Sickle cell disease

Sickle Cell Disease (SCD) is an inherited blood disorder that affects almost eight million[1] people worldwide, causing tremendous suffering. It's a life-threatening condition with chronic manifestations, including acute painful episodes, anemia and organ damage. The greatest burden of SCD lies in Africa where SCD complications lead to 500 child deaths per day[2].

For more than 50 years, Novartis has been committed to the fight against sickle cell disease. Novartis first supported SCD care with the development of chelators for the management of iron overload, initially injectable in 1968, and then oral in 2005.

Treatment options have increased since then, but access to these treatments has not kept pace.

Progress in high-income countries like the United States has shown that with newborn screening and comprehensive disease management, mortality in children with SCD under five years old has been reduced by 94%[3]. In stark contrast, it is estimated that 50-90% of infants born in Africa with SCD will die in childhood[4]. This highlights the health equity considerations that are needed to adequately address this crippling disease.

What is health equity? We've learned that the key to achieving health for all is in forging multisector partnerships.

Partnering to overcome obstacles to healthcare

To help bridge disease management and outcome disparities between Africa and the rest of the world, Novartis established the Novartis Africa Sickle Cell Disease program in 2019.

The program is a multisectoral collaboration to jointly address barriers and provide comprehensive end-to-end solutions, right from screening and diagnosis, through to treatment, capacity building and overall disease management. With these elements in place, a stronger foundation for bringing next-generation treatments to Africa can be created.

The Novartis Africa Sickle Cell Disease program was launched in Ghana in 2019 and now also includes Angola, Kenya, Tanzania, Uganda and Zambia.

Highlights of the program include:

- Lack of access to newborn screening is thought to be a primary driver of mortality for children in Africa with SCD[5]. We are working with partners including the American Society of Hematology's CONSA program to make screening and diagnosis more available and affordable.
- Expanding access to a generic medicine that has been shown to improve health outcomes of people living with SCD, as well as introducing a child-friendly formulation (currently launched in Ghana with plans to expand across Africa).
- Helping to strengthen health care systems to provide comprehensive management of SCD, including early interventions and the safe administration of existing and innovative treatments. For example, more than 100 SCD clinics in Ghana and Uganda have been trained to provide SCD care through our Ahodwo program.

- Novartis comprehensive approach to SCD disease management also includes a portfolio of digital initiatives. For example, more than 90,000 babies have been registered in an app Novartis supported the Sickle Cell Foundation of Ghana to develop and roll out that facilitates collection and management of data in the country's national newborn screening program. The app is being expanded to additional countries in Africa through our partnership with the American Society of Hematology.
- A research collaboration between the Hospital for Sick Children (Sick-Kids), the Navrongo Health Research Center, Hemex Health and Novartis evaluates the feasibility of integrating point-of-care SCD testing into primary care centers in Kassena-Nankana Districts in the Upper East Region of Ghana. 6,000 babies have been screened in the first year (already reaching the target set for the first 24 months' screening period) and 65 patients referred into comprehensive SCD care for early intervention strategies and long-term follow-up. A telehealth partnership with Vula Mobile supports the health workers to consult specialists elsewhere; as well as the patients and caregivers facing transport obstacles to reach the sickle cell clinic.
- Partnering with governments to ensure program sustainability and to support policy changes that lead to long-term improvements in healthcare for patients with SCD.

A blueprint for future global health: We've learned partnering across private and public sectors can help us navigate the complexities of accessing healthcare.

Partnering for renewed commitment to a neglected disease

In response to a call for a renewed commitment to tackling the rising toll of SCD from African health ministers and the WHO Africa Regional Office, Novartis Global Health joined forces with leaders in SCD to launch the World Coalition on Sickle Cell Disease.

Through the Coalition, we aim to decrease childhood mortality associated with SCD and improve the quality of life of people with the disease in low- and middle-income countries. It is our hope that the Coalition will provide a unique platform for elevating SCD and enhancing coordinated action on a global scale. As we look to the United Nations' prioritization of ensuring Universal Health Coverage, SCD is an important reminder of the work ahead to ensure no patient is left behind.

World Coalition on Sickle Cell Disease: multi-sector partnership to holistically address this largely forgotten injustice.

Gene therapy, hope for a cure

In 2021, Novartis entered into a grant agreement with the Bill & Melinda Gates Foundation with the shared goal to discover and develop a potential single-administration *in vivo* gene therapy for SCD that is accessible in resource-strained countries where SCD is most prevalent.

The Novartis-Gates Foundation collaboration envisions an *in vivo* gene therapy administered directly to patients, where the goal would be to mitigate the need for extended hospital stays or specialized infrastructures.

This contrasts with complex procedures associated with *ex vivo* gene therapies that require cells to be extracted from the body and are individually manufactured before treatment. *Ex vivo* gene therapies require long hospital stays and advanced laboratory and hospital infrastructure, which limit their application in places where resources are constrained.

Contributing to this program for an accessible *in vivo* gene therapy for SCD and other inherited hemoglobinopathies, Novartis recently entered into a collaborative agreement with Precision BioSciences to design a protein that can cut DNA at a specific site, allowing for the insertion of a functional gene that can act in place of the mutated gene that causes red blood cells to become sickle-shaped.

Partners

- American Society of Hematology
- Global Alliance of Sickle Cell Disease Organizations
- World Coalition on Sickle Cell Disease
- Sickle Cell Foundation Ghana
- Children Sickle Cell Foundation of Kenya
- Global Sickle Cell Disease Network
- Sick Kids
- Hemex Health
- MedShr
- Revvity
- Vula Mobile

News

- Bridging barriers to healthcare: A blueprint for future global health – watch the documentary
- Bridging the Access Gap for Comprehensive Sickle Cell Disease Management Across Sub-Saharan Africa: Learnings for Other Global Health Interventions? – Annals of Global Health
- Access to essential therapy for sickle cell disease in Africa: Experience from a national program in Ghana – ScienceDirect article
- World Coalition on SCD launches, sparking global focus on SCD diagnosis and care – American Society of Hematology publication
- Evaluation of treatment patterns, healthcare resource utilization and cost of illness for sickle cell disease in Ghana – BMC Health Services Research article
- Sickle cell screening urged for newborns in Africa
- Novartis and the Bill & Melinda Gates Foundation collaborate to discover and develop an accessible in vivo gene therapy for sickle cell disease
- Precision BioSciences Announces In Vivo Gene Editing Collaboration with Novartis to Develop Potentially Curative Treatment for Disorders Including Sickle Cell Disease
- Novartis announces partnership with the American Society of Hematology to fight sickle cell disease in Sub-Saharan Africa
- PerkinElmer and Novartis Collaborate to Address the Unmet Need of Sickle Cell Disease in Sub-Saharan Africa
- World Coalition on SCD launches, sparking global focus on SCD diagnosis and care
- Government of Ghana Launches First of Its Kind Public-Private Partnership With Novartis to Improve Diagnosis and Treatment of People With Sickle Cell Disease
- Government of Ghana Makes Hydroxyurea Available to People With Sickle Cell Disease Through First of Its Kind Public-private Partnership With Global Medicines Company Novartis
- Novartis expands Africa Sickle Cell Disease program to Uganda and Tanzania
- Bringing innovation to sickle cell disease patients in sub-Saharan Africa

- Breaking down barriers for patients with sickle cell disease
- Improving the lives of people with sickle cell disease in Ghana

Resources

Download the Sickle Cell Disease Factsheet

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2. The Lancet: Sickle cell disease: a new era
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