

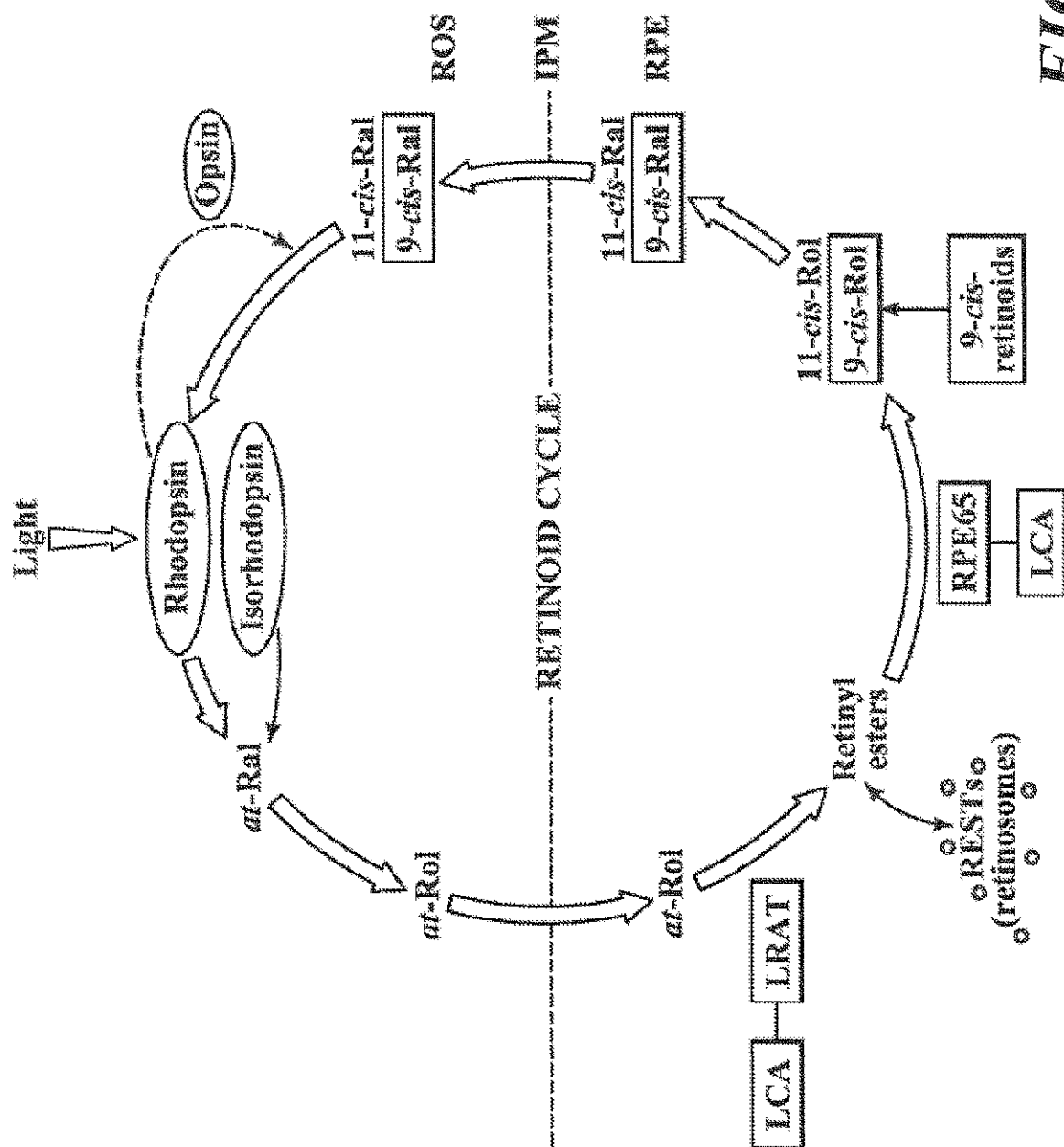


FIG. 1

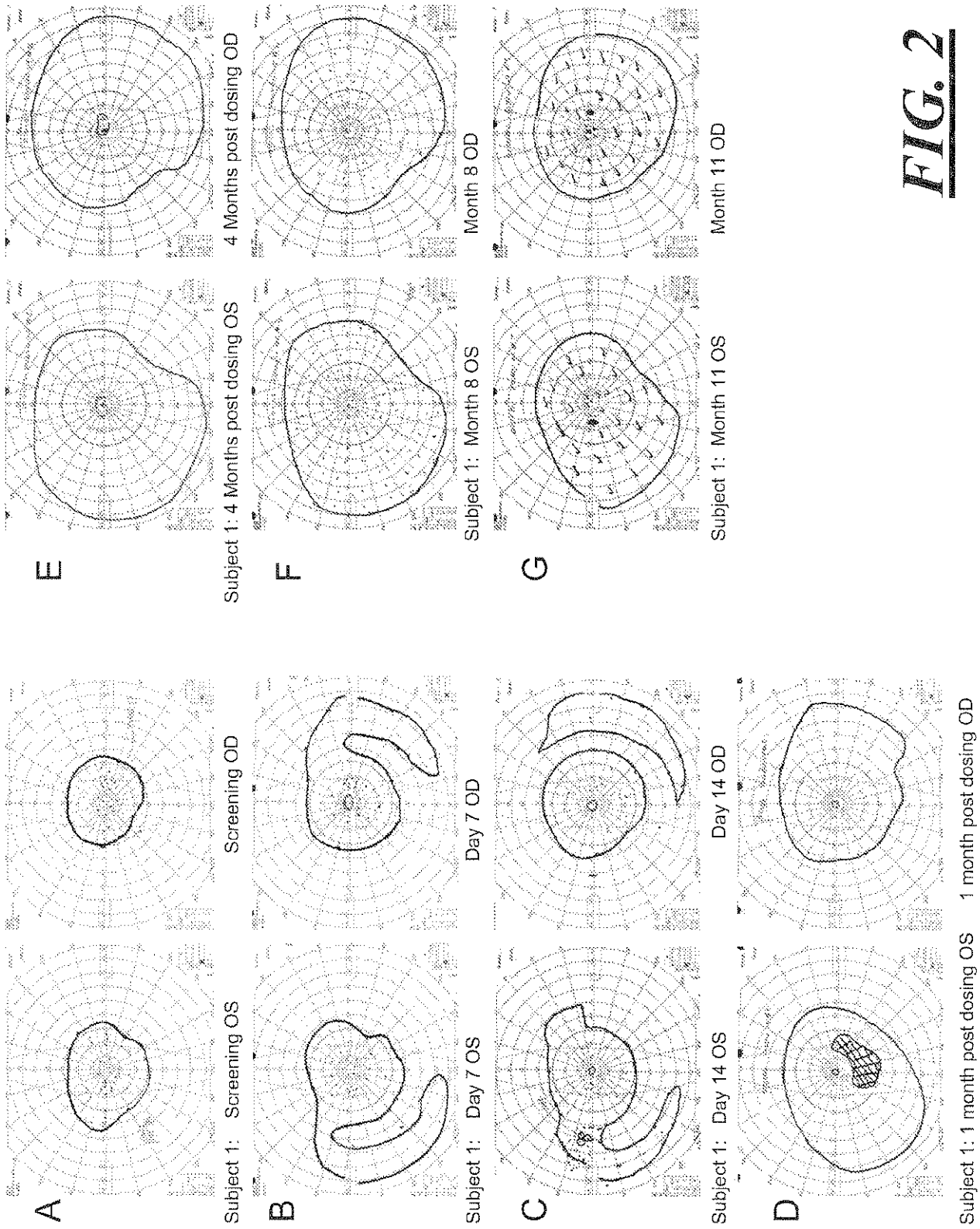


FIG. 2

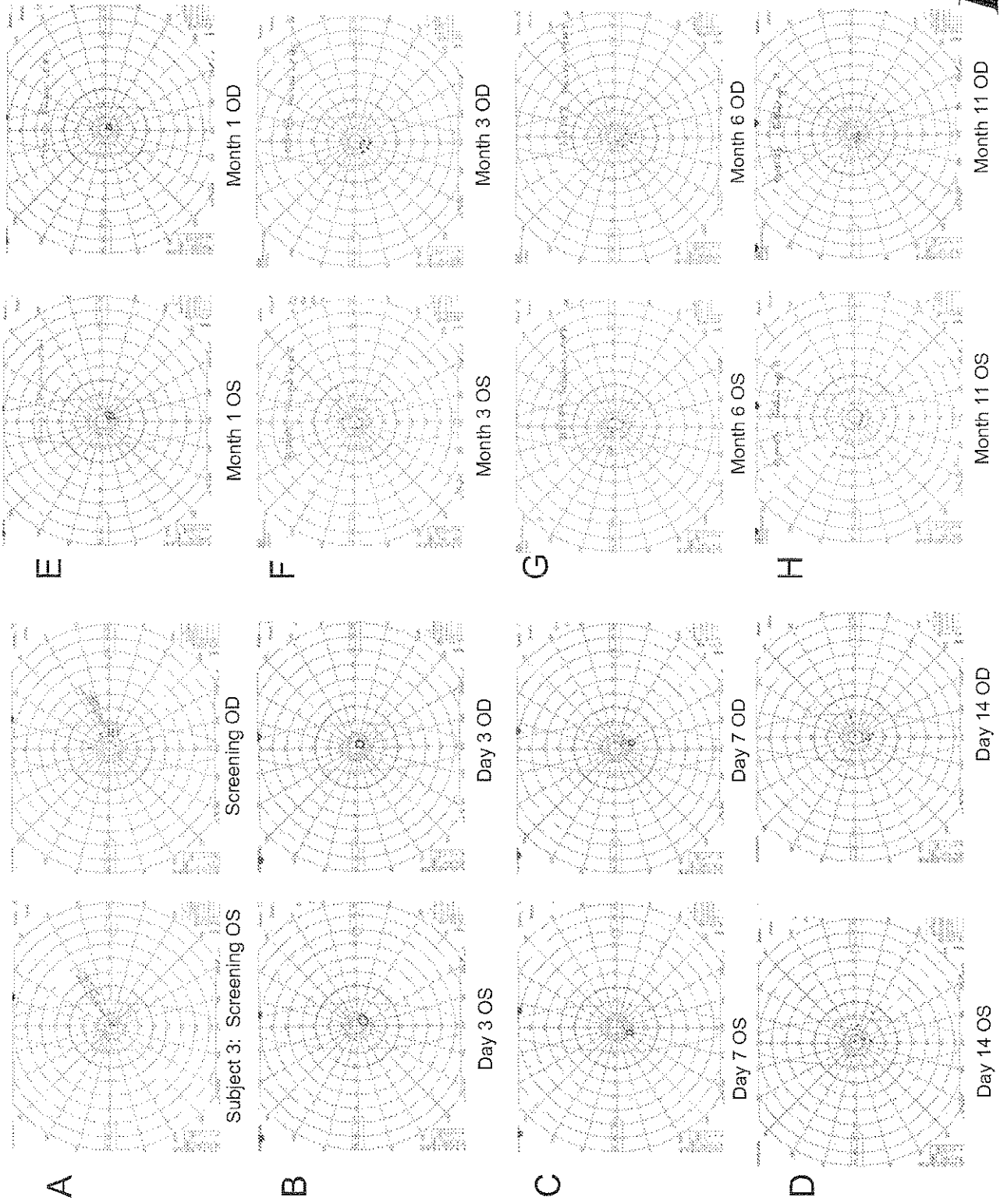


FIG. 3

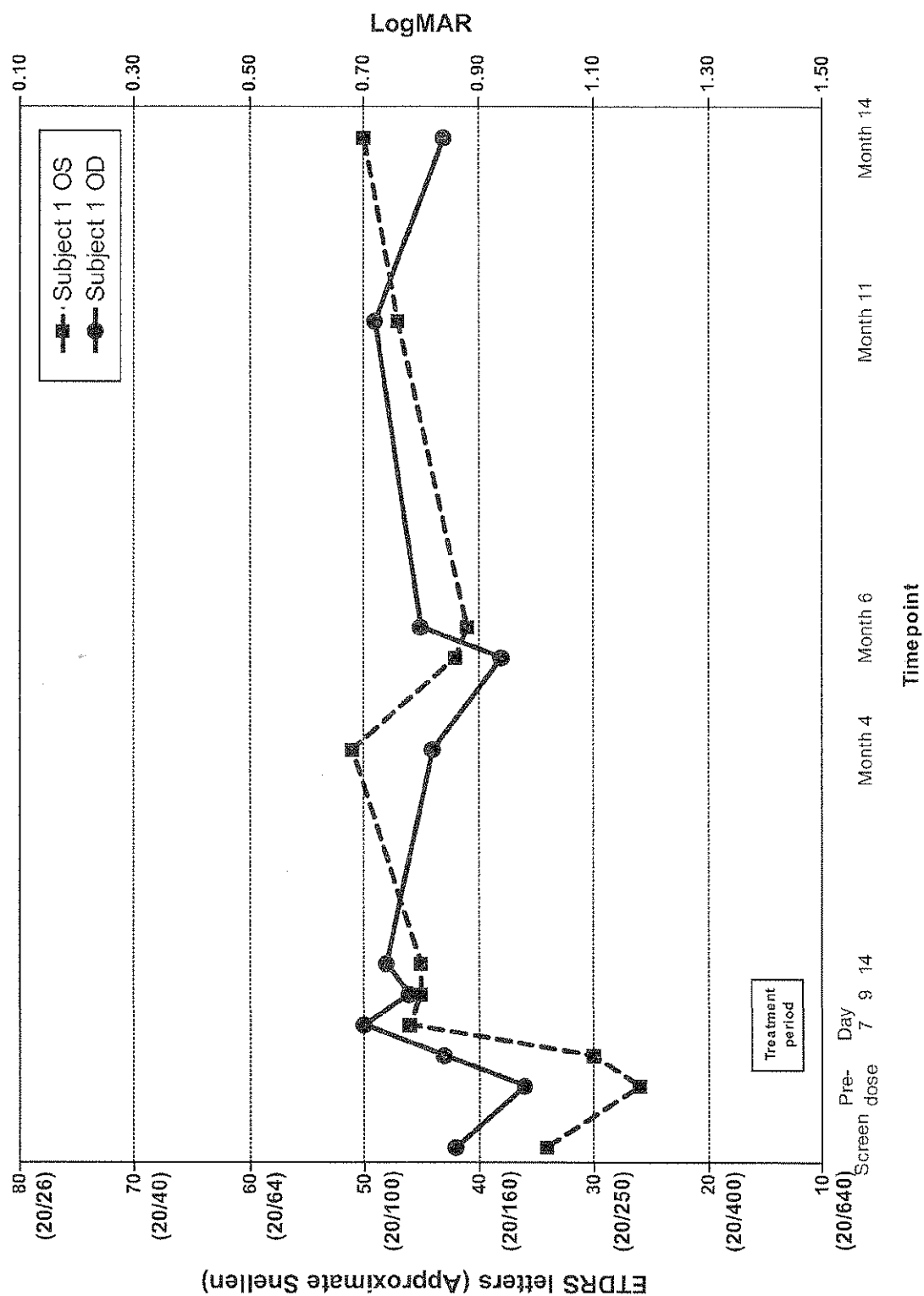


FIG. 4A

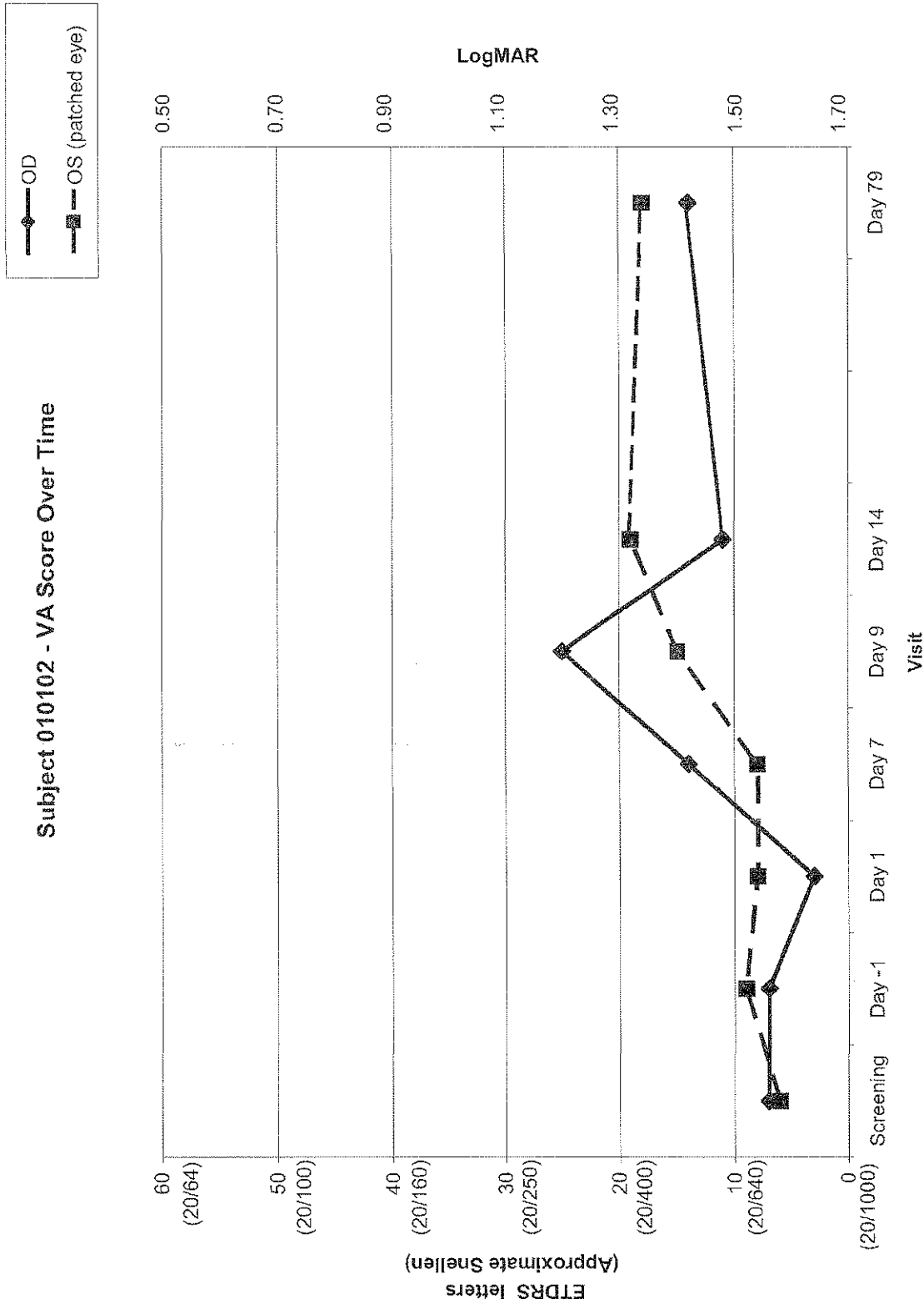


FIG. 4B

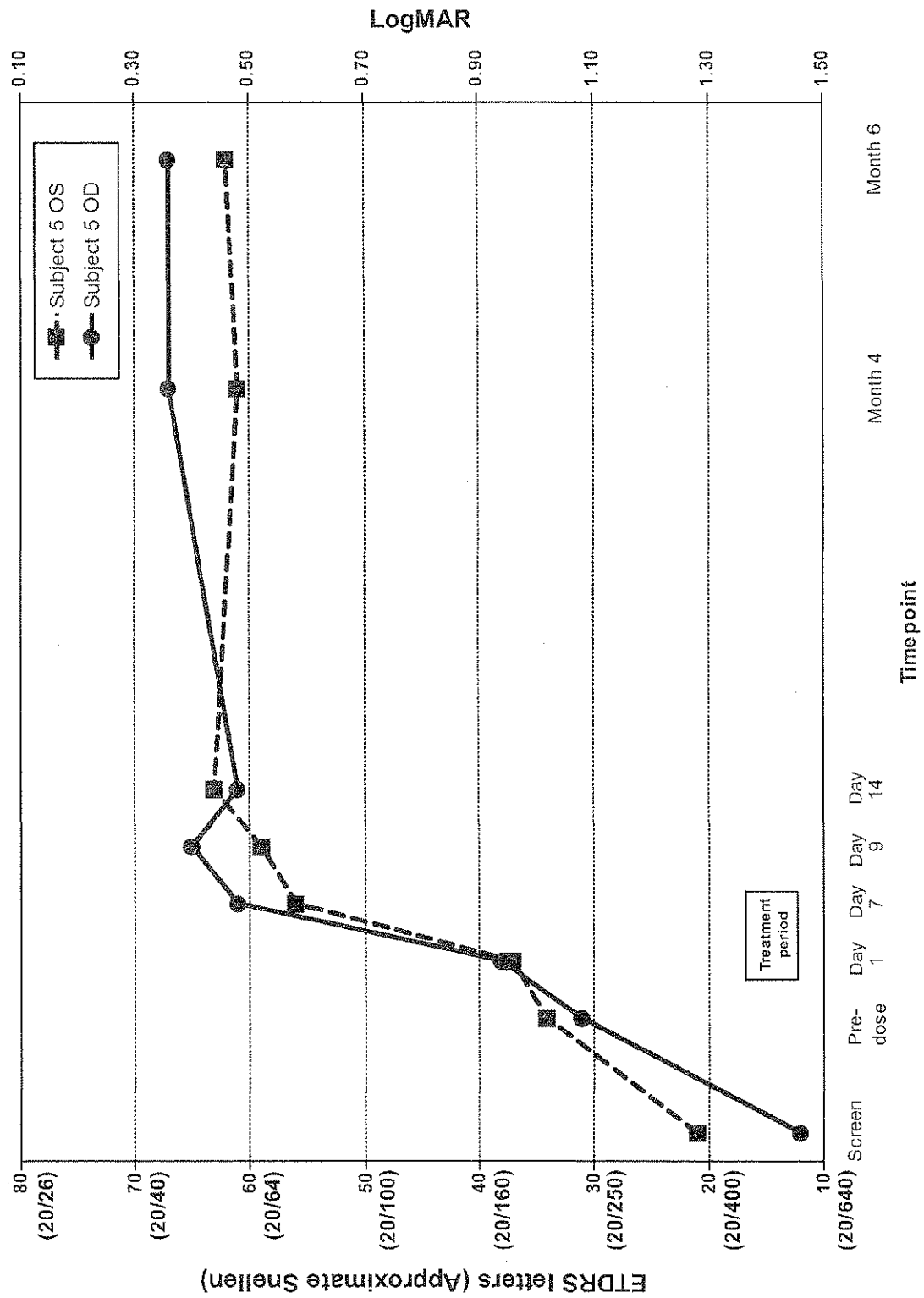
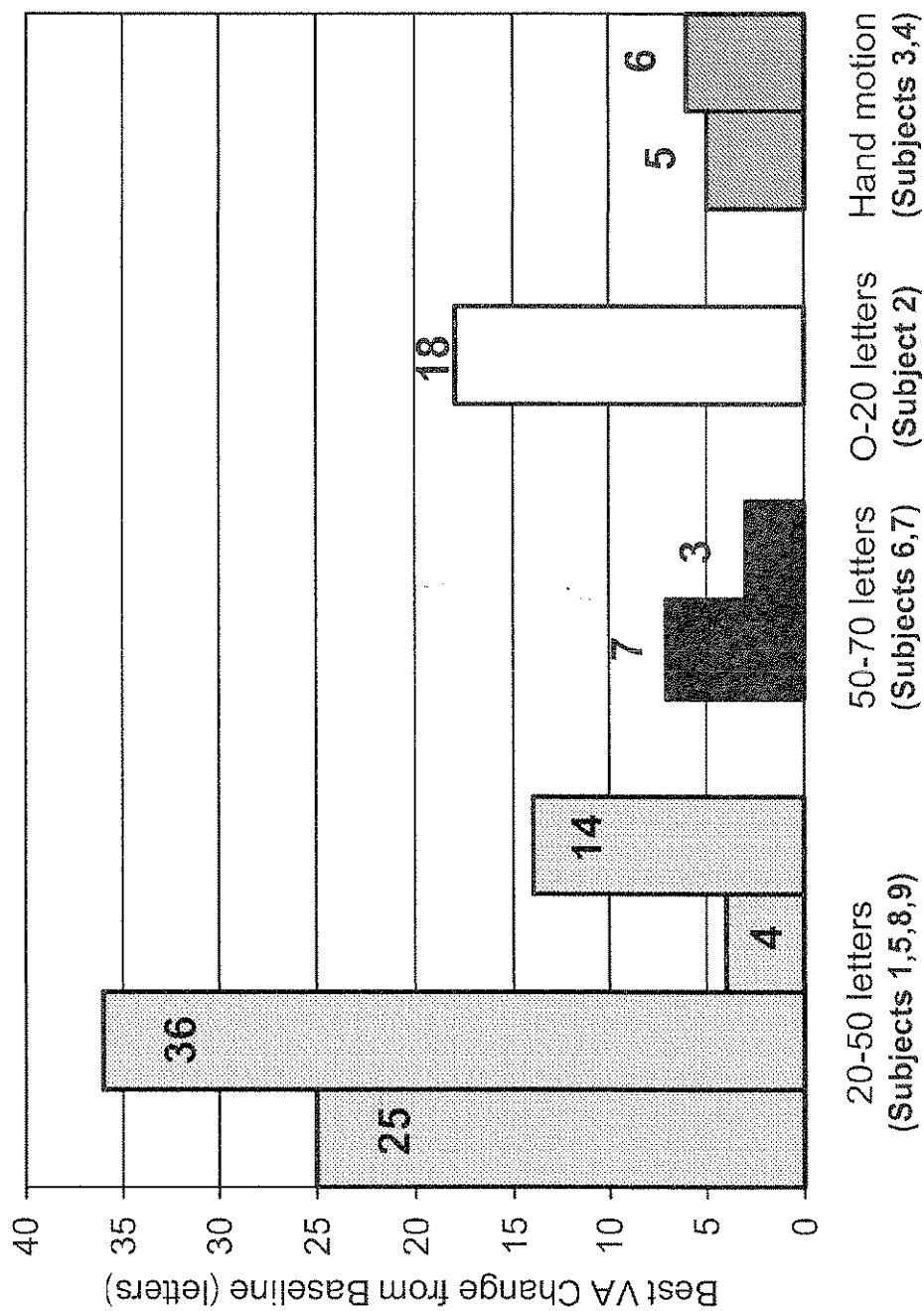


FIG. 5

Subject	Type	Age	Sex	Race	Dose (mg/m ²)	Baseline VA (letters)	Best Change from Baseline	Visit
1 LCA	LRAT	10	Female	White	40	OD 36	OD 51 (+15)	Day 8
	HOMOZYGOUS C.217_218DELAT, P.MET73ASPF SX47					OS 26	OS 51 (+25)	Month 4
2 LCA	LRAT	12	Male	White	40	OD 9	OD 18 (+9)	Month 6.5
	HOMOZYGOUS C.217_218DELAT, P.MET73ASPF SX47					OS 7	OS 25 (+18)	Day 9
3 LCA	LRAT	38	Female	White	40	OD 0	OD 5 (+5)	Month 6
	HOMOZYGOUS C.217_218DELAT, P.MET73ASPF SX47					OS 0	OD 3 (+3)	Month 2.5
4 LCA	RPE65	31	Male	Indian	40	OD 0	OD 1 (+1)	Month 2.5
	p.W331X (TGG>TAG) c.992G>A					OS 15	OS 21 (+6)	Day 14
5 LCA	RPE65	13	Female	Asian	40	OD 31	OD 67 (+36)	Month 4
	Leu67Arg CTG>CGG heterozygous - EPP=3					OS 34	OS 63 (+29)	Day 14
6 LCA	RPE65	6	Female	Asian	40	OD 64	OD 70 (+6)	Day 14
	Leu67Arg CTG>CGG heterozygous - EPP=3					OS 61	OS 68 (+7)	
7 LCA	RPE65	21	Female	Syrian	40	OD 60	OS 60 (0)	Day 14
	-/- V19DEL2BP, NT 57+58					OS 52	OS 55 (+3)	Day 9
8 LCA	RPE65	15	Female	Brazilian	10	OD 28	OD 32 (+4)	Day 9
	EXON 4 272>A R91Q GA/CT EXON 10 1022T>C L341S TC/AG					OS 25	OS 27 (+2)	
9 LCA	RPE65	14	Female	Hispanic	10	OD 37	OD 51 (+14)	Day 14
	EXON 4 272G, EXON 10 1022T					OS 47	OD 46 (-1)	
10 RP	LRAT	28	Male	Indian	40	OD 71	OD 82 (+11.5)	Day 9
	HOMOZYGOUS EXON 2 C.525T>A; P.SER175ARG					OS 60	OS 74 (+14.5)	Month 1.5
11 RP	LRAT	41	Male	White	40	OD 0	No change	Day 14
	HOMOZYGOUS EXON 2 C.181T>A3 P.TYR61ASP					OS 1.5	OD 26 (+24.5)	

FIG. 6



Baseline VA Category

FIG. 7

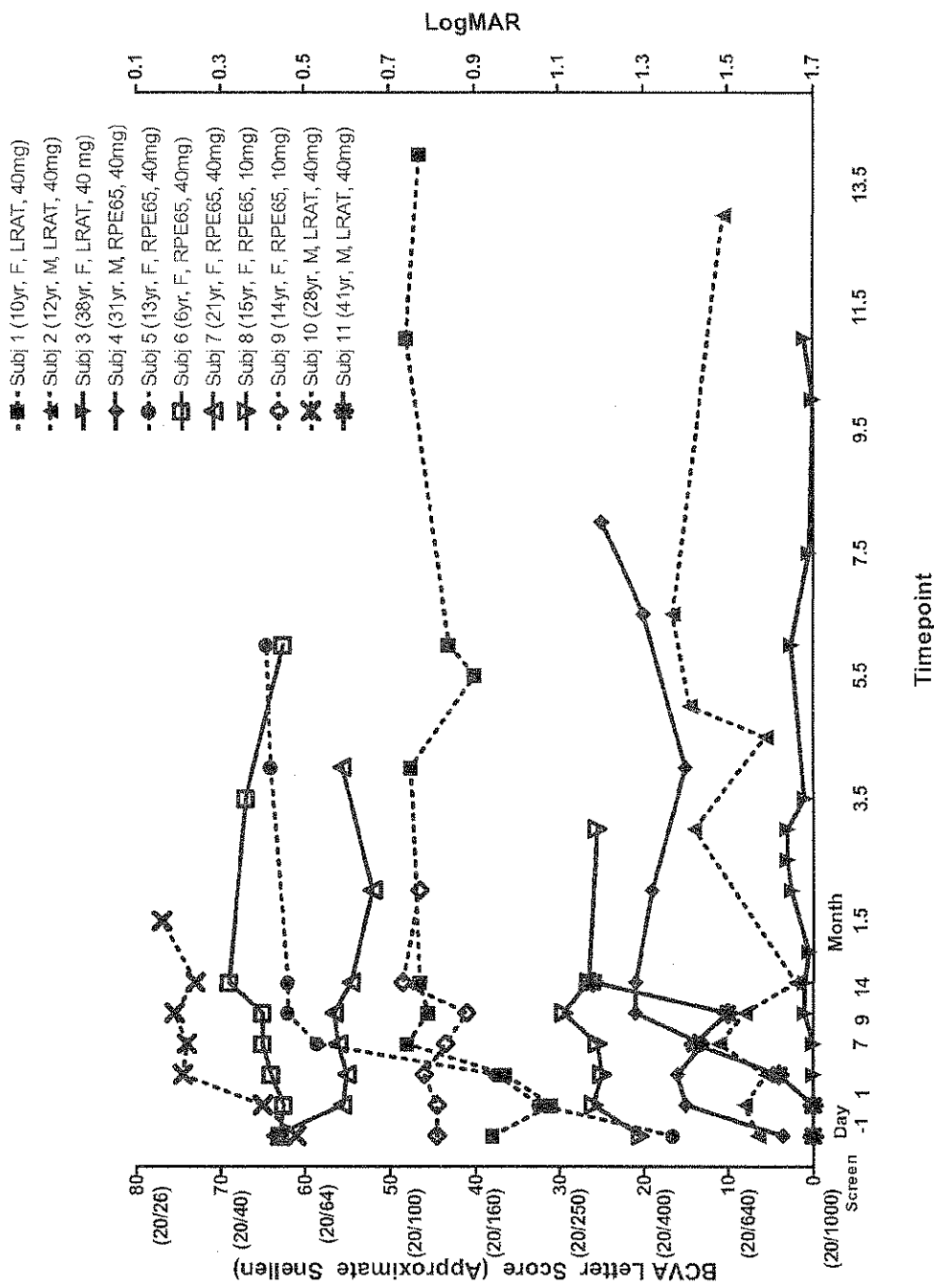


FIG. 8

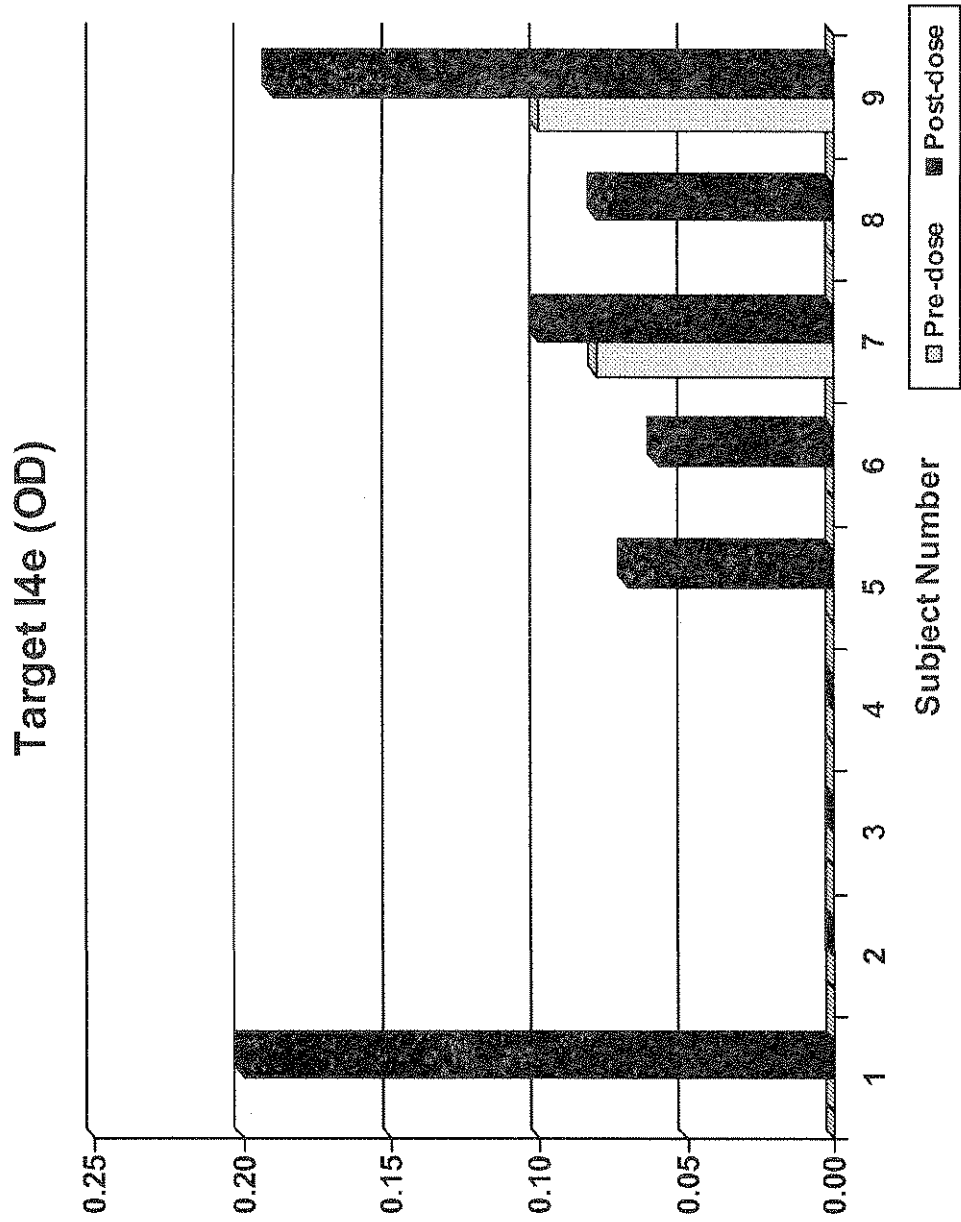
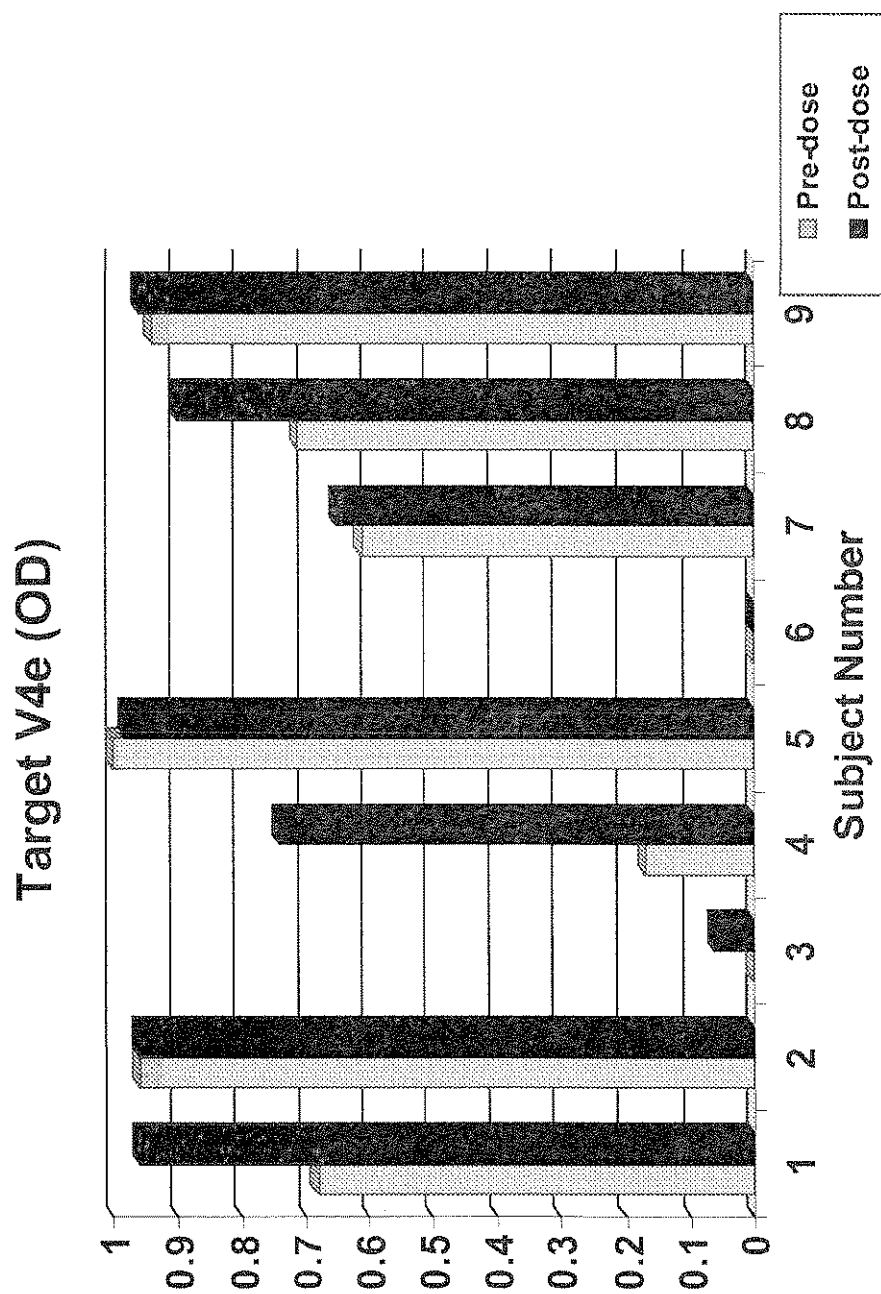


FIG. 9A

***FIG. 9B***

Triglycerides	Baseline	Day 3	%chg	Day 7	%chg	Day 9	%chg	Day 14	%chg
10 mg/m2 (n=2)	0.3 ± 0.0	0.5 ± 0.3	57.1%	0.6 ± 0.2	66.1%	0.4	17.6%	-	-
40 mg/m2 (n=9)	1.0 ± 0.5	1.5 ± 0.7	55.8%	2.0 ± 0.8	113.4%	1.6 ± 0.6	82.2%	1.2 ± 0.5	12.0%
Total (n=11)	0.9 ± 0.5	1.3 ± 0.8	56.0%	1.7 ± 1.0	103.9%	1.5 ± 0.8	73.0%	1.2 ± 0.5	12.0%

HDL	Baseline	Day 3	%chg	Day 7	%chg	Day 9	%chg	Day 14	%chg
10 mg/m2 (n=2)	1.3 ± 0.1	1.2 ± 0.2	-6.3%	1.2 ± 0.2	-2.5%	1.3	-2.9%	-	-
40 mg/m2 (n=9)	1.1 ± 0.2	0.9 ± 0.2	-16.2%	0.8 ± 0.1	-20.1%	1.0 ± 0.1	-5.6%	1.1 ± 0.2	5.8%
Total (n=11)	1.1 ± 0.2	1.0 ± 0.2	-13.7%	0.9 ± 0.2	-16.6%	1.0 ± 0.2	-5.0%	1.1 ± 0.2	5.8%

Cholesterol	Baseline	Day 3	%chg	Day 7	%chg	Day 9	%chg	Day 14	%chg
10 mg/m2 (n=2)	4.3 ± 0.0	4.3 ± 0.2	-1.2%	4.5 ± 0.3	3.4%	4.5	2.8%	-	-
40 mg/m2 (n=9)	4.0 ± 0.7	3.8 ± 0.6	-1.3%	4.3 ± 0.6	11.6%	4.5 ± 0.8	23.2%	4.7 ± 0.6	18.1%
Total (n=11)	4.1 ± 0.7	3.9 ± 0.6	-1.3%	4.3 ± 0.6	9.9%	4.5 ± 0.7	20.3%	4.7 ± 0.6	18.1%

LDL	Baseline	Day 3	%chg	Day 7	%chg	Day 9	%chg	Day 14	%chg
10 mg/m2 (n=2)	2.9 ± 0.1	2.8 ± 0.3	-2.4%	3.0 ± 0.3	2.2%	2.9	4.6%	-	-
40 mg/m2 (n=9)	2.5 ± 0.7	2.3 ± 0.4	-0.1%	2.6 ± 0.7	10.3%	3.0 ± 0.7	31.3%	3.0 ± 0.6	26.0%
Total (n=11)	2.6 ± 0.6	2.4 ± 0.5	-0.6%	2.7 ± 0.6	8.7%	3.0 ± 0.6	26.0%	3.0 ± 0.6	26.0%

FIG. 10