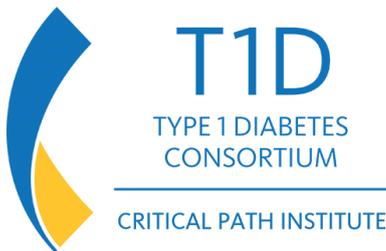

C-Path Receives Qualification Opinion from EMA on Type 1 Diabetes Biomarker Initiative



TUCSON, Ariz., April 5, 2022 — [Critical Path Institute](#)'s (C-Path) Type 1 Diabetes Consortium (T1DC) today announced that the European Medicines Agency (EMA) has issued a positive qualification opinion for pancreatic islet autoantibodies as enrichment biomarkers for type 1 diabetes (T1D) prevention trials. The purpose of this model-based qualification is to make publicly available tools to assist in the identification and selection of patients with a likelihood of progressing to a T1D clinical diagnosis, in trials of reasonable duration.

This regulatory endorsement will provide sponsors with the confidence to use islet autoantibodies in the optimization of clinical trials evaluating novel therapies focused on the delay and/or prevention of T1D. The models used to generate the underlying evidence for this EMA qualification opinion utilize islet autoantibody status with other patient features to identify a patient's risk of progressing to a T1D diagnosis. Positivity for two or more of the autoantibodies, together with other patient features, will be used for enrichment of clinical trials focusing on the delay or prevention of the clinical diagnosis of T1D.

In their qualification opinion, EMA reiterated the unmet drug development need that T1DC targeted with this work, stating, "There is clearly an unmet need for biomarkers to aid development in T1DM prevention, a field with a long history of failed trials." EMA's qualification opinion statement says, "Positivity to at least 2 of the following islet AAs; IAA, GAD65, IA-2, and ZnT8 is qualified for use as enrichment biomarker, in combination with clinical parameters (sex, baseline age, blood glucose measurements from the 120-minute timepoints of oral glucose tolerance test (OGTT), and hemoglobin A1c (HbA1c) levels) in T1D prevention trials targeting individuals at risk of developing T1D."

The incidence of T1D is on the rise worldwide, particularly in children. In Europe, incidence rates are between 0.2 and 0.5%, with steep rises in the number of children and young people being diagnosed in certain European countries. The ability to identify individuals at risk of progressing to a clinical diagnosis of T1D is a valuable opportunity to enrich clinical trials testing interventions that can potentially delay and ultimately prevent T1D.

"This qualification from the EMA would not have been possible without the tireless dedication and collaboration of the T1D research community," said Marjana Marinac, Pharm.D., JDRF Senior Director of Regulatory Affairs and T1DC Co-Director. "JDRF applauds EMA's decision to support and accelerate more innovation in therapies to delay and prevent T1D."

"This endorsement from EMA is a result of many years of extensive work and extraordinary collaboration among clinical researchers, patient advocacy groups, nonprofit organizations, and members of the biopharmaceutical industry facilitated by T1DC at C-Path," said Klaus Romero, M.D., M.S., F.C.P., C-Path Chief Scientific Officer. "This success was only possible through collaborative patient-level data sharing across stakeholders, and we are grateful to our many collaborators for their continued support. We look forward to the impact this tool will make in the development of novel therapies to treat the early

stages of T1D with the goal of delaying or preventing the clinical onset of this disease.”

The Qualification Opinion can be found on the EMA website [here](#), or on the T1D Consortium website [here](#).

The consortium is also working on the next regulatory milestone: the endorsement of these islet autoantibodies by the U.S. Food and Drug Administration. C-Path’s T1D Consortium will achieve the regulatory endorsement of the islet autoantibodies currently used in clinical practice to diagnose T1D by employing the resources of all its members and engaging with regulatory agencies at each step of the process with funding and input from [The Leona M. and Harry B. Helmsley Charitable Trust](#), [Janssen Research & Development, LLC](#), [JDRF International](#), [Novo Nordisk A/S](#) and [Provention Bio](#).



About C-Path

Critical Path Institute (C-Path) is an independent, nonprofit organization established in 2005 as a public and private partnership. C-Path’s mission is to catalyze the development of new approaches that advance medical innovation and regulatory science, accelerating the path to a healthier world. An international leader in forming collaborations, C-Path has established numerous global consortia that currently include more than 1,600 scientists from government and regulatory agencies, academia, patient organizations, disease foundations, and hundreds of pharmaceutical and biotech companies. C-Path U.S. is headquartered in Tucson, Arizona, C-Path in Europe is headquartered in Amsterdam, Netherlands and [C-Path Ltd.](#) operates from Dublin, Ireland with additional staff in multiple other locations. For more information, visit [c-path.org](#).

In the U.S., Critical Path Institute is supported by the Food and Drug Administration ([FDA](#)) of the Department of Health and Human Services ([HHS](#)) and is 54.2% funded by the FDA/HHS, totaling \$13,239,950, and 45.8% funded by non-government source(s), totaling \$11,196,634. The contents are those of the author(s) and do not necessarily represent the official views of, nor an endorsement by, FDA/HHS or the U.S. Government.



C-Path’s **Type 1 Diabetes Consortium** (T1DC) is a public-private partnership initiated in March 2017. Currently membership is composed of the following Industry and Foundation members: The Leona M. and Harry B. Helmsley Charitable Trust; Janssen Research & Development, LLC; JDRF International; Novo Nordisk; and Provention Bio. Other consortium members, participants and advisors include individuals from the following organizations: Benaroya Research Institute at Virginia Mason; Lund University, Sweden; Helmholtz Zentrum München; University of Bristol; University of Colorado Denver; University of Florida; University of Helsinki; University of Leuven; University of Munich; University of Oulu; University of Tampere; University of Turku; Critical Path Institute; FDA and NIH.



The Leona M. and Harry B. Helmsley Charitable Trust aspires to improve lives by supporting exceptional efforts in the

U.S. and around the world in health and select place-based initiatives. Since beginning active grantmaking in 2008, Helmsley has committed more than \$3 billion for a wide range of charitable purposes. The Helmsley Type 1 Diabetes Program is one of the largest private foundation funders of T1D in the nation focused on understanding the disease, developing better treatments, and improving care and access in the U.S and low- and middle- income countries. For more information on Helmsley and its programs, visit helmsleytrust.org



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JDRF's mission is to accelerate life-changing breakthroughs to cure, prevent, and treat T1D and its complications. To accomplish this, JDRF has invested more than \$2.5 billion in research funding since our inception. We are an organization built on a grassroots model of people connecting in their local communities, collaborating regionally for efficiency and broader fundraising impact and uniting on a national stage to pool resources, passion and energy. We collaborate with academic institutions, policymakers and corporate and industry partners to develop and deliver a pipeline of innovative therapies to people living with T1D. Our staff and volunteers throughout the United States and our five international affiliates are dedicated to advocacy, community engagement and our vision of a world without T1D. For more information, please visit jdrf.org or follow us on Twitter: [@JDRF](https://twitter.com/JDRF).



Novo Nordisk is a leading global healthcare company, founded in 1923 and headquartered in Denmark. Our purpose is to drive change to defeat diabetes and other serious chronic diseases such as obesity and rare blood and endocrine disorders. We do so by pioneering scientific breakthroughs, expanding access to our medicines, and working to prevent and ultimately cure disease. Novo Nordisk employs about 47,800 people in 80 countries and markets its products in around 170 countries. Novo Nordisk's B shares are listed on Nasdaq Copenhagen (Novo-B). Its ADRs are listed on the New York Stock Exchange (NVO). For more information, visit novonordisk.com, [Facebook](#), [Twitter](#), [LinkedIn](#), [YouTube](#).



Provention Bio, Inc. (Nasdaq: PRVB) is a biopharmaceutical company focused on advancing the development of

investigational therapies that may intercept and prevent debilitating and life-threatening immune-mediated disease. The Biologics License Application (BLA) for teplizumab, its lead investigational drug candidate, for the delay or prevention of clinical type 1 diabetes in at-risk individuals, has been filed by the U.S. Food and Drug Administration (FDA). The Company's pipeline includes additional clinical-stage product candidates that have demonstrated in pre-clinical or clinical studies proof-of-mechanism and/or proof-of-concept in other autoimmune diseases, including celiac disease and lupus. Visit www.Proventionbio.com for more information and follow us on Twitter: [@ProventionBio](https://twitter.com/ProventionBio).

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