

EFGCP ANNUAL

PAEDIATRIC CONFERENCE

EF  
GCP

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 Revision of the Paediatric & Orphan Regulations

12:45 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 (PDCO chair), Dr Sabine Scherer (PDCO vice-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**

**UZ LEUVEN**

Vienna Vaccine Safety Initiative



**University Hospital Basel**

**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 & Chair of EMA PDCO, NL  
EFGCP, EUCROF & ICON Plc., Germany  
NHS, United Kingdom  
Roche, Switzerland  
AstraZeneca, France  
Novartis, Switzerland

## Faculty

**Alexandru Costescu**  
**Ann Marie Janson Lang**  
**Angeliki Siapkara**  
**Begonya Nafria Escalera**  
**Christina Bucci-Rechtweg**  
**Christina Kyriakopoulou**  
**Claudio Fracasso**  
**Corinne de Vries**  
**Dimitrios Athanasiou**  
**Dominik Karres**  
**Gregory Reaman**  
**Gilles Vassal**  
**Gunter Egger**  
**Helen Mao**  
**Helen Shaw**  
**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**  
**Martine Dehlinger-Kremer**  
**Nick Sireau**  
**Nicola Ruperto**  
**Nicolas Frey**  
**Pamela Dicks**  
**Ralph Bax**  
**Rhian Thomas-Turner**  
**Sabine Fuerst-Recktenwald**  
**Sabine Scherer**  
**Sam Blackman**  
**Solange Corriol-Rohou**  
**Susan McCune**  
**Zhimin Yang**

European Commission, Belgium  
Swedish MPA, Sweden  
MHRA, United Kingdom  
Sjd Children's Hospital Barcelona, Spain  
Novartis, USA  
European Commission, Belgium  
Pfizer, Italy  
EMA, The Netherlands  
EURORDIS, Greece  
EMA, The Netherlands  
FDA, USA  
Gustave Roussy Institute, France  
Enpr-EMA, EMA, the Netherlands  
Health Canada, Canada  
Proveca, United Kingdom  
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 & Chair of EMA PDCO, The Netherlands  
Swedish MPA, Sweden  
Sanofi, France  
FDA, USA  
MYDB Paediatric Clinical Insights, France  
Liverpool University, United Kingdom  
EFGCP, EUCROF & ICON Plc., Germany  
AK, United Kingdom  
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Roche, Switzerland  
BfArM & Vice-Chair of EMA PDCO, Germany  
DayOne, USA  
AstraZeneca, France  
PPD, USA  
NMPA, China

## ◇ DAY ONE – 18 OCTOBER 2021 ◇

- 11:45**      **Opening of Conference**
- 12:00**      **Welcome and Introduction to the Conference**  
**Martine Dehlinger-Kremer**, *EFGCP, EUCROF & ICON Plc.*
- 12:10**      **Revision of the Paediatric & Orphan Regulations**
- 12:45**      **Implementation of the CT Regulation 536/2014 – Impact on Paediatric trials**  
◇ **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**, *IGR*  
◇ Panel: **Gregory Reaman**, *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* & **Rhian Thomas-Turner**, *NHS*
- Paediatric Lay Summaries**  
◇ Moderators: **Begonya Nafria Escalera**, *SJD Hospital* | **Nicola Ruperto**, *PRINTO*
- Is it time to prioritise?**  
◇ Moderators: **Gilles Vassal**, *IGR* & **Solange Corriol-Rohou**, *AstraZeneca*
- 17:15**      **Fireside Chat with**  
**Koenraad Norga**, *PDCO Chair* | **Sabine Scherer**, *PDCO Vice-Chair* | **Angeliki Siapkara**, *MHRA*  
◇ Moderator: **Claudio Fracasso**, *Pfizer*
- 18:15**      **End of Day 1**  
**Martine Dehlinger-Kremer**, *EFGCP, EUCROF & ICON Plc.*





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Conference Language: English

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