



Press release, March 10, 2015

## **The diabetes vaccine Diamyd<sup>®</sup> in new study to stop type 1 diabetes in children**

*Diamyd Medical (Nasdaq Stockholm First North, Ticker: DMYD B) informs that DiAPREV-IT 2 has started. In this new study, the second of its kind, the diabetes vaccine Diamyd<sup>®</sup> is tested to prevent or delay the onset of type 1 diabetes in children at very high risk of presenting with the disease.*

The first participant out of 80 has now been included in DiAPREV-IT 2, a placebo controlled Phase II study where the diabetes vaccine Diamyd<sup>®</sup> is administered with the aim of preventing or delaying type 1 diabetes. The children participating in the study are seemingly healthy, but have been identified to have a very high risk of presenting with type 1 diabetes. The participants will also be supplemented with Vitamin D with the aim of strengthening the efficacy of the diabetes vaccine.

The autoimmune process causing type 1 diabetes starts before the appearance of any clinical symptoms of the disease. Years or months prior to symptoms of insulin deficiency the immune system has already started to attack the insulin producing cells in the body. During this period there are not yet any noticeable symptoms of the disease but the autoimmune process can be detected by screening for certain markers in the blood. In large screening studies at Lund University, Sweden, children with these markers have been identified and those children will thus most probably present with symptomatic type 1 diabetes.

“We have noted a great interest to participate in the study, and there are many candidates in line,” says Helena Elding Larsson, pediatrician in Malmö and researcher at Lund University but also the lead investigator and sponsor for the study. “It is very satisfying to be able to offer these children a study with the aim of preventing or delaying the disease process.”

“Through a blood sample, it is today possible to identify individuals in which the autoimmune process leading to type 1 diabetes has started,” says Åke Lernmark, Professor of Experimental Diabetes Research at Lund University. “It is important to intervene before it is too late and two injections with the diabetes vaccine Diamyd<sup>®</sup>, which has been shown to be safe in studies with more than 1000 diabetes patients, seem to be the most promising treatment today.”

The new study, DiAPREV-IT 2, is a complement to the ongoing study DiAPREV-IT. When the children are included in the study they will be stratified according to which of the early stages of the autoimmune process leading to type 1 diabetes they are in. The first stage comprises children with two or more auto-antibodies directed at their own insulin-producing cells, but with normal glucose metabolism. The second stage comprises children with both auto-antibodies and impaired glucose metabolism. Vitamin D supplement is given to down-regulate the immune system’s inflammatory components in order to increase the diabetes vaccine’s tolerance-inducing effect regarding the preservation of the body’s insulin-producing capacity.

The study is double-blind and placebo-controlled. Half of the participants will be randomized to receive two injections of Diamyd<sup>®</sup> and half will be randomized to receive placebo (non-active substance), but no one will know which treatment group they are randomized to until the study is completed after 5 years. The children that present with symptomatic type 1 diabetes during the study will receive injections of active Diamyd<sup>®</sup> after diagnosis, regardless of what they received as preventative treatment. This way, the effect of the diabetes vaccine can be monitored also in new-onset patients.

DiAPREV-IT and DiAPREV-IT 2 are conducted by a research team at Lund University and co-funded by research grants. Diamyd Medical is providing the study drug and has the right to utilize the findings of the studies in a potential future market registration.

### **About type 1 diabetes**

Type 1 diabetes is an autoimmune disease where the immune system attacks the patients' own insulin producing beta cells. By analyzing markers in the blood it is possible to identify persons in whom this autoimmune process is ongoing, although has not yet caused clinical symptoms of diabetes. When type 1 diabetes presents with clinical symptoms, patients must be treated daily, for the rest of their lives, with insulin to sustain life. The importance of finding a cure is high for the world's health care systems and the wellbeing of patients. The annual market for an easy to use, successful therapeutic is estimated to several billion dollars.

### **About the diabetes vaccine Diamyd®**

Diamyd® is the world's furthest developed Antigen Based Therapy for preventing, delaying or stopping the autoimmune attack on beta cells in type 1 diabetes and other forms of autoimmune diabetes and thus preserving the body's own ability to produce insulin. The diabetes vaccine Diamyd® is easily administered in any clinical setting and has been used in studies with more than 1,000 diabetes patients and has shown a good safety profile. In a European Phase III study with children and adolescents recently diagnosed with type 1 diabetes, Diamyd® showed an overall 16% efficacy (p=0.10) versus placebo in preserving endogenous insulin secretion. Ongoing development work is aimed at enhancing the efficacy of the treatment by combining Diamyd® with other agents. Five clinical studies with Diamyd® are now ongoing and one is being launched.

- **DIABGAD-1.** A placebo-controlled study, where Diamyd® is being tested in combination with ibuprofen and vitamin D. The study comprises a total of 64 patients between the ages of 10 and 18 recently diagnosed with type 1 diabetes, and will continue for a total of 30 months. The aim of the combination treatment is to preserve the body's residual capacity to produce insulin. All of the participants have been enrolled in the study and the initial six-month results, focusing on immunological markers, are expected to be presented in the spring of 2015. The study runs at nine clinics in Sweden and is led by Professor Johnny Ludvigsson at Linköping University.
- **DIAPREV-IT.** A placebo-controlled study, where Diamyd® is being tested in children with very high risk of developing type 1 diabetes, meaning that they have been found to have an ongoing autoimmune process but do not yet have any clinical symptoms of diabetes. A total of 50 participants from the age of four have been enrolled in the study, which will last for five years. The aim of the study is to evaluate whether Diamyd® can delay or prevent the participants from presenting with type 1 diabetes. The study is taking place in Sweden led by Dr. Helena Elding Larsson at Lund University. Results are expected at the end of 2016.
- **DIAGNODE.** An open label study, where Diamyd® is administered directly into lymph nodes in combination with treatment with vitamin D. The study will comprise five patients between the ages of 18 and 30 who have been newly diagnosed with type 1 diabetes, and will continue for a total of 30 months. The aim of the study is to evaluate the safety of the combination treatment and the effect on the immune system and the patients' insulin producing capacity. The study is taking place in Sweden led by Professor Johnny Ludvigsson and enrolled the first patient in February 2015.
- **DIAMYD®/GABA.** A placebo-controlled study, where Diamyd® is being tested in combination with GABA. The study will comprise 75 patients between the ages of 4 and 18 recently diagnosed with type 1 diabetes, and will continue for a total of 12 months. The aim of the combination treatment is to preserve the body's residual capacity to produce insulin. The study is taking place in the US led by Professor Kenneth McCormick at the University of Alabama at Birmingham. The first patient was included in March 2015.
- **DIAPREV-IT 2.** A placebo-controlled study, where Diamyd® is being tested in combination with vitamin D in children with very high risk of developing type 1 diabetes, meaning that they have been found to have an ongoing autoimmune process but do not yet have any clinical symptoms of diabetes. A total of 80 participants between the ages of 4 and 18 will be enrolled in the study, which will last for five years. The aim of the study is to evaluate whether Diamyd® can delay or prevent the participants from presenting with type 1 diabetes. The study is taking place in Sweden led by Dr. Helena Elding Larsson. The first patient was included in March 2015.
- **EDCR IIa.** An open label study, where Diamyd® is combined with etanercept and vitamin D. The study will comprise 20 patients between the ages of 8 and 18 who have been newly diagnosed with type 1 diabetes, and will continue for a total of 30 months. The aim of the study is to evaluate the safety of the

combination treatment and the effect on the immune system and the patients' insulin producing capacity. The study is taking place in Sweden led by Professor Johnny Ludvigsson and is in the start-up phase.

#### **About Diamyd Medical**

Diamyd Medical is dedicated to fighting type 1 diabetes and to working toward a cure for the disease. Its projects include development of combination regimens with the GAD-based diabetes vaccine Diamyd® for arresting the successive destruction of insulin-producing beta cells. Diamyd Medical has an exclusive license to patent rights held by the UCLA related to the GAD molecule. The company has also an exclusive license from UCLA for GABA for the treatment of diabetes and other inflammation-related conditions.

Diamyd Medical is a shareholder in the stem cell company Cellaviva AB, which is establishing a Swedish commercial bank for private family saving of stem cells in umbilical cord blood and other sources of stem cells. Stem cells are expected to be used in Personalized Regenerative Medicine (PRM), for example, to restore beta cell mass in diabetes patients where autoimmunity has been arrested. Diamyd Medical also has an ownership stake in the US medical technology company Companion Medical, Inc., and a minor shareholding and other financial interests in the US gene therapy company Periphagen Holdings, Inc.

Remium Nordic AB is the Company's Certified Adviser.

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