

BCVA ETDRS Score Change from Baseline by Surgery site (All Treated Subjects)

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Visit	Oxford				Moorfields			
	Cohort 1 1x10^11 vg		Cohort 2 1x10^11 vg		Cohort 1 1x10^11 vg		Cohort 2 1x10^11 vg	
	Study Eye (N=12)	Fellow Eye (N=12)	Study Eye (N=12)	Fellow Eye (N=12)	Study Eye (N=18)	Fellow Eye (N=18)	Study Eye (N=16)	Fellow Eye (N=16)
Baseline								
Observed								
n	12	12	12	12	18	18	16	16
Mean (SD)	81.3 (3.72)	83.8 (2.05)	81.3 (3.72)	83.8 (2.05)	80.6 (6.71)	83.2 (6.27)	81.6 (6.28)	82.3 (5.86)
Median	83.0	84.0	83.0	84.0	79.0	83.5	81.0	82.5
Min, Max	75, 85	79, 87	75, 85	79, 87	70, 91	72, 94	72, 91	72, 92
Visit 4 (Day 7)								
Observed								
n	12	12	12	12	18	18	16	16
Mean (SD)	80.1 (7.67)	84.2 (3.30)	80.1 (7.67)	84.2 (3.30)	72.9 (14.14)	83.3 (5.18)	73.6 (14.90)	83.3 (5.46)
Median	83.0	85.0	83.0	85.0	76.5	84.0	78.0	84.0
Min, Max	58, 85	75, 88	58, 85	75, 88	38, 91	73, 92	38, 91	73, 92
Change from Baseline								
n	12	12	12	12	18	18	16	16
Mean (SD)	-1.2 (5.84)	0.4 (3.94)	-1.2 (5.84)	0.4 (3.94)	-7.6 (12.09)	0.1 (4.40)	-8.0 (12.81)	1.0 (3.33)
Median	-0.5	1.0	-0.5	1.0	-5.0	0.0	-5.0	1.0
Min, Max	-17, 6	-9, 6	-17, 6	-9, 6	-39, 4	-12, 7	-39, 4	-4, 7
95% C.I.	(-4.9, 2.5)	(-2.1, 2.9)	(-4.9, 2.5)	(-2.1, 2.9)	(-13.6, -1.6)	(-2.1, 2.3)	(-14.8, -1.2)	(-0.8, 2.8)

Cohort 1: All subjects; Cohort 2: Subset of subjects with randomized study eye.

Notes: [1] 95% Confidence Intervals (C.I.) of the mean change based on ttest.

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

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Visit	Oxford				Moorfields			
	Cohort 1 1x10^11 vg		Cohort 2 1x10^11 vg		Cohort 1 1x10^11 vg		Cohort 2 1x10^11 vg	
	Study Eye (N=12)	Fellow Eye (N=12)	Study Eye (N=12)	Fellow Eye (N=12)	Study Eye (N=18)	Fellow Eye (N=18)	Study Eye (N=16)	Fellow Eye (N=16)
Visit 5 (Month 1)								
Observed								
n	12	12	12	12	18	18	16	16
Mean (SD)	81.8 (6.07)	84.6 (3.26)	81.8 (6.07)	84.6 (3.26)	77.9 (9.89)	84.6 (5.52)	78.3 (10.17)	84.5 (5.69)
Median	83.0	84.5	83.0	84.5	80.5	84.5	81.0	84.5
Min, Max	67, 90	78, 90	67, 90	78, 90	51, 93	76, 94	51, 93	76, 94
Change from Baseline								
n	12	12	12	12	18	18	16	16
Mean (SD)	0.6 (3.73)	0.8 (3.13)	0.6 (3.73)	0.8 (3.13)	-2.7 (7.61)	1.3 (5.73)	-3.3 (7.67)	2.3 (4.75)
Median	1.0	1.0	1.0	1.0	-2.0	1.0	-2.0	2.0
Min, Max	-8, 5	-6, 6	-8, 5	-6, 6	-27, 8	-13, 12	-27, 8	-4, 12
95% C.I.	(-1.8, 3.0)	(-1.2, 2.8)	(-1.8, 3.0)	(-1.2, 2.8)	(-6.5, 1.1)	(-1.5, 4.2)	(-7.4, 0.8)	(-0.3, 4.8)

Cohort 1: All subjects; Cohort 2: Subset of subjects with randomized study eye.

Notes: [1] 95% Confidence Intervals (C.I.) of the mean change based on ttest.

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BCVA ETDRS Score Change from Baseline by Surgery site (All Treated Subjects)

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Visit	Oxford				Moorfields			
	Cohort 1 1x10 ¹¹ vg		Cohort 2 1x10 ¹¹ vg		Cohort 1 1x10 ¹¹ vg		Cohort 2 1x10 ¹¹ vg	
	Study Eye (N=12)	Fellow Eye (N=12)	Study Eye (N=12)	Fellow Eye (N=12)	Study Eye (N=18)	Fellow Eye (N=18)	Study Eye (N=16)	Fellow Eye (N=16)
Visit 6 (Month 3)								
Observed								
n	12	12	12	12	18	18	16	16
Mean (SD)	81.7 (5.87)	84.5 (2.28)	81.7 (5.87)	84.5 (2.28)	78.3 (10.81)	84.9 (5.95)	79.3 (11.00)	84.9 (6.24)
Median	82.5	84.0	82.5	84.0	80.5	84.0	82.0	84.0
Min, Max	69, 89	82, 89	69, 89	82, 89	50, 93	73, 93	50, 93	73, 93
Change from Baseline								
n	12	12	12	12	18	18	16	16
Mean (SD)	0.4 (4.78)	0.8 (2.53)	0.4 (4.78)	0.8 (2.53)	-2.2 (7.86)	1.7 (5.79)	-2.4 (8.28)	2.6 (4.94)
Median	2.0	0.5	2.0	0.5	-1.0	1.0	-1.0	2.0
Min, Max	-8, 6	-4, 4	-8, 6	-4, 4	-28, 6	-12, 12	-28, 6	-5, 12
95% C.I.	(-2.6, 3.5)	(-0.9, 2.4)	(-2.6, 3.5)	(-0.9, 2.4)	(-6.1, 1.7)	(-1.2, 4.5)	(-6.8, 2.0)	(-0.0, 5.3)

Cohort 1: All subjects; Cohort 2: Subset of subjects with randomized study eye.

Notes: [1] 95% Confidence Intervals (C.I.) of the mean change based on ttest.

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BCVA ETDRS Score Change from Baseline by Surgery site (All Treated Subjects)

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Visit	Oxford				Moorfields			
	Cohort 1 1x10^11 vg		Cohort 2 1x10^11 vg		Cohort 1 1x10^11 vg		Cohort 2 1x10^11 vg	
	Study Eye (N=12)	Fellow Eye (N=12)	Study Eye (N=12)	Fellow Eye (N=12)	Study Eye (N=18)	Fellow Eye (N=18)	Study Eye (N=16)	Fellow Eye (N=16)
Visit 7 (Month 6)								
Observed								
n	12	12	12	12	18	18	16	16
Mean (SD)	81.4 (6.75)	84.2 (3.56)	81.4 (6.75)	84.2 (3.56)	79.1 (10.94)	84.4 (5.48)	79.4 (11.57)	84.6 (5.62)
Median	83.0	84.5	83.0	84.5	79.5	86.0	80.0	86.0
Min, Max	63, 89	76, 90	63, 89	76, 90	49, 94	75, 93	49, 94	75, 93
Change from Baseline								
n	12	12	12	12	18	18	16	16
Mean (SD)	0.2 (4.45)	0.4 (3.68)	0.2 (4.45)	0.4 (3.68)	-1.5 (8.33)	1.2 (6.13)	-2.3 (8.55)	2.4 (4.83)
Median	0.5	0.5	0.5	0.5	-0.5	3.0	-1.5	3.5
Min, Max	-12, 6	-6, 6	-12, 6	-6, 6	-29, 10	-15, 11	-29, 10	-8, 11
95% C.I.	(-2.7, 3.0)	(-1.9, 2.8)	(-2.7, 3.0)	(-1.9, 2.8)	(-5.6, 2.6)	(-1.8, 4.3)	(-6.8, 2.3)	(-0.2, 4.9)

Cohort 1: All subjects; Cohort 2: Subset of subjects with randomized study eye.

Notes: [1] 95% Confidence Intervals (C.I.) of the mean change based on ttest.

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	Cohort 1 1x10 ¹¹ vg		Cohort 2 1x10 ¹¹ vg		Cohort 1 1x10 ¹¹ vg		Cohort 2 1x10 ¹¹ vg	
	Study Eye (N=12)	Fellow Eye (N=12)	Study Eye (N=12)	Fellow Eye (N=12)	Study Eye (N=18)	Fellow Eye (N=18)	Study Eye (N=16)	Fellow Eye (N=16)
Visit 8 (Month 9)								
Observed								
n	12	12	12	12	18	18	16	16
Mean (SD)	82.3 (5.73)	85.0 (3.05)	82.3 (5.73)	85.0 (3.05)	79.1 (11.35)	83.8 (7.55)	79.3 (12.04)	85.0 (6.47)
Median	83.0	85.0	83.0	85.0	80.5	84.0	82.5	85.0
Min, Max	68, 89	80, 89	68, 89	80, 89	49, 92	66, 95	49, 92	73, 95
Change from Baseline								
n	12	12	12	12	18	18	16	16
Mean (SD)	1.1 (4.12)	1.3 (2.99)	1.1 (4.12)	1.3 (2.99)	-1.5 (8.76)	0.6 (7.40)	-2.4 (8.92)	2.8 (3.75)
Median	1.0	2.0	1.0	2.0	1.0	2.0	1.0	2.0
Min, Max	-7, 10	-4, 5	-7, 10	-4, 5	-29, 7	-22, 13	-29, 7	-4, 13
95% C.I.	(-1.5, 3.7)	(-0.6, 3.1)	(-1.5, 3.7)	(-0.6, 3.1)	(-5.9, 2.9)	(-3.1, 4.3)	(-7.1, 2.4)	(0.8, 4.7)

Cohort 1: All subjects; Cohort 2: Subset of subjects with randomized study eye.

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	Cohort 1 1x10^11 vg		Cohort 2 1x10^11 vg		Cohort 1 1x10^11 vg		Cohort 2 1x10^11 vg	
	Study Eye (N=12)	Fellow Eye (N=12)	Study Eye (N=12)	Fellow Eye (N=12)	Study Eye (N=18)	Fellow Eye (N=18)	Study Eye (N=16)	Fellow Eye (N=16)
Visit 9 (Month 12)								
Observed								
n	12	12	12	12	18	18	16	16
Mean (SD)	82.2 (6.69)	84.7 (2.15)	82.2 (6.69)	84.7 (2.15)	77.1 (14.72)	84.7 (6.11)	77.4 (15.58)	84.6 (6.44)
Median	84.0	85.0	84.0	85.0	81.5	85.0	84.0	85.0
Min, Max	65, 91	81, 88	65, 91	81, 88	36, 93	73, 96	36, 93	73, 96
Change from Baseline								
n	12	12	12	12	18	18	16	16
Mean (SD)	0.9 (4.94)	0.9 (2.02)	0.9 (4.94)	0.9 (2.02)	-3.4 (11.54)	1.5 (5.61)	-4.3 (12.01)	2.4 (4.92)
Median	1.0	1.0	1.0	1.0	0.0	2.5	-0.5	3.0
Min, Max	-10, 8	-3, 4	-10, 8	-3, 4	-36, 10	-11, 12	-36, 10	-7, 12
95% C.I.	(-2.2, 4.1)	(-0.4, 2.2)	(-2.2, 4.1)	(-0.4, 2.2)	(-9.2, 2.3)	(-1.3, 4.3)	(-10.6, 2.1)	(-0.2, 5.0)

Cohort 1: All subjects; Cohort 2: Subset of subjects with randomized study eye.

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	Cohort 1 1x10^11 vg		Cohort 2 1x10^11 vg		Cohort 1 1x10^11 vg		Cohort 2 1x10^11 vg	
	Study Eye (N=12)	Fellow Eye (N=12)	Study Eye (N=12)	Fellow Eye (N=12)	Study Eye (N=18)	Fellow Eye (N=18)	Study Eye (N=16)	Fellow Eye (N=16)
Visit 10 (Month 18)								
Observed								
n	12	12	12	12	18	18	16	16
Mean (SD)	80.8 (7.63)	84.7 (4.60)	80.8 (7.63)	84.7 (4.60)	75.9 (19.84)	85.0 (5.03)	75.9 (21.06)	85.8 (4.81)
Median	84.5	85.0	84.5	85.0	83.0	86.0	84.0	86.5
Min, Max	67, 90	77, 91	67, 90	77, 91	12, 96	75, 92	12, 96	75, 92
Change from Baseline								
n	12	12	12	12	18	18	16	16
Mean (SD)	-0.5 (5.96)	0.9 (3.75)	-0.5 (5.96)	0.9 (3.75)	-4.6 (17.29)	1.8 (6.41)	-5.8 (18.05)	3.5 (4.20)
Median	0.0	1.5	0.0	1.5	1.5	2.0	1.0	2.5
Min, Max	-11, 8	-7, 5	-11, 8	-7, 5	-60, 10	-14, 11	-60, 10	-2, 11
95% C.I.	(-4.3, 3.3)	(-1.5, 3.3)	(-4.3, 3.3)	(-1.5, 3.3)	(-13.2, 4.0)	(-1.4, 5.0)	(-15.4, 3.9)	(1.3, 5.7)

Cohort 1: All subjects; Cohort 2: Subset of subjects with randomized study eye.

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	Cohort 1 1x10^11 vg		Cohort 2 1x10^11 vg		Cohort 1 1x10^11 vg		Cohort 2 1x10^11 vg	
	Study Eye (N=12)	Fellow Eye (N=12)	Study Eye (N=12)	Fellow Eye (N=12)	Study Eye (N=18)	Fellow Eye (N=18)	Study Eye (N=16)	Fellow Eye (N=16)
Visit 11 (Month 24)								
Observed								
n	12	12	12	12	18	18	16	16
Mean (SD)	83.4 (6.42)	86.0 (3.93)	83.4 (6.42)	86.0 (3.93)	74.7 (21.05)	86.2 (4.51)	74.4 (22.35)	86.0 (4.60)
Median	85.0	84.5	85.0	84.5	82.0	85.5	83.5	85.5
Min, Max	72, 92	80, 93	72, 92	80, 93	5, 93	78, 93	5, 93	78, 93
Change from Baseline								
n	12	12	12	12	18	18	16	16
Mean (SD)	2.2 (4.34)	2.3 (3.39)	2.2 (4.34)	2.3 (3.39)	-5.8 (18.76)	2.9 (4.75)	-7.3 (19.47)	3.8 (3.70)
Median	3.0	2.5	3.0	2.5	0.5	3.0	-1.0	3.5
Min, Max	-4, 9	-3, 7	-4, 9	-3, 7	-67, 11	-10, 11	-67, 11	-4, 11
95% C.I.	(-0.6, 4.9)	(0.1, 4.4)	(-0.6, 4.9)	(0.1, 4.4)	(-15.2, 3.5)	(0.6, 5.3)	(-17.6, 3.1)	(1.8, 5.7)

Cohort 1: All subjects; Cohort 2: Subset of subjects with randomized study eye.

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