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MGH Initiates Phase I Diabetes Trial

BOSTON, MA – Scientists at the Massachusetts General Hospital (MGH) have initiated a phase 1 clinical trial to reverse type 1 diabetes. The trial is exploring whether the promising results from the laboratory of Denise Faustman, MD, PhD, can be applied in human diabetes. Faustman’s previous studies have shown that mice with a form of diabetes that closely resembles type 1 diabetes in humans can be cured. In the animal studies, a commonly used vaccine that provides protection against tuberculosis, called Bacillus Calmette-Guerin (BCG) was used effectively to deplete the abnormal immune cells that attack and destroy the insulin producing cells of the pancreas. The first step in the human study, which is currently enrolling volunteers, is to determine whether the same strategy using BCG vaccination can be used to modify the abnormal autoimmune cells that are present in type 1 diabetes, sometimes called “juvenile-onset” diabetes.

“We are pleased to be starting human clinical trials,” said Faustman. “Human trials take time, but we are making the step from curing diabetes in mice to determining whether it will work in men and women with diabetes.”

Type 1 diabetes usually starts during childhood or adolescence and can cause a variety of severe complications including kidney failure, loss of vision, amputations, heart disease, and strokes. It occurs when a person’s immune system attacks and destroys the insulin-producing cells in the pancreas. In the absence of insulin, which is necessary for sugar and other nutrients to enter cells, blood sugar levels rise. The risk for developing complications is closely linked to the elevated blood sugar levels over time. If blood sugar levels are well controlled, the long-term complications can largely be avoided. However, the so-called intensive therapy that is required to maintain near-normal sugar levels requires life-long demands on the patient, including frequent blood sugar monitoring and at least 3 daily injections of insulin or use of an insulin pump, along

with restrictive diets. Insulin doses must be adjusted based on blood sugar levels, dietary factors, and anticipated exercise. Thus, a cure for diabetes has been highly sought after and has attracted much research interest.

The clinical trial is using the BCG vaccine for several reasons. BCG has been used safely for nearly 80 years as a tuberculosis vaccine. It is now being used in the human trial because it causes a low-grade inflammatory reaction, which in the mouse model of autoimmune diabetes lead to the destruction of the abnormal autoimmune cells.

David M. Nathan, MD, director of the MGH Diabetes Center, who is leading the human study at MGH, provides context, “This is the very first step in what is likely to be a long process in achieving a cure. We first need to determine whether the abnormal autoimmune cells that underlie type 1 diabetes can be knocked out with BCG vaccination, as occurred in the mouse studies.”

The Phase I trial is being supported largely through direct and fundraising support from the Iacocca Foundation, and through support from other donors and the Massachusetts General Hospital. The Iacocca Foundation was founded by Lee Iacocca and his family in 1984 to fund innovative approaches to a potential cure for diabetes. Trial information is available to the public at www.faustmanlab.org.

About the Iacocca Foundation

The Iacocca Foundation has been a leader in the battle against diabetes for over 20 years. The foundation has granted more than \$27 million to innovative and promising research designed to lead to a cure for diabetes and alleviate its complications. The Foundation was established by Lee Iacocca after his wife, Mary, died from complications of type 1 diabetes.

About the Massachusetts General Hospital

Founded in 1811, the MGH is the third oldest general hospital in the United States and the oldest and largest in New England. The 900-bed medical center offers sophisticated diagnostic and therapeutic care in virtually every specialty and subspecialty of medicine and surgery. Each year the MGH admits more than 46,000 inpatients and handles nearly 1.5 million outpatient visits at its main campus and health centers. Its Emergency Department records nearly 80,000 visits annually. The surgical staff performs more than 35,000 operations and the MGH Vincent Obstetrics Service delivers more than 3,500 babies each year. The MGH conducts the largest hospital-based research program in the country, with an annual research budget of approximately \$500 million. It is the oldest and largest teaching hospital of Harvard Medical School, where nearly all MGH staff physicians serve on the faculty. The MGH is consistently ranked among the nation’s top hospitals by *US News and World Report*.

