Cellular therapy in ALS: what’s next after face I and II clinical trials?

Amyotrophic lateral sclerosis (ALS) is a neurodegenerative disease of the motor system of unknown etiology. During the last 10 years, cell therapy has been proposed as a promising treatment for ALS. But after a significant number of clinical trials, the results have not been satisfactory as consolidated therapy, because: 1) the mechanisms of therapeutic action are largely speculative, and 2) the heterogeneity in the procedures of grafting.

We designed a phase I and phase II clinical trials to evaluate the efficacy of intraspinal bone marrow cellular (BMNC) grafts versus intra-thecal cell-grafts or placebo (saline-infusion) (ClinicalTrials.gov: NCT01254539). The histological analysis confirmed a neurotrophic activity of grafted cells to host motoneurons.

The differential vulnerability between MNs suggests the influence of muscle signals as a key factor to understanding MNs resistance. Then we have conducted phase I clinical trial (ClinicalTrials.gov Identifier: NCT02286011) with intramuscular infusion of BMNC, showing benefits in the MNs and muscle electromyography parameters in ALS patients. Thus, we have proposed a Phase II clinical trial with intramuscular infusion. At the same time, we are progressing in a fundamental research program aiming to understand the molecular and cellular factors underlaying MNs heterogeneous vulnerability of MNs.

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Aula Magna Facultat Medicina i Ciències de la Salut - Campus Clínic