

P2.221

Safety and Dosing of Autologous Neural Progenitors Injected Intrathecally in Multiple Sclerosis Patients: Results of a Pilot Study

Saud A. Sadiq, Sydney Chirls, Daniel Koffler, and
Violaine K. Harris

Tisch MS Research Center of New York

Funding: Damial Foundation
No disclosures.



TISCH MS
RESEARCH CENTER OF NEW YORK

Background

Mesenchymal stem cell-derived neural progenitors (MSC-NPs) are an autologous source of bone marrow stem cells proposed to promote repair and regeneration in MS.

Methods: Autologous MSC-NPs were given intrathecally (IT) as part of a pilot study to determine dosing and feasibility of administration. Subjects were evaluated for degree of disability (EDSS) and adverse events before and after each treatment.

Study subjects	4 SPMS, 2 PPMS, 1 spinal cord atrophy
Dosing (escalation)	5×10^4 to 1.6×10^7 autologous MSC-NPs
# of IT doses	Average 3 doses (range 2 to 5 doses)
Dosing frequency	Average 4.5 months apart (range 2 to 8 months)
Follow up	Average 7.4 years (range 6.7 to 8.4 years)



Results

- No serious adverse events.
- Transient fever (5%) and headache (23%)
- 5/7 patients showed measured clinical improvement
 - 4/7 improved EDSS
 - 3/7 improved bladder/bowel function
 - 1/7 moved R index finger and thumb and began speaking (patient quadriplegic and could not speak)



Conclusions/Future Directions

- Based on these initial findings, we determined that a dose of 2 to 10 million MSC-NPs administered by IT injection every 3 months would be safe and feasible.
- Phase I safety and tolerability study of autologous IT MSC-NP treatment for 20 MS patients has commenced (FDA approval of IND in Aug 2013)

