

Type 1 Diabetes Reversal Trials at Massachusetts General Hospital

What is the BCG Human Clinical Trial Program?

Led by Dr. Denise Faustman at Massachusetts General Hospital, the **BCG Human Clinical Trial Program** is testing Bacillus Calmette-Guérin (BCG), as a **treatment for longstanding type 1 diabetes**. The goal is to bring forward a new, inexpensive, non-toxic treatment for patients who have been living with type 1 diabetes for many years.

A Unique Type 1 Diabetes Research Program

- Our goal is to reverse advanced diabetes, not just temporarily halt new-onset disease or treat symptoms
- The trials do not require lifelong immunosuppressive drugs or cell transplants
- The trials are designed to kill only disease-causing cells, sparing healthy cells
- The trials employ BCG, a safe and inexpensive generic drug

Why test BCG in type 1 diabetes?

BCG is a **generic drug** that has been used for **nearly a century** in different ways, including as an anti-tuberculosis vaccine and as a bladder cancer treatment. BCG is derived from a strain of bacteria that is weakened so that it cannot make humans sick, but is still strong enough to prompt an immune response—including production of a natural substance in the body called **tumor necrosis factor (TNF)**. This response may be able to help **halt the disease process** in advanced type 1 diabetes. In preclinical studies¹⁻³ and in a Phase I human clinical trial,⁴ the Faustman Lab has shown that temporarily elevating TNF levels can **eliminate the “bad” white blood cells** (those that mistakenly attack and destroy the healthy cells of the pancreas that secrete insulin) in type 1 diabetes,⁴ which may have clinical benefits.

What did the Phase I human trial show?

Data from the first human study show that BCG may be a **promising treatment for advanced type 1 diabetes**.⁴ In this double-blind, placebo-controlled study, BCG was administered to adults who had been living with type 1 diabetes for an average of 15 years. BCG was shown to be **safe for people with type 1 diabetes**, with no severe reactions to the vaccine and only mild inflammation at the injection site, as expected. In addition, BCG treatment not only **helped to eliminate the “bad” white blood cells** that contribute to type 1 diabetes, but it also **temporarily restored the ability of the pancreas to produce small amounts of insulin**—an exciting finding in people who had been living with type 1 diabetes for so long!

What is the Phase II study?

A Phase II study is currently underway at Massachusetts General Hospital. The goal of this randomized, double-blind, placebo-controlled study is to see if repeat injections of BCG can **benefit people with longstanding type 1 diabetes** by putting the **disease into remission** and helping to **prevent diabetic complications**. The Phase II study will include approximately **150 people** with longstanding type 1 diabetes, ages 18-65. Participants will receive 2 injections of either **BCG or placebo** spaced 4 weeks apart during the first year, then 1 injection per year for the next 4 years. Over the course of the study, the investigators will see whether BCG vaccination is associated with **improvements in HbA1c** and look for **beneficial changes in autoimmune status**, such as continued elimination of the bad white blood cells that attack the pancreas.

Thank you for joining us in the fight against diabetes!

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HOW TO SUPPORT THIS PROGRAM

Philanthropy is a vital part of our program to **reverse advanced type 1 diabetes**. Please help us move these human studies forward with a tax-deductible donation.

1. To make a secure online donation, please visit www.faustmanlab.org and click on “Support.”
2. Donations by check, payable to “Massachusetts General Hospital” (subject line “Faustman Diabetes Trials”) can be mailed to:

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To learn more visit faustmanlab.org

Researching a cure for type 1 diabetes

References

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