



EFGCP ANNUAL PAEDIATRIC CONFERENCE

BETTER MEDICINES FOR CHILDREN

*COMMON ISSUES IN CLINICAL DEVELOPMENT AMPLIFIED IN
PAEDIATRIC DRUG DEVELOPMENT*

📅 22 & 23 October 2024

📍 Crowne Plaza Brussels Airport

> [CLICK HERE TO REGISTER](#) <





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 2023, 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.

Last year, when we met in October 2023, gave us the opportunity to finally reconnect in person after 3 years of virtual conferences due to the pandemic. This year will also be an in-person conference to happen in Brussels, at the Crowne Plaza Airport Hotel.

The conference will focus on **“Common issues in clinical development amplified in paediatric drug development”**, its challenges and solutions, and in particular its value, with a focus on what can be done and has been achieved in Europe, and this year also in the US and globally . To this effect, the conference will bring together distinguished speakers from all around the world and relevant stakeholders and experts involved in paediatric medicines development.

On **Day One**, further to a Key Note focusing on **Mental Health and Neurologic Disorders in Children**, there will be the opportunity to discuss implications of the new **Mechanism of Action Paediatric Investigation Plan**, which while considered a child-centric option, is a new option considered by Policy makers who are still working on the **revision of the Pharmaceutical Legislation**. **Examples of successful EU-funded paediatric projects**. One session will focus on multi-stakeholders’ collaboration knowing a number of initiatives are already planned to start in 2024, which will benefit all involved in medicines development including children. The last session will provide the opportunity to discuss the **Potential of Artificial Intelligence in Paediatric Drug Development**

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 childrens’ access to new medicines, with a focus on the important topics:

1. Inclusion of Pregnant and Breastfeeding Individuals in Clinical Trials.
2. Use of Real-World Data/Registries in Paediatric Drug Development.
3. Paediatric-only medicines development/First in child companies.

Day Two will provide the opportunity to share **Paediatric Collaboration Activities at the Global Level** with a panel of regulators from various regions, such as Africa & Middle East, Japan, Australia, Canada, Europe, and the USA. This session will be complemented by a session on **Childrens’ Access to Medicines in Developing Countries**. Prior to sharing the feedback from Day 1’s Breakout Sessions, there will be an interesting session, **Health Technology Assessment (HTA) of Paediatric Medicines**, which will be of help to understand what sponsors will be facing with the implementation of the HTA Regulation in early 2025.

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

Angeliki Siapkara
Anja Schiel
Begonya Nafria Escalera
Chrissi Pallidis
Christina Kyriakopoulou
Claudio Fracasso
Gesine Bejeuhr
Gilles Vassal
Kristina An Haack
Mark Turner
Martine Dehlinger-Kremer
Rhian Thomas-Turner
Sabine Fuerst-Recktenwald
Solange Corriol-Rohou
Thomas Severin

AstraZeneca
Norwegian Medicines Agency
Barcelona Children's Hospital, eYPAGnet, EFGCP
European Medicines Agency
European Commission
Hippocrates Research Srl
Bayer
Gustave Roussy Institute
Sanofi
Liverpool University Hospital
ICON, EUCROF, EFGCP
Noah's Ark Children's Hospital for Wales
Roche
AstraZeneca
Novartis

United Kingdom
Norway
Spain
The Netherlands
Belgium
Italy
Germany
France
France
United Kingdom
Germany
United Kingdom
Switzerland
France
Switzerland

FACULTY

Angelika Joos
Argyris Stingarisi
Benedetto Vitiello
Brian Aylward
Carmen Moreno
Cesare Spadoni
Corinne de Vries
Danielle Belgrave
Dimitrios Athanasou
Emmanuella Amoako
Fabio D'Atri
Fahimeda Ali
Jeffrey Barrett
Katherine Donegan
Lauren Wyatt
Lenias Hwenda
Louisa Braun Exner
Lynne Yao
Marc Ramis
Marie Teil
Marina Kolochavina
Martina Penazzato
Michiyo Sakiyama
Moy Bracken
Natsuko Hayama
Roberto De Lisa
Robyn Langham
Sam Blackman
Sara Carucci
Sylvie Benchetrit
Tyrza Boersma
Ulrike Bonati
Victoria Kitcatt
Yashwant Sinha

MDS
University College of London
University of Turin
EMA, HPRA
Hospital Gregorio Maranon
Oncoheros
EMA
GSK
EUPATI
Yemaachi Biotech
European Commission
MHRA
EMA
MHRA
ADHD Foundation
Medicines for Africa Foundation
Danish Medicines Agency, PDCO Member
FDA
Venture Capital
UCB Pharma
Five Voices Consortium
WHO
Pharmaceuticals and Medical Devices Agency
Access to Medicines Foundation
Pharmaceuticals and Medical Devices Agency
EMA
Therapeutic Goods Administration
Day One Biopharmaceutical
Università degli studi di Cagliari
National Agency for the Safety of Medicine and Health Products
Patient representative
Roche
Pfizer
Therapeutic Goods Administration

Belgium
United Kingdom
Italy
Ireland
Spain
Italy
The Netherlands
United Kingdom
Greece
Ghana
Belgium
United kingdom
The Netherlands
United kingdom
United Kingdom
South Africa
Denmark
USA
Spain
Belgium
Germany
Switzerland
Japan
The Netherlands
Japan
The Netherlands
Australia
USA
Italy
France
The Netherlands
Switzerland
United Kingdom
Australia

ACKNOWLEDGMENTS

Thanks its Partners and Sponsors for their continued support!

IN PARTNERSHIP WITH



WITH THE SUPPORT OF

GOLD





AGENDA

Day 1 - 22 October 2024

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 KEY NOTE – Treatment of Psychiatric Disorders in children and adolescents: current situation and needs for research

Carmen Moreno, *Hospital Gregorio Maranon*

10:45 Session 1: Neuro Health

Moderator: **Sabine Fuerst-Recktenwald**, *Roche* | **Sara Carucci**, *Università degli studi di Cagliari*

- Depression
Argyris Stingaris, *University College of London* | **Louisa Braun Exner**, *PDCO representative Denmark*
- Multiple Sclerosis
Ulrike Bonati, *Roche*
- Paediatric Irritability: From Definition and Assessment to Treatment
Benedetto Vitiello, *University of Turin*

11:45 Session 2: Mechanism of Action Paediatric Investigation Plan

Moderators: **Gesine Bejeuhr**, *Bayer* and **Gilles Vassal**, *Gustave Roussy Institute*

Panelists: **Angelika Joos**, *MDS* | **Chrissi Pallidis**, *EMA* | **Sylvie Benchetrit**, *PDCO, EMA*

12:45 LUNCH

13:45 Session 3: Examples of successful EU-funded paediatric projects

Moderators: **Christina Kyriakopoulou**, *European Commission* and **Mark Turner**, *Liverpool University Hospital*

- VISION-DMD
- ALPHA-MAN
- Lena Project
- HIP PUMA



AGENDA

Day 1 - 22 October 2024

14:45

Breakout sessions:

1. Inclusion of Pregnant and Breastfeeding Individuals in Clinical Trials: impact and benefits for both mother and child

Moderators: **Martine Dehlinger-Kremer**, *ICON & EUCROF* and **Solange Corriol-Rohou**, *AstraZeneca*

Panelists: **Corinne de Vries**, *EMA* | **Lynne Yao**, *FDA* | **Marie Teil**, *UCB* | **Tyrza Boersma**, *Patient representative*

2. Use of RWD/Registries in Paediatric Drug Development

Moderators: **Angeliki Siapkara**, *AstraZeneca* and **Jeffrey Barrett**, *Aridhia*

Panelists: **Katherine Donegan**, *MHRA* | **Marina Kolochavina**, *Five Voices Consortium* | **Roberto De Lisa**, *EMA*

3. Paediatric-only drug development First in child companies

Moderators: **Gesine Bejeuhr**, *Bayer* and **Rhian Thomas-Turner**, *Noah's Ark Children's Hospital for Wales*

Panelists: **Cesare Sapon**, *Oncohereos* | **Kristina An Haack**, *Sanofi* | **Marc Ramis**, *Venture capital* | **Sam Blackman**, *Day One Biopharmaceutical*

16:30

COFFEE BREAK

17:00

Session 4: The Potential of Artificial Intelligence in Paediatric Drug Development

Moderator: **Claudio Fracasso**, *Hippocrates Research Srl* and **Thomas Severin**, *Novartis*

Panelists: **Danielle Belgrave**, *GSK* | **Kristina An Haack**, *Sanofi* | **Roberto De Lisa**, *EMA*

18:00

Reflection from the PDCO Chair

Brian Aylward, *Health Products Regulatory Authority*

18:30

End of Day 1

Martine Dehlinger-Kremer, *ICON & EUCROF*



AGENDA

Day 2 - 23 October 2024

08:00

WELCOME COFFEE

08:30

Welcome and Debrief from Day 1

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

Martine Dehlinger-Kremer, *ICON & EUCROF*

08:45

Session 5: Regulatory updates

Moderators: **Fahimeda Ali**, *MHRA* and **Sabine Fuerst- Recktenwald**, *Roche*

Panelists: **Michiyo Sakiyama** and **Natsuko Hayama**, *Pharmaceuticals and Medical Devices Agency* | **Robyn Langham** and **Yashwant Sinha**, *Therapeutic Goods Administration*

09:45

Session 6: Pathways to Medicines in Developing Countries

Moderators: **Martina Penazzato**, *WHO* and **Rhian Thomas-Turner**, *Noah's Ark Children's Hospital for Wales*

Panelists: **Emmanuella Amoako**, *Yemaachi Biotech* | **Lenias Hwenda**, *Medicines for Africa Foundation* | **Moy Bracken**, *Access to Medicines Foundation*

- Access to Childhood Cancer Medicines (WHO)
- Access to Medicines Foundation. They have a published methodology on assessing medicines specifically in developing countries

10:30

COFFEE BREAK

11:00

Session 7: Multistakeholder Collaboration in 2024

Moderator: **Solange Corriol-Rohou**, *AstraZeneca*

Panelists: **Christina Kyriakopoulou**, *European Commission* | **Dimitrios Athanasiou**, *EUPATI* | **Mark Turner**, *Liverpool University Hospital*

12:00

LUNCH

13:00

Session 8: European Legislations impact on paediatric medicines

Moderators: **Angeliki Siapkara**, *AstraZeneca* and **Dimitrios Athanasiou**, *EUPATI*

Panelist: **Anja Schiel**, *Norwegian Medicines Agency* | **Brian Aylward**, *Health Products Regulatory Authority* | **Mark Turner**, *Liverpool University Hospital* | **Victoria Kitcatt**, *Pfizer*

14:00

Feedback from the Breakout Sessions:

1. Inclusion of Pregnant and Breastfeeding Individuals in Clinical Trials
2. Use of RWD/Registries in Paediatric Drug Development
3. Paediatric-only drug development First in child companies

15:00

End of Day 2 – Conclusion & Farewell

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

Martine Dehlinger-Kremer, *ICON & EUCROF*