EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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)
- Paediatric-specific guidelines (http://tinyurl.com/paedguidelines, http://tinyurl.com/paedguidelines2)
  - ✓ 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)

Expert meeting on clinical investigation of new drugs for the treatment of chronic hepatitis C in the paediatric population

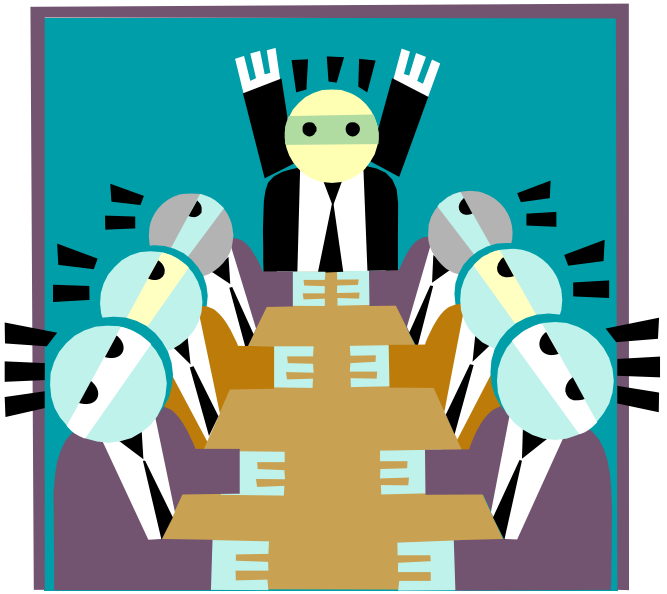
Email

Details | All documents

Name	Language	First published	Last updated
Agenda - Expert meeting on clinical investigation of new drugs for the treatment of chronic hepatitis C in the paediatric population	(English only)	12/07/2011	
Minutes of expert meeting on clinical investigation of new drugs for the treatment of chronic hepatitis C in the paediatric population: Monday 4 April 2011	(English only)	12/07/2011	
Presentation - European Medicines Agency/Committee for Medicinal Products for Human Use draft guidance on paediatric hepatitis C virus drug development; what, when, who, how?	(English only)	12/07/2011	
Presentation - Treatment of chronic hepatitis C virus infection in children and adolescents: Guidance for clinical trials	(English only)	12/07/2011	
Presentation - Paediatric-investigation-plan applications assessed by the Paediatric Commitee for the treatment of chronic hepatitis C in children	(English only)	12/07/2011	
Presentation - Objectives expert meeting: List of questions: Preliminary answers	(English only)	12/07/2011	

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)

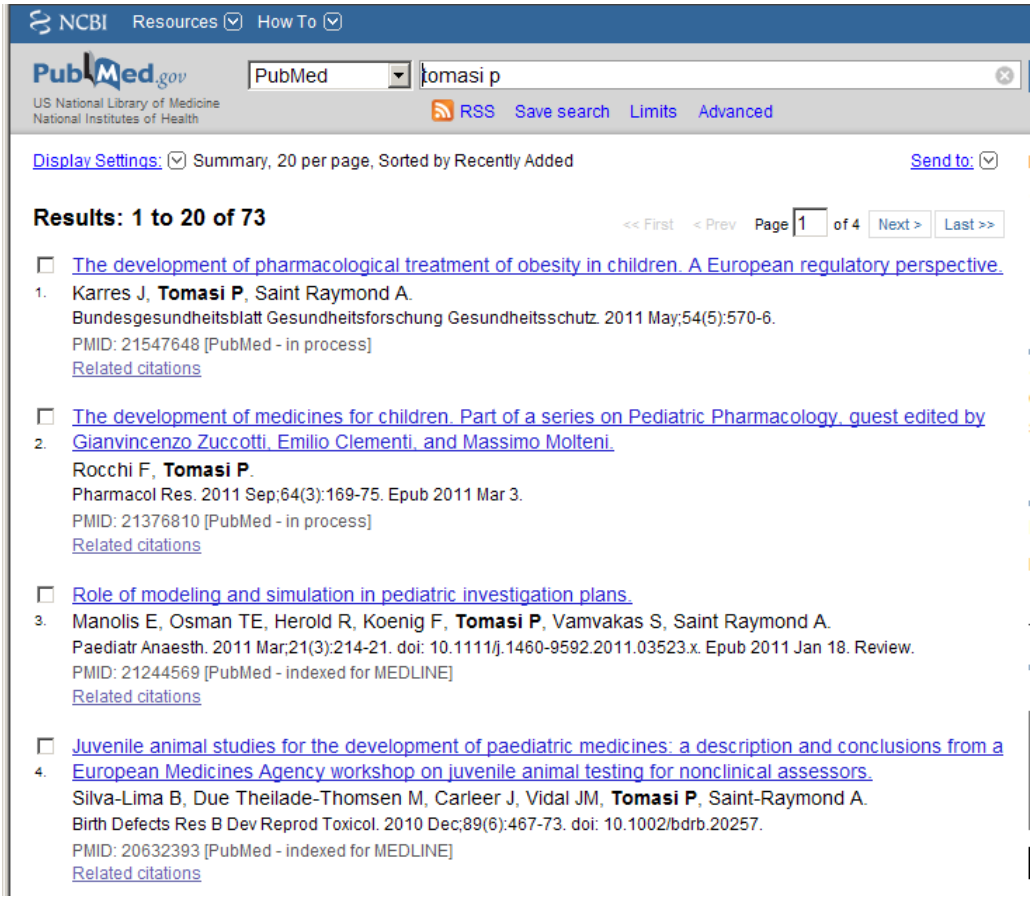


5) Model PIPs ("Standard PIPs")

- Two adopted so far (http://tinyurl.com/ModelPIPs):
  - Pandemic vaccines
  - Immunotherapy of oculorhinitis
- 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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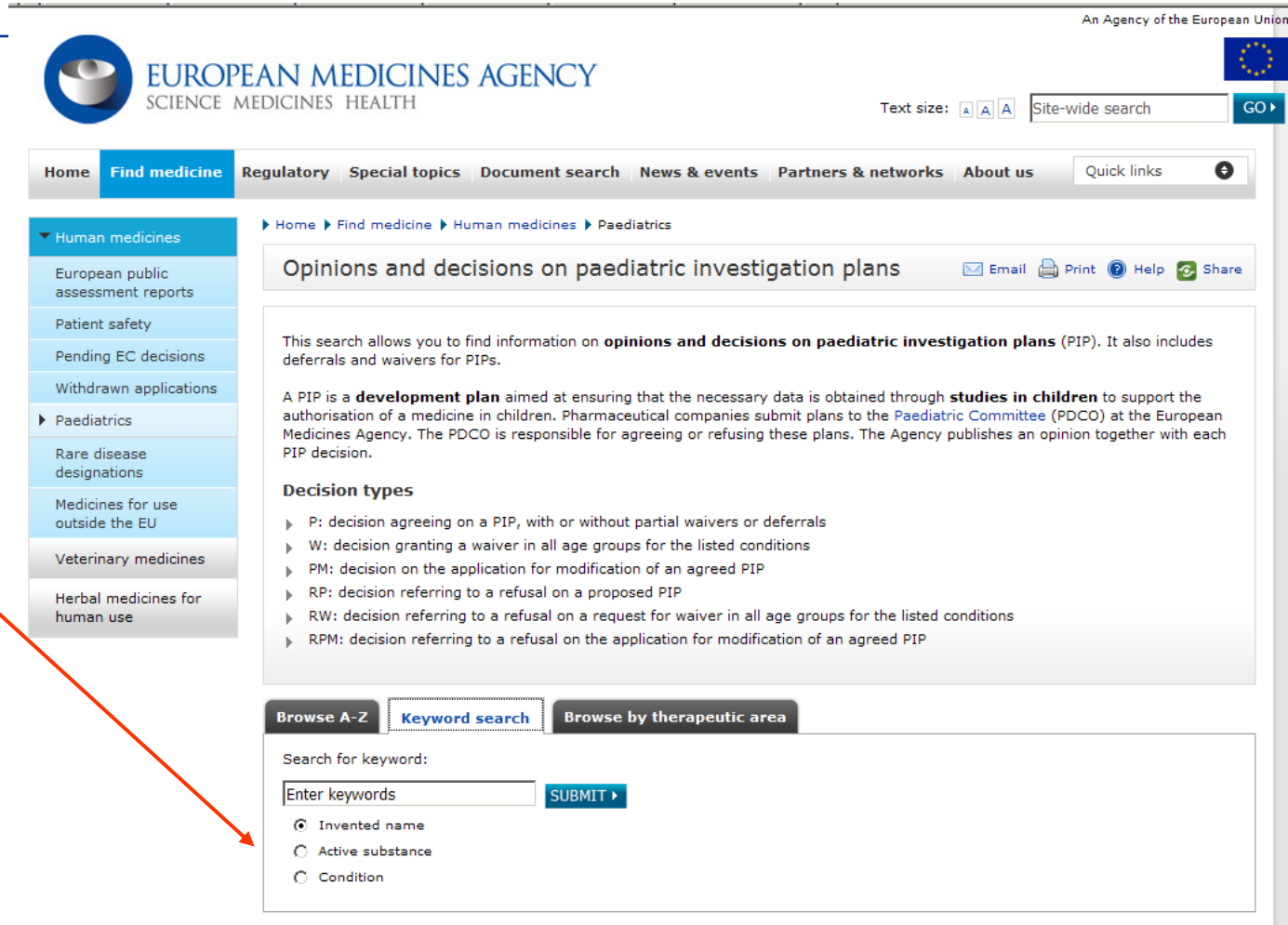
9a) List of Paediatric needs

- **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

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?