Supplementary Information

AAV Gene Therapy for MPS1-associated Corneal Blindness

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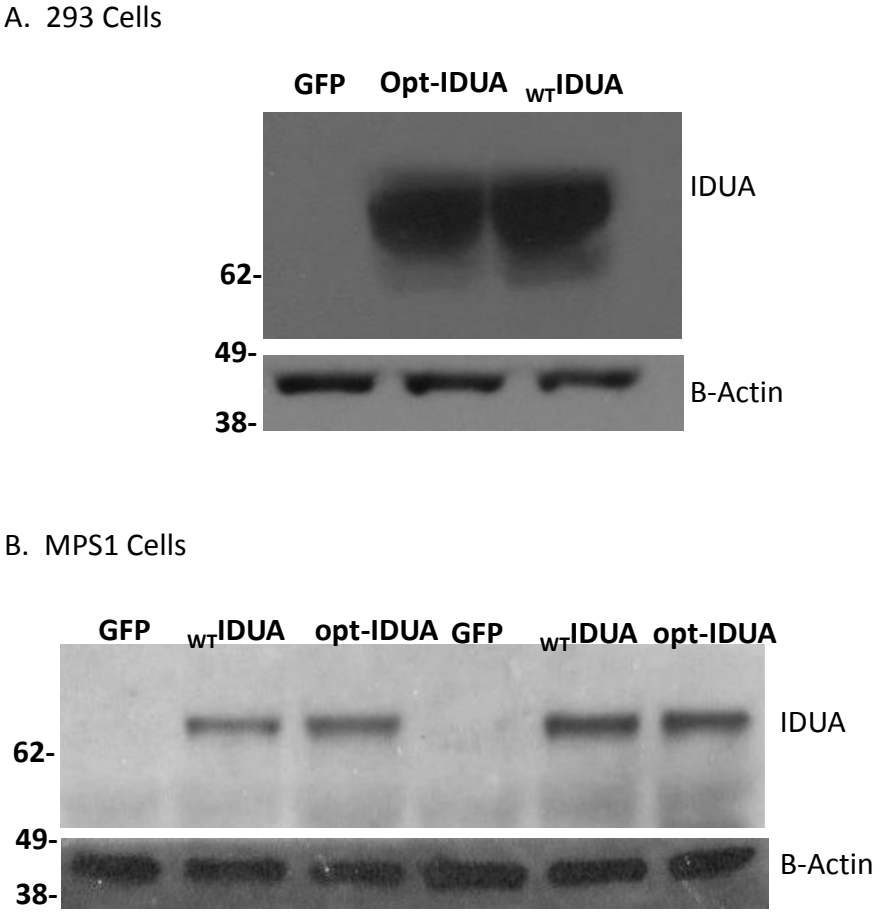

Figure S1. Evaluation of Optimized IDUA. 293 cells (A) or MPS1 human fibroblasts (B) were transfected with identical expression cassettes containing the wild type ($_{WT}$IDUA) or codon-optimized (opt-IDUA) IDUA open reading frames. Three days post-transfection Western blot analysis was used to determine the overall abundance of IDUA and B-actin. The sizes of a molecular weight marker is listed in kiloDaltons on the left.

Fig S1

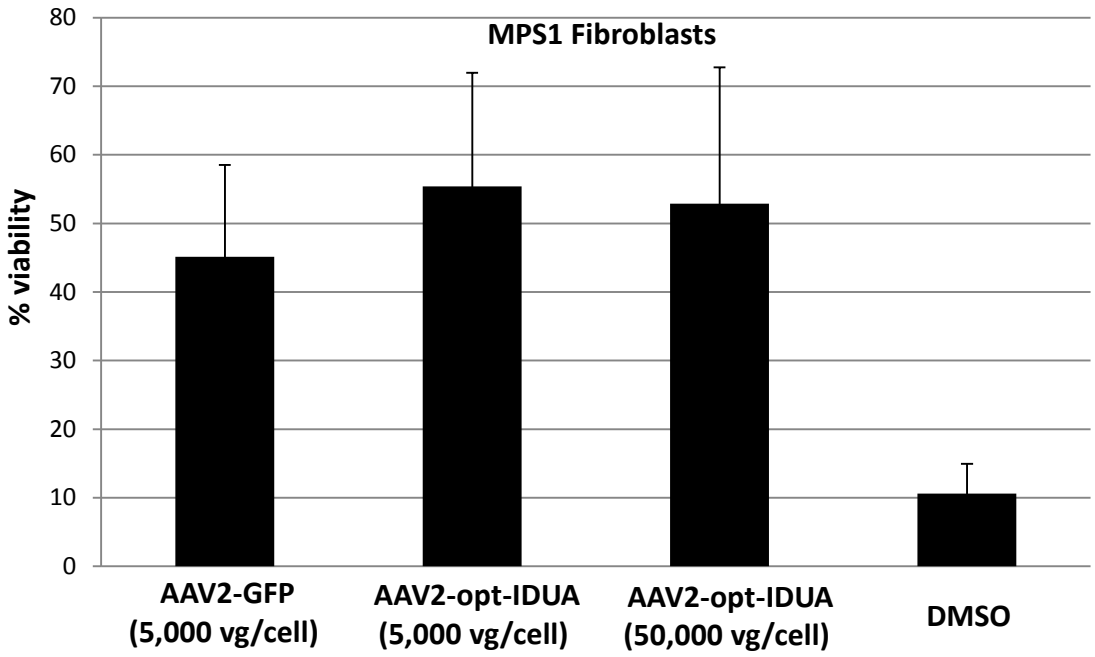

Figure S2. Analysis of AAV2-optIDUA toxicity in MPS1 patient fibroblasts. Trypan blue dye exclusion assay was performed on patient fibroblast cells following infection with AAV2 vector carrying opt-IDUA sequence or GFP reporter gene. Incubation with DMSO was used as a cell death control. Vg, viral genomes.

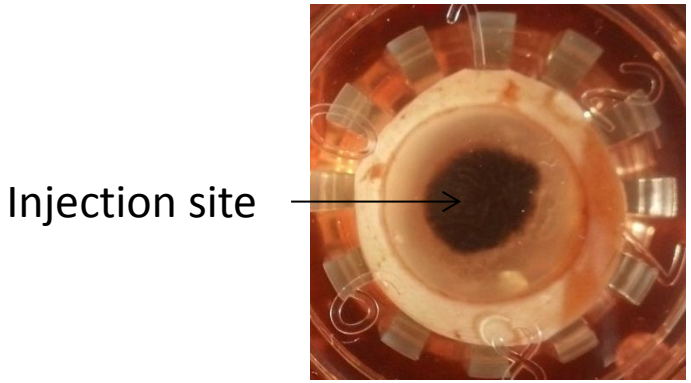

Figure S3. Intrastromal injection. Representative image demonstrating the dye distribution following an intrastromal vector injection in a cornea explant. India ink was used in a vector prep with a final volume of 50 µl for each injection.