DIA

Better Medicines for Children, a DIA/EFGCP Conference

Moving forward in times of change

16-17 September 2019 | NH Schiphol Airport, Amsterdam

PROGRAMME COMMITTEE

Heidrun Hildebrand
Global Program Head, TA Paediatric Development
Bayer, Germany

Mette Due Theilade Thomsen
Managing Director
PIP Adviser, Denmark

Geneviève Le Visage
Head EU Regulatory Policy
Novartis Pharma, Switzerland

Dimitrios Athanasiou,
Patient Advocate
Muscular Dystrophy Association Hellas, Greece

Martine Dehlinger-Kremer
Vice President, Pediatric Development, Synteract, Germany
President EUCROF, Netherlands

Siri Wang
Member PDCO
Scientific Director, Norwegian Medicines Agency, Norway

Key Topics

- Leap in scientific innovation – how to address it for children
- Future perspectives of the EU research landscape – IMI and beyond
- Orphan and Paediatrics - where do we stand?

Plan ahead and join us to reflect on the importance of two major headways that are currently affecting Paediatric research: Innovative approaches to Drug Development and impact of the current environment on the Regulatory and Research Infrastructure

Overview

This conference will focus on two major developments currently affecting Paediatric Development: Innovative approaches to Drug Development and the impact of the current environment on the Regulatory and Research Infrastructure. You will gain critical insights from Regulators and leading industry experts about the latest scientific innovations and have the opportunity to engage in lively discussions with Paediatric Committee (PDCO) members and other industry experts involved in paediatric drug development. The patient experience will be voiced throughout the conference.

Objectives

- Showcase latest developments with regards to innovation in how the paediatric studies are designed and conducted
- Update participants on current paediatric regulatory requirements, scientific and operational successes and challenges

Who Should Attend

- Regulatory, clinical and drug development professionals from Health Authorities and Industry interested in paediatric drug development
- Paediatricians, Representatives from Academia, Paediatric Societies and Networks, Patients Organisations
- Employees from Clinical Research Organisations (CROs) involved in paediatric clinical trials
- Stakeholders interested in the development of better medicines for children

DAY ONE | MONDAY, 16 SEPTEMBER 2019

08:00 REGISTRATION

08:45 WELCOME

09:00 SESSION 1

INNOVATIVE APPROACHES TO PAEDIATRIC DRUG DEVELOPMENT – STATE OF PLAY AND POSSIBLE IMPACT

Session Chair:
Martine Dehlinger-Kremer, Vice President, Pediatric Development, Synteract, Germany & President EUCROF, The Netherlands

EMA Regulatory Science Strategy 2025 - How will it Impact Paediatric Development?
Ralph Bax, Head of the Paediatric Medicines Office European Medicines Agency, EU

Panel Discussion and Q&A, with the participation of:
Dimitrios Athanasiou, Patient Advocate Muscular Dystrophy Association Hellas, Greece
Ralph Bax, Head of the Paediatric Medicines Office, European Medicines Agency, EU
Geneviève Le Visage, Head EU Regulatory Policy, Novartis, Switzerland
Siri Wang, Member PDCO Scientific Director, Norwegian Medicines Agency, Norway

10:00 COFFEE BREAK

10:30 SESSION 2

ADVANCED THERAPY MEDICINAL PRODUCTS (ATMPS) – DO THEY HOLD WHAT THEY PROMISE?

Session Chair:
Katie Rizvi, Founder, Asociatia Little People Romania & Temerarii - The Romanian Community of Cancer Survivor Adolescents and Young Adults, Romania

Advances in ATMP Development - Regulator Perspective
Anja Schiel, Senior Adviser/Statistician, Unit for HTA and Reimbursement Norwegian Medicines Agency (NoMA), Chair EMA Biostatistics Working Party, Chair EMA Scientific Advice Working Party, Norway

Paediatric ATMP Development– Industry Initial Learnings
Mette Due Theilade Thomsen, Managing Director, PIP Adviser, Denmark

Paediatric ATMP Development– Patients' Experience
Elizabeth Vroom, President, Duchenne Parent Project, The Netherlands

Panel discussion

12:00 LUNCH

13:30 SESSION 3

INNOVATION IN PEDIATRIC DEVELOPMENT: FORMULATION, TRIAL DESIGN AND REAL WORLD DATA

Session Chair:
Siri Wang, Member PDCO, Scientific Director, Norwegian Medicines Agency, Norway

Beyond Tablets and Liquids – Update on new Concepts for Paediatric Formulations
Joerg Breitkreutz, APV President Director, Institute of Pharmaceutics and Biopharmaceutics, Heinrich-Heine-University Duesseldorf, Germany

Innovative Trial Designs - How much can we use Data from Adults?
Anja Schiel, Senior Adviser/Statistician, Unit for HTA and Reimbursement Norwegian Medicines Agency (NoMA), Chair EMA Biostatistics Working Party, Chair EMA Scientific Advice Working Party, Norway

Registries, Apps, Wearables - How does Real World Data Change the way we do Studies?
Elin Haf Davies, Founder/CEO, Aparito, UK

15:00 COFFEE BREAK - POSTER SESSION WITH VOTING

Disclosure Policy

Unless otherwise disclosed, DIA acknowledges that the statements made by speakers are their own opinion and not necessarily that of the organisation they represent, or that of the DIA. Speakers and agenda are subject to change without notice. Recording during DIA sessions is strictly prohibited without prior written consent from DIA.

DAY ONE | MONDAY, 16 SEPTEMBER 2019

16:00 SESSION 4

PAEDIATRIC RESEARCH INITIATIVES – WHO ARE THEY AND WHAT DO THEY AIM FOR?

Session Chair:
Heidrun Hildebrand, Global Program Head, TA Paediatric Development Bayer, Germany

One of the critical factors for successful Paediatric Development is the availability of efficient Research Infrastructure across Europe and beyond. There are several initiatives ongoing globally to create this infrastructure. These include networks and research initiatives of all kind, receiving a mix of public and private funding. This session will provide some inside on these initiatives. Who they are, what are their goals, and how they are funded?

European Research Infrastructure Initiatives - who are they and what do they do?
Mark Turner, Co-Director International Neonatal Consortium, NIHR Clinical Research Network Children's Theme Liverpool Women's Hospital, UK

IMI Activities Related to Paediatric Research & Development (c4c, ITTC-P4)
Nathalie Seigneuret, Senior Scientific Project Manager, Innovative Medicines Initiative (IMI), Belgium

US Public-Private Partnerships Supporting Paediatric Research (C-Path; iACT, INC, Duke)
Samuel D. Maldonado, Vice President, Head of Child Health Innovation Leadership Department, USA

17:30 PDCO REVIEW AND SNEAK PREVIEW

Dirk Mentzer, PDCO Chair 2013-2019 & Head of Pharmacovigilance Unit, Paul-Ehrlich-Institut, Germany

17:45 Q&A - UPDATE FROM PDCO

Dirk Mentzer, PDCO Chair 2013-2019 & Head of Pharmacovigilance Unit, Paul-Ehrlich-Institut, Germany

18:30 END OF DAY ONE

DAY TWO | TUESDAY, 17 SEPTEMBER 2019

08:45 WELCOME COFFEE

09:00 SESSION 5

DIGITAL INNOVATION AND E-TOOLS – OPPORTUNITIES AND CHALLENGES

Session Chair:
Cécile Ollivier, Chief Operating Officer, Aparito, UK

Bring the Patient Experience with the Application
Alex Johnson, Co-founder and Joint CEO of Duchenne UK

Digital Tools and Drug Development: an Industry Perspective
Aude Clement, Global Regulatory Leader - Rare Diseases, F. Hoffmann-La Roche Ltd, Switzerland

EMA Qualification Procedure: Experience from a Medtech Company
Damien Eggenspieler, Healthcare Program Director, Sysnav

EMA Regulatory Perspective of Opportunities and Challenges
Ralph Bax, Head of the Paediatric Medicines Office, European Medicines Agency, EU

Panel discussion with Q&A

10:30 COFFEE BREAK

11:00 SESSION 6

NONCLINICAL STUDIES TO SUPPORT PAEDIATRIC DRUG DEVELOPMENT

Session Chair:
Mette Due Theilade Thomsen, Managing Director PIP Adviser, Denmark

ICH S11 Nonclinical Safety Testing in Support of Development of Paediatric Medicines
David R Jones, Expert Pharmaco-Toxicologist, Clinical Trials Unit, MHRA, UK

Update on PDCO Juvenile Animal Study Project for Oncology Products
Karen Van Malderen, Chair PDCO Non-Clinical Working Group; Member PDCO (alt); Non-Clinical Assessor, AFPMS/FAGG, Belgium

Preclinical Disease Models for Nonclinical Pharmacology Data on Drugs
Ralph Bax, Head of the Paediatric Medicines Office, European Medicines Agency, EU

Industry Perspective
Andreas Hartmann, Executive Director, Novartis, Switzerland

DAY TWO | TUESDAY, 17 SEPTEMBER 2019

12:30 SESSION 7

ORPHAN MEDICINES – DOING STUDIES IN SMALL POPULATIONS

Session Chair:
Geneviève Le Visage, Head EU Regulatory Policy Novartis Pharma, Switzerland

Feedback on the Joint Review of the Paediatric and Orphan Regulations
Fabio D'Atri, Deputy Head of Unit, Unit D6, Directorate General for Health and Consumers, EU

Orphan Medicines for Paediatric Use - A focus on the European Union
Thomas Kühler, Head Global Regulatory Science & Policy EU/AMEE, Sanofi R&D, France

Panel discussion

13:30 END OF THE CONFERENCE

EVALUATION

We value your feedback on the content and organisation of this conference. Please complete the electronic survey after the conference https://bit.ly/2lRejLP.

ACCESS PRESENTATIONS

As a benefit of your registration, presentations are made available on the DIA website.

To access presentations, go to www.diaglobal.org and click on Sign in at the very top. Once you have successfully logged in, click on the event page, then Resources on the left.

No paper copies of the presentations will be provided.

NOTE: If a presentation is not available, the speaker either did not agree to publish it or did not provide us with the presentation. Updated versions of the slides will be made available shortly after the conference.

CERTIFICATE OF ATTENDANCE

A Certificate of Attendance will be sent to all attendees electronically after the conference. Please note certification requires full attendance. For more information please liaise with our DIA Contact Centre on Basel@DIAglobal.org or call +41 61 225 51 51.

Conference Venue

NH SCHIPHOL AIRPORT
Kruisweg 495
2132 NA Hoofddorp
Tel: +31 20 655 0550
Email: **nhschipholairport@nh-hotels.com**

HOW TO GET THERE

From Amsterdam Schiphol Airport, the NH shuttle service is available 24/7. Free for guests. Contact the hotel directly for the schedule.

From the Amsterdam Central Station you can take the train to Schiphol Airport. If you're in the city for a few days buy a T-10, Multi-person card for 10 journeys, (€9.95). It's cheaper than buying singles. Click here for a Amsterdam Transport map.

ACCOMMODATION
NH SCHIPHOL AIRPORT
Kruisweg 495
2132 NA Hoofddorp

DIA has blocked a limited number of rooms for the participants. Please book through this link.

Standard Double room rate: € 196,90, including Breakfast & VAT, excluding 6.05% city tax.

What to see in Amsterdam?
Amsterdam has an array of sites and entertainment options spread all over the city. For more information check out our **Travel Guide** for Amsterdam, written by locals.

Continuous Education

DIA meetings and training courses are generally approved by the Commission for Professional Development (CPD) of the Swiss Association of Pharmaceutical Professionals (SwAPP) and the Swiss Society of Pharmaceutical Medicine (SGPM) and will be honoured with credits for pharmaceutical medicine. Participants are eligible for 8.5 credits.

SwAPP | Swiss Association of Pharmaceutical Professionals

WIFI Access

Username: **NH Hotel** Password: **meeting**

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