

# Novartis Hematology in Europe

**Proud  
of our  
Bloodline!**

**Blood cancers and serious blood disorders affect one or more parts of the blood, such as red blood cells, white blood cells, platelets and plasma, preventing the blood from functioning as it should.**

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

**Blood cancers and serious blood disorders affect many people across Europe**

**154,000** PEOPLE died in Europe in 2019<sup>i</sup> from blood cancers



This accounts for **8%** of all cancer deaths in the region<sup>i</sup>

**Placing a significant burden on patients and their families**



**82%** of patients with blood cancers experience disabling symptoms<sup>ii</sup>

**The effects of this are felt across society and the economy**

The total economic cost of all blood disorders and blood cancers in Europe is estimated to be

**€23 billion**  
per year<sup>iii</sup>



However, across Europe, significant progress has been made by Novartis and our partners in treating a range of blood disorders over the years.

<sup>i</sup> Global burden of 369 diseases and injuries in 204 countries and territories, 1990-2019: a systematic analysis for the Global Burden of Disease Study 2019. The Lancet (doi:10.1016/S0140-6736(20)30925-9). [https://www.thelancet.com/journals/lancet/article/PIIS0140-6736\(20\)30925-9/fulltext#supplementaryMaterial](https://www.thelancet.com/journals/lancet/article/PIIS0140-6736(20)30925-9/fulltext#supplementaryMaterial)

<sup>ii</sup> Blood Cancer. Blood Cancer in America 2018 [Internet]. 2018 [cited 22 December 2020]. Available from: <https://blood-cancer.com/infographic/life-changing-impact/>

<sup>iii</sup> Engert A, Balduini C, Brand A, et al. The European Hematology Association Roadmap for European Hematology Research: a consensus document. Haematologica. 2016;101(2):115-208

# We believe that anyone living with a blood cancer or serious blood disorder has the right to a life free from pain, free from symptoms and free from disease – this is our vision for the future.

Advances in hematology treatment give so much cause for hope. It's our responsibility to turn that hope into reality for every patient. Wherever they live and whomever they are.

Today Novartis Hematology has nearly 20 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.

## Therapy areas we are working in:

### CML

■ Our targeted therapies have helped transform CML from a life-limiting disease to a chronic one for many patients.<sup>iv</sup> 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.<sup>v</sup>

### MDS

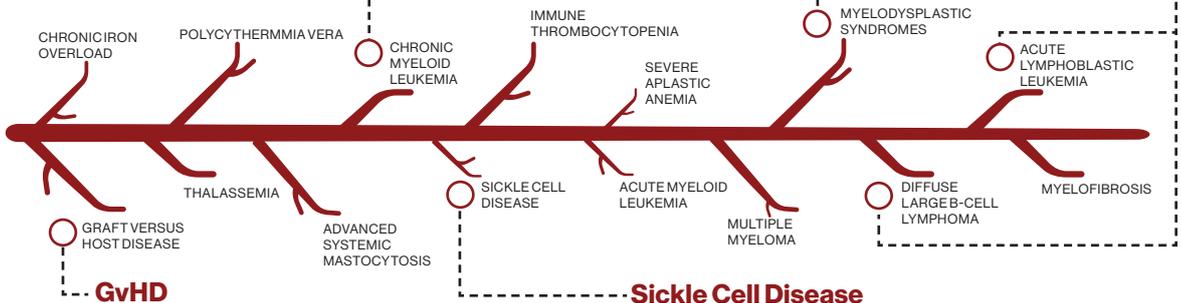
■ Immunotherapies are revolutionizing cancer care; however, most of the benefits to date have been seen in patients with solid tumors, and less so in blood cancers.

■ However, the discovery of TIM-3, an immune target, may change that. By blocking TIM-3 in 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.<sup>ix</sup>

### DLBCL, ALL and Follicular Lymphoma

■ We have revolutionized cancer care with CAR-T cell therapy, for the treatment of DLBCL and ALL.<sup>vi</sup> This is a cutting-edge therapy that uses specifically altered cells from the immune system to fight blood cancers.

■ Our focus is to continue researching potential next-generation CAR-T therapies and broaden their impact, and are researching its use for the treatment of relapsed and refractory follicular lymphoma.<sup>vii</sup>



**GvHD**

■ GvHD is a serious and common complication of stem cell transplants with no widely approved treatment options for patients who do not respond to steroids.

■ 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.<sup>x</sup>

**Sickle Cell Disease**

■ Sickle cell disease is a chronic, lifelong, debilitating disease that can range in clinical severity. Our scientists are working to evaluate potential disease-modifying therapies. Because sickle cell disease has a single genetic mutation, it provides unique opportunities in exploring gene editing technologies, such as CRISPR.<sup>viii</sup>

We care. Together we cure.  
We never stop.

Learn more at  
<https://www.novartis.com/our-company/novartis-oncology>

iv Harvard Health Publishing Harvard School of Medicine. FDA Approves Gleevec to Treat Leukemia [Internet]. 2001 [cited 4 December 2020]. Available from: <https://www.health.harvard.edu/digestive-health/fda-approves-gleevec-to-treat-leukemia>

v Novartis. Novartis investigational novel STAMP inhibitor asciminib (ABL001) meets primary endpoint of Phase III chronic myeloid leukemia study [Internet]. 2020. Available from: <https://www.novartis.com/news/media-releases/novartis-investigational-novel-stamp-inhibitor-asciminib-abl001-meets-primary-endpoint-phase-iii-chronic-myeloid-leukemia-study>

vi Novartis. Novartis receives first ever FDA approval for a CAR-T cell therapy, Kymriah(TM) (CTL019), 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-kymriahm-ctl019-children-and-young-adults-b-cell-all-refractory-or-has-relapsed-at-least-twice>

vii Novartis. CAR-T Cell Therapy: Pioneering Cancer Therapy | Novartis [Internet]. 2020 [cited 16 December 2020]. Available from: <https://www.novartis.com/our-focus/cell-and-gene-therapy/car-t-car-t-patients>

viii Novartis. Shaping the future of sickle cell disease [Internet]. 2020 [cited 7 December 2020]. Available from: <https://www.novartis.com/stories/discovery/shaping-future-sickle-cell-disease>

ix Novartis. A decades-long journey in blood cancer research [Internet]. 2019 [cited 10 December 2020]. Available from: <https://www.novartis.com/stories/discovery/decades-long-journey-blood-cancer-research>

x Novartis. Novartis announces Phase III study of Jakavi® in chronic graft-versus-host disease met primary and key secondary endpoints | Novartis [Internet]. 2020 [cited 22 December 2020]. Available from: <https://www.novartis.com/news/media-releases/novartis-announces-phase-iii-study-jakavi-chronic-graft-versus-host-disease-met-primary-and-key-secondary-endpoints>

