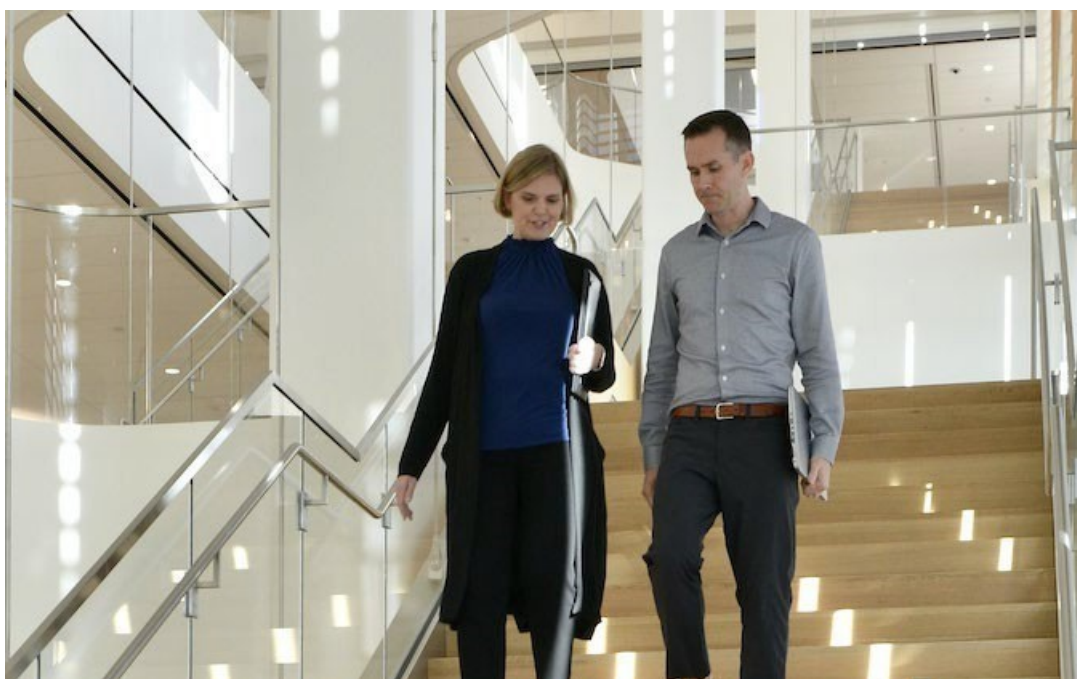


A decades-long journey in blood cancer research

Novartis immunologist describes the excitement of discovering a promising new immuno-oncology (IO) target in the treatment of acute myeloid leukemia (AML) and high-risk myelodysplastic syndrome (MDS).

Dec 09, 2019

It's the dream of every medical scientist: when the years of relentless effort, minor victories, setbacks and challenges of research finally yield a breakthrough that may one day change the way patients are treated. Catherine Sabatos-Peyton, Director, Exploratory Immuno-Oncology at the Novartis Institutes for BioMedical Research (NIBR), recalls her role in the discovery of TIM-3, an immunotherapy target that may bring new hope to patients with acute myeloid leukemia (AML) and high-risk myelodysplastic syndrome (MDS).



NIBR scientists Catherine Sabatos-Peyton and Mikael Rinne are working together to investigate a new approach for patients with AML and high-risk MDS.

"TIM-3 has been an important discovery particularly for hematologic malignancies like AML and MDS because of the intriguing role that it plays," Sabatos-Peyton says. "While we don't fully appreciate the mechanism yet, we think that, by targeting TIM-3, it may give us the opportunity to attack these cancers in two critical ways – by directly binding to the cancers cells themselves, as well as to the cells of the immune system."

I think that's the moment that made believers out of all of us.

Catherine Sabatos- Peyton, Director, Exploratory Immuno-Oncology, NIBR

AML and MDS affect the immune system and the ability of the bone marrow to generate platelets and red blood cells. Patients face significant challenges with their diagnosis; these diseases are aggressive and life-threatening.

"Immuno-oncology agents have really revolutionized the care of cancer for many patients," says Mikael Rinne, Senior Clinical Program Leader at NIBR, who leads early clinical development. However, most of the benefit

has been seen in patients with solid tumors, and less so in blood cancers. “When hematologists tried to treat AML and MDS with PD-1 inhibitors, a standard immunological approach, the response rates weren’t as promising.”

The discovery of TIM-3—a different immune target—may change that.

What exactly is TIM-3?

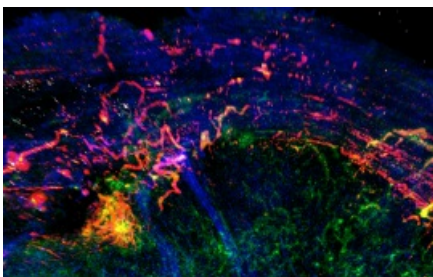
TIM-3 (T-cell immunoglobulin and mucin-domain-containing-3) is an inhibitory cell surface receptor that plays a key role in regulating adaptive and innate immune responses. Its role is to ensure that the immune system remains in balance, thereby avoiding tissue damage from an unchecked immune response.

What’s more, it turns out that TIM-3 exists on both the immune cells that fight cancer (T- cells, myeloid cells, and natural killer cells), and the cancer cells themselves (leukemic stem cells, but importantly, not on normal hematopoietic stem cells), according to Sabatos-Peyton.

“By blocking TIM-3 in this setting, we may have the opportunity to directly target cancer cells and also the immune response,” she says, “Taken together, this could potentially result in an anti-cancer immune response.”

To block TIM-3, Novartis has developed and is evaluating an investigational immunotherapy agent called MBG453.

On December 9, 2019, preliminary Phase I data for MBG453 with decitabine in patients with high-risk MDS and AML was presented at the American Society of Hematology Annual Meeting and Exposition (ASH), in Orlando, Florida, in the US. Phase II clinical trials are underway and will help to establish the safety and efficacy of this approach.



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