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Neural stem cellsmediated therapy of amyotrophic lateral sclerosis

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Amyotrophic lateral sclerosis (ALS) is a devastating neurodegenerative disorder characterized by progressive degeneration of motor neurons which leads to definitive muscular paralysis and finally to respiratory failure. We have previously shown that NSC are able to delay motor neuron degeneration after injection into the spinal cord of EAE multiple sclerosis mice model, to boost endogenous remyelination in the corpus callosum of focally demyelinated rats and to dampen microgliosis and astrogliosis into the ischemic brain of adult. These results together with studies from other labs showing the efficacy of neural precursors to delay ALS progression when transplanted into the spinal cord of ALS rodents have promoteD NSC transplantation as a therapeutic strategy.

Moreover, cell therapy is reaching the stage of clinical application, with the first few clinical trials already underway in some post traumatic, post-ischemic or neurodegenerative disorders. A standardized, clinical grade source of normal human CNS cells (hNSCs), combining the plasticity of foetal tissue with extensive proliferative capacity and functional stability would be of paramount importance in this field. We established continuous and stable hNSC lines from the foetal CNS and showed their ability to integrate in the spinal cord of ALS animal model after transplantation. We are currently investigating some of the NSC-mediated therapeutic effects, such as trophic support to host cells and immunomodulation of the inflammatory environment.

It has to be emphasized that hNSC lines were grown according rules of Good Manufacturing Practices (GMP) in medium containing EGF and b-FGF according to the neurosphere assay paradigm and they have been recently approved by the Italian Agency of Pharmacology (AIFA) for clinical trial phase I on ALS patients.

Biography

Lidia De Filippis is a senior Post-doc researcher by University Milan Bicocca, collaborator of Prof. Angelo Vescovi. She works on human neural stem cell-mediated therapy of neurodegenerative diseases and is currently involved in translational and pre-clinical research studies on Amyotrophic Lateral Sclerosis and Lysosomal Storage Diseases.