Novartis Hematology: Relentless Pursuit of a Cure

Novartis is committed to transforming the lives of people living with blood cancers and serious blood disorders.
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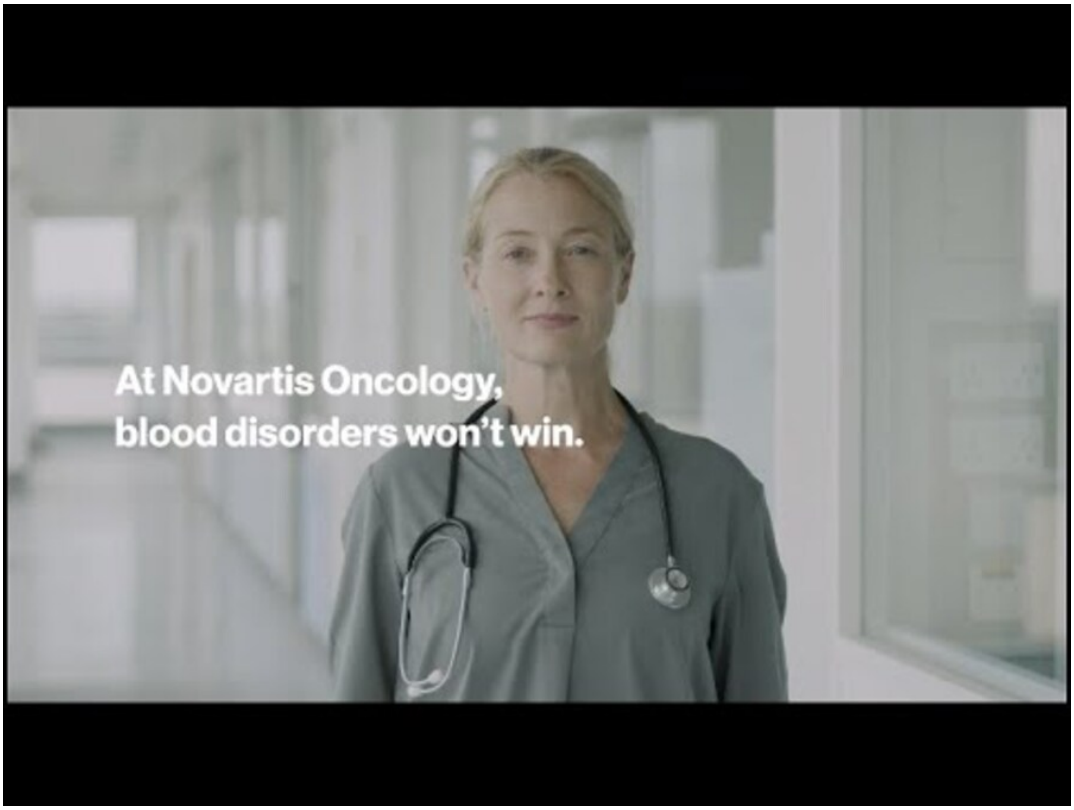

VIDEO

Novartis is deeply committed to transforming the lives of people living with blood cancers and life-threatening blood disorders. We believe that anyone living with these conditions has the right to a life free from pain, free from symptoms, and free from disease – this is our vision for the future.

Reimagining Myeloid Disorders

Novartis – together with its research partners – led the era of targeted therapies in cancer and, in investigating how these medicines could transform patient outcomes, helped chronic myeloid leukemia (CML) to become a chronic, versus a life-limiting, condition for many patients by increasing life expectancy near that of the general population. We have continued to revolutionize oncology research within CML by exploring molecules to address unmet needs for patients who face treatment resistance or intolerance to currently available therapies.

With our extensive experience in Janus kinase (JAK) inhibition, which has set the standard of care in myeloproliferative neoplasms (MPN), including myelofibrosis (MF) and polycythemia vera (PV), we are now breaking new ground in the treatment of graft-versus-host disease (GvHD) through the largest phase III program in this indication.

Novartis is committed to demonstrating its leadership in MPN care and improving the lives of patients with, and beyond, JAK inhibition by investigating multiple novel compounds in patients with MF. Working with hematologists and cardiologists, Novartis is also changing the treatment paradigm of PV, gaining momentum to improve the recognition and treatment of cardiovascular disease in these patients and help establish new risk stratification. By supporting novel projects, such as machine-learning approaches and investigator-initiated ground-breaking studies, Novartis hopes to contribute strongly to better the treatment of PV.

Separately, in myelodysplastic syndromes (MDS) and acute myeloid leukemia (AML), we are researching ways to target the dysfunctional immune system and leukemic stem cell proliferation, and are investigating an immuno-myeloid therapeutic approach that may have the potential to deliver durable responses beyond current treatments alone.

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Reimagining Nonmalignant (Benign) Hematology

More than 50 years ago, Novartis began to break down barriers in serious nonmalignant (or benign) blood disorders like sickle cell disease (SCD) and thalassemia, with the world's first treatment for chronic iron overload.

Over time, our understanding of chronic conditions like SCD, immune thrombocytopenia (ITP), and severe aplastic anemia (SAA) have improved considerably. Now, innovative Novartis medicines are options for thousands of patients with these serious nonmalignant blood disorders.

For inherited conditions like SCD, gene therapy may one day become a curative option. Novartis is at the leading edge, recently announcing a collaboration with the Bill & Melinda Gates Foundation to research an in vivo gene therapy for SCD with a focus on access in sub-Saharan Africa (SSA). Novartis is also researching a treatment for SCD with CRISPR technology licensed from Intellia Therapeutics.

Scientist at work

Reimagining our Responsibility to Patients

Novartis has enabled over 90,000 people in low- and middle-income countries (LMIC) to receive treatment for CML since 2002 through our GIPAP program. In 2017, GIPAP transitioned to the CMLPath to Care program, which has enabled access to CML treatment at no cost in over 80 countries.

In SCD, Novartis is continuing efforts on a child-friendly formulation of hydroxyurea and announced plans to conduct two clinical trials in Ghana and Kenya for SCD treatment. We have also started enrollment of clinical trials in SSA for a potentially cutting-edge biologic medicine for individuals living with SCD.

Novartis has conducted three major surveys (LANDMARK, I-WISh, SWAY) of nearly 5,000 people living with blood disorders in 26 countries around the world to help give a voice to the community and ensure their unique needs are not going unnoticed.

Reimagining our Responsibility to Society

Since 2007, Novartis has been a part of EUTOS, a collaboration first with the European Leukemia Net and now with Jena University, to improve the understanding of CML, promote best practice, and enhance treatment outcomes.

Novartis is also a part of the HARMONY Alliance, a European public-private partnership for Big Data in Hematology. We made the first transfer of data to the HARMONY Big Data Platform – the RATIFY trial – helping to better define clinical endpoints and outcomes for AML, and will continue contributing with data on other disease areas, like CML and MPN, as part of HARMONY PLUS.

In strong collaboration with academic partners throughout the world, Novartis has joined multiple projects to generate real-world evidence in MPN, including the ERNEST registry of MF patients.

Through the Novartis Africa Sickle Cell Disease program, Novartis has been focusing its efforts on establishing public-private partnerships to improve the lives of people with SCD in SSA. Specifically, we are focused on developing a comprehensive approach to screening and diagnosis; treatment and disease management; training and education; and elevating basic and clinical research capabilities. These efforts are intended to help prepare health care systems for future innovations in the treatment of SCD.

Additional resources

Novartis is a pioneer in the field of cell and gene medicine. To learn more about a one-time treatment, manufactured individually for each patient, see below:

CAR-T Cell Therapy and Beyond | Novartis
New frontiers of medicine – Cell and Gene Therapies
The Process of CAR-T Cell Therapy

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