SUPPLEMENTARY INFORMATION

Supplementary Table 1. Raw Scores on Vineland Adaptive Behavior Scales – Second Edition (Vineland-2)

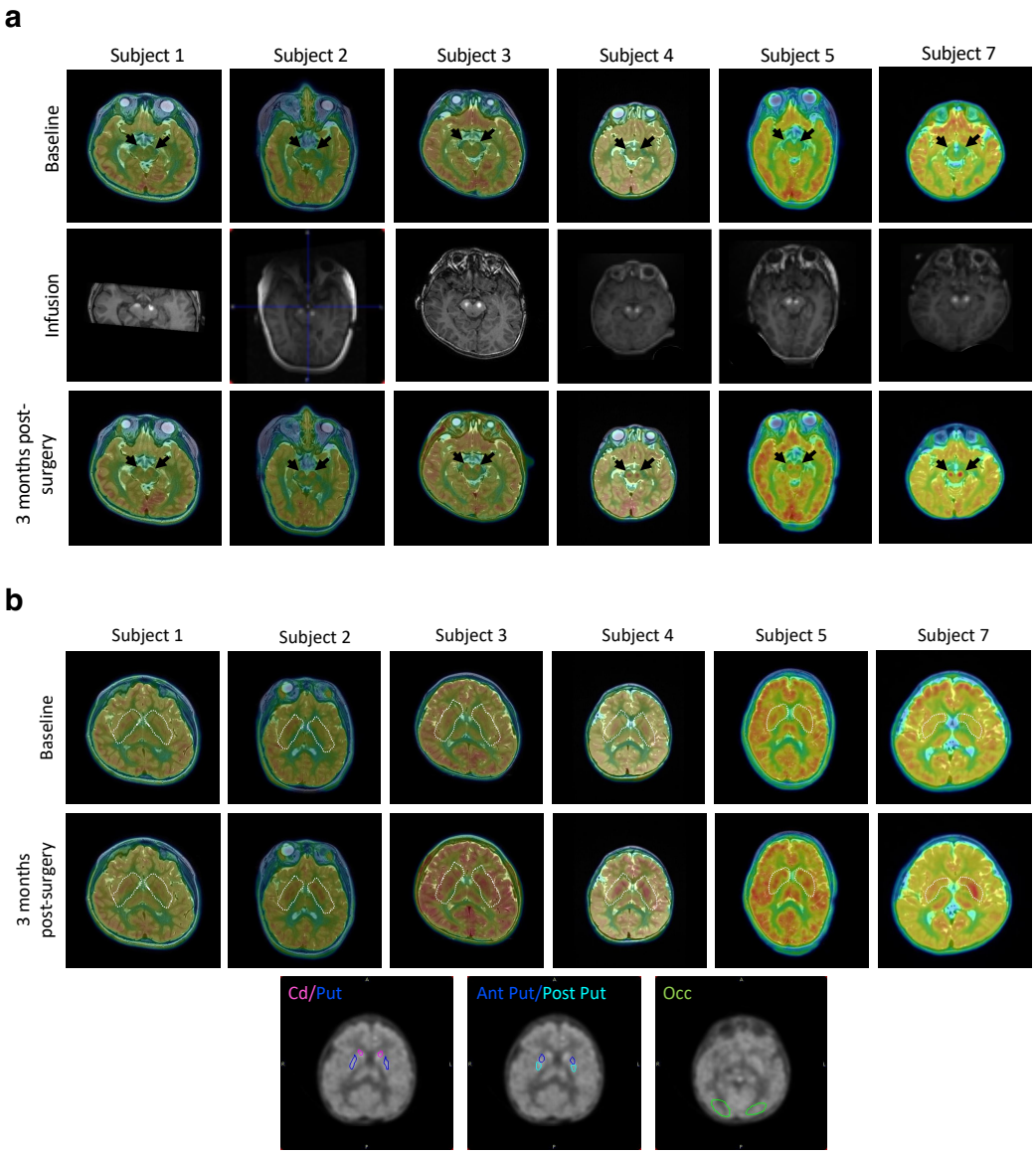
	Subject 2		Subject 3			Subject 4		Subject 5	
Skill Area	Month 12[#]	Month 24	BL	Month 12	Month 24	BL	Month 12	BL	Month 12
Receptive Communication	21	23	5	8	11	9	11	16	25
Expressive Communication	18	38	9	11	10	12	15	12	14
Written Communication	0	2	0	0	0	0	0	2	7
Personal DLS	4	9	0	5	5	0	4	4	8
Domestic DLS	0	0	0	0	0	0	0	0	0
Community DLS	2	5	0	0	1	0	0	1	1
Interpersonal Relationships	21	26	21	19	22	21	23	15	19
Play & Leisure Time	6	20	6	7	8	5	6	6	9
Coping Skills	10	17	4	4	4	3	5	4	4
Gross Motor	8	16	0	4	6	4	6	1	8
Fine Motor	10	17	0	0	1	6	9	1	12

DLS, Daily Living Skills

[#]BL assessment not performed

Note that data was not collected for Subject 1, and Subjects 6 and 7 are not included because they had not reached the Month 12 timepoint.

Supplementary Figure 1: FDOPA PET demonstrates increased AADC activity in midbrain and striatum 3 months after gene transfer.



a, Images in the top row show FDOPA uptake in the midbrain before AAV2-hAADC infusion (baseline). The center row shows representative slices of the infusion at the midbrain for each subject as visualized by the MR-tracer (white regions in midbrain). Bottom row images present the FDOPA uptake in the same area of the midbrain 3 months after surgery. Black arrows in top and bottom rows point to the infusion target (SNc and VTA). **b,** Images in top and center rows show FDOPA uptake at the level of striatum. White dotted line indicates the striatum (caudate nucleus and putamen), the postsynaptic nucleus of the nigrostriatal pathway. Images in bottom row show representative slices used to select VOIs. Ki data were normalized by referencing them to the FDOPA uptake in the occipital lobe (VOI/Occ ratio).