Innovation in CML at Novartis

The Development of the First TKI

In May 2001, Novartis received approval from the US Food and Drug Administration (FDA) for the first targeted therapy for cancer, known as a tyrosine kinase inhibitor (TKI). This was a watershed moment in drug discovery, transforming the treatment landscape for chronic myeloid leukemia (CML) and opening the door to reimagining possibilities for other forms of cancer and blood disorders.

The development of the first TKI was an unlikely success story. Back in the 1950s, scientists had a limited understanding of cancer and, specifically, how genes influence the disease. Nevertheless, research was intensified over the next few years, with hopes that the emerging understanding of genomics could bring forth lasting change. One particular gene, discovered by Drs Peter Nowell and David Hungerford from Philadelphia, was identified as a common factor in patients with CML. Soon after, it was learned that this gene, dubbed as the Philadelphia chromosome, was also the root cause of a faulty tyrosine kinase protein that was directly responsible for the blood cancer.

In the decades following Nowell and Hungerford's discovery, Novartis had started to look more closely into this class of proteins. We collaborated with scientists and clinicians, including Dr Brian Druker, researcher at Oregon Health and Science University. It was Dr Druker who suggested that Novartis explore ways of developing a molecule with the ability to inhibit the faulty kinase—a drug that would target this disease trigger, different than the therapies available at that time. There was a pressing need; CML was a fatal disease and in the mid-1970s, the estimated 5-year survival rate was only 22%.

> I had one goal at the time—to find a company that had an inhibitor for BCR-ABL and to bring it into the clinic.
>
> **Brian Druker**, MD, Director, Knight Cancer Institute at Oregon Health & Science University

Goal of Improving Patient Responses

This milestone approval helped evolve treatment goals in CML and demonstrated Novartis' commitment to research and innovation.

Novartis' Continuous Pursuit of BOLD Science

Today, the 5-year survival rate for patients with CML is estimated above 70%. Despite important advancements in CML care over the past 20 years, cancer and serious blood disorders are a devious enemy, and one breakthrough is not enough. Significant unmet needs still remain, particularly for patients in later lines of CML who have experienced resistance or intolerance to available treatments. That's why Novartis won't stop in the continuous pursuit of bold science and striving to transform the lives of patients with CML.

At Novartis, we are relentless in the pursuit of cutting-edge medicines, from targeted therapies to treatments that harness the immune system and beyond. Like with CML, we must continue to take a BOLD approach with science to tackle some of the toughest-to-treat diseases and seek solutions for incredibly challenging public health issues facing our society today.

> Novartis is a company for science and innovation. We have a big vision as a company, and that is to reimagine medicine—that is what we strive for. If you really want to change something, you must be BOLD and have the courage to take risks.
>
> **Marie-France Tschudin**, President, Innovative Medicines International and Chief Commercial Officer, Novartis

With a heritage in developing transformative, first-in-class therapies for blood disorders, we open our minds to all scientific possibilities and reach further for new and different endpoints in hematologic diseases.

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