

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

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

## Agenda – Day 2

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

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| 12:35 | <b><u>Session IV: Overall Issues of Study Design</u></b>                           | <b>Session Co-chairs:</b><br><br>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 Bahnson, 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<br><br><b>Panelists:</b> Panelists: Session IV speakers + Francisco Leon, Provention Bio |
| 14:30 | <b><u>Workshop Closing Remarks</u></b>                                             | Lisa Yanoff, FDA                                                                                                                 |