



Immunobiology Laboratory
Massachusetts General Hospital East
Building 149, Thirteenth Street, Room 3601
Charlestown, Massachusetts 02129
Tel: 617-726-4084

Denise Faustman, MD, PhD
Director, Immunobiology Laboratory
Associate Professor of Medicine
Harvard Medical School

BCG Turns Pancreas Back on in Phase I Study, Phase II Being Launched

Dear Friends,

We have great news to share with you. At the 2011 scientific meeting of the American Diabetes Association (ADA), we presented additional results from the Phase I human clinical trial testing BCG vaccination as a treatment for advanced type 1 diabetes. These data show the potential of BCG treatment to turn the pancreas "back on" briefly, even in people who have had type 1 diabetes for many years.

Data from the Phase I study show that BCG treatment:

- Eliminated the disease-causing T cells that attack the pancreas
- Increased the number of beneficial regulatory T cells (Tregs)
- Restored the ability of the pancreas to secrete insulin for a time

Notably, these participants had been living with type 1 diabetes for an average of 15 years.

These results are also strengthened by data from one placebo-treated patient who, after enrollment, unexpectedly developed Epstein-Barr virus (EBV) infection and then experienced the same beneficial effects as those who were treated with BCG. Like BCG vaccination, EBV infection boosts the level of tumor necrosis factor (TNF) in the body, which we believe is the mechanism through which BCG treatment benefits human type 1 diabetes.

In addition to these exciting results, we are also happy to announce the launch of the Phase II study. A major gift from the Iacocca Foundation, the support of other philanthropic organizations and the thousands of individuals who have generously donated money have helped us reach the \$8.5 million needed to support the first year of work. Although study participants won't be enrolled until next year, the trial team is now able to begin all of the necessary work that will lead up to patient enrollment. The Phase II study will validate the Phase I findings in a larger study. It will also look at what doses of BCG are needed to make this a functional and sustained type 1 diabetes therapy, and how frequently it would need to be given.

A total of \$25M will be needed to support all three years of the Phase II program. Please consider making a donation at www.faustmanlab.org to sustain this momentum.

Sincerely,

