

BCVA ETDRS Score Change from Baseline, Between Eye Comparison (All Treated Subjects)

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	Cohort 2 1x10^11 vg	
	Study Eye (N=28)	Fellow Eye (N=28)
Change from Baseline at Month 12		
ANCOVA [1]		
n	28	28
LS Mean (SE)	-1.9 (1.42)	1.6 (1.42)
95% C.I. for the LS Mean	-4.7, 1.0	-1.3, 4.4
LS Mean difference from Fellow Eye	-3.5	
95% C.I. for the LS Mean difference from Fellow Eye	-7.5, 0.6	
p-value (Study Eye vs Fellow Eye)	0.093	
MMRM [2]		
n	28	28
LS Mean (SE)	-2.2 (1.55)	1.9 (1.10)
95% C.I. for the LS Mean	-5.3, 0.9	-0.4, 4.1
LS Mean difference from Fellow Eye	-4.1	
95% C.I. for the LS Mean difference from Fellow Eye	-7.5, -0.6	
p-value (Study Eye vs Fellow Eye)	0.023	

Cohort 2: Subset of subjects with randomized study eye; LS: Least Squares; ANCOVA: Analysis of covariance; MMRM: Mixed model for repeated measures .

Notes: [1] The ANCOVA model includes eye (Study Eye vs Fellow Eye) as a factor and baseline BCVA as a covariate.

[2] The MMRM uses data available from all the visits (regardless of existence of missing data in those visits) after visit 4 (starting from visit 5). The model includes the terms of baseline BCVA, eye (Study Eye vs Fellow Eye), visit and visit-eye interaction, where both eyes and visits are considered as repeated measurements. An unstructured covariance matrix is used for the inter-eye correlation and longitudinal correlation of repeated measures respectively (i.e: UN@UN). The Kenward-Roger approximation is used to estimate the denominator degree of freedom. In case of convergence failure, the auto regression covariance structure is used for longitudinal correlation of repeated measures instead (i.e: UN@AR(1)). The current results use UN@UN.

Source: biib111chm/273ch202/csr/t-ef-bcva-chg-bl-eyecp-saf.sas **Run Date:** 03MAR2022

BCVA ETDRS Score Change from Baseline, Between Eye Comparison (All Treated Subjects)

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	Cohort 2 1x10^11 vg	
	Study Eye (N=28)	Fellow Eye (N=28)
Change from Baseline at Month 24		
ANCOVA [1]		
n	28	28
LS Mean (SE)	-3.0 (2.14)	2.9 (2.14)
95% C.I. for the LS Mean	-7.3, 1.3	-1.4, 7.2
LS Mean difference from Fellow Eye	-5.9	
95% C.I. for the LS Mean difference from Fellow Eye	-12.0, 0.2	
p-value (Study Eye vs Fellow Eye)	0.059	
MMRM [2]		
n	28	28
LS Mean (SE)	-3.3 (2.24)	3.2 (1.66)
95% C.I. for the LS Mean	-7.8, 1.2	-0.2, 6.6
LS Mean difference from Fellow Eye	-6.5	
95% C.I. for the LS Mean difference from Fellow Eye	-11.8, -1.1	
p-value (Study Eye vs Fellow Eye)	0.019	

Cohort 2: Subset of subjects with randomized study eye; LS: Least Squares; ANCOVA: Analysis of covariance; MMRM: Mixed model for repeated measures .

Notes: [1] The ANCOVA model includes eye (Study Eye vs Fellow Eye) as a factor and baseline BCVA as a covariate.

[2] The MMRM uses data available from all the visits (regardless of existence of missing data in those visits) after visit 4 (starting from visit 5). The model includes the terms of baseline BCVA, eye (Study Eye vs Fellow Eye), visit and visit-eye interaction, where both eyes and visits are considered as repeated measurements. An unstructured covariance matrix is used for the inter-eye correlation and longitudinal correlation of repeated measures respectively (i.e: UN@UN). The Kenward-Roger approximation is used to estimate the denominator degree of freedom. In case of convergence failure, the auto regression covariance structure is used for longitudinal correlation of repeated measures instead (i.e: UN@AR(1)). The current results use UN@UN.

Source: biib111chm/273ch202/csr/t-ef-bcva-chg-bl-eyecp-saf.sas **Run Date:** 03MAR2022