Immunotherapy drug delays onset of Type 1 diabetes in at-risk group

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More than five years after receiving an experimental immunotherapy drug, half of a group of people at high risk of developing Type 1 diabetes remained disease-free compared with 22% of those who received a placebo, according to a new trial overseen by Yale School of Medicine researchers.

And those who developed diabetes did so on average about five years after receiving the new drug, called teplizumab, compared with 27 months for those who received the placebo.

The study, which was done in collaboration with researchers from Indiana University, was published March 3 in the journal *Science Translational Medicine*.

"If approved for use, this will be the first drug to delay or prevent Type 1 diabetes," said Kevan Herold, the C.N.H. Long Professor of Immunobiology and of Medicine (Endocrinology) at Yale and co-senior author of the paper.

The drug, developed by biotechnology company Provention, has been awarded breakthrough status by the U.S. Food and Drug Administration and could be approved for general use by summer, Herold said.

In the trial, an analysis of the 76 subjects showed reduced levels of damage caused by T cells in response to the drug and improved functioning of insulin-producing beta cells in those who received teplizumab.

The subjects in the trial had a median age of 13 years and relatives with Type 1 diabetes.

The new study is the result of 30 years of work by Herold's lab to find new treatments for Type 1 diabetes. The findings are a follow-up to another clinical study organized by TrialNet, an international coalition dedicated to the study of the disease. That study, which was published in 2019, showed a delay in the onset of Type 1 diabetes among those who received teplizumab.

Type 1 diabetes is an autoimmune disease in which a person's own T

cells attack insulin-producing beta cells in the pancreas. Those diagnosed require lifelong insulin treatment and face higher risk of death and diseases affecting the heart, kidneys, and vision. Diagnosis of the disease often occurs during childhood or adolescence.

Herold stressed it is not known whether some of the subjects who received teplizumab will never develop Type 1 diabetes. But delaying onset of disease could have a big impact on the development of those at high risk.

"Any time without diabetes is important, but particularly so for those children who might have a chance to grow up without it," he said.

More information: E.K. Sims el al., "Teplizumab improves and stabilizes beta cell function in antibody-positive high-risk individuals," *Science Translational Medicine* (2021). stm.sciencemag.org/lookup/doi/… scitranslmed.abc8980

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