Stem cells for treatment of amyotrophic lateral sclerosis

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Amyotrophic lateral sclerosis (ALS) is a progressive neurodegenerative disorder leading to the death of motoneurons (MN). In our preclinical study human mesenchymal stem cells (hMSCs), were delivered intrathecally into SOD1 G93A transgenic rats. Survival in the hMSC-treated group was prolonged and rats showed better motility and grip strength. We found greater numbers of perineuronal nets (PNNs) in the hMSCs-treated animals and MSCs have antiapoptotic and immunomodulatory effects. The clinical study was designed as a prospective, non-randomized, open-label study (phase I/IIa, EudraCT No. 2011-000362-35) to assess the safety and efficacy of autologous BM-MSC in the treatment of ALS. BM-MSC were applied via lumbar puncture into the cerebrospinal fluid. During the 18-month follow-up period no serious adverse reactions or new cerebrospinal pathology on MR examinations were observed. The clinical outcome was evaluated by an ALS functional rating scale (ALSFRS), norris spinal and bulbar scale (NSS and NSB), forced vital capacity (FVC) and weakness scale (WS). In almost 80% of patients FVC values remained above 60% for a time period of 12 months. A group of 14 patients, with remarkable pretreatment decline in functional scales (ALSFRS + NSS), had significant reduction/stabilization in their total functional score decline at 3 months after application ($p<0.02$) that persisted for 6 months ($p<0.05$). In about 80% of the patients, FVC values remained stable or above 70% for a time period of 9 months. These results demonstrate that the intrathecal application of BM-MSC in ALS patients is a safe procedure and that it can slow down progression of the disease.

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