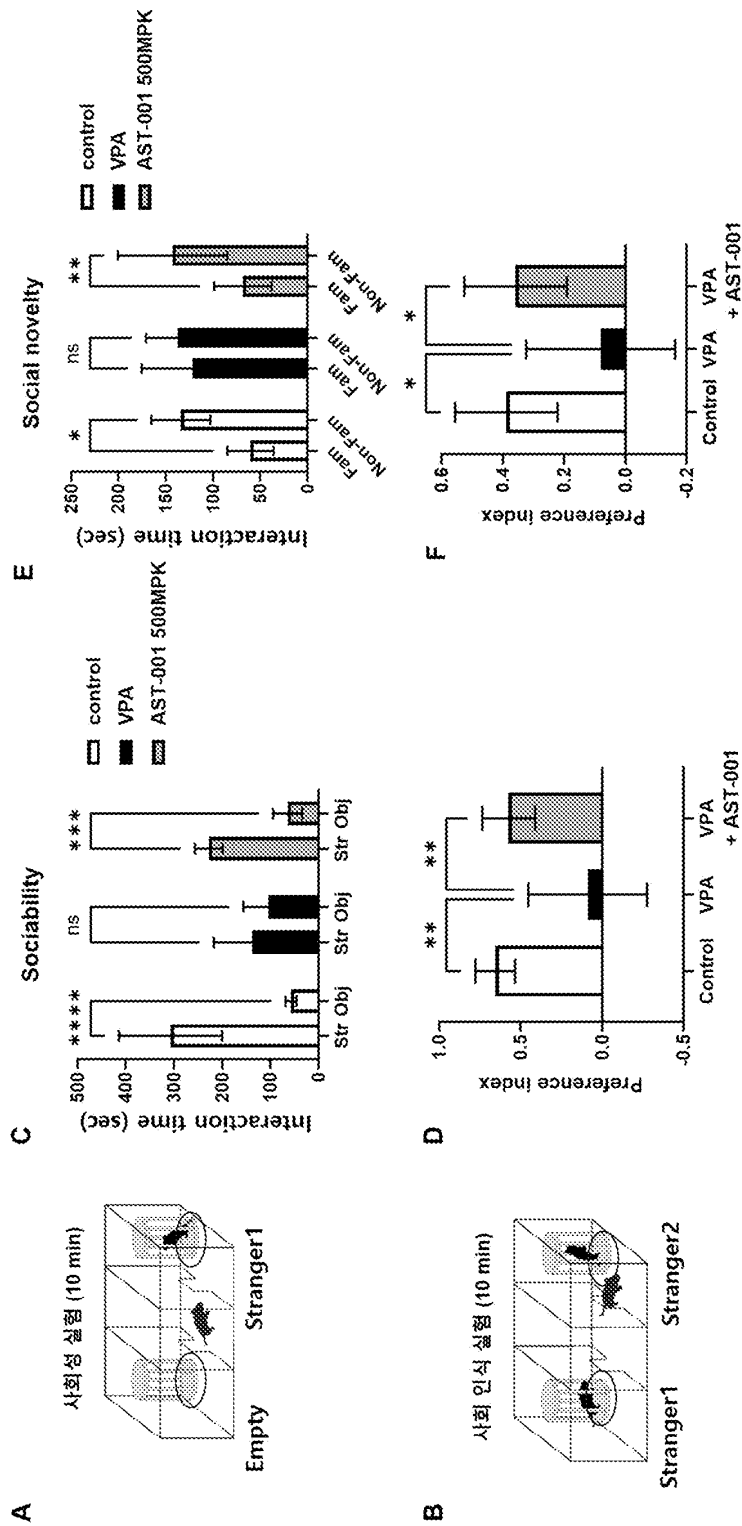
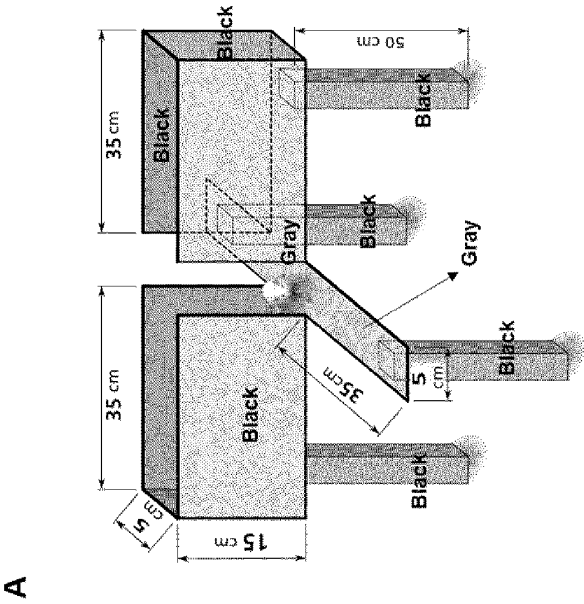
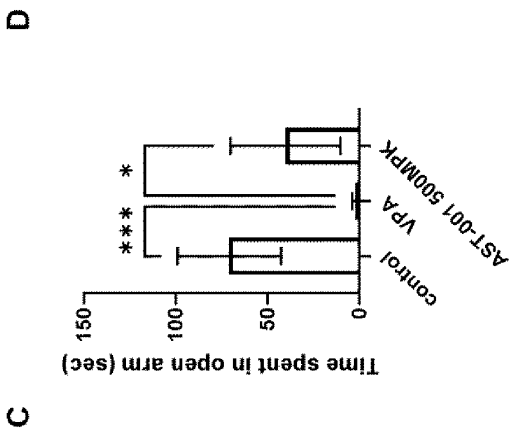
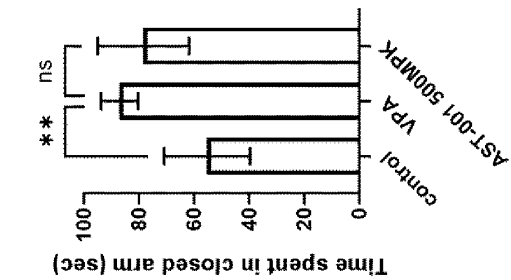
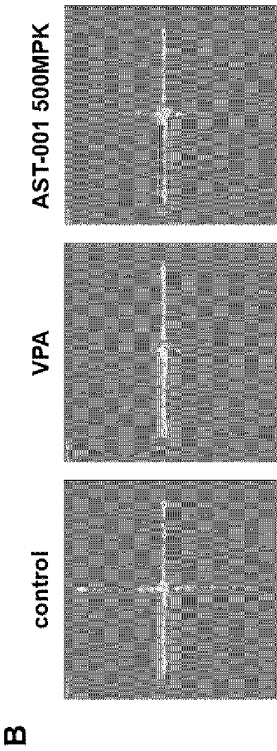


[도1]

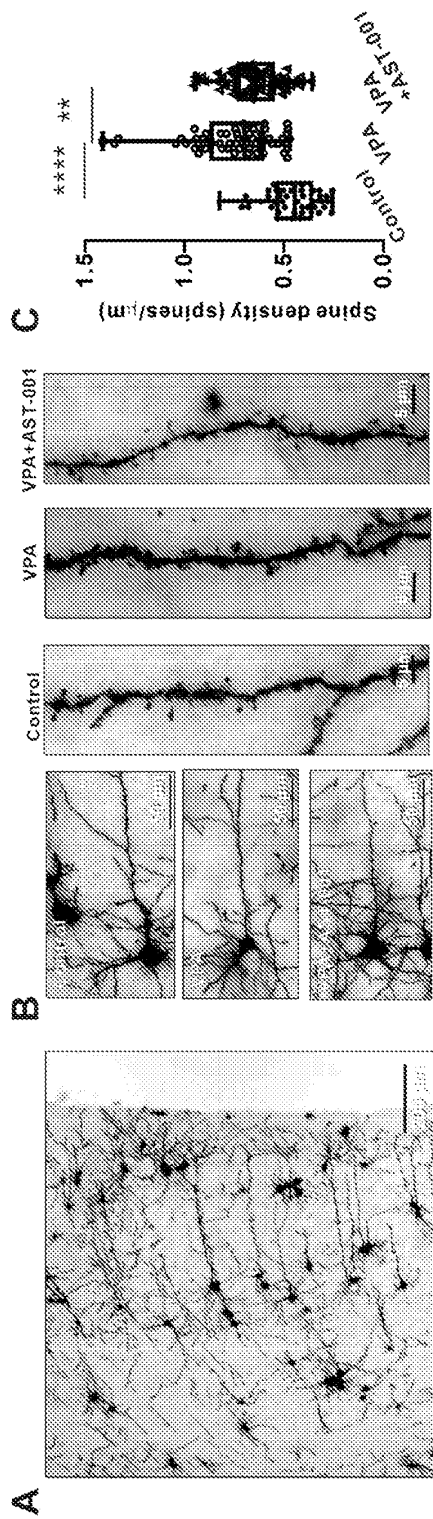


[52]

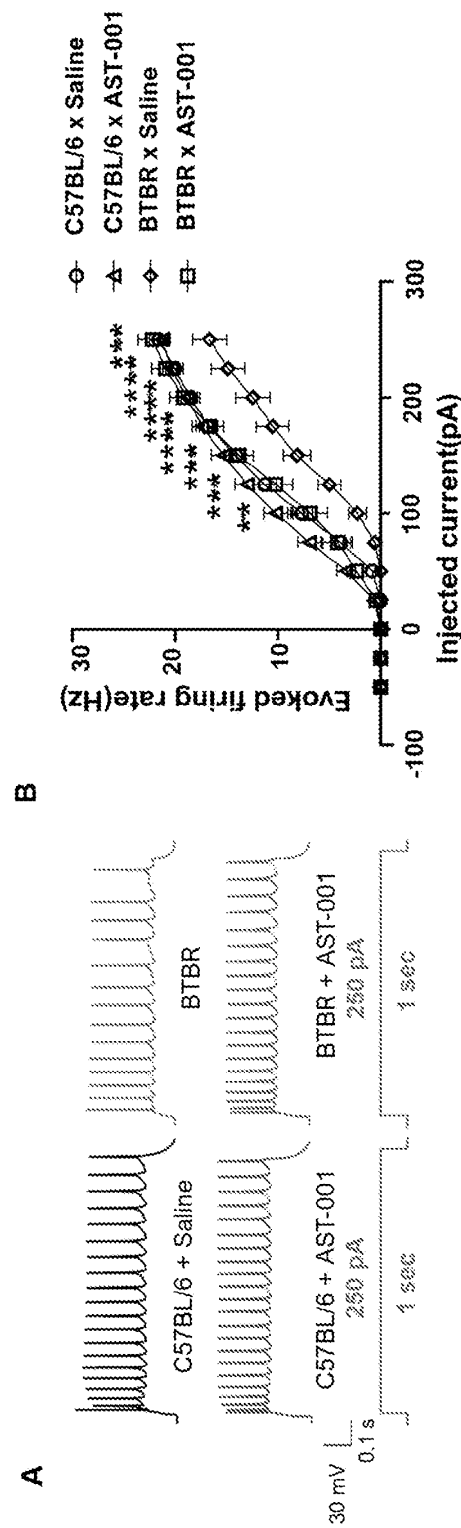


[도4]

규칙 91,
31.01.2024

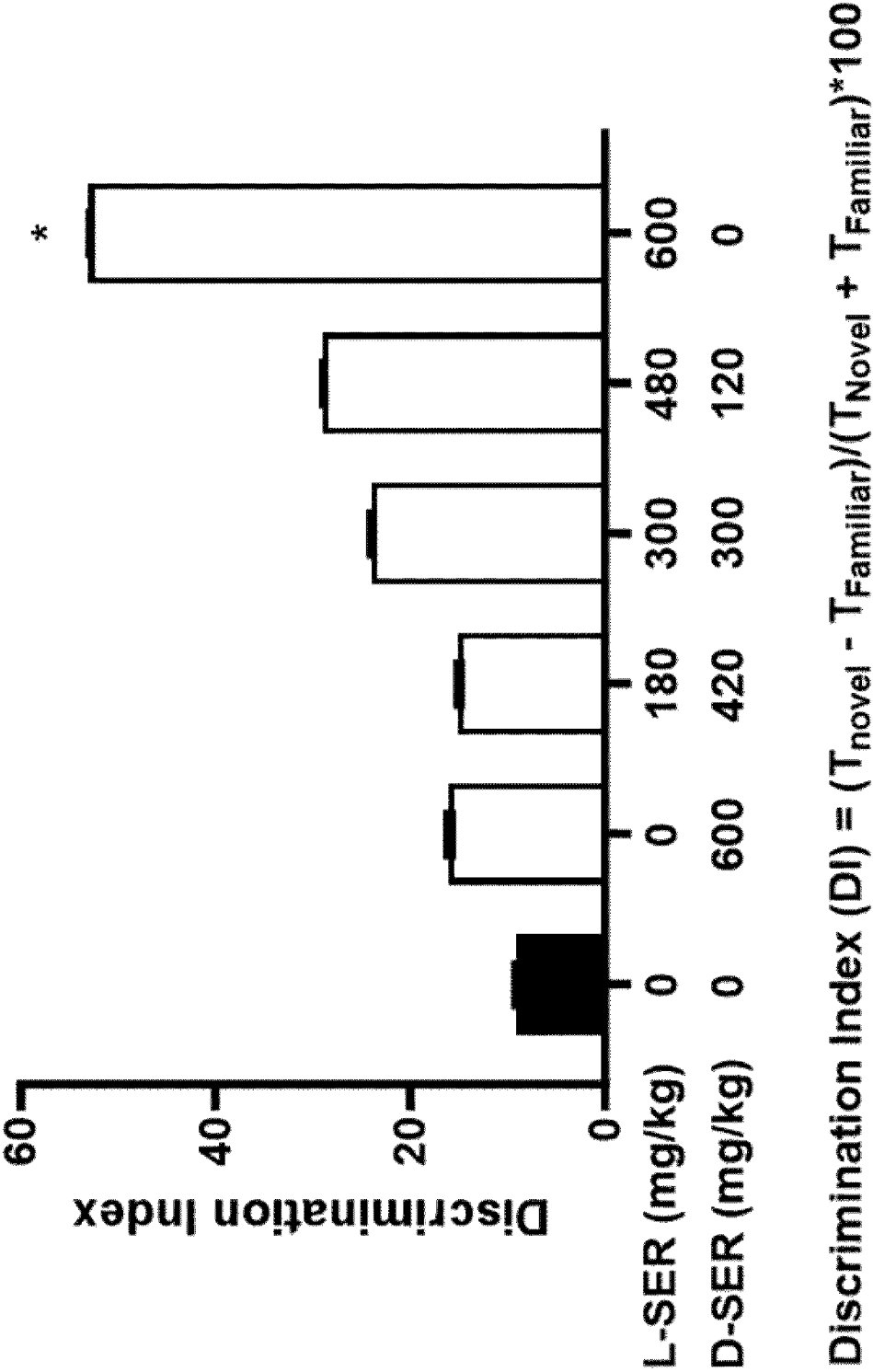


[도5]

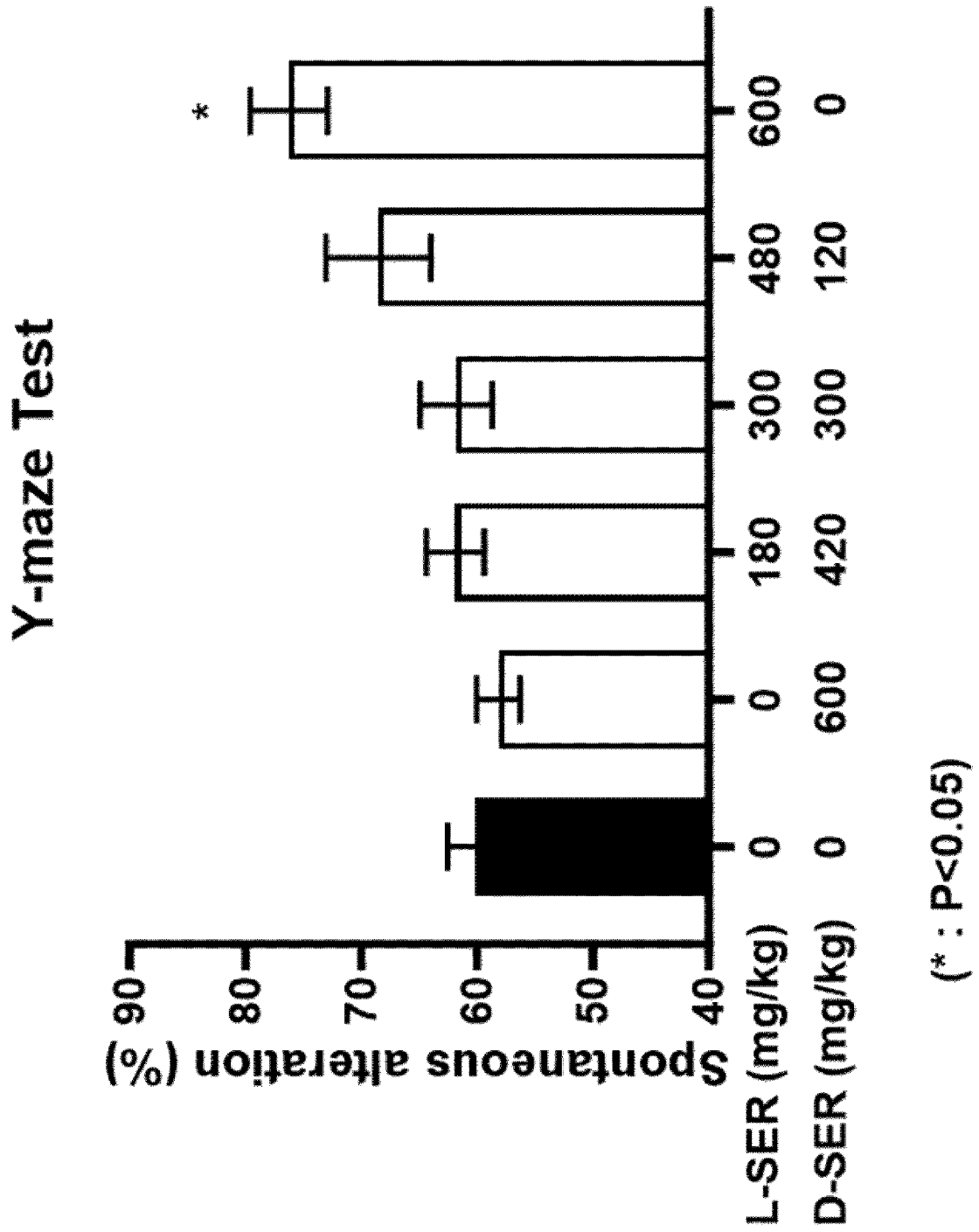


[도6]

Novel Object Recognition test



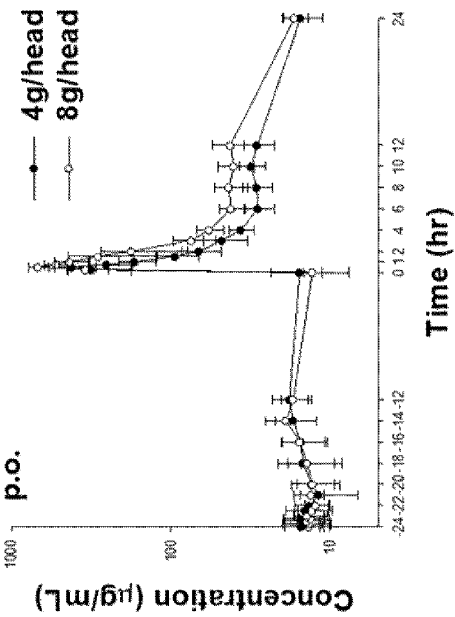
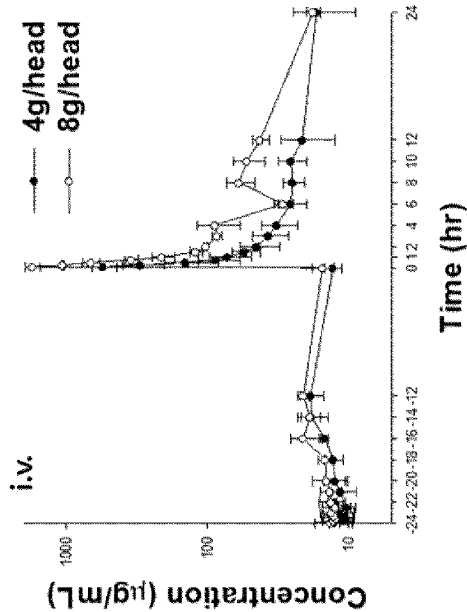
[도7]



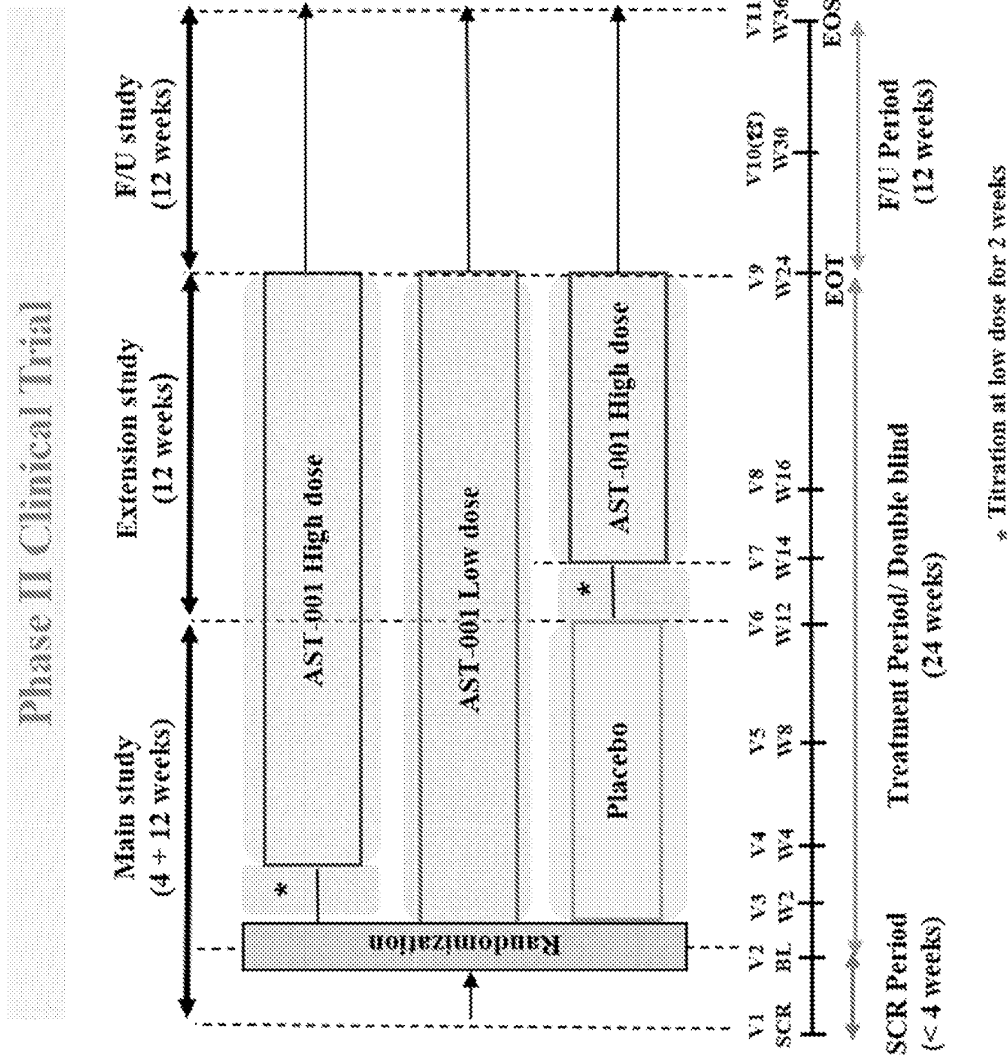
WO 2025/014008

PCT/KR2023/020475

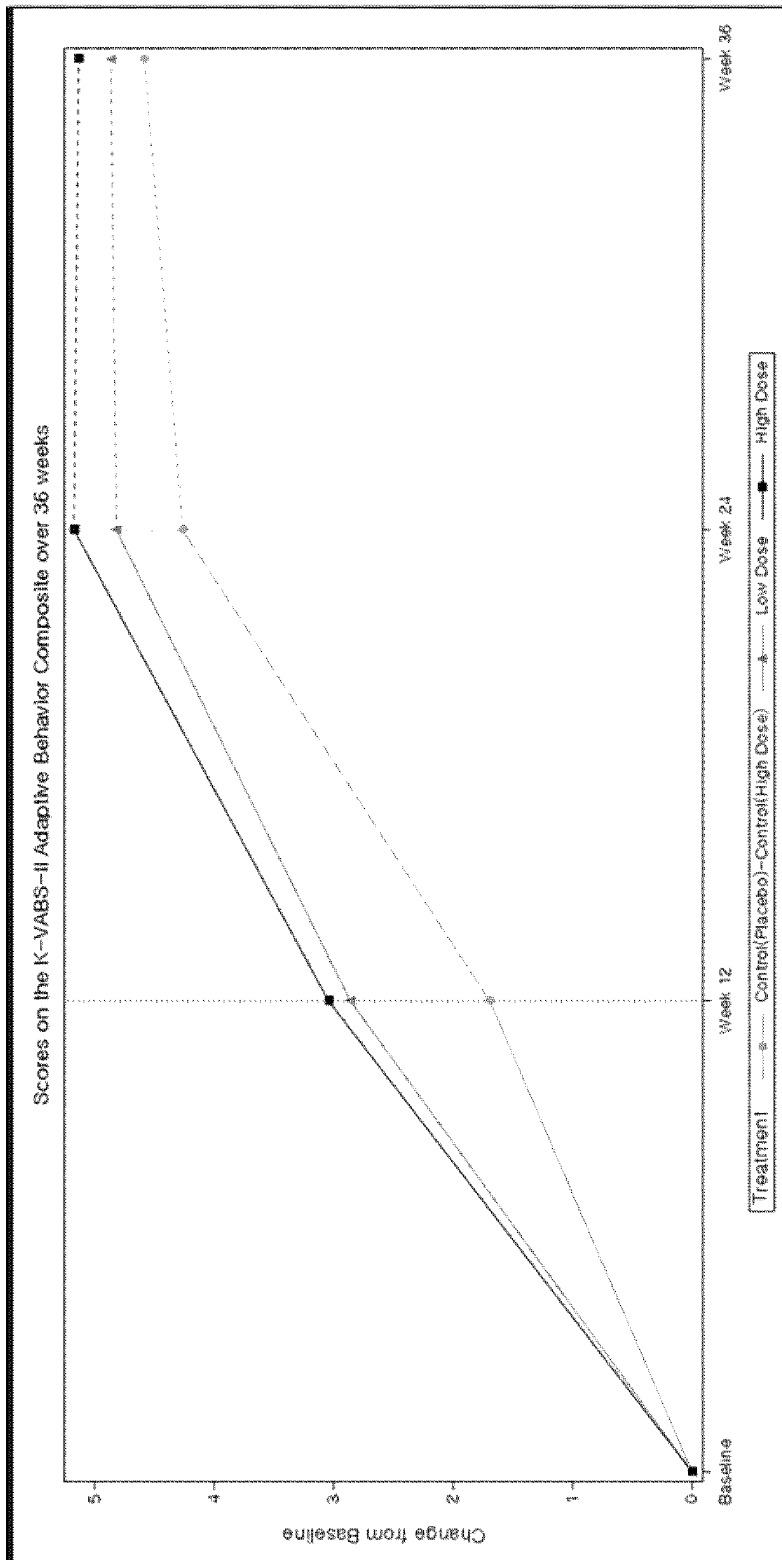
[58]



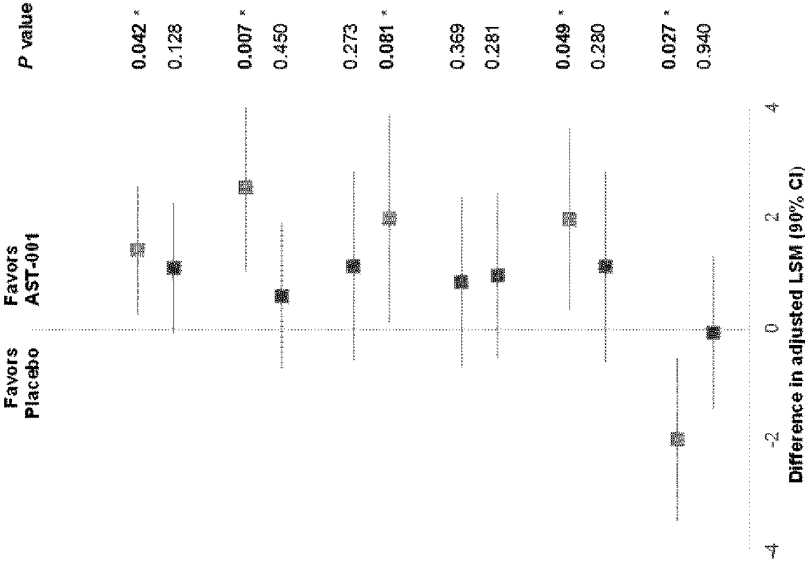
[도9]



[도10]



[511]



-4 -2 0 2 4
Difference in adjusted LSM (90% CI)

WO 2025/014008

PCT/KR2023/020475

[도12]

0-12주 (FAS: N=145)					
투여군	N	베이스라인	12주	베이스라인 대비 12주 변화량	Model- adjusted Treatment difference ¹⁾
위약	49	50.22±10.33	51.93±10.66	1.70±3.20	
저용량	50	52.28±11.56	54.65±12.14	2.37±3.79	0.71±0.71
고용량	46	51.53±9.89	54.60±11.66	3.27±3.78	1.96±0.73

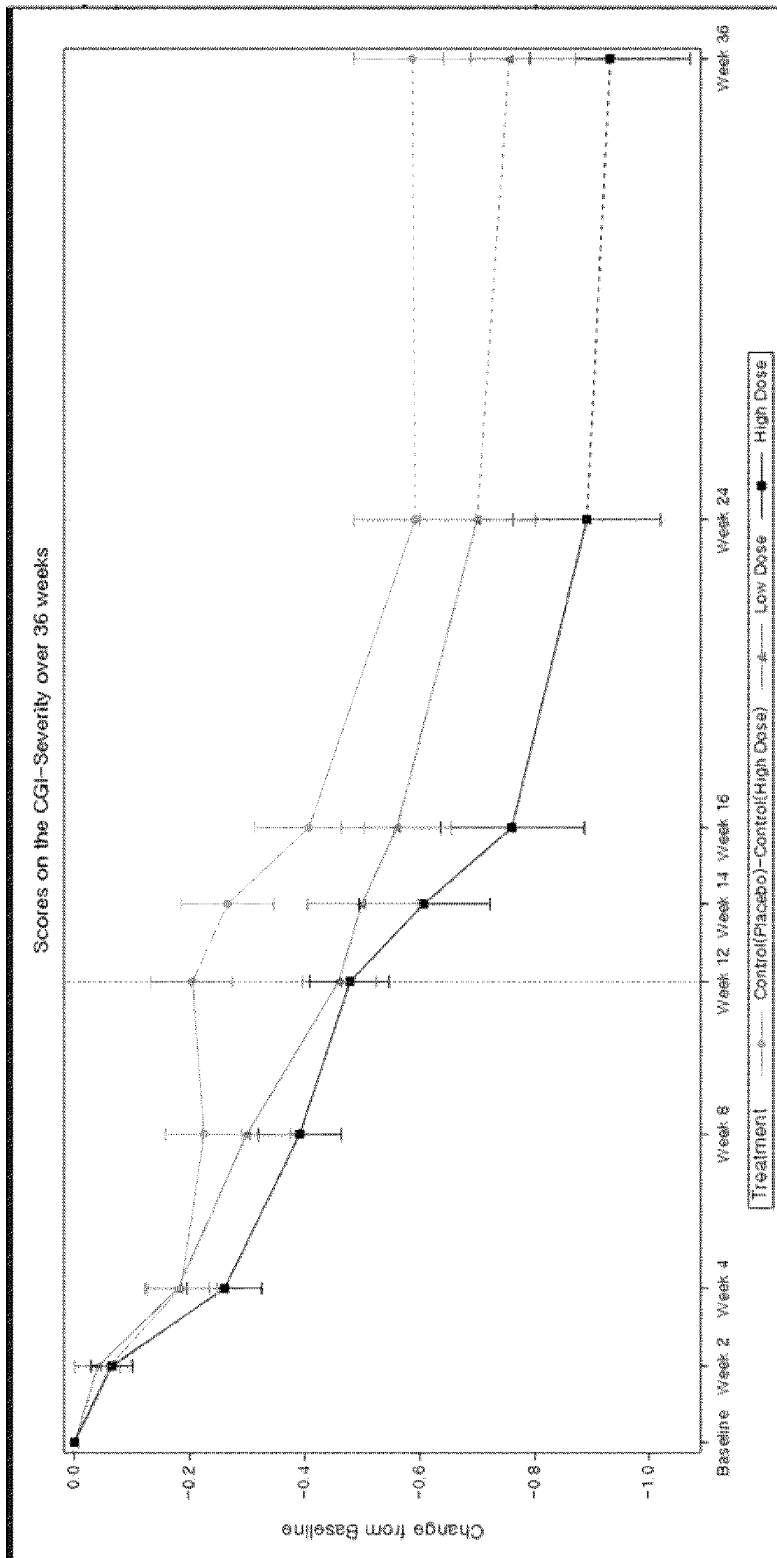
12-24주 (FAS: N=145)					
투여군	N	24주	베이스라인 대비 24주 변화량	Model- adjusted Treatment difference ²⁾	
고용량단기	49	54.12±12.04	3.90±4.94	1.41±1.05	
저용량단기	50	56.21±13.14	3.93±4.76	1.50±1.01	
고용량장기	46	56.66±12.57	5.13±5.29		

24-36주 (FAS: N=135)					
투여군	N	36주	베이스라인 대비 36주 변화량	Model- adjusted Treatment difference ²⁾	
고용량단기	46	54.76±12.43	3.91±5.66	1.24±1.18	
저용량장기	45	56.39±13.56	3.66±5.20	1.64±1.16	
고용량장기	44	56.35±13.24	4.78±6.00		

WO 2025/014008

PCT/KR2023/020475

[도 13]



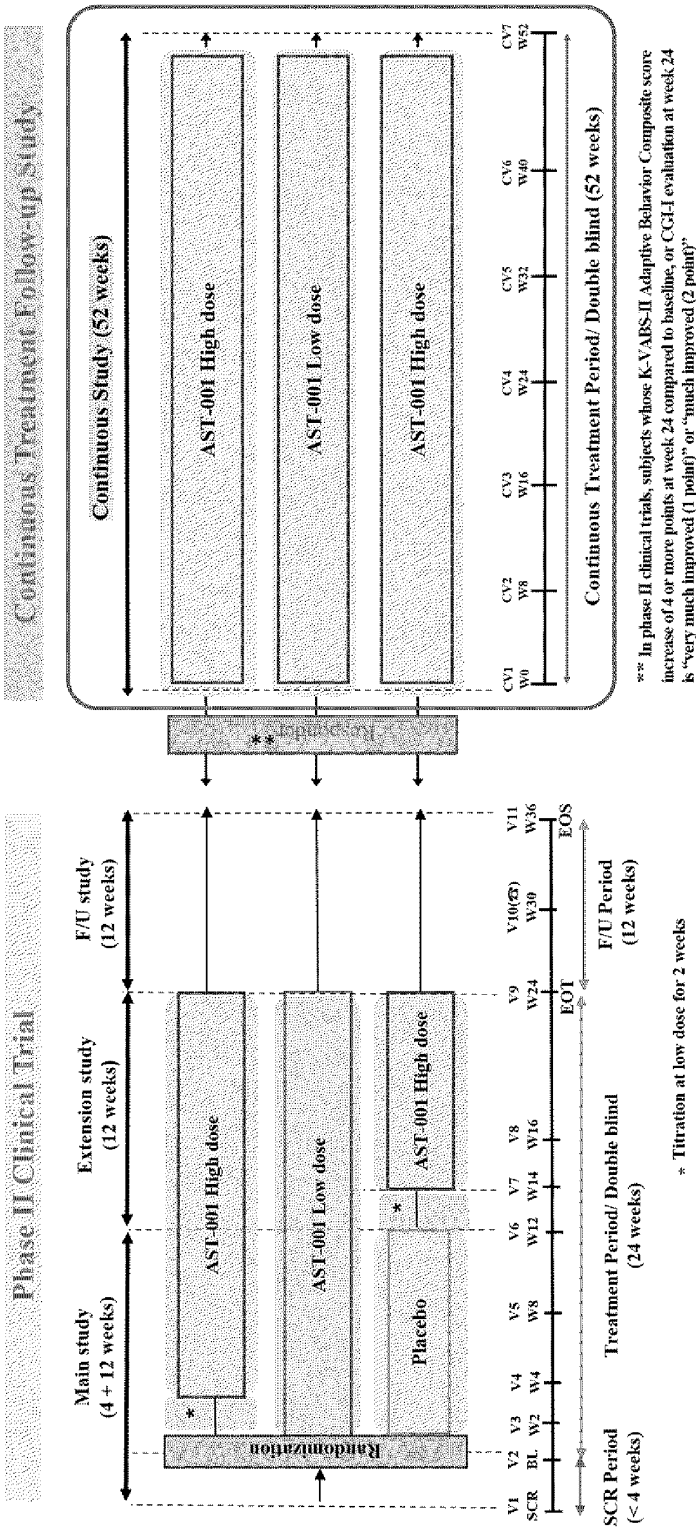
[도14]

0~12주 (PPS1, N=134)					
투여군	N	베이스라인	12주	베이스라인 대비 12주 변화량	Model- adjusted Treatment difference ⁽¹⁾
위약	43	47.91±10.70	49.30±11.19	1.40±2.95	
저용량	48	50.77±11.92	53.63±13.43	2.85±4.04	1.35±0.75
고용량	43	49.07±9.08	51.98±10.83	2.91±3.44	1.59±0.69

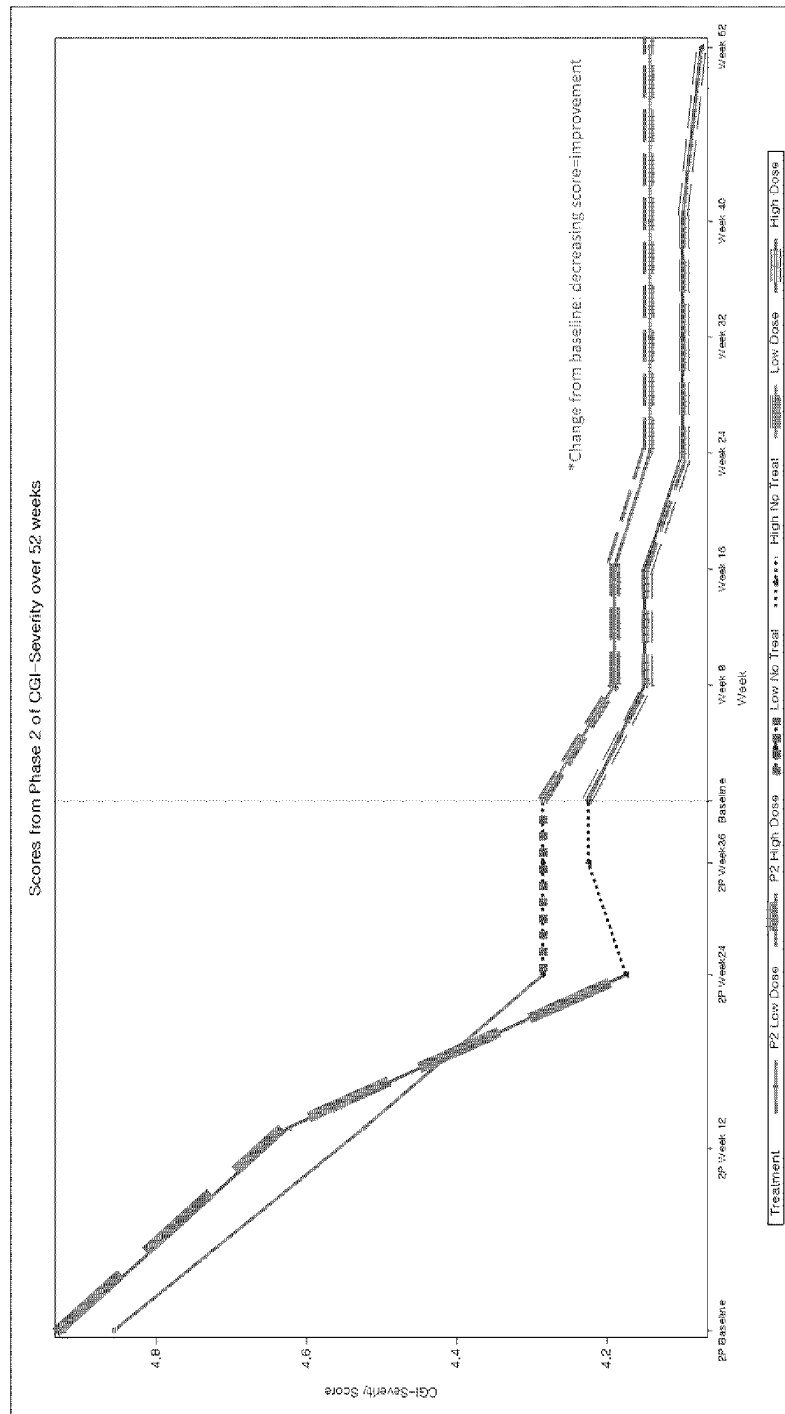
12~24주 (PPS2, N=124)				
투여군	N	베이스라인	24주	베이스라인 대비 24주 변화량
고용량단기	44	48.25±10.64	52.27±12.62	4.02±4.46
저용량장기	43	51.72±12.09	56.30±14.96	4.58±5.58
고용량장기	37	49.89±9.97	55.41±13.30	5.51±5.64

24~36주 (PPS3, N=114)				
투여군	N	베이스라인	36주	베이스라인 대비 36주 변화량
고용량단기	36	49.31±11.39	53.81±14.60	4.50±5.61
저용량장기	40	51.50±11.21	56.43±14.33	4.93±5.54
고용량장기	38	50.13±9.63	55.66±13.98	5.53±6.79

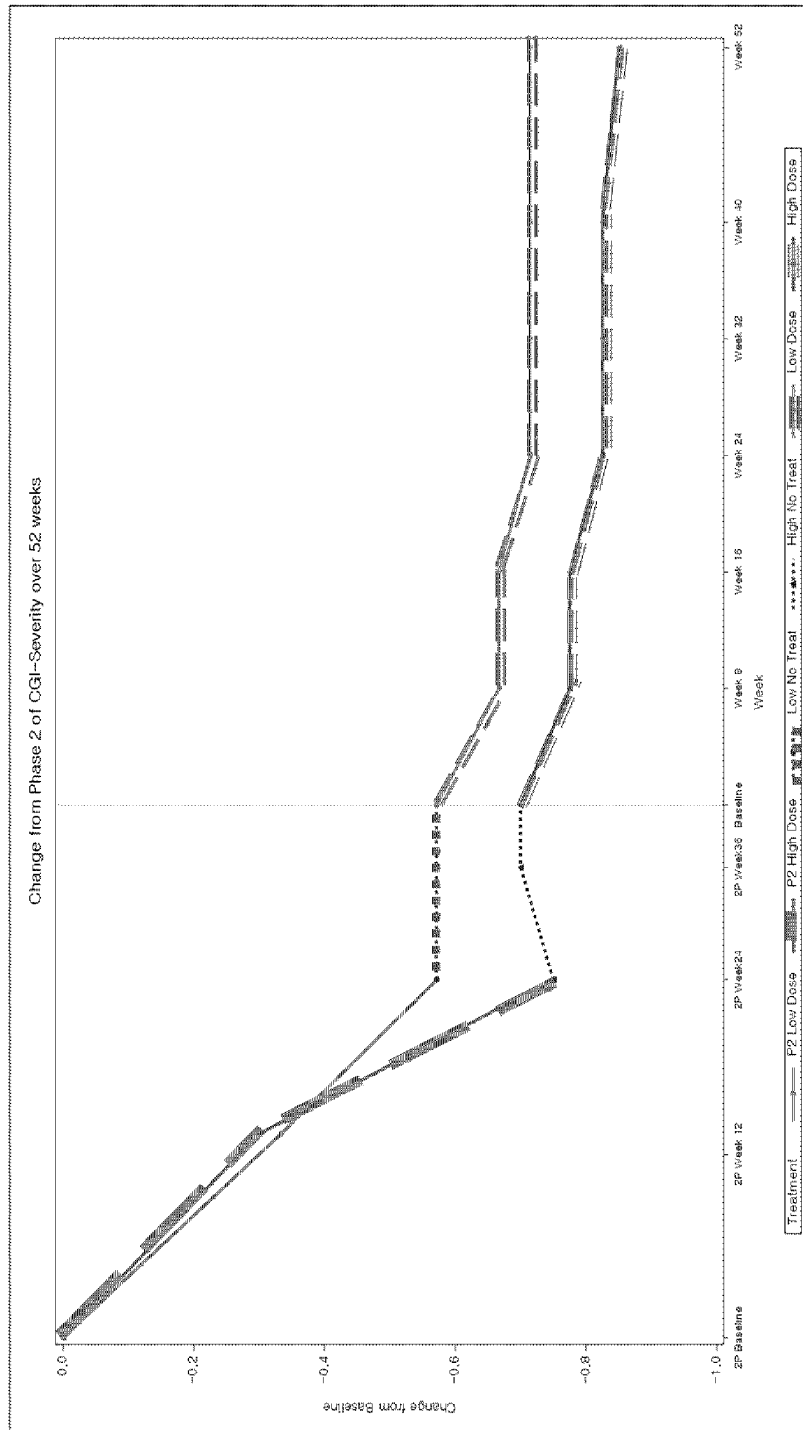
[도15]



[도 16]



[도17]



[도 18]

Simulation Scenario (Weight-based dosage 4)									
Weight range	Dose	Treatment				Placebo			
		AK-VABS-II-ABC at 12 week ^a			Target attainment (%) ^b	AK-VABS-II-ABC at 12 week ^a			Target attainment (%) ^b
		5%	50%	95%		5%	50%	95%	
10 ~ 13 kg	2g BID	-0.8	1.67	4.01	40.5	-1.75	0.92	3.34	19.5
14 ~ 20 kg	4g BID	-0.71	2.06	7.65	51.5	-1.06	1.15	3.3	29
21 ~ 34 kg	6g BID	-0.91	2.13	13.05	54	-1.69	1.02	3.74	29
35 ~ 49 kg	10g BID	-0.07	2.77	20.99	65	-1.56	0.98	3.78	29.5
50 kg ~	14g BID	-0.65	3.74	24.81	73	-1.57	0.96	3.65	27

Simulation Scenario (Phase 2 trial dosage)									
Weight range	Dose	Treatment				Placebo			
		AK-VABS-II-ABC at 12 week ^a			Target attainment (%) ^b	AK-VABS-II-ABC at 12 week ^a			Target attainment (%) ^b
		5%	50%	95%		5%	50%	95%	
10 ~ 14 kg	2g BID	-0.8	1.67	4.01	40.5	-1.75	0.92	3.34	19.5
15 ~ 24 kg	4g BID	-0.7	2.11	7.72	53	-1.06	1.15	3.3	29
25 ~ 37 kg	7g BID	-0.9	2.32	15.4	56	-1.69	1.02	3.74	29
38 ~ 51 kg	10g BID	-0.068	2.76	20.99	65	-1.56	0.98	3.78	29.5
52 kg ~	14g BID	-0.67	3.73	24.76	73	-1.57	0.96	3.65	27

Target attainment: 0주(베이스라인) 대비 12주의 K-VABS-II-ABC 점수 변화량이 2점 이상 증가한 소아 환자의 비율