# Novartis Hematology: Working to help ensure patients are getting the access they need

Developing treatments for blood cancers and serious blood disorders is important, but it is only part of the equation. We also need to help ensure those who may need our therapies can get them.

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Novartis is striving to tackle barriers to access and promote health equity in hematology through our three key access principles: research and development, affordability, and the strengthening of health care systems.

## Research & development in hematology

As a global medicines company, we aim to more quickly deliver potentially life-saving treatments to people who need them—regardless of where they are.

Continuously upgrading our technology and processes is an important part of delivering our medications to more people, with speed and convenience. With innovations like T-Charge™, a novel, Novartis-developed next generation CAR-T manufacturing platform that will be the foundation for various new investigational pipeline CAR-T cell therapies, we strive to bring CAR-T to more people in need. This investigational technology could potentially reduce the wait time between the collection of patients' T-cells and their infusion with the therapy and may help reduce burdens on the health care system. Our goals with T-Charge are to enable rapid cell manufacturing process time compared with traditional CAR-T; reliable delivery through a simplified process and streamlined quality control; and potentially offer flexibility in the setting of care, so people may be treated in both the inpatient setting and outpatient/community setting.

Our pursuit of innovation in cell and gene therapies, such as in-vivo gene therapies and CAR-T cell therapies,

may help to develop therapeutic options that aim to treat, prevent, or potentially cure blood cancers and series blood disorders, including initiatives in sickle-cell disease (SCD) and certain blood cancers.

Meanwhile, in paroxysmal nocturnal hemoglobinuria (PNH), a rare, but chronic blood disorder, we're exploring treatment alternatives to hospital procedures. With potential new approaches to treat hematological disorders like this, we may help alleviate the burden on local hospitals and treatment centers that are often required to facilitate care for patients—which in turn, could free up resources to address other health care needs.

## Affordability and opening more avenues to care with global partnerships

Investigating and developing new medicines is part of our heritage. As we bring new therapies to the market, we also aim to be part of the solution to help deliver them to people in need.

In addition to pricing our new medicines based on the value they deliver to individuals, health care systems, and society, we also work to make our medicines available through unique strategies to broaden access for people in lower- and middle-income countries (LMICs).

Through CancerPath to Care, an innovative, access initiative in partnership with The Max Foundation, people living with chronic myeloid leukemia, breast cancer, and certain rare cancers can be connected with effective treatments, professional medical capabilities, trained physicians, and hands-on support. Our partnership with The Max Foundation began over two decades ago with a focus on chronic myeloid leukemia (CML) and other rare cancers, has impacted thousands of patients since its initiation. Through our ongoing support for this program, we aim to provide access to care to ~36,000 patients in over 70 LMICs by 2025.

The Access to Oncology Medicines (ATOM) Coalition was launched by Union for International Cancer Control (UICC) and 30+ partners with a goal of improving access to essential cancer medicines in LMICs. Novartis has become the first pharmaceutical company to contribute an innovative treatment to ATOM. We're sharing the intellectual property of how this treatment is made with certain manufacturers who will independently manufacture and sell generic, lower-cost versions of the medicine in certain ATOM Coalition countries. Along with medicine, other coalition partners will contribute improvements in diagnosing cancer, the supply chain and beyond. The expectation is that these contributions will provide a new model—and a first step—for the pharmaceutical sector to help close the gaps in access to life-changing medicines for people in need.

## Helping to strengthen health care systems across the world

Health care systems are integral to creating thriving communities across the globe. That's why Novartis prioritizes efforts that strengthen health care systems—the people, institutions, organizations, and resources that manage the health of people—allowing them to effectively and efficiently provide care to the communities they serve. To do this, we identify barriers in the health system that prevent people from accessing needed treatment and work to systemically lower such barriers.

To help address the burden of SCD in sub-Saharan Africa, Novartis created the Novartis Africa Sickle Cell Disease Program. This program is an end-to-end effort that includes screening and diagnosis; research to investigate new treatments; and education and advocacy to improve access to existing therapies. It is implemented through public-private partnerships with local governments, as well as collaborations with universities, patient groups, professional societies and other organizations. It was launched in Ghana in 2019 and has expanded to Angola, Kenya, Tanzania, Uganda, and Zambia.

As part of the initiative, Novartis is partnering with governments to ensure program sustainability and to support policy changes that lead to long-term improvements in health care for patients with SCD. These efforts will help prepare health care systems for future innovation in the treatment of SCD. A new partnership with the American Society of Hematology will provide standard-of-care practices for screening and early intervention therapies at participating institutions in seven sub-Saharan African countries, while a further collaboration with PerkinElmer aims to accelerate access to SCD screening and treatment.

We are also 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 sub-Saharan Africa).

While we strive to open more avenues to make our therapies available, broadening awareness and education efforts around the treatments available is also key to ensuring patients and caregivers can make informed decisions about the best treatment options for them.

With programs like CAR-T Revelations, developed in collaboration with The Leukemia & Lymphoma Society (LLS), we asked real patients to share their experience with CAR-T cell therapy and shed light on important—and sometimes lesser-known—aspects of the CAR-T lymphoma treatment journey. People living with SCD shared their personal challenges in the "S word" webinar series. Developed in conjunction with advocacy group, Global Alliance of Sickle Cell Disease Organizations (GASCDO), myths were debunked, inspirational stories were shared, and the keys to a wholistic approach to tackling the disease were discussed. By connecting people to stories that may resonate with them, we aim to make education regarding serious blood disorders not only informational and accessible, but also relatable. It is our goal that this can help patients to feel empowered in their care.

## Additional resources

Read more about our dedicated work and programs in hematology.

CAR-T Cell Therapy

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