

## Phlebotomy as a treatment for hemoglobin SC disease (HbSC)

Every year, 300,000 babies are born with sickle cell disease worldwide and there are over 30 million people with sickle cell disease in the middle and low income countries. At Erasmus MC, there are about 600 sickle cell patients under treatment in the Center for Rare Diseases.

Sickle cell disease (SCD) is a severe hereditary disorder with various complications. Patients with sickle cell disease have chronic anemia and severe pain attacks, also called sickle cell crises. The disease can damage organs. As a result, the life expectancy of patients in the Western world is 20-30 years shorter than that of the general population. In low-income countries, where there is no proper care, 75% of patients die before they reach the age of five. Current treatment options are symptom-fighting drugs (antibiotics, folic acid, hydroxycarbamide), blood transfusions and stem cell transplantation, if a suitable donor can be found.

### Personalized treatment

Sickle cell anemia (SCA) is the most common and severe genotype, while hemoglobin SC disease (HbSC) is another genotype with different characteristics. Currently, all SCD patients are treated the same regardless of their specific type. There is no specific treatment for HbSC available. Even though new therapies are being developed for sickle cell anemia it's uncertain if these therapies will benefit individuals with HbSC or will be available for these patients (such as gene therapy or stem cell transplantation).

### Study treatment of complications

This study aims to assess the effectiveness of phlebotomy (drawing blood) in treating HbSC. Phlebotomy is known to reduce blood viscosity (thickness of the blood) and induce iron deficiency, which can be beneficial for HbSC. The study involves 60 adults with HbSC, who will either receive regular phlebotomy or standard care (no specific treatment). The main goal is to see if phlebotomy reduces the occurrence of complications like painful episodes, acute chest syndrome (a severe lung condition), irreversible damage to the bones, or irreversible damage to the eyes.

Participants will undergo regular phlebotomies until their hemoglobin levels reach a certain target. They'll also have eye exams, MRI scans, and other tests to monitor their red blood cells and thickness of the blood. Phlebotomy is generally considered safe, with low risks. If this study shows positive results, it could lead to implementing phlebotomy as a treatment for HbSC, that can be applied globally, especially in low-income countries, such as African countries, where HbSC is prevalent. Phlebotomy is a relatively inexpensive therapy that can be easily administered in such regions.

