Comparing Bone Marrow Transplant and Gene Therapy for Sickle Cell Disease

Understanding the similarities and differences between bone marrow transplant (BMT) and gene therapy for sickle cell disease (SCD) helps you and your loved ones **make an informed decision** about whether these curative (or transformative) therapies are right for you.

Similarities

The similarities between bone marrow transplant and gene therapy

- **Targeted treatment**: On a cellular level both treatments help a SCD patient produce non-sickling hemoglobin
- **Potential for long-term benefits**: Both have the potential to provide long-lasting or permanent relief from certain conditions
- **Personalized approach**: Both treatments utilize individualized medicine to tailored\ the therapy to the patient's condition and needs
- **Requires specialized care**: Each treatment requires specialized medical expertise and long-term follow-up care
- **Use of conditioning**: Both treatments often involve conditioning or chemotherapy to prepare the body for the therapy
- **Infertility**: The conditioning or chemotherapy required for both procedures can potentially lead to infertility

Differences

The differences between bone marrow transplant and gene therapy

- **Need for donor**: Gene therapy is simpler using the patient's own cells, collected and modified in a lab, while BMT needs a matched donor
- **Recovery Time**: Gene therapy may offer a shorter, less invasive recovery, while BMT has a longer recovery due to its invasive nature
- **Research**: BMT has been studied longer for SCD than gene therapy. More is known about long-term effects and outcomes for BMT
- **Availability**: Due to its longevity within sickle cell, BMT is more widely available than gene therapy, however there are expanding sites

Key Terms

Hemoglobin: A protein in red blood cells (RBCs) that helps carry oxygen. The sickle cell mutation causes hemoglobin to change the RBCs into sickle shape, making it difficult to carry oxygen and causing complications.

Stem cell: Special cells that can turn into different types of cells. They are used to replace the bone marrow that makes sickle red blood cells.

Conditioning: Uses chemotherapy and sometimes radiation to prepare the bone marrow. For BMT, it clears space for donor marrow, while for gene therapy, it makes room for the patient's modified stem cells.

What now?

Interested in learning more about curative therapy?
Connect with your sickle cell specialist or specialized center to get started.

Important Questions to Ask

What to ask when considering a curative therapy

Disease burden: Understanding how much SCD affects your life to help you decide if an option is right for you.

- How much does SCD impact my daily life?
- Does SCD stop me from reaching life milestones?
- How much does SCD affect my quality of life?
- How often do I experience severe or life-threatening complications from SCD?
- Are there other treatments that might be a better fit for me that I haven't tried?

Support system: Identifying who can support you and understanding the sacrifices they might need to make.

- Who can support me throughout this journey? What sacrifices might be necessary to ensure I have a strong support system?
- Who can provide additional insights into the true experience of receiving a curative therapy?
- What do my family, loved ones, and healthcare providers think about undergoing a curative therapy?
- What kind of support will I need during this process?

Access: Determining curative treatment options based on your individual needs and locality.

- Are there treatment centers available near me?
- What are the costs associated with the procedure, and what kind of coverage do I have?
- Would I prefer a commercially available curative therapy or one that's still in clinical trials?
- Which curative therapies are available near me? None, one, or both? Am I willing to travel?
- Do I have access to a specialist experienced in both the procedure and treating SCD?
- What other barriers and potential issues might I have, if I decide to undergo a curative therapy procedure?

Journey: Understanding your best curative option, as well as what the process and recovery will involve.

- Am I a suitable candidate for bone marrow transplant or gene therapy? What are the eligibility criteria?
- What are the benefits and risks of each option?
- What are the short-term and long-term effects of the curative therapies?
- If I choose bone marrow transplant, will there be a compatible donor available?
- What options do I have for fertility preservation?
- How long will the entire treatment process take? What follow-up care will be required?

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Sickle Cell 101

Ask our sickle cell experts:
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