

2024 STIFF PERSON SYNDROME SYMPOSIUM



Take-Away: The Potential of Hematopoietic Stem Cell Transplant in Treating SPS (Dr. Amanda Piquet, MD, FAAN)

Hematopoietic Stem Cell Transplant (HSCT) Overview:

Autologous HSCT: Uses the patient's cells to reset the immune system and aims to rebuild a healthy one.

Allogeneic HSCT: Uses donor cells to replace a damaged immune system, potentially curing or ameliorating conditions like Stiff Person Syndrome (SPS).

Benefits and Risks of HSCT:

- Offers the possibility of long-term, drug-free remission from autoimmune diseases.
- Early data shows significant benefits but also highlights risks such as higher mortality rates in the first-year post-transplant.

HSCT vs. Other Treatments:

- HSCT has shown superior long-term outcomes to traditional therapies in key studies in other autoimmune diseases such as scleroderma and Multiple Sclerosis (e.g., SCOT and HALT MS Clinical Trials).
- It is particularly effective in early-stage diseases, providing a better quality of life and reduced disease progression.

Guidance for Patients Considering HSCT:

- Only pursue FDA-approved treatments to ensure safety and efficacy.
- Be wary of unregulated stem cell therapies, especially those offered abroad without proper oversight.

The Future of HSCT for SPS:

- Research is ongoing with clinical trials assessing autologous and allogeneic HSCT.
- Additional cell-based therapies, such as CAR T therapy, is being explored for other autoimmune diseases and has potential as a therapeutic agent in SPS
- Focus is on developing precise, validated clinical outcome measures to assess treatment effectiveness in SPS, which will improve the development of treatment trials.