



RESPECT
patient needs



Promoting Transparency in European Paediatric Clinical Research

*Researchers Informing Patients
Patients Informing Health Science and Policy*

RESPECT Project Dissemination Meeting

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&



Strategic Initiative for Developing Capacity in Ethical Review

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The Request of the European Commission

HEALTH-2007-4.1-4

Identifying patients' needs in the clinical trials context.

- How can patients be better **mobilised** and **empowered**?
- How can patients get the **clinical outcomes** that really matter to them?
- How can the **patients needs** be integrated into clinical trials.?

These questions should be addressed involving patients, clinicians, regulators, and researchers (industrial and academic) on a **broad basis** in order to cover a broad spectrum of diseases.



Relating Expectations and Needs to the Participation and Empowerment of Children in Clinical Trials RESPECT

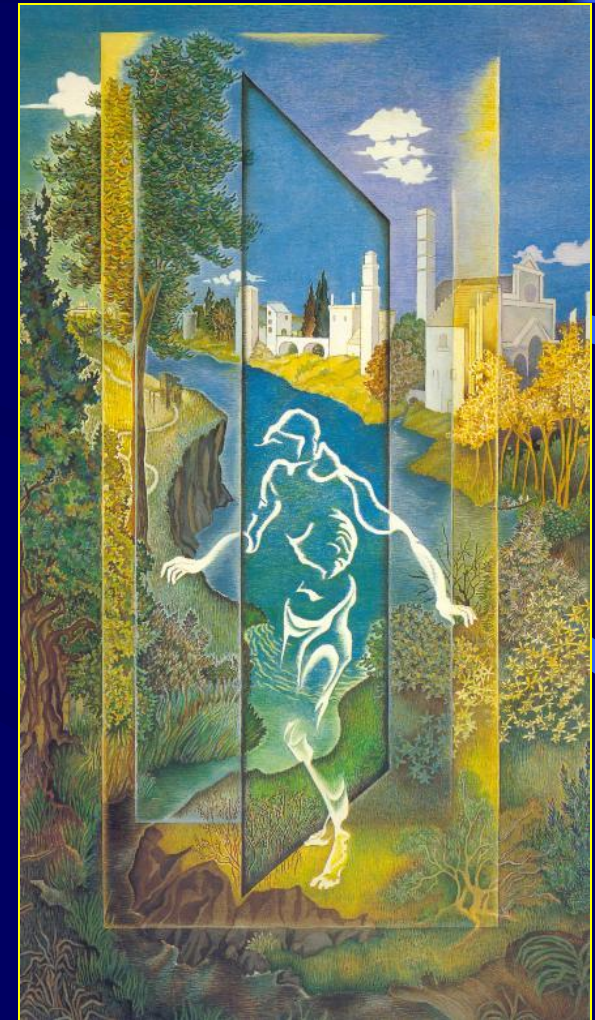
Project Objectives

1. to identify the **needs** of children and their families as related to **outcomes in clinical trials**
2. to identify **methods** by which these needs can be translated into **empowering and motivating participants** in future clinical trials research



What is our engagement in paediatric research ethics?

- Protecting children from/in health research?
- Justifying paediatric health research?
- Advancing children's health?



Rights & Responsibilities in Children's Health Research

- Health Research is a **public good**
→ the pursuit, and the results,
of paediatric health research science belong to
all children, European and beyond
- Improving children's health is an
obligation and a **responsibility**
- Research on human subjects is
never a right, *always* a responsibility
- We need to complement a rights-based
ethics with **an ethics of responsibility**
(the internalizing of the ethical disposition)



Why Paediatric Health Research Is Important

- An **ethical obligation** to improve child health in Europe and globally
- Health is essential to child **development** and happiness
- Children deserve the same **quality of medicines** as do adults



The Trust Deficit in (Paediatric) Health Research



A **tension** regarding what people trust
and the **reliability** of what is trusted.

Why the Trust Deficit in (Paediatric) Health Research

- The **vulnerability** of the child vs. the knowledge of the researcher
- The **interests** of science and society vs. the interests/vulnerabilities of children
- The challenge of (paediatric) **access** to **medical care** and **health research**
→ individual & societal dimensions
- The (historical?) lack of **transparency** in health research
→ secrecy = the undoing of trust



Building a European House for Paediatric Health Research

**Health Outcomes
*for Children***

Sponsors Investigators IRBs Regulators

Competence

**Independence
&
Inter-
dependence**

Efficiency

The Scientific Method & The Ethical Disposition (GCP)

Trust

Transparency, Education, Guidance & Regulation

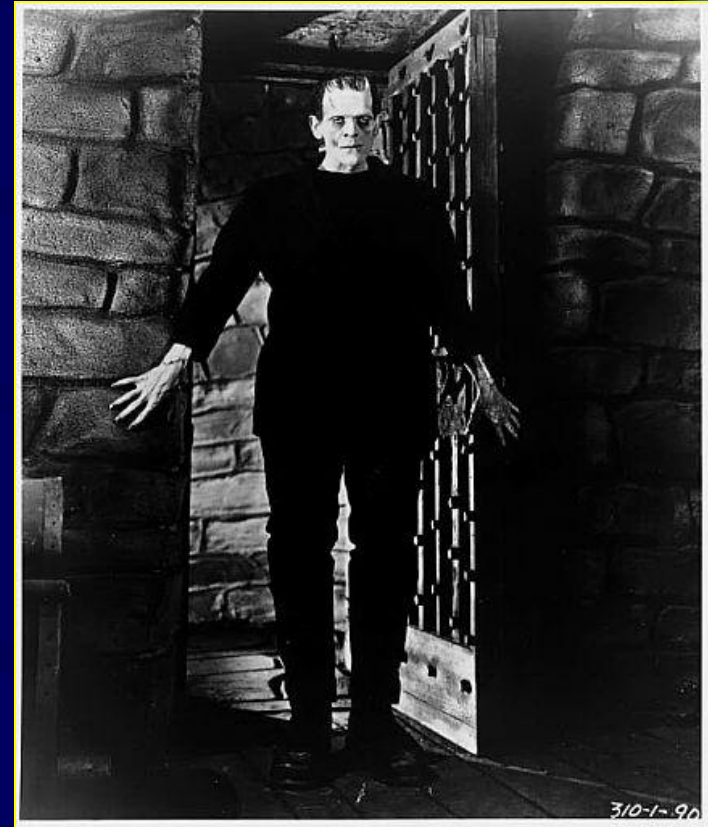
Responding to the Trust Deficit

Transparency

- an increasing **interest** by science and society to have paediatric health research studies and results published
- the need to **share knowledge** in paediatric health research
- a growing concern with **data**, its management and access
- the ongoing promulgation of **regulation** and **research ethics**
- advances in **medical publishing**: ICMJE, ISMPP
- advancing the role of clinicaltrials.gov, **Eudract**, & other registries (WHO ICTRP) for paediatric health research
- advancing the role(s) of **sponsors & researchers & ethics committees** in promoting transparency
- increasing **collaboration** between industry and academia
- developing the role of **regulatory authorities**
- developing the roles of **patients, communities, and ECs**

Making Trust Verifiable

- Shared **principles** → Common **practices**
requires operational guidance & education
- Scientific research may *never* be carried out in **secrecy**
each & every scientific experiment involving human subjects needs to be carried out in the daylight of full public knowledge
→ a culture of transparency
- A **Common Rule**
for all scientific research involving human subjects → national & transnational



UEMS-EAP WG Ethics (CESP) Guidelines for Informed Consent in Biomedical Research Involving Paediatric Populations as Research Participants (2002)

- ‘These Guidelines are intended to assist the European paediatrician in **inviting and enrolling children** in biomedical research projects by establishing appropriate informed decision and assent/consent procedures.’
- ‘These Guidelines provide **specific guidance for the paediatrician** regarding informed consent for research on children.’



EAP Ethics Working Group

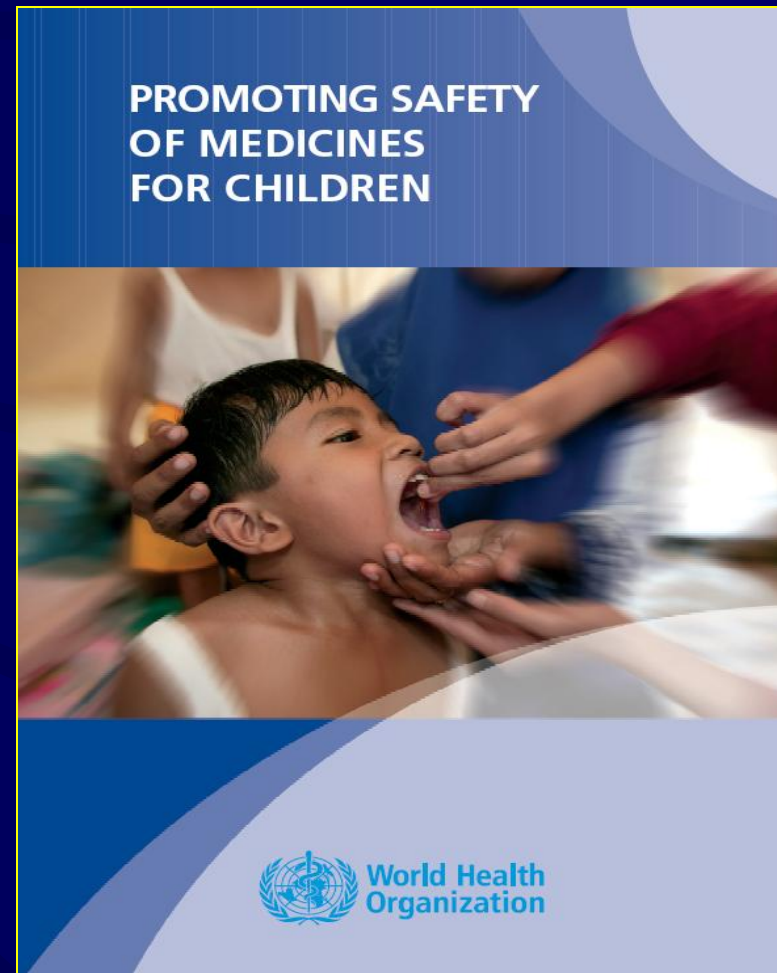
Informed Consent Principles



- Respect for the **dignity** of the child-participant
- Safe-guard the **best interests** of the child-participant
- **Protect** the child-participant from harm
- Assure and respect the **privacy** of the child-participant
- Protect the **confidentiality** of the child-participant

Transparency in Ethical Review

- Ethical review contributes to **safeguarding** . . . research participants.
- ECs should **ensure** independent, competent, and efficient practices in their ethical review procedures and decision-making processes.
- ECs are responsible for acting in the full **interests** of potential participants and concerned communities. . . .



Ethics Committees Need to Promote Transparency in EU Paediatric Health Research

- By helping science and society to understand the health **needs** of children
- By assisting government and institutions in adapting **research** to the needs of public health and patient care.
- By assisting society in creating an open **conversation** on paediatric health research.
- By insisting on **best practices** in paediatric health research
- By promoting **transparency and accountability** in paediatric health research



Quality in ethical review promotes trust in ethics committees.

- Based on an **understanding** of the **role** of ethical review in health research.
- Addresses the **expectations** for ethical review held by various parties
→ EC members, Investigators, Regulatory Authorities, Patients, Communities, Sponsors.
- Expresses an appreciation of the characteristics that contribute to **good ethical review practices**.



Building Transparency into Partnerships

European Network for Expertise for Rare Paediatric Neurological Disorders nEUroped

Objectives

1. Establishing a European network of **patients and researchers** with a common interest in rare paediatric neurological disorders
2. Establishing a **patient and research registry** for rare neurological disorders in the European Union



European Network for Alternating Hemiplegia in Childhood (ENRAH)
(Barcelona, Spain; 9 February 2006)



Transparency Empowers

Researchers empower patients.

Patients empower science and society.



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