

YMTHE, Volume 30

## **Supplemental Information**

### **Long-term efficacy and safety of eladocagene exuparvovec in patients with AADC deficiency**

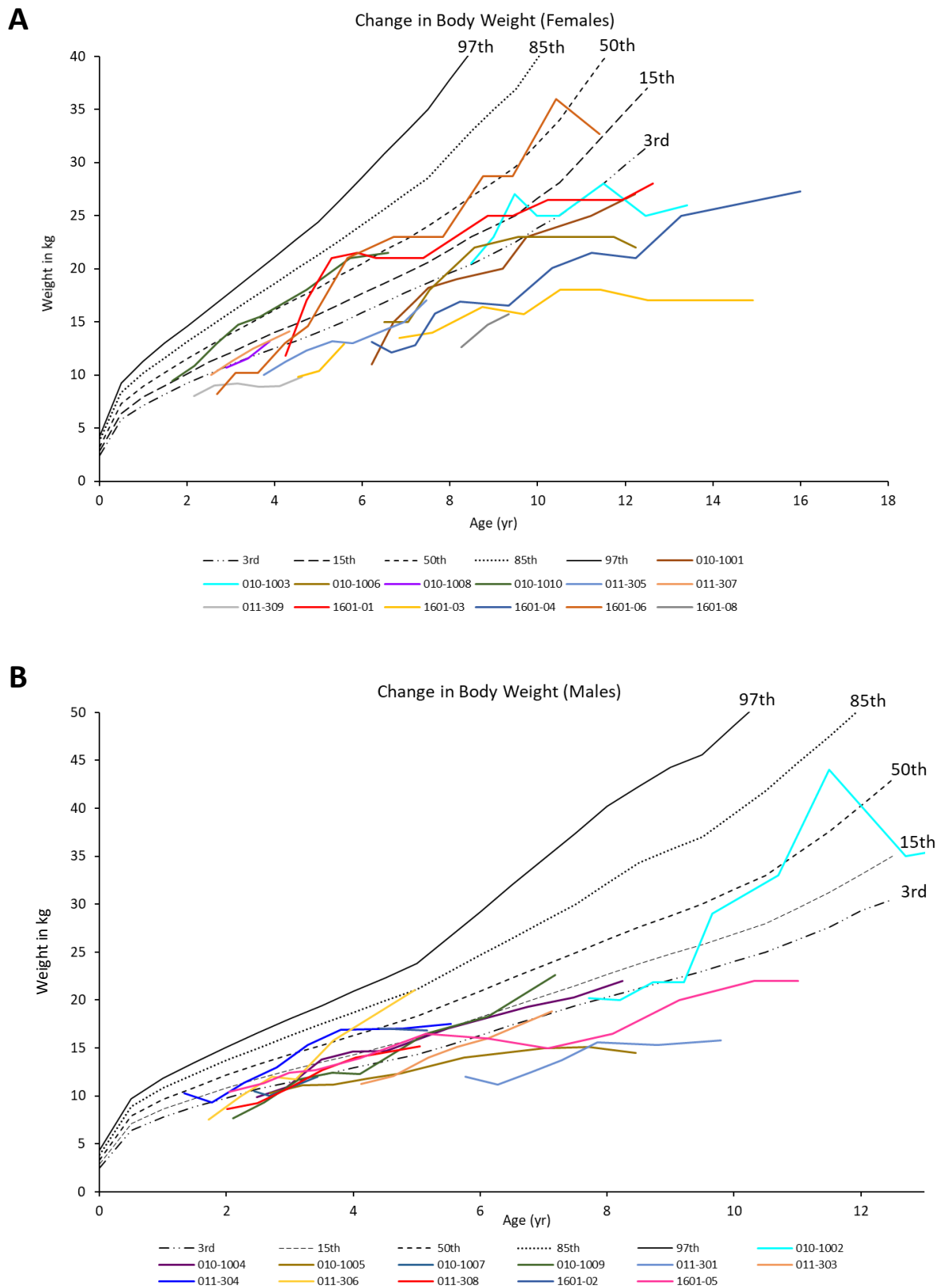
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**Table S1. Correlations Between Variables and Response to Eladocagene Exuparvovec**

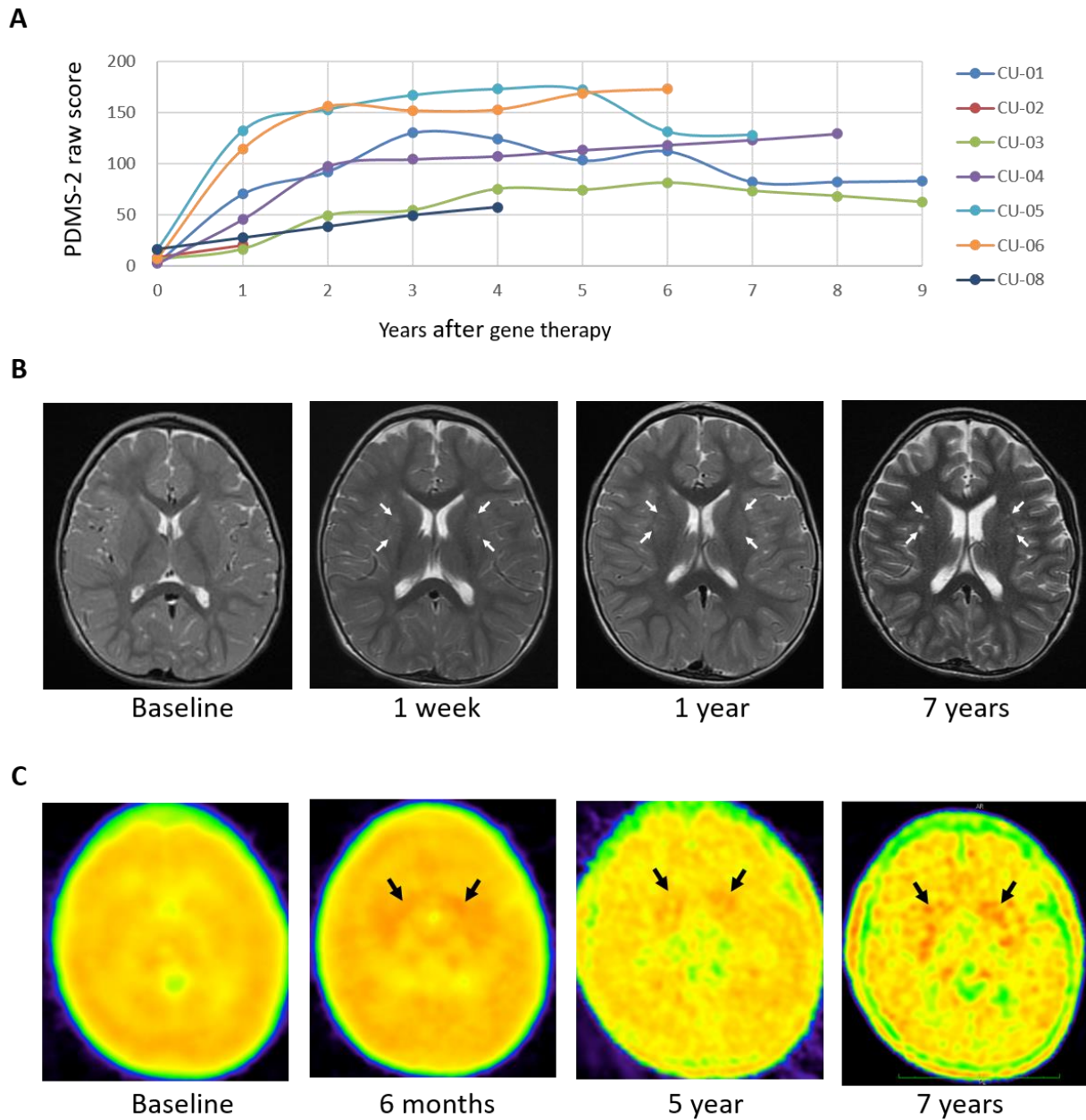
<b>Predicting Factor, Pearson Coefficient</b>	<b>First-Year PDMS-2 Score</b>	<b>Second-Year PDMS-2 Score</b>
Age at time of treatment, y	-0.698 ( $P<0.001$ )	-0.686 ( $P<0.001$ )
Pre-treatment CSF HVA	0.65 ( $P=0.001$ )	0.46 ( $P=0.04$ )
Post-treatment CSF HVA <sup>a</sup>	0.84 ( $P<0.001$ )	0.89 ( $P<0.001$ )
Pre-treatment PDMS-2 Score	0.21 ( $P=0.34$ )	0.25 ( $P=0.29$ )

CSF, cerebrospinal fluid; HVA, homovanillic acid; PDMS-2, Peabody Developmental Motor Scales–Second Edition.

<sup>a</sup>Post-treatment HVA measured at 1 year after therapy.



**Figure S1. Change in body weight following treatment with eladocogene exuparvovec.** (A) Change in body weight for 13 female patients  $\leq 9$  years after gene therapy plotted against patient age at time of measurement. (B) Change in body weight for 12 male patients  $\leq 9$  years after gene therapy plotted against age at time of measurement. Black solid and dashed lines indicate body weight growth curve percentiles for age- and gender-matched Taiwanese children without AADC deficiency.



**Figure S2. Efficacy and safety of eladocogene exuparvovec in the compassionate use.** (A) PDMS-2 score of the 7 patients  $\leq 9$  years after gene therapy. (B) Horizontal T2-weighted MRI images of CU-05 at baseline, 1 week, 1 year, and 7 years after gene therapy. Signals caused by injection in dorsal putamen are marked by arrows. (C)  $^{18}\text{F}$ -DOPA PET scans of the putamen at baseline, 6 months, 5 years, and 7 years after gene therapy. Black arrows indicate the observed signal.  $^{18}\text{F}$ -DOPA, L-6- $^{18}\text{F}$  fluoro-3, 4-dihydroxyphenylalanine; MRI, magnetic resonance imaging; PDMS-2, Peabody Developmental Motor Scales, Second Edition; PET, positron emission tomography.

**Video S1. Clips from the baseline motor evaluation of Patient 004, at the age of 2.5 years.**

**Video S2. Clips from the 5-year evaluation of the same individual.**