



Pediatric Research
INNOVATION FORUM



Advancing Pathways for the Development of Innovative Therapies for Children with IBD
Virtual Public Webinar Series

Data Extrapolation

Wednesday, November 10, 2021: 9:00 – 13:00 ET/15:00 – 19:00 CET

Agenda

9:00 – 9:10 ET
15:00 – 15:10 CET

Welcome—Gary Noel –*I-ACT for Children, Chief Medical Officer*
Meeting Logistics—Anushree Parikh –*I-ACT for Children, Clinical Project Manager*
Introductions—Collin Hovinga –*I-ACT for Children, SVP—Clinical & Scientific Development*
(*Co-Chairs—Andrew Mulberg and Nick Croft*)

9:11 – 10:00 ET
15:11 – 16:00 CET

Working Group Summary of Key Evidence

- (1) **Evidence Supporting Extrapolation of Efficacy** —Laurie Conklin –*Pharmaceutical Industry Physician & Researcher*
- (2) **Is there a PD-Measurement that Can Be Used to Predict Efficacy in Children?** – Johanna Escher –*Physician/Professor of Pediatrics* and Javier Martin de Carpi - *Physician/Associate Professor*
- (3) **Is it reasonable to Assume Similar Exposure-Response in Children and in Adults?** – Wallace Crandall –*Pharmaceutical Industry Physician & Researcher* and Edwin de Zoeten – *Physician/Associate Professor*

10:01 – 10:20 ET
16:01 – 16:20 CET

Key Evidence Panel and Q&A—Moderated by Andrew Mulberg and Nick Croft

Marc Gastonquay –*Biomedical Modeling & Simulation*; Jeffrey Barrett –*Data and Analytics Platform*; Insook Kim –*FDA Translational Sciences*; Melvin Heyman –*Professor of Pediatrics*; Harland Winter –*Associate Professor of Pediatrics*; David Wilson –*Professor of Paediatric Gastroenterology and Nutrition*; Gigi Veereman –*Pediatric Gastroenterology and Nutrition*; and Forrest Williamson –*Pharmaceutical Scientist, Statistics Data & Analytics*

10:21 – 10:30 ET/16:21 – 16:30 CET

Break

10:31– 11:20 ET
16:31 – 17:20 CET

Working Group Summary of Recommendations

- (4) **Extrapolation of Efficacy** —Laurie Conklin
- (5) **PD-Measurements for Prediction of Efficacy**—Johanna Escher and Javier Martin de Carpi
- (6) **Exposure-Responses** –Wallace Crandall and Edwin de Zoeten

11:21 – 11:40 ET
17:21 – 17:40 CET

Recommendations Panel and Q&A—Moderated by Andrew Mulberg and Nick Croft

Marc Gastonquay; Jeffrey Barrett; Insook Kim; Melvin Heyman; Harland Winter; David Wilson; Gigi Veereman; and Forrest Williamson

11:41 – 11:50 ET
17:41 – 17:50 CET

- (7) **Summary of Feedback and Discussion – Review** – Andrew Mulberg

11:51 – 12:00 ET
17:51 – 18:00 CET

Thank you— *Andrew Mulberg and Nick Croft*

Next Steps—Collin Hovinga – *I-ACT for Children, SVP—Clinical & Scientific Development*

Closing Remarks—Gary Noel – *I-ACT for Children, Chief Medical Officer*

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Working Group Members

I-ACT for Children Facilitator Collin Hovinga, PharmD, MS, FCCP Senior Vice President, Clinical and Scientific Development	
Co-Chair Andrew Mulberg, MD Senior Vice President, Regulatory Affairs Neurogene Inc.	Co-Chair Nick Croft MBBS, PhD Professor of Clinical Paediatric Gastroenterology Blizard Institute, Barts and the London School of Medicine and Dentistry Queen Mary University of London, London, UK
Members	
Andrew Grossman, MD Co-Director, Children’s Hospital of Philadelphia’s Center for Pediatric Inflammatory Bowel Disease	Athos Bousvaros, MD Vice Chair of Clinical Operations, Department of Pediatrics Boston Children’s Hospital, Professor of Pediatrics, Harvard Medical School
Marla Dubinsky, MD Professor of Pediatrics and Medicine at the Icahn School of Medicine at Mount Sinai, Chief, Division of Pediatric Gastroenterology and Nutrition Mount Sinai Kravis Children’s Hospital	Subra Kugathasan, MD Professor of Pediatrics & Human Genetics Marcus Professor of Pediatric Gastroenterology Scientific Director, Children’s Healthcare of Atlanta Combined Center for Pediatric Inflammatory Bowel Disease
Laurie Conklin, MD Director, Pediatric Immunology, Janssen Research and Development	Cecile Ollivier OCM Director - Regulatory Science Europe at Critical Path Institute (C-Path)
Wallace Crandall, MD Senior Medical Fellow, Senior Medical Lead, Pediatric Immunology, Eli Lilly	Edwin de Zoeten, MD, PhD Associate Professor-Pediatrics University of Colorado School of Medicine, Director-Pediatric Inflammatory Bowel Disease Center Colorado Children’s Hospital
Jenn Poole Associate Director, Global Regulatory Affairs Gastroenterology, Takeda	Richard Veselý MD Vice President Regulatory Strategy CATO SMS Amsterdam, The Netherlands
Javier Martín-de-Carpi, MD, PhD Associate Professor, University of Barcelona. Head of the Pediatric Gastroenterology, Hepatology and Nutrition Department, Sant Joan de Déu Hospital, Barcelona.	Johanna (Hankje) Escher, MD, PhD Professor of Pediatrics Head of Pediatric Gastroenterology Department Erasmus University Medical Center, Sophia Children’s Hospital Rotterdam, The Netherlands