



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

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- Pre-authorisation
- Post-opinion
- Post-authorisation
- Product information
- Scientific advice and protocol assistance
- Scientific guidelines
- Innovation Task Force
- Regulatory and procedural guidance
- SME office

▼ Paediatric medicine

- Paediatric Regulation
- Application guidance
- Opinions and decisions
- Post-assessment
- ▼ Related information
- Paediatric needs

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Paediatric workshops

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The European Medicines Agency regularly organises 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



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

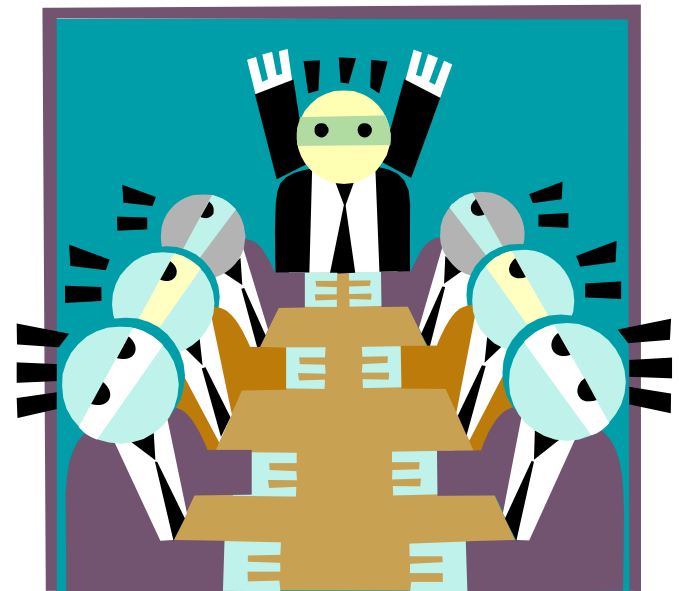
All documents

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 Committee 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

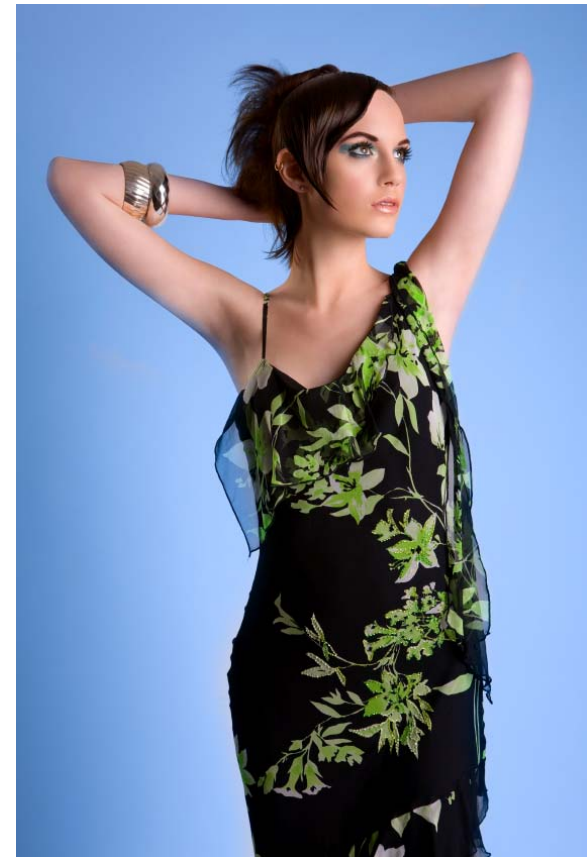
- Paediatric Pipeline meetings (any time)
- Presubmission meetings (before submission) Q&A 26 in <http://tinyurl.com/PIPQ-A>
- Clarification of the PDCO Request for Modification (during clockstop)
- Oral explanation meeting (D120, D90)





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

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National Institutes of Health

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☐ [The development of pharmacological treatment of obesity in children. A European regulatory perspective.](#)
1. Karres J, **Tomasi P**, Saint Raymond A.
Bundesgesundheitsblatt Gesundheitsforschung Gesundheitsschutz. 2011 May;54(5):570-6.
PMID: 21547648 [PubMed - in process]
[Related citations](#)

☐ [The development of medicines for children. Part of a series on Pediatric Pharmacology, guest edited by Gianvincenzo Zuccotti, Emilio Clementi, and Massimo Molteni.](#)
2. Rocchi F, **Tomasi P**.
Pharmacol Res. 2011 Sep;64(3):169-75. Epub 2011 Mar 3.
PMID: 21376810 [PubMed - in process]
[Related citations](#)

☐ [Role of modeling and simulation in pediatric investigation plans.](#)
3. Manolis E, Osman TE, Herold R, Koenig F, **Tomasi P**, Vamvakas S, Saint Raymond A.
Paediatr Anaesth. 2011 Mar;21(3):214-21. doi: 10.1111/j.1460-9592.2011.03523.x. Epub 2011 Jan 18. Review.
PMID: 21244569 [PubMed - indexed for MEDLINE]
[Related citations](#)

☐ [Juvenile animal studies for the development of paediatric medicines: a description and conclusions from a European Medicines Agency workshop on juvenile animal testing for nonclinical assessors.](#)
4. Silva-Lima B, Due Theilade-Thomsen M, Carleer J, Vidal JM, **Tomasi P**, Saint-Raymond A.
Birth Defects Res B Dev Reprod Toxicol. 2010 Dec;89(6):467-73. doi: 10.1002/bdrb.20257.
PMID: 20632393 [PubMed - indexed for MEDLINE]
[Related citations](#)



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 PDCCO (Q4 2011)



9b) EMA Decisions on PIPs, waivers

(<http://tinyurl.com/PIPDecisions>)

Search by
condition to
evaluate
previous PIPs-
waivers

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Opinions and decisions on paediatric investigation plans

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This search allows you to find information on **opinions and decisions on paediatric investigation plans (PIP)**. It also includes deferrals and waivers for PIPs.

A PIP is a **development plan** aimed at ensuring that the necessary data is obtained through **studies in children** to support the authorisation of a medicine in children. Pharmaceutical companies submit plans to the [Paediatric Committee \(PDCO\)](#) at the European Medicines Agency. The PDCO is responsible for agreeing or refusing these plans. The Agency publishes an opinion together with each PIP decision.

Decision types

- ▶ P: decision agreeing on a PIP, with or without partial waivers or deferrals
- ▶ W: decision granting a waiver in all age groups for the listed conditions
- ▶ PM: decision on the application for modification of an agreed PIP
- ▶ RP: decision referring to a refusal on a proposed PIP
- ▶ RW: decision referring to a refusal on a request for waiver in all age groups for the listed conditions
- ▶ RPM: decision referring to a refusal on the application for modification of an agreed PIP

[Browse A-Z](#) [Keyword search](#) [Browse by therapeutic area](#)

Search for keyword:

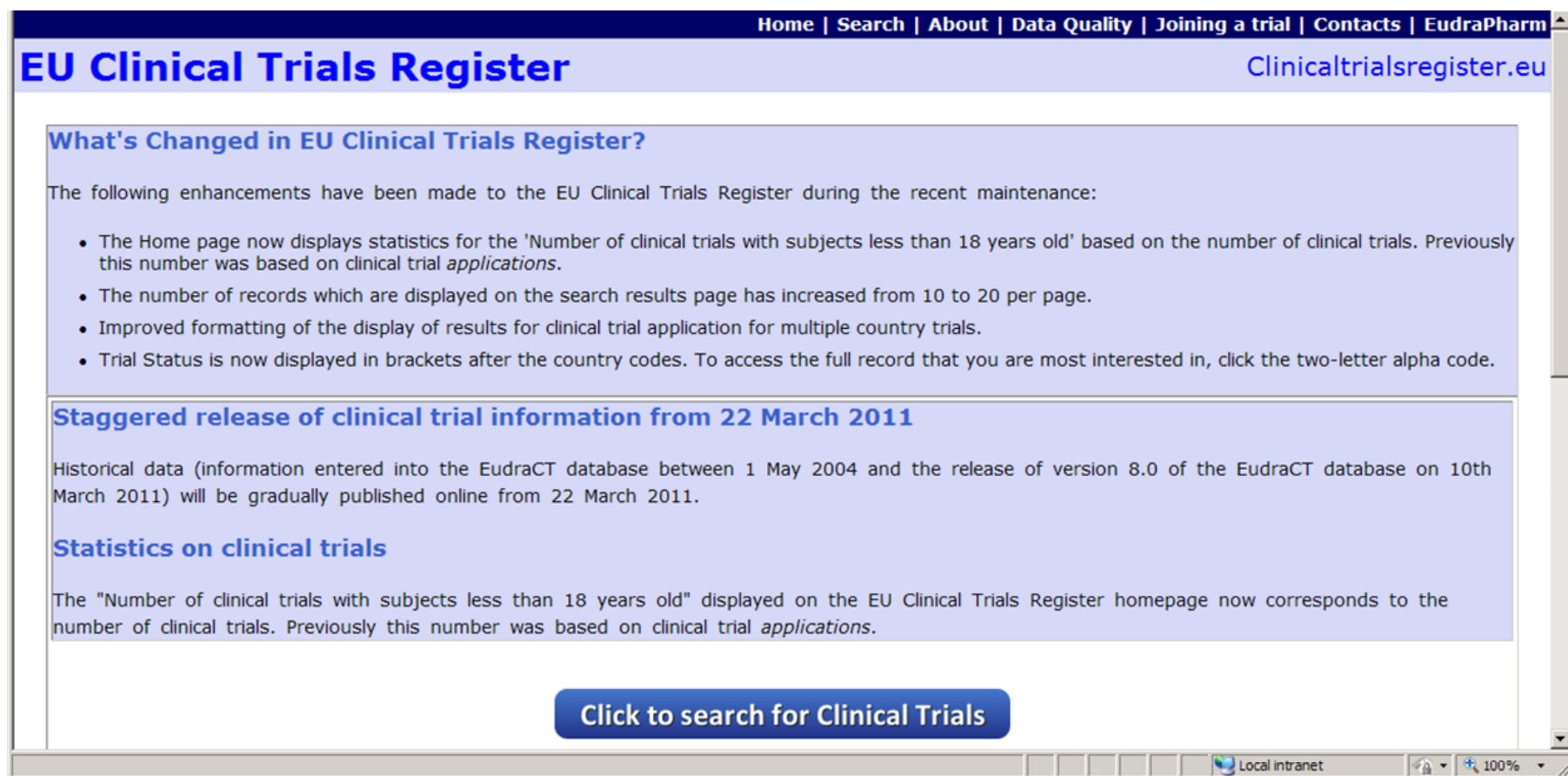
[SUBMIT](#)

☒ Invented name
☐ Active substance
☐ Condition



9c) European Clinical Trials Register

<https://www.clinicaltrialsregister.eu>



The screenshot shows the homepage of the EU Clinical Trials Register. At the top, there is a navigation bar with links: Home | Search | About | Data Quality | Joining a trial | Contacts | EudraPharm. Below this, the title 'EU Clinical Trials Register' is displayed on the left, and the URL 'Clinicaltrialsregister.eu' is on the right. The main content area is divided into sections. The first section, 'What's Changed in EU Clinical Trials Register?', lists four enhancements: 1. 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 based on clinical trial applications. 2. The number of records displayed on the search results page has increased from 10 to 20 per page. 3. Improved formatting of the display of results for clinical trial application for multiple country trials. 4. 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. The second section, 'Staggered release of clinical trial information from 22 March 2011', states that 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. The third section, 'Statistics on clinical trials', states that 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 based on clinical trial applications. At the bottom, there is a large blue button that says 'Click to search for Clinical Trials'. The browser's status bar at the bottom shows 'Local intranet' and '100%' zoom.

Home | Search | About | Data Quality | Joining a trial | Contacts | EudraPharm

EU Clinical Trials Register

Clinicaltrialsregister.eu

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

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Statistics on clinical trials

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[Click to search for Clinical Trials](#)

Local intranet 100%



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?

