Treatments for blood cancers and serious blood disorders are advancing rapidly – Novartis is helping lead the way

Novartis has been at the forefront of advances in how blood cancers and serious blood disorders are treated, helping patients live their best lives.

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The last few decades have brought remarkable advances in how blood cancers and serious blood disorders are treated. New types of medicines have changed some diseases, like chronic myeloid leukemia (CML), from acute – and often fatal – diagnoses into chronic conditions patients can live with for many years.[1] Other areas of hematology research have brought new treatment options for people with rare and nonmalignant (benign) blood disorders.[2-4]

And even more critical research is underway to support the millions of people globally living with blood cancers or disorders.[5] Novartis has been at the forefront of this field, developing medicines that have helped transform treatment paradigms and helped patients with blood diseases and disorders live their best lives. And we don't plan on slowing down.

A history of innovation in hematology care

In the mid-1970s, the estimated five-year survival rate for patients with CML was only 22%.[6] Today, the five-year survival rate is estimated above 70%.[6] This shift has been due in part to the introduction of targeted therapies, like tyrosine kinase inhibitors (TKIs), which Novartis and its partners worked to advance. The first one, approved by the FDA in 2001, made CML a chronic, rather than life-limiting, condition.[7] A few years later came a selective TKI that introduced the possibility of treatment-free remission for CML[8] – and more recently, a CML medicine with a new mechanism of action expanded care for patients with resistances and/or intolerances to currently available TKI therapies.[9]

> Meeting with hematologists in the field or at conferences, I feel their sense of urgency to find the next best medicine for their patients and their dedication to changing patients' lives. It fuels our

> commitment to this area and makes me excited for the promise of our ongoing innovation to further build our legacy in advancing care for blood cancers and hematologic diseases. And I have absolutely no doubt that we can deliver even more transformative medicines for patients in the near future.
>
> **Victor Bultó**, *President of Innovative Medicines US*

Another innovation that changed the landscape of blood cancer care was the development of CAR-T therapy, a groundbreaking form of therapy – gene therapy – that enables the immune system to fight cancer cells more effectively. The first CAR-T therapy was developed by Novartis and approved by the FDA in 2017 for pediatric and young adult patients (up to 25 years in age) with refractory Acute Lymphoblastic Leukemia (ALL).[10] And to expand the reach of CAR-T, we developed T-Charge™, a next-generation CAR-T manufacturing platform that has the potential to be the foundation for a number of investigational pipeline CAR-T cell therapies.[11]

Other advances in recent years have spanned Acute Myeloid Leukemia (AML),[12-14] myeloproliferative neoplasms (MPN),[1] graft-versus-host disease (GvHD),1,15 immune thrombocytopenia (ITP),[1,2] and chronic iron overload.[16,17] We've been heartened to have been part of each of these developments and are committed to continuing our innovation in the lab so that we can have even more meaningful impact on patients' lives in the future.

A pipeline of hope

> Significant developments in hematology have meant patients' expectations for treatment have fundamentally shifted. We're no longer just talking about extending lives, but about helping people with blood cancers and disorders live symptom-free and even potentially treatment-free, as part of our commitment to the next generation of innovation.
>
> **Jeff Legos**, *Executive Vice President, Global Head of Oncology and Hematology Development at Novartis*

Across the globe, Novartis is conducting clinical trials with the aim of turning data into real-world treatment options for patients. One way to do this is to advance care where existing treatments may not fully control symptoms or may be inconvenient or cumbersome for patients.[18,19] Paroxysmal nocturnal hemoglobinuria (PNH) is a rare, chronic, and serious blood disorder, in which some red blood cells more susceptible to being destroyed by the immune system's complement system.[20] In PNH, red blood cells break apart (hemolysis), leading to blot clots (thrombosis) and impaired bone marrow function, as well as anemia and fatigue.[20] Although PNH treatments exist, they require infusions at healthcare centers, and may leave some patients still anemic, in need of blood transfusions, and fatigued.[18,19]

We believed a more convenient and more effective option was possible. A molecule discovered at Novartis is being studied for a range of complement-system-mediated diseases, including PNH.[21,22] Because it is an oral investigational treatment that may significantly reduce the destruction of red blood cells – and the need for blood transfusions – in PNH, it has the potential to change how the disease is treated and improve patients' everyday lives.[21,22]

Immune thrombocytopenia is another rare blood disorder – affecting platelet cells – that can leave patients with symptoms that affect their quality of life, including significant fatigue.[23] Having been a pioneer in ITP treatment years ago and understanding there were still opportunities to advance care, we knew we could do more. So we've been advancing a molecule engineered to have a unique structure, known as a B-cell-

depleting antibody, that may provide a more effective option for patients with ITP.[24]

And we've got a number of other promising medicines in the pipeline, spanning CML, AML, myelodysplastic syndrome, atypical hemolytic uremic syndrome and warm autoimmune hemolytic anemia.[25] In one case, we're investigating our approved third-line CML treatment across multiple treatment lines, to bring patients an effective option earlier on in care.[9] Whether life-threatening or benign, we believe that uncovering the mechanisms that drive disease and listening to what patients most need from their treatments can drive fundamental advances in care.

Novartis hematology pipeline

Benefiting from our continued focus on innovation, Novartis has one of the industry's most competitive pipelines.

Learn more

Working closely with patients to advance medicine together

To help us carry out our mission of improving and extending people's lives, we actively seek out the perspectives of patients, whose critical insights can inform our work. In 2022, the majority (87%) of our early research programs in general medicines captured patient insights before carrying out the first trials in humans.[26] Eighty-five of our clinical trials used patient insights to inform how clinical trials were designed and/or carried out.[26] And 202 clinical trials across 77 countries included patient-reported outcomes (PRO), to ensure we're including the outcomes that matter most to people living with the condition.[26]

We also carry out global surveys to help us understand patients' perceptions of living with a disease, as well as the benefits and burdens of existing treatments. For instance, our most recent ITP World Impact Survey outlined how ITP can affect patients' day-to-day experiences, emotional well-being, ability to work, and feelings about current treatments.[27] Another recent survey focused on unmet needs in CML from the perspectives people living with CML and treating physicians.[28] We found key disconnects, which pointed to a need for greater communication and shared decision-making, as well as treatment options that don't sacrifice quality of life for efficacy.[28] Surveys like this can be helpful, not only to support patients and providers but also to help the industry gain a clearer understanding of what's important to patients as well as any opportunities to advance care.

> We've come a long way in terms of how we manage CML, but there's still work to be done. The CML survey of unmet needs revealed what patients need from their providers and what they want in treatments, especially over time. It's clear that patients really need to play a strong role, not just in their own treatment decisions but to help inform our larger understanding of CML.
>
> **Lisa Machado**, *CML SUN Steering Committee member, founder of the Canadian CML Network and executive producer of Healthing.ca*

We are convinced that only by working together can we improve outcomes for patients and become a trusted partner in changing the practice of medicine.

Global partnerships to expand access to life-changing care

We know providing life-changing medicines to patients can't be done alone. That's why in addition to our in-

house work, we collaborate with partner organizations to expand care where it's needed and address the barriers that prevent people from benefitting from the medicines they need, no matter where they live or their income.[11] Since 2021, we've worked with the Bill & Melinda Gates Foundation to research a novel gene therapy for sickle cell disease with a focus on access in sub-Saharan Africa.[11] We also collaborate with CancerPath to Care, an access initiative with The Max Foundation, to connect people living with CML and other cancers with effective treatments and hands-on support.[11]

Novartis has joined forces with 40 public and private partners, as a founding member of the Access to Oncology Medicines (ATOM) Coalition.[11] We were the first pharmaceutical company to contribute an innovative treatment, by granting the Medicines Patent Pool (MPP) a non-exclusive license for nilotinib – becoming the first company to sign a non-exclusive voluntary license for an oncology medicine. The agreement allows the MPP to sub-license nilotinib to generic manufacturers, who will be able to manufacture and commercialize the medicine in all 44 ATOM Coalition countries. We hope this approach can serve as a new model for the pharmaceutical sector to help close the gaps in access to life-changing medicines for non-communicable diseases.

This is an exciting time for hematology, with new developments paving the way for even more effective medicines and technologies. Through bold science and bold approaches, we'll continue to reimagine medicine and transform care for people living with blood cancers and serious blood disorders.

Hematology

Reimagining medicine to transform the treatment of blood cancers and serious blood disorders.

Learn more

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