DIA

Pediatric Drug Development Workshop

Conference 28-29 | Hyatt Regency Bethesda | Bethesda, MD

PROGRAM CHAIR

Christina Bucci-Rechtweg, MD
Head, Pediatric and Maternal Health Policy
Global Drug Development Regulatory Affairs
Pediatric Center of Excellence, Novartis Pharmaceuticals Corporation

PROGRAM COMMITTEE

Samuel Blackman, MD, PhD
Head of Clinical Development
Mavupharma, Inc.

Edward Connor, MD, MBE, FAAP
President, Clinical Research Alliance; Chairman and President
I-ACT for Children

Thomas Miller, PhD
Vice President and Global Head, Pediatrics
Bayer

Andrew Mulberg, MD
Head, Senior Vice President, Global Regulatory Affairs
Amicus Therapeutics

Yeruk (Lily) Mulugeta, PharmD
Associate Director, Division of Pediatric and Maternal Health
FDA

Robert Nelson, MD, PhD
Senior Director, Pediatric Drug Development
Johnson and Johnson

Overview

The convergence of policy reform, the evolution of highly targeted therapies, and innovations in regulatory and development science has created the potential for a new era in pediatric drug development. Development of highly targeted therapies for small research populations has led to unimaginable innovation in regulatory and development science and revolutionized how treatments for the most complex diseases can be developed.

DIA's *Pediatric Drug Development Workshop* brings together the pediatric research to evaluate and discuss how various solutions can be applied to advance the development of biopharmaceutical therapies for pediatric patients.

Highlights

- Leveraging pre-existing data for modeling and simulation
- Utilizing molecular targeting
- Pragmatic solutions to address the complexities of pediatric drug development
- Tools to facilitate the evaluation of cutting-edge therapeutic discoveries

Who should attend

Professionals involved in:

- Regulatory, clinical, and drug development professionals from health authorities and within industry
- Employees from clinical research organizations (CRO) and individuals involved in pediatric clinical trials
- Pediatricians
- Representatives from academia, pediatric societies, and networks
- Parents and patient advocacy organizers
- Any stakeholder interested in the development of better pediatric research programs

Schedule At-A-Glance

DAY ONE | MONDAY OCTOBER 28		ROOM
7:00AM-5:00PM	Registration	Regency Foyer IV
7:00-8:00AM	Continental Breakfast and Networking	Regency IV
8:00-8:15AM	**Welcome and Opening Remarks**	Regency III
8:15-9:00AM	**Keynote Address:** A Family's Outlook on the SMA Diagnoses of Their Daughter and Son	Regency III
9:00-10:30AM	**Session 1:** Developing Innovative Technologies for Children: Pediatric-Directed Drug Development	Regency III
10:30-11:00AM	Refreshment and Networking Break	Regency IV
11:00AM-12:30PM	**Session 2:** Innovation Through Extrapolation: Improving the Efficiency and Effectiveness of Pediatric Drug Development	Regency III
12:30-1:30PM	Luncheon and Networking	Regency IV
1:30-3:00PM	**Session 3:** Leveraging Innovative Trials Design I: Product Development-Centric Registries and Platforms	Regency III
3:00-3:30PM	Refreshment and Networking Break	Regency IV
3:30-5:00PM	**Session 4:** Leveraging Innovative Trials Design II Panel Discussion	Regency III
5:00-6:00PM	Networking Reception	Regency IV

DAY TWO | TUESDAY OCTOBER 29		ROOM
7:00AM-12:00PM	Registration	Regency Foyer IV
7:00-7:55AM	Continental Breakfast and Networkin	Regency IV
7:55-8:00AM	Welcome to Day Two	Regency III
8:00-9:30AM	**Session 5:** Making Innovative Infrastructure for Pediatric Product Development a Reality	Regency III
9:30-10:00AM	Refreshment and Networking Break	Regency Foyer IV
10:00AM-12:00PM	**Session 6:** Innovative Business Models for Pediatric Therapeutic Development	Regency III
12:00-12:15PM	Closing Remarks	Regency III

Learning Objectives

At the end of this workshop participants should be able to:

- Describe how novel technologies such as gene therapy are being applied to the development of therapies for serious pediatric illnesses
- Explain how innovative uses of methods such as extrapolation and modeling and simulation can leverage existing adult data and decrease the need for pediatric data to facilitate the study of therapies in pediatric populations
- Discuss how the latest generation of clinical development plans, clinical trial designs, and organizational structures can enable accelerated development and approval of therapeutics for children
- Describe how companies make decisions regarding inclusion of pediatric development programs within their portfolios and the unique and evolving roles of large pharma, venture capital, and academic Institutions in the advancement of new medications for children

Continuing Education Credit

DIA is accredited by the Accreditation Council for Pharmacy Education as a provider of continuing pharmacy education. This workshop is designated for up to 10.5 contact hours or 1.05 continuing education units (CEU's). Type of Activity: Knowledge

ACPE CREDIT REQUESTS MUST BE SUBMITTED BY DECEMBER 13, 2019

DIA is required by the Accreditation Council for Pharmacy Education (ACPE) to report pharmacy-requested CEUs through the CPE Monitor system. All ACPE-certified activity credit requests need to be submitted through DIA's My Transcript within 45-days post activity. If ACPE credit is not requested by December 13, 2019, the CEU request will not be transmitted through to the CPE Monitor. Pharmacists will need to provide their National Association of Boards of Pharmacy (NABP) e-Profile ID and date of birth (MMDD) to ensure the data is submitted to the ACPE and NABP properly. If you need to obtain your NABP e-Profile, please visit **www.cpemonitor.net**.

DIA has been accredited as an Authorized Provider by the International Association for Continuing Education and Training (IACET).

As an IACET Authorized Provider, DIA offers CEUs for its programs that qualify under the ANSI/IACET Standard. DIA is authorized by IACET to offer 1.1 CEUs for this conference. Participants must complete the entire conference in order to be able to receive an IACET statement of credit. No partial credit will be awarded.

Continuing Education Credit Allocation

Day One: 6.75 Contact Hours 6.75 CEUs, UAN: 0286-0000-19-079-L04-P

Day Two: 3.75 Contact Hours 3.75 CEUs, UAN: 0286-0000-19-080-L04-P

TO ACCESS MY TRANSCRIPT

- Visit DIAglobal.org
- **Sign In** with your DIA User ID and Password
- Select the Welcome Menu in the upper right hand corner (where your name appears)
- Select **My Account** from the menu
- Select **My Transcripts** then **Manage My Transcripts**

ACCESS PRESENTATIONS

- Visit DIAglobal.org
- **Sign In** with your DIA User ID and Password
- Select the Welcome Menu in the upper right hand corner (where your name appears)
- Select **My Account** from the menu
- Choose **My Presentation**

Please Note: DIA User ID and Password are needed to access presentations. If you have forgotten your DIA User ID and Password, or this is your first time logging into the DIA website, please use our Login Reminder. **Presentations will be available for six months post conference.*

DAY ONE | MONDAY OCTOBER 28

7:00AM-5:00PM — **Registration**

7:00-8:00AM — **Continental Breakfast and Networking**

8:00-8:15AM — **Welcome and Opening Remarks**

Christina Bucci-Rechtweg, MD, Head, Pediatric and Maternal Health Policy, Global Drug Development Regulatory Affairs, Pediatric Center of Excellence, Novartis Pharmaceuticals Corporation

8:15-9:00AM — **Keynote Address:** A Family's Outlook on the SMA Diagnoses of Their Daughter and Son

Session Chair

Christina Bucci-Rechtweg, MD, Head, Pediatric and Maternal Health Policy, Global Drug Development Regulatory Affairs, Pediatric Center of Excellence, Novartis Pharmaceuticals Corporation

Cheryl Yoder will talk about the difference in outlook their family had between the SMA diagnoses of their daughter and son. The Yoder's daughter was diagnosed in 2012, at a time when no treatments were on the horizon. Just three years later, in 2015, her son was able to take part in a Phase II clinical trial of nusinersen.

Cheryl Yoder, Patient Advocate Cure SMA

9:00-10:30AM — **Session 1:** Developing Innovative Technologies for Children: Pediatric-Directed Drug Development

Session Chair
Andrew Mulberg, MD, Head, Senior Vice President, Global Regulatory Affairs, Amicus Therapeutics

This session will explore the innovative use of technologies in pediatric drug development. A presentation of scientific underpinnings of novel technology for the treatment of inborn errors of metabolism and CNS neurodegenerative diseases will be presented. Overcoming the technical challenges of gene therapy to the CNS, approaches for improved protein expression targeting, and stability for novel gene therapy. The presentations will be followed by a panel discussion.

James Wilson, MD, PhD, University of Pennsylvania, Orphan Disease Center

Kathrin Meyer, DrSc, PhD, Principal Investigator, Assistant Professor, Pediatrics, The Ohio State University, Nationwide Children's Hospital

Panelist
Cheryl Yoder, Patient Advocate, Cure SMA

10:30-11:00AM — **Refreshment and Networking Break**

11:00AM-12:30PM **Session 2:** Innovation Through Extrapolation: Improving the Efficiency and Effectiveness of Pediatric Drug Development

Session Co-Chairs
Yeruk (Lily) Mulugeta, PharmD, Associate Director, Division of Pediatric and Maternal Health, OCP, OTS, CDER, FDA

Robert Nelson, MD, PhD, Senior Director, Pediatric Drug Development, Johnson and Johnson

This session will explore the innovative use of extrapolation in pediatric drug development. A presentation of the conceptual, clinical, and ethical justification for the use of extrapolation will be followed by presentations exploring: (1) the use of modeling and simulation to establish and/or assess pediatric exposure and response for both small molecules and biologics and (2) innovative statistical approaches, including Bayesian designs, to borrowing source data to reduce the need for data from the target pediatric population. The presentations will be followed by a panel discussion.

The Use of Extrapolation in Pediatric Drug Development
Robert Nelson, MD, PhD, Senior Director, Pediatric Drug Development, Johnson and Johnson

Marc R. Gastonguay, PhD, Chief Executive Officer, Metrum Research Group

Lisa Hampson, Associate Director, Statistical Methodology and Consulting, Novartis, Switzerland

Nikolay Nikolov, MD, Associate Director for Rheumatology, Division of Pulmonary, Allergy, and Rheumatology Products (DPARP), FDA

12:30-1:30PM **Luncheon and Networking**

1:30-3:00PM **Session 3:** Leveraging Innovative Trials Design I: Product Development-Centric Registries and Platforms

Session Co-Chairs
Edward Connor, MD, MBE, FAAP President, Clinical Research Alliance; Chairman and President, I-ACT for Children

Andrew Mulberg, MD, Head, Senior Vice President, Global Regulatory Affairs, Amicus Therapeutics

This session will explore the innovative use of trial designs and registry development to address clinical issues including understanding natural history and outcome assessment for effective endpoint development in pediatric drug development. A presentation of scientific underpinnings of effective and existing registry development for the treatment of pediatric diseases will be presented.

Laura Schanberg, MD, Associate Professor and Co-chief, Pediatric Rheumatology, Duke University Medical Center

John-Michael Sauer, PhD, Biomarkers Program Officer and Executive Director, Predictive Safety Testing Consortium and Inflammatory Bowel Disease Group, Critical Path Institute

Abby Bronson, MBA, Senior Vice President, Research Strategy, Parent Project Muscular Dystrophy

3:00-3:30PM **Refreshment and Networking Break**

3:30-5:00PM **Session 4:** Leveraging Innovative Trials Design II Panel Discussion

Session Co-Chairs
Robert Nelson, MD, PhD, Senior Director, Pediatric Drug Development, Johnson and Johnson

Christina Bucci-Rechtweg, MD, Head, Pediatric and Maternal Health Policy, Global Drug Development Regulatory Affairs, Pediatric Center of Excellence Novartis Pharmaceuticals Corporation

This session will explore the use of innovative trial designs that have the strong potential to accelerate approval of new pediatric therapeutics. Panelists will be challenged to reflect on strategies that are being discussed with regulatory agencies, and employed across small, focused biotech companies and large pharma to address varying pipeline needs within today's complex development environment.

Edward Connor, MD, MBE, FAAP President, Clinical Research Alliance; Chairman and President, I-ACT for Children

Thomas Miller, PhD, Vice President and Global Head, Pediatrics, Bayer

Andrew Mulberg, MD, Head, Senior Vice President, Global Regulatory Affairs, Amicus Therapeutics

Yeruk (Lily) Mulugeta, PharmD, Associate Director, Division of Pediatric and Maternal Health, OCP, OTS, CDER, FDA

Robert Nelson, MD, PhD, Senior Director, Pediatric Drug Development, Johnson and Johnson

5:00-6:00PM **Networking Reception**

DAY TWO | TUESDAY OCTOBER 29

7:00AM-12:00PM **Registration**

7:00-7:55AM **Continental Breakfast and Networking**

7:55-8:00AM **Welcome to Day Two**

8:00-9:30AM **Session 5:** Making Innovative Infrastructure for Pediatric Product Development a Reality

Session Chair
Edward Connor, MD, MBE, FAAP President, Clinical Research Alliance; Chairman and President, I-ACT for Children

A major gap in the development of innovative drugs for children has been lack of a sustainable pediatric-focused clinical trials infrastructure. Over the past few years, significant progress has been made in making such infrastructure a reality on a global scale. This session will cover the challenges in execution of regulatory-grade clinical trials in children, public-private initiatives that are now being implemented in the US and Europe to address these challenges, and a view of the innovative capabilities and resources available to facilitate planning and execution of pediatric clinical trials.

Ensuring Capability for Regulatory Grade Trials in Children Through Public Private Collaboration
Ronald Portman, MD, FAAP, FASN, FASH, Executive Director, Pediatric Development, Science and Innovation, Pediatric Center of Excellence, Clinical Development & Analytics, Novartis Pharmaceuticals Corporation

Pediatric Regulatory Considerations and the Need for Collaboration
Susan McCune, MD, Director, Office of Pediatric Therapeutics, OCPP, OC, FDA

Global Initiatives to Maximize Efficiency, Quality, and Impact of Pediatric Clinical Trials
Mark Turner, MD, PhD, MRCP, FFPM, Co-Director International Neonatology Consortium, NIHR Clinical Research Network Children's, Theme Liverpool Women's Hospital, United Kingdom

Edward Connor, MD, MBE, FAAP President, Clinical Research Alliance; Chairman and President, I-ACT for Children

Real World Data: A Critical Element of Pediatric Trial Success
Vicki-Seifert Margolis, PhD, CEO, MyOwnMed

9:30-10:00AM **Refreshment and Networking Break**

10:00AM-12:00PM **Session 6:** Innovative Business Models for Pediatric Therapeutic Development

Session Co-Chairs
Thomas Miller, PhD, Vice President and Global Head, Pediatrics, Bayer

Christina Bucci-Rechtweg, MD, Head, Pediatric and Maternal Health Policy, Global Drug Development Regulatory Affairs, Pediatric Center of Excellence Novartis Pharmaceuticals Corporation

In recent years, deliberate prioritization of medication development for children has accelerated broadly across the life sciences ecosystem. This session will explore evolving, innovative business models for pediatric therapeutics. Presentations will focus on the increasingly important role of key stakeholders including large pharma, venture capital, and new academic medical center business models relating to pediatric therapeutics development. Presentations will be followed by an engaging panel discussion.

Pharma Portfolio Considerations for Pediatric Development Programs
Thomas Miller, PhD, Vice President and Global Head, Pediatrics, Bayer

TRINITY Whitepaper: What We Value – the Proposition Behind the Price
Gavin Miyasato, MS, Associate Director of Statistics, Trinity

Redefining the Approach for Pediatric Academic Medical Centers
Dan Fields, JD, MBA, Vice President, Business Innovation, Children's Hospital of Philadelphia

12:00-12:15PM **Closing Remarks**

Summary of Take-a-Ways and Calls to Action