

Conquering Diabetes with Sweet Success

By Megan McDermott

Massachusetts General Hospital is pleased to highlight the clinical Trial success of the Faustman laboratory to reverse even established Type 1 diabetes.

Life After Insulin: The Faustman Lab Sets its Sights on a Vaccine for Type I Diabetes

For decades, patients with type I diabetes have been assured that a cure would be just around the corner, but such promises have always led to disappointment. A unique clinical research project underway at Mass General might finally change all that — reversing, even preventing, the autoimmune disorder and weaning type 1 sufferers off insulin for good.

Director of the MGH Immunobiology Lab Denise L. Faustman, MD, PhD, has teamed up with David Nathan, MD, director of the MGH Diabetes Center and the MGH Clinical Research Center, in a decades-long investigation that found that they could kill off a subpopulation of the disease-causing T-cells in lab mice by temporarily elevating levels of a natural protein found in the immune system called tumor necrosis factor (TNF). At the same time that TNF destroys “bad” T-cells, healthy islet cells appear to flourish. Protected from the devastation of an immune system gone bad, the pancreas in a mouse with end-stage disease seemed to regenerate on its own, virtually reversing type I diabetes.

TNF, unfortunately, is not readily available in drug form, but an effective substitute now seems to be one long-heralded by medical experts to be the safest vaccine ever developed. Bacillus Calmette-Guérin, or BCG, is administered as a tuberculosis (TB) vaccine. BCG is cheap to produce and, in small doses, it temporarily stimulates the body’s production of TNF. The question remained, however: TNF cured type I diabetes in mice — but will it work in people?

By June 2010, the Faustman lab had completed phase I of planned clinical trials, proving that multiple, low-dose injections of the BCG vaccine were safe in people with type 1 diabetes. The success of phase I clears the way for planning the larger phase II trials. In phase II, Dr. Faustman’s team will continue tracking the effectiveness of BCG as a therapeutic agent for advanced type I diabetes as they tinker with the



exact dose and timing of vaccine administration. If all goes well, a third-phase trial will test the drug in even larger groups of patients across the country.

The most formidable challenge to the Cure Diabetes Now Project, as it is known, is a financial one, according to Dr. Faustman. Volunteers for the trial are lining up around the block, but the three-year phase II program requires, among other things, a highly specialized staff of experts, contracts for the manufacturing of BCG for type 1 diabetes, the establishment of a Data Safety Monitoring Board and a dedicated, large-scale automated blood handling process at the Faustman lab.

“Fundraising for Cure Diabetes Now has been an amazing, international effort,” says Dr. Faustman. “Our benefactors range from the Iacocca Foundation to grassroots philanthropists — kids holding bake sales and people climbing mountains to raise money for us. Every dollar counts on the way to our \$25 million goal.”