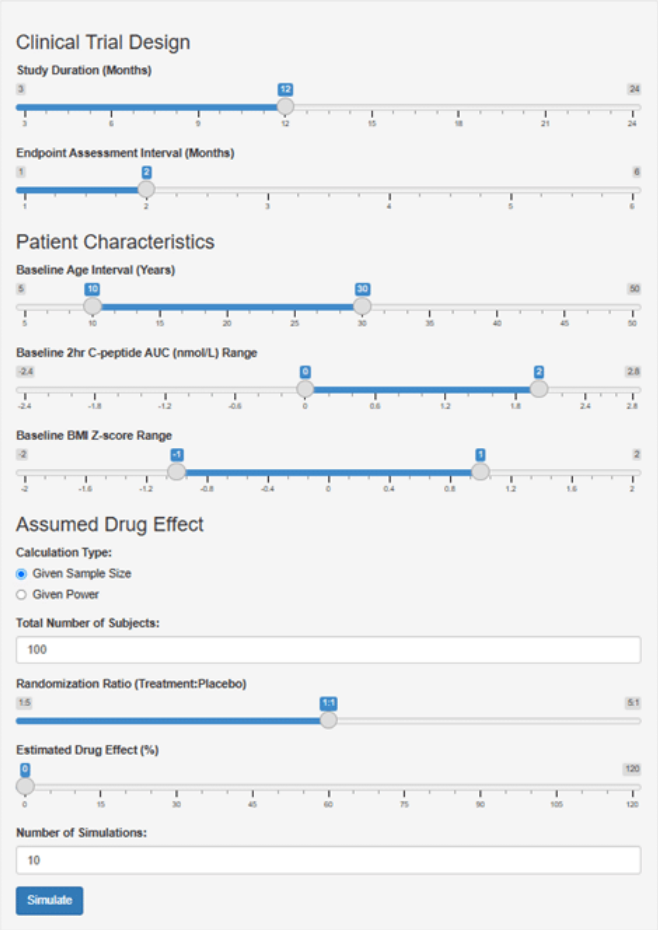
CRITICAL PATH INSTITUTE

C-Path's Initiative to Optimize Clinical Trial Design in New-Onset Type 1 Diabetes

In honor of World Diabetes Day and the need to improve diabetes treatment, Critical Path Institute's Type 1 Diabetes Consortium (T1DC) would like to highlight its development of a regulatory grade clinical trial simulation tool to inform trial design in new onset type 1 diabetes (T1D). This revolutionary tool uses a drug-disease trial model measuring C-peptide area under the curve (AUC) during the 2-hour mixed-meal tolerance test (MMTT). The tool will be beneficial to drug developers who are looking to design better clinical trials for patients with new-onset T1D.



The tool, developed from control arms of 20 randomized clinical trials, included 781 individuals from various sources. The model was developed on 80% of the data based on disease progression of C-peptide AUC measured by a 2-hour MMTT and the model was validated on the other 20% of the data. Stepwise covariate model building examined the following covariates: baseline C-peptide, age, BMI Z-score,disease duration, sex, race, and ethnicity. Operable within a graphical user interface, the tool allows users to specify factors including sample size, duration, and various other predictors to create a simulation of clinical trials.

T1DC staff members recently presented this work at the Immunology of Diabetes Society in Bruges, Belgium. I was fortunate enough to be an attendee and could see first-hand how this serves as a critical and informative tool for investigators, developers, and other interested parties seeking confidence in their clinical trial design for new-onset T1D. There are currently no approved therapies to treat this stage of disease, and the intent of developing this tool is to facilitate drug development here. As a person living with T1D for 27 years, I know my community is eager for these much-needed therapies.

T1DC plans to submit this univariate model to the U.S. Food and Drug Administration (FDA) for qualification, so that the tool can be used by drug developers to design more efficient clinical trials. In the meantime, the consortium will continue to develop a multivariate model that will still include C-peptide, along with HbA1C and insulin models to form a more robust simulation for clinical trials. When complete, the team will also seek regulatory approval from FDA and European Medicines Agency to qualify the multivariate clinical trial simulation tool.

A pilot univariate model will be released to the public ahead of regulatory approval in the coming months, following a public webinar on its development and use. Follow C-Path's social channels for registration details and to stay updated on the tool's official release.

About the Author

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Paul Belmonte, Ph.D., is the Scientific Director for the Type 1 Diabetes Consortium at Critical Path Institute. He is an immunologist with over six years of experience conducting science at the bench in T cell development and animal models of T1D, three years of experience working in medical affairs, and is heavily involved in patient advocacy as a person living with type 1 diabetes himself. His focus and career are dedicated to improving the lives of those living with T1D.