Novartis has long been committed to understanding and developing treatments for hemoglobinopathies, a group of inherited blood disorders that includes sickle cell disease (SCD). SCD is a life-threatening condition with chronic debilitating manifestations, including acute painful episodes, anemia, organ damage, chronic pain, and fatigue. It is regarded by the World Health Organization (WHO) as a public health priority and a neglected health problem in sub-Saharan Africa, where 80% of babies with SCD globally are born each year. Today, Novartis is a leader in addressing critical unmet needs in SCD. We are continually seeking new approaches and strategic partnerships to help make diagnosis and treatment more accessible and affordable to patients in countries where SCD remains a challenge for health care systems.

Building a solid, sustainable foundation

SCD is a global health problem, yet sub-Saharan Africa (SSA) bears the highest burden of disease. 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. The program is implemented through public-private partnerships with local governments, as well as collaborations with universities, patient groups, professional societies and other organizations. A key element of our strategy is a comprehensive approach to disease management that encompasses early intervention strategies, such as screening and diagnosis; research to investigate new treatments; and education and advocacy to improve access to existing therapies.

The program was launched in Ghana in 2019 and expanded to Angola, Kenya, Tanzania, Uganda and Zambia, with further plans to reach at least 10 countries in SSA.

Highlights of the program include:

- Working with partners to make diagnosis more available, accessible and affordable— currently, lack of...
access to newborn screening, and thus delayed diagnosis, is thought to be a primary driver of mortality for children in Africa with SCD (it is estimated that 50-90% of infants born in Africa with SCD will die before their fifth birthday)²

- Expanding access to hydroxyurea (a generic medicine that has been shown to improve health outcomes of individuals living with SCD), as well as making available a child-friendly formulation (currently approved in Ghana with submissions ongoing in other countries in SSA)
- Helping to strengthen health care systems to provide comprehensive management of SCD, including early interventions and the safe administration of existing and innovative treatments
- Partnering with governments to ensure program sustainability and to support policy changes that lead to long-term improvements in healthcare for patients with SCD

Download the sickle cell disease fact sheet

Gifty, a sickle cell patient from Ghana

Enabling access to next-generation therapies

Novartis is also working to bring innovative therapies to sickle cell patients in sub-Saharan Africa. For example, Korle Bu Teaching Hospital in Accra, Ghana, is one of the sites participating in an ongoing Phase III trial to assess the efficacy of our next-generation biologic therapy³. Until the medicine is commercially available, eligible patients can be enrolled in a Managed Access Program to receive the medicine.

To help ensure medicines are adopted and used successfully, we are working with local partners to strengthen healthcare system capabilities. For example, we support programs to train healthcare professionals and are establishing coordinated SCD clinics and centers of excellence. To date, more than 20 SCD clinics in Ghana have been trained to provide SCD care through the Ahodwo program.

Leveraging technology with the goal of improving outcomes and helping to optimize safety

Our comprehensive approach to SCD disease management includes a portfolio of digital initiatives, including information-, advocacy-, and support-oriented websites tailored to country needs, as well as mobile solutions to better serve the global sickle cell community.

For example, as part of the Africa Sickle Cell Disease Program, Novartis supported the Sickle Cell Foundation of Ghana to develop and roll out a digital phone-based application (App) to facilitate collection and management of data in the country’s national newborn screening program. To date, more than 25,000 babies have been registered in the App, which is being expanded to additional countries in Africa. In addition, the partners have developed a clinical management App to help ensure safe and effective use of hydroxyurea. A third App is being tested to provide healthcare professionals with ready access to a digital version of the hydroxyurea prescribing guidelines on their phones.

Addressing understanding: Making education a priority

There’s a world of myths and misconceptions about SCD. Many of them are harmful and create barriers to early diagnosis and treatment.

In 2019, Novartis conducted the Sickle Cell World Assessment Survey (SWAY), one of the largest global
surveys in sickle cell disease, which provided valuable information about the impact of the disease on patients and their families and caregivers worldwide. Insights were collected from more than 2100 patients and 360 health care providers to help inform the management of this lifelong condition. This included more than 500 patients and more than 40 healthcare professionals from Ghana and Nigeria. Here are five things we learned.

We have long sponsored educational initiatives to bridge the voices of those with SCD with the world at large. One is example is notaloneinsicklecell.com, a website available in 15 different languages featuring real stories from patients with SCD from around the globe.

In addition, we work with partners to develop educational content and tools that help inform patients and healthcare providers about the pathophysiology and management of SCD. For example, together with the Global Alliance for Sickle Cell Disease Organizations (GASCDO), we are conducting a series of virtual dialogues where healthcare professionals, patients and other key stakeholders in sub-Saharan Africa (SSA) engage in proactive conversations about SCD and the difficulties patients face in navigating their disease, including topics such as screening and stigma. In addition, in collaboration with the Sickle Cell Disease Association of America (SCDAA), we conducted educational workshops to strengthen the capacity and skills of patient associations across SSA.

**Gene therapy: Hope for a cure**

While the genetic cause of SCD has been known for decades, only recently has medical science gained the tools to potentially mitigate the biological effects of the errant gene that causes the disease. We are pursuing an ex vivo hematopoietic stem cell program for SCD, using CRISPR gene editing technology licensed from Intellia Therapeutics, and in 2021 we initiated patient dosing.

We also entered into a grant agreement with the Bill & Melinda Gates Foundation with the shared goal to discover and develop a novel in vivo gene therapy to cure the clinical manifestations of SCD.

Existing clinical-stage gene-based therapies involve removing cells from a patient, modifying those cells in a laboratory, and then reintroducing them to the patient through a procedure similar to a bone marrow transplant. If approved, this complex procedure may be challenging to introduce in many parts of sub-Saharan
Africa due to current limitations of local healthcare systems. 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.

As part of the early drug design strategy, Novartis will prioritize addressing access and distribution hurdles posed by limited healthcare infrastructure in low- and middle-income countries.

At Novartis, we remain committed to providing hope and improving outcomes for anyone affected by SCD. There is more to be done. That is why we continue to join forces with partners from around the world to help bring sickle cell disease out of the shadows.

Partners

Novartis partner, Hemex Health, develops point of care diagnostic

Watch Video

News

- Sickle cell screening urged for newborns in Africa
- Bringing innovation to sickle cell disease patients in sub-Saharan Africa
- Novartis expands Africa SCD program to East Africa
- Government of Ghana makes hydroxyurea available to people with sickle cell disease through the first of its kind public-private partnership with global medicines company Novartis
- Improving the lives of people with sickle cell disease in Ghana
- Government of Ghana launches first of its kind public-private partnership with Novartis to improve diagnosis and treatment of people with sickle cell disease
- Novartis announces partnership with the American Society of Hematology to fight sickle cell disease in Sub-Saharan Africa | Novartis
- PerkinElmer and Novartis Collaborate to Address the Unmet Need of Sickle Cell Disease in Sub-Saharan Africa
- Breaking down barriers for patients with sickle cell disease

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3. www.clinicaltrials.gov, accesses last June 2021

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