



EFGCP ANNUAL PAEDIATRIC CONFERENCE **BETTER MEDICINES FOR CHILDREN** *INNOVATION IN PAEDIATRIC DRUG DEVELOPMENT AS A DRIVER FOR CHANGE*

 21 & 22 October 2025

 Hotel NH Amsterdam Schiphol Airport

> REGISTER HERE <





RATIONALE

Thank you for joining us!

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 2024, 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 for advancing paediatric research and innovation across Europe and beyond.

This year will be a hybrid conference but we strongly encourage you to attend in-person in Amsterdam at the NH Amsterdam Schiphol Airport Hotel as this would be a great opportunity to connect with key stakeholders in paediatric drug development and create the all-important relationships with other delegates.

The conference will focus on **“Innovation in paediatric drug development as a driver for change”**. At the heart of the Conference lies a resolute commitment to patient-centricity—placing the voices, needs, and lived experiences of children and their families at the forefront of paediatric research and innovation. The conference will bring together distinguished speakers from all around the world and relevant stakeholders and experts to resolve the most urgent and complex challenges in paediatric drug development.

Day One, will start with a Keynote speech from a parent on the impact of trial participation, experience and solutions for study success. As the conference continues there will be the opportunity to discuss novel approaches to drug development in paediatric chronic kidney disease as well as key opportunities in streamlining global clinical trials. We will continue to unpack the implications of evolving EU legislation with a discussion amongst all stakeholders affected by it. Finally, we will explore the integration of artificial intelligence (AI) and machine learning (ML) in paediatric data ecosystems.

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

1. Pediatric Extrapolation.
2. Innovative approaches in clinical trials for small populations.
3. Paediatric medical devices.

At the end of Day 1, there will be the opportunity for **an EMA PDCO member**, to share their views and perspective on paediatric drug development and also on the EMA Paediatric Committee (PCDO) work.



RATIONALE

Thank you for joining us!

Day two will start with the feedback and key deliverables from the Breakout Sessions. Then it will be the opportunity to engage on the status of Europe as a **competitive/innovative region for paediatric research** with a panel of experts including regulators from various regions, such as Europe, the UK, Canada and Japan. Further to this session, there will be two sessions that will focus on **Optimising the Clinical Development Path of Orphan Medicines and also on Drug Repurposing**, highlighting the outcomes of several projects and real-life examples that enhance delegates expertise in these fields. These sessions will be then complemented by **Global Regulatory Alignment** and the critical role of these partnerships in derisking drug development. Finally, as a conference first we are proud to present **New voices in Paediatric Research with a focus on real world examples of successful paediatric drug development**.

The Programme Committee looks forward to welcoming you all at 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 and experts from all over of the world.



PROGRAMME COMMITTEE

Angelika	Joos	<i>MSD</i>	Belgium
Angeliki	Siapkara	<i>AstraZeneca</i>	United Kingdom
Begonya	Nafria Escalera	<i>Barcelona Children's Hospital, eYPAGnet, EFGCP</i>	Spain
Christina	Kyriakopoulos	<i>EU Commission</i>	Belgium
Claudio	Fracasso	<i>Hippocrates Research Srl</i>	Italy
Donato	Bonifazi	<i>TEDD - European Network of Excellence for Paediatric research</i>	Italy
Emmanuela	Amoako	<i>Paediatric Oncologist and Yemaachi Biotech</i>	Germany
Fahimeda	Ali	<i>MHRA</i>	United Kingdom
Kristina	An Haack	<i>Sanofi</i>	France
Mark	Turner	<i>conect4children Stichting</i>	United Kingdom
Martine	Dehlinger-Kremer	<i>ICON & EUCROF</i>	Germany
Mette	Due Theilade Thomsen	<i>Pip Adviser</i>	Denmark
Rhian	Thomas-Turner	<i>Noah's Ark Children's Hospital for Wales</i>	United Kingdom
Sabine	Fuerst-Recktenwald	<i>Roche</i>	Switzerland
Solange	Corriol-Rohou	<i>Consultant</i>	France
Thomas	Severin	<i>Novartis</i>	Switzerland
Yuri	Kiselev	<i>EMA</i>	The Netherlands



FACULTY

Alberto	Tozzi	<i>Ospedale Pediatrico Bambino Gesù</i>	Italy
Andrea	Manfrin	<i>MHRA</i>	United Kingdom
Bethan	Phillips	<i>Cardiff and Vale UHB - Children and Young adult research unit</i>	United Kingdom
Dominik	Karres	<i>EMA</i>	The Netherlands
Donato	Bonifazi	<i>TEDDY</i>	Italy
Franca	Ligas	<i>EMA</i>	Italy
Franz	Schaefer	<i>Heidelberg University</i>	Germany
Gilles	Vassal	<i>Gustave Roussy Institute</i>	France
Gunter	Egger	<i>EMA</i>	The Netherlands
Helena	Luning	<i>EUCROF</i>	Sweden
Jari	Petaja	<i>HUS for PHEMS Initiative</i>	Finland
Jennifer	McKenzie	<i>Boehringer-Ingelheim</i>	United States of America
Jyothsna	Krishnan	<i>EFPIA</i>	Belgium
Maria	Baselga	<i>Patient Representative</i>	Spain
Michelle	Haber	<i>FAHMS</i>	Australia
Michiyo	Sakiyama	<i>PMDA</i>	Japan
Ming	Su	<i>Health Canada</i>	Canada
Oscar	Della Pasqua	<i>GSK</i>	United Kingdom
Ralph	Bax	<i>EMA</i>	The Netherlands
Sebastian	Honoré	<i>Cure Lowe Foundation</i>	Denmark
Thorsten	Ruppert	<i>VfA - German Association of research-based Pharmaceutical Companies</i>	Germany

ACKNOWLEDGMENTS

Thank you to our Partners for their continued support!

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AGENDA

Day 1 - 21 October 2025

09:00 REGISTRATION AND WELCOME COFFEE

10:00 Introduction to the Conference

Martine Dehlinger-Kremer, *ICON & EUCROF*

Begonya Nafria-Escalera, *Barcelona Children's Hospital & eYPAGnet*

10:15 KEYNOTE – Patient

Maria Baselga, *Patient Representative*

10:45 Session 1: Challenges and opportunities for paediatric drug development in chronic kidney disease - Panel Discussion

Franz Schaefer, *Heidelberg University* | Gunter Egger, *EMA*

Jennifer Mckenzie, *Boehringer-Ingelheim* | Sebastian Honoré, *Cure Lowe Foundation*

11:45 Session 2: Actions to streamline and facilitate paediatric clinical trial

Bethan Phillips, *Cardiff and Vale UHB - Children and Young adult research unit*

Michelle Haber, *FAHMS*

12:45 LUNCH

13:45 Session 3: EU legislation updates

Gilles Vassal, *Gustave Roussy Institute* | Jyothsna Krishnan, *EFPIA* | Ralph Bax, *EMA*

14:45 Breakout sessions:

1. Pediatric extrapolation

Dominik Karres, *EMA* | Oscar Della Pasqua, *GSK*

Sabine Fuerst-Recktenwald, *Novartis*

2. Innovative approaches in clinical trials for small populations

Donato Bonifazi, *TEDDY* | Solange Corriol-Rohou, *Consultant*

3. Paediatric medical devices and in vitro diagnostics

16:30 COFFEE BREAK

17:00 Session 4: The role of AI within the Paediatric Data Ecosystem

Alberto Tozzi, *Bambino* | Jari Petaja, *HUS for PHEMS Initiative*

18:00 Reflection from the PDCO Chair

18:30 Closing Remarks End of Day 1

19:00 Get-together and Networking



AGENDA

Day 2 - 22 October 2025

08:00 WELCOME COFFEE

08:30 **Welcome and Debrief from Day 1**

08:40 **Feedback from breakout sessions**

09:15 **Session 5: EU as a competitive/innovative region for paediatric research**
Andrea Manfrin, MHRA | Helena Luning, EUCROF | Mark Turner, conect4children
Stichting
Thorsten Ruppert, VfA - German Association of research-based Pharmaceutical
Companies

10:15 COFFEE BREAK

10:35 **Session 6: Optimising the clinical development path of orphan medicine**
Kristina An Haack, Sanofi

11:35 **Session 7: Paediatric Drug repurposing**
Solange Corriol-Rohou, Consultant

12:00 LUNCH

13:30 **Session 8: Global regulatory alignment**
Fahimeda Ali, MHRA | Franca Ligas, EMA | Michiyo Sakiyama, PMDA | Ming Su,
Health Canada

14:30 **Session 9: New voices in paediatric research**

15:30 **End of Day 2 - Conclusion & Farewell**

15:45 END OF CONFERENCE



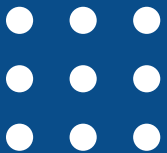
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CONFERENCE LANGUAGE: ENGLISH

EVENTS@EFGCPEU



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