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Sickle Cell Disease (SCD), including Sickle Cell Anemia

Sickle cell disease (SCD), an umbrella group of hemoglobinopathies that includes sickle cell anemia, is an inherited disorder caused by an abnormal form of a protein called beta-globin. This can cause red blood cells to become sickle (crescent)-shaped and inflexible. Because of their abnormal shape, red blood cells have problems carrying oxygen and traveling through blood vessels. As a result, certain tissues in the child's body do not receive enough blood. This can cause serious problems, including severe pain, stroke, or bacterial infections. People with SCD may have pain in the hands, arms, legs, and other parts of the body; chest pain with breathing problems; nervous system problems, from minor ones to stroke; and an enlarged spleen.

SCD is typically detected through routine screening of newborns. When you bring your child to MSK Kids, we'll do a complete medical work-up to assess your child's health and the effects of SCD on his or her body, since symptoms tend to differ from person to person. Your child's treatment will depend on his or her needs. Treatment can reduce the risk of complications, extend lives, and in the case of stem cell transplantation, cure SCD. At MSK Kids, we offer SCD treatments that include:

Supportive care. We do all we can to monitor your child's health through frequent screening exams. Some children benefit from periodic blood transfusions, which temporarily lower the percentage of sickled red blood cells in the body and higher-than-normal iron levels. Medications are available to manage pain and keep your child comfortable.

Stem cell transplant. Also called a bone marrow transplant, this is the only known cure for SCD. We use it for children with severe SCD. Your child first receives chemotherapy to wipe out the diseased blood cells, and then receives healthy stem cells from a donor to rebuild the blood system. The best donor of healthy stem cells is a matched sibling who doesn't have SCD. But at MSK Kids, we have experience offering

cutting-edge stem cell transplants for patients with related and unrelated matched donors with variable levels of compatibility, and we've completed these with excellent results. If your child has severe SCD, your care team will connect you with a transplant doctor right away so you can learn the risks and benefits of transplantation and see if it is a treatment option for your child.

Gene therapy. This new treatment is very promising for children with severe SCD who cannot be helped by stem cell transplantation, including those without a compatible donor. Through a clinical trial, a child would have blood cells removed, modified in a laboratory, and returned to the body to re-establish a blood system with healthy red blood cells. We will let you know if MSK Kids is conducting such studies and if so, whether your child can participate.

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Beta Thalassemia (Thalassemia Major/Transfusion-dependent Thalassemia)

Beta thalassemia is an inherited blood disorder caused by a defect in the gene that helps control the production of hemoglobin, which is needed for red blood cells. As a result, people with beta thalassemia have fewer healthy red blood cells.

Beta thalassemia is typically detected through routine screening of newborns. Children with this condition develop anemia and may have symptoms such as pale skin and fatigue. They may also have weakness and enlargement of organs such as the heart, spleen, and liver. When you bring your child to MSK Kids, we'll do a complete medical work-up to assess your child's health.

Some children with very serious beta thalassemia can be cured with a bone marrow transplant, also called a stem cell transplant. Your child first receives chemotherapy to wipe out the diseased blood cells, and then receives healthy stem cells from a healthy matched donor (ideally a sibling) or umbilical cord blood to rebuild the blood system. At MSK Kids, we have completed cutting-edge stem cell transplants for patients with beta thalassemia with excellent results. If your child has beta thalassemia, your care team will connect you with a transplant doctor right away so you can discuss the risks and benefits.

After a transplant, children might need follow-up treatments to reduce excess iron in their blood. This can decrease the symptoms and complications of thalassemia.

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Other Hemoglobinopathies

MSK Kids cares for children with two other rare hemoglobinopathies:

Congenital sideroblastic anemia

Congenital dyserythropoietic anemia

Both disorders result in low levels of functioning red blood cells and often high levels of iron in the body. People with these hemoglobinopathies may experience fatigue, weakness, pale skin, and other complications. MSK Kids offers treatments such as supportive care and stem cell transplantation that are tailored to each child"s needs.

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New Patient Appointments

Call 833-MSK-KIDS Available Monday through Friday, 9 a.m. to 5:30 p.m. (Eastern time)

Make an Appointment

PREVIOUS

Fanconi Anemia and Other Inherited Bone Marrow Failure Syndromes (BMFS)

NEXT

Primary Immunodeficiencies (PIDs), Primary Immune Regulatory Disorders (PIRDs) and White Blood Cell Disorders