THE AGE

National Cancer

Australian-made cancer drug gets billion-dollar US FDA approval

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A drug invented in Melbourne to treat a lethal bone marrow cancer is set to become a billion-dollar global blockbuster after US regulators on Friday gave it the green light to be sold there.

Momelotinib was invented by an Australian team in Burnley in the late 1990s, and the drug was purchased by pharma giant GSK in 2022 for $US1.9 billion. It is used to treat myelofibrosis, a rare and devastating disease that leads to bone marrow burnout.

Professor Andrew Wilks, managing director of SYNthesis BioVentures, was involved in the breakthrough research.

Getting a drug from lab bench to patients is a long, expensive and usually unsuccessful process. For all the medical discoveries made around the world every day, Australia approves only about 40 new drugs every year.

After winning approval in the US to treat myelofibrosis, GSK expects the drug to generate more than $US1 billion in sales each year. The company is expected to apply for approval globally. Both the key underlying cause of myelofibrosis and the treatment were discovered in Melbourne.

“There are few stories that really encapsulate how great Australian science can be better than this one,” said Professor Mark Dawson, co-head of the Cancer Biology and Therapeutics Program at the Peter MacCallum Cancer Centre. He was not involved in the drug’s development.

On Monday afternoon, Professor Andrew Wilks was drinking champagne to celebrate an achievement three decades in the making.

Wilks discovered the Janus kinases in 1989. Momelotinib is named after Melbourne, the city where he and chemist Dr Chris Burns first sketched out the molecule.

“How long’s that, 35 years? I was young and lovely when I started this thing,” Wilks said.

Myelofibrosis is rare, poorly understood, and devastating. The disease appears driven by a dysfunction in a family of proteins known as Janus kinases.

Ken Young has one of the diseases that is likely to develop into myelofibrosis. JOE ARMAO

These proteins are named after the Roman god of duality because they play key roles in both manufacturing and regulating blood cell production.

In the early 2000s, researchers around the world linked genetic dysfunction in the newly discovered kinases to a pair of diseases in which the proteins go rogue, pumping out far too many blood cells.
The bone marrow strains under the demands of excess production, and begins to inflame. Over time, this inflammation scars the sensitive tissue.

Eventually, the marrow reaches what Dawson described as the "burnout phase". Where once they poured out too many red blood cells, now they make too few, leaving patients anaemic.

Ken Young was diagnosed with polycythemia vera, one of the diseases that can lead to myelofibrosis, in 1998 after a routine doctor's visit.

"One of the doctors said 'your blood is like jam, it's very thick'," said Young, who is a committee member of advocacy group MPN Alliance Australia.

Like many people with the disease, Young has had no choice but to wait to see if myelofibrosis develops.

"When I was first diagnosed, there were no treatments for it. It was quite medieval in a way – the main treatment was venesection, old-fashioned bloodletting," he said.

Momelotinib treats myelofibrosis by calming those overactive kinases. While not a cure, clinical trials show it significantly improves some patients' conditions compared to existing therapies. Importantly, it is also designed to cut treatment side effects dramatically – its major advantage.

Associate Professor David Ross led the Australian arm of the drug's clinical trials at Flinders Medical Centre. He recalled one patient, in his '70s, found he no longer needed frequent blood transfusions.

"That means he's no longer tied to the hospital every few weeks for a transfusion, he's got more energy, he can get out and do more," Ross said. "It can really make a difference to the lives of some patients."

Wilks and Burns developed the drug at a local start-up until 2008, when the global financial crisis meant they were unable to get funding to run further clinical trials.

The drug passed through a few companies until it was bought by GSK last year; it struck gold with US regulatory approval.

Having sold the patent, Wilks won't enjoy any of the revenue. "I'm consoling myself from the fact that this will probably be a billion-dollar drug by the fact it's all about the patient."

Wilks, who now heads venture fund SYNthesis BioVentures, said momelotinib's story was a lesson for Australian science about the importance of commercialising discoveries and actually taking drugs from the lab to the patients.

"We need to change the attitude a little bit. We've shown we can do it."

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