Treatment reverses young man's type 1 diabetes. Will it last?

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(HealthDay)—After starting a drug that's officially approved to treat a

type of blood cancer, a young man with type 1 diabetes was able to stop using insulin.

He's been off insulin since August 2018—more than two years.

Dr. Lisa Forbes—his doctor and co-author of a letter describing his case in the Oct. 8 issue of the *New England Journal of Medicine*—stopped short of calling the drug a cure for type 1 diabetes.

But Forbes, an assistant professor of pediatrics, immunology, allergy and rheumatology at Baylor College of Medicine in Houston, said the patient's diabetes appears to have been reversed. She hopes it will stay that way as long as he keeps taking the oral medication called ruxolitinib (Jakafi). It's in a class of medications known as JAK inhibitors.

Whether this drug can help others with type 1 diabetes isn't yet known. This patient had a genetic mutation that ruxolitinib is known to work on. Forbes said it's not clear if other people with type 1 diabetes also have this specific genetic mutation.

Type 1 diabetes is believed to be an autoimmune disease, though the exact cause is unknown. It develops when the immune system mistakenly attacks insulin-producing beta cells in the pancreas. Insulin is a hormone that ushers the sugars from foods into the body's cells to be used as fuel.

People with type 1 diabetes produce little to no insulin and must take multiple daily injections of insulin (or use an insulin pump) to survive. No treatments are approved for reversing type 1 diabetes.

At 15, Forbes' patient had been experiencing chronic yeast infections (of skin, nails, mouth and throat), chronic diarrhea, oral and rectal ulcers, recurrent sinus and lung infections and another autoimmune condition called hypogammaglobulinemia. At 17, he was diagnosed with type 1

diabetes.

Because he had multiple conditions, his doctors ordered whole genome sequencing to see if they could pinpoint a root cause. They saw one particular genetic mutation and thought ruxolitinib might help. He started the drug nine months after being diagnosed with type 1 diabetes.

"The drug had an unbelievable effect on his type 1 diabetes," Forbes said. "A year after starting ruxolitinib, we took him off insulin, and he's been insulin-free ever since."

The patient is in college now, and Forbes said he calls the drug a "game-changer" because it's a pill and so easy to take.

Forbes said this case provides potentially important information into a pathway that leads to type 1 diabetes. But more research is needed, she added.

Because ruxolitinib acts on the immune system, patients have a higher risk of certain infections. And their white blood cells, liver function and kidney function have to be checked every few months, according to Forbes.

She isn't the only one excited about the potential of JAK inhibitors in type 1 diabetes.

JDRF (formerly the Juvenile Diabetes Research Foundation) has been funding research into JAK inhibitors for years, and will soon start a clinical trial in Australia for people with newly diagnosed type 1 diabetes, according to Frank Martin, director of research at JDRF.

"We're hoping JAK inhibitors will have a really profound effect in type 1 diabetes," Martin said. He noted that they've been used to treat other

autoimmune conditions, too.

In type 1 diabetes, "They tone down the immune system response, tamping down the strength of the immune cells, and improve beta cell survival," Martin said.

Although the Australian trial will focus on people who are newly diagnosed, Martin suspects even people with long-standing type 1 diabetes may benefit from JAK inhibitors.

"They may still need to be on insulin, but less, depending on what their beta cell reserve is," he said.

Like Forbes, Martin also hesitated to call ruxolitinib a cure.

"People have to continue taking the drug, but they don't appear to become resistant to it," he said. "We still want a permanent cure, but this may be a step in the right direction."

More information: Learn more about type 1 diabetes from JDRF.