

*Developing Clinical Pharmacokinetics and Safety Trials in Down syndrome*

PTN DOWN SYNDROME 2020 VIRTUAL MEETING – SYLLABUS

NOVEMBER 2, 2020

9:00 AM – 1:30 PM ET

**COURSE DESCRIPTION:**

The Pediatric Trials Network (PTN) was established by the National Institute of Child Health and Human Development (NICHD) for the Best Pharmaceuticals for Children Act (BPCA) program to serve as a focal point for pharmaceutical clinical trials in pediatric populations. This half-day virtual meeting will focus on presentations and discussions about therapeutic challenges in treating children with DS; community advocacy and partnering with communities to engage patients with DS in clinical trials; parent/family engagement in current and future research; lessons learned from current efforts to enroll patients with Down syndrome into an active PTN study; and request input on a prospective randomized clinical trial the PTN is interested in conducting.

**TARGET AUDIENCE:**

Clinicians and researchers working with patients with Down syndrome (DS), advocates for people living with Down syndrome, and individuals/parents of children living with Down syndrome.

**MEETING GOALS:**

1. To increase dialogue and collaboration between key stakeholders involved in the care of children with Down syndrome with the shared goal of improving clinical outcomes in this population
2. To raise awareness of the therapeutic challenges faced by children with Down syndrome
3. To develop educational and research strategies that include individuals with Down syndrome as contributors and participants
4. To discuss challenges, lessons learned, and best practices among new PTN sites enrolling individuals with Down syndrome in clinical research studies
5. To solicit input on a prospective randomized clinical trial in development by the PTN

**LEARNING OBJECTIVES:**

By the end of this meeting attendees will:

1. Have a better understanding of the clinical complexity of down syndrome and the importance of involving patients with Down syndrome into clinical research.
2. Learn how to overcome some of the challenges with engaging the DS population in research.

**AGENDA:**

TIME	DISCUSSION	SPEAKER
9:00 am	Welcome	Mara L Becker, MD, MSCE <i>Duke Clinical Research Institute (DCRI)</i>
9:05 am	Brief Overview of the Pediatric Trials Network	Kanecia Zimmerman, MD, MPH <i>DCRI</i>
9:15 am	INCLUDE Project Overview	Melissa Parisi, MD, PhD <i>Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD)</i>

9:25 am **Clinical Overview: Clinical variability and  
Therapeutic Challenges in individuals with Down  
syndrome** **Priya Kishnani, MD**  
*Duke University Medical Center*

9:40 am **Q & A – Open discussion**

**Session: Community Engagement and Readiness**

9:45 am **Community Advocacy Perspective: Working with  
Advocacy Partners to Advance Clinical Trials in  
Individuals with Down syndrome** **Ashley Helsing**  
**Adrian Forsythe**  
*National Down Syndrome Society (NDSS)*  
**Tavrick Lawless**  
**Jacob Murasko**  
**Marla Murasko**  
**Carol Nye**  
**Sara Nye**  
*NDSS DS-AMBASSADORS®*

10:05 am **Individual/Family Perspective: How Can We  
Engage Families Meaningfully for Current and  
Future Research?** **Michelle Pfeiffer**  
**Matthew Schwab**  
**Michelle Schwab**  
**Emily Mondschein**  
*Gigi's Playhouse*

10:25 am **DS Connect: Connecting Families to Research  
Opportunities that INCLUDEs Them** **Sujata Bardhan, PhD**  
*NICHHD*

10:45 am **Moderated Q&A Session with stakeholders** **Moderator:**  
**Maria Stanley, MD**  
*University of Wisconsin-Madison*

**Panelists:**  
**Julie Hajewski**  
*University of Wisconsin-Madison*  
**Sujata Bardhan, PhD**  
*NICHHD*  
**NDSS DS-AMBASSADORS®**  
**Gigi's Playhouse**

11:15 – 11:30 am **BREAK**

**Session: Lessons Learned/ Best Practices from POP02 and PTN**

11:30 am	<b>Lessons Learned/Best practices from the Pharmacokinetics, Pharmacodynamics, and Safety Profile of Understudied Drugs Administered to Children Per Standard of Care (POP) study</b>	<b>Elena Soler, MPH</b> <i>DCRI</i>
11:40 am	<b>POP02 Down syndrome Cohort: Lessons Learned and Looking Forward</b>	<b>Jessica L. Van Bree, BS</b> <i>University of Wisconsin-Madison</i>
12:10 pm	<b>Q &amp; A – Open discussion</b>	<b>Kate Pawlowski</b> <i>Boston Children’s Hospital</i>

**Session: Down syndrome Protocol and Future Considerations**

12:15 pm	<b>ADHD in children with Down syndrome</b>	<b>Anna Esbensen, PhD</b> <i>Cincinnati Children’s</i>
12:35 pm	<b>Protocol Discussion: Guanfacine for Hyperactivity in Children with Down Syndrome (HYPEbeGONE_DS)</b>	<b>Mara Becker, MD, MSCE</b>
12:55 pm	<b>Q&amp;A and Feedback on the protocol</b>	<b>Moderator: Jenny Jackman</b> <i>DCRI</i>
1:15 – 1:30 pm	<b>Wrap up/adjourn – Next Steps</b>	<b>Panelists:</b> <b>Mara Becker, MD, MSCE</b> <b>Kylie Opel, JD</b> <i>DCRI</i> <b>Ravinder Anand</b> <i>The Emmes Company</i> <b>Mara Becker, MD, MSCE</b>

**EDUCATION CREDITS:**

\* All targeted audience members are eligible to receive Joint Accreditation credits for this activity. No attendance certificates are issued for this course. See below for CME/MOC credit statements.



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It is the CME activity provider's responsibility to submit participant completion information to ACCME for the purpose of granting ABIM or ABP MOC credit."



#### PRESENTERS, PANEL MEMBERS, AND PLANNING COMMITTEE W/O DISCLOSURES:

- Ravinder Anand, PhD, Principal Investigator BPCA Data Coordinating Center, The Emmes Company, LLC
- Sujata Bardhan, PhD, National Institute of Child Health and Human Development
- Nicole Baumer, MD, Med, Director, Boston Children's Hospital Down Syndrome Program
- Mara Becker, MD, MSCE, Vice Chair, Faculty, Duke University School of Medicine
- George Capone, MD, Director, Down Syndrome Clinic and Research Center / Medical Director, Pediatric Feeding Disorders Program, Kennedy Krieger Institute
- Anna Esbensen, PhD Assistant Professor, UC Department of Pediatric
- Sara Hart, PhD, Certified Genetic Counselor, Duke University School of Medicine
- Ashley Helsing, Director of Government Affairs, National Down Syndrome Society
- Jenny Jackman, PhD, Medical Writing Manager, Duke Clinical Research Institute
- Priya Kishnani, MD, Division Chief, Medical Genetics, Duke University Medical Center
- Kylie Opel, JD, Regulatory Affairs Scientist, Duke Clinical Research Institute
- Melissa Parisi, MD, PhD, Chief, Intellectual and Developmental Disabilities Branch, National Institute of Child Health and Human Development
- Kate Pawlowski, Research Manager, Boston Children's Hospital
- Michelle Pfeiffer, Parent Advocate, Gigi's Playhouse – Raleigh location President
- Elena Soler, MPH, PMP, Project Leader III, Duke Clinical Research Institute
- Nasreen Talib, MD, MPH, Professor of Pediatrics / Medical Director, Down Syndrome Clinic, Children's Mercy Hospital – Kansas City
- Jessica Van Bree, BS, MA, Research Specialist, University of Wisconsin-Madison
- Kanecia Zimmerman, MD, MPH, Associate Professor of Pediatrics, Duke Clinical Research Institute

**PRESENTERS, PANEL MEMBERS, AND PLANNING COMMITTEE WITH DISCLOSURES:**

- Brian Skotko, MD, MPP, Medical Geneticist / Director, Down Syndrome Program, MassGeneral Hospital
  - AC Immune
  - Woodbine House, Inc
  - Gerson Lehrman Group
  - F. Hoffman La Roche, Inc
  - LuMind IDSC Foundation
- Michelle Palumbo, MD, Instructor, Harvard Medical School Assistant Pediatrician, Mass General Hospital
  - Otsuka
- Maria Stanley, MD, Clinical Professor and Division Chief, Department of Pediatrics, University of Wisconsin-Madison
  - Octapharma
  - Shire Pharmaceuticals

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1. Participants must sign-in
2. Attend the virtual meeting
3. Complete evaluation

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