



# Sickle cell disease

## Reimagining the management of sickle cell disease in Africa

Sickle cell disease is a hereditary and life-threatening condition that causes ongoing vascular damage and repeated injury to blood vessels and organs<sup>1,2</sup>. This lifelong illness often takes an extreme emotional, physical, and financial toll on patients and their families<sup>3,4</sup>.

The World Health Organization (WHO) recognizes sickle cell disease (SCD) as a public health priority and a neglected health problem in sub-Saharan Africa. However, it is still largely absent from the global health agenda.

~80%

**OF PEOPLE WITH SCD**  
live in sub-Saharan Africa<sup>5</sup>

~1 000

**CHILDREN IN AFRICA**  
are born with SCD every day<sup>6</sup>

>50%

**OF CHILDREN WITH SCD**  
in Africa die before their fifth birthday  
due to preventable complications<sup>6</sup>

~14 million

**NEWBORNS GLOBALLY**  
will be affected by SCD  
between 2010 and 2050<sup>7</sup>

For more than 40 years, Novartis has been committed to the fight against sickle cell disease. Through research, access programs and educational resources, we support patients, healthcare providers and caregivers.

SCD is a global health problem, yet sub-Saharan Africa bears the highest burden of disease. There is a clear disparity between Africa and other parts of the world, where the condition is managed as a chronic disease.

### **Building a holistic approach for sickle cell disease**

SCD management requires an integrated, holistic approach. Early diagnosis through newborn screening is the first step, but currently only an estimated 4% of children get tested. The second step is to link diagnosis with effective treatment, yet access to medicine is a challenge for patients in lower-income countries.

To achieve long-term gains, it is also important to strengthen health systems to better manage SCD, including through data collection and analysis, impact and outcomes monitoring, and scientific research, training and collaboration.

### **Launching the Novartis Africa Sickle Cell Disease program**

Against this background, Novartis launched a first-of-its-kind effort to manage the disease holistically in Africa. First launched in Ghana in 2019, the Novartis Africa SCD program is now expanding to Uganda, Tanzania and Kenya. Novartis plans to roll the program out to a total of 10 African countries by 2022.

The program consists of public-private partnerships between Novartis, ministries of health, patient groups and nongovernmental organizations to improve outcomes for SCD patients. The focus is on making diagnosis and treatment available, accessible and affordable for patients and their families; promoting scientific research, training and education; and pursuing robust monitoring and evaluation of the program.

### **Making an impact for patients in Ghana**

With the launch of the Novartis Africa SCD program, Ghana became the first country in Africa to offer hydroxyurea, the global standard of care for people with SCD. More than 3 400 patients are currently being treated with hydroxyurea in 11 treatment centers

across Ghana that have been trained on using the drug. Discussions are also underway to include the medicine and associated laboratory testing in the national health insurance scheme. Further, Novartis and the Sickle Cell Foundation of Ghana are rolling out a clinical management app to help ensure hydroxyurea is administered safely and that patients are gaining the maximum benefit from the treatment.

Novartis partner Hemex Health is also working with the Ghana Food and Drug Administration to launch an affordable point of care diagnostic to help improve access to SCD screening.

### **Researching next-generation treatments**

In addition to Ghana, hydroxyurea is now also registered as a treatment for SCD in Uganda, Tanzania and Kenya. While expanding access to hydroxyurea, we are also investing in research for new treatment options to address unmet medical needs. SCD is the single most important genetic cause of childhood mortality globally, and Novartis is currently developing a child-friendly formulation of hydroxyurea that would facilitate administration for children unable to swallow capsules.

Further, we plan to conduct two clinical trials in Ghana and Kenya for our next-generation treatment for SCD. This will be the first time a biologic therapy, which is not a vaccine, enters multicenter clinical trials in sub-Saharan Africa (excluding South Africa). This is an important step in bringing this innovation, once approved, closer to people with SCD in sub-Saharan Africa.

### **References**

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