EFGCP ANNUAL PAEDIATRIC CONFERENCE

In Person Event

Better Medicines for Children

Global Paediatric Drug Development

The value of multi-stakeholders' collaboration

17&18 October 2023

AGENDA AT A GLANCE

TUESDAY 17 OCTOBER 2023

09:00	**Registration and welcome coffee**
10:30	Opening of the Conference and Introduction
10:45	Revision of the Pharmaceutical Legislation – Impact on Paediatric & Orphan Drug Development
11:45	Panel discussion: Stepwise Paediatric Investigation Plan
12:45	**Lunch**
14:00	Panel discussion: The value of Multi-stakeholders' Collaboration for Paediatric Drug Development at the Global Level
15:30	Breakout Sessions: • Inclusion of Adolescents in Adult Trials • Data Collection and Data Sharing • Paediatric Extrapolation – What progress has been made since 2022?
17:00	Coffee Break
17:30	Fireside Chat with PDCO chair and vice-chair
18:30	**End of Day 1**

WEDNESDAY 18 OCTOBER 2023

08:00	**Welcome Coffee**
08:30	Welcome and Debrief from Day 1
08:45	Panel Discussion: The Enpr-EMA Initiative on Site Quality Criteria/ Standards for Paediatric Trials
09:30	Panel Discussion: The Value of Digital Health Technologies in Paediatric Drug Development
10:30	**Coffee Break**
11:00	Panel Session: The Rare Disease MOONSHOT Initiative: how to make rare diseases more common through drug development optimisation?
11:30	Paediatric formulation development between patients' needs, methodology and regulatory requirements
12:30	**Lunch**
14:00	Panel Discussion: Neonatal Research – What progress has been made since the 2022 Paediatric Conference?
15:00	Feedback from the Breakout Sessions
16:15	**End of Day 2 – Conclusion & Farewell**

RATIONALE

This year will give us the opportunity to reconnect in person after 3 years of virtual conferences due to the pandemic.

The EFGCP "Better Medicines for Children" Conference is an annual flagship event providing a unique opportunity to share best practices, reflect on what has happened since we last met in October 2022, and discuss paediatric updates with a truly global outreach. Despite an ever changing and challenging environment, we believe we have been able to organise a dynamic, innovative, and highly engaging conference to inform our delegates of the latest scientific and regulatory developments in the paediatric space.

The Conference will focus on **Multi-stakeholders' collaboration**, its challenges and solutions, and in particular its values, with a focus on what has been achieved in Europe, in the US and globally, whether for example, through Public-Private Partnerships, e.g., the EU Innovative Health Initiative or the US Critical Path Institute. To this effect, the Conference will bring together not only distinguished speakers from all around the world but also all the relevant stakeholders and experts involved in paediatric drug development.

Day One will be the opportunity to hear from the EU Commission, where they are with the **revision of Pharmaceutical Legislation** and its impact on paediatric drug development. The stakeholders who have already used the **Stepwise Paediatric Investigation Plan Pilot**, established by EMA in February 2023, will share their experience so far. Stakeholders from around the world will share their view on the **value of multi-stakeholders' collaboration**, what can be learnt, and what could be expected in the future to address unmet needs of underserved populations, such as neonates or those with rare diseases.

Active engagement of conference delegates will be then welcomed in **Breakout Sessions** to discuss challenges and identify solutions on what could be done to optimise children's access to new medicines, with a focus on important topics:

1. Inclusion of Adolescents in Adult Trials.
2. Data collection and Data sharing.
3. Paediatric Extrapolation: What progress has been made since 2022?

At the end of Day 1, **a fireside chat** will be the opportunity to hear from Brian Aylward (PDCO chair) and Sylvie Benchetrit (PDCO vice-chair) about their respective activities and priorities, and to hear their views and answers to questions raised ahead of the conference.

Day two will be the opportunity to keep the momentum with some of the experts who joined the 2022 Conference. Hot paediatric topics for which multi-stakeholders' collaboration is more than ever valued to address gaps in scientific knowledge and find synergies to optimise patients' access to new medicines, will explore innovative development avenues that could foster paediatric drug development. A panel committed to finding solutions for **rare diseases**, which present a unique public health challenge, will discuss the latest information on rare diseases. Similarly, new initiatives will be discussed, and their respective panels will explain their strategy to deliver tangible and innovative changes, whether for **neonates**, for the **selection of investigational sites** through quality criteria and standards for paediatric trials, or for **the implementation of a platform for the use of Paediatric Oncology RWE in regulatory and clinical decision making.**

The programme committee looks forward to welcoming you all to the Conference for a global educational and networking event that is a unique opportunity to learn from one another, to meet and interact with experienced colleagues from all parts of the world.

NEWS!
For those interested in paediatric drug development, a pre-conference Workshop on **"Dose-finding/Dose selection"** has been set up on **October 12th, 2023**. Determining the appropriate dose and regimen is one of the hardest and most important tasks during the development of new paediatric drugs to ensure an appropriate balance of risks and benefits for children. This workshop will be the opportunity to learn from experts how to identify the right dose to be prescribed in children depending on their age/weigh, a topic detailed in the ICH E4 guideline which should be revisited in the future, according to the ICH Model Informed Drug Development (MIDD) Road Map released by the MIDD discussion group in March 2022.

ACKNOWLEDGEMENT

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◇ PROGRAMME COMMITTEE ◇

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Virginie Hivert	Pfizer, The Netherlands
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Yann Le Cam	Eurordis, France
Yury Kiselev	European Medicines Agency, The Netherlands

DAY ONE – 17 OCTOBER 2023

09:00 **Registration and welcome coffee**

10:30 **Introduction to the Conference**

Martine Dehlinger-Kremer, *EFGCP, EUCROF & ICON Plc.* **and Begonya Nafria Escalera**, *Barcelona Children's Hospital & eYPAGnet*

10:45 **Revision of the Pharmaceutical Legislation – Impact on Paediatric & Orphan Drug Development**

Moderator: **Alexandru Costescu,** *European Commission*
Panellists: **Fabio d'Atri,** *European Commission, DG Santé***; Gilles Vassal,** *Gustave Roussy***; Victoria Kitcatt,** *Pfizer;* **Yann Le Cam,** *Eurordis*

11:45 **Stepwise Paediatric Investigation Plan**

Moderator: **Gilles Vassal,** *Gustave Roussy* **and Karl-Heinz Huemer,** *former PDCO member*

Panellists: **Brian Aylward,** *PDCO chair***; Giovanni Lesa,** *EMA***; and Gesine Bejeuhr,** *Bayer*

12:45 **Lunch break**

14:00 **Panel session**

The Value of Multistakeholders' Collaboration for Paediatric Drug Development at the Global Level

Moderators: **Alexandru Costescu,** *European Commission* **and Angeliki Siapkara,** *AstraZeneca*

Panellists: **Andy Pearson,** *ACCELERATE;* **Begonya Nafria Escalera,** *Barcelona Children's Hospital & eYPAGnet;* **Cecile Ollivier,** *C-Path Institute***; Heidrun Hildebrand,** *Bayer***; Lynne P. Yao,** *FDA***; Mark Turner,** *Conect4Children***; Martina Penazzato**, *WHO;* **Nathalie Seigneuret,** *IHI***; Ralph Bax,** *EMA*

15:30 **Breakout Sessions**

- Inclusion of Adolescents in Adult Trials
  Moderators: **Rhian Thomas Turner,** *Noah's Ark Children's Hospital for Wales* **and Solange Corriol-Rohou,** *AstraZeneca*
  Panellists: **Amy Cheung,** *Certara;* **Max Williamson,** *ACCELERATE FAIR Work Group*

- Data Collection and Data Sharing
  Moderators: **Angeliki Siapkara,** *AstraZeneca* **and David Sibbald,** *Aridhia*
  Panellists: **Alexandre Bétourné,** *C-Path Institute;* **Becca Leary,** *Conect4Children;* **Jeffrey Barrett**, *Arhidia*

- Paediatric Extrapolation – What progress has been made since 2022?
  Moderators: **Richard Vesely,** *Allucent* **and Sabine Fuerst-Recktenwald,** *Roche*
  Panellists: **Andrew Thomson,** *EMA;* **Eric Zuckerman,** *Pediatric IBD Foundation;* **Lynne P. Yao,** *FDA*

17:00 **Coffee break**

17:30 **Fireside Chat with**
Moderator: **Mark Turner**, *Conect4Children*

Brian Aylward, *PDCO Chair* **and Sylvie Benchetrit,** *PDCO Vice-Chair*

18:30 **End of Day 1**
Martine Dehlinger-Kremer, *EFGCP, EUCROF & ICON Plc.*

◇ DAY TWO – 18 OCTOBER 2023 ◇

08:00 **Welcome Coffee**

08:30 **Welcome and Debrief from Day 1**
Martine Dehlinger-Kremer, *EFGCP, EUCROF & ICON Plc.* **and** Begonya **Nafria Escalera,** *Barcelona Children's Hospital & eYPAGnet*

08:45 ***The Enpr-EMA Initiative on Site Quality Criteria/ Standards for Paediatric Trials***
Moderators: **Gunter Egger,** *EMA* **and Pirkko Lepola,** *FINPEDMED*
Panellists: **Begonya Nafria Escalera,** *Barcelona Children's Hospital & eYPAGnet;* **Ensio Norjavaara,** *AstraZeneca***; Kirsten Sherman Cervati,** *ICON***; Eva Degraeuwe,** *Ghent University* **and Ricardo Fernandes,** *STAND4Kids*

09:30 **Panel Session**
The Value of Digital Health Technologies in Paediatric Drug Development
Moderators: **Angeliki Siapkara,** *AstraZeneca* **and Laurent Servais,** *Oxford University*
Panellists: **Niina Kolehmainen,** *Newcastle University*; **Tim Buckinx,** *Epihunter*; **Yury Kiselev,** *EMA*

10:30 **Coffee Break**

11:00 **Panel Session**
The Rare Disease MOONSHOT Initiative: How to make rare diseases more common through drug development optimisation?
Moderators: **Cecile Ollivier**, *C-Path Institute* **and Solange Corriol-Rohou,** AstraZeneca
Panellists: **Christina Kyriakopoulou,** *European Commission* **and Yanis Mimouni,** *EJP RD*

11:30 **Paediatric Formulation Development between Patient's needs, Methodology and Regulatory Requirements**
Moderators: **Martine Dehlinger-Kremer**, *ICON, EUCROF &EFGCP* **and Martina Penazzato**, *WHO*
Panellists: **Sybille Reidemeister,** *Novartis*; **Viviane Klingmann,** *Paediatric University Hospital, Düsseldorf;* **Patient representative,** *TBA*

12:30 **Lunch Break**

14:00 ***Neonatal Research – What progress has been made since the 2022 Paediatric Conference?***
Moderators: **Mark Turner,** *Conect4Children* **and Solange Corriol-Rohou,** *AstraZeneca*
Panellists: **Christina Tischer,** *EFCNI;* **Jeff Barrett,** *Aridhia***; John van den Anker,** *Children's National Medical Center Washington***; Ralph Bax,** *EMA* **and Susan McCune,** *PPD*

15:00 **Feedback from the Breakout Sessions**

- Inclusion of Adolescents in Adult Trials
- Data Collection and Data Sharing
- Paediatric Extrapolation – What progress has been made since 2022?

16:15 **End of Day 2 – Conclusion & Farewell**
Martine Dehlinger-Kremer, *EFGCP, EUCROF & ICON Plc.*

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ANNUAL PAEDIATRIC CONFERENCE

Better Medicines for Children
Global Paediatric Drug Development
The value of multi-stakeholders' collaboration

Conference Language: English

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- Click Here -

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