

EFGCP ANNUAL

PAEDIATRIC CONFERENCE



VIRTUAL EVENT

BETTER MEDICINES FOR CHILDREN

Global Paediatric Drug Development

Progress made & always remaining challenges

18&19

October

2022



AGENDA AT A GLANCE

TUESDAY 18 OCTOBER 2022

11:45 Opening of Conference

12:00 Welcome and Introduction to the Conference

12:10 PDCO Vision

12:40 Implementation of the CT Regulation 536/2014 – Impact on Paediatric trials

13:30 Panel discussion: Global / transatlantic alignment for the development of paediatric products – what can be learnt from paediatric oncology drug development?

14:30 Coffee break

14:45 Panel session: Rare diseases – what is happening and can be expected?
EU Commission/EU joint program on rare diseases

15:45 Breakout sessions:

- How to improve Patient & Parents involvement in Paediatric Research?
- Paediatric Lay Summaries
- Is it time to prioritise?

17:15 Fireside Chat

18:15 End of Day 1

WEDNESDAY 19 OCTOBER 2022

11:45 Opening of Conference

12:00 Welcome and Debrief from Day 1

12:10 Panel Session: Optimising Paediatric Drug Development – what progress has been made since the 2021 Paediatric Conference?

13:30 Panel Session: Neonatal Research – where are we and what could be next steps?

14:30 Panel Session: What's new at ICH for paediatric drug development?

15:30 Coffee break

15:50 Panel Session: Can drug repurposing benefit drug development in Children & in Paediatric Rare Diseases?

16:50 Feedback from the Breakout Sessions

17:50 End of Day 2 – Conclusion & Farewell

The EFGCP “Better Medicines for Children” Conference is an annual flagship event providing a unique opportunity to share best practice, reflect on what has happened since we last met in October 2021, and discuss paediatric updates with a truly global outreach. Despite an ever changing and challenging environment, and the need to meet virtually again, 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.

The conference will focus on **Global Paediatric Development**, its challenges and solutions, and in particular on what has been achieved during the pandemic, and the remaining opportunities and challenges. To this effect, the conference brings together not only distinguished speakers from all around the world but also all the stakeholders involved in paediatric drug development.

Day One will bring regulators and patient representatives to discuss the potential impact on R&D of the **revision of paediatric regulation** and of the **implementation of the Clinical Trial Regulation**. Stakeholders from around the world while focusing on **global alignment** will share their view on what can be learnt from paediatric oncology 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.

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. How to improve Patient and parents’ involvement in Paediatric research?
2. Paediatric Lay Summaries
3. Is it time to prioritise?

At the end of Day 1, a **fireside chat** will be the opportunity to hear from Dr Koen Norga (previous PDCO chair), and Dr Angeliki Siapkara (MHRA and former UK representative on PDCO) about their respective activities and priorities; and also to hear their view and answers to questions raised ahead of the conference.

Day two will be the opportunity to keep the momentum with experts who joined the 2021 conference. Hot paediatric topics for which multi-stakeholders’ collaboration is more than ever valued to address gaps in scientific knowledge, will explore innovative development avenues that could foster paediatric drug development.

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 colleagues from all parts of the world.

We do hope that the 2022 conference will be the last one organised remotely, and 2023 will give us to go back to face-to-face meetings.

NEWS! For those interested in paediatric drug development, a pre-conference Workshop on **the "Fundamentals of Paediatric Extrapolation"** has been set up on October 17. This will be the opportunity to learn from the experts the value of paediatric extrapolation, a topic detailed in the ICH E11 guideline recently released for public consultation. For further details on this pre-conference Workshop, please visit the webpage.

Thank you for joining us!

ACKNOWLEDGEMENT

EFGCP Thanks its Partners & Members for their continued support

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UiO : University of Oslo



Alexandru Costescu
Angelika Joos
Angeliki Siapkara
Begonya Nafria Escalera
Gilles Vassal
Helen Shaw
Koenraad Norga
Martine Dehlinger-Kremer
Rhian Thomas-Turner
Sabine Fuerst-Recktenwald
Solange Corriol-Rohou
Thomas Severin

European Commission, Belgium
 MSD, Belgium
 MHRA, United Kingdom
 Sjd Children's Hospital Barcelona, Spain
 Gustave Roussy Institute, France
 Proveca, United Kingdom
 Antwerp University Hospital, Belgium & Former Chair of EMA PDCO, NL
 EFGCP, EUCROF & ICON Plc., Germany
 NHS, United Kingdom
 Roche, Switzerland
 AstraZeneca, France
 Novartis, Switzerland

Faculty

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Angeliki Siapkara
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Billy Amzal
Brian Aylward
Christina Bucci-Rechtweg
Christina Kyriakopoulou
Claudio Fracasso
Corinne de Vries
David Hayes
Dimitrios Athanasiou
Dominik Karres
François Houyez
Gilles Vassal
Gunter Egger
Helen Mao
Hannah Batchelor
Isaac Rodriguez-Chavez
Jeffrey Barrett
John van den Anker
Kaye Robertson
Koenraad Norga

Kristin Karlsson
Kristina An Hack
Lynne Yao
Marie-Yvonne Douste-Blazy
Mark Turner
Martha Donoghue
Martine Dehlinger-Kremer
Nicola Ruperto
Nicolas Frey
Pamela Dicks
Ralph Bax
Rhian Thomas-Turner
Sabine Fuerst-Recktenwald
Sam Blackman
Solange Corriol-Rohou
Susan McCune
Zhimin Yang

European Commission, Belgium
 Swedish MPA, Sweden
 MHRA, United Kingdom
 Sjd Children's Hospital Barcelona, Spain
 Quinten Health, France
 Chair PDCO, EMA
 Novartis, USA
 European Commission, Belgium
 Pfizer, Italy
 EMA, The Netherlands
 AstraZeneca, USA
 EURORDIS, Greece
 EMA, The Netherlands
 EURORDIS, France
 Gustave Roussy Institute, France
 Enpr-EMA, EMA, the Netherlands
 Health Canada, Canada
 Institute of Pharmacy and Biomedical Sciences, University of Strathclyde, Scotland
 ICON, USA
 Critical-Path Initiative, USA
 Children's National Medical Center Washington, DC, USA
 TGA, Australia
 Antwerp University Hospital, Belgium & previous Chair of EMA PDCO, The Netherlands
 Swedish MPA, Sweden
 Sanofi, France
 FDA, USA
 MYDB Paediatric Clinical Insights, France
 Liverpool University, United Kingdom
 FDA, USA
 EFGCP, EUCROF & ICON Plc., Germany
 PRINTO, Italy
 Roche, Switzerland
 NHS, United Kingdom
 EMA, the Netherlands
 NHS, United Kingdom
 Roche, Switzerland
 DayOne, USA
 AstraZeneca, France
 PPD, USA
 NMPA, China

- 11:45** **Opening of Conference** *with a video with the young people from eYPAGnet*
European Young Person's Advisory Groups Network
- 12:00** **Welcome and Introduction to the Conference**
Martine Dehlinger-Kremer, EFGCP, EUCROF & ICON Plc.
- 12:10** **PDCO Vision**
Brian Aylward, Chair PDCO, EMA
- 12:40** **Implementation of the CT Regulation 536/2014 – Impact on Paediatric trials**
◇ Moderator: **Martine Dehlinger-Kremer**, EFGCP, EUCROF & ICON Plc.
◇ **Ann Marie Janson-Lang**, Swedish MPA & **Nicola Ruperto**, PRINTO
- 13:30** **Panel discussion**
Global / transatlantic alignment for the development of paediatric products – what can be learnt from paediatric oncology drug development?
◇ Moderator: **Gilles Vassal**, Gustave Roussy Institute
◇ Panel: **Martha Donoghue**, FDA | **Dominik Karres**, EMA | **Helen Mao**, Health Canada | **Angeliki Siapkara**, MHRA | **Zhimin Yang**, NMPA | **Kaye Robertson**, TGA
- 14:30** **Coffee break**
- 14:45** **Panel session**
Rare diseases – what is happening and can be expected?
EU Commission/EU joint program on rare diseases
◇ Moderator: **Angeliki Siapkara**, MHRA
◇ Panel: **Kristina An-Haack**, Sanofi | **Christina Kyriakopoulou**, EU Commission | **Dimitrios Athanasiou**, EURORDIS
- 15:45** **Breakout sessions**

How to improve Patient & Parents involvement in Paediatric Research?
◇ Moderators: **Pamela Dicks**, NHS, eYPAGnet & **Rhian Thomas-Turner**, NHS

Paediatric Lay Summaries
◇ Moderators: **Begonya Nafria Escalera**, SJD Hospital, eYPAGnet | **Nicola Ruperto**, PRINTO

Is it time to prioritise?
◇ Moderators: **Gilles Vassal**, Gustave Roussy Institute & **Solange Corriol-Rohou**, AstraZeneca
- 17:15** **Fireside Chat with**
Koenraad Norga, Former PDCO Chair | **Angeliki Siapkara**, MHRA
◇ Moderator: **Claudio Fracasso**, Pfizer
- 18:15** **End of Day 1**
Martine Dehlinger-Kremer, EFGCP, EUCROF & ICON Plc.

◇ DAY TWO – 19 OCTOBER 2021 ◇

- 11:45** **Opening of Conference** *with a video with the young people from eYPAGnet*
European Young Person's Advisory Groups Network
- 12:00** **Welcome and Debrief from Day 1**
Martine Dehlinger-Kremer, EFGCP, EUCROF & ICON Plc.
- 12:10** **Panel Session**
Optimising Paediatric Drug Development – what progress has been made since the 2021 Paediatric Conference?
◇ Moderators: **Sabine Fuerst-Recktenwald**, Roche & **Gilles Vassal**, Gustave Roussy Institute
- **Innovative Techniques towards Drug Development:** **Hannah Batchelor**, Institute of Pharmacy and Biomedical Sciences, University of Strathclyde
 - **Use of RWD in paediatric drug development:** **Marie-Yvonne Douste-Blazy**, MYDB Paediatric Clinical Insights
 - **Decentralised Clinical Trials:** **Isaac Rodriguez-Chavez**, ICON
 - **Adolescents in Adult Clinical Trials:** **Christina Bucci-Rechtweg**, Novartis
- 13:30** **Panel Session**
Neonatal Research – where are we and what could be next steps?
◇ Moderators: **Solange Corriol-Rohou**, AstraZeneca & **Mark Turner**, Liverpool University
◇ Panel: **Jeff Barrett**, C-Path Initiative | **John van den Anker**, Children's National Medical Center Washington | **Ralph Bax**, EMA | **Susan McCune**, PPD
- 14:30** **Panel Session**
What's new at ICH for paediatric drug development?
◇ Moderators: **Solange Corriol-Rohou**, AstraZeneca & **Sabine Fuerst-Recktenwald**, Roche
- **ICH E11A:** **Lynne Yao**, FDA
 - **MIDD:** **Kristin Karlsson**, MPA & **Nicolas Frey**, Roche
 - **Pregnant & Breast-feeding Individuals in CTs:** **Corinne de Vries**, EMA
- 15:30** **Coffee break**
- 15:50** **Panel Session**
Can drug repurposing benefit drug development in Children & in Paediatric Rare Diseases?
◇ Moderators: **Alexandru Costescu**, EU Commission & **David Hayes**, AstraZeneca
◇ Panel: **Billy Amzal**, Quinten Health | **François Houyez**, EURORDIS | **Gunter Egger**, Enpr-EMA, EMA | **Sam Blackman**, DayOne
- 16:50** **Feedback from the Breakout Sessions**
- How to improve Patient involvement in Paediatric Research?
 - Paediatric Lay Summaries
 - Is it time to prioritise?
- 17:50** **End of Day 2 – Conclusion & Farewell**
Martine Dehlinger-Kremer, EFGCP, EUCROF & ICON Plc.



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