

Updates

Fall 2015



MASSACHUSETTS
GENERAL HOSPITAL



HARVARD
MEDICAL SCHOOL

from the Faustman Laboratory at Massachusetts General Hospital

A Note from Dr. Faustman

This has been an incredible year. In June, our Phase II trial protocol was approved by the U.S. Food and Drug Administration (FDA), allowing us to fully launch the Phase II trial testing BCG in type 1 diabetes. The five-year trial will investigate whether repeat BCG vaccinations can improve type 1 diabetes in patients with longstanding disease.

Since that time, we've received literally thousands of emails and phone calls from individuals wanting to participate in the study. What an amazing response! We are still working to respond to each person that has contacted us, and we have already enrolled many patients into the study.

In total, we anticipate enrolling approximately 150 people in the Phase II study. These will be adults who still have detectable levels of insulin secretion from the pancreas. Those who fulfill such criteria usually have had type 1 diabetes for 25 years or less.

Many have been asking why we will be studying this specific population of people with type 1 diabetes. Why not patients with 40 years of diabetes? Why not children? One of the biggest reasons is that these are the same characteristics as the patients in our Phase I study. By studying patients with the same characteristics, we will be able to submit combined data from the Phase I and Phase II trials to the FDA to seek drug approval. We believe that this approach will make this program advance more quickly towards the goal of bringing BCG forward for type 1 diabetes. As we progress in the Phase II trial, we will work closely with the FDA to discuss including other groups of patients in additional BCG studies.

Thank you to everyone who has helped us launch this trial—whether by spreading the word about this research, by offering financial support or simply by



letting us know how much this program means to you. Every kind word and shared story has been an inspiration to us here at the lab.

If you have questions about the Phase II trial or other aspects of the BCG research program, please don't hesitate to email us at diabetestrial@partners.org.

Thank you again!

Sincerely,

Denise L. Faustman, MD, PhD

Update on the BCG and Autoimmunity Working Group

The BCG and Autoimmunity Working Group is a global coalition of researchers investigating the use of BCG and similar immune boosting strategies to prevent or reverse autoimmune diseases. As part of this group, Dr. Faustman and other experts met this October to discuss research and progress in type 1 diabetes, multiple sclerosis, Sjögren's syndrome and other forms of autoimmunity.

The group last met in 2013 and collaborated on a scientific book, "The Value of BCG and TNF in Autoimmunity," which was published in 2014. With the growing public and research interest in BCG and similar approaches, this year's meeting includes even more investigators and experts who are active and interested in moving this field forward.

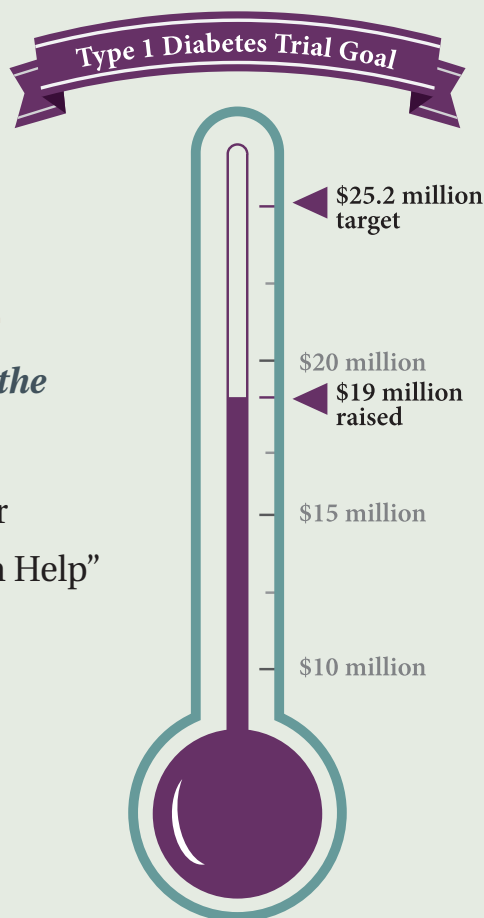
Protecting Patients from Type 1 Diabetes Complications

Our new paper, "Low levels of C-peptide have clinical significance for established type 1 diabetes," was published in the journal *Diabetic Medicine* this summer. In it, we report on our findings that even low levels of insulin production can be protective against the development of diabetic complications and hypoglycemia. This finding is significant in two important ways. First, it suggests that using new tests that can better measure low levels of C-peptide may help doctors identify patients who are at higher risk of complications. It also suggests that helping patients maintain even low levels of insulin production—such as through interventions like the BCG vaccine—can help patients avoid complications and improve blood sugar control. As we progress through the Phase II trial, we hope to expand on these findings.

Help Us Reach Our Funding Goal

We now have over \$19 million raised for the Phase II clinical trial.

To find out how you can help us reach our \$25.2 million goal, please see "How You Can Help" on the last page of this newsletter.



Conquering Diabetes One Mountain at a Time

Rick Noble has been supporting the Faustman Lab's research for over a decade. His inspiration is his daughter, Kate, who was diagnosed with type 1 diabetes in 1992, when she was only 1 year old.

"Our doctors at the time said that we would be hearing about a potential cure in about a decade," said Rick. "To mark the end of that decade, we launched Kiss the Sky (KTS) to Conquer Diabetes in 2002, climbed Mount Whitney in California and pledged to keep climbing until we conquered diabetes. We joined the effort to help finance the Faustman Lab's human clinical trials in 2004, and have been part of it ever since."

Since that first climb, KTS teams have climbed mountains from Washington state to Bolivia. They have also held numerous other fundraising events—from dinners featuring mountaineers as guest speakers to a hike around the monuments in Washington, DC. This summer, the KTS team headed back to California to climb 14,042-foot Mount Langley.

"Our team included my daughter Sarah, friends Lindsay, Mackenzie and Taylor Kingston, and their mother Heather Pierce, all making their first attempt at a fourteener (a mountain 14,000 feet or more in elevation). My wife Karen and daughter Kate helped

send the team off, and then hiked to Rainbow Falls," said Rick. "We brought prayer flags with quotes from other KTS climbs, including one of my favorites from T.S. Eliot—'Only those who will risk going too far can possibly find out how far one can go.'" This latest effort raised more than \$50,000 to help the Faustman Lab conquer diabetes.



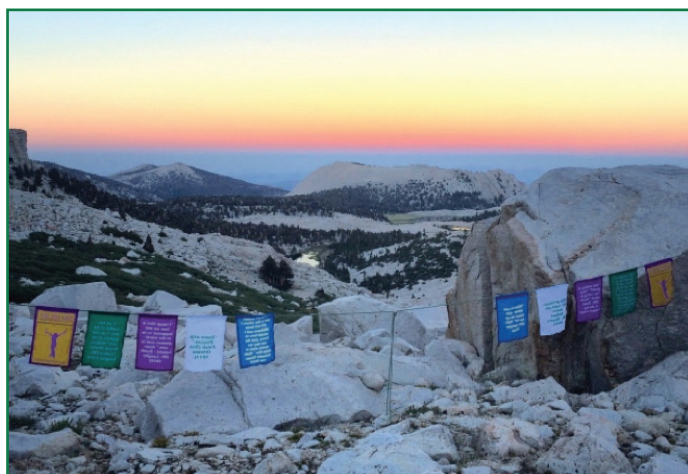
The team reached the summit under clear blue skies.

Spotlight on Some of Our Dedicated Supporters

There are so many people who care about this research and who work hard to make sure we continue to move forward. (You may have read some of their stories in our "Spotlight on a Supporter" series on Facebook.) We want to take this opportunity to acknowledge a few groups that have fundraising activities every year on behalf of our Lab, each donating over \$20,000:

- Brendan's Brigade - Annual bike ride put together by the Buono family in honor of their son, Brendan
- Drive to Cure Diabetes - Annual golf tournament and auction produced by the Kearney family, in honor of their daughter, Casey
- Mr. & Mrs. Lijewski - In honor of their son, Brian White

Thank you to these individuals and all who have given their time and support to help further a cure for type 1 diabetes!



Prayer flags with inspirational sayings brought by the KTS team.

Phase II BCG Clinical Trial Program

Led by Dr. Denise Faustman, the Phase II clinical trial is a randomized, double-blinded, study being conducted at Massachusetts General Hospital. It will include approximately 150 people with type 1 diabetes, ages 18-65. The purpose of this study is to see if repeat injections of bacillus Calmette-Guérin (BCG) are beneficial in longstanding type 1 diabetes. Published Phase I data on repeat BCG vaccinations in long-term diabetics show that BCG can specifically eliminate disease-causing “bad” white blood cells (misprogrammed cells that attack and kill the insulin-producing cells of the pancreas) while also temporarily restoring a small degree of insulin secretion. In the Phase II study, participants will receive more frequent injections to see if these desirable effects can be sustained.

Eligible volunteers 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 any beneficial changes in autoimmune status (such as continued elimination of bad white blood cells).

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

Seeking more information about this type 1 diabetes research?
Visit www.faustmanlab.org.

Have questions about participating in the research?
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: “Type 1 diabetes research.”

Thank you for joining us in the fight against diabetes!

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