

***Investment Brief for  
A Treatment to Prevent Type 1 Diabetes***

***NSW  
AREA HEALTH  
SERVICES***

***Office of Commercialisation***

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# ***A Treatment to Prevent Type 1 Diabetes***

## ***Summary***

Type I diabetes is mediated by the destruction of beta cells. Proteins which control the two major processes implicated in the pathogenesis of Type I diabetes have been identified and characterized. This protein has been shown to be naturally occurring in the body and to be well tolerated in higher doses.

## ***Market***

Type I diabetes occurs widely, especially in children, and currently affects about 4.9 million people worldwide. Its incidence in children under 5 has grown by 30% in the last 5 years. This technology would target the at-risk portion of the population with the potential to prevent or delay the onset of the condition.

## ***Benefits***

The treatment of Type I diabetes meets an unmet need, as to date there are no effective treatments against this disease, which is increasing in prevalence.

## ***The Opportunity***

For the first time, we have found strong evidence that a known protein prevents or delays the onset of Type 1 diabetes in an effective animal model for the disease.

Drs Chris Jackson and Meilang Xue's research at Royal North Shore Hospital and the University of Sydney, has found the ideal candidate to prevent Type 1 diabetes. The protein has the potential to prevent the immune and inflammatory destruction of beta cells, the two major processes implicated in the development of the disease, and it has a proven track record as a safe therapeutic agent.

Northern Sydney Central Coast Area Health Service is seeking to license the technology to a drug development company interested in taking the project to clinical trial stage.

## Background

Type 1 diabetes is believed to be caused by interplay between genetic susceptibility and environmental factors. The disease is lifelong and incurable, striking people in their childhood years and making them insulin dependent for life.

### **What are the current and future market opportunities?**

Type 1 diabetes accounts for up to 15% of all diabetes and currently affects 4.9 million people around the world. Young relatives of the 4.9 million people with Type 1 diabetes are more likely to get the disease, and if they have antibodies that indicate an autoimmune attack against the insulin-producing cells in their own pancreas, their risk is very high.

This is the 'highly at risk' target market for applying the protein to prevent the onset of type 1 diabetes. By taking advantage of the relatively long pre-diabetic period, their diabetes related autoantibodies can be measured and if found to be in a pre diabetic state, treatment with the protein can be given to prevent onset of the disease.

The Type 1 diabetes market is growing with a 30% increase in children under 5 years developing the disease in the last five years.

### **What is the competition?**

There is still nothing on the market to prevent Type 1 diabetes. Competing approaches have had mixed success. Trials of antigen based treatment with insulin in the 1990's and the nicotinamide trial reported in 2002, did not, overall, slow the progression of diabetes.

Recently, other potential therapeutic agents such as an antibody to the CD3 receptor, an antibody to prevent RAE-1 from binding its receptor on the CD8 lymphocytes, dendritic cells to stimulate T-cells, and a nasal insulin spray are showing more promising results to prevent diabetes.

Other agents have been reported to prevent or delay the onset of diabetes in animal models. These include active Vitamin D, a green tea derivative -epigallocatechin gallate, streptozotocin-treated islet cells, and a diet containing low advanced glycolation end products. The effect of these agents is yet to be tested in human diabetes.

### **What qualities of the product make it unique or provide a competitive edge over other potential technologies in the market place?**

In contrast to the current approaches which focus on the immune response in diabetes, this protein is unique in that it not only targets the abnormal immune response but also directly inhibits inflammation, which is associated with insulinitis. Thus, the protein controls the two major processes implicated in the pathogenesis of Type1 diabetes, inflammation and auto-

immunity. This explains how it dramatically reduced blood glucose levels and prevented diabetes in non-obese diabetic mice.

Unlike many of the other proposed agents to prevent diabetes, this protein has a proven track record as a safe therapeutic agent, a very important consideration when treating children.

The protein is a naturally occurring protein circulating in the blood and it can be produced in large quantities using recombinant techniques. It is well-tolerated when used to treat patients with a severe illness at relatively high doses. Its safety data indicates that it may also be suitable for use in high-risk pregnancies which develop gestational diabetes.

### **Has the technology been proven?**

The protein has been proven in vitro and in vivo in a highly regarded animal model for the disease, the non-obese diabetic mouse model. It has been shown to prevent the immune and inflammatory destruction of beta cells and has a proven track record as a safe therapeutic agent.

It has yet to be trialed in humans to prevent Type 1 diabetes.

### ***Patent Pending***

An International (PCT) Patent Application No PCT/AU2006/000009 was filed on 9 January 2006 with priority from Australian Provisional Patent Application No 2005 900073 "Treatment for Autoimmune and Inflammatory Conditions " filed by Northern Sydney Central Coast Area Health Service on 7 January, 2005.