

Updates

Fall 2017



MASSACHUSETTS
GENERAL HOSPITAL



HARVARD
MEDICAL SCHOOL

from the Faustman Laboratory at Massachusetts General Hospital

A Note from Dr. Faustman

Another year has passed too quickly, but we are happy to share a number of major updates. We continue to move forward with the clinical trial “for the people, by the people.” Our goal is to prove the hypothesis that the BCG vaccine can reverse type 1 diabetes and provide a safe and affordable treatment option for this disease.

Our work is part of growing global consortium of researchers who are testing BCG in the treatment and prevention of a variety of autoimmune diseases, including type 1 diabetes and multiple sclerosis.

If BCG is approved by the U.S. Food and Drug Administration,



it will not be sold by a pharmaceutical company, but rather it will be made available based simply on the price to produce. We have gone to great lengths to establish labeling and manufacturing to ensure that BCG will remain available and affordable. It also means we need to fund this research with donations from individuals and private philanthropies.

We hope you will help us continue to move this important work forward. Please contact us if you have any questions:

diabetestrial@partners.org.

Thank you again!

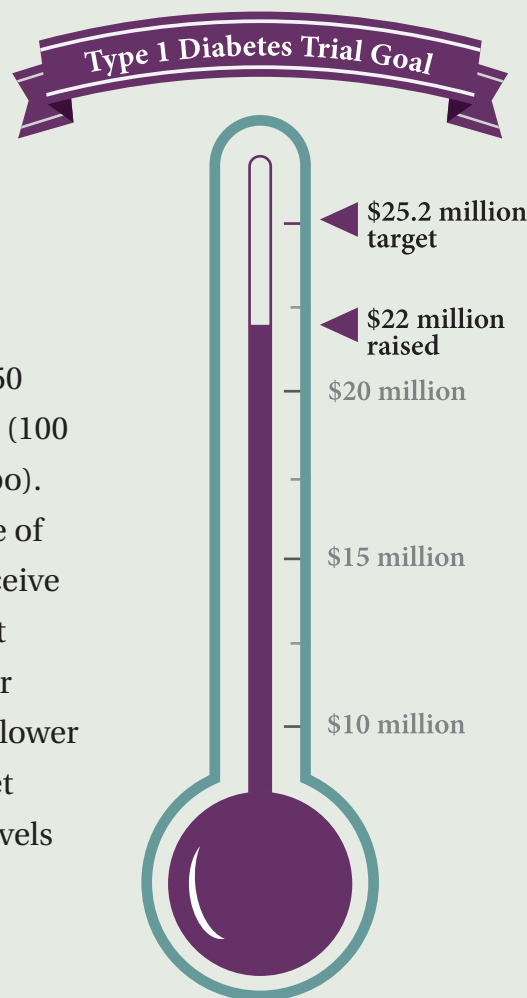
Sincerely,

Denise L. Faustman, MD, PhD

Phase II Trial Updates

We now have over \$22 million raised for the Phase II clinical trial and are working toward our \$25 million goal.

We are also pleased to announce that the Phase II trial is fully enrolled as of the summer of 2017, with 150 volunteers with type 1 diabetes taking part in the trial (100 who will receive BCG and 50 who will receive a placebo). All of the BCG patients have received at least one dose of BCG. Over the next five years, the participants will receive a total of six vaccinations: two vaccinations in the first year and one vaccination a year for the remaining four years. The primary endpoint will be to see if BCG can lower HbA1c. HbA1c is the measure that clinicians use to get an overall picture of what your average blood sugar levels have been over a period of weeks/months.



New Data Coming from Phase I Trial Placebo Group

As part of moving from Phase I to Phase II clinical testing, we were able to give BCG to the three patients who received placebo in the Phase I trial under a special “open label” study. Data from these patients, along with long-term follow up data from the BCG-treated patients who participated in the Phase I trial, will continue to inform us of the efficacy of BCG.

We Are Seeking Patients for Future Study

Our Phase II trial is fully enrolled, but we are still looking for patients for future study groups. For the Phase II trial, the FDA recommended we test in a narrowly defined population of patients with type 1 diabetes, one that has similar characteristics to the patients we enrolled in the Phase I trial (i.e., similar ages, years since diagnosis, etc.). That does not mean we think BCG will only work in those patient groups. We are actively planning and designing new cohorts and study groups. If you are interested, please contact the lab at diabetestrial@partners.org.

BCG Working Group and Web Site

The global consortium of BCG researchers we helped organize has met again, during our third biennial “BCG and TNF Signalling in the Treatment and Prevention of Autoimmune Diseases” conference, held on October 7th in Athens, Greece. At the conference, we shared updates on BCG trials and on the effects that BCG may have on the immune system that could prevent and reverse autoimmunity. We also launched a new web site for the working group, www.bcgandautoimmunity.org.

Updates on How BCG Changes the Immune System

Over the past year, Dr. Faustman has presented updates on the BCG clinical trial at three international diabetes conferences:

- Immunology of Diabetes Society
- The American Diabetes Association
- European Association for the Study of Diabetes

One of the key updates was new data on the mechanism by which the BCG vaccine may restore the proper immune response to the insulin-secreting islet cells of the pancreas. In their research, Dr. Faustman and team show that BCG may induce a permanent increase in the expression of genes that restore the beneficial regulatory T cells (Tregs) that prevent the immune system from attacking the body's own tissues.



“We and other global efforts have known for some time that restoring beneficial Treg cells might halt the abnormal self-reactivity in type 1 diabetes and other autoimmune diseases, but therapies to restore this immune balance have not achieved long-lasting results,” Dr. Faustman stated at the American Diabetes Association conference. “The discovery that BCG restores Tregs through epigenetics – a process that modulates whether or not genes are expressed – is exciting. This now provides a better idea of how BCG vaccination appears to work by powerfully modulating Treg induction and resetting the immune system to halt the underlying cause of the disease.”

Review Article Published

Recently, Dr. Faustman published a new review article in *Diabetes/Metabolism Research and Reviews* on the role of BCG and TNF in type 1 diabetes. The article is called “TNF, TNF Inducers and TNFR2 Agonists: A New Path to Type 1 Diabetes Treatment.” It provides a great overview of our work and the potential of BCG:

<http://onlinelibrary.wiley.com/doi/10.1002/dmrr.2941/full>

More Data on Long-Term Pancreatic Function

One of the key learnings from the Phase I clinical trial has been the ability of type 1 diabetics to keep producing insulin years after diagnosis. This was first discovered and published by the Faustman Lab in 2012. Multiple labs have now confirmed these results and expanded on what we know about the importance of blood sugar control, insulin production and outcomes in type 1 diabetes. This information has been vital as we begin to design new treatment groups for future studies. So, remember, your pancreas is still working long after you are diagnosed.

Prince of Egypt Event

On October 12th, Stephen Schwartz hosted a special fundraising event in California as part of the world premiere of his new musical, Prince of Egypt, at the Mountain View Center for the Performing Arts in Mountain View, CA. This was our third event with Mr. Schwartz following our fantastic Pippin event in 2013 and Schikaneder event in 2016.



Drive to Cure Diabetes

Kathy Kerney and her team put together another incredible golf tournament. This year was the ninth tournament they have organized, and the date is already set for next year. Mark your calendar for July 30, 2018.

[facebook.com/
drivetocurediabetes/](https://facebook.com/drivetocurediabetes/)



Overcoming Obstacles for Diabetes

Some of our more adventurous supporters convened in Hingham, MA on October 22nd for the annual Renegade Run. Participants overcame a four mile obstacle course to help raise funds for our research.

<http://typeonerenegaderun.com/>

Seeking more information about this type 1 diabetes research?
Visit www.faustmanlab.org or Email: DiabetesTrial@partners.org.

How You Can Help

Please consider making a tax-deductible donation to sustain the momentum of this type 1 diabetes research program. Every gift makes a difference.

1. To make a secure online donation, visit www.faustmanlab.org and click on "Support."
2. You may make a gift by check (**payable to "Massachusetts General Hospital"**) and mail your check to:

*Diabetes Clinical Trial
c/o Dr. Denise Faustman
Immunobiology Laboratory
Massachusetts General
Hospital-East
Building 149, 13th Street, CNY-3601
Charlestown, MA 02129*

On the memo line of your check, please write: "Faustman T1D research."

Thank you for joining us in the fight against diabetes!

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