Ethical considerations for paediatric trials - how can Ethics Committees (ECs) in the European Member States and the Paediatric Committee (PDCO) at the European Medicines Agency work together?

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#	Time	Agenda	Speaker	Min
--	09:00-09:05	Welcome		5
1	09:05-09:20	Role of the PDCO in the European regulatory system	Daniel Brasseur	15
2	09:20-09:35	Legal-regulatory framework for the assessment of paediatric trials in Europe and feed back on EMA meeting on third country clinical trials	Agnès Saint Raymond	15
3	09:35-09:45	Discussion		10

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Role of the PDCO in the EU Regulatory System

Daniel Brasseur
PDCO Chair
European Medicines Agency

27.12.2006 EN Official Journal of the European Union L 378/1

I

(Acts whose publication is obligatory)

REGULATION (EC) No 1901/2006 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL

of 12 December 2006

on medicinal products for paediatric use and amending Regulation (EEC) No 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004

6 EN Official Jour

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on medicinal products for paediatric use and amending Regulation (EEC) No 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004

(Text with EEA relevance)

Key objectives of the Regulation

– **To improve the health of the children of Europe, by:**

- **increasing high quality research for medicinal products for children**
- **promoting the development and authorization of such medicines at the EU level**
- **improving the information on medicines designed for children**

– **While *avoiding unnecessary studies* in children and not delaying the authorization of medicines for adults**

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Key Tools of the Regulation

- **Mandatory paediatric development for *new* products according to a PIP agreed upon by the PDCO (possible deferrals or waivers)**
- **Mandatory submission of paediatric data when filing new applications unless waiver or deferral approved by the PDCO**
- **New Marketing Authorisation Procedure for off-patent products (PUMA)**

3 Key Pillars of the Regulation

- **Obligation : Paediatric Investigation Plan [PIP] (agreed and compliant in its conduct)**
- **Reward [incentives] for studies conducted (6 months patent extension, only once)**
- **Paediatric Committee [PDCO] at the EMEA (advisory body to EMEA in its executive role)**

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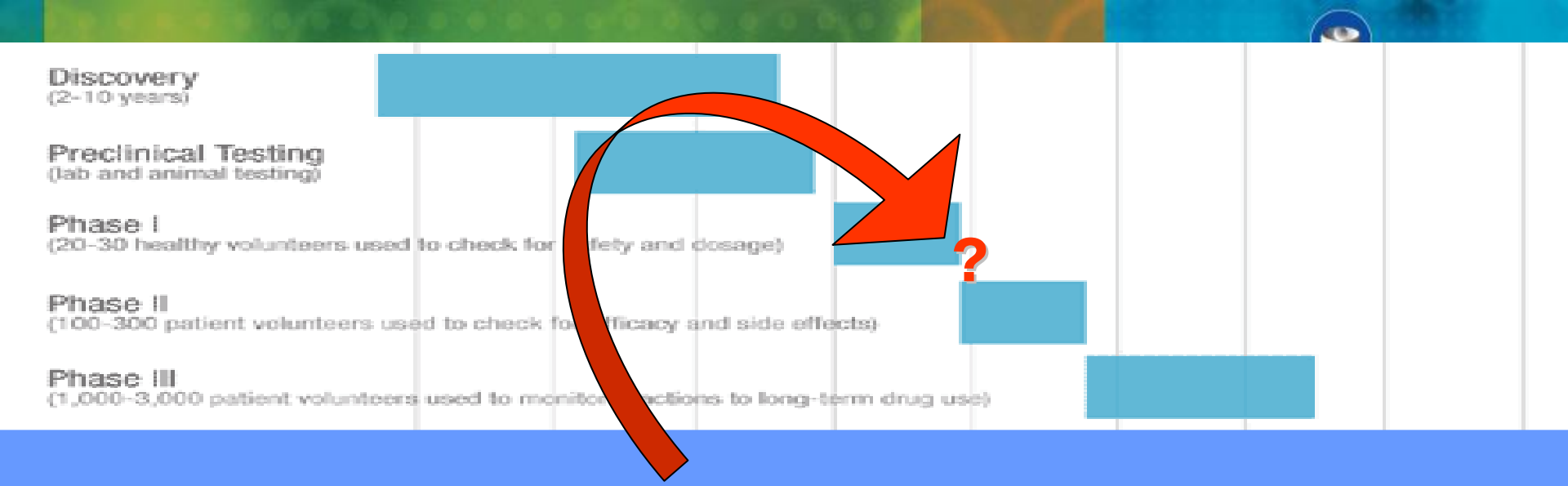

PIP... is a plan

including formulations, preclinical safety, clinical efficacy, and some pharmacovigilance

describing the drug development

-Timing of studies: start, duration and end, with respect to submission of Marketing Authorisation applications

-Population (age groups) to be studied

PDCO

5 CHMP members
+
1 members per Member State not yet represented
+
6 members from families & HCP associations

Each member has an alternate

Article 6

1. The tasks of the Paediatric Committee shall include the following:

(a) to assess the content of any paediatric investigation plan for a medicinal product submitted to it in accordance with this Regulation and formulate an opinion thereon;

(b) to assess waivers and deferrals and formulate an opinion thereon;

Roles pertaining to fulfilling the needs

Article 17

1. Following receipt of a proposed paediatric investigation plan which is valid in accordance with the provisions of Article 15(2), the Paediatric Committee shall appoint a rapporteur and shall within 60 days adopt an opinion as to whether or not the proposed studies will ensure the generation of the necessary data determining the conditions in which the medicinal product may be used to treat the paediatric population or subsets thereof, and as to whether or not the expected therapeutic benefits justify the studies proposed. When adopting its opinion, the Committee shall consider whether or not the measures proposed to adapt the formulation of the medicinal product for use in different subsets of the paediatric population are appropriate.

Roles pertaining to fulfilling the needs

PDCO
PIPlan

Roles pertaining to fulfilling the needs

PDCO
PIPlan

SAWP
R&D

Roles pertaining to fulfilling the needs

PDCO
PIPlan

SAWP
R&D

EC
CT advice

NCA
CT auth

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PDCO
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EC
CT advice

SAWP
R&D

NCA
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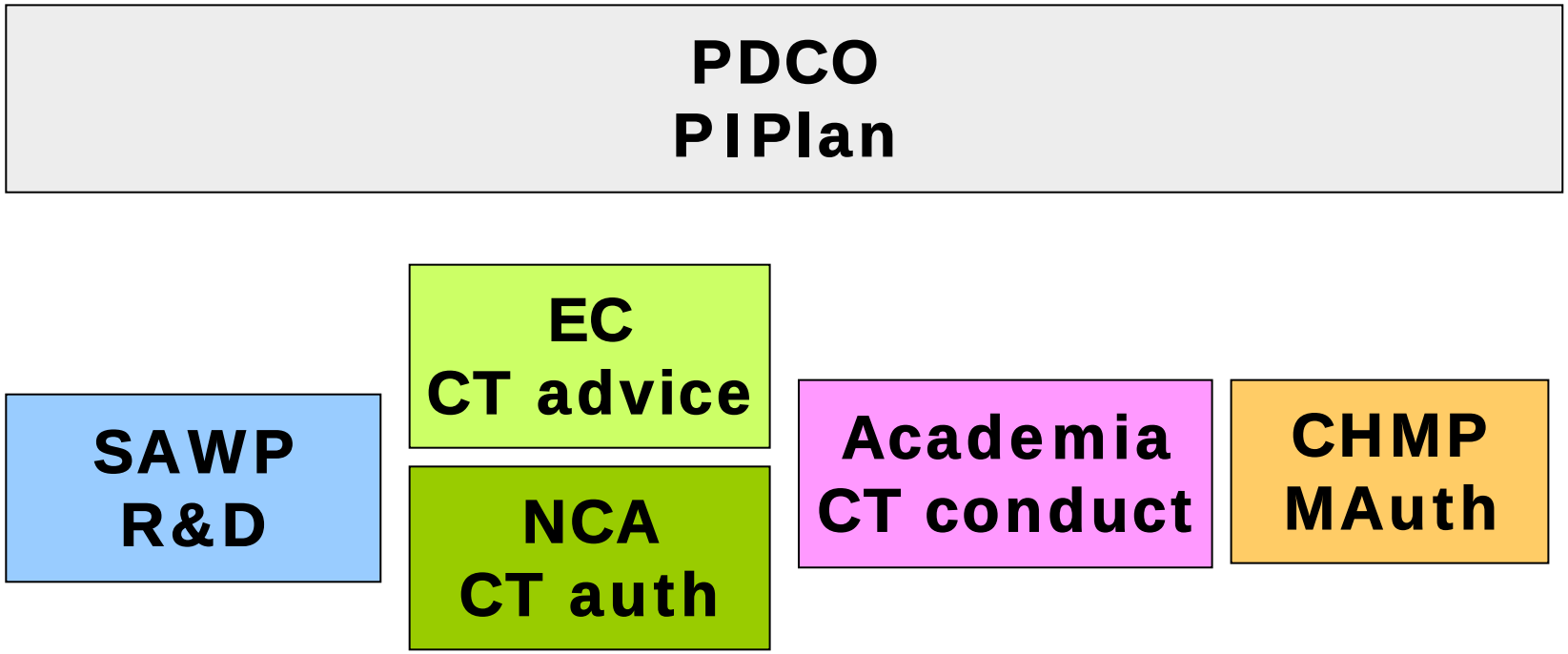
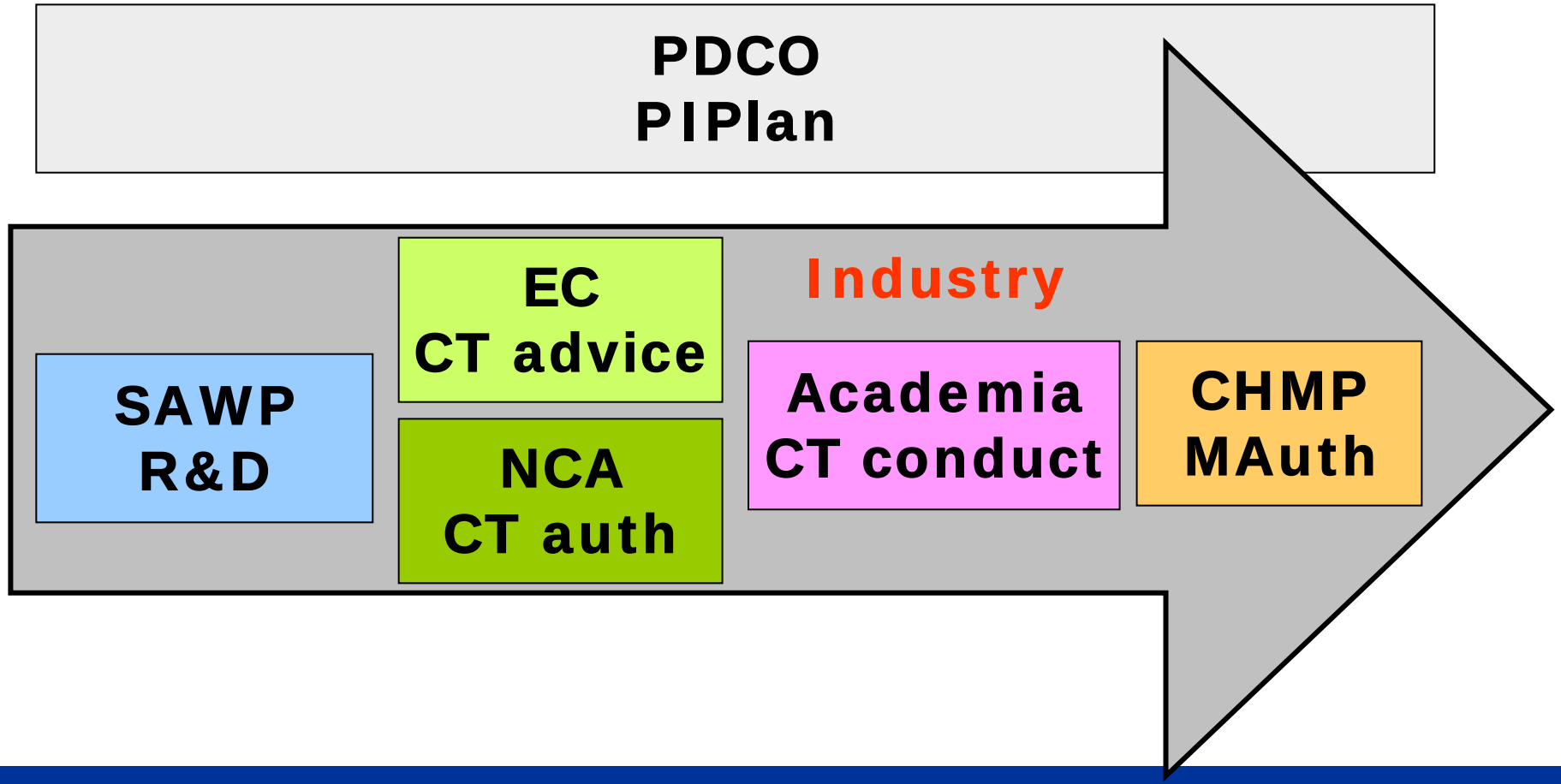
Academia
CT conduct

Roles pertaining to fulfilling the needs



Roles pertaining to fulfilling the needs



Roles pertaining to fulfilling the needs

(c) at the request of the Committee for Medicinal Products for Human Use, a competent authority or the applicant, to assess compliance of the application for a Marketing Authorisation with the agreed paediatric investigation plan concerned and formulate an opinion thereon;

(d) at the request of the Committee for Medicinal Products for Human Use or a competent authority, to assess any data generated in accordance with an agreed paediatric investigation plan and formulate an opinion on the quality, safety or efficacy of the medicinal product for use in the paediatric population;

(f) to support and advise the Agency on establishing the European network referred to in Article 44;

(g) to assist scientifically in the elaboration of any documents related to the fulfilment of the objectives of this Regulation;

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Penta was established in 1999 as a collaboration between paediatric HIV centres in Europe. Trials are principally funded by the European Union through BIOMED II, by governmental bodies in a number of European countries and by support from the pharmaceutical industry.

The PENTA foundation for the treatment and care of children with HIV serves as an umbrella for the PENTA clinical trials network as well as for future work in training and cohort collaborations.

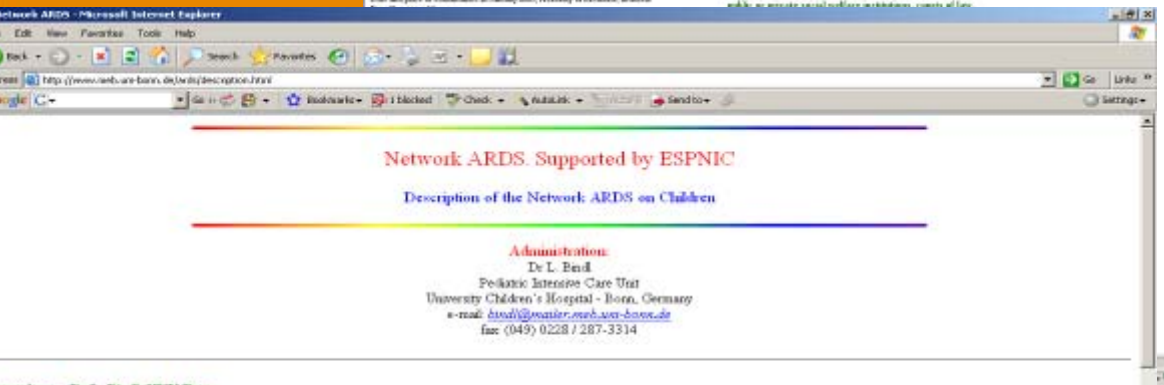
Many questions about treatment for HIV can be answered in adult trials and it is unnecessary to repeat all trials in children. However, the natural history of vertical HIV infection differs from that in adults in some important ways and the tolerance of drugs in children may also be different.

European Medicines Agency

What's new?

London, 15 January 2008
Doc. Ref. EMEA/MB/543523/2007

The Network of Paediatric Networks at the EMEA Implementing Strategy



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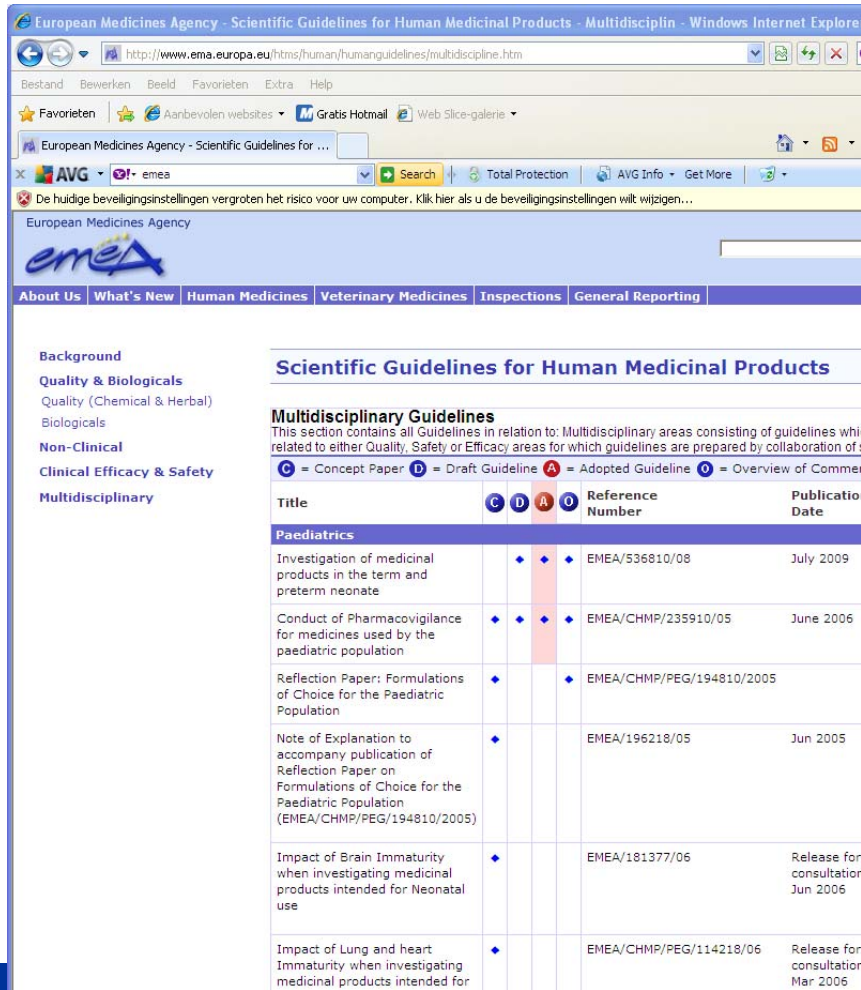

Roles pertaining to establishing needs

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

10 December 2010
EMA/794083/2009
Human Medicines Development and Evaluation

(e) to advise on the content and format of data to be collected for the survey referred to in Article 42;

(i) to establish a specific inventory of paediatric medicinal product needs and update it on a regular basis, as referred to in Article 43;

(j) to advise the Agency and the Commission regarding the communication of arrangements available for conducting research into medicinal products for use in the paediatric population;

Report on the survey of all paediatric uses of medicinal products in Europe

Established according to article 42-43 of Regulation (EC) No 1901/2006 of the European Parliament and of the Council on medicinal products for Paediatric use

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European Medicines Agency
Evaluation of Medicines for Human Use

London, 04 July 2008
Doc. Ref. EMEA/224688/2006/rev1

ASSESSMENT OF THE PAEDIATRIC NEEDS
DIABETES TYPE I AND II

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EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

26 July 2010*
EMA/480197/2010
Human Medicines Development and Evaluation

Revised priority list for studies into off-patent paediatric medicinal products

for the 5th call 2011 of the 7th Framework Programme of the European Commission

This priority list of off patent medicines is the basis only for the 5th Call 2011 of the 7th Framework Programme of the European Commission.

The 6th call 2012 will be based on a separate priority list. Please refer to the website of the European Medicines Agency.

Priority List

In accordance with the Paediatric Regulation, an inventory of the needs for medicines intended for children, as established by the Paediatric Committee, will be published shortly after 26 January 2009.
A similar exercise to establish paediatric needs was carried out between 2001 and 2007 by the Paediatric Working Party (PEG) — a temporary working party of the CHMP, established prior to implementation of the Paediatric Regulation. The lists of paediatric needs as adopted by the PEG will serve as a basis for the inventory of needs to be established by the Paediatric Committee.

List of paediatric needs (as established by the Paediatric Working Party)

Please refer to 'EMEA/PEG procedure for identifying paediatric needs' (EMEA/175192/2004/rev2) before reviewing any of the documents in the table below.

Roles pertaining to establishing needs

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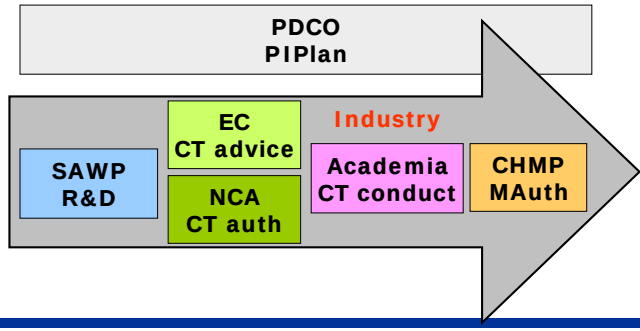
The EMA/PDCO regularly hold workshops on paediatrics-related topics

Expert Meeting on Clinical Investigation of New Drugs for the Treatment of Chronic Hepatitis C in the Paediatric Population, April 2011 High grade glioma expert group, December 2010 Expert Group Meeting on Paediatric Heart Failure, November 2010 Paediatric Rheumatology Experts Group Meeting, November 2010 Expert meeting on gastroenterology and rheumatology, June 2010 Expert meeting on neonatal and paediatric sepsis, June 2010 Expert meeting on specific immunotherapy, January 2010 Workshop on paediatric formulations for assessors in national regulatory agencies, 2010 Second workshop on European Paediatric Network, 2010 Paediatric Rheumatology Experts Group Meeting, 2009 Paediatric Epilepsy Experts Group Meeting, 2009 Meeting of the Paediatric Diabetes Mellitus Experts Group, 2009 Meeting of the Paediatric Human Immunodeficiency Virus (HIV) Experts Group First European Medicines Agency Workshop on European Paediatric Network, 2009 European Medicines Agency workshop on modelling in paediatric medicines, 2008 Workshop on FP7 and off-patent medicines developed for children, 2007 Workshop on Neonates, 2006 Workshop on Paediatric pain, 2004

Roles pertaining to fulfilling the needs



Conclusion

The role of PDCO is to foster / coordinate

research
in children
on medicinal products
allowing the conduct of sound programmes
generating useful & Q information
for a safe and easy use,

placing such medicinal products on the market


Thank
You!