BDA WORKSHOP

IMPROVING ONCOLOGY DRUG DEVELOPMENT FOR CHILDREN AND ADOLESCENTS

18-19 NOVEMBER 2013 | PARIS, FRANCE

PROGRAMME

BDA
BIOTHERAPY DEVELOPMENT ASSOCIATION

encca

In partnership:

Innovative Therapies for Children with Cancer
ITCC
European Consortium

SIOPE
SIOP Europe
the European Society for Paediatric Oncology

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Content

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

Organising Committee

Workshop Chairs

- Raphael Rousseau (Genentech, a member of the Roche group, 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

Metro: Line 8 – Station Liberté

Programme Overview

	Monday, 18 November 2013		Tuesday, 19 November 2013
8:00	Registration opens		
9:00	Welcome and introduction	9:00	Innovative and appropriate designs and methodology (I)
9:15	5 Years after the launch of the pediatric medicines regulation	10:30	Break
11:15	Break	11:00	Innovative and appropriate designs and methodology (II)
11:45	Round table and discussion	12:30	Conclusion of day 2
12:45	New incentives: the us creating hope act	13:00	Sum up of the meeting and action plan
13:15	Lunch	13:30	Adjourn and 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		

Programme

MONDAY, 18 NOVEMBER 2013 *ROOM DÉCOUVERTE 2*

9:00 WELCOME AND INTRODUCTION

Heinz Zwierzina (BDA) &
Gilles Vassal (ITCC – SIOPE – ENCCA)

9:15 5 YEARS AFTER THE LAUNCH OF THE PEDIATRIC MEDICINES REGULATION: WHERE ARE WE IN PEDIATRIC ONCOLOGY?

Session Chairs: *Ralf Herold (European Medicines Agency)*
Ruth Ladenstein (SIOPE, Austria)
Stefan Schwoch (Eli Lilly, UK)

9:15 **Parents' standpoint**
Patricia Blanc (Imagine for Margo, France)

9:30 **European Medicines Agency's experience**
Ralf Herold (European Medicines Agency)
Koenraad Norga (Vice-Chair Paediatric Committee EMA - University Hospital Antwerp, Belgium)

9:45 **A vision from Academia**
Gilles Vassal (European Network of Cancer Research in Children and Adolescents-ENCCA, France)

10:00 **Industry's perspective**
Mary Brigid Bradley-Garelik (Bristol-Myers Squibb, USA)

10:15 **The point of view of the European Commission**
Florian Schmidt (European Commission, Belgium)

10:30 **Discussion**
Moderators: Session Chairs

11:00 **Conclusions and action points**
Speakers: Session Chairs

11:15 Break

11:45 ROUND TABLE AND DISCUSSION: Key challenges to make the pediatric medicine regulation a success for children and adolescents with cancer

Participants:
- *Gerlind Bode (Member of the Paediatric Committee at the EMA; International Confederation of Childhood Cancer Parent Organizations, Germany)*
- *Mary Brigid Bradley-Garelik (Bristol-Myers Squibb, USA)*
- *Ralf Herold (European Medicines Agency)*
- *Martin Schrappe (University Medical Center Schleswig-Holstein, Germany)*
- *Hendrik van den Berg (Member of the Paediatric Committee at the EMA for The Netherlands; College ter Beoordeling van Geneesmiddelen, The Netherlands)*
- *Gilles Vassal (ITCC – SIOPE – ENCCA)*

12:45 NEW INCENTIVES: THE US CREATING HOPE ACT

Nancy Goodman (Kids V Cancer, USA)

13:15 Lunch *ROOM FORUM*

14:15 NEW WAYS OF COOPERATION

Session Chairs: *Bouchra Benettaib (Celgene, USA)*
Vaskar Saha (Institute of Cancer Sciences, UK)
Jaroslav Sterba (Member of the Paediatric Committee at the EMA for the Czech Republic; University Hospital Brno, Czech Republic)

14:15 **Opportunities for new models of public private partnership**
Raphael Rousseau (Roche - Genentech, USA)

14:30 **The Global Academic Alliance for early drug development**
David Ashley (Australian Children's Cancer Trials, Australia)

14:45 **Discussion**
Moderators: Session Chairs

15:15 **Conclusions and action points**
Speakers: Session Chairs

15:30 MECHANISM OF ACTION BASED AND BIOLOGY DRIVEN PEDIATRIC ONCOLOGY DRUG DEVELOPMENT

Session Chairs: *Bouchra Benettaib (Celgene, USA)*
Vaskar Saha (Institute of Cancer Sciences, UK)
Jaroslav Sterba (Member of the Paediatric Committee at the EMA for the Czech Republic; University Hospital Brno, Czech Republic)

15:30 **The medical and scientific rationale for biology-driven pediatric drug development**
Andy Pearson (The Institute of Cancer Research, UK)

15:45 **Feasibility from an industry standpoint.**
Raphael Rousseau (Roche - Genentech, USA)

16:00 **Discussion**
Moderators: Session Chairs

16: 30 **Conclusions and action points**
Speakers: Session Chairs

16:45 Break

17:15 BREAK-OUT SESSIONS

Break out session 1 *ROOM DÉCOUVERTE 4*
How to improve cooperation between all stakeholders?

Moderators: *Kevin and Karen Capel (Christopher's Smile, UK)*
Bouchra Benettaib (Celgene, USA)

Break out session 2 *ROOM DÉCOUVERTE 2*
How to design and implement mechanism-of-action based and biology driven research & development plans?

Moderators: *Jacqueline Carleer (Member of the Paediatric Committee at the EMA for Belgium; Agence Fédérale des Médicaments et des Produits de Sante, Belgium)*
Vaskar Saha (Institute of Cancer Sciences, UK)

18:15 Reports from Breakout sessions to the Plenary session *ROOM DÉCOUVERTE 2*

Speakers: Breakout session moderators

18:45 **CONCLUSION OF DAY 1**
Speakers: Workshop chairs

19:00 End of the Day 1

19:30 Reception and Networking Dinner *HOTEL RESTAURANT*

TUESDAY, 19 NOVEMBER 2013 *ROOM DÉCOUVERTE 2*

9:00 INNOVATIVE AND APPROPRIATE DESIGNS AND METHODOLOGY

Session Chairs: *Ralf Herold (European Medicines Agency)*
Raphael Rousseau (Roche - Genentech, USA)
Martin Schrappe (University Medical Center Schleswig-Holstein, Germany)

9:00 EXTRAPOLATING EFFICACY FROM ADULTS TO CHILDREN IN ONCOLOGY

9:00 **The experience in hematological malignancies**
Vaskar Saha (Institutes of Cancer Sciences, UK)

9:15 **Extrapolating from a regulatory standpoint**
Koenraad Norga (Vice-Chair Paediatric Committee EMA - University Hospital Antwerp, Belgium)

9:30 **How and when to address extrapolation from adults to children in malignant solid tumors**
Speaker to be announced

9:45 **Discussion**
Moderators: Session Chairs

10:15 **Conclusion and action points**
Speakers: Session Chairs

10:30 Break

11:00 ADDRESSING THE RARITY OF PATIENTS

11:00 **Experience from development of medicines in orphan diseases**
Ralf Herold (European Medicines Agency)

11:15 **Innovative designs to define a recommended dose in children and adolescents**
Xavier Paoletti (Institut Curie, France)

11:30 **Innovative designs for efficacy trials in rare conditions**
Marie-Cécile Le Deley (Institut Gustave Roussy, France)

11:45 **Discussion**
Moderators: Session Chairs

12:15 **Conclusions and action points**
Speakers: Session Chairs

12:30 **CONCLUSION OF DAY 2**
Speakers: Workshop Chairs

13:00 SUM UP OF THE MEETING AND ACTION PLAN

Raphael Rousseau (Roche - Genentech, USA) &
Gilles Vassal (Institut Gustave Roussy, France)

13:30 Lunch *ROOM FORUM*

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WORKSHOP SECRETARIAT

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