Novartis
Hematology in Europe

Proud of our Bloodline!

We Care. Together we cure.
We never stop.

NOVARTIS
“I barely even knew what cancer meant, what leukemia was, how it affected my blood, what it did to me. I didn’t know what any of that meant, and I didn’t know how hard it was going to be”

– Acute Lymphoblastic Leukemia (ALL) patient
The impact and burden of blood cancers and blood disorders in Europe

Blood cancers and serious blood disorders place a huge burden on individual patients, their families and on wider society.

While considerable progress has been made in improving outcomes for blood cancers, malignant blood cancers are still responsible for almost 8% of all cancer deaths across the European region\(^i\), affecting about 154,000 people.\(^i\)

The effects of this burden are felt across society and the economy – the total economic cost of all blood disorders and blood cancers in Europe is approximately €23 billion.\(^ii\)

We have a responsibility to every patient, wherever they live and whomever they are – and to society. Everyone benefits when we work together – with patients, HCPs and healthcare systems.

“We put on a brave face, and everyone thinks we’re ok and we’re strong. But it’s not always the case… we’re only human”

— Sickle cell disease patient

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About Novartis Hematology in Europe

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

Across Europe, significant progress has been made by Novartis and our partners in treating a range of blood disorders.

Using our experience and expertise, we continually innovate and grow our pipeline of medicines for the future. We are leading the science, supported by the latest data and digital advances, to reimagine medicine for patients.

We are committed to ensuring sustainable access to our treatments

Innovation doesn’t stop here – we are always looking for new ways to help patients get sustainable access to our broad range of innovative treatments. Better access to treatment means better health. We must work on all ends of the healthcare system to ensure that patients can access the therapies they need, transforming what it means to live with a blood disease. We are proud to work with The Max Foundation on CMLPath to Care, which provides treatment and support to people living with chronic myeloid leukemia (CML) in low-income countries around the world, at no cost. To date, this program has reached 75,000 patients in nearly 68 countries across four continents.

“I’m a patient. I have cancer. But I can lead a normal life”

—Chronic myeloid leukemia (CML) patient
Disease areas we are working in

Our energy is focused on areas where we can resolve the greatest unmet need.

Rarity or complexity of disease does not deter us. We pursue innovation that resolves the greatest unmet need – wherever it is and whomever it affects. Advances in hematology treatment, from targeted therapies to cell and gene therapy, and immunotherapy, give so much cause for hope.

**Advanced Systemic Mastocytosis (SM)**

**CAR-T therapy**
- Acute Lymphoblastic Leukemia (ALL)
- Diffuse Large B-cell Lymphoma (DLBCL)

**Leukemia**
- Acute Myeloid Leukemia (AML)
- Chronic Myeloid Leukemia (CML)

**Immune Thrombocytopenia (ITP)**

**Myeloproliferative Neoplasms (MPNs)**

**Severe Aplastic Anemia (SAA)**

**Sickle Cell Disease (SCD)**

**Graft Versus Host Disease (GvHD)**

“Know that you’re not alone... You should know that there are people in that exact same situation”

— Myeloproliferative Neoplasm (MPN) patient
At Novartis, our expertise, determination and partnerships are what can turn this hope into reality.

HARMONY

Big Data To Enable Better And Faster Treatments For Patients With Hematologic Malignancies (HM)

We are proud to work with HARMONY, a European Network of Excellence for Big Data in Hematology.

Collaborating with over 90 public and private partners, HARMONY gathers data at scale to help define clinical endpoints and outcomes, helping to inform advances in treatment and care.

Novartis is committed to utilizing real-world evidence to help provide real-time oncology solutions, achieving the best results through collaboration.

www.harmony-alliance.eu

“At HARMONY we believe in the power of Big Data, collating data from across Europe to help us better understand blood cancers and disorders and how best to manage them.”

– Dr Jesus Hernandez, Lead Coordinator HARMONY

EUTOS

European Treatment and Outcome Study

Novartis has been part of EUTOS since 2007, a collaboration first with European Leukemia Net, and now with Jena University, to improve understanding and treatment of chronic myeloid leukemia (CML).

“I was young, only 16, when I was first diagnosed with ITP and it scared me a lot. My whole family were also very worried. We had very little knowledge of blood diseases and we only knew about leukemia”

– Immune Thrombocytopenia (ITP) patient
Looking to the future

Our vision in hematology is to transform the lives of people living with blood cancers and serious blood disorders.

Today, Novartis has nearly 20 different approved medicines for hematological conditions, across dozens of blood cancers and serious blood disorders, but we won’t stop there. We are bold in our relentless pursuit to develop and bring to patients breakthrough therapies for blood cancers and disorders, from targeted therapies, to precision cell and gene therapy, to immunotherapy.

We are conducting clinical trials into investigational therapies across multiple blood cancers and serious blood disorders, pursuing discovery until it makes a meaningful difference in the real world.

Cell & Gene Therapy

Cell therapy and gene therapy are overlapping fields of biomedical research and treatment. Both therapies aim to treat, prevent, or potentially cure diseases, and both approaches have the potential to alleviate the underlying cause of genetic diseases and acquired diseases.

- We have revolutionized cancer care with the first ever FDA approved CAR-T cell therapy.\(^3\) CAR-T cell therapy is a cutting-edge therapy that uses specifically altered cells from the immune system to fight blood cancers.\(^4\)

- Our focus is to continue researching potential next-generation CAR-T therapies, and we are now looking to expand CAR-T therapy, for the treatment of relapsed and refractory follicular lymphoma.

- We are also investigating the application of gene editing technologies to treat Sickle Cell Disease, a chronic, lifelong, debilitating disease that can range in clinical severity.

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\(^3\)Novartis. Novartis receives first ever FDA approval for a CAR-T cell therapy. Kymriah\textsuperscript{TM} (CTLO19), for children and young adults with B-cell ALL that is refractory or has relapsed at least twice | Novartis [Internet]. 2017 [cited 16 December 2020]. Available from: https://www.novartis.com/news/media-releases/novartis-receives-first-ever-fda-approval-car-t-cell-therapy-kymriahctlo19-children-and-young-adults-b-cell-all-refractory-or-has-relapsed-least-twice

**Immunotherapy**

Cancers can evade our immune systems with many molecular tricks. Scientists are investigating several approaches to increase the body’s immune response to cancer, and exploring the role of inflammation in cancer.

- Immunotherapies are revolutionizing cancer care; however, most of the benefits to date have been seen in patients with solid tumors, and less so in blood cancers.¹
- The discovery of TIM-3, a different immune target, may change that. By blocking TIM-3 in AML and MDS, we may have the opportunity to directly target cancer cells and also the immune response. Novartis is continuing to evaluate the use of TIM-3 for treating MDS and encouraging response rates have been seen so far.²⁻³

**Targeted Therapy**

Targeted therapies treat cancer by blocking the pathways or mutations driving cancer growth. Understanding the biology of cancer helps tailor treatments to specifically target the cancer.

- Our targeted therapies have helped transform CML from a life-limiting disease to a chronic one for many patients.⁴ However, many patients are still at risk of disease progression, and we are committed to further transforming CML care – this time through the development of an inhibitor specifically targeting the ABL myristoyl pocket (STAMP) which may help address tyrosine-kinase inhibitor (TKI)-resistance and intolerance in later treatment lines of CML.⁵⁻⁶
- The prognosis for Graft Versus Host Disease (GvHD) patients is poor. However, studies with one of our existing therapies nearly doubled overall response rates in steroid-resistant or steroid-dependent patients with chronic GvHD compared to the best available therapy.⁷⁻⁸

As long as patients continue to suffer from hematological diseases, **Novartis will continue to develop innovative and accessible therapies.**

We care. Together we cure. **We never stop.**
