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Supplemental information

**Human iPSC-derived neural stem cells engraft
and improve pathophysiology of MPS I mice**

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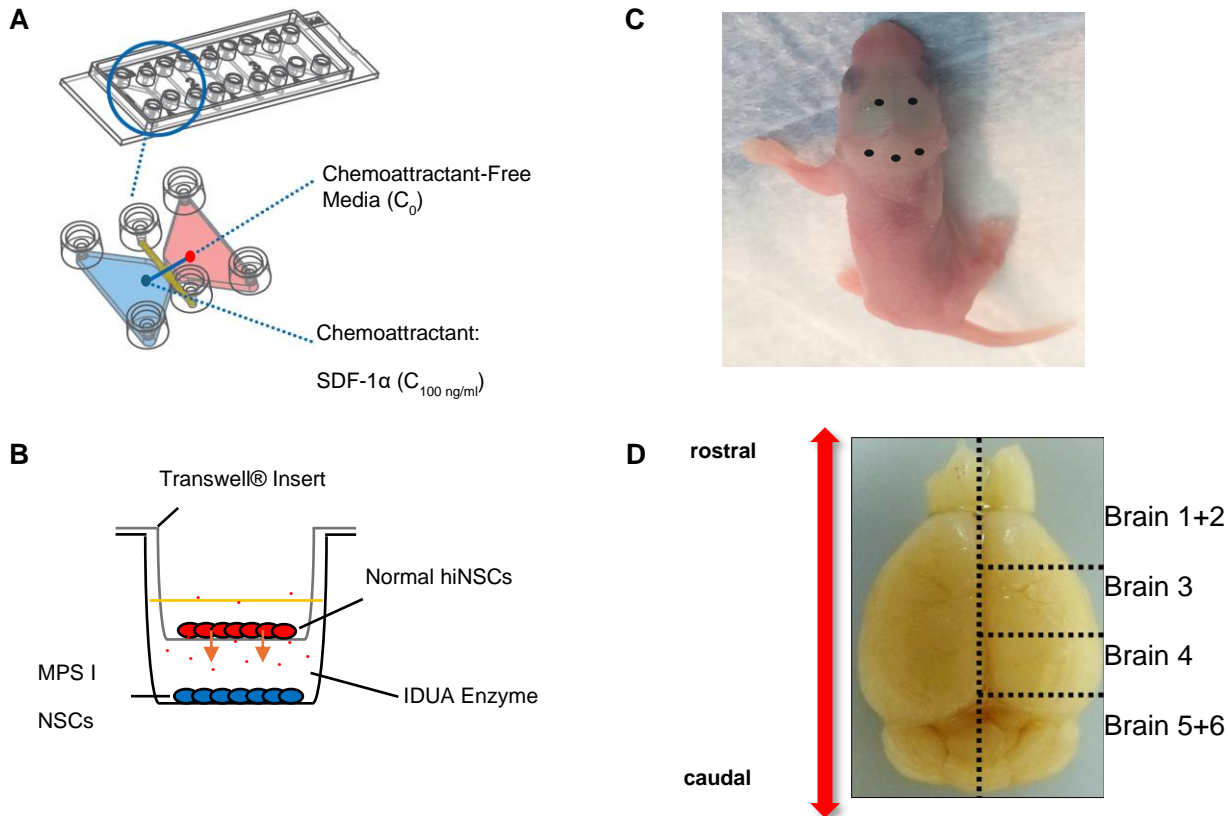


Figure S1:

A. ibidi μ -Slide Chemotaxis assay set-up used to measure qualitative migration. HiNSCs were seeded in the center observation chamber (yellow); chemoattractant SDF-1 α on the left side of reservoir chamber (blue); chemoattractant-free media on the right side of the reservoir chamber (pink). Image was authorized for use with minor modifications from ibidi GmbH.

B. Transwell $\text{\textcircled{R}}$ co-culture setup used to measure cross-correction efficacy. MPS I-NSCs (GM00415) at the bottom of the well (blue); hiNSCs in the Transwell $\text{\textcircled{R}}$ insert on top (red).

C. Immunodeficient MPS I knockout (*Idua*^{-/-}) mice aged 1-2 days received hiNSCs via bilateral injections into both lateral ventricles and intraparenchymally at three sites in the cerebellum (1×10^5 hiNSCs per site).

D. Top view of an adult mouse brain from an engrafted animal at 8 months post-transplantation. Mouse brain was dissected sagittally along the midline. The right hemispheres were coronally sectioned at 2 mm thickness and fractionated for biochemical analysis. Left hemispheres were fixed in 4% PFA for histological examination.

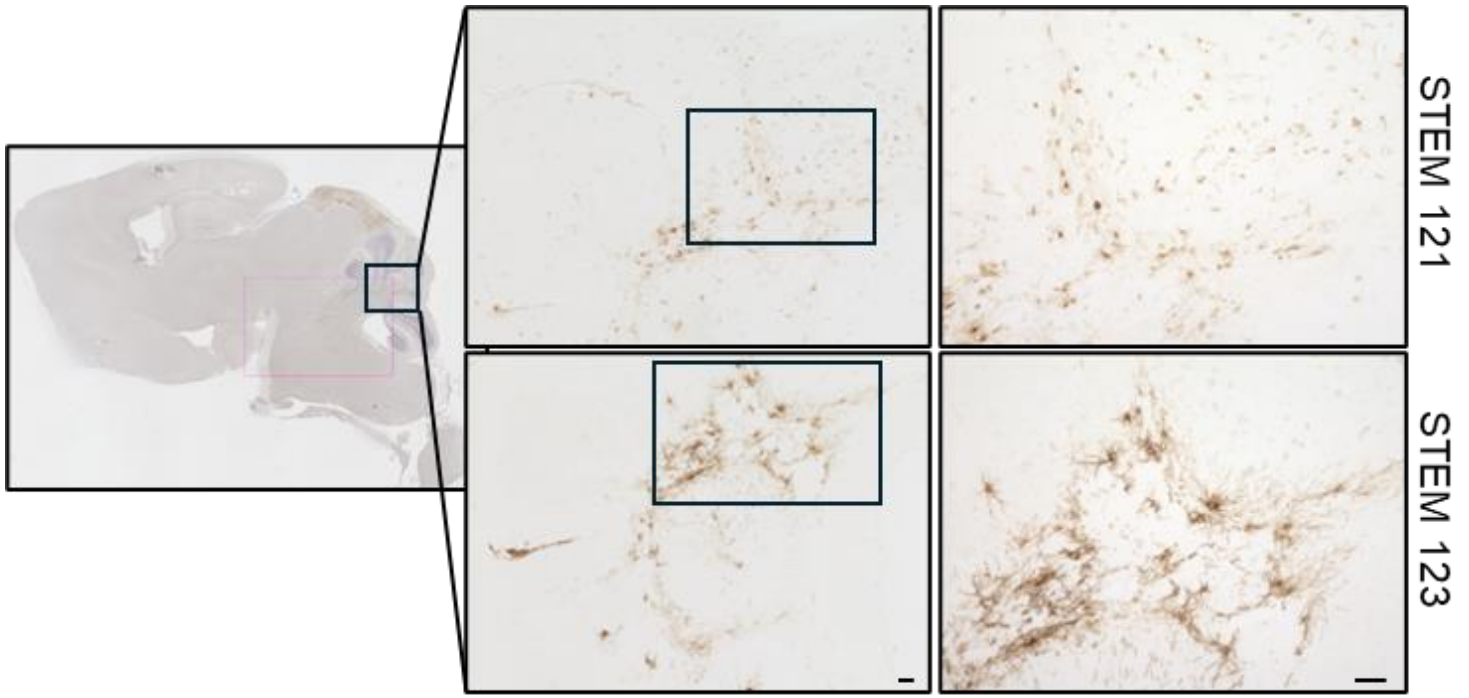


Figure S2:

Representative images show immunohistochemical staining for STEM121 and STEM 123 in the cerebellum area. To determine the differentiation status of hiNSCs after 8 months engraftment in the *Idua*^{-/-} mouse brain, sections were stained with STEM123, a marker specific to human glial fibrillary acidic protein (GFAP). Scale bars, 50 μ m

Video S1:

Description: Time-lapse video of hiNSCs (center) migrating towards the chemoattractant SDF-1 α (left) in an ibidi μ -slide chemotaxis assay, with no chemoattractant present in the right reservoir.