

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

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

Agenda – Day 2

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

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