Young Person´s Advisory Groups enhancing clinical trials

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Our mission

Contribute to the process to ensure the **paediatric patient-centricity** of any medicine or medical devices addressed to the children and young people.

Facilitate the **involvement** of young people and families with an **ethical approach** that will guarantee their rights in the health field.
The 4 Qs

✓ WHY?
✓ WHO?
✓ WHEN?
✓ HOW?
WHY?

- Health Care Providers
  - Clinical Research
  - Real world data

- Basic Research
  - Elucidate mechanisms of disease
  - Discover disease pathways
  - Discover biomarkers

- Regulatory entities
  - Provide a framework of ethical and scientific investigation to advance patient care
  - Assess and approve new medicines
  - Protect the public health

- Pharmaceutical Development
  - Create novel molecules
  - Develop and conduct clinical trials research
  - Validate basic research hypothesis
  - Generate clinical data for further research

Source: Doi: 10.1016/J.JID.2020.01.007  Accessed: April, 2022
The 4 Qs

- WHY?
- WHO?
- WHEN?
- HOW?

- Young patients
- Parents
- Young Person’s Advisory Groups
- National and international perspective
eYPAGnet

+ 30 YPAGs across Europe

- Belgium
- Czech Republic
- Denmark
- Finland
- France
- Ireland
- Italy
- Netherlands
- Poland
- Portugal
- Spain
- UK
Ad hoc groups of patients and parents:

- All therapeutic areas in paediatrics and pregnant women
- Some therapeutic areas in adults
- Involving patients from different regions of the world
The 4 Qs

✓ WHY?
✓ WHO?
✓ WHEN?
✓ HOW?

WHY INVOLVE CHILDREN AND YOUNG PEOPLE?

AWARENESS  DESIGN  RECRUIT  CONSENT  RETAIN  FOLLOW UP

UNMET MEDICAL NEEDS

REVIEW OF PROTOCOLS

TRIAL STEERING COMMITTEE
DATA & SAFETY MONITORING COMMITTEE

“Adapt the clinical trial to the patients, no the patients to the clinical trial”
Co-design of protocols. Why is important involving patients?

- 57% of protocols can have at least one substantial amendment.
- 45% of these amendments were deemed “avoidable”.
- Phase II and III protocols: mean of 2.2 and 2.3 global amendments.
- Global amendments means longer recruitment time.

Think in terms of patient experience...

- Recommendations in terms of feasibility, study visits, schedules of procedures
- Involve patients allows think beyond the disease factors: **patient experience**
- Put the patient in the heart of your project

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Benefits of involving paediatric patients

ROE
WHEN?

Co-design of protocols. Why is important involving patients?

- **Assessment** of clinical trial applications
- Article 9.3 “…At least one layperson shall participate in the assessment…”

- **Trial design** as described in the protocol
- Annex I, Application dossier, Protocol 17 (e) “...where patients were involved in the design of the clinical trial, a description of their involvement…”
WHEN?

Lay summaries: patients involvement is required.

European Regulation EU N° 536/2014

- Article 37 requires trial sponsors to submit a “lay summary” that is understandable to laypersons.

- The LS must be submitted to CTIS via the EU Portal no later than 12 months from the protocol-defined end of the clinical trial:
  - 6 months for paediatric studies, and up to 30 months
  - for non-therapeutic Phase 1 trials.
The 4 Qs

✓ WHY?
✓ WHO?
✓ WHEN?
✓ HOW?

- Focus group
- Questionnaires
- Interviews
- Patient journey
- Advisory boards
- Steering committees
- Simulation

- F2F - virtual - blended
More examples

Patients’ information and education resources

- Assent form for paediatric patients
- Consent form
- Patient Diary
- Patients training in clinical trials
- Others
More examples

Better medicines for babies, children and young people through a pan-European clinical trial network
The Young Person Advisory Group (YPAG) of Sant Joan de Déu Hospital (Barcelona-Spain) has written some guidelines with a collection of recommendations to be considered by the sponsors of the clinical trials (drug companies, researchers, etc.) and for the ethics committees of the research centres, with the aim that the informed consent document (or assent document in the case of paediatric patients) be fully adapted to the children’s necessities of information.

The participation of a child in a clinical trial must be voluntary, and for this reason his/her consent is necessary from a certain age. This is an altruistic act that contributes to the advancement of science in the treatment of a specific disease.

It must also be ensured that the child or young patient knows his/her rights, what will happen during participation in the study and the important contribution of clinical trials for medical science.

Recommendations on the content
- Concise content.
- Use easy to understand vocabulary.
- Include a general explanation of what a clinical trial is.
- Easy understandable explanation of the possible side effects of the drug under study.
- Include a glossary with definitions of the most difficult words to understand.
- Detail the basic contact data of:
  - Principal investigator (PI)
  - Hospital responsible

Recommendations on the format
- Length from 2 to 5 pages.
- Font size between 12 and 14 points.
- Always address the child in the second person singular.
- Do not use the term “subject”.
- Use the term “patient” or “boy / girl”.
- Use colour, drawings, photos or infographics to facilitate the understanding of the information.

CLINICAL TRIAL GUIDE

A CLINICAL TRIAL GUIDE which can be easily used by children, that can be carried, may facilitate their participation.

- Include a calendar
- A space for taking notes
- A glossary with scientific terms
- With more visual than written content

These recommendations have the approval of the Ethics and Research Committee of Hospital Sant Joan de Déu.
More examples
More examples
More examples

YEAH
Youngsters Engagement in Health

DESIGNING RESEARCH PROJECTS WITH CHILDREN AND YOUNG CITIZENS

QUALITY OF LIFE AND PROMS

CO-CREATING HEALTH PROTOCOLS WITH YOUNG CITIZENS

RESEARCH INFORMATION FOR YOUNG PATIENTS
Patient involvement: quantitative approach

Benefits of involving paediatric patients

INVESTMENT $100K

ENPV $50M

ENPV (Expected Net Present Value)

Importance of diversity

Children and young people have the right to **freely express their views** (CRC art. 12), the right to the highest attainable standard of health and to facilities for the treatment of illness and rehabilitation of health (including **research**).

We spend a lot of time designing the bridge, but not enough time thinking about the people who are crossing it”.

Dr. Singh, Director of System Design at the Earth Institute
Nothing for the patients, without us!

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