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Ethical, Legal and social issues in paediatric research: the proposed ELSI service

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EPTRI Stakeholders Roundtable Virtual Meeting July 9th, 2020

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Disclaimer

This presentation reflects only the author(s)'s view

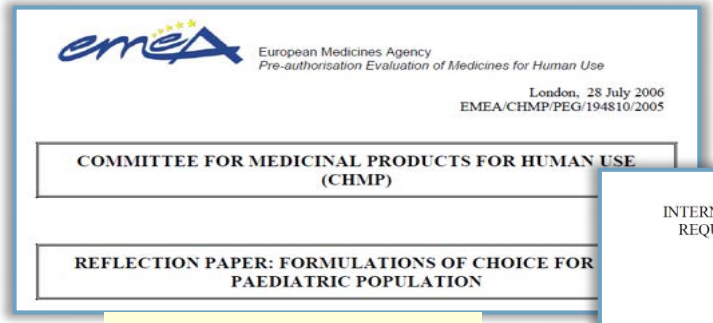
DOI
https://www.ema.europa.eu/sites/default/files/ContactsAndExperts_CVs_and_DOIs/giannuzziv1_DI_en.pdf

R&D in paediatrics

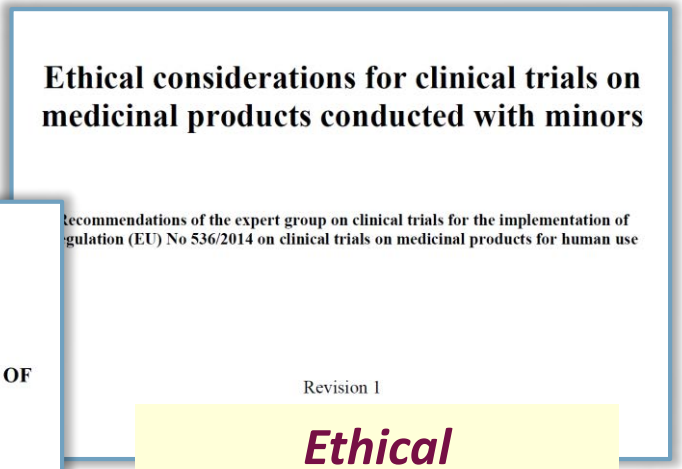
more difficult, takes longer and costs more

drugs and devices to be adequately developed, studied and used

Paediatric-specific regulatory provisions



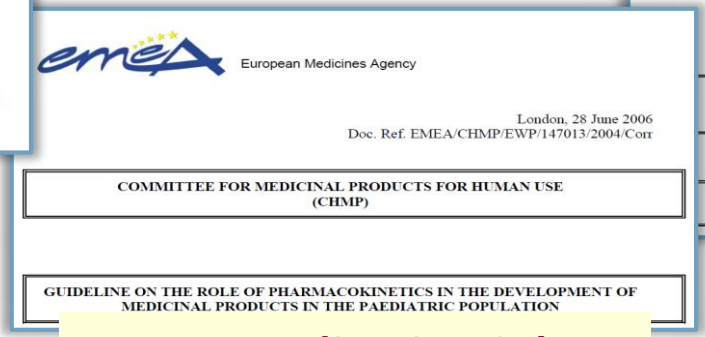
Formulations



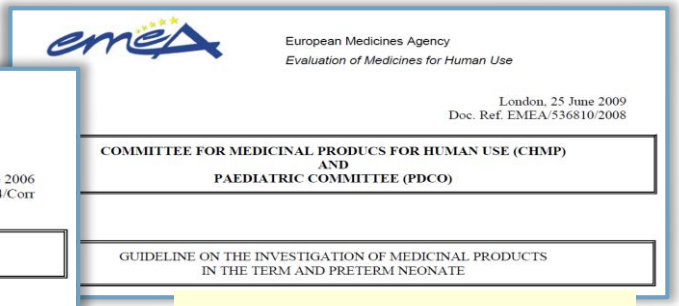
Ethical Recommendations

ICH Topic E11

Pharmaceutical development



PK paediatric trials



Trials in neonates

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..and further regulatory provisions

ATMP

Orphans

Formulations

Biomarkers

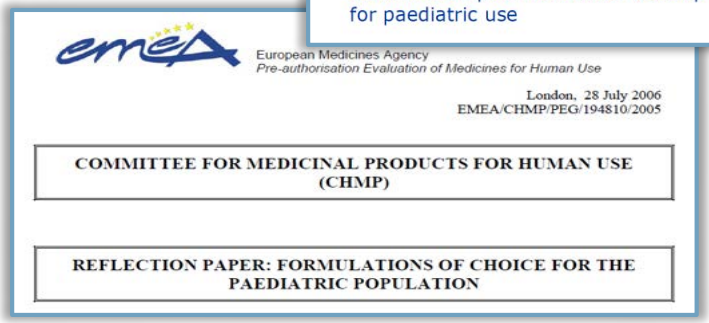

10.12.2007 EN Official Journal of the European Union L 324/121

REGULATION (EC) No 1394/2007 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL
of 13 November 2007
on advanced therapy medicinal products and amending Directive 2001/83/EC and Regulation (EC) No 726/2004
(Text with EEA relevance)


EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

1 August 2013
EMA/CHMP/QWP/805880/2012 Rev. 2
Committee for Medicinal Products for Human Use (CHMP)
Paediatric Committee (PDCO)

Guideline on pharmaceutical development of medicines for paediatric use


European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

London, 28 July 2006
EMEA/CHMP/PEG/194810/2005

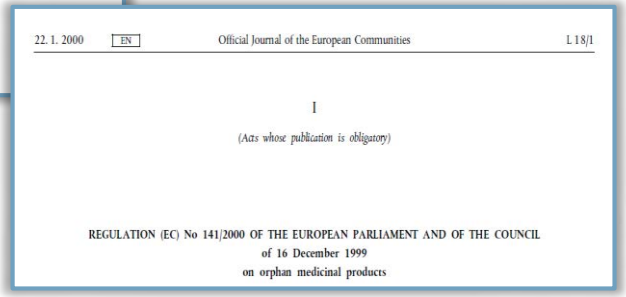
COMMITTEE FOR MEDICINAL PRODUCTS FOR HUMAN USE (CHMP)

REFLECTION PAPER: FORMULATIONS OF CHOICE FOR THE PAEDIATRIC POPULATION


EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

10 November 2014
EMA/CHMP/SAWP/72894/2008
Revision 1: January 2012[1]
Revision 2: January 2014[2]
Revision 3: November 2014[3]
Scientific Advice Working Party of CHMP

Qualification of novel methodologies for drug development: guidance to applicants


22. 1. 2000 EN Official Journal of the European Communities L 18/1

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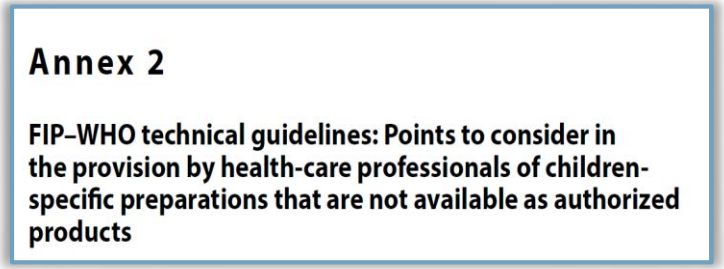
(Acts whose publication is obligatory)

REGULATION (EC) No 141/2000 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL
of 16 December 1999
on orphan medicinal products


Annex 2

FIP–WHO technical guidelines: Points to consider in the provision by health-care professionals of children-specific preparations that are not available as authorized products

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Safeguarding animals performing non-clinical studies

Ethical justification

3Rs

(i) REDUCTION

(ii) RENEMENT

(iii) REPLACEMENT

20.10.2010 EN Official Journal of the European Union L 276/33

DIRECTIVES

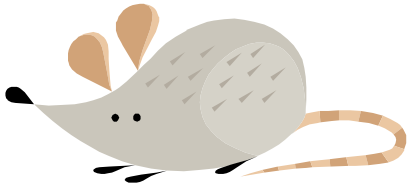
DIRECTIVE 2010/63/EU OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL
of 22 September 2010
on the protection of animals used for scientific purposes
(Text with EEA relevance)

European Medicines Agency

London, 24 January 2008
Doc. Ref. EMEA/CHMP/SWP/169215/2005

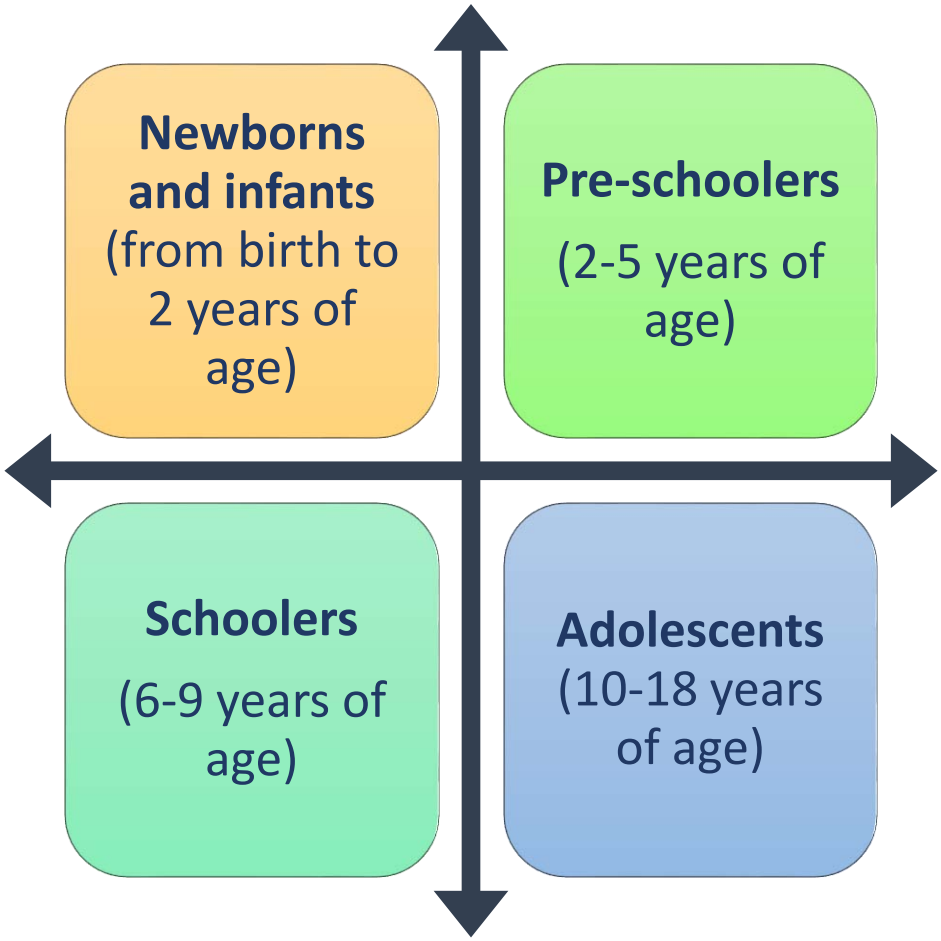
COMMITTEE FOR HUMAN MEDICINAL PRODUCTS
(CHMP)

GUIDELINE ON THE NEED FOR NON-CLINICAL TESTING IN JUVENILE ANIMALS OF PHARMACEUTICALS FOR PAEDIATRIC INDICATIONS



Strengthening children participation in the decision-making process

Only consent from parent(s)/legal representative



Only consent from parent(s)/legal representative

Assent + consent from parent(s)/legal representative

Group considered differently across Member States…

EC Ethical Recommendations, 2017
from A. Didio lesson - ARISE project, July 2020

Strengthening children participation in the decision-making process

European Network of Paediatric Research at the European Medicines Agency

26 June 2018

Informed Consent for Paediatric Clinical Trials in Europe 2015[i]

Developed by the Working Group on Ethics

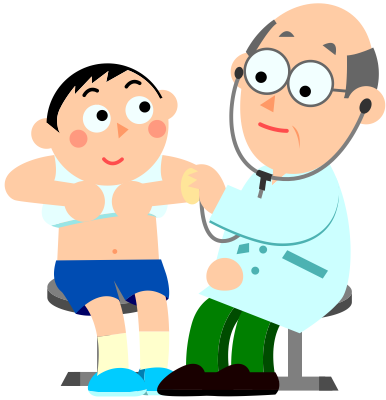
	Consent / assent from child		Consent from parent(s) / guardian(s)	General informed consent information	
Country	Legal age of consent	Mandatory / suggested age ranges defined for assent (or consent if assent not used)	Number of required signatories	Official language requirements	IC template(s) / guidelines / information sources

Protecting children's wellbeing

Procedures for paediatric studies ⇨ collection of human tissues and cells

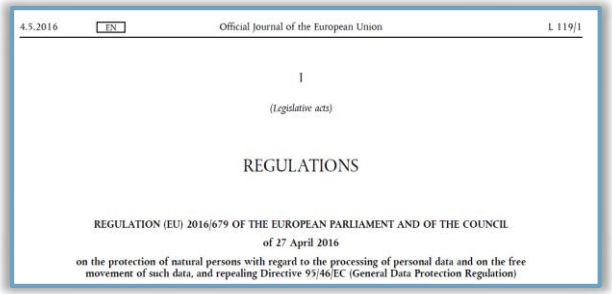
Pain, discomfort, fear, distress and any other foreseeable risk to be minimized:

- Study-related procedures limited to most necessary avoiding repeated invasive procedures
- Volume of blood withdrawing minimized



EC Ethical Recommendations, 2017

Protecting children's personal data



4.5.2016 EN Official Journal of the European Union L 119/1

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(Legislative acts)

REGULATIONS

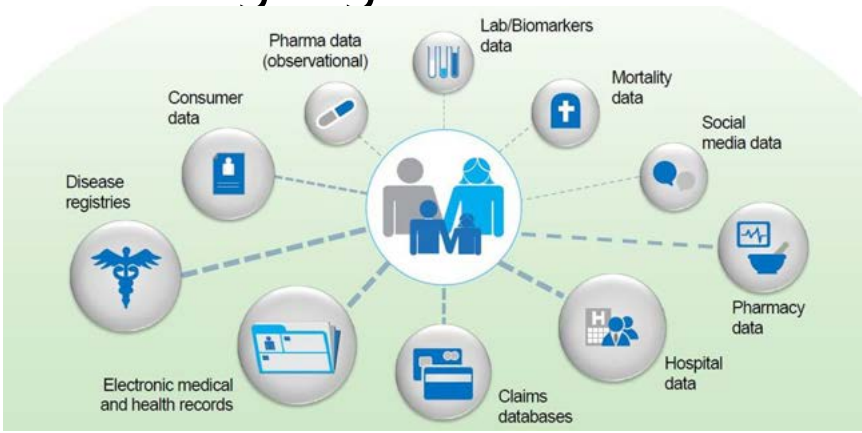
REGULATION (EU) 2016/679 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL
of 27 April 2016
on the protection of natural persons with regard to the processing of personal data and on the free movement of such data, and repealing Directive 95/46/EC (General Data Protection Regulation)

Subjects ≥16 years old can provide consent to process personal data *(possible* lower age nationally - above 13 years)

Information in a clear and plain language

Children may be less aware of risks, consequences and safeguards and their rights ⇨ later may want to remove data

More and more secondary use of health data

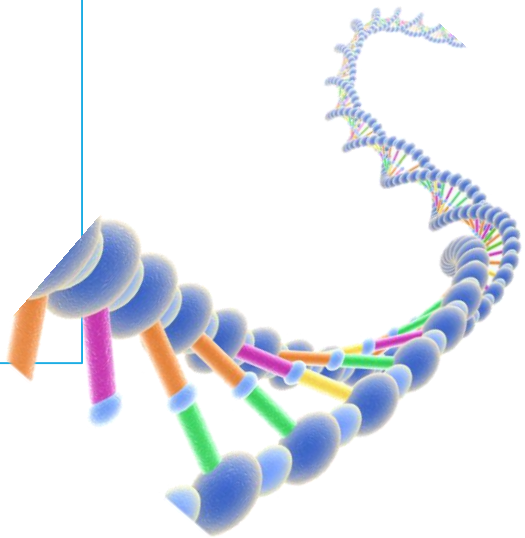


Protecting children's personal data

Genetic data

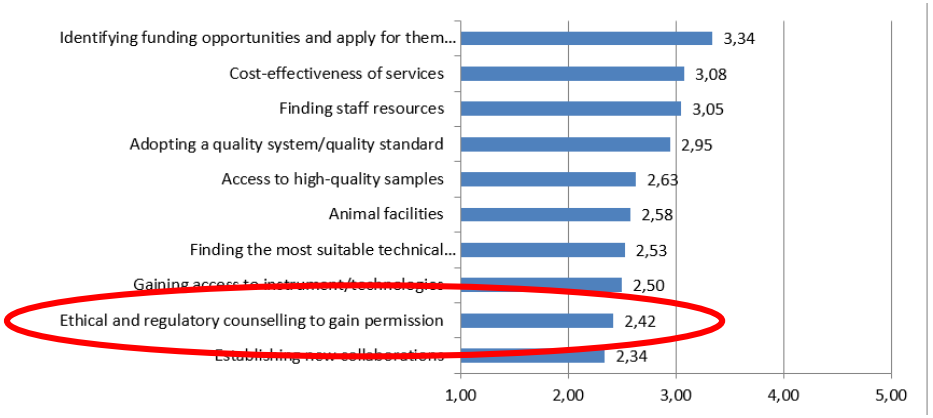
ad hoc procedures:

- Separate information
- Separate informed consent and assent
- Genetic counselling
- Incidental findings

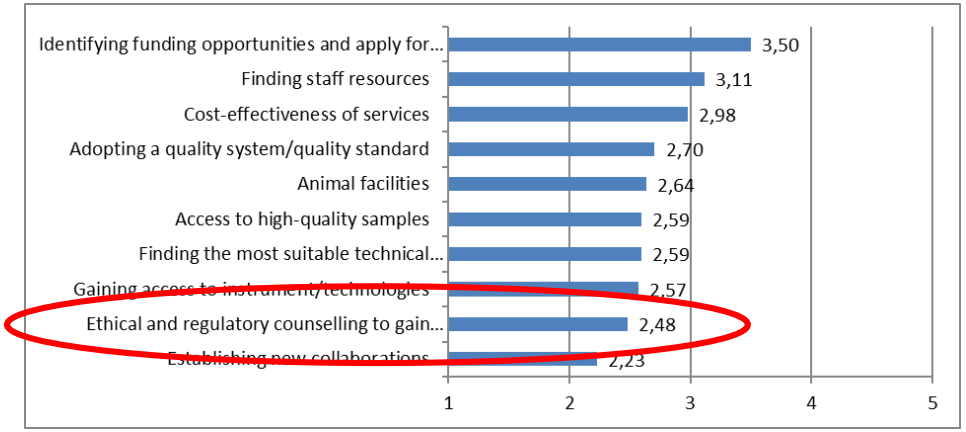


Need for ethics and regulatory support

Research services needed in the framework of a research infrastructure



Difficulties relevant to research activities



Specific expertise, resources and facilities currently not available/accessible in paediatric research activity

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An ELSI service dedicated to paediatric translationalresearch

- To guarantee that ethical/regulatory requirements are fulfilled and adopted
- To promote appropriate research participation of minors

- ✓ Research misconduct minimized
- ✓ Rights of children as “future generation” protected
- ✓ Translation of basic research findings into deliverable solutions facilitated

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An ELSI service dedicated to paediatric translational research

Paediatric Medicines Discovery

Paediatric Biomarkers and Biosamples

Developmental Pharmacology

Paediatric Medicines Formulations

NON-CLINICAL RESEARCH

CLINICAL PROCEDURES CONSENT/ASSENT

DATA PROTECTION

BIOSAMPLES HANDLING

GENETIC TESTING

ADVANCED/NEW PRODUCTS

An ELSI service dedicated to paediatric translational research

Multidisciplinary expertise

RESEARCH

SUPPORT (helpdesk)

TRAINING

NON-CLINICAL RESEARCH

CLINICAL PROCEDURES CONSENT/ASSENT

DATA PROTECTION

BIOSAMPLES HANDLING

GENETIC TESTING

ADVANCED/NEW PRODUCTS

Cooperation & synergies

- Complementary effort on paediatric paeculiarities in these fields
- Collaborating with other RIs and initiatives and institutions according to the topic and competences

Cooperation & synergies

BBMRI-ERIC → existing ELSI service

collaborating to cover unmet paediatric ethical needs and integrating in the paediatric sector BBMRI biobanks tools and quality criteria

Cooperation & synergies

ECRIN
EUROPEAN CLINICAL RESEARCH INFRASTRUCTURE NETWORK

→ Collection of regulatory documents

collaborating to cover unmet paediatric ethical needs and integrating in the paediatric sector ECRIN clinical trial tools and quality criteria

Something more…

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Deliverable 10.5 "Ethical/regulatory review of the CDR"

H2020-INFRADEV-2017-1

Concluding…

A RI devoted to paediatrics research will help to addressing ethical and legal uncovered issues related to research with a translation approach in paediatrics

- ⇨ to bridge between preclinical and clinical R&D
- ⇨ to strengthen the efforts avoiding duplications in research, speeding up the availability of proper health products for children / robust data for paediatric medicines and medical devices development

Viviana Giannuzzi

PharmD, PhD

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TEDDY member

EPTRI EAB member

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