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

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
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<tbody>
<tr>
<td>7:00AM-5:00PM</td>
<td>Registration</td>
<td>Regency Foyer IV</td>
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<tr>
<td>7:00-8:00AM</td>
<td>Continental Breakfast and Networking</td>
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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

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

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<td>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.</td>
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<td>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.</td>
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<td><strong>James Wilson, MD, PhD</strong>, University of Pennsylvania, Orphan Disease Center</td>
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<td><strong>Kathrin Meyer, DrSc, PhD</strong>, Principal Investigator, Assistant Professor, Pediatrics, The Ohio State University, Nationwide Children’s Hospital</td>
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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