

Updates

Fall 2016



MASSACHUSETTS
GENERAL HOSPITAL



HARVARD
MEDICAL SCHOOL

from the Faustman Laboratory at Massachusetts General Hospital

A Note from Dr. Faustman

Great things have happened this year! Most importantly, we began enrolling participants in our Phase II trial. This double-blind, randomized, placebo-controlled study in 150 participants will continue to investigate the ability of the BCG vaccine to reverse type 1 diabetes. Supported entirely by philanthropy, it is a clinical trial by the people, for the people.

With this trial, I hope that we will find a way to stop the body from destroying the insulin-producing cells of the pancreas and help the body restore blood sugars to normal levels. A decade ago, that may have seemed unachievable, but I believe it is a goal that we are coming closer to every day.

We are very proud to be doing this work and thank all who

have helped us get here. We are touched by all of the individuals and families we hear from and see who are affected by type 1 diabetes, from the patients that donate blood for our research to those who send in messages of support. The dedication and energy we are giving to this project is matched—if not exceeded—by the dedication and energy of our supporters. Thank you all!

On a more sober note, we are very deeply saddened each time we learn about a life lost to type 1 diabetes complications. As you read further, you will see that this is something we touch upon in one of the updates in this newsletter. The loss of life caused by this disease makes our research goals very real to us and very clear – we must find a way to stop type 1 diabetes.



I know that we all share this hope, and the Faustman Lab will continue to work towards this goal.

Please contact us with any questions at diabetestrial@partners.org.

Thank you again!

Sincerely,

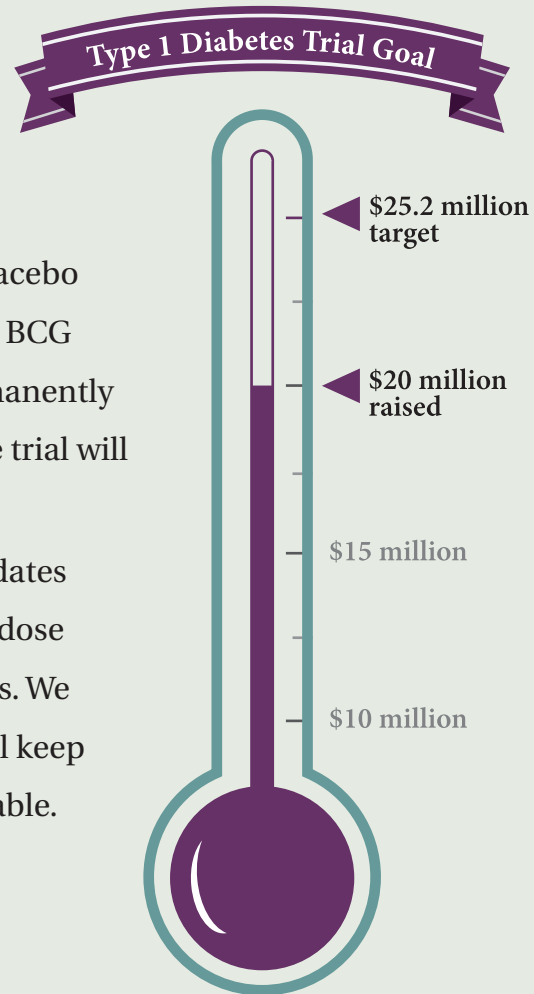
Denise L. Faustman, MD, PhD

Phase II Trial Updates

We now have over \$20 million raised for the Phase II clinical trial.

The Phase II trial is a double-blind, randomized, placebo controlled study that will investigate the ability of the BCG vaccine to help those living with type 1 diabetes permanently lower their blood sugars. The primary endpoint of the trial will be to see if BCG helps to lower HbA1c.

To date, we have enrolled over 130 of the 150 candidates that we need for the trial, and have given at least one dose of the BCG vaccine or placebo to over 100 participants. We will follow participants over a five-year period. We will keep everyone updated with trial updates as they are available.



BCG Trials in Multiple Sclerosis Moving Forward in Italy

Our colleagues in Italy are moving forward with a Phase III trial testing BCG in patients with multiple sclerosis (MS). Led by Dr. Giovanni Ristori at Sapienza University in Rome and supported by the Multiple Sclerosis Society of Europe, the trials will look at the clinical effect of BCG vaccinations in patients with early signs of MS. This is a follow up to their Phase II study, which showed that even a single dose of BCG stopped multiple sclerosis progression in over half of patients compared to placebo.

Long-Term Follow Up of the Phase I Trial Continues

We continue to follow the progress of the patients who participated in the Phase I BCG study. All patients who were randomized to receive placebo during the blinded portion of the Phase I study have now received two doses of the BCG vaccine, just as the patients randomized to BCG did. We expect to publish data on our long-term follow up of all of the Phase I patients in 2017.

Faustman Immunobiology Lab Research Identifies & Corrects Regulatory T Cell (Treg) Defects in Type 1 Diabetes.

Regulatory T cells or “Tregs” are a type of white blood cell that helps regulate the immune system, and they normally should prevent autoimmune conditions like type 1 diabetes. In our latest type 1 diabetes research paper, we identified a defect in the Tregs of people with type 1 diabetes. Using human blood samples (thanks to everyone who donates blood for our research), we showed how tumor necrosis factor (TNF) is one way to correct this Treg defect. Specifically, TNF treatment of type 1 diabetic Treg cells “awakens” the Tregs so that they become potent and functional again, fighting the cells that cause autoimmunity. Part of what we hope to do in our trials with the BCG vaccine (which is a known way to stimulate the body to produce TNF) is to awaken these cells to put up a fight. Our paper was published online in *Nature Clinical & Translational Immunology* in January this year.



Dr. Faustman and Charlotte, a young supporter whose very successful rollerskating fundraiser helped benefit the Faustman Lab’s research.

Kiss the Sky to Conquer Diabetes: A Tribute to Murphy (Murph) Roberts

We featured the amazing efforts of Kiss the Sky to Conquer Diabetes (KTS) in our newsletter last year. For those who don’t know, KTS is a mountain climbing group led by Rick Noble. They joined the effort to help raise awareness for and finance our human clinical trials in 2004, and they have since taken on some incredible climbing challenges in support of these goals.

This summer, KTS took on the *Six-Pack of Peaks Challenge*, which Rick described as “6 peaks, 82 miles, over 27,000’ of vertical gain and one epic challenge to conquer diabetes!”

Right before setting out on the fifth and biggest climb of the challenge, the KTS team sadly learned of the passing of Murphy (Murph) Roberts, the brother of a friend of Rick’s daughter, Sarah. While hiking in Utah, Murph experienced a seizure related to his type 1 diabetes. The seizure caused a fall that resulted in a series of complications, leading to his death.

Murph, a member of the class of 2017 at Middlebury College in Vermont, is remembered as a great student and person, and an avid adventurer. He was also passionate about finding a cure for type 1 diabetes. As a tribute to Murph and with the blessing and support of Murph’s family, the KTS team has renewed their pledge to “keep climbing until we conquer type 1 diabetes.”

New Musical from Stephen Schwartz Benefits Our Lab's Research

In September, musical theater lyricist and composer Stephen Schwartz together with type 1 diabetes advocates Barbara and Michael Herscovici and their daughter Zoé (an upcoming musical performer) hosted a fundraising event for the Faustman Lab at the Raimund Theater (managed by "Vereinigte Buehnen Wien") in Vienna, Austria.

One hundred donors to the lab were greeted by Stephen Schwartz, Barbara, and Zoé. The guests enjoyed the preview show of Stephen's new musical, "Schikaneder," which premiered September 30th. At the after-show reception, Stephen gave a special performance for the guests, who also had the opportunity to mingle with the cast.



Photo: Rolf Bock

The Herscovici family with Stephen Schwartz and cast member at a fundraising preview of Stephen's new musical "Schikaneder."

Interview with *Diabetes in Control*

Diabetes in Control recently published a three-part interview with Dr. Faustman. In the interview, Dr. Faustman discusses why the BCG vaccine may lead to a cure for type 1 diabetes, the past challenges of dosing BCG correctly, and how a cure based on the inexpensive BCG vaccine could permanently change type 1 treatment. You can find the full 3-part interview at www.faustmanlab.org in our "News" section, under "Media Coverage."

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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