Sickle Cell Disease

For SCD warriors like Porscha Burks, VBRI research represents a hopeful path forward and a new standard of care.

Porscha's Story

Porscha Burks was diagnosed with sickle cell disease (SCD) at 4 years old. For more than 20 years, Porscha has lived with constant, chronic pain typical of SCD. By early adulthood the pain was unmanageable, and prevented Porscha from living a normal life.

Porscha manages her SCD with monthly rounds of red blood cell exchange. The exchange is a time-locked, life-long process, requiring Porscha to remain in constant close proximity to a medical center. SCD is progressive, with risks of serious complications worsening over time. Today, with over two decades of SCD treatment behind her, Porscha lives with an increased risk of acute chest syndrome, a pneumonia-like illness which is the leading cause of hospitalization and death in SCD patients, while still experiencing painful symptoms of SCD.

Porscha is one of more than 8 million hoping that researchers will discover a cure for SCD.

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> *I definitely thought I was headed in a direction where I might not make it, and my sickle cell disease was going to be fatal.*
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Porscha Burks
Sickle Cell Disease Patient

The Search for a Cure for a Devastating Disease

SCD is a devastating disease that affects over 100,000 people in the US per year, and millions worldwide. An incredibly severe and painful disease, it affects all aspects of patients' lives from mental and physical health to fertility, and often their overall ability to live and enjoy a normal life.

SCD disproportionately affects people of color – with more than 90% of cases presenting in Black or African American people. By targeting a group historically left behind by the US medical system, SCD facilitates a culture of stigma, creating an additional barrier for treatment.

Delivering Hope for Sickle Cell Disease Patients

At VBRI, **we are developing treatments for sickle cell disease that can impact patients now** by increasing their quality of life, decreasing the amount of time they spend in the hospital, and reducing the amount of pain medication needed.

The Search for Treatments That Can Have an Impact Now

Currently, the only cure for SCD is a bone marrow transplant, but the majority of patients will never find a match. Matchless patients are sentenced to a lifetime of red blood cell therapy, an expensive inpatient treatment the timing of which often leads to lost days of work and a decreased quality of life.

“We are trying to understand how we can improve quality of life, reduce the time spent in hospital, and overall improve the lives of sickle cell patients.”

Prithu Sundd, PhD
Senior Investigator

The Race Against Pneumonia

Prithu Sundd, PhD is working to find new treatments for SCD patients, focused on preventing the most severe forms of pneumonia from developing. Dr. Sundd’s research helped uncover the mode of action and limitations of P-selectin antibody therapy — the first targeted therapy developed in recent years to prevent hospitalization of SCD patients. Despite early promise, Dr. Sundd’s team demonstrated that P-selectin therapy provides partial protection and requires a long hospital stay with each treatment. In practice, this meant a high-cost for patients receiving treatment and time away from friends and family.

In response to findings from P-selectin antibody therapy research, Dr. Sundd’s team is pursuing new directions to develop therapies that work for all patients, and that can be developed more quickly, cheaply, and easily. Dr. Sundd and his team are pioneers in precision medicine – an approach that uses a range of powerful scientific tools: genomic research, patient biobanks, which are huge databases that can be mined for patterns, and custom-built mouse models with human genes that mimic the disease. **The team collaborates directly with patients to bring understanding to the research**, using blood samples to understand which factors cause a patient to go into crisis or experience pain. They are also working to understand why only a subset of SCD patients develop lung thrombosis. Their hope: to use this personalized information to design new therapies and treatments.

Turning Back The Clock

Philip Doerfler, PhD joined VBRI in August 2024, drawn to the community in the Milwaukee area. Working in such close proximity to patients with SCD has allowed his lab’s work to consider the whole lifetime of the patient to understand the disease.

His lab studies why SCD develops as people reach adulthood. Sickle cell disease happens when the body makes sickle hemoglobin in red blood cells. A type of hemoglobin called fetal hemoglobin can help prevent this, but its levels naturally decrease after birth, allowing symptoms to develop. Dr. Doerfler’s team is focused on the genetic switch that controls how much fetal hemoglobin is made, and whether treatments can be developed to turn this switch back on in red blood cells, boosting fetal hemoglobin to help reduce the effects of SCD. These treatments promise a new hope for patients who do not find a match with a bone marrow donor. Dr. Doerfler’s team is not only developing the gene therapy, but also working to make it as safe as possible and understand how it affects patients on an individual level.

“It takes specialized facilities and expertise to do this. There aren’t many places that are like VBRI.”

Philip Doerfler, PhD
Associate Investigator

Unparalleled Expertise Fueled by Compassion

VBRI investigators are among the world leaders in innovative science that promises to shape the future of sickle cell disease treatment. Our approach is driven by a unique understanding of what our patients are going through, and what hope looks like for them. **Together, we research cures**.