CURATIVE TREATMENTS FOR SICKLE CELL DISEASE

AT HACKENSACK MERIDIAN CHILDREN'S HEALTH

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WHAT IS SICKLE CELL DISEASE?

Sickle cell is an inherited blood disorder affecting the red blood cells. When a patient has two copies of the sickle cell gene, their red blood cells are not shaped normally and are instead shaped like a crescent or sickle.

WHAT HAPPENS AS A RESULT?

Due to their sickle shape, the red blood cells may stick together and block blood flow to the blood vessels of different organs, tissues, and bones.

Patients with sickle cell disease may experience serious medical complications, including repeated painful crises, strokes, infections, anemia, and blood clots.

Hackensack Meridian Children's Health

WHY SEEK CURATIVE TREATMENT FOR SICKLE CELL DISEASE?

Despite advances in medications and disease management, sickle cell remains a lifelong disease that is associated with a high risk of serious complications and decreased life expectancy.

- Patients may suffer organ damage to the lungs, heart, kidneys, eyes, and central nervous system.
- Patients may develop chronic pain due to recurrent pain crises.
- More than 50 percent of patients with sickle cell disease die before 45 years of age.

There are currently four medications approved for sickle cell disease that help improve symptoms. However, these medications do not prevent stroke or reduce the organ damage caused by the disease.

Bone marrow transplant and gene therapy are the only therapies available that may cure sickle cell disease.

WHY CHOOSE US FOR TREATMENT?

- We have a multidisciplinary team of specialists that provide a comprehensive and personalized approach to curative therapy.
- We are the only pediatric stem cell transplant and cellular therapy program in New Jersey. We perform an average of more than 30 pediatric transplants each year. We are a joint program with the Adult Stem Cell Transplantation and Cellular Therapy team at the John Theurer Cancer Center and are accredited by Foundation for the Accreditation for Cellular Therapy (FACT).
  - We have been performing bone marrow transplant for patients with sickle cell disease since 2002 and have transplanted more than 70 pediatric patients.
- We are active in national organizations focused on expanding research in bone marrow transplant for sickle cell disease, including the Sickle Cell Transplant, Advocacy and Research (STAR) Alliance and Blood & Marrow Transplant Clinical Trials Network (BMT-CTN).
- We are a Qualified Treatment Center for Lyfgenia, the FDA-approved gene therapy for sickle cell disease.
  - Our first pediatric patient received gene therapy for sickle cell disease in 2019 as part of the only clinical trial of its kind in New Jersey.
  - Our center has one of the largest groups of patients in the nation treated with gene therapy for sickle cell anemia on research studies.
  - We currently have multiple open clinical research trials for new gene therapies for sickle cell disease.

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CURATIVE TREATMENTS

BONE MARROW TRANSPLANT

How does bone marrow treatment work?

- Bone marrow transplant is the process of infusing stem cells from a healthy donor into a patient to replace bone marrow that is not working properly.
- A suitable donor is found by matching specific inherited markers on the surface of their cells to those same markers on the cells of the patient.
- To prepare for bone marrow transplant, patients receive chemotherapy to lower their immune system and make room in their bone marrow for new stem cells.
- The donor stem cells are then infused, travel to the bone marrow, and produce new red blood cells, white blood cells, and platelets.
- With a new bone marrow present, patients will not have red blood cells that sickle and thus are cured of their sickle cell disease.

PROS / CONS

Pros:

- Because bone marrow transplant offers a cure, patients will not experience any new complications from sickle cell, such as new pain crises.
- Bone marrow transplant for sickle cell disease started in the mid-1980s and has been practiced for more than 40 years in the United States. There have been many advancements during that time, which have decreased the risk of complications and improved outcomes following transplant.

Cons:

- Difficulty finding a fully or well-matched donor. It is estimated that less than 25 percent of patients with sickle cell disease will find a fully matched donor.
- Risk of short- and long-term side effects due to chemotherapy.
- Risk of graft rejection or failure. This could happen if donor stem cells do not succeed at making new white blood cells, red blood cells, or platelets so the patient still has sickle cell disease.
- Risk of graft-versus-host disease. This happens when donor stem cells attack different organ systems of the patient, like the skin, gastrointestinal tract, or liver.
- Patients are at an increased risk of infections after treatment and are unable to return to school or work for a few months.
- Patients will need to take multiple medications and follow up in the clinic for months after bone marrow transplant.

GENE THERAPY

How does gene therapy work?

- After receiving a medication to help mobilize their stem cells from their bone marrow into their bloodstream, stem cells are collected from patients via an IV or temporary line in their veins connected to an apheresis machine
- The stem cells are then sent to a special lab where the sickle cell gene is modified or changed.
- Patients are prepared before gene therapy by receiving chemotherapy that makes room in their bone marrow for new blood cells to form.
- The gene therapy is then infused and the gene-modified stem cells travel to the bone marrow and produce new white blood cells, red blood cells, and platelets.
- With a new bone marrow present, patients will be cured of their sickle cell disease.

PROS / CONS

Pros:

- There is no need to find a matched donor as the patient's own cells are collected and modified.
- There is no risk of graft-versus-host disease since there is no donor.
- Because gene therapy offers a cure, patients will not experience any new complications from sickle cell, such as new pain crises.

Cons:

- Risk of short and long-term side effects due to chemotherapy
  - The risk of infertility is higher due to the dose of chemotherapy used for gene therapy.
- The first research studies for gene therapy for sickle cell disease started in 2015. Because there has only been approximately eight years of follow-up, the potential for long-term complications is currently unknown.
- Patients are at an increased risk of infections after treatment and are unable to return to school or work for a few months.
- Patients will need to take preventative medications for the first few months following gene therapy. They will be monitored in our clinic periodically for a total of 15 years following gene therapy.

CELEBRATE THE CURE — PATIENT JOURNEYS

5-Year-Old Boy Cured From Sickle Cell Disease

FOR QUESTIONS OR TO SCHEDULE AN APPOINTMENT FOR EVALUATION

You can call the office of the Pediatric Blood and Marrow Transplantation and Cellular Therapy team at **551-996-5600** to schedule an initial consultation to learn more about curative therapy options for sickle cell disease.

To learn more

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