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Mutual Respect and Shared Goals for Clinical Trials on Children

Catriona Chaplin*, David Neubauer, Falk Wulf***, Sylvia v. Mackensen***,**

Liuska Sanna** and John Eric Chaplin***

*Göteborg Pediatric Growth Research Center, Sahlgrenska Academy at University of Gothenburg,
**Department of Child, Adolescent & Developmental Neurology, University Children's Hospital, Ljubljana,
***Institute of Medical Psychology, University Medical Centre Hamburg-Eppendorf,
****European Patients Forum, Brussels

The Sahlgrenska Academy

UNIVERSITY OF GOTHENBURG

EU project: RESPECT

Relating Expectations and needS to the Participation and Empowerment of Children in clinical Trials

Aims

- to identify children's needs and expectations from clinical trials
- to gather best practice examples and suggestions for empowering parents and children to have a say in future clinical trials
- to give recommendations to all stakeholders on how to achieve outcomes that really matter to young patients

Method

- case study interviews with families participating in clinical trials
- interviews with paediatricians and nurses running trials
- interviews with trial sponsors
- surveys and workshops with patient organisations

Themes that emerged

Paediatricians reported that parents did not always understand the difference between clinical trials and regular treatment. They felt that many parents did not see the importance of medical research and did not always have an altruistic attitude.

Many of the **parents** we interviewed indeed saw the trial as a way to get the best treatment for their child (thus misunderstanding the principle of equipoise that is fundamental to clinical trials). Both children and parents were surprised to find that some of the procedures were painful.

However, other parents saw themselves as research partners in the trial and wanted to feel that their input was valued, although they did not always feel that their contribution was appreciated sufficiently. They wanted to receive the study results but several paediatricians confirmed that they do not inform parents about the results unless specifically asked.

Several **patient organisations** reported that they were ill-equipped to give input on which trials and which outcomes have the highest priority for their members. This is something that could be improved through surveys of their members as a basis for contact with pharma companies.

We found good examples of trials where patients felt empowered to make valuable contributions. We also found patient organisations giving input into trial design and protocols, helping to define outcome measures and to recruit trial participants, as well as **pharma companies** making their trial results accessible to all via their website.

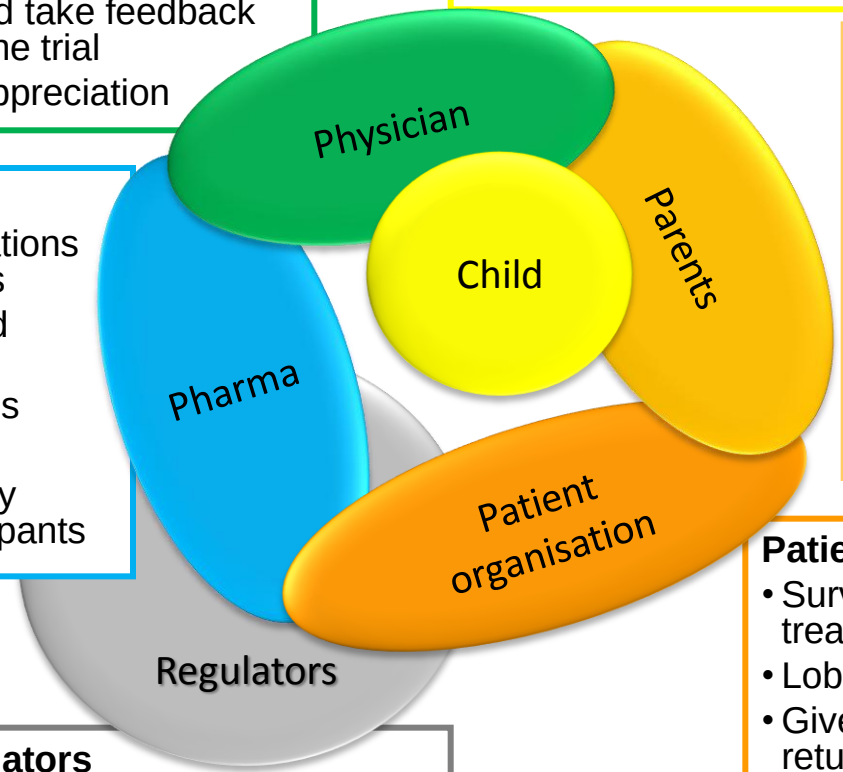
Good practice examples: fostering mutual respect

Physician
- Inform pharma of unmet needs that families report
- Review trial protocol for endpoints that matter
- Be honest about pain
- Give and take feedback during the trial
- Show appreciation

Child
- is the best judge of their own pain and distress
- Child panels:
  - ➢ can indicate which outcomes really matter to them
  - ➢ can review informed consent materials
  - ➢ can tell others what it is like to be in a trial

Parents
- Need education about:
  - ➢ trials vs. treatment
  - ➢ randomisation
- Want to be partners in the trial:
  - ➢ internet search for trial details or potential trials
  - ➢ make sacrifices (e.g. time off work) for the trial
  - ➢ expect to get study results

Pharma
- Consult patient organisations on unmet medical needs
- Develop patient-reported outcome measures
- Less invasive procedures
- Reduce inconvenience
- Make study results easily accessible to trial participants



Patient organisation
- Survey members for unmet treatment needs
- Lobby pharma companies
- Give input on the trial design in return for recruitment help
  - ➢ relevance for their members
  - ➢ outcome measures
- Support families looking for trials

Regulators
- Include patient organisation representatives on committees

Clinical implications

- Physicians are in a unique position to inform trial sponsors about the child's unmet medical needs and to influence which trials are conducted.

- It is important to ensure that families understand the need for research and are respected for their valuable contribution to the improved safety of medicines for children.

For additional information, please contact:

Catriona Chaplin, PhD
catriona.chaplin@vgregion.se

Göteborg Pediatric Growth Research Center
The Sahlgrenska Academy at University of Gothenburg
Institute of Clinical Sciences
Department of Pediatrics
The Queen Silvia Children's Hospital
416 85 Göteborg, Sweden

Project website: www.patientneeds.eu
email: respect@patientneeds.eu