DIA/EFGCP/EMA Conference on How to Optimise Children's Access to Innovative Medicines

16-17 October 2017

European Medicines Agency, London, UK

PROGRAMME COMMITTEE

DIA

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Vice President, Global Medical and Regulatory Affairs
SynteractHCR, Germany; President of EUCROF, The Netherlands

OVERVIEW

In 2017, the DIA/EFGCP/EMA Annual Paediatric Conference will focus on how to optimise children's access to new medicines.

With more than 10 years of the EU Paediatric Regulation, all stakeholders involved in paediatric drug development have seen progress in developing new medicines for children.

This conference will explore how to build on the successes from the past 10 years of EU Paediatric Regulation, and how to overcome challenges that still exist.

Day 1: 10 years of Paediatric Regulation: Lessons Learnt from All Stakeholders

- Real world examples of what challenges are associated with different regulatory pathways and development scenarios as well as how to overcome them. Case studies will be presented to foster the discussion in rare diseases/small populations as well as oncology
- Discussions on tools and methods appropriate to optimise drug development such as extrapolation
- Concluding session on patient engagement within decision-making processes. What are patients' groups expectations regarding availability of new medicines and how do they define benefit/risk?

Day 2: Looking Toward the Future

Updates from ongoing European and International initiatives related to paediatric, including clinical trials

- Discussions on possible opportunities with the new European Clinical Trials Regulation when conducting paediatric clinical trials
- Quest for solutions within paediatric pharmacovigilance with a view to promote shared understanding with all parties
- Concluding sessions on exploring the future of children's access to new medicines. Different stakeholders face different challenges that are unique to Europe, while paediatric medicines development is global. This session aims to promote a common understanding and alignment on important points from different stakeholders and will set the scene for future dialogue.

There will be a specific Q&A session with EMA on paediatric procedures.

OBJECTIVES

- To update participants on current paediatric regulatory requirements, scientific and operational success and challenges
- To exchange experiences with regulatory authorities, academia and industry when developing medicines for children globally
- To discuss visions, daily challenges and potential ways to move forward and further improve processes for paediatric drug development

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
- Employees from Clinical Research Organisations (CROs) involved in paediatric clinical trials
- Any stakeholder interested in the development of better medicines for children

How to Optimise Children's Access to Innovative Medicines

DAY ONE I MONDAY, 16 OCTOBER

08:00 REGISTRATION AND WELCOME COFFEE

08:30 OPENING REMARKS

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

Solange Corriol-Rohou, Senior Director, Global Regulatory Affairs & Policy, Europe, AstraZeneca Global Medicines Development, France

Enrica Alteri, Head of Human Medicines Research and Development Support Division, European Medicines Agency (EMA), European Union

09:00 KEYNOTE SPEECH

10 YEARS OF PAEDIATRIC REGULATION - WHERE WE ARE TODAY (CHALLENGES AND OPPORTUNITIES)

Florian Schmidt, Deputy Head of Unit B5 – Medicines: Policy, Authorisation and Monitoring, DG SANTE, European Commission, Belgium

09:30 SESSION 1

DOES THE REGULATION DELIVER ON ITS GOAL OF IMPROVING AVAILABILITY OF NEW MEDICINES TO PAEDIATRIC PATIENTS IN EUROPE?

Session Chair: **Solange Corriol-Rohou**, Senior Director, Global Regulatory Affairs & Policy, Europe, AstraZeneca Global Medicines Development, France

Setting the scene...With 10 years of paediatric regulation, hundreds of agreed Paediatric Investigation Plans and a growing number of ongoing paediatric clinical trials one question has still to be answered: will all these efforts also lead to increased availability of new medicines to patients and prescribers in Europe? What has been achieved during these period as a result of the Regulation? The session will give an overview on the state of the play from the different stakeholders involved.

A Regulator's View
Michael Berntgen, Head of Product Development Scientific Support Department, European Medicines Agency (EMA), European Union

An Academia/Prescriber's View
Gilles Vassal, Head of Clinical Research Division, Institute Gustave Roussy, France

An Industry View
Bruno Flamion, VP, Head of Strategic Development, Idorsia Pharmaceuticals, Switzerland

A Patient's View
Dimitrios Athanasiou, Patient Advocate, Muscular Dystrophy Association Hellas, Greece

Panel Discussion with Q&A

10:45 COFFEE BREAK

11:15 SESSION 2

HOW TO OPTIMIZE DRUG DEVELOPMENT UP TO APPROVAL AND BEYOND - PARALLEL BREAKOUT SESSIONS WITH CASE STUDIES

Session Chair: **Martine Dehlinger-Kremer**, Vice President, Global Medical and Regulatory Affairs, SynteractHCR, Germany; President of EUCROF, The Netherlands

In the breakout sessions real world cases will give an overview of the challenges to overcome when developing medicinal products for children with rare diseases or cancer. When and how to use extrapolation, which could help optimise drug development, will also be addressed.

SESSION 2A

RARE DISEASE/SMALL POPULATION

Session Chair: **Lutz Harnisch**, Senior Director, Global Clinical Pharmacology/Pharmacometrics, Pfizer, United Kingdom

The session will highlight EMA's current approach on the development and approval of medicines for rare diseases/small populations, specifically how this approach translates into a development programme not only supporting the Health Authorities' approval, but also the acceptance by payers and HTA bodies. What all that means in practice will be illustrated by two recent cases, which not only managed the Regulatory approval, but also successfully went through the HTA assessment, leading to ultimate availability of those new treatment options to children, their parents, and their doctors. Learnings from the two case studies and the experience from the audience will be discussed to better understand what is needed to improve the development and availability of new, innovative medicines for children.

Presenters:

Laura Fregonese, Scientific Officer, European Medicines Agency (EMA), European Union

Stephanie Rosenfeld, Director, Evidence Based Medicine/ Health Economics and Outcomes Research, Sanofi-Aventis, Germany

Dunja Pfeiffer, Head of Market Access DACH Area, Pierre Fabre Pharma, Germany

Rapporteurs:

Laura Fregonese, Scientific Officer, European Medicines Agency (EMA), European Union

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

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SESSION 2B

ONCOLOGY PAEDIATRIC DEVELOPMENT: LEARNING FROM EXPERIENCE

Session Chair: **Solange Corriol-Rohou**, Senior Director, Global Regulatory Affairs & Policy, Europe, AstraZeneca Global Medicines Development, France

Through two case examples (nilotinib in chronic myeloid leukaemia (CML), and selumetinib for neurofibroma and glioma), the session will be an opportunity to share learnings and to understand how two different companies are developing paediatric plans having in mind the final objective, which is to facilitate children's access to important medicines while addressing important medical needs. In addition, the use of physiologically based pharmacokinetic (PBPK) modelling to support paediatric development will also be addressed.

Presenters:

Meike Angstenberger, Senior Global Program Regulatory Manager, RA Oncology, Novartis Pharma, Switzerland

George Kirk, Global Medicines Lead, AstraZeneca, United Kingdom

Amy Cheung, Senior Pharmacometrician, Early Clinical Development, AstraZeneca, United Kingdom

Rapporteur:

Solange Corriol-Rohou, Senior Director, Global Regulatory Affairs & Policy, Europe, AstraZeneca Global Medicines Development, France

SESSION 2C

EXTRAPOLATION – APPLICATION OF THE EMA EXTRAPOLATION FRAMEWORK

Session Chairs:

Cécile Ollivier, Scientific Officer, Science and Innovation Support Office, European Medicines Agency (EMA), European Union

Christina Bucci-Rechtweg, Global Head, Pediatric & Maternal Health Policy, Drug Regulatory Affairs, Novartis Pharmaceuticals, United States

The EMA will release for public consultation an update of the EMA Extrapolation Reflection Paper. The proposed EMA Extrapolation Framework will be presented at the session, followed by a case study. The session is aiming at illustrating and discussing with different stakeholders choices companies may have to face when extrapolation approaches are planned as part of a paediatric development, particularly considering that the selected strategy need to address requirements for the regulatory decision–making.

Presenters:

Lynne Yao, Director, Division of Pediatric and Maternal Health, Office of New Drugs, Food and Drug Administration (FDA), United States - *presenting remotely*

Darren Austin, Senior Fellow and Senior Director, Clinical Pharmacology, GSK, United Kingdom

Sabine Derey, Regulatory Manager, Respiratory Therapeutic Group, GSK, United Kingdom

Rapporteur:

Andrew Thomson, Statistician, Biostatistics & Methodology Support Office, European Medicines Agency (EMA), European Union

13:15 LUNCH

14:00 SESSION 3

REPORTS AND CONCLUSION FROM BREAKOUT SESSIONS & EMA UPDATE

Session Chair:

Mette Due Theilade Thomsen, Managing Director, PIP Adviser, Denmark

Following the feedback from the 3 breakout sessions, the session will be the opportunity for EMA Paediatric Division to provide the attendees with an update on EMA paediatric activities, and to answer specific questions sent by the attendees for EMA and/or PDCO prior to the Conference.

Feedback from the Breakout Sessions

Rapporteurs of the BOS

Update from EMA on Paediatric Related Matters and Q&A

Ralph Bax, Head of the Paediatric Medicines Office, European Medicines Agency (EMA), European Union

Peter Karolyi, Scientific Officer, European Medicines Agency (EMA), European Union

Koenraad Norga, Head of Clinic, Paediatric Oncology, Antwerp University Hospital, Belgium; Paediatric Committee (PDCO) Vice-Chair

15:30 COFFEE BREAK

16:00 SESSION 4

HOW ARE CHILDREN, CARERS AND FAMILIES CONTRIBUTING TO THE DECISION MAKING?

Session Chair:

Katie Rizvi, Founder, Little People Association & Temerarii Club, Romania

The session will focus on both the technical and practical aspects of involving children and young persons' advisory groups in decision-making processes whether at regulatory, health technology assessment (HTA) or medicines research and development (R&D) levels. Discussions will include the point of view and expectations of different stakeholders.

Panel Discussion with Q&A

Panellists:

Nathalie Bere, Patient Relations Coordinator, European Medicines Agency (EMA), European Union

Yvonne Schmidt, Scientific Advisor, Pharmaceuticals Department, Federal Joint Committee (G-BA), Germany

Pamela Dicks, Network Manager, Scottish Children's Research Network (ScotCRN), United Kingdom

Anna Sherriffs, Young Patient Advocate, Young Persons' Advisory Group (YPAG) Scotland, United Kingdom

17:30 NETWORKING RECEPTION

All delegates are invited to take part in the networking reception, which will be hosted at the EMA. This will be a very special occasion for continuing the discussions with colleagues. During this reception, a lecture on "Challenges and Opportunities/Solutions in Treatment of Neonatal Patients" will be given by **Mark Turner**, Senior Lecturer in Neonatology, University of Liverpool, United Kingdom.

19:00 END OF DAY ONE

DAY TWO | TUESDAY, 17 OCTOBER

08:30 REGISTRATION AND WELCOME COFFEE

09:00 SESSION 5

PAEDIATRIC RELATED INITIATIVES: UPDATE ON PROGRESS

Session Chairs:

Janina Karres, Scientific Officer, Paediatric Medicines Office, European Medicines Agency (EMA), European Union

Roberto De Lisa, Scientific Officer, Paediatric Medicines Office, European Medicines Agency (EMA), European Union

This session will give an overview of currently ongoing initiatives that foster understanding and development of new medicines for children and discuss how all these initiatives support the goals of the Paediatric Regulation.

New GVP – Population-Specific Considerations, Paediatric Population
Jolanta Gulbinovič, PRAC Member, State Medicines Control Agency, Lithuania

Industry Experience to PV / Risk Management in Paediatrics
Jacqueline Phillips, Director, Pediatric Product Development, Johnson & Johnson, United States

The Patient Registry Initiative and Paediatric Aspects
Patricia McGettigan, Clinical Pharmacologist, Pharmacoepidemiology Group, European Medicines Agency (EMA), European Union

Upcoming New Clinical Trials Regulation
Seán Kilbride, Assessor (Clinical Assessment), Health Products Regulatory Authority (HPRA), Ireland

10:30 COFFEE BREAK

11:00 SESSION 5 CONTINUED

ICH S11 Guideline
Georg Schmitt, Head of Toxicology (Operations) and Nonclinical Paediatrics, F. Hoffmann-La Roche, Switzerland; EFPIA Deputy Topic Leader

Revision of the ICH E11 Guideline
Solange Corriol-Rohou, Senior Director, Global Regulatory Affairs & Policy, Europe, AstraZeneca Global Medicines Development, France; EFPIA Topic Leader

European Projects of Interest:

1. **IMI2: Pan European Paediatric Clinical Trials Network**
   Heidrun Hildebrand, Global Program Head, TA Paediatric Development, Bayer, Germany; Co-Lead EFPIA Consortium
2. **IMI2: Paediatric Preclinical Proof of Concept (POC) Platform**
   Stefan Pfister, Head of Division Paediatric Neurooncology, German Cancer Research Center (DKFZ) Heidelberg, Germany
3. **The FP7 Advances in Small Trials dEsign for Regulatory Innovation and eXcellence (ASTERIX) Project**
   Kit Roes, Professor, Clinical Trial Methdology, University Medical Center Utrecht, The Netherlands

13:00 LUNCH

14:00 SESSION 6

LOOKING AT THE FUTURE

Session Chair: **Mark Turner**, Senior Lecturer in Neonatology, University of Liverpool, United Kingdom

This session will be the opportunity to look at the future and discuss how to overcome the remaining challenges and optimise opportunities to deliver innovative and efficient drugs that address children's needs. All stakeholders have key roles and responsibilities in bringing new and beneficial medicines to Children in Europe. They face different challenges that are unique to Europe, while medicines are developed for global purpose. This panel discussion aims to promote common understanding and alignment on some important points from different stakeholders and will set the scene for future dialogue.

Panellists:

- **Ralph Bax**, Head of the Paediatric Medicines Office, European Medicines Agency (EMA), European Union
- **Gilles Vassal**, Head of Clinical Research Division, Institute Gustave Roussy, France
- **Dimitrios Athanasiou**, Patient Advocate, Muscular Dystrophy Association Hellas, Greece
- **Vinciane Pirard**, Senior Director, Public Affairs (Europe & International), Sanofi-Genzyme, Belgium
- **Siri Wang**, Scientific Director, Norwegian Medicines Agency, Norway; Paediatric Committee (PDCO) Member

Introduction

Panellists' view

- Based on the experience so far, what could be the future for drug development and drug access in paediatrics?
- How to plan for a fit for purpose and successful clinical programme
- Targeting stakeholder's interactions and engagement
- Roles of existing networks and (future) EU initiatives

Panel discussion with Q&A

Conclusions

16:00 END OF CONFERENCE