Press Release, September 14, 2020

Phase IIb topline results demonstrate a significant treatment effect of Diamyd® in a previously identified genetic subgroup of individuals with type 1 diabetes

Diamyd Medical today announced the topline results from the placebo-controlled Phase IIb trial DIAGNODE-2, where the diabetes vaccine Diamyd® (GAD-alum) was injected directly into a lymph node in individuals with recently diagnosed type 1 diabetes. In line with previous large-scale analysis of trials involving subcutaneous administration of Diamyd®, the reults, encompassing a total of 109 patients, showed a statistically significant effect in the predefined HLA (Human Leukocyte Antigen) subgroup of trial participants. Specifically, this trial demonstated a preservation of beta cell function at 15 months post-diagnosis, as measured by meal stimulated C-peptide. The primary endpoint, defined as meal stimulated C-peptide in the entire trial population was not met. Importantly, no related severe adverese events were observed in the trial. Based on these results, Diamyd Medical will pursue the HLA restricted responder subgroup in an upcoming pivotal Phase III program.

"I am very pleased and these results make perfect sense and are aligned with all our previous data", says Ulf Hannelius, CEO of Diamyd Medical. "Supported by these positive results and the superior safety profile, we will now move forward with a Phase III program in this patient population, representing close to one-half of all individuals with type 1 diabetes".

"I am overjoyed by the positive results", says Johnny Ludvigsson, Professor at Linköping University and Coordinating Investigator of the trial. "What we see now is that the GAD treatment works for nearly half of the patients with type 1 diabetes. It is important that this patient group may soon benefit from an effective, safe and convenient treatment that does not suppress the immune system, especially now in the era of COVID-19."

103 out of 109 patients were evaluated as part of the topline results for the primary endpoint: preserving beta cell function at 15 months, as measured by meal stimulated C-peptide. 55 individuals of the 103 patients evaluated received active treatment while 48 received placebo treatment. 46 patients out of 103 evaluated were positive for the HLA DR3-DQ2 haplotype; a genetically predefined subgroup of high responding patients identified based on data from previous trials focusing on subcutaneous administration of Diamyd®. Out of the 46 patients, 29 received active treatment and 17 received placebo.

While a limited positive but non-significant treatment effect was observed in the 103 patients evaluated as part of the topline results, a statistically significant ($p < 0.01$) treatment effect was observed in the predefined subgroup of patients positive for HLA DR3-DQ2. In this subgroup of patients, more than 50% greater preservation of C-peptide was observed in those that received active treatment compared to placebo. Likewise, positive trends in patients positive for HLA DR3-DQ2 were observed for all the important secondary endpoints; change in blood glucose levels as determined by HbA1c (an important marker of diabetes management), insulin dose and insulin-adusted HbA1c compared to placebo treated patients. No benefit was seen in patients negative for HLA DR3-DQ2.

"In my 37 years in type 1 diabetes research, these are some of the most promising results I have seen in terms of the potential for providing benefit to those recently diagnosed with type 1 diabetes," says Mark Atkinson, Ph.D., Director of the Diabetes Research Institute at the University of Florida and Diamyd Medical Board Member. "These findings emphasise the importance of genetic analysis and personalized medicine in the development of treatments for type 1 diabetes and could explain conflicting results of previous trials in the field."

A short presentation of the results will be given today at 18:00 CET, at https://tv.streamfabriken.com/2020-09-14-press-conference or dial in; SE +46 856642651 PIN: 20322624#, UK +44 3333000804 PIN: 20322624#, US +18558570686 PIN: 20322624#

On Thursday September 17 at 15:00 CET, CEO Ulf Hannelius, Professor Johnny Ludvigsson and Professor Mark Atkinson invite you to a webcast presentation of the results.

About DIAGNODE-2

The double-blind, placebo-controlled, European phase IIb trial DIAGNODE-2 included 109 patients aged 12–24 years from 18 clinics in Spain, the Czech Republic, Sweden and the Netherlands. The patients included in the trial were those with new-onset type-1 diabetes and diagnosed within 6 months of therapeutic intervention, along with their having a fasting C-peptide of ≥0.12 nmol/l. These patients were given the diabetes vaccine Diamyd® (or placebo) directly into a lymph node, following ultrasound guidance, on three occasions at one-month intervals. The trial participants were also given oral vitamin D (or placebo) supplements for four months, with vitamin D supplementation started 30 days before the first injection of Diamyd®. The patients were followed for 15 months in order to evaluate their endogenous insulin production, as measured by C-peptide. Of the initial 109 patients who entered the trial, 107 patients completed all visits up to 15 months; only two patients chose to terminate the trial prematurely. Of these 107 patients, 103 patients were available for evaluation of the primary endpoint. Four samples could not be analyzed since they arrived late at the laboratory or because the tests could not be performed on the patient. Of the 103 patients, 46 were positive for the HLA DR3-DQ2 haplotype. From the Autumn of 2019, patients who had not yet completed their last visit at 15 months were offered to participate in an extension of the trial for another 9 months. 53 patients agreed to participate in the extension trial and 15 of these patients have already been followed for 24 months. Results of this extended trial should be available in Q3 2021. Coordinating Investigator of the trial is Professor Johnny Ludvigsson at Linköping University. The Sponsor of the trial is Diamyd Medical.

About Diamyd Medical
Diamyd Medical develops therapies for type 1 diabetes. The diabetes vaccine Diamyd® is an antigen-specific immunotherapy for the preservation of endogenous insulin production. Significant results has been shown in a genetically predefined subgroup in the Company's European Phase IIb trial DIAGNODE-2, where the diabetes vaccine is administered directly into a lymph node in children and young adults with newly diagnosed type 1 diabetes. A new facility for vaccine manufacturing is being set up in Umeå for the manufacture of recombinant GAD65, the active ingredient in the therapeutic diabetes vaccine Diamyd®. Diamyd Medical also develops the GABA-based investigational drug Remygen® as a therapy for regeneration of endogenous insulin production and to improve hormonal response to hypoglycaemia. An investigator-initiated Remygen® trial in patients living with type 1 diabetes for more than five years is ongoing at Uppsala University Hospital. Diamyd Medical is one of the major shareholders in the stem cell company NextCell Pharma AB and has holdings in the medtech company Companion Medical, Inc., San Diego, USA.
Diamyd Medical's B-share is traded on Nasdaq First North Growth Market under the ticker DMYD B. FNCA Sweden AB is the Company's Certified Adviser; phone: +46 8-528 00 399, e-mail: info@fnca.se.

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