CDDF-ITCC-SIOPE 4TH ANNUAL PAEDIATRIC ONCOLOGY CONFERENCE

ACCELERATING THE DEVELOPMENT OF NEW ONCOLOGY DRUGS FOR CHILDREN AND ADOLESCENTS

BRUSSELS, BELGIUM 20–21 JANUARY 2016

PROGRAMME
**DAY 1  Wednesday 20\textsuperscript{th} January 2016**

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
<th>Speaker(s)</th>
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<tbody>
<tr>
<td>09:30</td>
<td><strong>Introduction</strong></td>
<td>Martin Schrappe (SIOPE President, Germany) and Heinz Zwierzina (CDDF Chairman, Austria)</td>
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<tr>
<td>09:40</td>
<td><strong>SESSION 1: ACCELERATING DRUG DEVELOPMENT FOR CHILDREN WITH LIFE-THREATENING DISEASES</strong></td>
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<td></td>
<td><strong>Session chair:</strong> Martin Schrappe (SIOPE President) and Heinz Zwierzina (CDDF President)</td>
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<td>09:40</td>
<td><strong>Working together - a report from The CDDF–SIOPE–ITCC multistakeholder paediatric platform</strong></td>
<td>Gilles Vassal (Gustave Roussy, France)</td>
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<td>10:00</td>
<td><strong>Unite2cure - mobilizing the general public and decision makers</strong></td>
<td>Debbie Binner (Create for Chloe Foundation, UK) and Nicole Scobie (Zoé4Life, Switzerland)</td>
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<td>10:20</td>
<td><strong>Improving the implementation of the Paediatric Medicine Regulation – recent changes made by the PDCO</strong></td>
<td>Dirk Mentzer (Paul-Ehrlich-Institut, Germany &amp; Paediatric Committee at the EMA, UK)</td>
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<td>10:40</td>
<td><strong>Improving the regulatory environment in the US</strong></td>
<td>Gregory Reaman (Food &amp; Drug Administration - FDA, USA)</td>
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<td>11:00</td>
<td><strong>Round table discussion with all stakeholders</strong></td>
<td>Participants: Session speakers and chairs</td>
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<td>Patricia Blanc (Imagine for Margo, France)</td>
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<td>12:00</td>
<td><strong>Lunch</strong></td>
<td>The Twelve Restaurant, Thon Hotel EU</td>
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**SESSION 2: PRIORITISATION THROUGH MECHANISM OF ACTION – WHY? HOW?**

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<thead>
<tr>
<th>Time</th>
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<tbody>
<tr>
<td>13:00</td>
<td><strong>Prioritisation through mechanism of action in paediatric malignancies: the CDDF-ITCC-SIOPE proposal</strong></td>
<td>Andy Pearson (Institute for Cancer Research, UK)</td>
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<td>13:20</td>
<td><strong>Prioritising drug development for children with rhumatologic diseases</strong></td>
<td>Nicola Ruperto (Paediatric Rheumatology international Trials Organisation - PRINTO, Italy)</td>
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<td>13:40</td>
<td><strong>How to evaluate the relevance of a drug target for paediatric malignancies?</strong></td>
<td>Stefan Pfister (German Cancer Research Center - DKFZ, Germany)</td>
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<td>14:00</td>
<td><strong>Is it safe to recruit teenagers in adult early drug trials?</strong></td>
<td>Natalie Gaspar (Gustave Roussy, France)</td>
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SESSION 3: LONG TERM FOLLOW UP OF PATIENTS RECEIVING INNOVATIVE ONCOLOGY DRUGS

Session chair: Peter Adamson (The Children’s Hospital of Philadelphia, USA) and Jaroslav Sterba (University Hospital Brno, Czech Republic)

16:10 Being a childhood cancer survivor in daily life
Jaap den Hartogh (Dutch Childhood Cancer Parent Organization, The Netherlands)

16:30 Toxicology of innovative oncology drugs and new European pharmacovigilance provisions
Jacqueline Carleer (Agence Fédérale des Médicaments et des Produits de Santé, Belgium & Paediatric Committee at the EMA, Belgium)

16:50 Setting up long term follow up measures: the industry perspective
Jürgen Maares (Novartis, Switzerland)

17:10 Long term follow up and quality of survivorship – a major goal
Lars Hjorth (Lund University, Sweden)

17:30 Proposals from Working Group 3
Raphaël Rousseau (Genentech/Roche, USA)

17:50 Round table discussion with all stakeholders
Participants: Session speakers and chairs
Danielle Horton Taylor (Patient Advocate, UK)

18:50 End of first day

19:30 Networking dinner
The Twelve Restaurant, Thon Hotel EU
DAY2 Thursday 21st January 2016

SESSION 4: NEW INCENTIVES FOR SPECIFIC PAEDIATRIC ONCOLOGY DRUG DEVELOPMENT

Session chairs: Ralf Herold (European Medicines Agency, UK) and Pamela Kearns (University of Birmingham, UK)

08:30 Paediatric rewards, incentives and obligations: How they work and how their impact is assessed?
Florian Schmidt (European Commission, Belgium)

08:50 Creating hope act in the US – where are we after 2 years?
Nancy Goodman (Kids v Cancer, USA)

09:10 Is the Orphan regulation working for paediatric malignancies?
Gilles Vassal (Gustave Roussy, France)

09:30 Orphan Drug Regulation, Paediatric Medicine Regulations and Paediatric cancer: the industry perspective
Jeffrey Skolnik (TetraLogic Pharmaceuticals, USA)

09:50 Results of the Platform survey and Proposals from Working Group 2
Patricia Blanc (Imagine for Margo, France)

10:10 Round table discussion with all stakeholders
Participants: Session speakers and chairs
Angela Polanco (Bethanys Wish, UK)

11:10 Break

SESSION 5: WRAP UP AND 2016 ACTION PLAN

11:40 Wrap up and further development of the multistakeholder paediatric oncology platform
Gilles Vassal (Gustave Roussy, FR)

12:10 End of meeting
The Cancer Drug Development Forum (CDDF), in partnership with ITCC and SIOPE, is organising the 2016 edition of the Paediatric Oncology Conference on ‘Accelerating the Development of New Oncology Drugs for Children and Adolescents’. The platform is an innovative multi-stakeholder initiative that has over the past years established a work programme and concrete action plan to improve the development of new oncology drugs for children and adolescents.

The conference will for the 4th time convene an exchange platform for all actors – academia, parents and survivors, industry and regulators, policymakers and charities – to evaluate progress and define further steps to foster more and better cure, including with regards to the paediatric medicines legislation.

The 2016 conference will share progresses and discuss the following proposals made by the working groups:

- New strategies for improved development of oncology drugs for children and adolescents:
  - Mechanism of action based development strategy
  - Compound prioritization
  - Changes needed in the paediatric medicine regulation
- New incentives for specific paediatric drug development and drug repositioning.
- Implementation of long-term follow up measures of children and adolescents receiving new anticancer drugs.
- Recent successes in new drug development will be shared.

**Scientific and Programme Committee**

- **Conference Chair:** Gilles Vassal (Gustave Roussy, France)
- Patricia Blanc (Imagine for Margo, France)
- Gerlind Bode (Childhood Cancer International, Germany)
- Silvia Chioato (Pfizer, Italy)
- Ralf Herold (European Medicines Agency, UK)
- Koenraad Norga (Antwerp University Hospital, Belgium & vice-chair PDCO, EMA, UK)
- Andrew Pearson (The Institute of Cancer Research, UK)
- Jeffrey Skolnik (TetraLogic, USA)
- Stefan Schwoch (Eli Lilly, UK)
- Raphael Rousseau (Genentech/Roche, USA)
- Heinz Zwierzina (CDDF chairman, Austria)
Venue
Thon Hotel EU
Rue de la Loi 75, B-1040 Brussels, Belgium
Phone: +32 (0)2 204 3911
Website: www.thonhotels.be

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This Conference has received the funding support of VisitBrussels