

Additional file 1

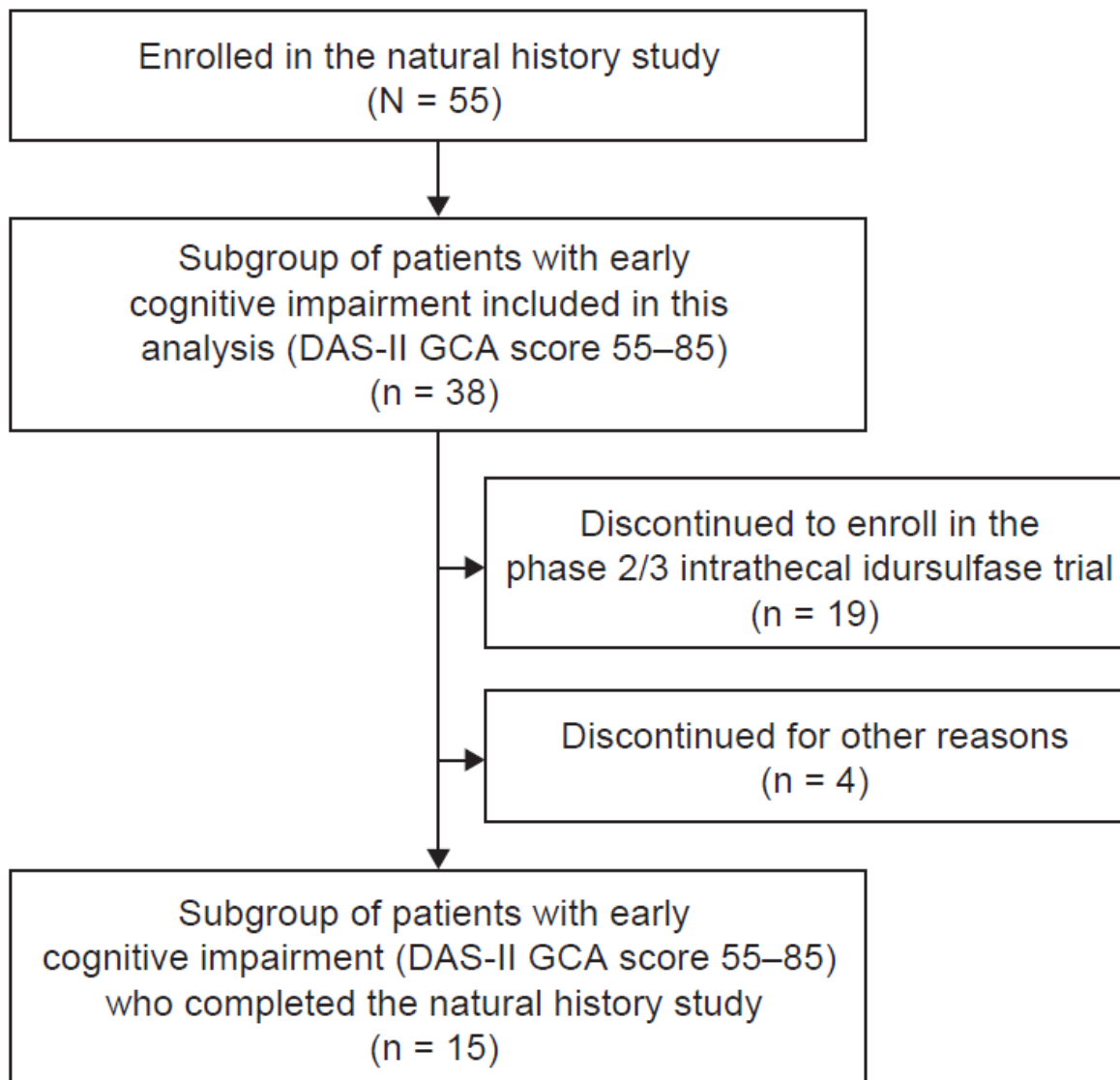
Table S1. Individual patient early years and school age DAS-II GCA scores by study visit

Patient	Study visit (month)								
	0	3	6	9	12	15	18	21	24
#1*	60	64	72	67	-	-	-	-	-
#2*	87	83	66	75	77	-	-	-	-
#3*	89	80	63	98	84	80	-	-	-
#4*	95	87	88	-	74	-	-	-	-
#5*	83	66	-	-	-	-	-	-	-
#6	91	-	77	-	-	-	-	-	-
#7	59	-	-	-	-	39	42	32	33
#8	70	-	-	-	-	-	-	-	34
#9*	66	-	-	-	-	-	-	-	-
#10	81	82	83	85	89	86	84	83	87
#11	62	63	-	73	72	63	68	68	66
#12	79	-	-	-	-	-	-	-	-
#13*	56	46	50	33	45	-	-	-	-
#14*	90	92	90	84	91	87	71	-	88
#15*	68	63	65	63	-	-	-	-	-
#16*	92	87	91	70	77	79	-	-	-
#17*	-	-	77	64	69	76	56	61	-
#18	-	78	86	95	87	87	89	94	92
#19*	64	63	64	-	-	-	-	-	-
#20	55	47	55	51	48	51	47	56	-
#21	79	84	84	89	89	82	-	-	-
#22	59	42	52	37	-	-	-	-	-
#23*	65	-	-	-	-	-	-	-	-
#24*	-	73	-	55	-	-	-	-	-
#25	81	80	80	73	77	73	76	76	74
#26*	-	84	91	86	-	-	-	-	-
#27*	-	83	-	-	-	-	-	-	-
#28*	-	-	78	-	-	-	-	-	-
#29*	63	75	-	-	-	-	-	-	-
#30*	82	90	90	52	-	-	-	-	-
#31	105	76	119	115	113	116	111	115	118
#32	55	59	60	71	-	-	-	-	-
#33	48	62	69	61	58	44	47	45	37
#34	86	84	82	82	78	85	81	81	78
#35	75	77	74	81	77	82	77	76	74
#36	72	69	74	68	68	73	63	75	71
#37	56	51	43	48	51	47	46	49	52
#38	65	55	55	55	66	61	65	63	66

Asterisks indicate the 19 patients who discontinued to participate in the phase 2/3 intrathecal idursulfase trial (HGT-HIT-094; ClinicalTrials.gov identifier: NCT02055118). Hyphens denote that data were not available for that visit.

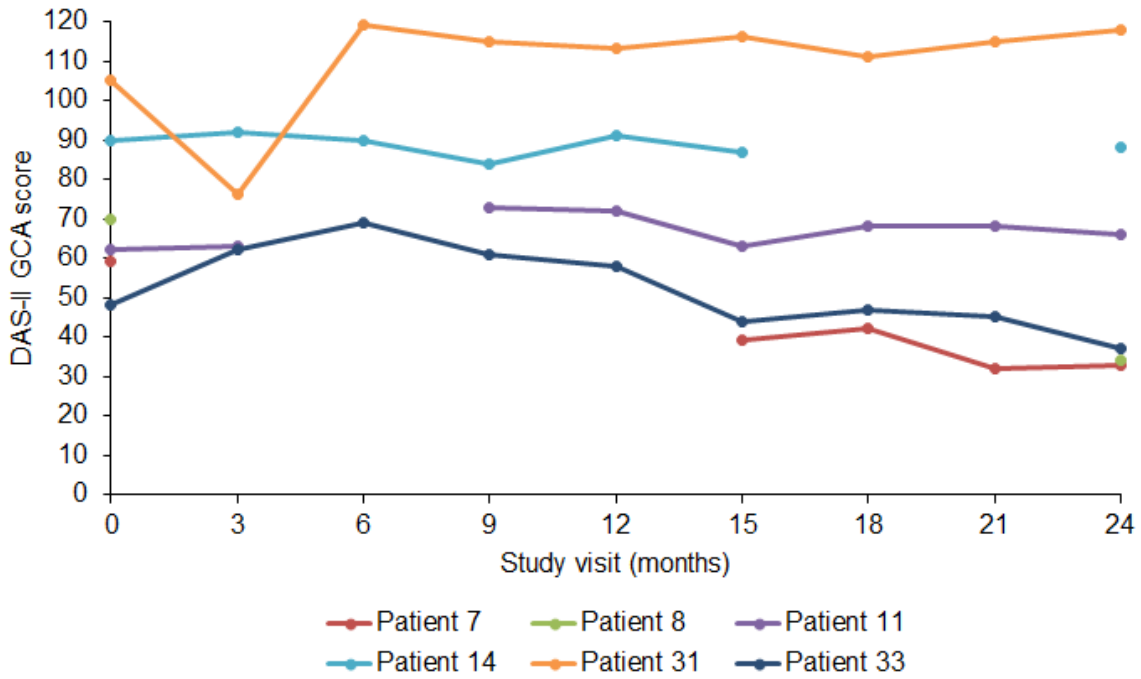
DAS-II, Differential Ability Scales, second edition; GCA, General Conceptual Ability

Fig. S1 Patient study flow



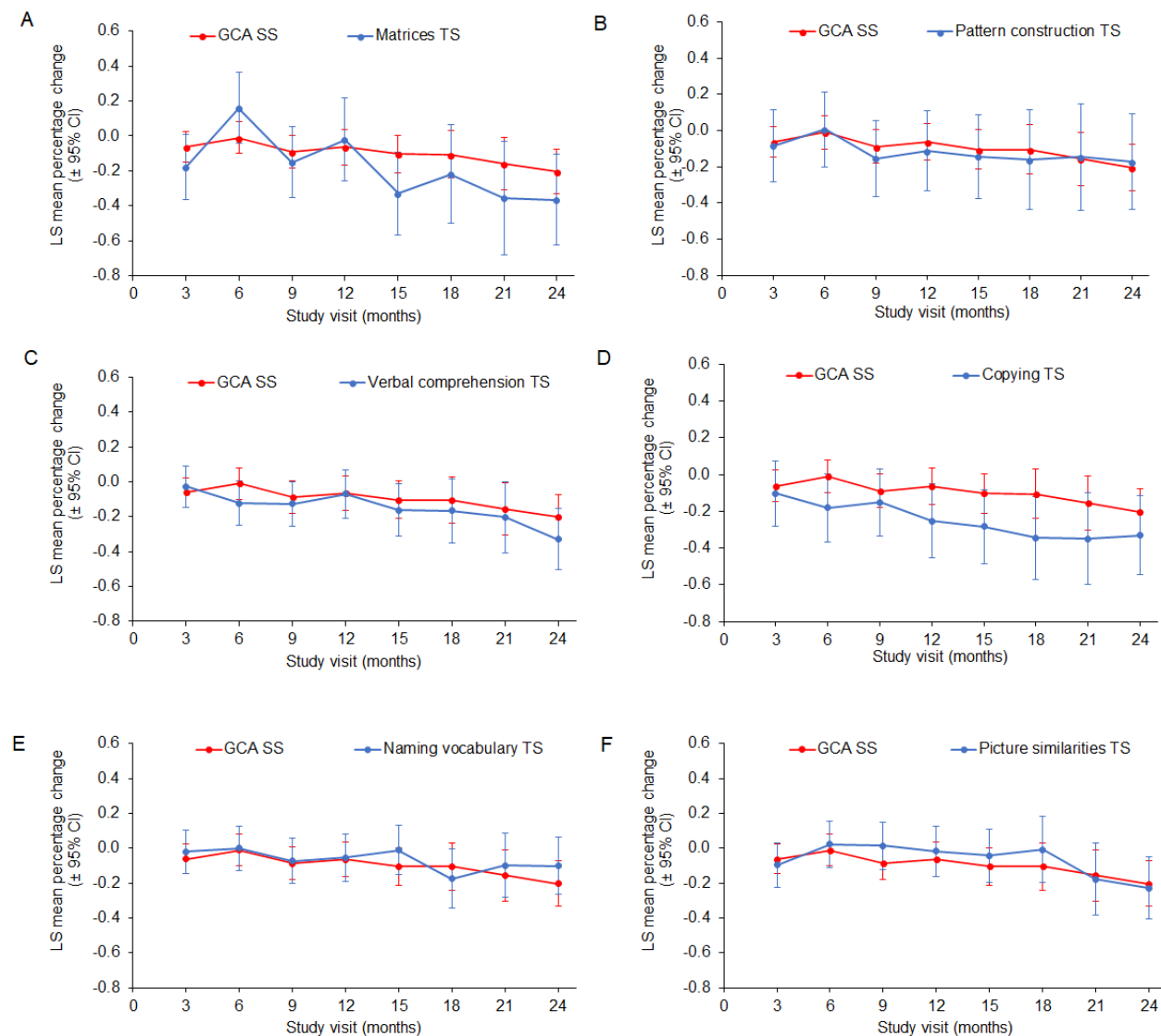
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Fig. S2 Individual early years DAS-II GCA scores for patients with assessments available at baseline and month 24



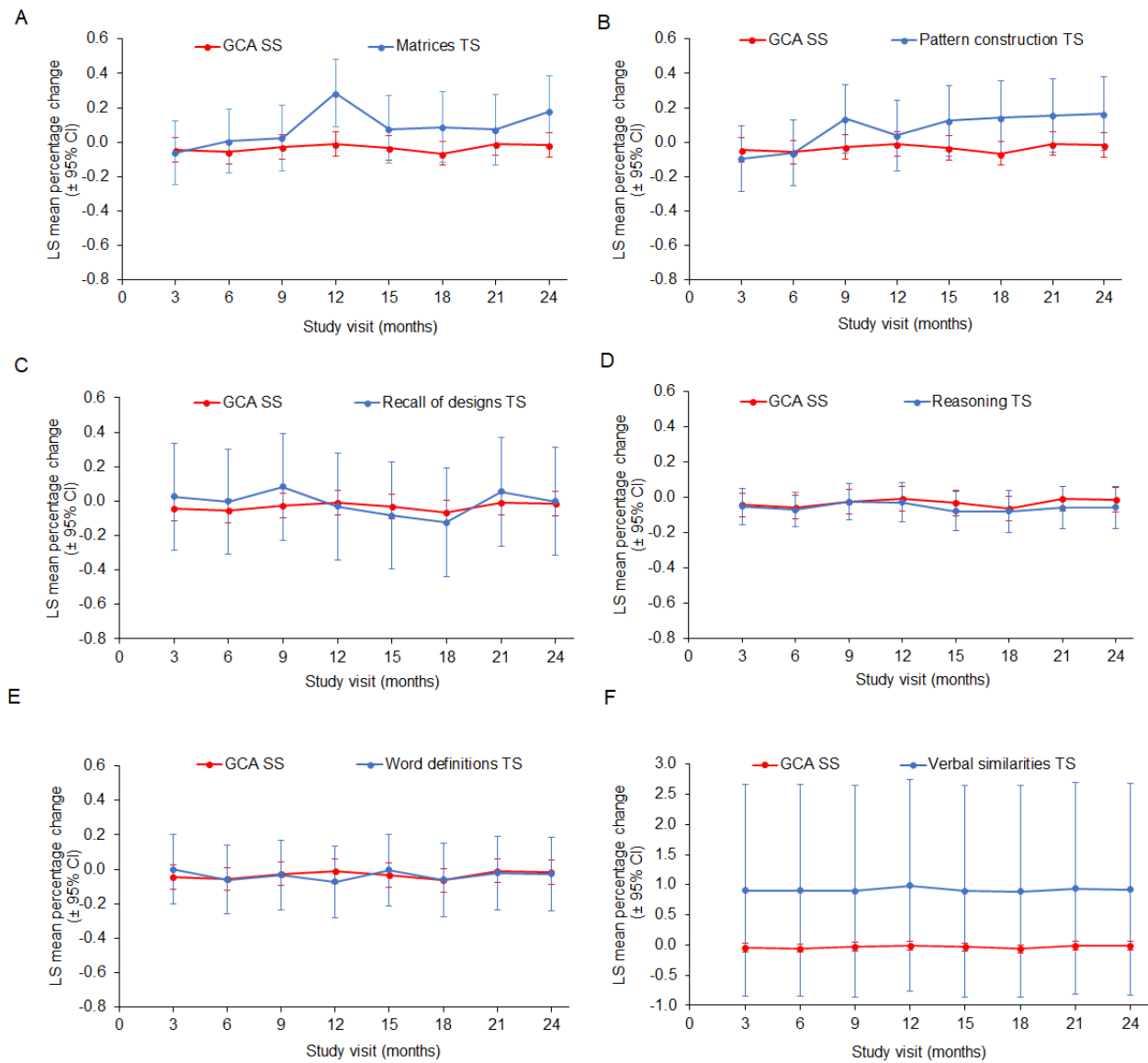
DAS-II, Differential Ability Scales, second edition; GCA, General Conceptual Ability

Fig. S3 Adjusted LS mean percentage change from baseline in DAS-II early years subtest standard scores compared with the DAS-II early years GCA standard score: (A) matrices, (B) pattern construction, (C) verbal comprehension, (D) copying, (E) naming vocabulary and (F) picture similarities



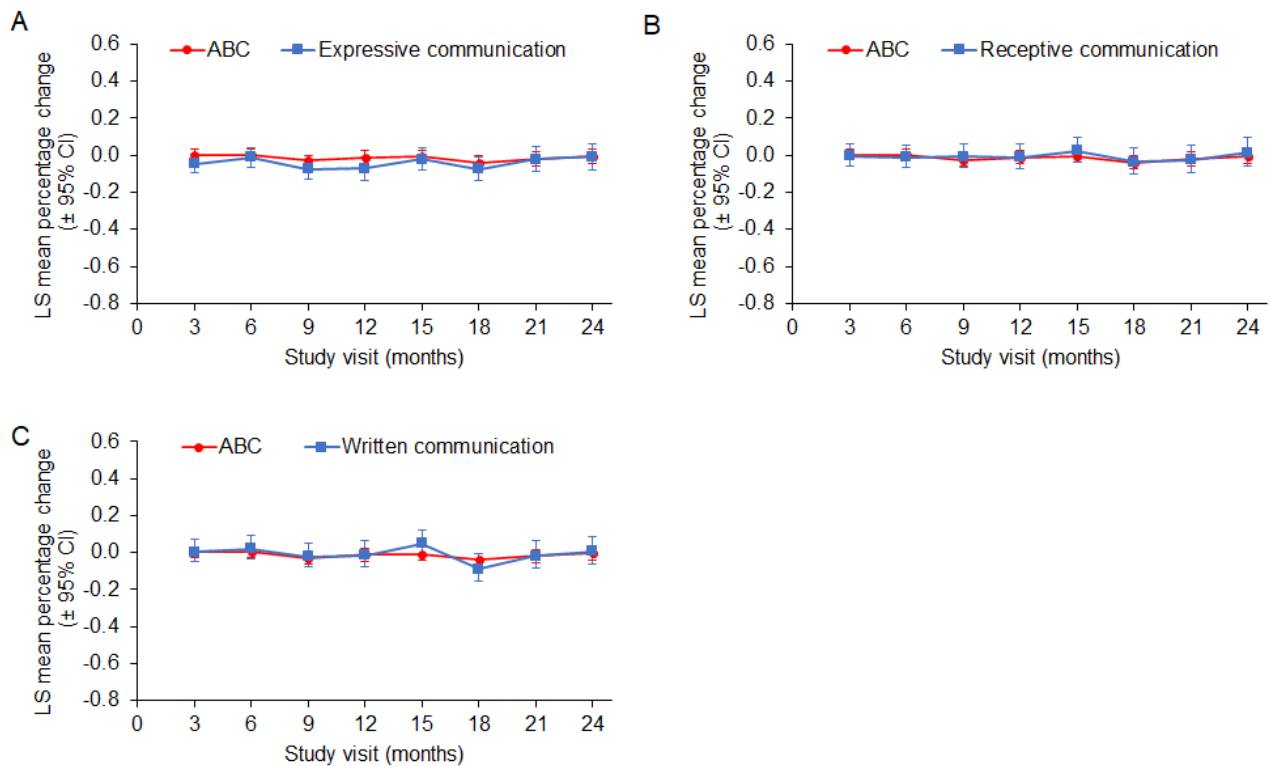
CI, confidence interval; DAS-II, Differential Ability Scales, second edition; GCA, General Conceptual Ability; LS, least-squares; SS, standard score; TS, *T* score

Fig. S4 Adjusted LS mean percentage change from baseline in DAS-II school age subtest standard *T* scores compared with the DAS-II school age GCA standard score: (A) matrices, (B) pattern construction, (C) recall of designs, (D) reasoning, (E) word definitions and (F) verbal similarities



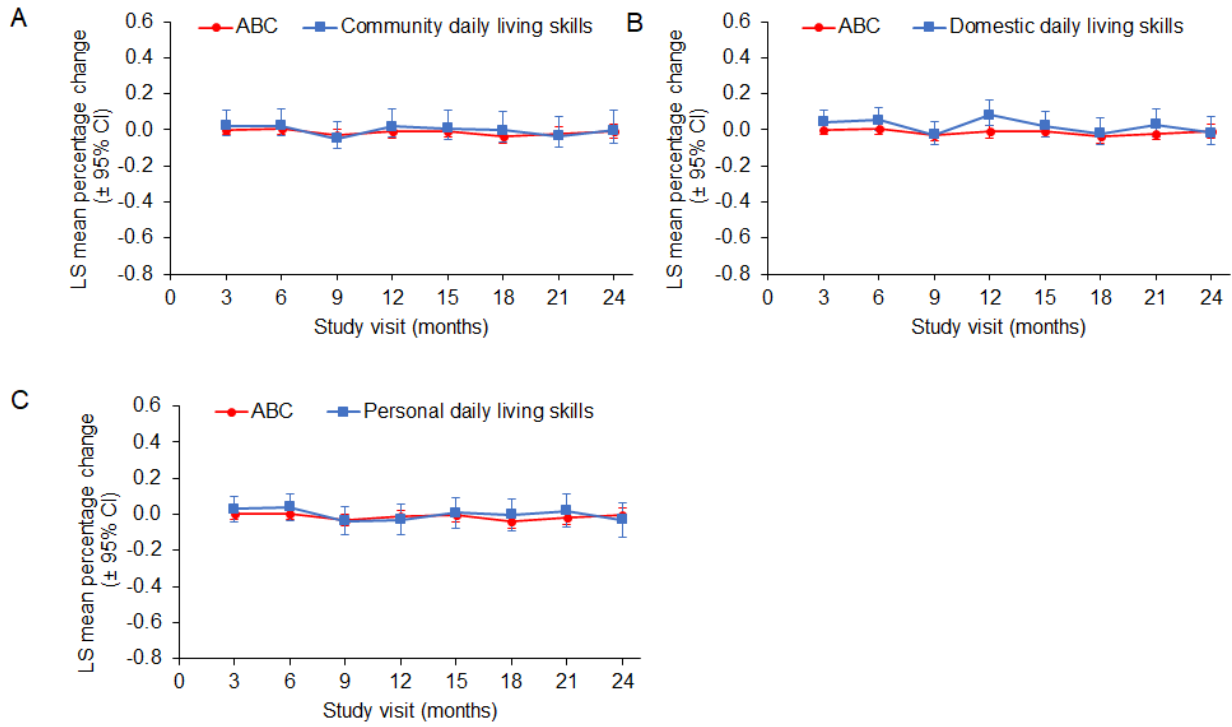
CI, confidence interval; DAS-II, Differential Ability Scales, second edition; GCA, General Conceptual Ability; LS, least-squares; SS, standard score; TS, *T* score

Fig. S5 Adjusted LS mean percentage change from baseline in VABS-II communication subdomain V-scale scores compared with the VABS-II ABC score: (A) expressive communication, (B) receptive communication and (C) written communication



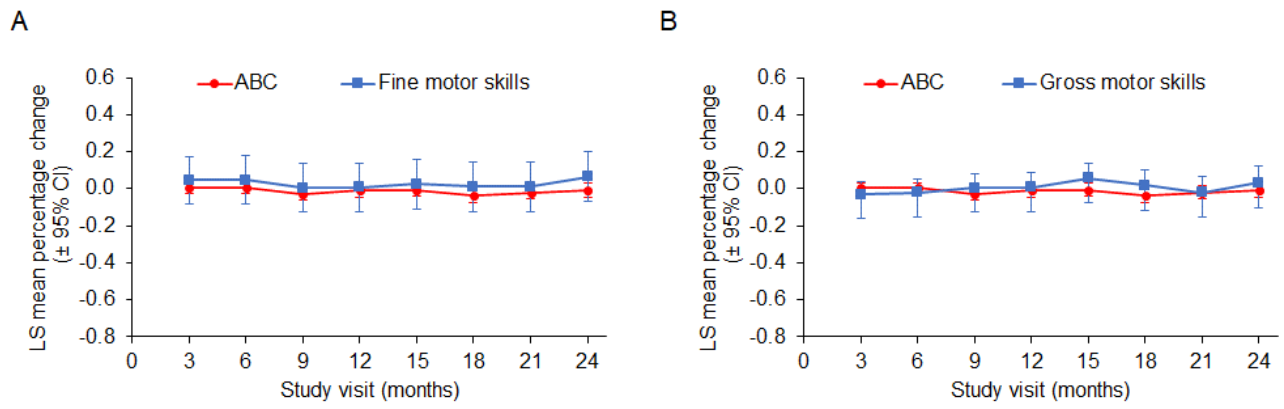
ABC, Adaptive Behavior Composite; CI, confidence interval; LS, least-squares; VABS-II, Vineland Adaptive Behavior Scales, second edition

Fig. S6 Adjusted LS mean percentage change from baseline in VABS-II daily living skills subdomain V-scale scores compared with the VABS-II ABC score: (A) community daily living skills, (B) domestic daily living skills and (C) personal daily living skills



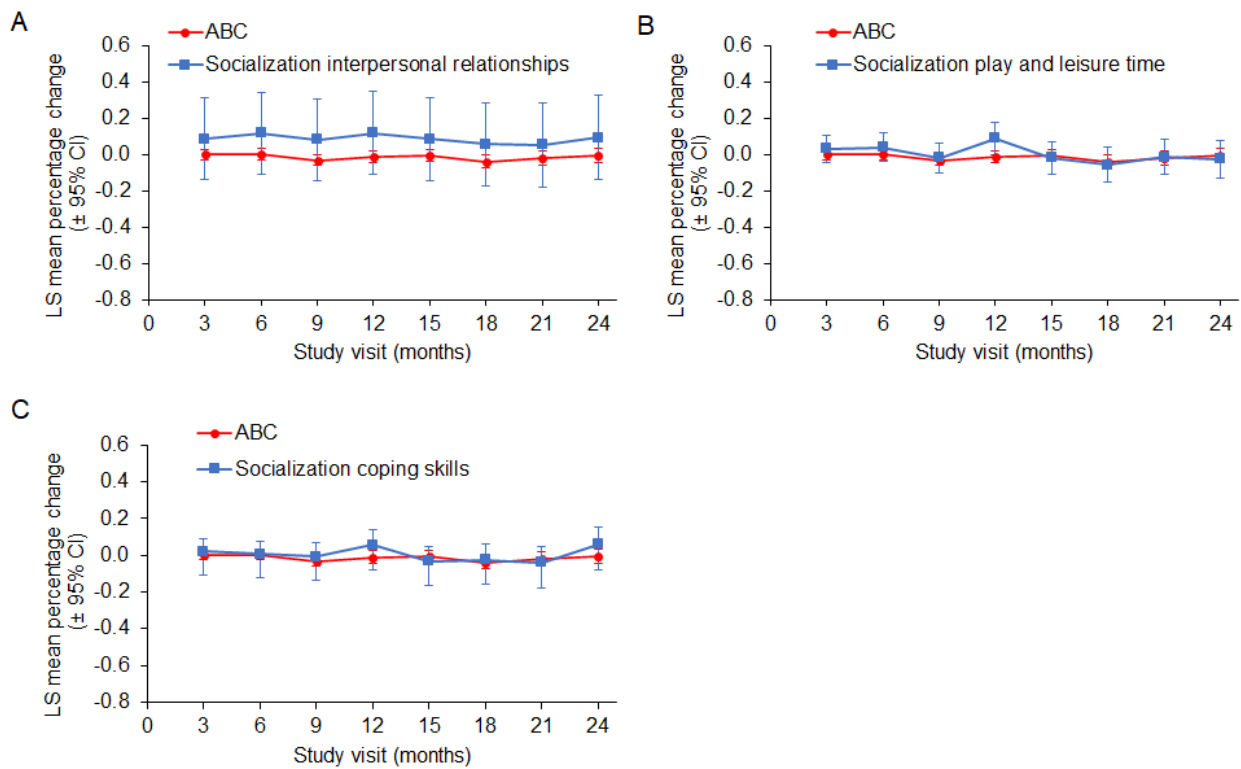
ABC, Adaptive Behavior Composite; CI, confidence interval; LS, least-squares; VABS-II, Vineland Adaptive Behavior Scales, second edition

Fig. S7 Adjusted LS mean percentage change from baseline in VABS-II motor skills subdomain V-scale scores compared with the VABS-II ABC score: (A) fine motor skills and (B) gross motor skills



ABC, Adaptive Behavior Composite; CI, confidence interval; LS, least-squares; VABS-II, Vineland Adaptive Behavior Scales, second edition

Fig. S8 Adjusted LS mean percentage change from baseline in socialization subdomain V-scale scores compared with the VABS-II ABC score: (A) socialization interpersonal relationships, (B) socialization play and leisure time and (C) socialization coping skills



ABC, Adaptive Behavior Composite; CI, confidence interval; LS, least-squares; VABS-II, Vineland Adaptive Behavior Scales, second edition

Fig. S9 HS-FOCUS domain and item scores: (A) activities, (B) breathing, (C) grip/reach, (D) school/work and (E) walking/standing

