

## Supplementary Information

**Supplementary Figure S1. Vector genome biodistribution in CNS and PNS tissues.** Vector genome copy (GC) quantification by quantitative PCR in specimens collected at tissue harvest, three years and eight months post-intrathecal vector administration in infant rhesus monkeys. Fr. Frontal, Temp. Temporal, prox. proximal, dist. Distal. Dotted line: limit of detection 0.0001 GC/dg.

**Supplementary Table S1. Study design overview**

Animal #	Day 1 (birth)	1 month	1-9 months	1-3 years	3 years 9 months
1 F	AAV8.TBG.hIDUA 4.6x10 <sup>11</sup> GC*	AAV9.CB7.hIDUA 1.7x10 <sup>12</sup> GC <sup>†</sup>	Weekly to monthly blood and CSF sampling	Yearly blood sampling	Blood, CSF, PBMC, tissue collection
2 M	AAV8.TBG.hIDUA 3.8x10 <sup>11</sup> GC*	AAV9.CB7.hIDUA 1.8x10 <sup>12</sup> GC <sup>†</sup>			
3 M	AAV8.LSP.hFIX 4.7x10 <sup>11</sup> GC*	AAV9.CB7.hIDUA 2.2x10 <sup>12</sup> GC <sup>†</sup>			
4 M	AAV8.LSP.hFIX 5x10 <sup>11</sup> GC*	AAV9.CB7.hIDUA 2.1x10 <sup>12</sup> GC <sup>†</sup>			

\* 1x10<sup>12</sup> GC/kg

<sup>†</sup> 3x10<sup>12</sup> GC/kg

GC=genome copies, CSF=cerebrospinal fluid, PBMC=peripheral blood mononuclear cells