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# STEM CELL CLINICAL TRIAL:

# Mesenchymal Stem Cell Transplantation in Multiple Sclerosis

NCT01228266

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## BACKGROUND:

- **Mesenchymal Stem Cells (MSCs) have been used in the past for cellular therapies due to their ability to differentiate into different tissue types.**
- **However more recently, MSCs have emerged as a potential therapy for Multiple Sclerosis (MS) based on not their tissue replacement properties, but rather their ability to inhibit pathogenic immune responses and promote tissue protection and repair.**
- **In this study a randomized, double blind, crossover, placebo controlled phase II trial was conducted using autologous MSC transplantation in patients with relapsing remitting MS (RRMS).**

## METHOD:

- Participants were eligible for the trial if their MS was defined as relapsing-remitting and they were not responsive to an approved therapy for at least a year.
- All participants must also have had a clinically documented relapse and/or at least 1 gadolinium-enhancing lesion (GEL) on MRI within the last 12 months, be aged 18-50 years old and have had the disease for a duration of 2 to 10 years.
- Bone marrow was aspirated from the posterior-superior iliac spine (80-100 mL) with the mononuclear cell fraction being isolated via Ficoll density gradient centrifugation.

# METHOD: CONTINUED

- Cells were grown in culture until the defined cellular doses were reached after which the cells were either administered as baseline or cryopreserved for reverse administration at 6 months.
- All cells were previously analyzed by flow cytometry for the presence and absence of specific cell surface markers.
- Cells were administered at a dose of 1-2 million MSCs/kg through a peripheral venous cannula at baseline; placebo group received cell suspension media only.
- Safety assessments were conducted at 1, 3, 6, 7, 9, and 12 months as well as MRIs being obtained at screening, baseline and every 3 months following.
- Peripheral blood mononuclear cells were also isolated from whole blood to evaluate T and B cell population frequency in blood.

# RESULTS:

- **Overall, there were no delayed adverse events for the duration of the 12 month study. Patients treated with MSCs reported an overall trend in lowering the number of GEL at 6 months, which continued to the end of the study.**
- **The significant trend to lower the mean number of GEL in the second period indicates a potential carryover effect of the MSC transplant.**
- **Data from the immunological analysis support immunological changes that are consistent with a proinflammatory T cell profile.**
- **This indicates that MSCs induce their therapeutic effect through immunomodulatory and immunosuppressive processes rather than just tissue replacement.**

## RESULTS: CONTINUED

- **In addition, the immunological profile of those treated with freshly prepared MSCs or those treated with cryopreserved MSCs did no have any significant differences, supporting the notion that cryopreservation has no negative effect on the properties of the MSCs.**

## CONCLUSION:

- The outcome of this trial confirmed that MSCs are a safe therapy for the treatment of RRMS and that their administration intravenously is well tolerated.
- Observations of this study also indicate the immunomodulatory effect that MSCs can have with such a disease.
- Overall, the data generated from this trial justifies the need for further clinical testing for the therapeutic benefit of MSC administration for MS.

## ASSOCIATED PUBLICATIONS:


- https://clinicaltrials.gov/ct2/bye/rQoPWwoRrXS9-i-wudNgpQDxudhWudNzlXNiZip9Ei7ym67VZR0wSg05SKCVA6h9Ei4L3BUgWwNG0it