Supporting Information

Hinderer et al. 10.1073/pnas.1413645111

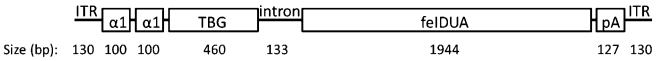

Fig. S1. Vector design. The vector consisted of the codon optimized feline α-l-iduronidase (IDUA) sequence (feIDUA) expressed from a liver-specific promoter consisting of the thyroxine-binding globlulin promoter (TBG) with two α1-microglobulin/bikunin enhancers (α1) and a chimeric intron. The polyadenylation signal (pA) was taken from the rabbit β-globin gene. The construct was flanked by AAV2 inverted terminal repeats (ITR).

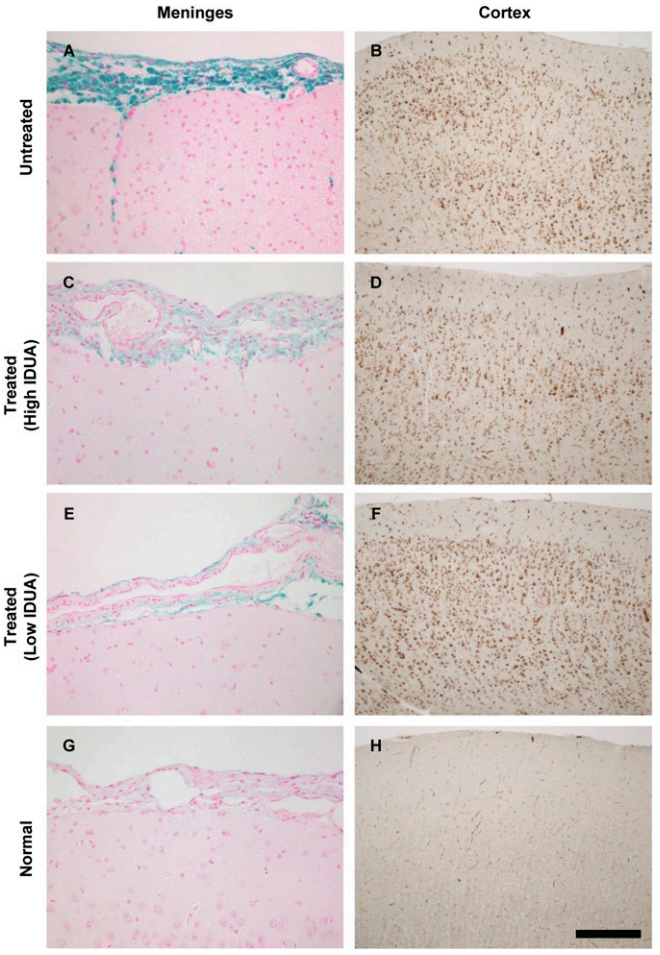

Fig. S2. Reduced storage in meninges but not brain parenchyma. Cortical brain sections with associated meninges were stained with Alcian blue (*A*, *C*, *E*, and *G*) or immunostained for the ganglioside GM3, which accumulates in neurons of mucopolysaccharidosis type I (MPS I) animals (*B*, *D*, *F*, and *H*). (Scale bar, 100 μm for *A*, *C*, *E*, and *G*; and 450 μm for *B*, *D*, *F*, and *H*.)

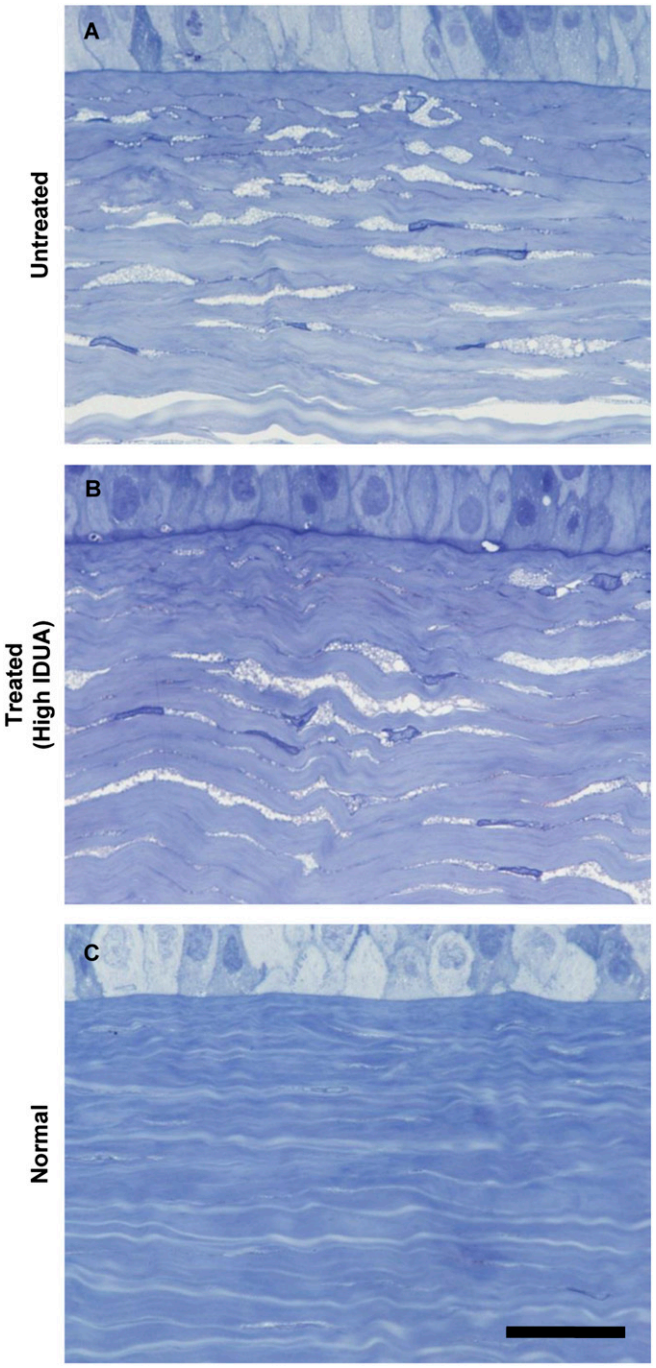

Fig. S3. Persistent corneal storage lesions in treated cats. Cornea sections were stained with toluidine blue. (Scale bar, 30 μm.)

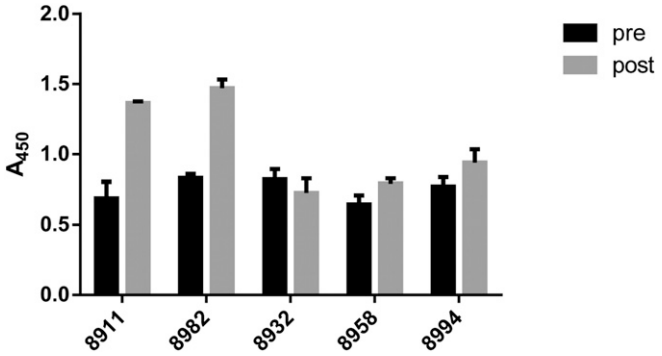

Fig. S4. Absence of detectable anti-IDUA antibodies. An indirect ELISA was performed with serum samples taken before vector administration and at the time of necropsy using purified his-tagged feline IDUA as a target antigen. Animals 8911, 8982, and 8932 were described in a previous study (1) in which 8911 and 8982 were found to develop antibodies against feline IDUA, which coincided with a lower circulating cerebrospinal fluid and serum IDUA activity.

1. Hinderer C, et al. (2014) Intrathecal gene therapy corrects CNS pathology in a feline model of mucopolysaccharidosis I. *Mol Ther*, 10.1038/mt.2014.135.