Subretinal Delivery of Investigational Sura-vec Gene Therapy for Neovascular Age-Related Macular Degeneration: Long-Term Follow-Up of Phase 1/2a Participants

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Sura-vec, surabgene lomparvovec.

Disclosures

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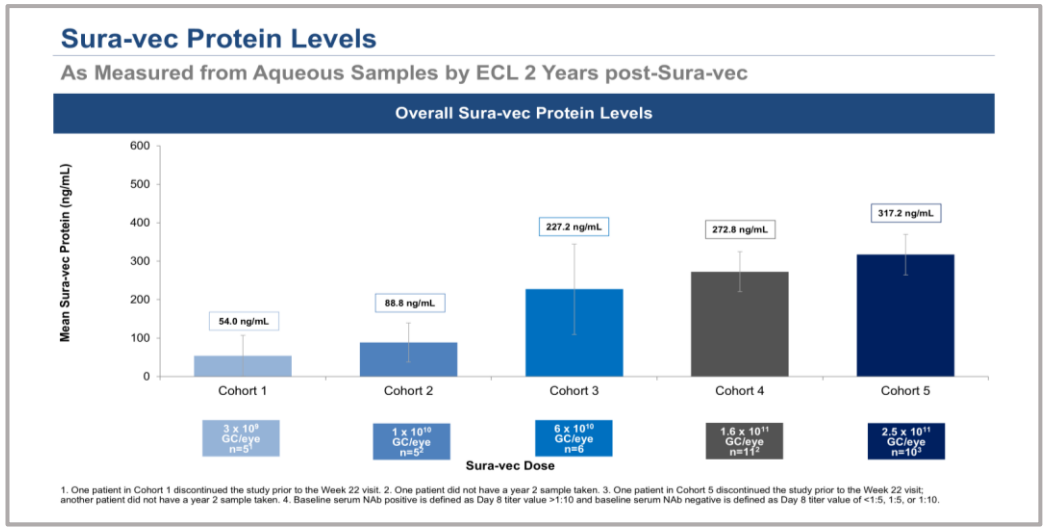
Background

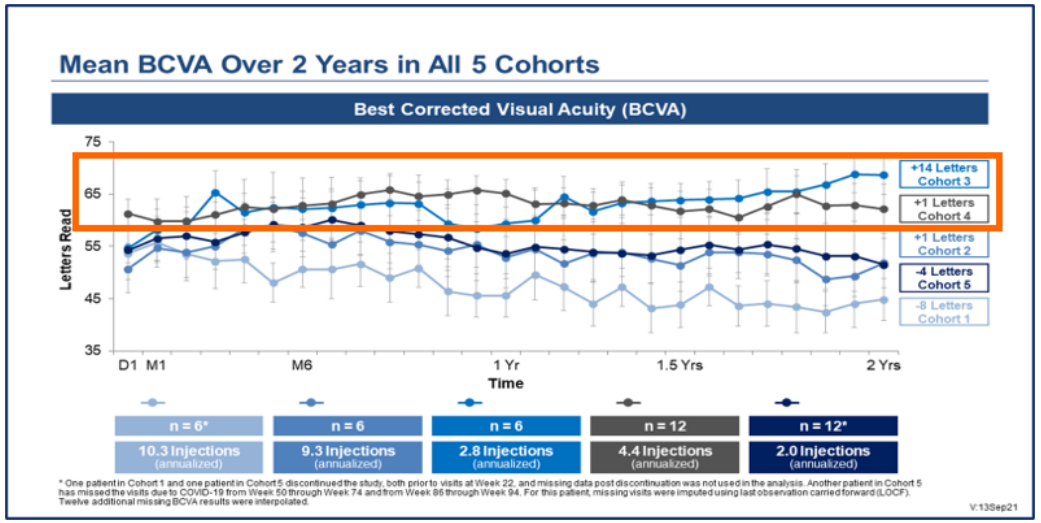
Surabgene Lomparvovec (Sura-vec; ABBV-RGX-314) Product Candidate

- **Mechanism of action:** reducing leaky blood vessel formation by giving retinal cells the ability to produce an anti-VEGF fab
- **Subretinal route of administration**
- **NAV® vector:** AAV8
- **Gene:** anti-VEGF fab

Phase I/IIa nAMD Study 2-Year Outcomes[1]

- Dose-dependent increases in protein levels were observed
- Doses similar to Cohorts 3 and 4 were moved into pivotal trials

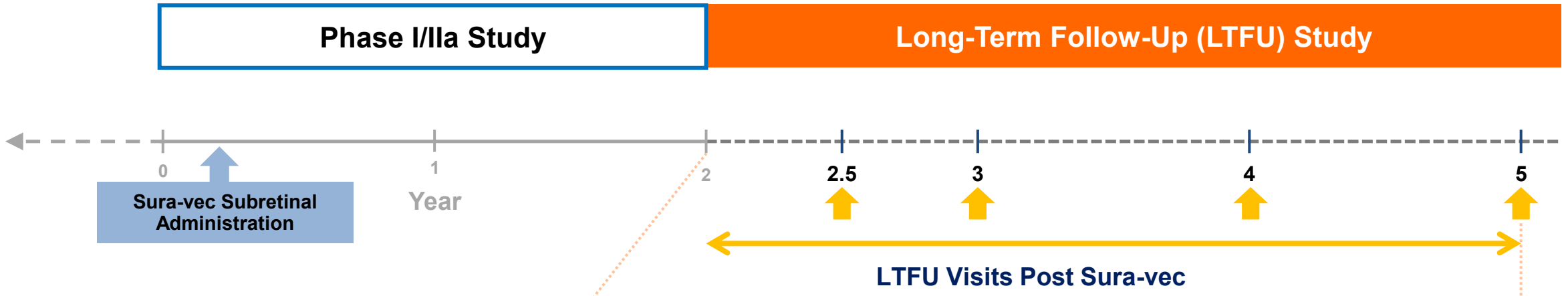




1. Campochiaro P, Avery R, Brown D et al. Gene therapy for neovascular age-related macular degeneration by subretinal delivery of RGX-314: a phase 1/2a dose-escalation study. *The Lancet*. 2024; 403, 1563-1573. doi: 10.1016/S0140-6736(24)00310-6.

AAV, adeno-associated virus; BCVA, best corrected visual acuity; ECL, electrochemiluminescence; fab, fragment antigen-binding; GC, genome copies; nAMD, neovascular age-related macular degeneration; VEGF, vascular endothelial growth factor.

Purpose and Methods



Key Phase I/IIa Eligibility Criteria

- Male or female, **50-89 years**
- nAMD with **response to anti-VEGF** at trial entry
- **BCVA: 19 - 63 ETDRS letters** (initial participant), **19 - 73 ETDRS letters** (all other participants)
- **≥ 4** anti-VEGF injections in the **8 months prior** to Visit 1
- **No exclusion for length of disease history**

LTFU

- **Purpose:** assess safety and efficacy
- **No supplemental injection criteria** (per PI discretion)

Visit Schedule & Assessments

Visit Schedule:

- Every 6 months Y2-Y3
- Annually Y3-Y5/EOS

Assessments:

- Ophthalmic exam, vision, imaging
- AEs

Adapted from Ho, Angiogenesis 2023.

AE, adverse event; BCVA, best corrected visual acuity; EOS, end of study; ETDRS, early treatment diabetic retinopathy study; LTFU, long-term follow-up; nAMD, neovascular age-related macular degeneration; OCT, optical coherence tomography; PI, principal investigator; VEGF, vascular endothelial growth factor; Y, year.

Results: Baseline Characteristics of Participants in LTFU

- 37 of the 42 participants from Ph I/IIa (RGX-314-001) across all cohorts enrolled in LTFU
- Long history of nAMD treatment

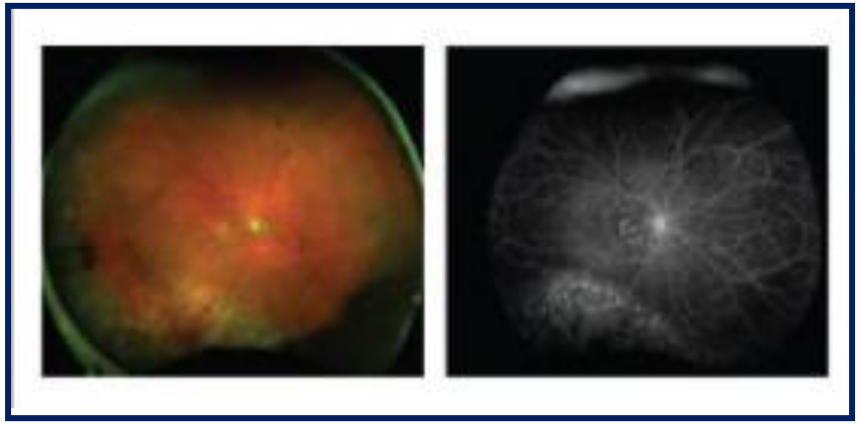
	Parameter	Cohort 1 (n=5)[a]	Cohort 2 (n=6)	Cohort 3 (n=6)	Cohort 4 (n=12)	Cohort 5 (n=8)[b]	Total (N=37)
Baseline	Mean age, years	79.0	78.0	80.0	80.3	79.9	79.6
	Baseline BCVA, letters	52.4	50.7	54.7	61.3	55.6	56.1
	Baseline CRT, µm	376.8	413.2	359.8	411.3	463.9	409.9
Prior Therapy	Months since 1st anti-VEGF injection, mean (min, max)	62.3 (16.0, 122.0)	58.5 (15.0, 131.0)	70.6 (20.0, 144.0)	57.2 (5.0, 145.0)	57.3 (9.0, 145.0)	60.3 (5.0, 145.0)
	Number of anti-VEGF injections since nAMD diagnosis, mean	48.0	32.5	34.2	35.7	31.5	35.7
	Annualized anti-VEGF injection rate in the prior year, mean	8.4	10.0	6.7	9.6	9.5	9.0

[a]1 participant died during the study.
[b]4 participants did not enroll in LTFU (3 participants did not enroll and 1 participant died in the parent study).
BCVA, best corrected visual acuity; CRT, central retinal thickness; LTFU, long-term follow-up; max, maximum; min, minimum; nAMD, neovascular age-related macular degeneration; Ph, phase; VEGF, vascular endothelial growth factor.

Results: Safety in LTFU

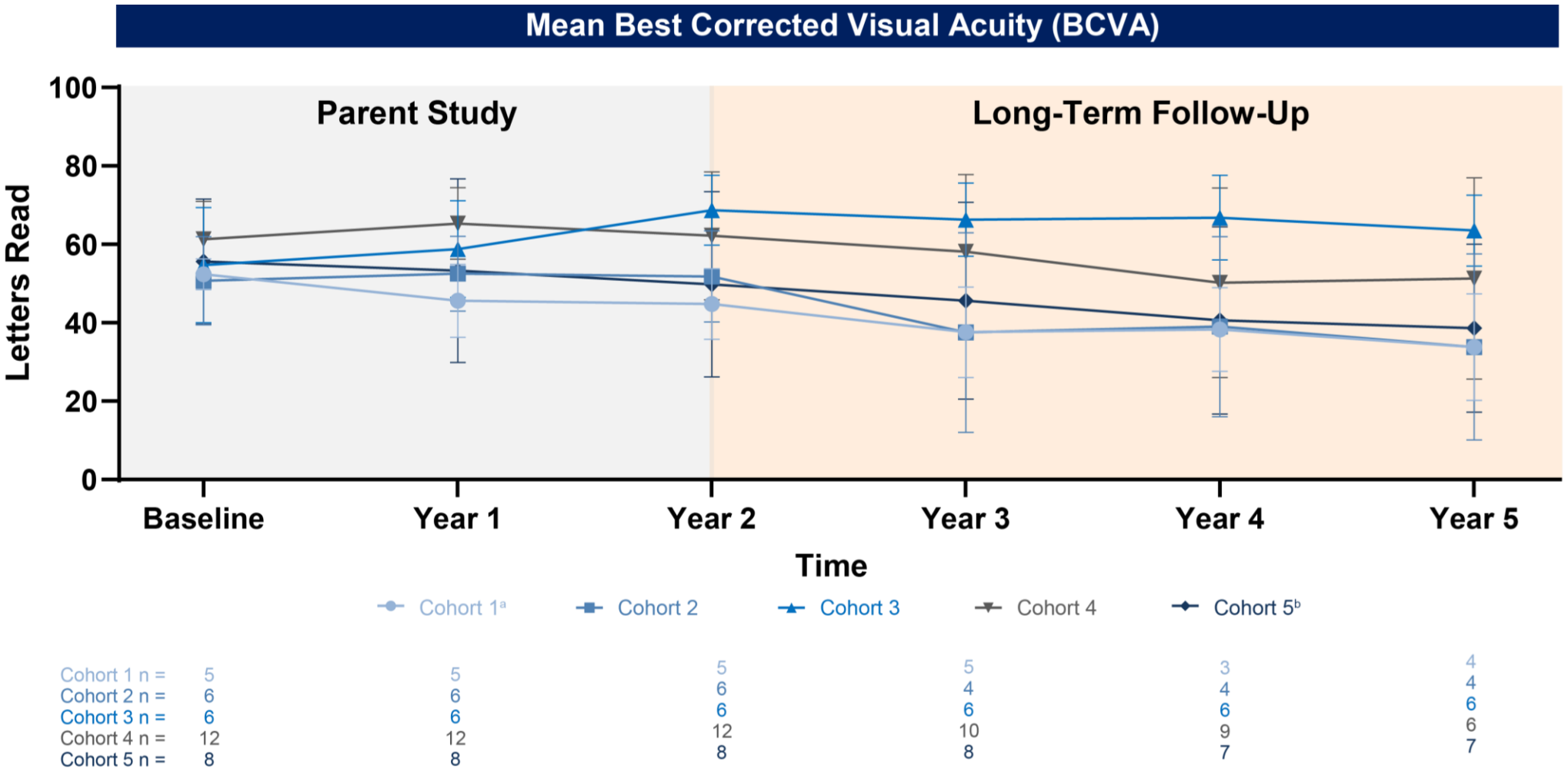
- The most frequent ocular AESI in the study eye was retinal pigmentary changes (26/37, 70.3%)
- After Year 2 in the LTFU:
  - 1 new case of peripheral retinal thinning (Cohort 4)
  - 1 new case of significant vision decrease (Cohort 5) in a participant who had macular pigmentary changes after a superior bleb in the Phase I/IIa study
- No post-operative intraocular inflammation (IOI) events deemed related to sura-vec
- 4 deaths occurred, none related to sura-vec
- 5 ocular SAEs were reported:
  - 3 were not related to sura-vec
  - 2 visual impairment events in Cohort 5 were considered related by the investigator

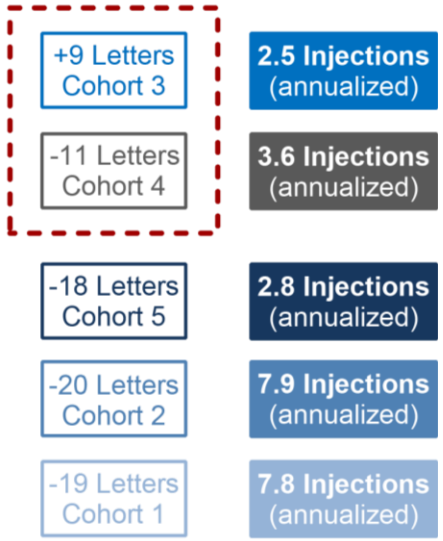
Example of Pigmentary Changes



Images from: Campochiaro P, Avery R, Brown D et al. Gene therapy for neovascular age-related macular degeneration by subretinal delivery of RGX-314: a phase 1/2a dose-escalation study. *The Lancet*. 2024; 403, 1563-1573. doi: 10.1016/S0140-6736(24)00310-6. AESI, adverse event of special interest; IOI, intraocular inflammation; LTFU, long-term follow-up; SAEs, serious adverse events.

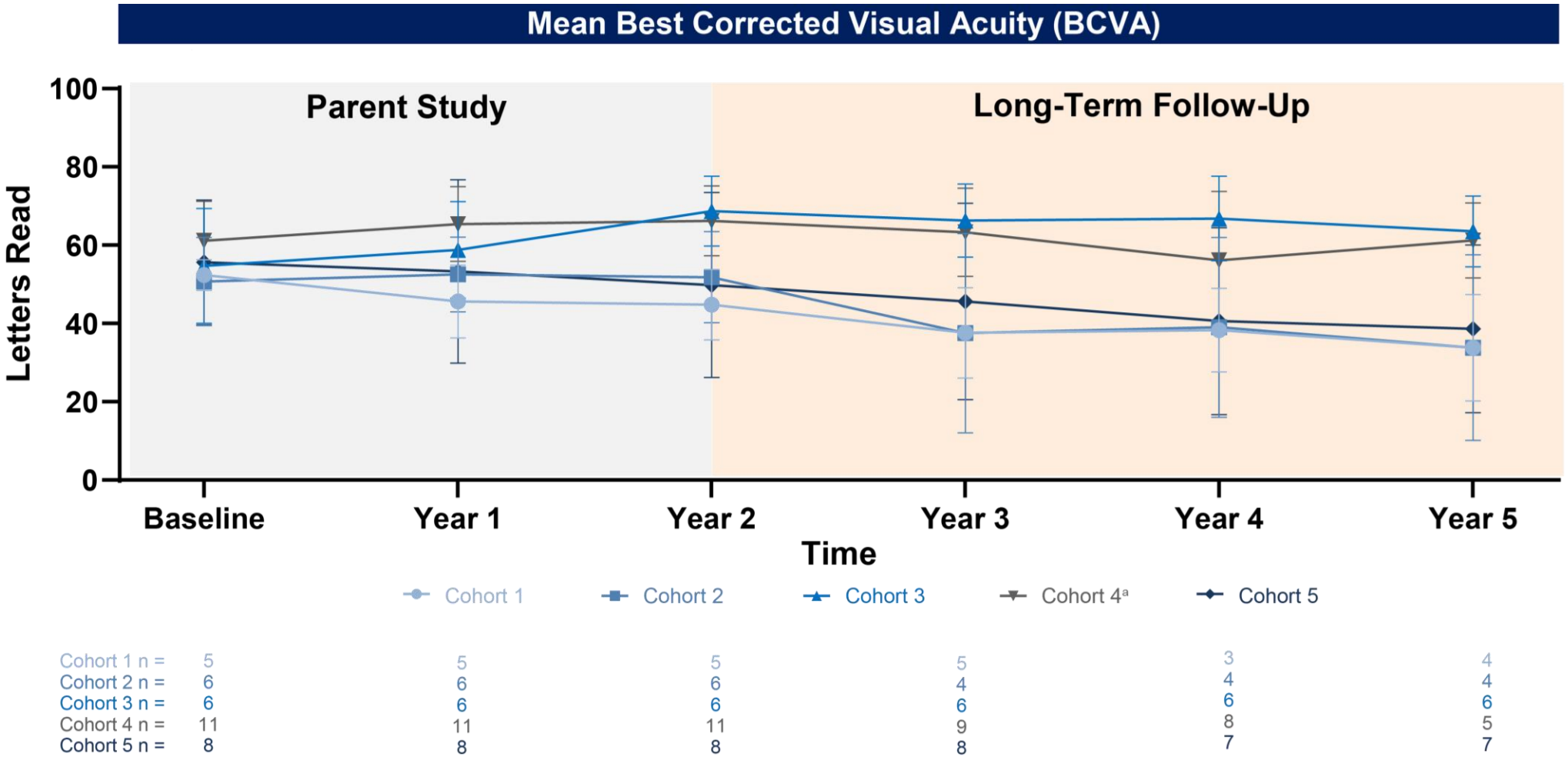
Results: Mean BCVA Through 5 Years in All 5 Cohorts





Note: Error bars represent standard deviation.
[a]1 participant died during the study.
[b]4 participants did not enroll in LTFU (three participants did not enroll and one participant died in the parent study).
BCVA, best corrected visual acuity.

Results: Mean BCVA Through 5 Years
Without PCV (refractory to anti-VEGF) Participant in Cohort 4[a]



+9 Letters Cohort 3 — 2.5 Injections (annualized)

-1 Letters Cohort 4[a] — 2.9 Injections (annualized)

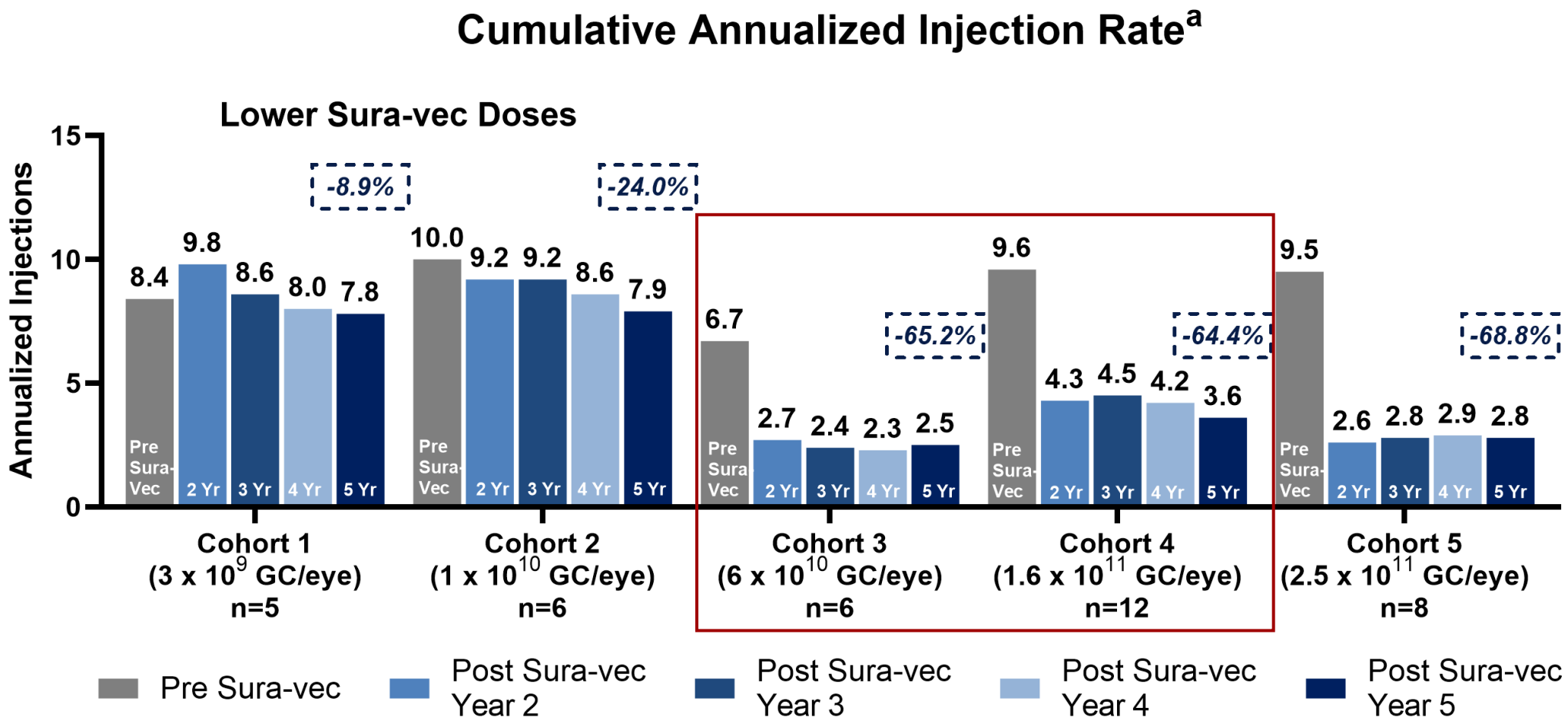
-18 Letters Cohort 5 — 2.8 Injections (annualized)

-20 Letters Cohort 2 — 7.9 Injections (annualized)

-19 Letters Cohort 1 — 7.8 Injections (annualized)

Note: Error bars represent standard deviation.
[a]Participant with PCV (refractory to anti-VEGF therapy) was excluded from Cohort 4.
BCVA, best corrected visual acuity; PCV, polypoidal choroidal vasculopathy; VEGF, vascular endothelial growth factor.

Results: Annualized Injection Rate Pre and Post Sura-Vec Through 5 Years



Note: The dashed boxes contain the injection burden reduction in Year 5 compared to the year prior.

[a]Percent reduction is calculated as (annualized anti-VEGF injection rate in the prior year - annualized supplemental anti-VEGF injection rate through the respective annual visit) / (annualized anti-VEGF injection rate in the prior year) * 100.

GC, genome copies; VEGF, vascular endothelial growth factor.

Conclusions: 5-Year Results of the Phase I/IIa Study

- No new safety signals were identified for participants who completed the study
- Cohorts 3 and 4 demonstrated stable to improved visual acuity (apart from a PCV outlier in Cohort 4)
- Cohorts 3 and 4 had > 64% reductions in injection burden after a single injection of sura-vec
- ATMOSPHERE® and ASCENT® pivotal studies are ongoing with doses similar to Cohorts 3 and 4

PCV, polypoidal choroidal vasculopathy.

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