



aGvHD

## ASH 2018 | MSC-FFM for the treatment of pediatric and adult patients with steroid-refractory graft-versus-host disease

 Anna Bartus  Elina Simanovits | Jan 08, 2019

Mesenchymal stromal cells (MSCs) are multipotent progenitor cells that showed promising activity for the treatment of steroid-resistant acute graft-versus-host disease (SR aGvHD). MSCs modulate immune responses by secreting soluble factors that can modify the stimulation, proliferation and maturation of T- and B-lymphocytes, natural killer cells, and dendritic cells. Furthermore, MSCs also induce the differentiation of regulatory T cells and regulate the Th1/Th2 ratio. At present, there are several ongoing randomized trials investigating the impact of MSCs on GvHD prevention or treatment.

At the [60<sup>th</sup> American Society of Hematology Annual Meeting & Exposition](#), [Peter Bader](#) from Hospital for Children and Adolescents; Division for Stem Cell Transplantation and Immunology, [University Hospital Frankfurt](#), Frankfurt, Germany, presented treatment data of pediatric and adult patients with either SR GvHD (27%) or GvHD treated with MSC-Frankfurt am Main (MSC-FFM), a novel MSC manufacturing protocol characterized by high *in vitro* potency and near-identity of individual doses developed by Professor Bader and colleagues.

### Patients and methods

- N = 61 pediatric patients
  - Median age = 7.7 years (range, 0.5–0)
- N = 31 adult patients
  - Median age = 42.4 years (range, 18.4–65.6)
- Patients from 23 centers in 6 countries were included in this study
- Severe GvHD: 37% grade III, 59% grade IV
- Donors: MSD (n = 21, 23%), MUD (n = 56, 61%), haploidentical (n = 14, 15%), and MMUD (n = 1, 1%)
- Myeloablative conditioning with TBI-(treosulfan, busulfan and fludarabine) based regimen
- 89% of patients had received immunosuppression for GvHD prophylaxis
- Recommended dose: 4 weekly doses of 1-2M MSC/kg
- Patients received 3 doses on average

### Key findings

- Response 28 days after MSC:

- CR: 28%
- PR: 54%
- NR: 15%
- Response at last follow-up:
  - CR: 51%
  - PR: 30%
  - NR: 17%
- Day-28 response rates were highly predictive of long-term responsiveness
- Six patients relapsed and died
- Twenty-eight deaths were treatment-related
- 6-month overall survival for children and adults: 68% and 54%, respectively
- Adverse events: headache, nausea/vomiting were reported shortly after infusion

Professor Bader concluded that MSC-FFM is effective for the treatment of severe pediatric and adult advanced GvHD. "The very low relapse mortality may suggest that severe GvHD effectively suppresses leukemic recurrence." A randomized trial for the treatment of SR aGvHD is planned to start in 2019.

## Reference

Bader P. *et al.* Children and adults with steroid-refractory acute graft-versus-host disease respond to treatment with the mesenchymal stroma cell preparation "MSC-Ffm": Treatment results for 92 consecutive patients. 2018 Dec 3. [60<sup>th</sup> American Society of Hematology Annual Meeting & Exposition](#), San Diego, CA.

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