Support offered by the Agency for the development of paediatric medicines

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Support offered from the Agency

1. Scientific Guidelines
2. Procedural guidance
3. Proceedings from Expert groups
4. Meetings/TC with applicants
5. Model PIPs ("standard" PIPs)
6. Publications from PDCO members / EMA staff on relevant regulatory/scientific aspects
7. EMA/national Scientific Advice
8. Enpr-EMA network
9. Other published information
1) EMA Scientific Guidelines

- General guidelines on quality, nonclinical and clinical development (http://tinyurl.com/EMAguidelines)

  
  ✓ Draft guideline “Pharmaceutical Development of Medicines for Paediatric Use” (http://tinyurl.com/draftqualitypaeds), deadline for comments 31 Dec 2011

  ✓ “Investigation of medicinal products in the term and preterm neonate”, (http://tinyurl.com/EMAneonates), 2010
2) EMA Procedural Guidance
for PIPs, waivers, modifications, compliance checks

- EU Commission guideline
  (http://tinyurl.com/ECGuidancePIP)

- Procedural advice Q&As
  (http://tinyurl.com/PIPQ-A)
  26 Q&A, recently updated (September 2011)

- Other documents:
  - All Templates and Deadlines for applications
    (http://tinyurl.com/PaedTemplatesDates)
  - New Q&A on PUMAs
    (http://tinyurl.com/PUMAQ-A) recently published September 2011
  - Q&A on Compliance Check
    (http://tinyurl.com/CC-Guidance) recently updated
3) Proceedings from Expert groups
(http://tinyurl.com/PaedExpGroups)

Not binding for PDCO, but provide general guidance for PIP development
3) Proceedings from Expert groups

(http://tinyurl.com/PaedExpGroups)
4) Meetings with applicants

- Paediatric Pipeline meetings (any time)
- Presubmission meetings (before submission) Q&A 26 in http://tinyurl.com/PIPQ-A
- Clarification of the PDCO Request for Modification (during clockstop)
- Oral explanation meeting (D120, D90)
5) Model PIPs (“Standard PIPs”)

• Two adopted so far (http://tinyurl.com/ModelPIPs):
  – Pandemic vaccines
  – Immunotherapy of ocularrhinitis

• Future developments:
  – Oncology: Acute myeloid leukaemia, Rhabdomyosarcoma, High-grade glioma, others
  – Vaccines: standardised vaccine schedules for PIP studies
  – Border between model PIP and guideline to be defined
6) Publications by PDCO members / EMA staff

- On relevant scientific / regulatory aspects
- Draft prepared
- Soon to be published on EMA website (including link to Pubmed abstract) after agreement by authors
7) EMA / National Scientific Advice

- Free EMA Scientific Advice for questions related to Paediatric development
- PDCO delegate and Paediatric coordinator participate in EMA Scientific Advice procedure
8) Enpr-EMA
European Network of Paediatric Research at EMA

Key operational goals

- To link together existing networks

- **To provide expertise and access to infrastructure for industry to conduct studies in children**

- To define consistent and transparent quality standards

- **To harmonise clinical trial procedures**

- To define strategies for resolving major challenges

- **To communicate with external stakeholders**
Support offered from Authorities

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9a) List of Paediatric needs

- **List of Paediatric Needs** by EMA’s Paediatric Expert Group (2006)
- **Aim:** to identify the needs in the different therapeutic areas where there should be research and development of medicinal products, either **old** (i.e. off patent) or **new** ones.
- Consultation of EU member states, learned societies.
- To be updated soon by EMA’s PDCO (Q4 2011)
9b) EMA Decisions on PIPs, waivers

(http://tinyurl.com/PIPDecisions)

Search by condition to evaluate previous PIPs-waivers
9c) European Clinical Trials Register

https://www.clinicaltrialsregister.eu

EU Clinical Trials Register

What's Changed in EU Clinical Trials Register?

The following enhancements have been made to the EU Clinical Trials Register during the recent maintenance:

- The Home page now displays statistics for the 'Number of clinical trials with subjects less than 18 years old' based on the number of clinical trials. Previously this number was based on clinical trial applications.
- The number of records which are displayed on the search results page has increased from 10 to 20 per page.
- Improved formatting of the display of results for clinical trial application for multiple country trials.
- Trial Status is now displayed in brackets after the country codes. To access the full record that you are most interested in, click the two-letter alpha code.

Staggered release of clinical trial information from 22 March 2011

Historical data (information entered into the EudraCT database between 1 May 2004 and the release of version 8.0 of the EudraCT database on 10th March 2011) will be gradually published online from 22 March 2011.

Statistics on clinical trials

The "Number of clinical trials with subjects less than 18 years old" displayed on the EU Clinical Trials Register homepage now corresponds to the number of clinical trials. Previously this number was based on clinical trial applications.
9c) Paediatric clinical trials in European Clinical Trials Register (ECTR)

(https://www.clinicaltrialsregister.eu)

- Includes:
  - protocol-related information (and results in future) of all clinical trials and of other trials submitted to NCAs
  - Additionally, for paediatric studies:
    - Third countries trials linked to a PIP;
    - Paediatric studies initiated before 2004, if included in a PIP.
- Currently holding approx. 15,500 trials, of which approx. 2000 include paediatric patients
- If a study is included in a PIP and has been submitted to a NCA, the protocol can be found in ECTR
Thank you

Any questions?