

Once shelved as too risky, blood cancer drug discovered in San Diego gets FDA approval

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Cancer cell during cell division. Credit: National Institutes of Health

A promising cancer drug, discovered in San Diego and later discarded for safety concerns, has at last been approved for use. The drug, fedratinib, treats certain kinds of a bone marrow cancer called

myelofibrosis.

On Friday, the U.S. Food and Drug Administration approved fedratinib, which will be sold by Celgene under the brand name Inrebic. It had originally been developed by San Diego's TargeGen, purchased by Sanofi in 2010 for up to \$635 million.

The drug is the first new therapy approved for myelofibrosis in nearly a decade, Celgene said in a statement.

Myelofibrosis causes blood-forming bone marrow to be replaced with fibrous scar tissue. This deprives patients of energy and makes them vulnerable to infection. Blood-forming cells move to the spleen, liver and other parts of the body, causing additional complications.

Fedratinib targets a mutation called JAK2 that drives certain kinds of myelofibrosis. In [clinical trials](#), oncologists including UC San Diego's Dr. Catriona Jamieson reported patient responses including complete remissions that no other drug delivered.

But after Sanofi withdrew the drug from clinical testing in 2013, some patients who had thrived on it died. Others survived on alternatives. Since then, Jamieson and John Hood, who led early research on the drug, had searched for ways to bring it back.

Fedratinib has an unusually complicated history. It sprang out of research at San Diego's TargeGen, was tested by Sanofi, then shelved because a few patients developed a dangerous brain inflammation, with one death. Were it not for Hood, that would have been the end.

Jamieson said one of her first patients on the fedratinib trial, Theresa Blanda, bounced back on the drug after being very seriously ill. Her symptoms included a hugely swollen liver and knee, filled with displaced

blood-forming stem cells.

Blanda went on the clinical trial in 2008. But when the drug was put on hold five years later, supplies dried up. Blanda relapsed.

"It looked like she may succumb to the disease," Jamieson said. "So I called John and said, What are we going to do about this? And John said, Well, we're going to have to acquire the drug."

Hood said he acted because it was the right thing to do.

"When you have patients who are truly responding to a [drug](#) that you're behind, who desperately need it, you do whatever you can to make sure they get it," Hood said. "That's the moment people in this industry live for. If you don't drop everything, you're in the wrong industry."

"I quit my job, worked out a deal with Sanofi, the local San Diego VCs and a third party to fund it, and started a company."

The effort took longer than anticipated, in part because of the need to answer the safety concerns. But in 2017, Hood founded Impact Biomedicines to bring back fedratinib. Impact was purchased by Celgene in January 2018 for \$1.1 billion upfront, plus milestones that could bring the total price to \$7 billion.

It was too late for Blanda. She died in 2016.

On Sunday, fedratinib's approval was celebrated at the 26th annual Luau and Legends of Surfing Invitational, a fundraiser for UCSD Moores Cancer Center held near Scripps Pier. Jamieson and Hood attended.

Fedratinib is another step toward personalized [cancer](#) therapy, said Dr. Brian Druker, a UCSD alumnus who was honored along with Salk

Institute cancer researcher Tony Hunter.

Druker's research led to Gleevec, which treats chronic myelogenous leukemia. It's considered the first genetically targeted [cancer therapy](#).

For his part, Hunter performed research that led to an entirely new category of cancer drugs called tyrosine kinase inhibitors. That category includes fedratinib.

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