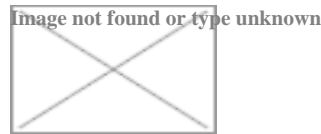
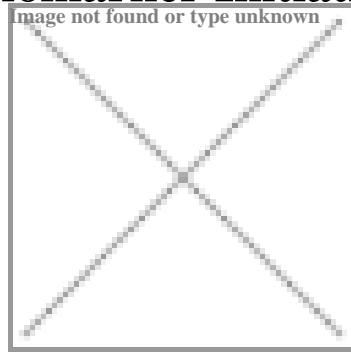


## Critical Path Institute Encouraged by FDA to Move Forward on Type 1 Diabetes Biomarker Initiative



### *Biomarker potentially could identify individuals at risk of developing T1D*

**TUCSON, Ariz., December 18, 2018** — [Critical Path Institute](#) (C-Path) announced today that its Type 1 Diabetes (T1D) Consortium has received a positive response to its [Letter of Intent \(LOI\)](#) from the U.S. Food and Drug Administration (FDA) detailing the FDA’s decision to accept the consortium’s Biomarker Initiative project into the Center for Drug Evaluation and Research (CDER) Biomarker Qualification Program (BQP). In its LOI, the T1D Consortium provided information to support its proposed qualification of islet autoantibodies (AAs) as susceptibility/ risk biomarkers that could identify individuals who are likely to develop a clinical diagnosis of T1D.

The FDA indicated in its LOI decision letter that it supports the consortium’s plan to pursue biomarker qualification aimed at providing clinical validation of the islet AAs. The FDA stated, “Based on our review of the LOI, we agree there is an unmet need and agree that development of the islet AAs as a susceptibility/risk biomarker of T1D would enable proper patient selection for clinical investigations of earlier interventions for T1D.”

An estimated 1.25 million Americans are living with T1D, and by the year 2050 the number of youth diagnosed with T1D in the U.S. is projected to more than triple. Islet AAs provide a means to better identify individuals at risk of progressing to a clinical diagnosis of T1D, providing a valuable opportunity to identify patients for early intervention and prevention of the progression of the disease.

“The encouraging statements that FDA incorporated in the LOI decision letter add weight to the recognition of this significant unmet medical need as well as the critical importance of identifying factors for those at risk of developing T1D,” said C-Path President and CEO Martha Brumfield, Ph.D. “This early support can serve to encourage those with data in this space to participate with C-Path to positively impact the lives of individuals affected by the disease, and it also represents a meaningful advance in the prevention of T1D.”

The islet AAs represent a panel of biomarkers: including antibodies to insulin, glutamic acid decarboxylase 65 (GAD-65), insulinoma antigen-2 (IA-2) and zinc transporter 8 (ZnT8). This model-based qualification effort, with both the FDA and the European Medicines Agency (EMA) will be based on a disease progression model that will quantitatively describe the relationship between the presentation of two of the

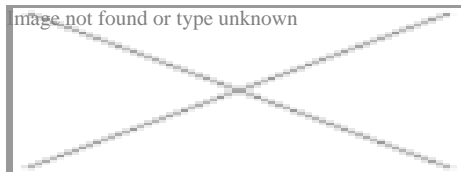
four islet AAs, other disease relevant features and the likelihood of developing clinical T1D over time in order to reliably identify patients for clinical trials evaluating novel therapies focused on the prevention of T1D.

C-Path's T1D Consortium is working to qualify islet AAs by employing the resources of its members, engaging with regulatory agencies at each step of the process, and with funding from [Janssen Research & Development, LLC](#); [JDRF International](#); [The Leona M. and Harry B. Helmsley Charitable Trust](#); [Novo Nordisk](#) and [Sanofi](#). In September 2018, Provention Bio joined the T1D Consortium as its newest member, demonstrating the community's increasing confidence in, and support of, this effort.

As part of the 21st Century Cures Act, public-private partnerships consisting of government entities, including the FDA, and the pharmaceutical and biotechnology industries, healthcare providers, and patient organizations are encouraged to work together to foster innovation in drug development and regulatory review through advancing drug development tools that give patients more timely access to diagnostic and therapeutic technologies.

“The efforts of the T1D Consortium support our collective goal to translate the work of the T1D community into an accepted drug development tool to support clinical trials of new medicines for T1D patients,” said Inish O’Doherty, Ph.D., executive director of the T1D Consortium.

The consortium is currently working on the next regulatory milestone: the development of a Qualification Plan for submission to the FDA and Letter of Intent/ Briefing Package for submission to the EMA.



### **About Critical Path Institute**

**C-Path (Critical Path Institute)** 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 over 1,500 scientists from government and regulatory agencies, academia, patient organizations, disease foundations, and dozens of pharmaceutical and biotech companies. C-Path is headquartered in Tucson, Arizona, with additional staff in multiple remote locations. For more information, visit [www.c-path.org](http://www.c-path.org).

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