Press Release, March 4, 2021

Update on the design of a Phase III Precision Medicine trial with the Diabetes Vaccine Diamyd®

The upcoming Phase III trial with Diamyd® in new-onset type 1 diabetes will be based on the first precision medicine approach in the field. The trial is designed to confirm the effect and safety of Diamyd® in individuals recently diagnosed with type 1 diabetes who carry the genetic HLA DR3-DQ2 haplotype.

The Phase III trial is designed to enroll approximately 330 individuals aged 12 to 28, recently diagnosed with type 1 diabetes, who carry the HLA DR3-DQ2 haplotype. This patient population is based on clinical efficacy and safety results from the Phase IIa and Phase IIb trials DIAGNODE-1 and DIAGNODE-2, as well as the large-scale meta-analysis encompassing data from more than 600 individuals from previous Phase II and Phase III trials using Diamyd®. A further stratification for HLA haplotypes will be included in order to evaluate the potential super responder group of individuals who are positive for HLA DR3-DQ2 and negative for HLA DR4-DQ8.

”We are breaking new ground and are with this comprehensive data package very excited to advance the first ever precision medicine approach for type 1 diabetes”, said Ulf Hannelius, CEO of Diamyd Medical. “We have taken into account valuable input on the trial design and efficacy endpoints provided by the regulatory authorities, payers and diabetes experts in order to optimize the likelihood of a successful phase III trial. We look forward to continuing a collaborative dialogue with the regulatory authorities throughout the pivotal phase III program. We will launch the trial during 2021, and with the support of a partner and institutional investors secure the further required operational and strategic resources to maximize the potential in Diamyd“.

Co-primary endpoints consisting of Change in endogenous insulin production and Long-term blood sugar (measured as stimulated C-peptide and HbA1c respectively), measured at 24 months from baseline, will be used to support the biological and clinical relevance of the treatment. This is in line with guideance from the United States Food and Drug Administration (FDA) and the European Medicinal Authority (EMA) as well as from a Health Technology Assesment panel hosted by Diamyd Medical.

The Phase III trial will be conducted at approximately 50 clinical sites in Europe and the United States. Following a run-in period where all subjects receive vitamin D for one month, subjects will be randomized 2:1 to receive three intralymphatic injections of Diamyd® or matching placebo given one month apart, with a primary efficacy readout at 24 months from baseline. The design provides, based on efficacy data from previous trials on the HLA restricted patient population, a high probability to reach its co-primary endpoints regarding preservation of stimulated C-peptide and lower HbA1c.

Accelerated approval pathways based on the comprehensive data package supporting clinical efficacy and safety in individuals carrying the HLA DR3-DQ2 haplotype will be evaluated in parallel to the Phase III trial.

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 have been shown in a genetically predefined patient group in a large-scale meta-analysis as well as in the Company’s European Phase IIb trial DIAGNODE-2, where the diabetes vaccine was administered directly into a lymph node in children and young adults with recently 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.

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

For further information, please contact:
Ulf Hannelius, President and CEO
Phone: +46 736 35 42 41
E-mail: ulf.hannelius@diamyd.com

Diamyd Medical AB (publ)
Kungsgatan 29, SE-111 56 Stockholm, Sweden. Phone: +46 8 661 00 26, Fax: +46 8 661 63 68
E-mail: info@diamyd.com Reg. no.: 556242-3797 Website: https://www.diamyd.com

The information was submitted for publication, through the agency of the contact person set out above, at 10:15 CET on March 4, 2021.