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RESPECT: Good practice in empowering families in clinical trials

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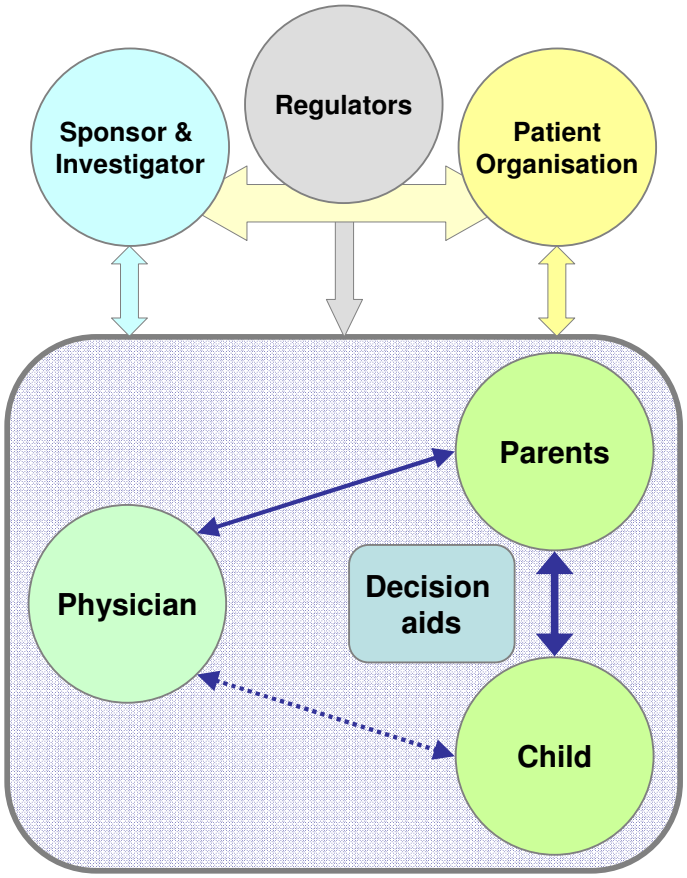
Background: The EC Paediatric Regulation requires increased participation of children in clinical trials in order to reduce off-label use of medicines. An exploration of the needs of these young patients and their parents is the basis of the European RESPECT project (**R**elating **E**xpectations and need**S** to the **P**articipation and **E**mpowerment of **C**hildren in clinical **T**rials).

Aim: To identify best practice examples and give recommendations.

Method: Through case study interviews with families participating in clinical trials and interviews with paediatricians and nurses running trials, as well as with trial sponsors, we explored their perspectives on medical research. We also ran surveys and a workshop with patient organisations to gather their insights into the support needs of children and parents and how to turn these needs into good practice for the conduct of clinical trials.

Results: We identified different actors in the CT process and found examples of good practice from each of them. They also gave concrete suggestions of what they wanted from each other. Some of these examples are shown in this diagram.

Sponsor & Investigator
- Consult patient organisations and physicians for unmet medical needs
- Develop patient-reported outcomes
- Minimise invasive procedures
- Reduce inconvenience
- Provide the results of the study to the participating families.



Patient Organisation
- Survey members for unmet treatment needs and outcome priorities for young patients
- Lobby pharma companies
- Assess the relevance of a trial for their members before offering recruitment help.
- Give input on the trial design and protocol
- Support families participating in trials

Parents
- Education helps them to distinguish trials from treatment and to see the importance of randomised clinical trials
- Many want to be partners in the trial
  - they research potential trials and details of a particular trial if asked to participate.
  - give detailed observations during the trial
  - expect to get the results of the study

Physician
- Inform pharma of the unmet needs that families actually report in consultations
- Review the trial protocol for endpoints that matter to young patients
- Empower families to ask and discuss if they feel uncertain
- Give and take feedback from families before, during and after the trial.
- Be honest and gain their trust
- Show appreciation for their contribution
- Report back their comments to the sponsor after the trial

Decision aids
- Decision aids help the family to
  - become aware of their attitudes, knowledge and emotions concerning participation
  - learn about clinical trials and form an opinion
  - identify further information and support needs
  - secure two-way communication with the clinical staff

Child
- Should be consulted as the best judge of their own pain and distress
- Even small children can indicate which outcomes really matter to them
- Child panel can review informed consent materials
- Children can act as 'ambassadors' and tell others how it is to be in a trial

Conclusions

These best practice examples and recommendations should be combined into a single process supported by regulators.

Empowering parents and children to have a say in the design and execution of the trial will motivate their participation and increases the likelihood of achieving outcomes that really matter to patients.