RESEARCH FRONTLINE

The JDRF Research Newsletter

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## JDRFI Partner Bayhill Enters Collaboration with Genentech to Develop Type 1 Vaccine

For the fourth time in 18 months, one of JDRFI's biotechnology partners has signed a collaboration agreement with a large pharmaceutical company to move research on type 1 diabetes into the final phases of clinical testing.

This summer, JDRFI industry partner Bayhill Therapeutics Inc., based in California, entered into an exclusive agreement with Genentech, Inc., a wholly owned member of the Roche Group, to further develop and potentially commercialize a novel immunotherapeutic designed to reverse the immune response that causes type 1 diabetes. The development is the latest success in JDRFI's innovative Industry Discovery and Development Partnership (IDDP) program. In IDDP grants, JDRFI provides early stage research funding to biotech companies pursuing technologies and therapeutics that could cure, treat, or prevent type 1 diabetes and its complications.

Bayhill's immunotherapy, an antigen-specific DNA vaccine, is currently being tested in phase I/II clinical trials supported by JDRFI. Antigens are substances, usually proteins, that trigger an immune response; the goal of designing a vaccine that is antigen-specific to type 1 diabetes is to selectively turn off the autoimmune response that attacks the pancreas and causes diabetes.

In just four years, JDRFI has awarded more than $29 million (US) in research funding to 25 companies through its IDDP program. JDRFI supported the current Bayhill clinical trial with an IDDP grant in October 2008.

"We began the IDDP program to help small companies demonstrate proof-of-principle for innovative ideas and products to treat and cure type 1 diabetes, in order to attract funding for potential commercialization from large drug companies," said Richard Insel, executive vice president of research at JDRFI. "This latest agreement further demonstrates that the strategy is successful in accelerating the pace of research leading to a cure for diabetes."

Dr. Insel noted the development underscores JDRFI's unique role in helping to accelerate and translate research discoveries into cures and better treatment of type 1 diabetes and its complications. By supporting early stage research at small biotech companies, JDRFI looks to lower the risk for larger pharmaceutical companies, which can then move to create biotech partnerships and carry the products through expensive, late-stage trials and regulatory approvals. The goal is to bring cures and treatments for type 1 diabetes to market faster.

Under the terms of the agreement, Genentech will make an upfront payment of $25 million (US) in cash and equity, with additional development, regulatory, and sales milestone payments potentially exceeding $325 million (US). Bayhill will also receive royalties on sales of any product that is developed. Bayhill will be responsible for completing the ongoing phase I/II trial, while Genentech will be responsible for all future research, development, manufacturing, and commercialization efforts.

If this partnership leads to the successful commercialization of a cure or treatment for type 1 diabetes, JDRFI shares in the financial results of that process, enabling the Foundation to recoup its support of those projects and fund other research programs leading to a cure.

Other IDDP partners that have created similar agreements include Tolerx, based in Massachusetts, which has joined with GlaxoSmithKline to develop an anti-CD3 antibody to preserve beta cell function in newly diagnosed patients; Maryland based MacroGenics, which is developing a similar antibody with Eli Lilly & Co.; and Canada's Transition Therapeutics, which has signed a commercialization agreement for a beta cell regenerative therapy with Lilly.

### Clinical Trial Evaluations

The Bayhill trial uses an antigen-specific DNA vaccine designed to reverse the underlying autoimmune component of type 1 diabetes, and slow or stop loss of function of the insulin-producing pancreatic beta cells. In the ongoing trial, begun in 2006, people with type 1 diabetes receive a weekly injection of one of four increasing doses of the vaccine (or placebo) for 12 weeks.

The vaccine is designed to selectively turn off the autoimmune response against insulin. This highly specific

immunemodulating therapy has the potential to preserve pancreas function, and could lead to improved long-term health in type 1 patients. The compound was efficient in animal trials.

### The 69th ADA Scientific Sessions

Interim results of the trial were presented earlier this year at the American Diabetes Association's annual scientific sessions. The data, which included assessments of pancreatic function as measured by C-peptide and safety profiles, support the vaccine's potential to preserve beta cell function and improve blood sugar control. Data was available ranging from six to 12 months of therapy.

Patients received either 0.3, 1, 3 or 6 mg of the vaccine weekly. Patients at all four dosage levels have experienced a significantly higher preservation of their C-peptide levels, compared to placebo, according to the interim results. The data also showed the vaccine is safe and well tolerated, as no serious adverse events were associated with the treatment. ■

### Key Point:

The new alliance between Bayhill and Genentech is important strategically because it shows the success of JDRFI's innovative efforts to partner with small biotech companies in order to accelerate the pipeline of drugs and treatments for type 1 diabetes. It also underscores scientific consensus on the potential of Bayhill's vaccine as a disease-modifying agent for people with type 1 diabetes.