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Drug Offers Hope In Diabetes Study

By [RON WINSLOW](#)

Researchers said a cheap generic drug used as a vaccine against tuberculosis showed promise in a small human study as a potential therapy for Type 1 diabetes, one of medicine's most-difficult-to-treat diseases.

The drug, called BCG, for bacillus-Calmette-Guerin, appeared to halt the process that causes the disease, in which the immune system mounts an attack on the pancreas, destroying its ability to produce insulin required to regulate blood sugar.

In addition, in four of the six treated patients, who had been living with the disease for an average of 15 years, the treatment enabled the pancreas to temporarily begin producing insulin again, researchers said.

"These were people with almost-dead pancreases," said Denise Faustman, director of the immunobiology laboratory at Massachusetts General Hospital, Boston, who led the study. "The data suggest that the pancreas can kick back in even 15 years after you get the disease. That surprised us."

Results of the new study are being unveiled in two presentations during the annual meeting of the American Diabetes Association, which began Friday in San Diego.

Dr. Faustman cautioned that the results don't amount to a cure and they need to be confirmed in a larger study, which is expected to begin enrolling patients within a year. Further research is required to determine what dose and how often injections of the drug would be necessary to maintain the benefit of the treatment. "You can't yet throw your insulin syringes away," Dr. Faustman said.

Patients in the current study were given two injections of BCG four weeks apart. Dr. Faustman said the drug costs about \$15 a vial. In the larger study, Dr. Faustman said, the plan is for participants to get injections six weeks apart for as long as 18 months.

Robert Henry, president for medicine and science with the American Diabetes Association and a diabetes researcher at University of California, San Diego, said the findings are based on a small number of patients and should be considered "preliminary." But "if this is real" and is validated in larger studies, "we might have a very cheap and effective way to treat and perhaps prevent Type 1 diabetes."

About 3 million Americans have Type 1 diabetes, formerly known as juvenile diabetes because it is typically diagnosed in children. Globally, about 70,000 new cases are diagnosed each year. The more common type of diabetes is Type 2, which unlike Type 1, is driven by obesity and metabolic disorders that are typically preventable with exercise and diet.

Type 1 is caused by an immune system gone awry. Managing the disease typically requires frequent daily blood tests and insulin injections. Long-term consequences include blindness, kidney failure, heart disease and amputations. Dr. Faustman said treating the disease in the U.S. costs about \$17 billion annually.

The new results come on the heels of three late-stage trials in which other medicines failed to stop or prevent the immune system's attack on the pancreas, in particular the so-called islet cells that produce the body's supply of insulin. Researchers' efforts to develop an artificial pancreas or methods for transplanting islet cells as possible treatments forge ahead but a cure has remained elusive.

The new findings "add to our knowledge of how we can potentially prevent the autoimmune destruction of islet cells in Type 1 diabetes," said Tom Donner, acting director of the diabetes center at Johns Hopkins University Medical Center, who wasn't involved in the research.

It was especially interesting, he said, that pancreatic function was at least transiently restored in people who have had the disease for years. He and other researchers said few would expect that pancreatic function could be restored after such a long period under immune-system attack.

Most studies of drug therapies are undertaken in newly diagnosed patients whose pancreases are still producing some insulin. The hope in those cases is to prevent further damage.

Eva Mezey, an immunology expert at the National Institutes of Health who is familiar with Dr. Faustman's work, said even if such a treatment strategy allows restoration of only a small amount of a person's own insulin production capacity, it would likely be a benefit. Instead of relying solely on insulin injections to manage blood sugar, which don't typically conform to the body's natural rhythms, "it would give the body a chance to have the normal physiological regulation of insulin levels," she said.

Dr. Faustman said she and her colleagues identified BCG as a possible therapy while screening a library of generic drugs. As a vaccine for tuberculosis, its effect is based on its ability to raise an immune modulator called tumor necrosis factor, or TNF.

In Type 1 diabetes, an abnormal version of an immune system cell called a T-cell mistakenly sees the insulin-producing cells in the pancreas as an enemy and attacks them. Earlier research in mice by Dr. Faustman showed that such cells are vulnerable to elevated levels of TNF.

The new study offers "proof of concept" that the same dynamic is at work in people.

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