effects of repeated autologous mesenchymal stem cells transplantation on cognition and serum biomarkers in progressive multiple sclerosis: interim analysis of an open label extension trial

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Introduction:

Intrathecal injection (IT) of autologous, bone marrow-derived mesenchymal stem cells (MSC) was shown robust clinical and radiological effects in a previous double-blind randomized study from our center (NCT02166021).

Objectives/Aims:

We evaluate here the effect of repeated MSC transplantations on cognition and objective serum biomarkers of neuroinflammation and neurodegeneration, namely, neurofilaments light chains (NFL) and glial fibrillary acidic protein (GFAP), in an open-label extension trial.

Methods:

48 patients with progressive MS (PPMS and SPMS) who participated in the previous double-blind trial with MSC injections, were included in the current extension study. Four cognitive tests (SDMT, CVLT, BVMT, COWAT) and testing for serum NFL and GFAP levels using Quanterix technology (SIMOA) were performed at baseline before treatment, and at 4-5 time points following the first MSC-injection.

Results:

17 patients were treated with at least 2 intrathecal injections of MSC, 3-6 months apart, and 12 received 3 MSC injections. Nine out of 15 tested patients, treated by at least two injections of MSC, improved in 25 feet walking, by 5-18 %. The average z-score of 4 cognitive tests (SDMT, CVLT, BVMT, COWAT) improved from 0.11 at baseline to 0.33 after three MSC injections, in a period of one year. Thirteen out of 22 patients who received at least one MSC treatment, showed improvement in the SDMT scores. Six out of 17 patients improved by more than 4 degrees in SDMT, in three consecutive tests over a year. The NFL levels were reduced from a mean of 15.7 pmol/ml at baseline to 12.8 pmol/ml during the whole post-treatment period. GFAP levels were reduced from 191.4 pmol/ml at baseline, to 155.4 pmol/ml during the whole post-treatment year.

Conclusion:

The interim analysis of this extension trial reveal indications of significant beneficial effects on cognition, and on objective biomarkers of neuroinflammation and neurodegeneration, in patients treated with repeated IT injections of autologous MSC.

Disclosures:

Nothing to disclose