C-Path Receives Letter of Support from EMA on Type 1 Diabetes Biomarker Initiative

TUCSON, Ariz., April 28, 2020 — The Critical Path Institute (C-Path) today announced that its Type 1 Diabetes (T1D) Consortium has received a letter of support from the European Medicines Agency (EMA) to facilitate the development and validation of the proposed regulatory qualification of pancreatic islet autoantibodies commonly used in clinical practice to diagnose T1D: insulin autoantibodies, glutamic acid decarboxylase 65, and insulinoma antigen-2 autoantibodies as enrichment biomarkers for T1D clinical trials.

In their response to the T1D Consortium Letter of Intent (LOI) and Briefing Package, the EMA stated, "[Therapies that preserve endogenous ?-cell function and can prevent, halt or slow T1D disease progression in a clinically meaningful way would constitute a significant advancement in T1D care. If successful, the quantitative tools proposed by this Consortium have the potential to facilitate the streamlined design, execution, and review of clinical trials targeting this goal.]."

By the year 2050, the number of people diagnosed with T1D in the U.S. is projected to quadruple from an estimated 1.6 million in 2020 to 5 million in 2050. The ability to screen for subjects with early stages of T1D prior to the appearance of clinical symptoms is a valuable opportunity to potentially delay, and ultimately prevent, symptomatic T1D. The islet autoantibodies provide a means to identify patients at risk of progressing to a clinical diagnosis of T1D.

C-Path's T1D Consortium will achieve the regulatory qualification 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.

"JDRF is pleased the T1DC received a letter of support from EMA," says Jessica Dunne, Ph.D., Senior Director, JDRF and Co-Director of the T1DC. "This is an example of collaboration between regulators and researchers, in the public and private sectors, working together to accelerate delivery of therapies into the hands of patients."

This model-based qualification, will provide a tool endorsed by both the EMA and U.S. Food and Drug Administration (FDA) that utilizes islet autoantibody status, along with other relevant patient features, to identify and select patients with a likelihood of progressing to a T1D clinical diagnosis. 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 prevent T1D.

“We are delighted that the EMA is strongly supporting the development of quantitative tools that can accelerate drug development in T1D,” said C-Path’s Executive Director of the T1D Consortium Inish O’Doherty, PhD. “This work has been enabled through the collaboration of the T1D community and the sharing of patient level data. We’re excited to move forward with this important project which will help pave the way for forthcoming therapies with the ability to treat early stages of T1D and delay or prevent the clinical, currently irreversible, stage of the disease.”

The consortium is currently working on the next regulatory milestones: the execution of the modeling analysis plan to inform the full Briefing Package for submission to the EMA and development of a Qualification Plan for submission to the FDA.

The Letter of Support can be found on the EMA website here or on the T1D Consortium website here.

About the Organizations:

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 dozens of pharmaceutical and biotech companies. C-Path US is headquartered in Tucson, Arizona and C-Path, Ltd. EU is headquartered in Dublin, Ireland, with additional staff in multiple other locations. For more information, visit c-path.org and c-path.eu.

The Type 1 Diabetes (T1D) Consortium 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 $2 billion for a wide range of charitable purposes. Learn more about Helmsley at helmsleytrust.org.

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Learn more at www.janssen.com. Follow us at www.twitter.com/JanssenGlobal. Janssen Research & Development, LLC is one of the Janssen Pharmaceutical Companies of Johnson & Johnson.

JDRF is the leading global organization funding type 1 diabetes (T1D) research. Our mission is to accelerate life-changing breakthroughs to cure, prevent and treat T1D and its complications. To accomplish this, JDRF has invested more than $2.2 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 six 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.

Novo Nordisk is a global healthcare company with more than 95 years of innovation and leadership in diabetes care. This heritage has given us experience and capabilities that also enable us to help people defeat obesity, haemophilia, growth disorders and other serious chronic diseases. Headquartered in Denmark, Novo Nordisk employs approximately 42,700 people in 80 countries and markets its products in around 170 countries. For more information, visit novonordisk.com, Facebook, Twitter, LinkedIn, YouTube.

Provention Bio, Inc. (Nasdaq: PRVB) is a clinical-stage biopharmaceutical company leveraging a transformational drug development strategy that is focused on the prevention or interception of immune-mediated disease. Provention's mission is to in-license, transform and develop therapeutic candidates targeting the high morbidity, mortality and escalating costs of autoimmune and inflammatory diseases including: type 1 diabetes (T1D), celiac disease and lupus. Provention's diversified portfolio includes advanced-stage product development candidates that have undergone clinical testing by other companies. For more information, please visit proventionbio.com.

Benaroya Research Institute at Virginia Mason (BRI) is a world-renowned, non-profit research institute committed to advancing the science that will predict, prevent, reverse and cure diseases of the immune

system. BRI researchers uniquely study the immune system in both health and disease, with the ultimate goal of achieving a healthy immune system for each individual. Diseases we study include type 1 diabetes, rheumatoid arthritis, lupus, multiple sclerosis, Crohn's and colitis as well as allergies and cancer. BRI accelerates discovery through laboratory breakthroughs in immunology that can be translated to clinical therapies. A leader of collaborative initiatives such as the Immune Tolerance Network and Type 1 Diabetes TrialNet, BRI frequently partners with global research institutes, pharmaceutical and biotech companies. Visit BenaroyaResearch.org or follow us on the Autoimmune Life blog, Facebook, Instagram, YouTube, LinkedIn or Twitter.

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