Bestrophin 1 treatment trial on the effectiveness of Ravicti
ISRCTN15509883 Basic Results Summary

Participant Flow Diagram

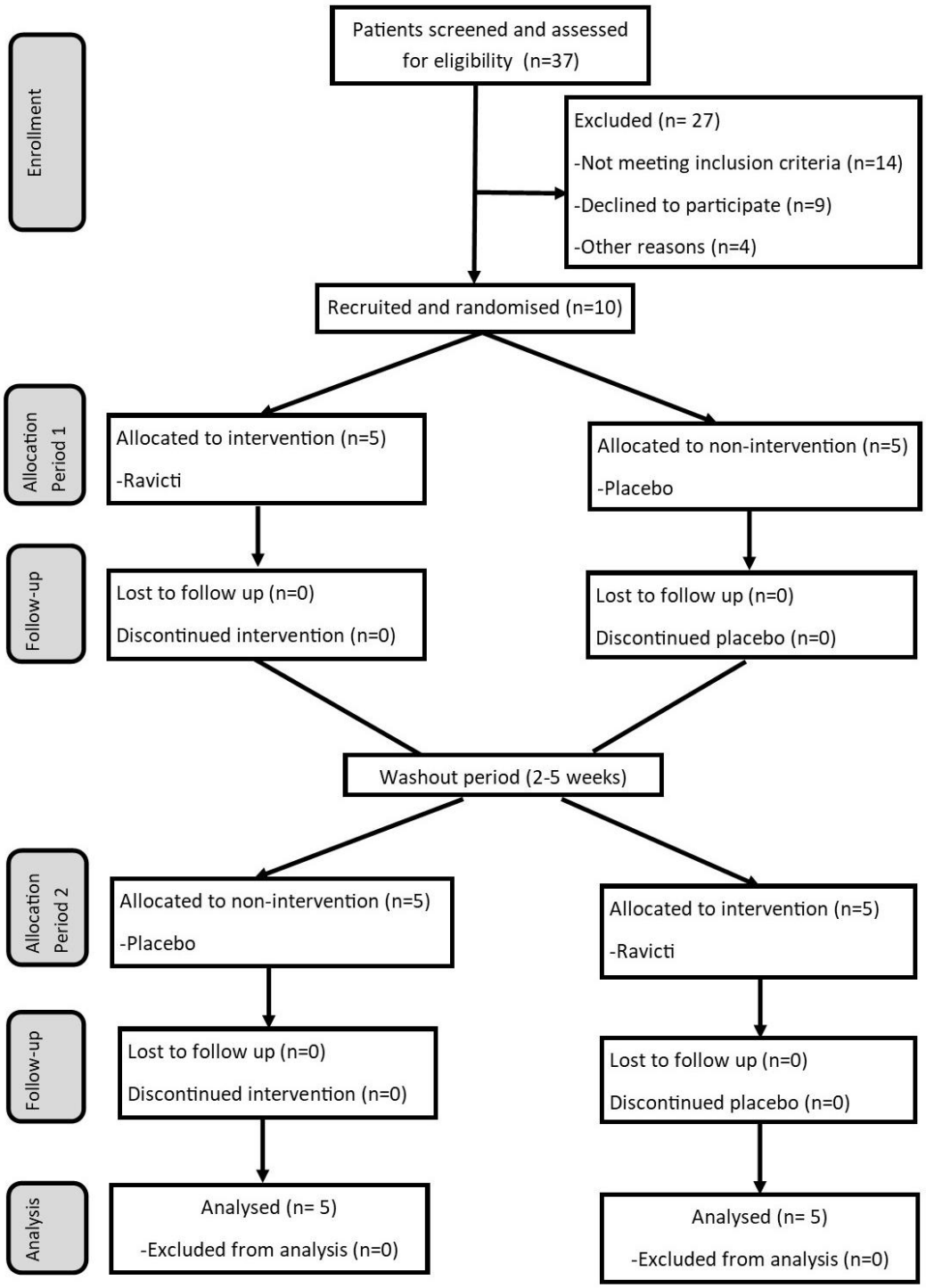


Table: Participant descriptive statistics (baseline)

Participant variable at baseline	Full cohort
Age, years; mean (SD)	45.0 (11.2)
Male gender, n (%)	7 (70.0%)
Height, cm; mean (SD)	174.1 (10.4)
Weight, kg; mean (SD)	80.8 (17.0)
Visual acuity OD, logMar; mean (SD)	0.31 (0.28)
Visual acuity OS, 1logMar; mean (SD)	0.56 (0.37)
Average eye arden index	1.43 (0.27)
Left eye central retinal thickness (µm), mean (SD)	277.4 (66.7)
Right eye central retinal thickness (µm), mean (SD)	334.4 (117.6)
Left eye total macular volume (mm^3), mean (SD)	8.5 (0.8)
Right eye total macular volume (mm^3), mean (SD)	9.1 (1.1)

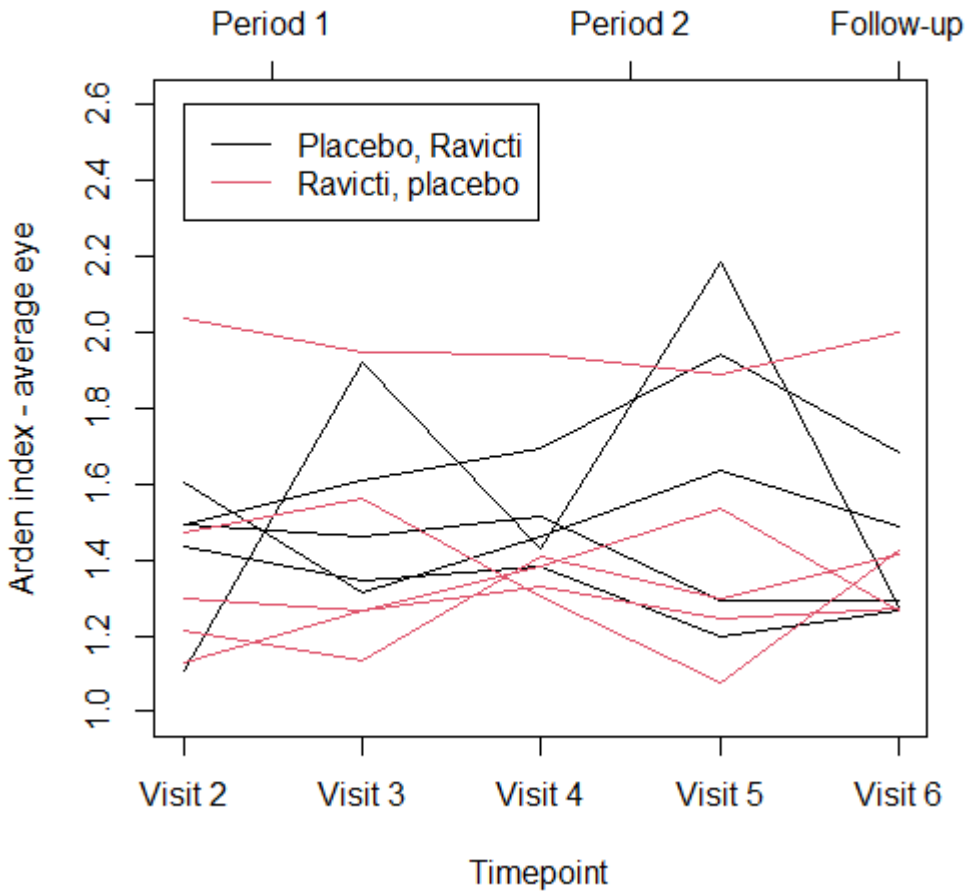
Primary Outcome – Arden index averaged across both eyes

Timepoint	Ravicti	Placebo	p-value
Outcome	1.43 (1.27 – 1.87)	1.40 (1.30 – 1.59)	0.9219

p-value from Wilcoxon signed rank test

Secondary outcome measures: average Arden index by timepoint

Period	**Timepoint**	**Placebo / Ravicti** mean (SD)	**Ravicti / Placebo** mean (SD)
1	Visit 2 – baseline	1.43 (0.19)	1.43 (0.36)
	Visit 3 – outcome	1.51 (0.17)	1.45 (0.38)
2	Visit 4 – baseline	1.53 (0.25)	1.44 (0.33)
	Visit 5 – outcome	1.43 (0.15)	1.49 (0.31)
Follow-up	Visit 6 – follow-up	1.50 (0.12)	1.48 (0.26)


Period 1
Period 2
Follow-up
Placebo, Ravicti
Ravicti, placebo
Arden index - average eye
1.0
1.2
1.4
1.6
1.8
2.0
2.2
2.4
2.6
Visit 2
Visit 3
Visit 4
Visit 5
Visit 6
Timepoint

Raw adverse events

Patid	Timepoint	Start date	Drug	Details	Severity
1	Period 1, prior to visit 4	NK/09/2024	Placebo	Occasional heartburn (gastrointestinal discomfort)	mild
	Period 2, visit 4	10/10/2024	Placebo	Slightly increased ALT (elevated Alanine Transaminase result)	mild
3	Period 2, visits 4 - 5	28/10/2024	Placebo	Slight bloating	mild
4	Period 2, visits 4 - 5	22/11/2024	Ravicti	Light headache	mild
5	Period 1, visits 2-3,	01/02/2025	Placebo	Mild headache	mild
	Period 2, visit 5	27/02/2025	Ravicti	Mild headache	mild
6	Period 1, visits 2-3	03/02/2025	Ravicti	Flatulence	mild
7	Period 1, visits 3-4	15/06/2025	Placebo	diarrhoea	mild
		19/06/2025		raised ALT	mild
8	Period 1, visit 1	19/05/2025	Baseline	Rhinorrhoea	mild
	Period 1, visit 2	20/05/2025	Ravicti	Nausea (mild)	mild
	Period 1, visits 2-3	22/05/2025	Ravicti	Constipation	mild
		22/05/2025		Headache	mild
		22/05/2025		Mild rash on wrist	mild
	Period 2, visits 5-6	01/07/2025	Placebo	Mild abdominal pain	mild
9	Period 1, visits 2-3,	26/05/2025	Placebo	Nauseous	mild
		26/05/2025		Vomiting (peak, worst day)	mild
	Period 2 visit 4	20/06/2025	Placebo	Vomiting	mild

	Period 1, visits 2-3	27/05/2025	Ravicti	Heartburn	mild
10	Period 1, visit 2	17/06/2025	Placebo	Mild headache	mild
	Period 1, visits 2-3,	22/06/2025	Placebo	Slight vertigo	mild
	Period 2, visits 4-5	13/07/2025	Ravicti	Ache in eye	mild
	Period 1, visits 2-3	20/06/2025	Placebo	Mild headache	mild

Drug	*Details*	*Total participants*	*Total count (n=23)*
Placebo	*Gastrointestinal*	*5 (50.0%)*	*7 (30.4%)*
Ravicti	*Gastrointestinal*	*3 (30.0%)*	*4 (26.1%)*
Placebo	*Elevated ALT*	*2 (20.0%)*	*2 (8.7%)*
Ravicti	*Headache*	*2 (20.0%)*	*2 (8.7%)*
Placebo	*Headache*	*3 (30.0%)*	*4 (26.1%)*
Baseline	*Rhinorrhoea*	*1 (10.0%)*	*1 (4.3%)*
Ravicit	*Rash*	*1 (10.0%)*	*1 (4.3%)*
Placebo	*Vertigo*	*1 (10.0%)*	*1 (4.3%)*
Ravicti	*Eye pain*	*1 (10.0%)*	*1 (4.3%)*