Young Person´s Advisory Groups enhancing clinical trials

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Sant Joan de Déu Children’s Hospital (Spain)

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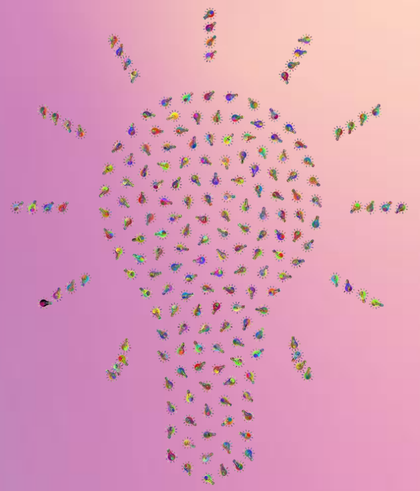
Our mission

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?

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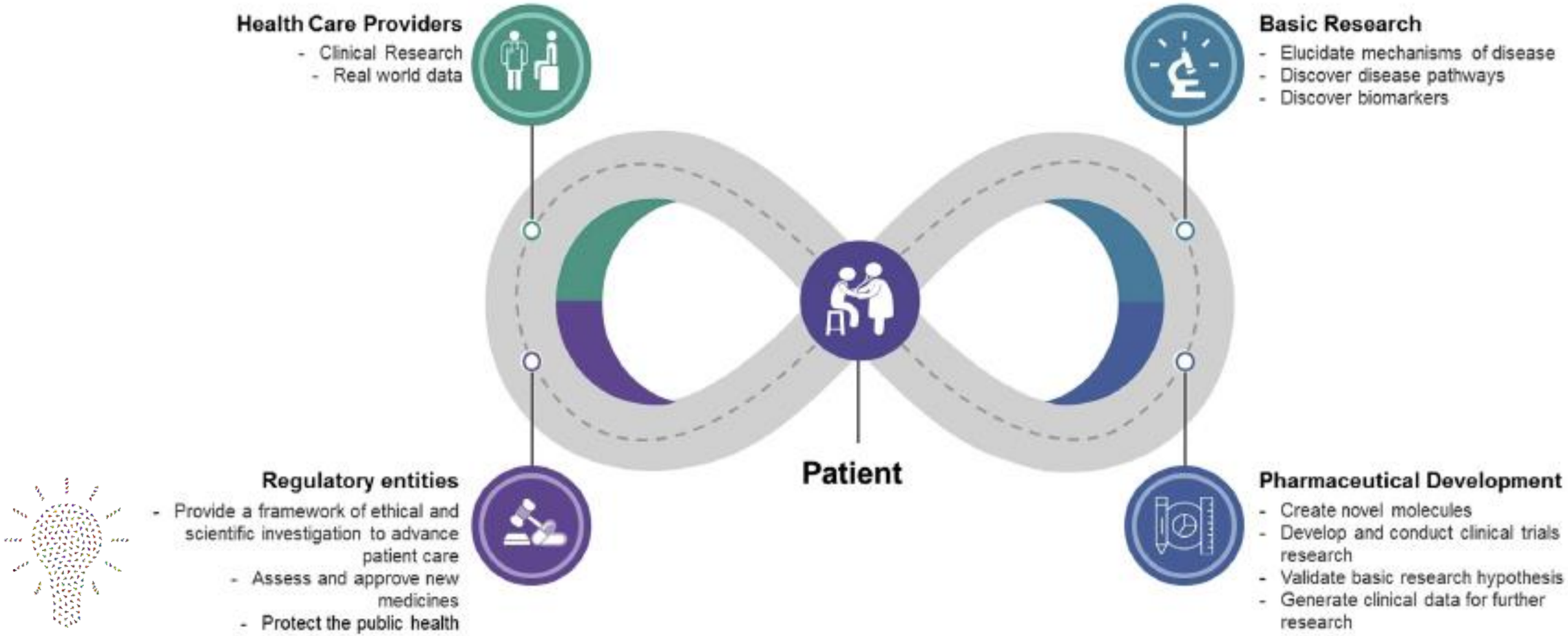
Patient Engagement in Research Area



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

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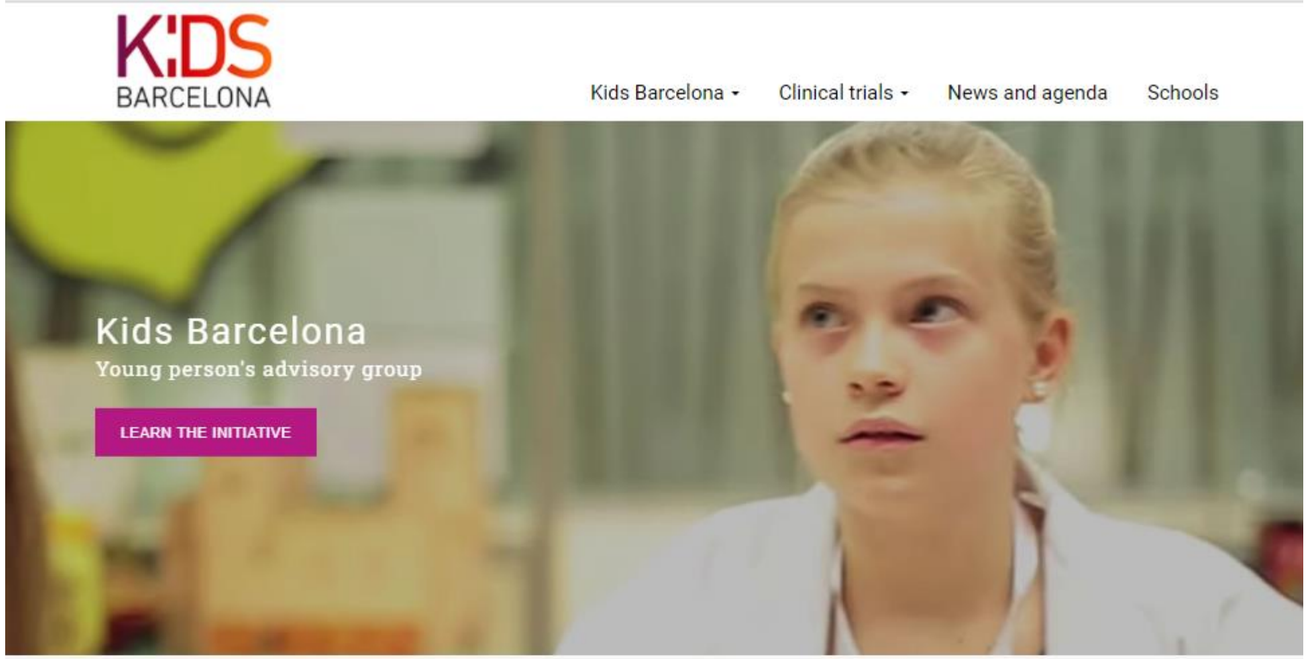
Fundació de Recerca

Patient Engagement
in Research Area

KIDS
BARCELONA



Clinical trials

What is a clinical trial?

From 8 to 12 years old

Why are clinical trials needed?

From 13 to 18 years old

Why are clinical trials necessary in children?

From 8 to 12 years old

Where can you find information about current clinical trials?

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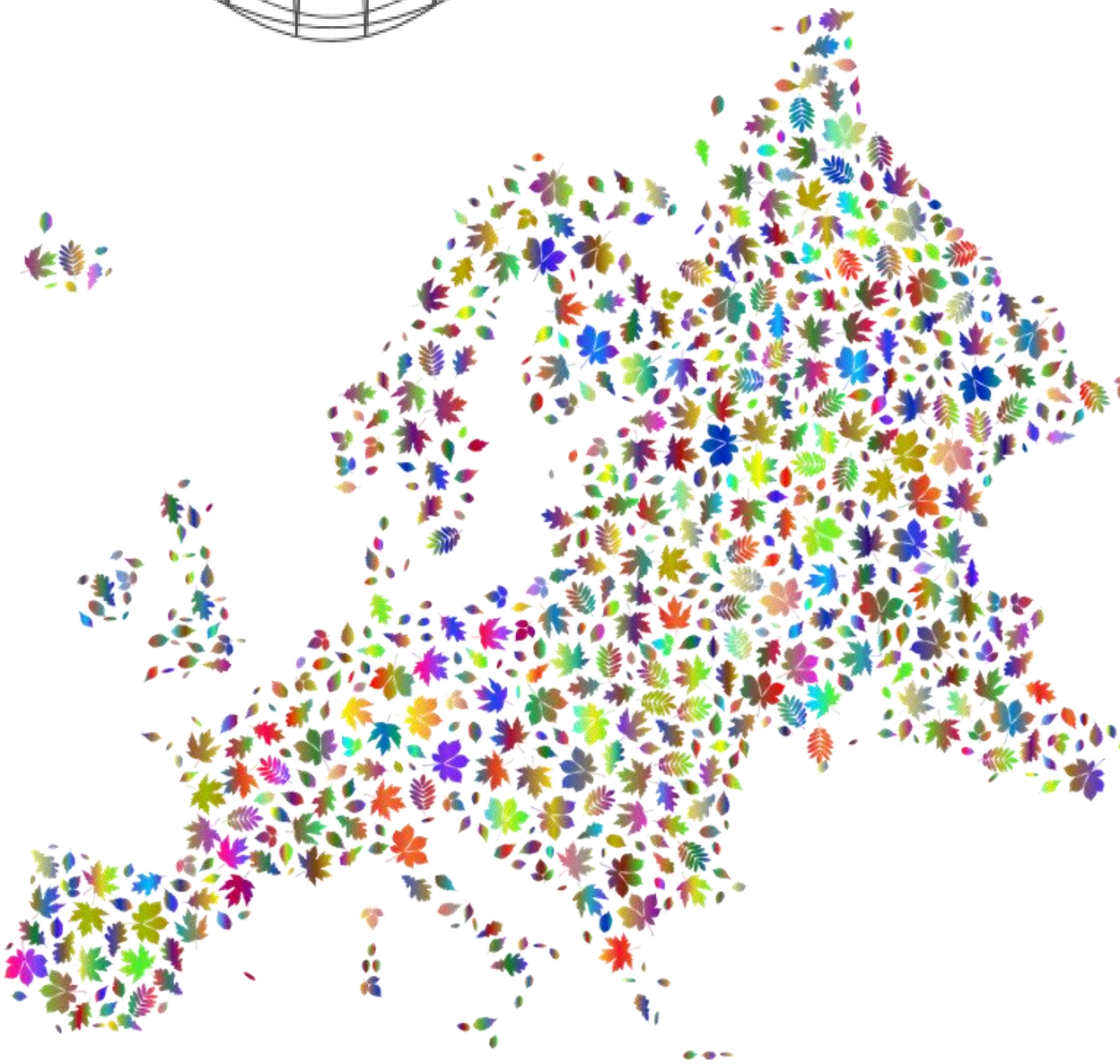
Patient Engagement in Research Area

eYPAGnet

+ 30 YPAGs accross Europe

- Belgium
- Czech Republic
- Denmark
- Finland
- France
- Ireland
- Italy
- Netherlands
- Poland
- Portugal
- Spain
- UK



EnprEMA
European network of paediatric research at the European Medicines Agency

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Patient Engagement in Research Area

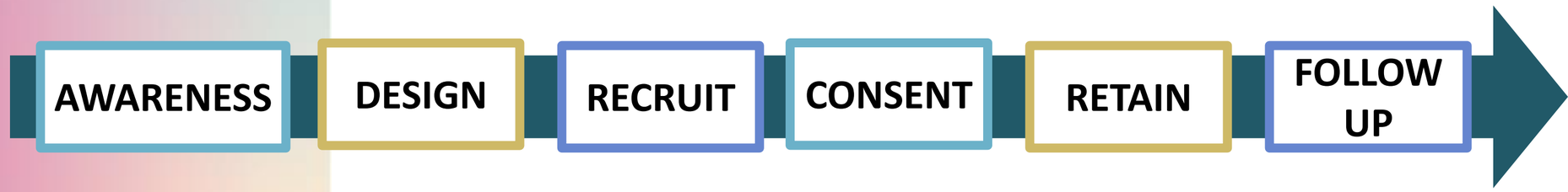
Ad hoc groups of patients and parents:

- 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?



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"

✓ WHEN?

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Patient Engagement in Research Area

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.

Source: DOI: 10.1177/2168479016632271 Accessed: April, 2022

Benefits of involving paediatric patients

ROE

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

✓ WHEN?

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

- **Asessment** 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?
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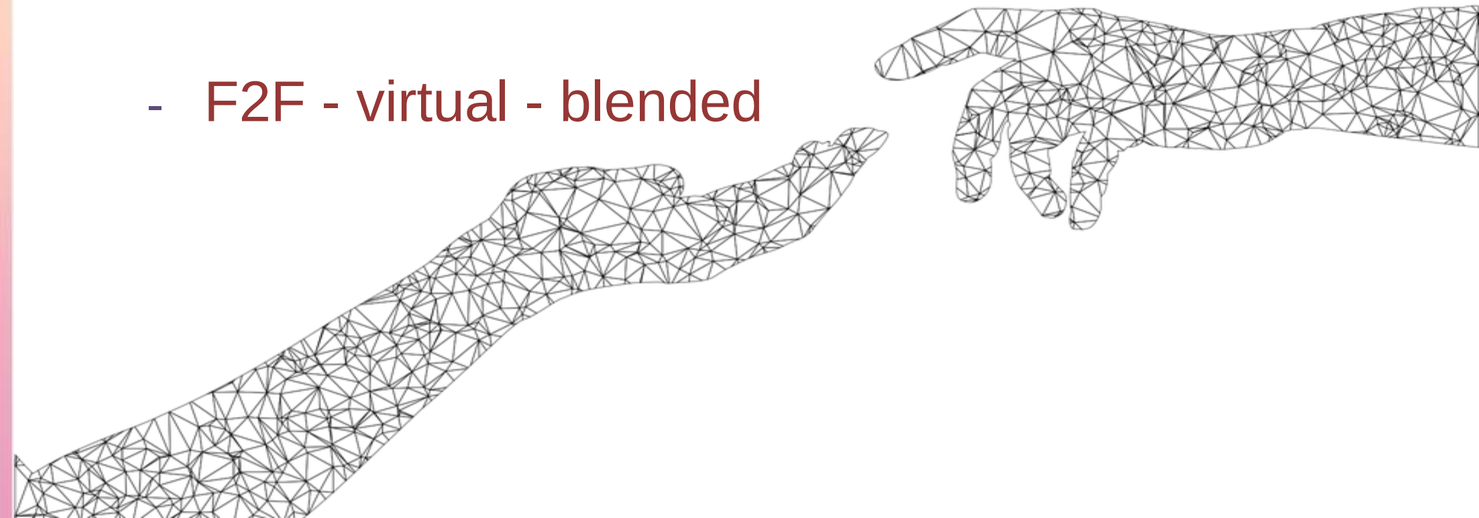
- ❑ Focus group
- ❑ Questionnaires
- ❑ Interviews
- ❑ Patient journey
- ❑ Advisory boards
- ❑ Steering committees
- ❑ Simulation

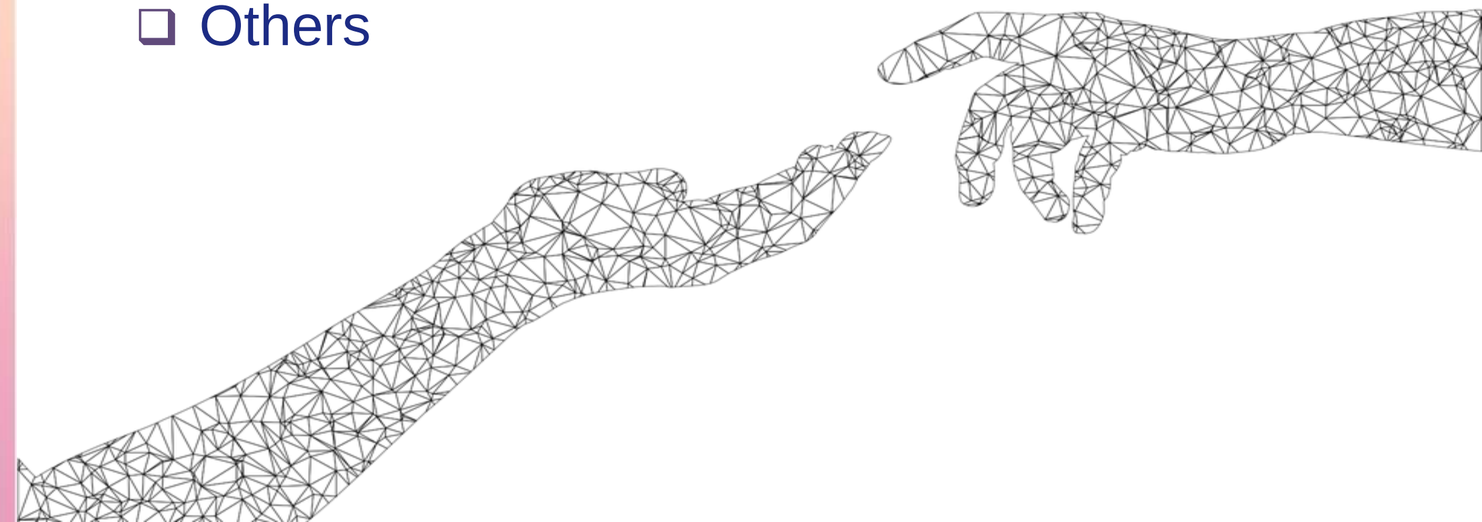
- F2F - virtual - blended



More examples

Patients' information and education resources

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



More examples

More examples

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Patient Engagement in Research Area

The **Young Person Advisory Group** (YPAG), Kids Barcelona, 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
- Describe what to do in case of emergency, who to connect, how to proceed.
- Audio-visual resources, such as a video or cartoon can make it easier to understand the purpose of the study.

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.

agencia española de medicamentos y productos sanitarios

More examples

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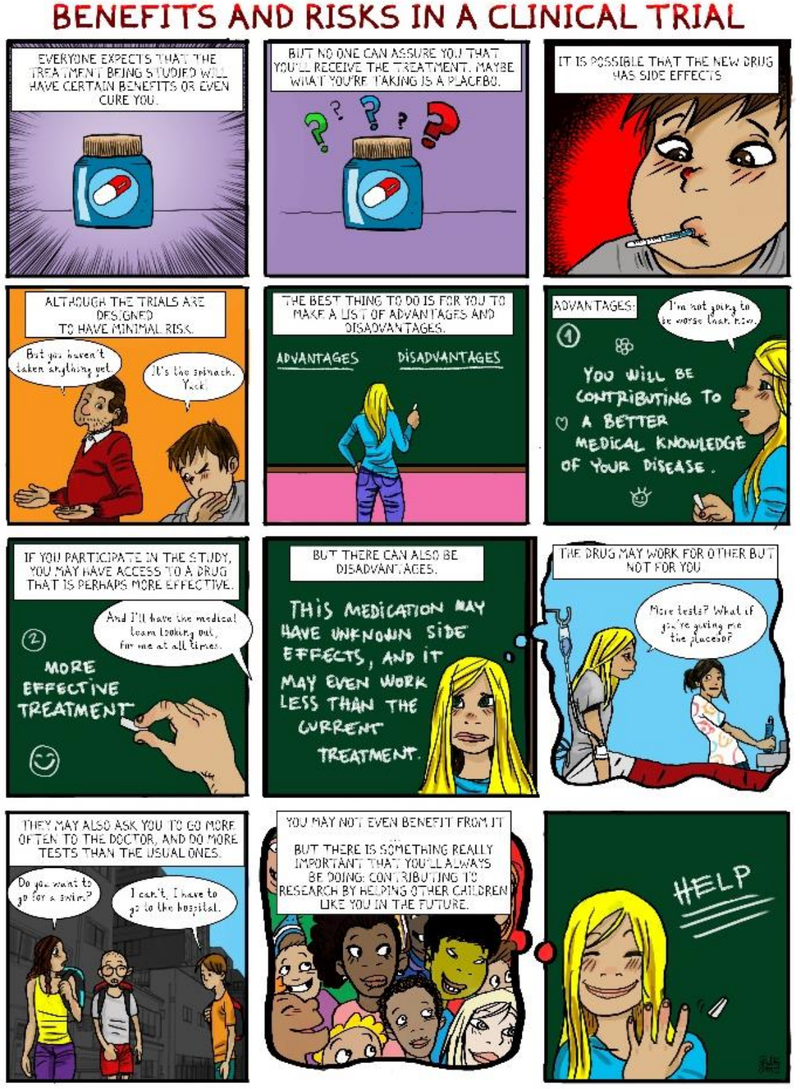

More examples

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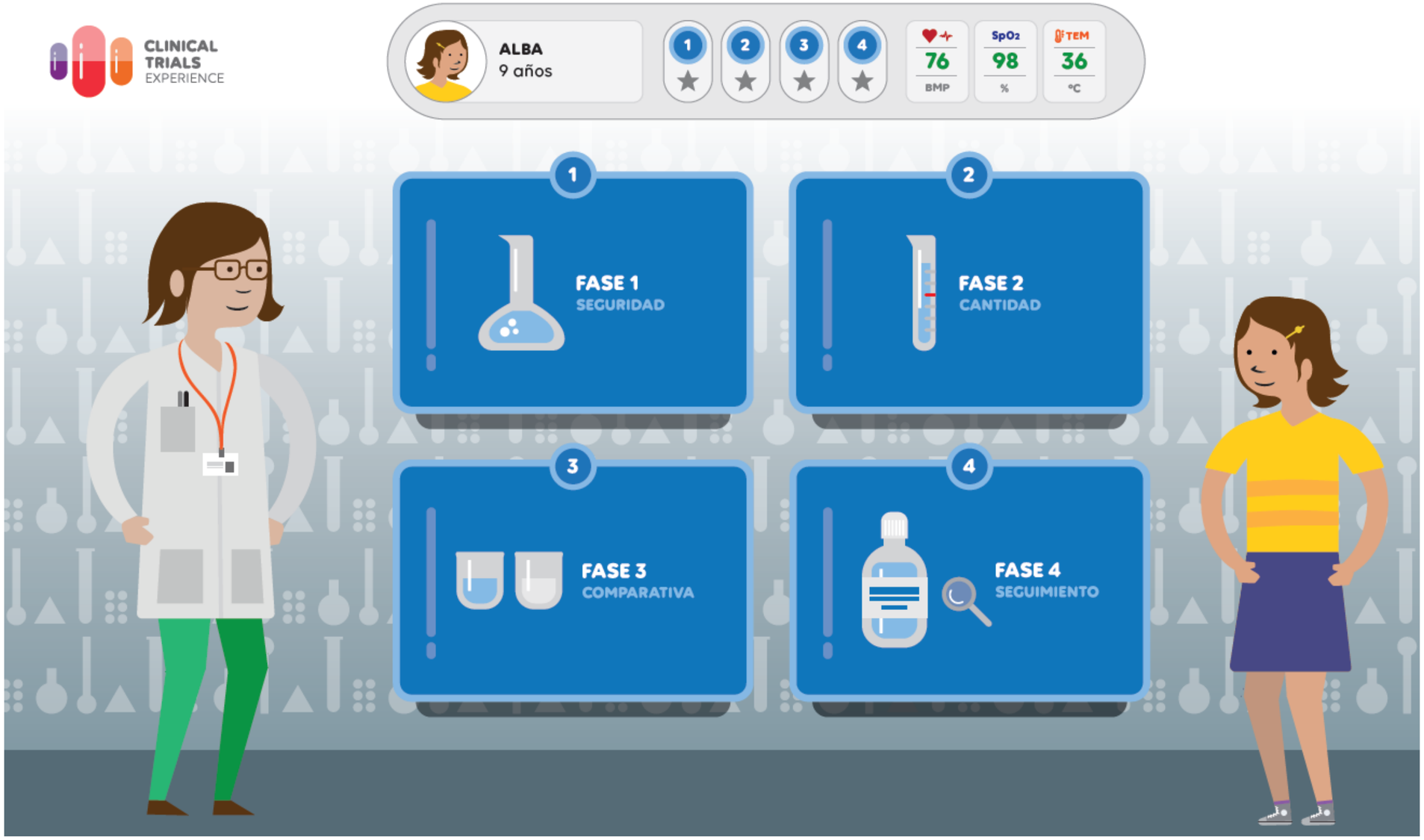

More examples

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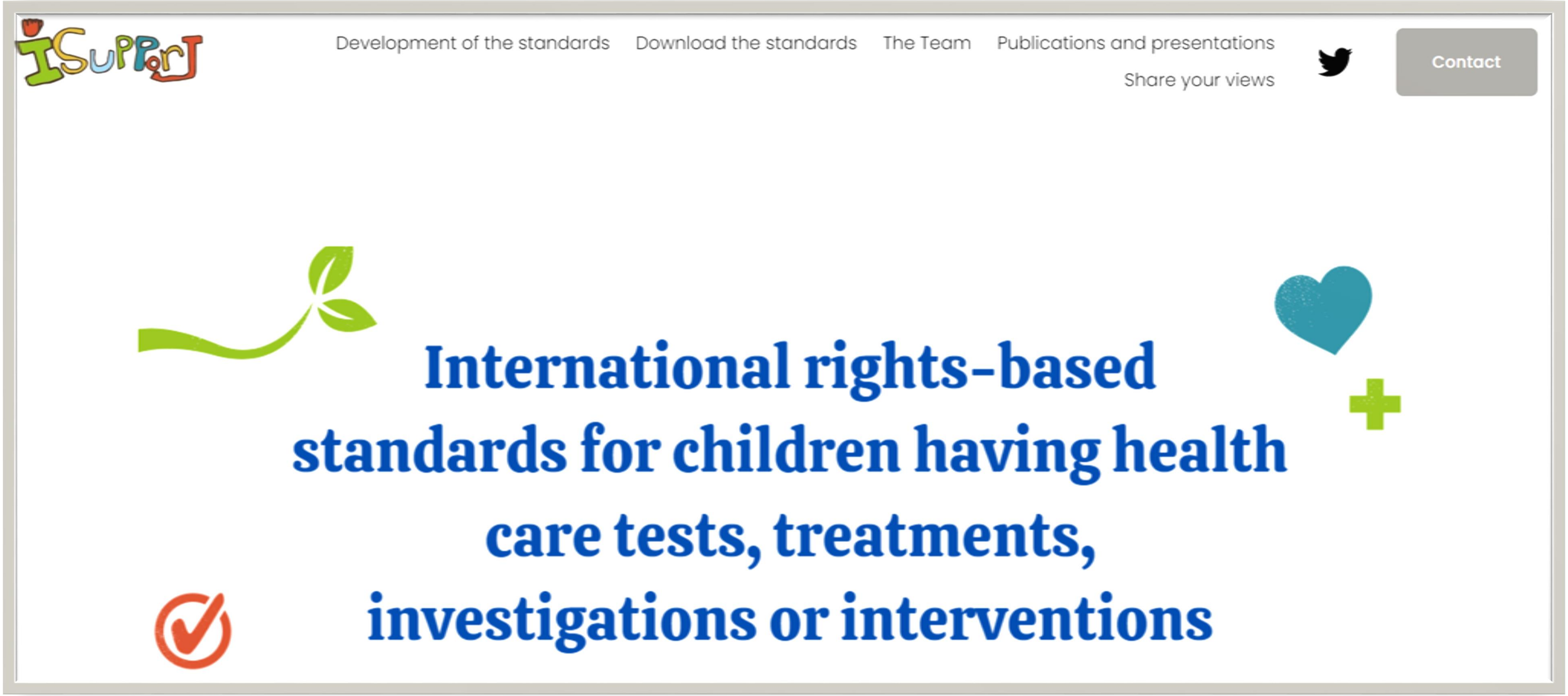


Patient involvement: quantitative approach

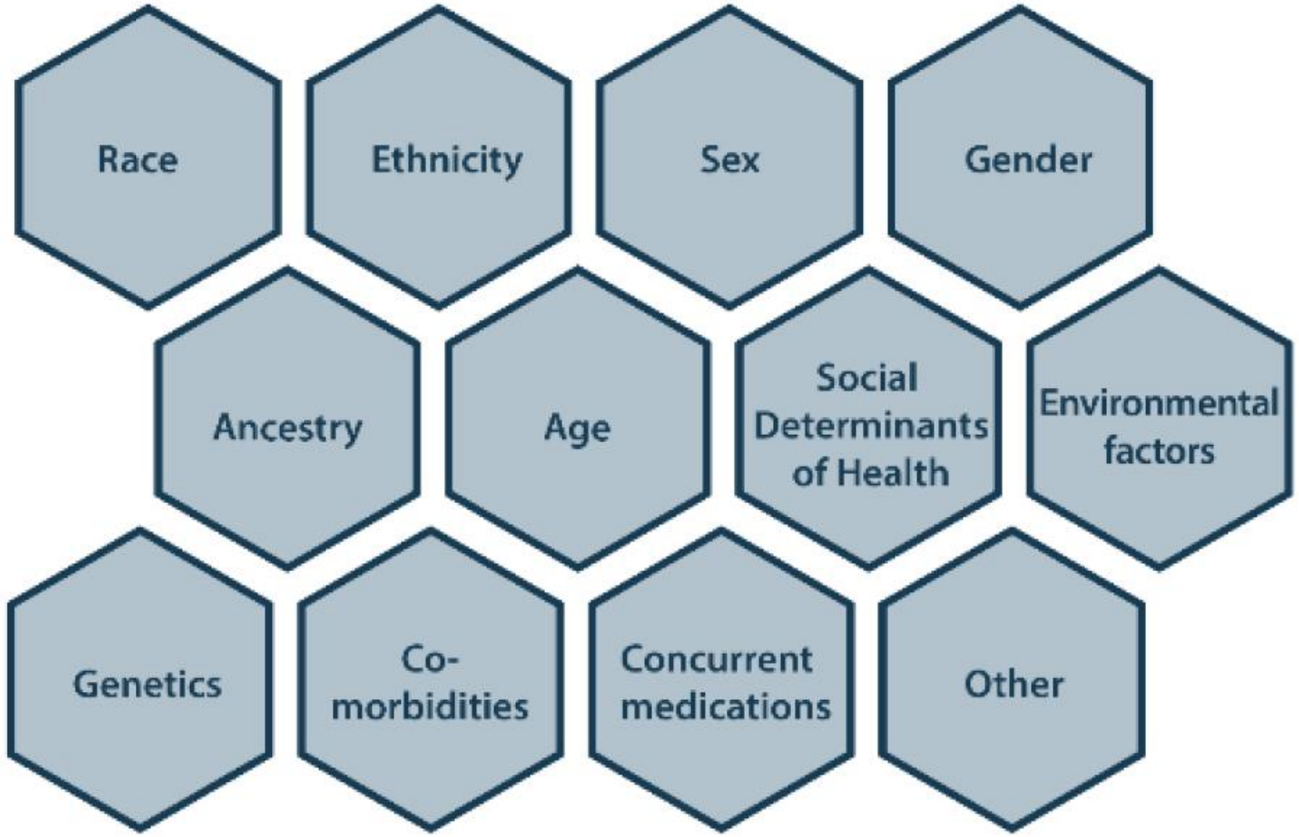
ENPV (Expected Net Present Value)

Source: Levitan B, Getz K, Eisenstein EL et al. Assessing the financial value of patient engagement: a quantitative approach from CTTI's patient groups and clinical trials project. Ther Innov Regul Sci 2018

Benefits of involving paediatric patients

ROI

Importance of diversity



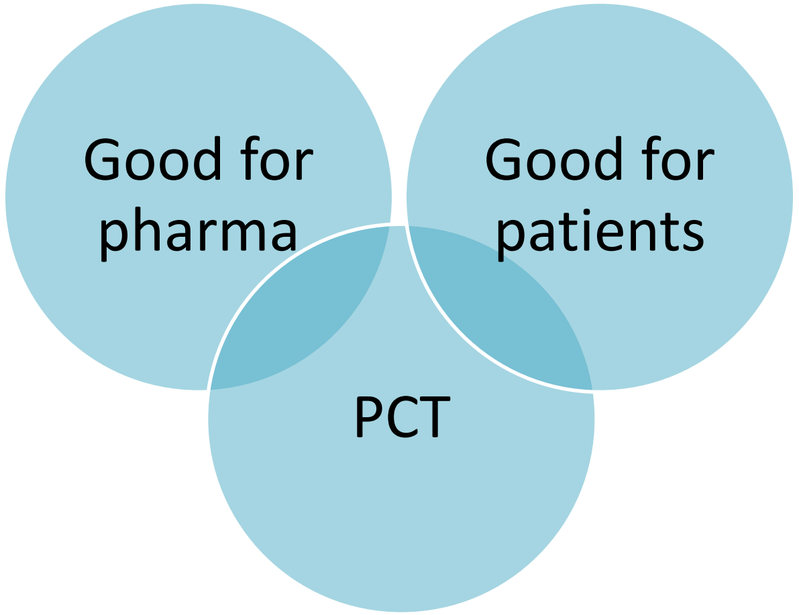
Source: Bierer B.E., White S.A., Meloney L.G., Ahmed H.R., Strauss D.H., Clark L.T., (2021). Achieving Diversity, Inclusion, and Equity in Clinical Research Guidance Document Version 1.2

Rigth to Science and Research

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**).

Convention of the Rights of the Child- 1989 – United Nations

Real value of patients involvement



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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