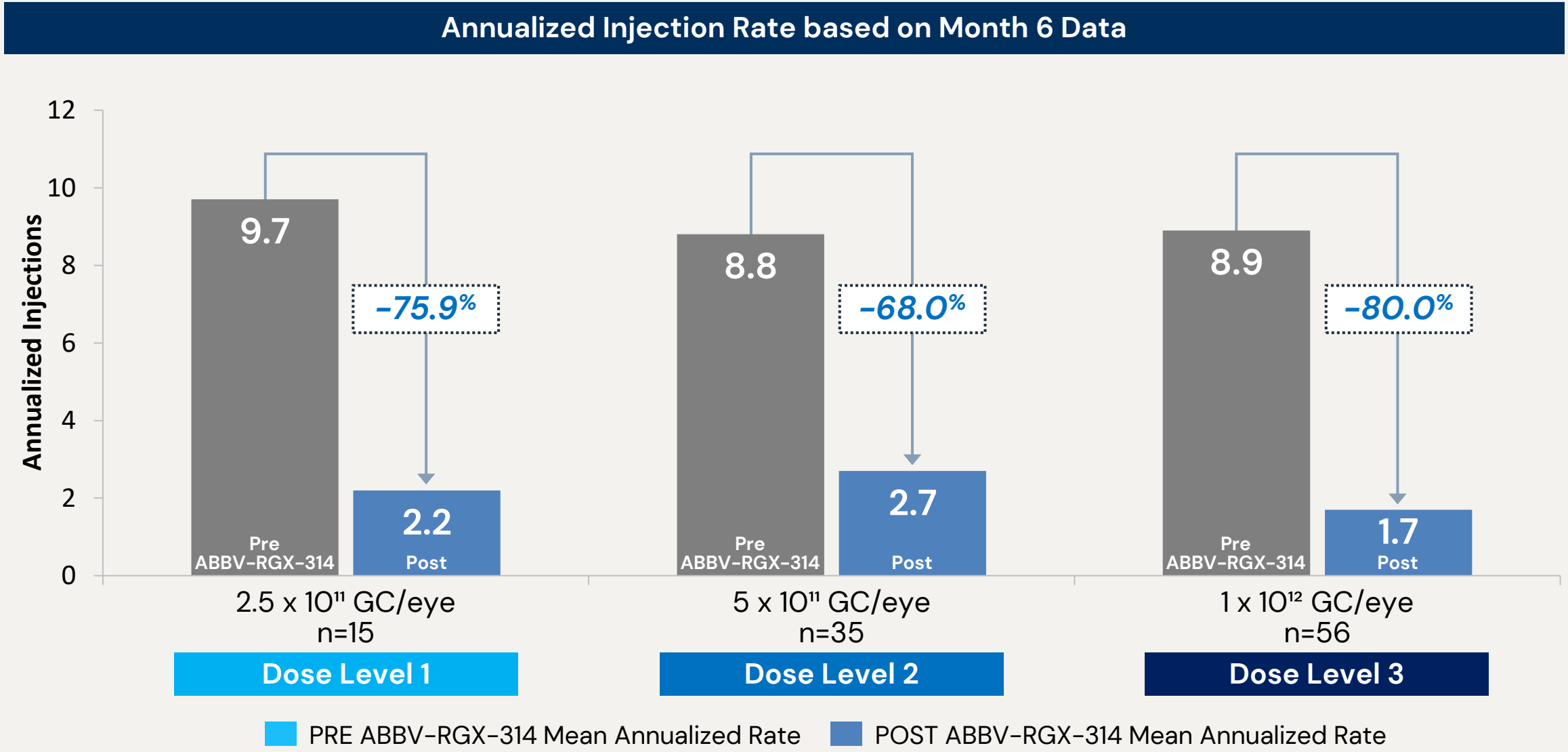
Data cut: November 06, 2023.
*Topical steroids PRN.
1. Subject received an incomplete dose of ABBV-RGX-314
IOI: Intraocular Inflammation: anterior chamber cells and flare, vitreous chamber cells and haze. Timepoints are post-dosing.

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.

Mean Change in Annualized Injection Rate PRE and POST ABBV-RGX-314 by Dose Level



Data cut: November 06, 2023.
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 screening visit 1 (Week -2). Post-dosing annualized rate is calculated based on supplemental injections at Month 6.

Dose Level 3: Injections Pre and Post ABBV-RGX-314 (n=56) – 6 Month Data

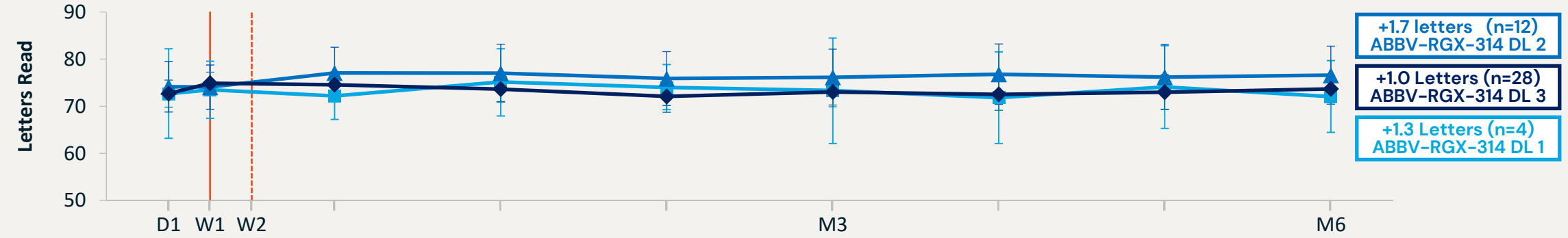
Data cut: November 06, 2023.

1. Protocol specified Ranibizumab injections included either 1 run-in injection or 2 run-in injections and 1 post ABBV-RGX-314 injection.

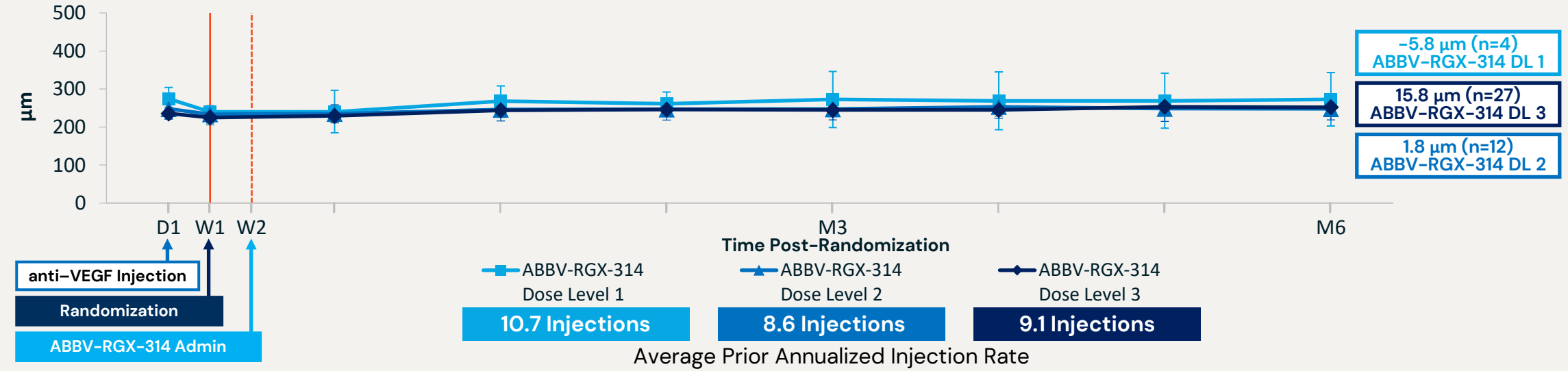
Dose Levels 1–3: No Anti-VEGF Injections over 6 Months

Mean BCVA and CRT from Day 1

Best Corrected Visual Acuity (BCVA) 95% CI



Central Retinal Thickness (CRT) 95% CI



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.

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.