VIRTUAL CONFERENCE

Joint EFGCP/DIA Conference "Better Medicines for Children"

Global Paediatric Drug Development Challenges & Solutions

The Path Forward

EFGCP
where science and ethics meet

DIA

18-19 October 2021

Agenda at a Glance

MONDAY 18 OCTOBER 2021

11:45	Opening of Conference
12:00	Welcome and Introduction to the Conference
12:10 12:45 13:00 13:15 14:15	• Revision of the Paediatric Regulation • UK experience in paediatric development • Impact of the RACE for Children Act on paediatric development • Panel session with regulators: Paediatrics in Japan, China and Brazil • Panel session: Impact of regulatory changes for the patients
15:00	Break
15:15	Breakout sessions: • Public Private Partnerships • Paediatric formulations • Remote Decentralised Clinical Trials in Paediatrics
17:00	Break
17:10	Fireside Chat with PDCO Chair & UK Paediatric Regulator
18:10	End of Day 1

TUESDAY 19 OCTOBER 2021

11:45	Opening of Conference
12:00	Welcome and Debrief from Day 1
12:10	Panel Session: Optimising Paediatric Drug Development
13:20	Panel Session - Vaccines (incl.COVID-19) in paediatrics
14:20	Panel Session: Funding Paediatric Research in Europe
15:00	Panel Session: Pregnant women in clinical research (including in-utero treatment)
15:40	Feedback from the Breakout Sessions
16:00	Break
16:15	Panel Session: Paediatric Unmet Needs - Interaction with HTA bodies & payers
17:15	Key Note: Developing a vaccine for COVID-19 in the midst of the public health emergency
17:45	Key Note: Paediatric Development for the Future; some personal reflections
18:15	End of Day 2 - Conclusion & Farewell

Introduction

The Paediatric "Better Medicines for Children" Conference is an annual flagship event providing a unique opportunity to share best practice updates with a truly global outreach. Despite a challenging environment, and the need to meet virtually, we believe have been able to organise a dynamic, innovative, and highly engaging conference to inform our delegates of the latest scientific and regulatory developments.

This year the conference will focus on Global Paediatric Development, its challenges and solutions. To this effect, the conference brings together not only distinguished speakers from all around the world but also all the stakeholders involved, namely regulators, HTA body and payer representatives, industry, academia and patient representatives.

While focusing on paediatric research, i.e. what is happening, and how the future could look, Day One will bring regulators and patient representatives from around the world to discuss the impact of existing European and US paediatric regulations on paediatric R&D. The Programme Committee is proud to welcome Fabio d'Atri who will update us on the work done by the European Commission to revisit the Paediatric Regulation. He will be joined by regulators from the UK MHRA and the US FDA, while a panel moderated by a WHO representative will include regulators from Japanese PMDA, Chinese NMPA and Brazilian ANVISA, all involved in the review of paediatric dossiers and matters.

Active engagement of conference participants will be 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:

1. *Public Private Partnerships*
2. *Paediatric Formulations*
3. *Remote Decentralised Clinical Trials.*

At the end of Day 1, a fireside chat moderated by Elin Haf Davies (Aparito) will be the opportunity to hear from Dr Koen Norga (PDCO chair) and Dr Angeliki Siapkara (MHRA and former UK representative on PDCO) about their respective activities and priorities; and also to discuss the changing European landscape post-Brexit.

Day 2 will focus on hot topics in paediatric R&D showing the value of multi-stakeholders' collaboration to address gaps in scientific knowledge, explore new innovative development avenues, and foster paediatric drug development.

The programme committee looks forward to welcoming you all to the conference for a global educational and networking event that will present a unique opportunity to learn from each other, to meet and interact with colleagues from all parts of the world.

Thank you for your attendance.

Acknowledgement

EFGCP thanks its Partners & Members for their continued support

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Programme Committee

Dimitrios Athanasiou	PDCO Patient Representative & EURORDIS, Greece
Solange Corriol-Rohou	AstraZeneca, France
Fabio d'Atri	European Commission, Belgium
Elin Haf Davies	Aparito, United Kingdom
Martine Dehlinger-Kremer	EFGCP, EUCROF & ICON, Germany
Sabine Fuerst-Recktenwald	Roche, Switzerland
Angelika Joos	MSD, Belgium
Begonya Nafria Escalera	SJD Children's Hospital Barcelona, Spain
Koenraad Norga	Antwerp University Hospital, Belgium & Chair of EMA PDCO, The Netherlands
Helen Shaw	Proveca, United Kingdom
Angeliki Siapkara	MHRA, United Kingdom
Rhian Thomas-Turner	NHS, United Kingdom

Faculty

Dina Apele-Freimane	PDCO Member & Pauls Stradins Clinical University Hospital, Latvia
Dimitrios Athanasiou	PDCO Patient Representative & EURORDIS, Greece
Jeff Barrett	Critical Path Institute, USA
Simon Broady	AstraZeneca, United Kingdom
Christina Bucci-Rechtweg	Novartis, United Kingdom
Didier Caizergues	Genethon, France
Amy Cheung	Certara, United Kingdom
Solange Corriol-Rohou	AstraZeneca, France
Alexandru Costescu	European Commission, Belgium
Fabio d'Atri	European Commission, Belgium
Harris Dalrymple	ICON, United Kingdom
Elin Haf Davies	Aparito, United Kingdom
Martine Dehlinger-Kremer	EFGCP, EUCROF & ICON, Germany
Franck Devaux	HUDERF, Belgium
Michael Ermisch	GKV-Spitzenverband / National Association of Statutory Health Insurance Funds, Berlin, Germany
Sabine Fuerst-Recktenwald	Roche, Switzerland
Ying Geng	NMPA, China
Nancy Goodman	CEO of Kids v Cancer, USA
Frederik Grell Nørgaard	DKMA, Denmark

Tony Hall	Healx, United Kingdom
Niklas Hedberg	EunetHTA, TLV, Sweden
Ralf Herold	EMA, The Netherlands
Heidrun Hildebrand	Bayer, Germany
Xing Huanping	Meier SMA Care Center, China
Karl-Heinz Huemer	EMA PDCO, Austria
Angelika Joos	MSD, Belgium
Shinichi Kijima	PMDA, Japan
Priscila Lemos Costa	ANVISA, Brazil
Pirkko Lepola	FINPEDMED, Finland
Linda Lewis	Clinton Health Access Initiative, USA
Stephen P. Lockhart	Vaccine Clinical R&D Europe and Asia-Pacific Head at Pfizer, United Kindgom
Begonya Nafria Escalera	SJD Children's Hospital Barcelona, Spain
Janet Nooney	MHRA, UNITED KINDGOM
Koenraad Norga	Antwerp University Hospital, Belgium & Chair of EMA PDCO, The Netherlands
Cecile Ollivier	Critical-Path Institute, The Netherlands
Gregory Reaman	FDA, USA
Katie Rizvi	Youth Cancer Europe, Romania
Isaac Rodriguez-Chavez	ICON, USA
Agnes Saint Raymond	Former EMA, France
Michiyo Sakiyama	PMDA, Japan
Claudia Scharl	Austrian Federation of Social Insurance; MEDEV, Austria
Anja Schiel	SAWP Chair, NoMA, Norway
Nathalie Seigneuret	IMI, Belgium
Helen Shaw	Proveca, United Kingdom
Angeliki Siapkara	MHRA, United Kingdom
Rhian Thomas-Turner	NHS, United Kingdom
Catherine Tuleu	European Paediatric Formulation Initiative, United Kingdom
Mark Turner	University of Liverpool, United Kingdom
Marie Valentin	WHO, Geneva, Switzerland
John van den Anker	Children's National Hospital, Washington (D.C.), USA
Christa Van Kan	Healx, United Kingdom
Marieke van Meel	NephcEurope Foundation, The Netherlands
Sheila Varadan	Leiden University, Netherlands based in Thailand
Siri Wang	Norwegian Medicines Agency, PDCO Member, Norway
Katharine Wright	The Nuffield Council on Bioethics, United Kingdom
Lynne Yao	FDA, USA
Mira Zuidgeest	Utrecht University, The Netherlands

Monday 18 October 2021

11:45 Opening of Conference

12:00 **Welcome and Introduction to the Conference**
Martine Dehlinger-Kremer, *EFGCP, EUCROF & ICON, Germany*

12:10 **Revision of the Paediatric Regulation**
Fabio d'Atri, *European Commission, Belgium*

12:45 **UK experience in paediatric development**
Angeliki Siapkara, *MHRA, United Kingdom*

13:00 **Impact of the RACE for Children Act on paediatric development**
Gregory Reaman, *FDA, USA*

13:15 **Panel session with regulators: Paediatrics in Japan, China and Brazil**
Moderator: **Marie Valentin,** *WHO, Switzerland*

- **Shinichi Kijima,** *PMDA, Japan*
- **Michiyo Sakiyama,** *PMDA, Japan*
- **Priscila Lemos Costa,** *ANVISA, Brazil*
- **Ying Geng,** *NMPA, China*

14:15 **Panel session: Impact of regulatory changes for the patients**
Moderators: **Dimitrios Athanasiou,** *PDCO Patient Representative & EURORDIS, Greece*
& Begonya Nafria-Escalera, *SJD Children's Hospital Barcelona, Spain*

- **Nancy Goodman,** *CEO of Kids v Cancer, US*
- **Marieke van Meel,** *NephcEurope Foundation, The Netherlands*
- **Xing Huanping,** *Meier SMA Care Center, China*

15:00 Break

15:15 **Breakout sessions:**

Public Private Partnerships

Moderators: **Cecile Ollivier,** *Critical-Path Institute, the Netherlands,*
Heidrun Hildebrand, *Bayer, Germany*
& Mark Turner, *University of Liverpool, United Kingdom*

with **Nathalie Seigneuret,** *IMI, Belgium*
Marie Valentin, *WHO, Geneva, Switzerland*
and **Jeff Barrett,** *Critical Path Institute, USA*

Paediatric formulations

Moderators: **Catherine Tuleu,** *European Paediatric Formulation Initiative, United Kingdom*
& Simon Broady, *AstraZeneca, United Kingdom*

with **Linda Lewis,** *Clinton Health Access Initiative, USA*
and **Siri Wang,** *Norwegian Medicines Agency, PDCO Member, Norway*

Remote Decentralised Clinical Trials in Paediatrics

Moderators: **Mira Zuidgeest,** *Utrecht University, The Netherlands*
& Solange Corriol-Rohou, *AstraZeneca, France*

with **Frederik Grell Nørgaard,** *DKMA, Denmark,*
Tony Hall, *Healx, United Kingdom;*
Christa Van Kan, *Healx, United Kingdom,*
Elin Haf Davies, *Aparito, United Kingdom*
and **Isaac Rodriguez-Chavez,** *ICON, USA*

17:00 Break

17:10 **Fireside Chat with PDCO Chair & UK Paediatric Regulator**

Moderator: **Elin Haf Davies,** *Aparito, United Kingdom*

- **Koenraad Norga,** *Antwerp University Hospital, Belgium & Chair of EMA PDCO, The Netherlands*
- **Angeliki Siapkara,** *MHRA, United Kingdom*

18:10 **End of Day 1**

Martine Dehlinger-Kremer, *EFGCP, EUCROF & ICON, Germany*

Tuesday 19 October 2021

11:45 Opening of Conference with a video with the young people from eYPAGnet (European Young Person's Advisory Groups Network) and ICAN (International Children Advisory Network)

12:00 **Welcome and Debrief from Day 1**

Martine Dehlinger-Kremer, *EFGCP, EUCROF & ICON, Germany*

12:10 **Panel Session: Optimising Paediatric Drug Development**

Moderators: **Solange Corriol-Rohou,** *AstraZeneca, France*
& Sabine Fuerst-Recktenwald, *Roche, Switzerland*

- **ICH E11A Paediatric Extrapolation Guideline - Lynne Yao**, *FDA, USA*
- **Use of quantitative approaches - Amy Cheung,** *Certara, United Kingdom*
- **Complex Innovative Trial Designs - Anja Schiel,** *SAWP Chair, NoMA, Norway*
- **Use of RWE - Ralf Herold,** *EMA, The Netherlands*
- **Adolescents in Adult Clinical Trials - Christina Bucci-Rechtweg,** *Novartis, USA*

13:20 **Panel Session: Vaccines (incl.COVID-19) in paediatrics**

Moderators: **Rhian Thomas-Turner,** *NHS, United Kingdom* **& Angelika Joos,** *MSD, Belgium*

- **Katharine Wright,** *The Nuffield Council on Bioethics, United Kingdom*
- **Sheila Varadan,** *Leiden University, Netherlands based in Thailand*
- **Franck Devaux,** *HUDERF, Belgium*
- **Dina Apele-Freimane,** *PDCO Member & Pauls Stradins Clinical University Hospital, Latvia*

14:20 **Panel Session: Funding Paediatric Research in Europe**

Moderators: **Pirkko Lepola,** *FINPEDMED, Finland* **& Katie Rizvi,** *Youth Cancer Europe, Hungary*

- **Nathalie Seigneuret,** *IMI, Belgium*
- **Alexandru Costescu,** *European Commission, Belgium*
- **Didier Caizergues,** *Genethon, France*

15:00 **Panel Session: Pregnant women in clinical research (including in-utero treatment)**

Moderators: **Angeliki Siapkara,** *MHRA, United Kingdom* **& Solange Corriol-Rohou,** *AstraZeneca, France*

- **John van den Anker,** *Children's National Hospital, Washington (D.C.), USA*
- **Lynne P. Yao,** *FDA, USA*
- **Janet Nooney,** *MHRA, United Kindgom*
- **Harris Dalrymple,** *ICON, United Kingdom*

15:40 **Feedback from the Breakout Sessions:**

- **Public Private Partnerships**
- **Paediatric formulations**
- **Remote Decentralised Clinical Trials in Paediatrics**

16:00 Break

16:15 **Panel Session: Paediatric Unmet Needs - Interaction with HTA bodies & payers**

Moderators: **Fabio d'Atri,** *European Commission* **& Helen Shaw,** *Proveca, United Kingdom*

- **Anja Schiel,** *SAWP Chair, NoMA, Norway*
- **Niklas Hedberg,** *EunetHTA, TLV, Sweden*
- **Michael Ermisch,** *GKV-Spitzenverband / National Association of Statutory Health Insurance Funds, Berlin, Germany*
- **Claudia Scharl,** *Austrian Federation of Social Insurance; MEDEV, Austria*
- **Karl-Heinz Huemer,** *EMA PDCO, Austria*

17:15 **Key Note: Developing a vaccine for COVID-19 in the midst of the public health emergency**

- **Stephen P. Lockhart,** *Vaccine Clinical R&D Europe and Asia-Pacific Head at Pfizer, United Kindgom*

17:45 **Key Note: Paediatric Development for the Future; some personal reflections**

- **Agnès Saint Raymond,** *Former EMA, France*

18:15 **End of Day 2**
Conclusion & Farewell

Martine Dehlinger-Kremer, *EFGCP, EUCROF & ICON, Germany*

Biographies

Dina Apele-Freimane, MD

She is a head of the Neonatal Intensive Care Unit, Women and Child Health Clinic, Pauls Stradins Clinical University Hospital (Riga, Latvia) and lecturer in Riga Stradins University. She has more than 25 years' experience in neonatology and paediatric intensive care working as a clinical practitioner, scientists and regulator supervising paediatric clinical trials. She has been a national member of EMA Paediatric Committee (PDCO) since its establishment in 2007, and Chair of PDCO Neonatal Working Group. She graduated as a Medical Doctor from Medical Academy of Latvia in 1994, and further specialised as a Paediatrician and Neonatologist. Since 1998, she holds a postgraduate degree in Anaesthesiology and Intensive Care from Riga Stradins University. She used to work as a neonatologist in the Neonatal Intensive Care Unit of Riga Maternal Hospital and since 2003 she is a Head of NICU in Pauls Stradins Clinical University Hospital. Having a passionate personality, she has always been actively involved in various scientific projects and has focused more on RSV immunisation projects over the last decade. She is a member of National COVID-19 Vaccines Working Group acting under the umbrella of State Agency of Medicines of Latvia, having a particular interest in COVID-19 vaccination aspects for younger paediatric patients, including risk/benefit assessment.

Jeff Barrett

Dr. Jeff Barrett is Senior Vice-President at the Critical Path Institute serving as the Executive Director of the Rare Disease Cures Accelerator, Data Analytics Platform and a critical liaison between C-Path and the pharmaceutical industry, foundations, and other key stakeholders, helping grow C-Path's portfolio in drug development solutions, with a focus, but not limited to model-informed drug development (MIDD) and real-world data (RWD) technologies. Jeff was previously Head of Quantitative Sciences at the Bill & Melinda Gates Medical Research Institute. In this role he was responsible for implementing model-based drug development, employing PK/PD modeling, statistics, and clinical trial simulations to advance the discovery and development of new medicines and vaccines. Prior to MRI, he was Vice President, of Translational Informatics at Sanofi Pharmaceuticals. He led various aspects of model-based decision-making spanning and provided leadership for Sanofi's cloud-based, high-performance computing and "big data" initiatives. Jeff spent 10+ years at the University of Pennsylvania where he was Professor, Pediatrics and Director, Laboratory for Applied PK/PD at the Children's Hospital of Philadelphia. Jeff received his B.S. in Chemical Engineering from Drexel University and Ph.D. in Pharmacokinetics from University of Michigan. He has co-authored over 175 manuscripts, is fellow of ACCP and AAPS and the recipient of numerous honors including ACCP awards for Young Investigator (2002) and Mentorship in Clinical Pharmacology (2007) and the AAPS Award in Clinical Pharmacology and Translational Research (2011). Dr. Barrett was awarded for Exceptional Innovation and Advancing the Discipline of Pharmacometrics at the International Society for Pharmacometrics (2013) and elected ISOP Fellow (2017). He is co-Specialty Chief Editor of Frontiers in Obstetric and Pediatric Pharmacology Journal and an active member of the Child Health and Human Development Pediatrics Subcommittee as a study section reviewer. He was a past acting chair of the FDA Advisory Committee for Pharmaceutical Science and Clinical Pharmacology; a voting member of the committee for 8 years.

Biographies

Christina Bucci-Rechtweg, MD
Dr. Bucci-Rechtweg graduated with a MD from the University of Rochester School of Medicine & Dentistry and was Residency trained in Pediatrics and Fellowship trained in Pediatric Critical Care Medicine at the State University of New York @ Buffalo. She has over 20 years of pharmaceutical industry experience with roles in Clinical Development & Medical Affairs as well as Regulatory and Development Policy where she was responsible for the oversight and registration of global clinical development programs. In her career she has developed and implemented clinical programs as a Global Medical Director for pediatric and women's health in phase II and III, including those with pediatric regulatory obligations in the EU and US, and is widely regarded for her negotiating skills in adult and pediatric drug development, as well as health policy and international regulatory consensus building. Christina is actively involved in numerous external organizations advancing the regulatory and development environment for pediatric and maternal health globally, including the U.S. National Advisory Council for Child Health and Human Development, ICH Pediatric Standing Advisory Cmte, ICH E11A Pediatric Extrapolation Expert Working Group, Critical Path Institute's International Neonatal Consortium, EFGCP Children's Medicines Working Party, the IQ Consortia's Pediatric Clinical Pharmacology Leadership Group, HHS Task Force on Research in Pregnant Women & Lactating Women, as well as numerous professional societies and the trades. Christina is currently the Global Head for Pediatric and Maternal Health Policy at Novartis Pharmaceuticals Corporation.

Amy Cheung, PhD
S. Y. Amy Cheung is a Senior Director in Integrated Drug Development with responsibility of Quantitative Sciences lead for the UK. She is a core member of the Pediatric Integrated Practice Area at Certara. She has over a decade of Pharma experience (AstraZeneca). She obtained her PhD from the University of Manchester. After receiving her PhD, she worked at the Centre for Applied Pharmacokinetic Research (CAPKR) at The University of Manchester. Followed by a postdoc on mechanistic modelling. She has expertise in pediatric drug development, oncology, infection, CNS, vaccine, mAb, MIDD, PBPK, and extrapolation. She is a member of the IQ CPLG pediatric working group and chair of the IQ TALG CPLG Pediatric PBPK group.

Fabio d'Atri
Fabio D'Atri is policy officer in the unit «Medicines: policy, authorisation and monitoring» in the Health and Food Safety Directorate General of the European Commission (DG SANTE).
Fabio holds a PhD in biochemistry from the University of Geneva and a master in management of biotech companies from the Grenoble School of Business.
After working several years as university researcher and then both in the public and private sector, he joined the European Commission in 2004.
In Directorate General Health and Food Safety he has worked on several areas of the food legislation. In 2011 he has joined the units dealing with pharmaceutical products. He has worked on the quality of medicines, falsified medicines and clinical trials. During 2016 and 2017 Fabio was seconded to the European Centre for Disease Prevention and Control (ECDC) in Stockholm and worked on vaccination and antimicrobial resistance related issues. He is now in charge of the paediatric medicines file.

Biographies

Harris Dalrymple

Dr. Harris W. Dalrymple has nearly 40 years' experience in the pharmaceutical and CRO industries, and has been involved in pediatric clinical trials for over 20 years. Originally a pharmacologist, he holds a Masters' in Medical Law and Ethics and has been awarded PhDs in both Medicine and Law, the latter for his thesis on legal and ethical issues raised by the conduct of clinical trials involving pregnant women. Dr Dalrymple worked for Pfizer for almost 25 years, mostly in Clinical Development, following which he joined PPD where he served as the vice-chair of the Pediatric Working group which he helped to establish there. Since March 2017, he has held the position of Executive Director, Scientific Affairs and Vice-Chair, Centre for Pediatric Clinical Development. His particular interests include assent/consent/dissent, clinical trials in pregnancy, data privacy and ethical issues in clinical trials generally. He lectures on medical law and ethics in the clinical trial setting for the British Association of Pharmaceutical Physicians Faculty of Pharmaceutical Medicine and is a member of a Research Ethics Committee.

Elin Haf Davies

Dr Elin Haf Davies began her career as a Paediatric Nurse at Great Ormond Street Hospital, moving into research and spending six years managing clinical and academic drug trials, in metabolic, neurology, endocrinology and pain.

Her PhD at University College London focused on the development of age-appropriate and disease specific biomarkers and clinical endpoints in neurometabolics (lysosomal storage disorders).

In 2007 Elin Haf moved to work at the European Medicine Agency Paediatric Team where, amongst other projects she evaluated over a 100 Paediatric Investigation Plans, working closely with Scientific Advice and the Orphan team.

She founded Aparito in 2015, a digital health company focused on developing patient centric digital outcomes to support decentralised trials. She also serves as the Chair of Board of Trustees for Metabolic Support UK, a patient advocacy group supporting 400 rare inherited metabolic diseases.

Martine Dehlinger-Kremer, PhD, MS

Vice President of Scientific Affairs, Pediatric Subject Matter Expert, ICON

Dr. Dehlinger-Kremer's expertise spans more than 30 years in the research industry, including 29 years of experience in global regulatory affairs, medical affairs, and pediatric leadership. Prior to joining ICON, she served in several executive leadership roles at global CROs, and has experience in global drug development in more than 40 countries. She has contributed hands-on to the global development of numerous products, including medicines for children, drugs for orphan diseases and biosimilars. Her vision and leadership extend to service with a number of professional organizations – she is an observer member of the Coordinating Group of the European Network of Pediatric Research (Enpr-EMA) at the European Medicines Agency, chair of the Pediatric Working Group and also President of the European CRO Federation (EUCROF), serves as chair of the European Forum for Good Clinical Practice (EFGCP) Children Medicines Working Party and Board Member of the association, and is active in the International Children's Advisory Network (iCAN).

In 2015, Dr. Dehlinger-Kremer was named one of PharmaVOICE's 100 Most Inspiring People in Life Sciences.

Dr. Dehlinger-Kremer earned a Doctorate in Sciences from the J. W. Goethe University in Francfort on the Main, Germany; a Degree of Advanced Study in Neurophysiology from the University Louis-Pasteur, Strasbourg, France; and a Master of Sciences degree from University Moulin de la Housse in Reims, France.

Biographies

Michael Ermisch
Dr Michael Ermisch is a pharmacist by training who specialised in drug information. His doctoral thesis dealt with the regulation of nuclear receptors. Since 2013, he has been a specialist consultant in the Department of Drugs and Remedies of GKV-Spitzenverband with various thematic responsibilities in the assessment of medicines and European affairs.

Sabine Fuerst-Recktenwald
Dr. Sabine Fürst-Recktenwald is a Principal Medical Director in the Personalized Health Care (PHC) Safety Interface team within Roche, a team that works to better understand patient-centric drivers of response to optimize the benefit/risk balance of medicines and drive precision medicine within Roche.
Sabine is a board certified pediatrician with speciality in pediatric diabetology from the University of Erlangen/Germany. She has over 19 years of experience in drug development in the pharmaceutical industry, including strategic roles in both early and late clinical research and medical affairs.
Sabine joined industry in 2001 with Sanofi-Aventis and served on different positions as Medical Advisor for Diabetology & Metabolism, as European Scientific Director for Diabetes and finally as Clinical Research Director in the department of Clinical and Exploratory Pharmacology. There, she also set up and chaired the international clinical development pediatric network for several years, advising project teams on their pediatric plans.
Sabine joined Roche in May 2006 as Translational Medicine Leader in the early development Cardio-Vascular & Metabolism group and has been responsible as the early clinical leader for various programs in Diabetes/Metabolism and Opthalmology.
Moving to the innovative Pediatric Oncology Drug Development team (iPODD) in 2014, she was the Clinical Science Leader for the pediatric development of Avastin, Cobimetinib and Hemlibra. In 2017 she joined PD Neuroscience, where she acted as Global Clinical Development Team Leader for Olesoxime and most recently Clinical Science Leader for Risdiplam in Spinal Muscular Atrophy.
Sabine has a broad experience in the management of international pediatric trials, served as EFPIA representative on the ICH E11 guideline update from 2013-2017 and serves as EFPIA representative on the ICH E11A pediatric extrapolation guideline, which is currently prepared.

Frederik Grell Nørgaard is Project Manager for decentralised trials at the Clinical Trial Unit, Danish Medicines Agency (DKMA). Frederik is working as a regulatory assessor and coordinator of clinical trials applications and runs several Danish initiatives such as the dialogue forum for decentralised trials (DCT). He is also the co-author and coordinator of the Danish DCT guidance. Frederik is a member of several workgroups within the Clinical Trials Facilitation and Coordination Group (CTFG) being involved in development of DCT guidance and a Q&A regarding the IVDR-CTR interface. Frederik has a background as Master in Pharmacy.

Niklas Hedberg
He is the Chief Pharmacist at the Swedish national governmental authority, the Dental and Pharmaceuticals Benefits Agency (TLV).
From 2018 to 2021 Niklas was the Chair of the EUnetHTA Executive Board.
Niklas has been working with pricing and reimbursement since 2001. Niklas started as a medical assessor and project leader in the agency, he was the Head of the Department for New Submissions between 2009 and 2014 and is since in his current position.
Niklas has a broad experience of different aspects of value based evaluation and over time has seen the increasing importance for health technology assessment (HTA) both across health care systems and on local level to prepare accurate decision making.

Biographies

Heidrun Hildebrand, Pediatric Development Alliance Management; Research and Development Pharmaceuticals ; Bayer AG, Germany
20 plus years' experience in drug development.
Heidrun started her carrier in the Pharmaceutical industry as Quality Manager within Clinical Development and held different positions in Clinical Development before moving into Global Project Management, leading global multi-indication drug development programs in different Therapeutic Areas. Since February 2017 Heidrun is part of the Paediatric Development Group at Bayer solely focusing on drug development for Children.
Heidrun is representing Bayer in a number of external organizations and public private partnerships in the field of Paediatric development and currently co-leading conect4children (c4c) an IMI2 initiative to create a pan-European Paediatric Clinical Trials Network.

Xing Huanping, Executive director of Meier Advocacy & Support Center for SMA
Over 17 years' experience working with patients of leprosy, Alzheimer's disease, osteogenesis imperfecta and spinal muscular atrophy as a social worker.
Starting 2016, she has provided services for more than 3,000 SMA families in China.

Shinichi Kijima is Associate Senior Scientist for clinical pharmacology and pharmacokinetics at PMDA, Japan. He have over 10 years' experience reviewing the pharmacokinetics, clinical pharmacology and pharmacometrics of new drugs. He is representative from PMDA/MHLW as Topic Leader for ICH E11A «Pediatric Extrapolation».
From 2016 to 2017, he worked as ORISE Fellow for Division of Pharmacometrics, Office of Clinical Pharmacology/CDER/U.S.FDA. In PMDA, he is in charge of clinical pharmacology and pharmacometrics in the Modeling & Simulation project team. And, he contributed to develop Japanese pharmacometrics related guidelines including PBPK reporting guideline, Exposure-Response guideline and Population approach (PopPK, PopPK/PD) guideline.

Priscila Lemos Costa is pharmacist and Health Regulation Specialist at Brazilian Health Regulatory Agency (Anvisa) since 2014. She performs reviews of safety and efficacy data of synthetic medicines

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Pirkko Lepola, Executive Secretary of FINPEDMED, General Secretary of NORDICPEDMED at Helsinki University Hospital, Department of Children and Adolescents, Finland. Chair of the Enpr-EMA Coordinating Group.
Education: 1995: B.Sc. (Health Care), 2006: M.Sc. (Biotechnology), 2012: Teacher (Pedagogical qualification). Courses in BioMedical Law and Pharmaceutical Medicine. Professional Experience: 1995-2006: Various management positions (Clinical Trials) in Pharma Industry and CRO companies, and University Hospital´s Research Organizations. Since 2007 -present: Executive Secretary of FINPEDMED (Finnish Investigators Network for Pediatric Medicines), 2010-2012: Project Manager of FinnTrials–project (Finland), 2013-2017: Researcher in EU GRiP -project (Global Research in Paediatrics), 2016-present: General Secretary, NordicPedMed (Nordic Investigators Network for Pediatric Medicines), 2017-2021: Consortium Partner in EU PedCRIN (The Paediatric Clinical Research Infrastructure Network)–project; 2018-present: Consortium Partner in the EU/IMI2 c4c (Conect4Children-PanEuropean Pediatric Clinical Research Infrastructure), 2014-2019: Conference creator/co-creator & producer: National Pediatric Drug Therapy Conferences, Nordic Conferences on Pediatric Medicines with Pharma Industry Finland. Memberships & Expert work: 2010-present: EMA (European Medicines Agency) European Expert, 2010-present: Chair, Enpr-EMA (European Network of Paediatric Research at the European Medicines Agency), 2013-present: Chair, Enpr-EMA Working Group of Ethics. 2013-present: Member of the EFGCP MCWP (European Forum for Good Clinical Practice, Medicines for Children Working Party), 2019-present: Member of the Advisory Board SwissPedNet (The Swiss Research Network of Clinical Pediatric Hubs), Teaching: 2007-present: Lecturer at Universities, University Hospitals and Universities of Applied Sciences in Finland. Publications: Articles 10, Book chapters 2, Scientific presentations 50+.

Biographies

Stephen P. Lockhart, MA, BM BCh, DM, MRCP, FFPM is Vice President, Vaccine Clinical Research and Development at Pfizer Vaccine R&D.
Stephen qualified as a physician in 1980. Following work in hospital internal medicine and research he joined the pharmaceutical industry in 1986. He has over 35 years of experience in pharmaceutical and vaccine development and has worked on development of vaccines against a wide range of bacterial and viral pathogens for adult and paediatric populations. Stephen, based in the UK, is Pfizer global clinical program lead for the Pfizer/BioNTech mRNA COVID-19 vaccine. He has led the clinical program from phase 1, through the first initial authorisation for any COVID-19 vaccine by a robust regulatory authority and now into use into broader populations, including paediatrics.

Begonya Nafria Escalera
She is Patient Engagement in Research Coordinator at Sant Joan de Déu Chidren's Hospital (Spain). She has long experience in the field of the involvement of patients and families in research initiatives. She has also a personal story as a caregiver and patient advocate because is the sister of a young adult with cerebral palsy.
Her areas of expertise are focused in paediatric patients involvement in research and specifically in the field of clinical trials.
Other relevant background of her profile is: Fellow of EUPATI (first cohort), Coordinator of eYPAGnet (European Young Patients Advisory Group Network – www.eypagnet.eu),
Coordinator of Kids Barcelona (YPAG of Sant Joan de Déu Children's Hospital- www.kidsbarcelona.org), member of Children's Medicines Working Party of EFPGCP, volunteer member of the Editorial Board of Center for Information & Study on
Clinical Research Participation (CISCRP), member of the Patients and Families working group of EnprEMA (European Network of Paediatric Research of EMA).
Coordinator of the cross-cutting theme of patients involvement in Conect4Children project (pan-European paediatric clinical trials network).
The main aim of all the activities leaded or participated by Begonya is to achieve a better quality of life for the paediatric patients thanks to research and new therapies.

Cecile Ollivier
Cecile is a senior health engineer with 15 years of global drug development experience in children & rare diseases. She recently joined C-Path as scientific director and was previously Chief Innovation Officer for Aparito a medtech company for 2 years and scientific officer in the Paediatric division of Science & Innovation at EMA for 12 years where Cecile has been leading the EMA extrapolation strategy and was an expert for the ICH E11(R)1 guideline

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Gregory Reaman
Heis the Associate Director for Pediatric Oncology in the FDA's Oncology Center of Excellence, Office of the Commissioner and Associate Director for Pediatrics in the Office of Oncologic Diseases, Center for Drug Evaluation and Research. He is the Executive Director emeritus of the Center for Cancer and Blood Disorders and senior attending physician at Children's National, Washington, D.C.. He has held numerous academic and clinical research leadership positions. He was the Inaugural Chair of the Children's Oncology Group and previously served as the Associate Chair for New Agent Studies and Vice Chair for Scientific Affairs of the Children's Cancer Group. He served on the national Board of Directors of the American Cancer Society and chaired its Task Force on Cancer in Children, the Board of Directors of the American Society of Clinical Oncology and the Board of Directors of the International Society of Pediatric Oncology. He was the first pediatrician member of the FDA's Oncologic Drugs Advisory Committee and the first chair of its Pediatric Subcommittee. He is a Professor of Pediatrics at the George Washington University School of Medicine and Health Sciences. His research interests are in the immunobiology and therapy of acute leukemia and the development of new cancer therapeutics for children. He has authored more than 350 peer-reviewed publications.

Biographies

Agnes Saint Raymond
She is an MD and qualified French Paediatrician. She worked in a paediatric Hospital in Paris, France, then in the pharmaceutical industry for 5 years,, and moved to the French Medicines Agency in 1995. In 2000 she joined the European Medicines Agency (EMA). She was responsible for Orphan Medicines, Scientific Advice, the Small & Medium-sized Enterprises Office, and Paediatric Medicines and implemented the EU Regulation on paediatric medicines. In 2013, she became Head of the Portfolio Board and from September 2016, she was also the EMA Head of the International Affairs Division. She retired from EMA on 1 October 2021.

Michiyo Sakiyama
She is a pediatrician and her area of expertise is pediatric oncology.
She has been working in the Pharmaceuticals and Medical Devices Agency (PMDA) since 2010.
She is an associate senior scientist for clinical medicine of Office of Vaccines and Blood Products and Office of Anti-Cancer Drugs. She is involved in reviewing vaccines and pediatric oncology drugs.
She also works as the PMDA Pediatric Drugs Working Group leader.

Nathalie Seigneuret
She is a Senior Scientific Project Manager at IMI (Innovative Medicines Initiative) responsible for the coordination and management of projects within the scientific operations team.
Before joining IMI in 2011, she was a scientific administrator at the European Medicines Agency, involved in various activities over 15 years including the implementation of the EU Paediatric Regulation at the Agency level.
Nathalie is a state-certified Doctor in Pharmacy and holds a Post-Graduate degree in International Development and Drug Registration.

Angeliki Siapkara
Dr Siapkara obtained her medical degree from the University of Athens, and completed her postgraduate qualification in Orthopaedic Surgery with a special interest in Paediatrics at hospitals in Athens, Glasgow and at Great Ormond Street Hospital. In 2010 she undertook postgraduate research leading a MSc in International Child Health from University College London. Dr Siapkara joined the MHRA in 2008 leading the Paediatric Unit which delivers the implementation of regulatory activities for innovating paediatric medicines and improved paediatric pharmacovigilance. She is currently the Group manager for MHRA's Benefit Risk Group. She was the UK delegate at EMA's Paediatric Committee (PDCO) and member of the CMDh/EMA Working Party on Paediatric Regulation. Dr Siapkara is an observer at Joint Standing Committee on Medicines of the Royal College of Paediatrics and Child Health.

Catherine Tuleu, Docteur en Pharmacie, PhD
She is Professor in Paediatric Pharmaceutics at UCL School of Pharmacy, UK. Her research, inherently translational, ranges from formulation, methodology development to clinical implementation, integrating the following themes: children centric excipient research; repurposing by reformulating better medicines for children; development of innovative age appropriate dosage forms (especially for under 5s); administration issues and devices; and sensory pharmaceuticsTM (dosage form acceptability and in vitro/in vivo taste assessment). Her spin out company senCeuTics Ltd. specializes in pharmaceutical sensory evaluation and offers a full spectrum of preclinical, clinical and paediatric formulation services under one roof. She is the founder and chairperson of the European Paediatric Formulation Initiative (EuPFI), a consortium working in a pre-competitive way on paediatric drug formulations.
https://www.ucl.ac.uk/pharmacy/people/professor-catherine-tuleu

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Biographies

Marie Valentin, Technical Officer, Regulatory Convergence and Networks Team [RCN], Regulation and Safety Unit [REG], Regulation and Prequalification Department [RPQ], World Health Organization, Geneva, Switzerland
Pharmacist specialized in drug product regulation with over 20 years of experience in regulatory affairs and product development, acquired both in the private and public sectors.
At WHO, Marie works in the Regulatory Convergence and Network Team towards convergence, harmonization and system strengthening activities, supporting different regional regulatory networks and supporting Member States to strengthen their regulatory systems. She was actively involved in the preparation and finalization of the WHO documents on Good Regulatory Practices and Good Reliance Practices. She is also in charge of the Secretariat for the WHO paediatric regulatory network, a global network of regulators and other interested stakeholders supporting the availability of quality assured medical products for children. She is the regulatory lead of the Global Accelerator for Paediatric formulations (GAP-f), a WHO led-network which goal is to enhance coordination between partners for an optimal development of formulations for children in need.
Before joining the WHO in May 2019, Marie worked for 8.5 years at the European Medicines Agency in London as a Regulatory Affairs Officer where she was in charge of providing regulatory and procedural advice in relation to the development, evaluation and surveillance of medicinal products in the European Union. Before that, she worked in the pharmaceutical industry, contract research organization and consultancies in the United Kingdom, Spain and France.

John van den Anker, MD, PhD, FAAP, FCP
Dr. John van den Anker is a Professor of Pediatrics, Pharmacology, Physiology, Genomics and Precision Medicine at the George Washington University School of Medicine and Health Sciences, Washington, DC and holds the Evan and Cindy Jones Endowed Chair in Pediatric Clinical Pharmacology. He also is the Eckenstein-Geigy Distinguished Professor of Pediatric Pharmacology at the University Children's Hospital of Basel, University of Basel, Switzerland.
Dr. van den Anker has been the President of the American College of Clinical Pharmacology (2016-2018) and twice the President of the European Society of Developmental, Perinatal and Paediatric Pharmacology (2006-2008 and 2017-2019). His awards include the Distinguished Investigator Award from the American College of Clinical Pharmacology (2008), the Distinguished Researcher award of the George Washington University (2012), and the Sumner J. Yaffe Lifetime Achievement Award in Pediatric Pharmacology and Therapeutics (2019) from the Pediatric Pharmacy Association.
Over the past 30 years, Dr. van den Anker' research has focused on developmental, neonatal and pediatric pharmacology. He has authored over 500 peer reviewed publications and has received NIH funding as well as funding from the European Union to support his research and the development of training programs in Pediatric Clinical Pharmacology.

Lynne Yao, MD
She is the Director, Division of Pediatric and Maternal Health in the Office of New Drugs, Center for Drug Evaluation and Research. Dr. Yao received a B.S. degree in Biology from Yale University, and an M.D. degree from the George Washington University School of Medicine. She is board certified in both Pediatrics and Pediatric Nephrology. Prior to joining FDA, Dr. Yao was the Director of Dialysis and Associate Pediatric Residency Program Director at the Inova Fairfax Hospital for Children in Fairfax, VA. She has been with the FDA since 2008. The Division of Pediatric and Maternal Health oversees quality initiatives which promote and necessitate the study of drug and biological products in the pediatric population; and improve collection of data to support the safe use of drugs and biological products in pregnant and lactating individuals. She collaborates with numerous stakeholders both inside and outside of FDA to advance development of safe and effective therapies for children, and pregnant and lactating women.

Biographies

Mira Zuidgeest, PhD, PharmD, MSc epidemiology,
works as assistant professor at the Julius Center for Health Sciences and Primary Care. Trained both as a pharmacist and epidemiologist and with a PhD in pharmacoepidemiology in the field of paediatric asthma, her current work focuses on clinical trial innovation, both methodological and operational. As core team member and scientific taskforce lead in the IMI GetReal Initiative, her work focusses on randomised clinical trials integrated into routine clinical practice (also called pragmatic trials) and the methodological and operational consequences of introducing real world elements in trial design, for which she has developed a decision-support tool (www.getrealtrialtool.eu). And as overall scientific lead and WP3 lead for the IMI Trials@Home project (www.trialsathome.com), she looks into the possibilities of centring trials around patients rather than clinical sites by use of innovative technologies (so called Remote Decentralised Clinical Trials).

EFGCP/DIA Conference
"Better Medicines for Children"
Global Paediatric Drug Development Challenges & Solutions
The Path Forward

Conference Language
The language of the conference will be English.

More Information: events@efgcp.eu

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