CRITICAL PATH INSTITUTE

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