

62. Stem cells and therapy Clinical Trials

1 unit, Viviane Tabar, December 2, 2025

- Cell Sources
 - Fetal tissue, postmortem donors (eye banks, etc)
 - Pluripotent stem cells
 - Induced Pluripotent stem cells (iPS)
 - The role of immunological matching
- Fate specification and Cell function
 - Fate Commitment, Environmental Factors
 - 3D Tissue engineering: matrices, scaffolds
 - Functional Assays
 - in vivo disease modeling and behavioral studies
- Stem cells as vehicles for gene / enzyme delivery
- Preclinical studies
 - Proof of Concept
 - Safety and side effects
 - Tumorigenicity / Strategies to mitigate risk (suicide genes, safety switches)
 - Good Manufacturing Practice (GMP)
- Clinical Trials
 - Trial Designs
 - The role of the FDA
- Discussion of selected previous and ongoing trials and preclinical studies
 - Macular degeneration
 - Parkinson's disease
 - Spinal cord injury
 - Brain tumors

Discussion paper:

[Preclinical Efficacy and Safety of a Human Embryonic Stem Cell-Derived Midbrain Dopamine Progenitor Product, MSK-DA01](#). Piao J, Zabierowski S, Dubose BN, Hill EJ, Navare M, Claros N, Rosen S, Ramnarine K, Horn C, Fredrickson C, Wong K, Safford B, Kriks S, El Maarouf A, Rutishauser U, Henchcliffe C, Wang Y, Riviere I, Mann S, Bermudez V, Irion S, Studer L, Tomishima M, Tabar V. *Cell Stem Cell*. 2021 Feb 4;28(2):217-229.e7. doi: 10.1016/j.stem.2021.01.004

Required Reading:

Tabar V, Sarva H, Lozano AM, Fasano A, Kalia SK, Yu KKH, Brennan C, Ma Y, Peng S, Eidelberg D, Tomishima M, Irion S, Stemple W, Abid N, Lampron A, Studer L, Henchcliffe C. Phase I trial of hES cell-derived dopaminergic neurons for Parkinson's disease. *Nature*. 2025 May;641(8064):978-983. doi: 10.1038/s41586-025-08845-y. Epub 2025 Apr 16. PMID: 40240592; PMCID: PMC12095069.