BDA
PAEDIATRIC ONCOLOGY WORKSHOP

New Oncology Drug Development for Children and Adolescents in Europe: Current Status and Where to Go?

LONDON, UNITED KINGDOM
5-6 DECEMBER 2011

PROGRAMME

BDA
BIOTHERAPY DEVELOPMENT ASSOCIATION

www.bdaoncology.org

ITCC

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Thanks to the work of all, we have adopted the European Union Paediatric Regulation in 2006 which was an essential step forward for the health in Europe. As rapporteur of this text to the European Parliament, it was inconceivable for me that appropriate therapies were not available for our children. This was nevertheless a reality before this Regulation.

Thanks to the European Union our children benefit now from appropriate and safe medicines, which is a major advancement for Science and Health..

Through a combination of legal obligations and well suited incentives, we have encouraged the development of these medicines while guaranteeing the quality of appropriately authorised research.

Yet, in spite of this regulation, certain children and adolescents with cancer in Europe are still denied access to innovative therapies.

I hope with all my heart that this workshop, in which essential stakeholders in the health area at the European and international levels participate, will come up with concrete solutions to solve the difficulties encountered in this disease by the health professionals, the families and the patient/parent advocacy groups.

With best wishes,

Françoise Grossetête
Member of the European Parliament

There is a major need for new oncology drugs for children and adolescents with cancer to increase cure rate and quality of cure.

In January 2007, the European Union Paediatric Regulation was introduced with the objective to improve the health of children in Europe by:

- Facilitating the development and availability of medicines for children aged 0 to 17 years;
- Ensuring that medicines for use in children are of high quality, ethically researched and authorised appropriately;
- Improving the availability of information on the use of medicines for children.

In 2011, ENCCA, an EU-funded Network of Excellence, was launched to structure clinical and translation research in paediatric and adolescent oncology in Europe.

The Workshop will:

- share recent advances in the field of paediatric oncology,
- evaluate the impact of the EU and US regulations on paediatric oncology,
- propose solutions to identified bottlenecks and hurdles.

All stakeholders will participate: Academia, Parents and Patients, Pharma Industry, European Medicine Agency, EMA Paediatric Committee, Government bodies, Members of the Parliament
The goal of the meeting is to provide an action plan in order to increase the likelihood that children and adolescents with cancer will benefit from new, safe, efficacious and age-appropriate medicines and from EU funded structuring activities along the lines of the EU Paediatric Medicine Regulation.

The meeting will be published in European Journal of Cancer.

Monday, 05 December 2011

09.30AM – 09.40AM

Welcome and Introduction of the meeting:
Heinz Zwierzina (BDA), Gilles Vassal (ITCC-ENCCA)

I. Session — 09.40AM – 10.40AM

Chair: Pierre Demolis, CHMP

Setting up the landscape

09:40 The needs for innovative therapies for children and adolescents with cancer. *Andy Pearson, Royal Marsden, ITCC*

10.00 Developing oncology drugs in adults in the era of personalised medicine. *Raphael Rousseau, Roche*

10:20 Approving oncology drugs in the era of personalised medicine. *Ralf Herold, EMA*

II. Session — 10.40AM – 12.00PM

Chair: Kathy Pritchard Jones, UCL, ENCCA

New oncology drugs for children and adolescents: Where are we? What are the issues?

10:40 Impact of the EU and US regulatory initiatives: point of view from each stakeholder
EMA – *Ralf Herold*
EU Cooperative groups – *Bruce Morland, Birmingham, ITCC*
FDA – *Greg Reaman*
COG – *Peter Adamson, Chair of COG (USA)*
Industry 1 – *Max Wegner, Bayer*
Industry 2 – *Angela Howes, Janssen R&D*

Coffee Break

Round table discussion — 12.15PM – 01.45PM

Chair: Gilles Vassal, ITCC

Patricia Blanc – Imagine for Margo
Daniel Brasseur – PDCO
Ruth Ladenstein – CCRI, Vienna, ENCCA
Agnès Saint Raymond – EMA
Stefan Schwoch – Lilly

Lunch Break

Parallel breakout sessions — 02.30PM – 04.15PM

Breakout session 1
Chairs: Andy Pearson, ITCC; Katrin Rupalla, Celgene; Lynley Marshall, EMA
How to improve early access to innovative drugs and meeting the needs of children and adolescents in Europe?

Breakout session 2
Chairs: André Baruchel, ITCC; Henk van den Berg, PDCO; Stephan Schwoch, Lilly
How to prioritise oncology compounds for development in children and adolescents with cancer?

Coffee Break

Wrap up — 04.30PM – 06.30PM

Chair: Lothar Bergmann, BDA

Early access to innovative drugs for children and adolescents in Europe

04:30 The Strategy for New Drug development by disease
Gilles Vassal, ITCC-ENCCA

04:45 Integrating paediatric oncology drug development in the strategy of Pharmaceutical companies
Raphael Rousseau, Roche

05:00 Summary and proposal by the Breakout session group 1 – chairs

Prioritisation oncology drugs

05:30 The PPTP and TARGET NCI initiatives
Malcolm Smith, Bethesda

05:45 The KidsCancerKinome project
Huib Caron, Amsterdam

06.00 Summary and proposal by the Breakout Session group 2 - chairs

Reception

Tuesday, 06 December 2011

How safe is the drug — 08.30am – 09.15am

Chair: Malcolm Smith, Bethesda

08:30 Handling early non-clinical and clinical Safety signals
Jacqueline Carleer, PDCO

08:50 Paediatric safety: a sponsor perspective
Jeffrey Skolnik, Astra-Zeneca

Parallel breakout sessions — 9.15am – 10.45am

Breakout session 3

Chairs: Angela Howes, Janssen R&D; Koen Norga, PDCO; Lars Hjorth, Pancare

How to set up long term follow up of children and adolescents exposed to new drugs and make it available for all stakeholders?

Breakout session 4

Chairs: *Bruce Morland, ITCC; Stephanie Mondabon, Bayer; Ralf Herold, EMA*

How to facilitate cooperation and collaboration between all stakeholders?

Coffee Break

Wrap up — 11.00am – 12.00pm

Chair: Heinz Zwierzina, BDA

Long term follow-up

11:00 Summary and proposal by the Breakout session group 3 - chairs

Cooperation between all stakeholders

11:30 Summary and proposal by the Breakout session group 4 - chairs

Conclusion and action plan — 12.00pm – 12.30pm

12:00 **Conclusion and action plan**
Gilles Vassal, Ralf Herold, Lothar Bergmann

General information for breakout sessions

These sessions are expected:
- to be interactive in between all stakeholders,
- to answer questions and to propose actions.

The conclusions and proposed actions will be presented in the General Session. It is suggested one of the three co-chairs presents on behalf of the break out session.

The introductory presentation of the session will include:
- description of the situation and questions to be addressed (see after)
- this is aimed at avoiding spending time on sharing views on the topic to be addressed

Chairpersons may present a few slides to provide information related to the topic and to stimulate discussion. Lengthy presentations should be avoided to favour interactive discussions.

Breakout session 1

How to improve early access to innovative drugs and meeting the needs of children and adolescents in Europe?

Chairs: Andy Pearson, ITCC; Katrin Rupalla, Celgene; Lynley Marshall, EMA

This session is aiming at addressing the following situation:
- Despite the Pharma obligation of submitting a PIP as early as end of phase I trial in adults, the number of new drugs in early development in children in Europe did not increase yet, as compared to the situation in the US.
- Patients in relapse have an extremely limited access to new drugs in early paediatric development in Europe which is often started late as compared to the US.
- Due to the insufficient number of early clinical trials in Europe, paediatric oncologists are tempted to prescribe new marketed oncology drugs off-label and off-prospective clinical studies that would warrant patient protection, safety evaluation and relevant efficacy information.

Thus, early access means: availability of new drugs for paediatric development early during their development in adults.

The questions to be answered are:
- What are the Pros and Cons for starting the paediatric development early during the drug development process in adults?
- How to increase the number of new oncology drugs in early development in children and adolescents in Europe in a timely fashion?

Propose 4 actions to increase the number of new oncology drugs in early development in children and adolescents in Europe

Breakout session 2

How to prioritise oncology compounds for development in children and adolescents with cancer?

Chairs: André Baruchel, ITCC; Henk van den Berg, PDCO; Stephan Schwoch, Lilly

This session is aiming at addressing the current situation:
- More than 800 oncology drugs are in development worldwide with an anticipated 5 to 10% of them eventually marketed in adults.
- Several companies are developing drugs against the same target and compete to be first in class
- Paediatric cancers are rare, even rarer when considering molecular sub-groups

The questions to be addressed are:
- What are the criteria upon which a targeted oncology drug should be prioritized among others for its entry in paediatric clinical development?
  - Preclinical therapeutic evaluation, tumour biology, safety, …….
- How cooperative groups prioritize among several drugs targeting the same relevant target which is relevant for one or several paediatric malignancies?
- How EMA and PDCO prioritize among several drugs targeting the same relevant target for one or several paediatric malignancies that are in development even though not all yet submitted for a PIP?

Propose 4 actions to facilitate prioritisation of oncology drugs for their development in children and adolescents.

Breakout session 3

How to set up long term follow up of children and adolescents exposed to new drugs and make it available for all stakeholders?

Chairs: Angela Howes, Janssen R&D; Koen Norga, PDCO; Lars Hjorth, Pancare

This session is aiming at addressing the current situation:

- Most new oncology drugs have a new mechanism of action. Their long-term toxicity is unknown.
- There is a need for long term follow up of children and adolescents exposed to new drugs, up to their adulthood and beyond. This is a key binding element in most oncology PIPs.
- Paediatric oncologists are used to follow their patients on the long term and long-term toxicities of chemotherapy and radiotherapy have been identified through cohort studies.

The questions to be addressed are:

- What are the specific needs for long term up of and adolescents exposed to new drugs? From each stakeholder point of view.
- What can industry do and not do?
- What can academia do and not do?

Propose 4 objectives and actions upon which a proposal for a joint program between academia and industry can be designed in order to guarantee adequate long-term follow up of patients.

Breakout session 4

How to facilitate cooperation and collaboration between all stakeholders?

Chairs: *Bruce Morland, ITCC; Stephanie Mondabon, Bayer; Ralf Herold, EMA*

This session is aiming at addressing the current situation:

- The major stakeholders for the implementation of new oncology drug development for children and adolescents are: academia, industry, parents/ patients, regulatory, funding organisations and health technology assessment bodies.
- Designing, developing and authorising new oncology drugs for paediatric malignancies with the EU regulation is a brand new activity for each stakeholder.

The goals of the session are:

- Identify the current ways of cooperation and collaboration between stakeholders
- Identify the limits, hurdles and needs for improvement

Propose 4 actions to facilitate cooperation and collaboration between all stakeholders

BDA, Biotherapy Development Association (www.bdaoncology.net/); CCRI, Children's Cancer Research Institute; CHMP, Committee for Medicinal Products for Human Use; COG, Children's Oncology Group; DG Sanco, EC Directorate General Health and Consumers; EMA, European Medicine Agency (www.ema.europa.eu); ENCCA, European Network for Cancer research in Children and Adolescents; FDA, US Food and Drug Administration; ITCC, network for Innovative Therapies for Children with Cancer (www.itcc-consortium.org); NCI, National Cancer Institute; PDCO, Paediatric Committee; PIP, Paediatric Investigation Plan; PPTP, Paediatric Preclinical Testing Programme

BDA

Scientific Organising Committee

Academia G. Vassal – (chair)
Institut Gustave Roussy – ITCC, ENCCA
A. Pearson
Institute for Cancer Research, ITCC

EMA R. Herold – (co-chair)

PDCO H. van den Berg
Amsterdam Medical Center

BDA L. Bergmann – (co-chair)
H. Hendriks

Industry A. Howes *(Janssen Research & Development)*
S. Schwoch *(Lilly)*
L. Narburgh *(Roche)*
S. Mondabon *(Bayer Healthcare)*
K. Rupalla *(Celgene)*
A. De Bock *(Astra-Zeneca)*
J. Henslee-Downey *(EMD Serono)*

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