C-Path's T1D Consortium to Release a Clinical Trial Simulation Tool for New-Onset Trial Design

The tool will help researchers design more efficient, effective clinical trials in new-onset type 1 diabetes.

TUCSON, Ariz., June 20, 2025 — Critical Path Institute's® (C-Path) Type 1 Diabetes Consortium (T1DC) today announced the release of a pilot version of their regulatory-grade new-onset clinical trial simulation tool (CTST). The pilot CTST is designed to provide valuable insights into clinical trial size and duration, utilizing C-peptide as a direct reflection of beta cell function. As an endpoint in individuals with stage 3 type 1 diabetes studies, C-peptide allows investigators to make faster decisions regarding study design or modifications, given its direct correlation with ?-cell function. This pilot CTST will aid researchers and drug developers in designing more efficient and effective clinical trials which will have a direct benefit to individuals with T1D.

The pilot CTST was developed from individual-level control arm data of 20 randomized clinical trials, which included 810 trial participants from various sources. The pilot CTST was developed on 80% of the data based on longitudinal area-under-the-curve data of C-peptide, measured by a 2-hour MMTT and then validated on the other 20% of the data. Covariates examined included: baseline (C-peptide, age, BMI Z-score, and disease duration), sex, race, and ethnicity. Operable within a graphical user interface (GUI), the pilot CTST allows users to specify factors including sample size, duration and various other predictors to create a simulation of clinical trials. The work was completed by several C-Path teams including T1DC, the Quantitative Medicine Program and Data Collaboration Center, as well as T1D consortium members.

T1DC has submitted the C-peptide univariate model to the U.S. Food and Drug Administration (FDA) for its review and endorsement as a drug development tool to design more efficient clinical trials. Regulatory feedback will be key to finalizing the CTST. In the meantime, the consortium will continue to develop a multivariate CTST that will include C-peptide, HbA1C and insulin to form an even more sophisticated solution to optimize clinical trials. When complete, C-Path will also submit this multivariate CTST to both FDA and the European Medicines Agency for regulatory review.

"This tool, which leverages clinical data acquired over decades of research, will enable researchers seeking to design new studies to leverage this hard-won knowledge," said Joseph Hedrick, Ph.D., Executive Director of Critical Path Institute's T1D Consortium. "Employing tools such as this can enable all stakeholders to make more informed decisions and accelerate the development of novel treatments."

"The new-onset clinical trial simulation tool marks a pivotal advancement in T1D research as it aims to shed light towards non-traditional endpoints in T1D such as C-peptide levels," said Mark Peakman, M.B.B.S., B.Sc., M.Sc., Ph.D., FRCPath, Head, Immune Tolerance & Autoimmunity Research, R&D at Sanofi.

Elisabeth Niemoeller, M.D., Therapeutic Area Head, Development, Diabetes, Cardiovascular and Metabolism, R&D at Sanofi added, "The evolution of clinical endpoints are relevant for the development of novel medicines that aim to avoid autoimmune-mediated pathways and preserve glucose homeostasis, such as the insulin-producing ?-cell function. At Sanofi, we're proud to support this collaborative initiative, and are looking forward to hearing feedback from regulatory authorities and other relevant stakeholders for the benefit of society."

“The tool will address critical questions related to testing upcoming therapies in the new-onset stage,” said Esther Latres, Ph.D., Vice President for Research at Breakthrough T1D, the leading global type 1 diabetes research and advocacy organization. “It signifies an unprecedented collaboration among stakeholders to bring together data and expertise to accelerate drug development in the type 1 diabetes field.”

C-Path and the additional project collaborators are excited to put this new pilot CTST into the hands of researchers and industry to help accelerate drug development in T1D. The work of T1DC is supported by Breakthrough T1D, Diamyd Medical, Helmsley Charitable Trust, Novo Nordisk and Sanofi; development of the CTST for new-onset T1D was specifically supported by Breakthrough T1D and Sanofi.

The pilot CTST and recorded webinar are available on C-Path’s website, here.

About Critical Path Institute

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.

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