

## **VIEW NOW | Design of Clinical Trials in New-Onset Type 1 Diabetes: Regulatory Considerations for Drug Development**

Critical Path Institute held a free virtual workshop, Design of Clinical Trials in New-Onset Type 1 Diabetes: Regulatory Considerations for Drug Development, June 15-16, 2021.

### Workshop Summary

The purpose of this scientific workshop was to discuss the existing evidence regarding the role of C-peptide in clinical trials intended to support regulatory decision making, unique regulatory considerations from FDA and EMA and next steps for the T1D drug development community.

The preliminary agenda included:

- Welcome and Introductory Remarks
- Session I: Regulatory Framework for Clinical Investigations in New/Recent Onset T1D
- Session II: Scientific Framework: The rationale for C-peptide preservation and use as a clinical trial endpoint
- Session III: Establishing/Confirming Clinical Benefit
- Session IV: Overall Issues of Study Design

**View the available sessions below.**

## **Agenda – Day 1**

Time (EST)	Title	Presenter
10:00	<a href="#"><u>Welcoming Remarks and Housekeeping</u></a>	Inish O'Doherty, C-Path
10:05	<a href="#"><u>FDA Introductory Remarks</u></a>	Ilan Irony, FDA
10:15	<a href="#"><u>Patient Perspective Opening Remarks: Unmet Need</u></a>	Aaron Kowalski, JDRF
10:25	<a href="#"><u>Session I: Regulatory Framework for Clinical Investigations in New/Recent Onset T1D</u></a>	
10:25	<a href="#"><u>FDA perspective</u></a>	Kristen Pluchino, FDA
10:45	<a href="#"><u>EMA perspective</u></a>	Peter Mol, EMA
11:05	<b>Break: 20 minutes</b>	
11:25	<a href="#"><u>Session II: Scientific Framework: The rationale for C-peptide preservation and use as a clinical trial endpoint</u></a>	<b>Session Co-chairs:</b>  Chantal Mathieu, INNODIA + Patricia Beaston, FDA
11:30	<a href="#"><u>C-Peptide as Primary Endpoint &amp; Natural History</u></a>	Kevan Herold, Yale University

12:00	Islet Transplantation: Relationship of C-peptide and clinically meaningful outcomes	Michael Rickels, University of Pennsylvania
12:20	<a href="#"><u>Differential Rates of C-Peptide Decline</u></a>	Carla Greenbaum, Benaroya Research Institute
12:40	<a href="#"><u>C-Peptide as a Primary Endpoint</u></a>	Stephen Gough, Novo Nordisk
13:00	<a href="#"><u>Panel Discussion</u></a>	<b>Moderators:</b> Session II co-chairs  <b>Panelists:</b> Panelists: Session II speakers + Mark Peakman, Sanofi
13:40	<a href="#"><u>Day 1 Closing Remarks</u></a>	

## Agenda – Day 2

Time (EST)	Title	Presenter
10:00	<a href="#"><u>Day 2 Opening Remarks</u></a>	
10:10	<a href="#"><u>Session III: Establishing/Confirming Clinical Benefit</u></a>	<b>Session Co-chairs:</b>  Lisa Yanoff, FDA + Colin Dayan, Cardiff University
10:15	<a href="#"><u>Perspective from people living with T1D: Clinically meaningful measures</u></a>	Chantal Mathieu, INNODIA;  Marjana Marinac, JDRF;  Kyle Jacques Rose, INNODIA PAC;  Melissa Schwaber
10:45	<a href="#"><u>FDA Perspective: Clinical endpoints and validated surrogates</u></a>	Lauren Wood Heickman, FDA
10:55	<a href="#"><u>EMA Perspective: Clinical endpoints and validated surrogates</u></a>	Carine de Beaufort, EMA
11:05	<a href="#"><u>General considerations for trial design for confirmatory endpoints</u></a>	Allison Goldfine, Novartis Institutes of Biomedical Research
11:20	<a href="#"><u>Additional Clinical Outcomes: Considerations and current limitations</u></a>	Joe Hedrick, Janssen
11:35	<a href="#"><u>Panel Discussion: Discuss relevant perspectives</u></a>	<b>Moderators:</b> Session III co-chairs  <b>Panelists:</b> Session III speakers
12:15	<b>Break: 20 Minutes</b>	

12:35	<b>Session IV: Overall Issues of Study Design</b>	<b>Session Co-chairs:</b> Inish O'Doherty, C-Path + Peter Gottlieb, University of Colorado
12:40	<a href="#"><u>Ethical Considerations of Trial Design</u></a>	Donna Snyder, FDA
13:00	<a href="#"><u>Statistical Considerations for Trial Design and Feasibility</u></a>	Tee Bahnsen, Benaroya Research Institute
13:15	<a href="#"><u>Considerations for Trial Design and Feasibility</u></a>	Regine Bergholdt, Novo Nordisk
13:30	<a href="#"><u>Final Panel Discussion/Open Comment</u></a>	<b>Moderators:</b> Session IV co-chairs <b>Panelists:</b> Panelists: Session IV speakers + Francisco Leon, Provention Bio
14:30	<a href="#"><u>Workshop Closing Remarks</u></a>	Lisa Yanoff, FDA