BDA WORKSHOP

IMPROVING ONCOLOGY DRUG DEVELOPMENT FOR CHILDREN AND ADOLESCENTS
18-19 NOVEMBER 2013 | PARIS, FRANCE

ADVANCE PROGRAMME

In partnership:

SIOP
SIOP Europe
the European Society for Paediatric Oncology

encca
European Network for Cancer Research in Children and Adolescents

Funded by the European Union’s Seventh Framework Programme (FP7/2007-2013) under the project ENCCA, grant agreement HEALTH-F2-2011-261474.
The Biotherapy Development Association

The Biotherapy Development Association (BDA) is a not-for-profit organization. Our mission is to provide a unique platform to facilitate interactions between all stakeholders: academia, regulatory authorities, the pharmaceutical industry, patient advocates and policymakers in order to improve the efficiency of cancer drug development.

BDA organizes regular meetings and workshops where all stakeholders can meet and discuss the latest challenges in oncology drug development with the goal to create an "ideal" scientific, regulatory and commercial environment for the development of cancer drug.

For more information about BDA, please visit: www.bdaoncology.org

Introduction

The EU Paediatric Medicines Regulation came into force on 26th January 2007, aiming to provide better medicines for children. This Regulation is based on rewards, incentives and obligations for pharmaceutical companies; its intention was to accelerate the development of drugs for paediatric diseases, such as malignancies, with no expected direct return on investment for pharmaceutical companies. Warmly welcomed by the paediatric community, the Regulation was expected to facilitate access to anticancer drugs, which are in development in adults and to significantly increase the number of those drugs in clinical development for children and adolescents in Europe.

However, the number of new oncology drugs in paediatric development remains low in Europe. There is still a 10-fold difference between Europe and the US in the number of new anticancer drugs available for clinical research.

In June 2013, the European Commission published the interim report on the first 5 years of the implementation of the Pediatric Regulation. This report and additional publications showed positive changes in the field of pediatric drug development and identified hurdles and bottlenecks in pediatric oncology drug development.

The goal of the meeting is to state where we are, identify how the strategy for pediatric oncology drug development should be further defined and to propose solutions that may improve the implementation of the regulation in order to better meet the needs of children and adolescents with cancer.

This is a meeting that will provide a forum for discussion for all stakeholders, namely academia, parents and patients, industry, regulators, policymakers and others to share their views and challenges, to interact and propose solutions and actions for the future.

This meeting is not aimed to be a consensus meeting.

Meeting Objectives

The meeting will first address where we are at year 5 of the European Pediatric Regulation in the field of oncology and will follow up on the actions proposed during the first BDA meeting in December 2011.

Discussions will be focused on three major topics of interest for the future:

- Mechanism-of-action and biology driven development of oncology drugs for children and adolescents
- Partnerships for improving cooperation between stakeholders
- Novel designs and development plans to speed up introduction of new drugs in standard care
Programme

MONDAY, 18 NOVEMBER 2013

9:00 WELCOME AND INTRODUCTION
9:15 5 YEARS AFTER THE LAUNCH OF THE PEDIATRIC MEDICINES REGULATION: WHERE ARE WE IN PEDIATRIC ONCOLOGY?
9:15 Parents' standpoint
9:30 European Medicines Agency's experience
9:45 A vision from Academia
10:00 Industry's perspective
10:15 The point of view of the European Commission
10:30 Discussion
11:00 Conclusions and action points
11:45 ROUND TABLE AND DISCUSSION:
Key challenges to make the pediatric medicine regulation a success for children and adolescents with cancer
12:45 NEW INCENTIVES: THE US CREATING HOPE ACT
13:15 Lunch
14:15 NEW WAYS OF COOPERATION
14:15 Opportunities for new models of public private partnership
14:30 The Global Academic Alliance for early drug development
14:45 Discussion
15:15 Conclusions and action points
15:30 MECHANISM OF ACTION BASED AND BIOLOGY DRIVEN PEDIATRIC ONCOLOGY DRUG DEVELOPMENT
15:30 The medical and scientific rationale for biology-driven pediatric drug development
15:45 Feasibility from an industry standpoint
16:00 Discussion
16:30 Conclusions and action points
16:45 Break
17:15 BREAK-OUT SESSIONS
- Break out session 1: How to improve cooperation between all stakeholders?
- Break out session 2: How to design and implement mechanism -of-action based and biology driven research & development plans?
18:15 Reports from Breakout sessions to the Plenary session
18:45 CONCLUSION OF DAY 1
19:00 End of the Day 1 meeting
19:30 Reception and Networking Dinner

TUESDAY, 19 NOVEMBER 2013

9:00 INNOVATIVE AND APPROPRIATE DESIGNS AND METHODOLOGY
9:00 EXTRAPOLATING EFFICACY FROM ADULTS TO CHILDREN IN ONCOLOGY
9:00 The experience in hematological malignancies
9:15 Extrapolating from a regulatory standpoint
9:30 How and when to address extrapolation from adults to children in malignant solid tumors
9:45 Discussion
10:15 Conclusions and action points
10:30 Break
11:00 ADDRESSING THE RARITY OF PATIENTS
11:00 Experience from the Orphan Drug Regulation
11:15 Innovative designs to define a recommended dose in children and adolescents
11:30 Innovative designs for efficacy trials in rare conditions
11:45 Discussion
12:15 Conclusions and action points
12:00 CONCLUSION OF DAY 2
12:15 SUM UP OF THE MEETING AND ACTION PLAN
12:30 Adjourn and Lunch
Programme Overview

### Monday, 18 November 2013

- **8:00**  Registration opens
- **9:00**  Welcome and introduction
- **9:15**  5 Years after the launch of the pediatric medicines regulation
- **11:15**  Break
- **11:45**  Round table and discussion
- **12:45**  New incentives: the us creating hope act
- **13:15**  Lunch
- **14:15**  New ways of cooperation
- **15:30**  Mechanism of action based and biology driven pediatric oncology drug development
- **16:45**  Break
- **17:15**  Break-out sessions
- **18:15**  Reports from breakout sessions to the plenary session
- **18:45**  Conclusion of day 1
- **19:30**  Reception and networking dinner

### Tuesday, 19 November 2013

- **9:00**  Innovative and appropriate designs and methodology (I)
- **10:30**  Break
- **11:00**  Innovative and appropriate designs and methodology (II)
- **11:45**  Round table and discussion
- **12:45**  New incentives: the us creating hope act
- **12:30**  Conclusion of day 2
- **13:00**  Sum up of the meeting and action plan
- **13:15**  Adjourn and lunch

Organising Committee

- **Workshop Chairs**
  - Raphael Rousseau (Genentech, USA)
  - Gilles Vassal (Institut Gustave Roussy, France)

- **Scientific Committee**
  - Mary Brigid Bradley-Garelik (Bristol-Myers Squibb, USA)
  - Ralf Herold (European Medicines Agency, UK)
  - Andy Pearson (The Institute of Cancer Research, UK)
  - Vaskar Saha (Institute of Cancer Sciences, UK)
  - Martin Schrappe (University Medical Center Schleswig-Holstein, Germany)
  - Stefan Schwoch (Eli Lilly, UK)

Conference Venue

Hotel Novotel Charenton
3 - 5 place des Marseillais
94227 CHARENTON LE PONT
France

[www.novotel.com](http://www.novotel.com)

Registration

Register online at: [www.bdaoncology.org](http://www.bdaoncology.org)

Registration Fees

Registration fee includes attendance to the 1.5 day workshop as well as all meals (lunches and networking dinner)

<table>
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<th>Category</th>
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<tbody>
<tr>
<td>Parents and Patient Advocates</td>
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<tr>
<td>ITCC members</td>
<td>150 EU</td>
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<tr>
<td>Academia and Regulatory bodies</td>
<td>300 EU</td>
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<td>Industry</td>
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**Deadline for registration:** 12 November 2013
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WORKSHOP SECRETARIAT

Marjorie Recorbet
Biotherapy Development Association (BDA)
Avenue Mounier, 83
1200 Brussels
Belgium

Direct phone +32 2 775 02 15
marjorie.recorbet@ecco-org.eu

www.bdaoncology.org