

Children and Young Patients and family Involvement: Why? and How?

Patient Organisation Workshop
Train the trainers
16 & 17 September 2020, online



Introduction to the presenters



Senior Patient & Public
Involvement Manager



Patient Engagement in Research
Coordinator



Senior Project Manager for
Maternal and Newborn Health



Pre-training material

Article about PPI:

- Preston, J., Stones, S.R., Davies, H. & Philips, B. (2019). How to involve children and young people in what is, after all, their research. Archives of Disease in Childhood 104(5): 494-500. Doi: 10.1136/archdischild-2018-315118.

Case study for the discussion:

- What's it all about (synopsis of a paediatric clinical trial for children and young people with asthma).



“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

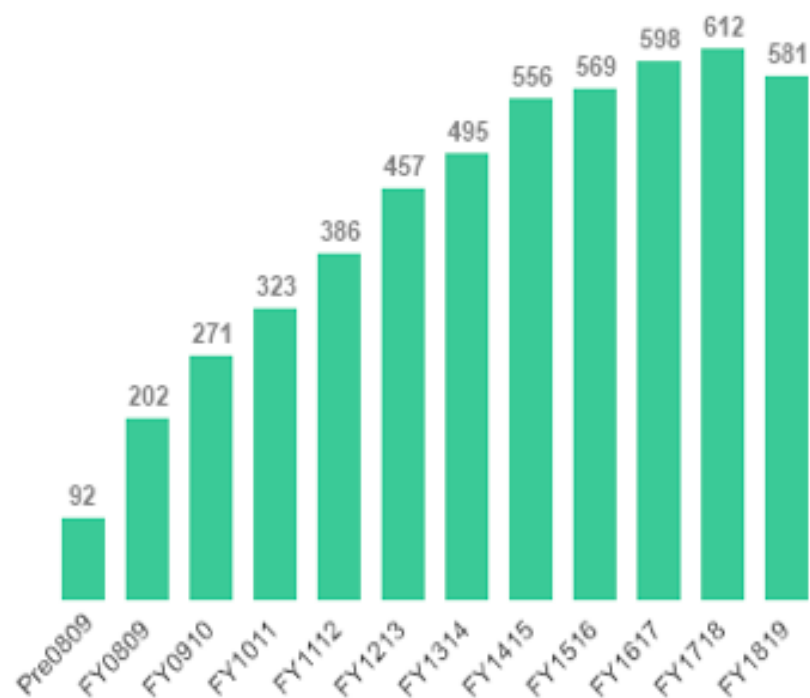




50%

90%

Studies Recruiting in Each FY (Anywhere in UK)



State of Paediatric Medicines in the EU

10 years of the EU
Paediatric Regulation

Report from the Commission
to the European Parliament
and the Council

COM (2017) 626

Clinical Trials: The Nation's Perspective



National Institute for
Health Research

Common misconceptions about clinical trials

2/3

of adults think
that you have to
be invited to
participate in a
clinical trial



58%

incorrectly think that
children are not
allowed to
participate in
clinical trials



38%

think all clinical
trials involve
testing a new drug



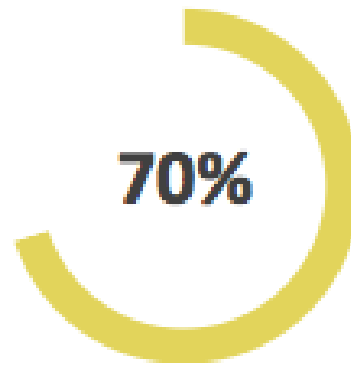
4 in 10

do not realise that
most hospitals in this
country undertake
clinical trials

Challenges of clinical trials in children



**delayed due to patient
recruitment**



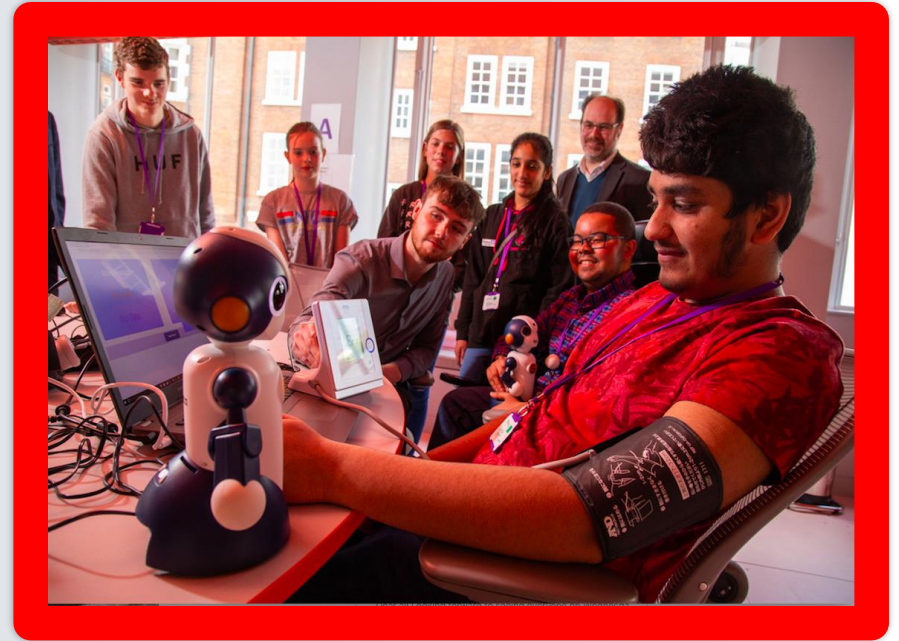
**delayed by at least one
month**



**failing to retain enough
patients**

Source: Clinical trials and their patients: The rising costs and how to stem the loss, 2016.

Involving patients, carers and the public, who have personal experience of or an interest in a health condition(s), in research or research related activities



An active partnership between patients and the public and researchers, doctors and other NHS staff

Keep in mind this generic definitions of key concepts...

ENGAGEMENT

where information and knowledge about research is provided and disseminated.

INVOLVEMENT

where members of the public are actively involved in research projects and in research organizations.

PARTICIPATION

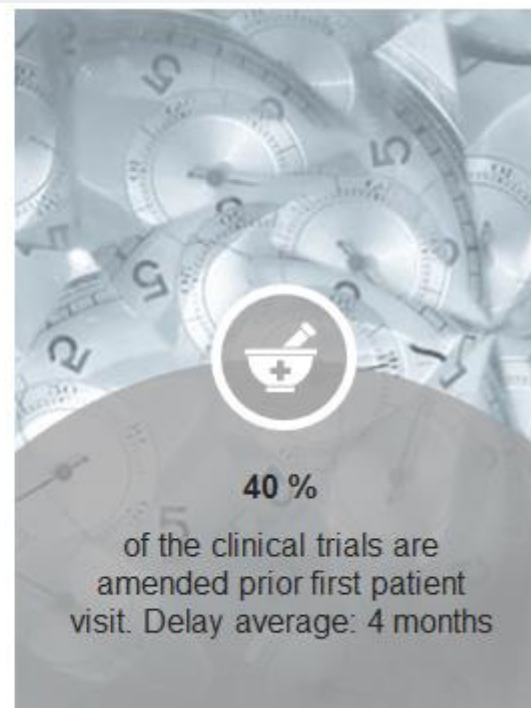
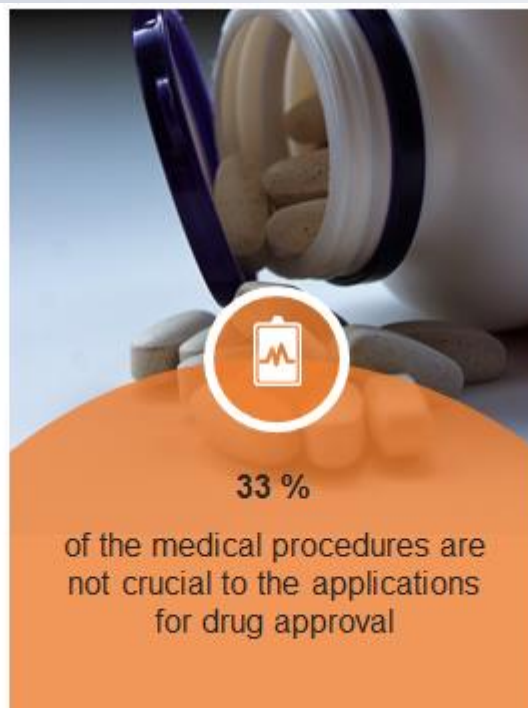
where people take part in a research study.

ADVOCACY



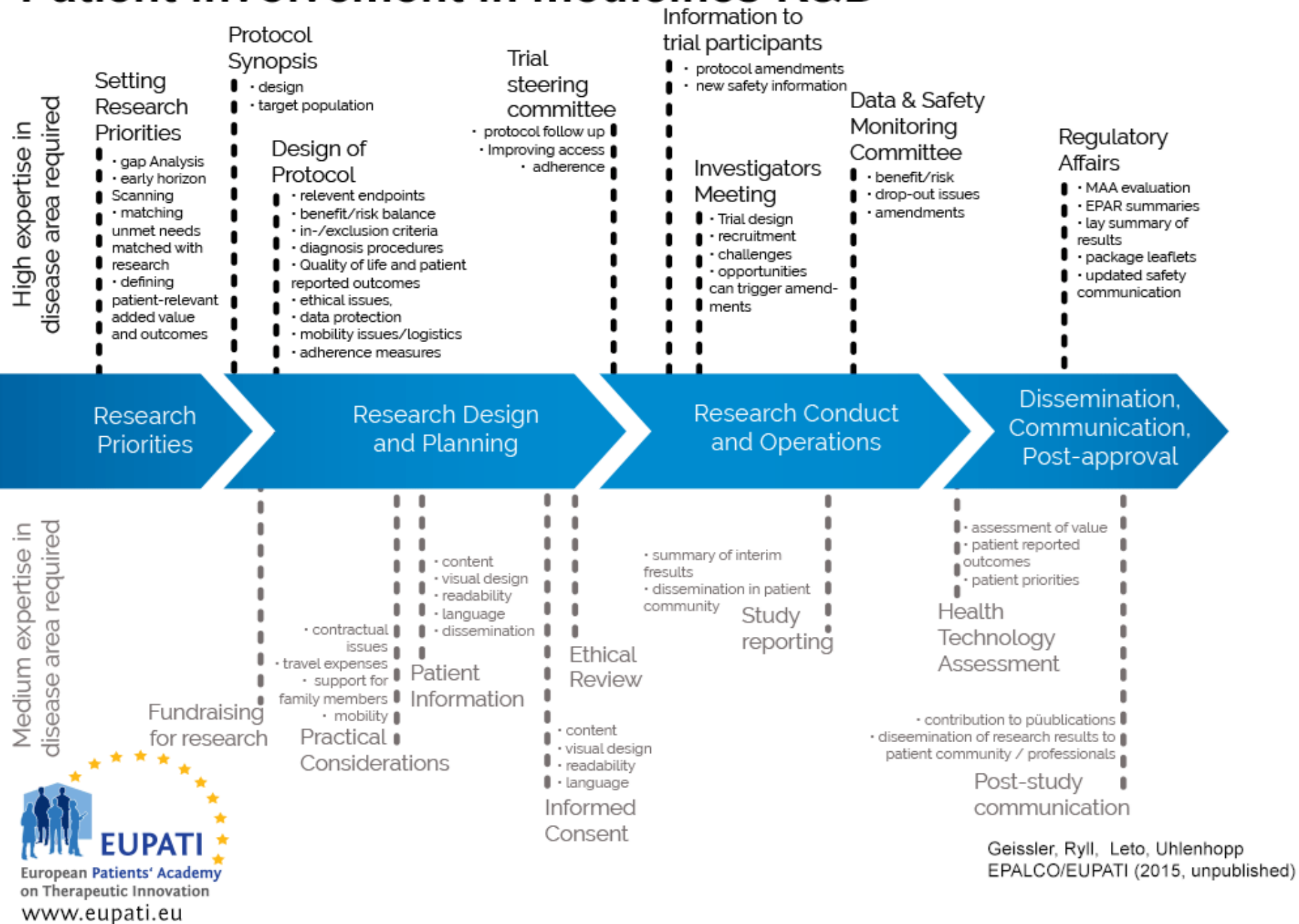
Why involve children and young people in clinical trials?





Source: Getz K.A., Zuckerman R., Cropp A.B., Hindle A.L., Krauss R., Kaitlin K.I. Measuring the incidence, causes, and repercussions of protocol amendments. Drug Inf. J. 2011;45:265–275.

Patient involvement in medicines R&D



How to involve children and young people in clinical trials?

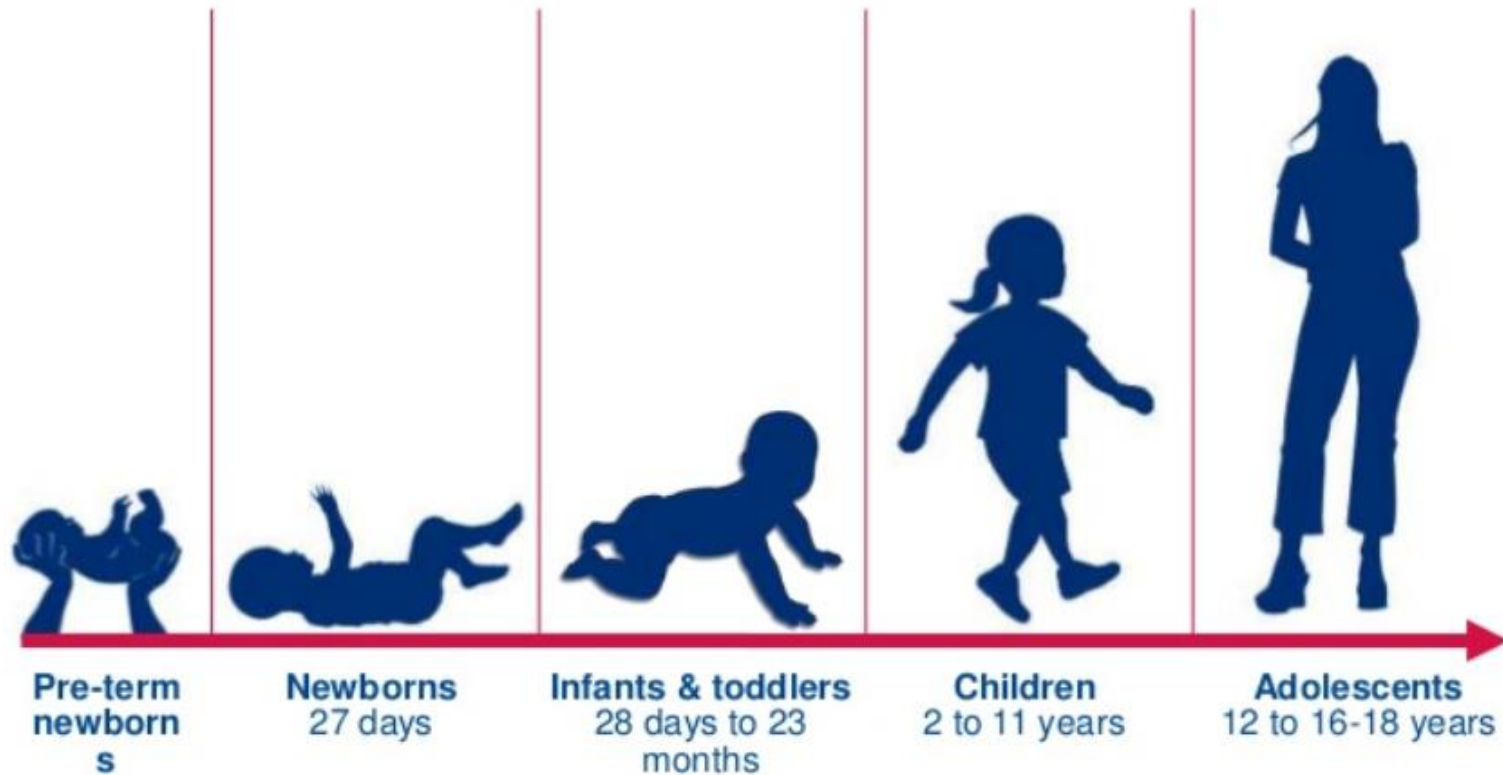
How...?

Before you answer this question think about the **patients:**

- Vulnerable group of population
- Ethics principles
- Risk/benefit
- Rights of the children:
 - To be heard
 - To good quality health care to stay healthy
 - To research

How...?

Before you answer this question think about the **patients**:



How...?

Before you answer this question think about the **patients**:



What does involving paediatric patients mean?

- Different **ages**, from preterm newborn babies to teenagers.
- Different **conditions**.
- **Legal consent age** different across the different European countries.
- Always the role of the **parents/legal guardians**.
- **Need of targeted treatment** for many conditions.
Need of the use of **placebo**.

How...?

- The best **methodology** needs to be considered to achieve a meaningful involvement
- Every project is different: age, country, disease, protocol design, etc...
- Take your time to **design, execute** and **evaluate** the PE activity

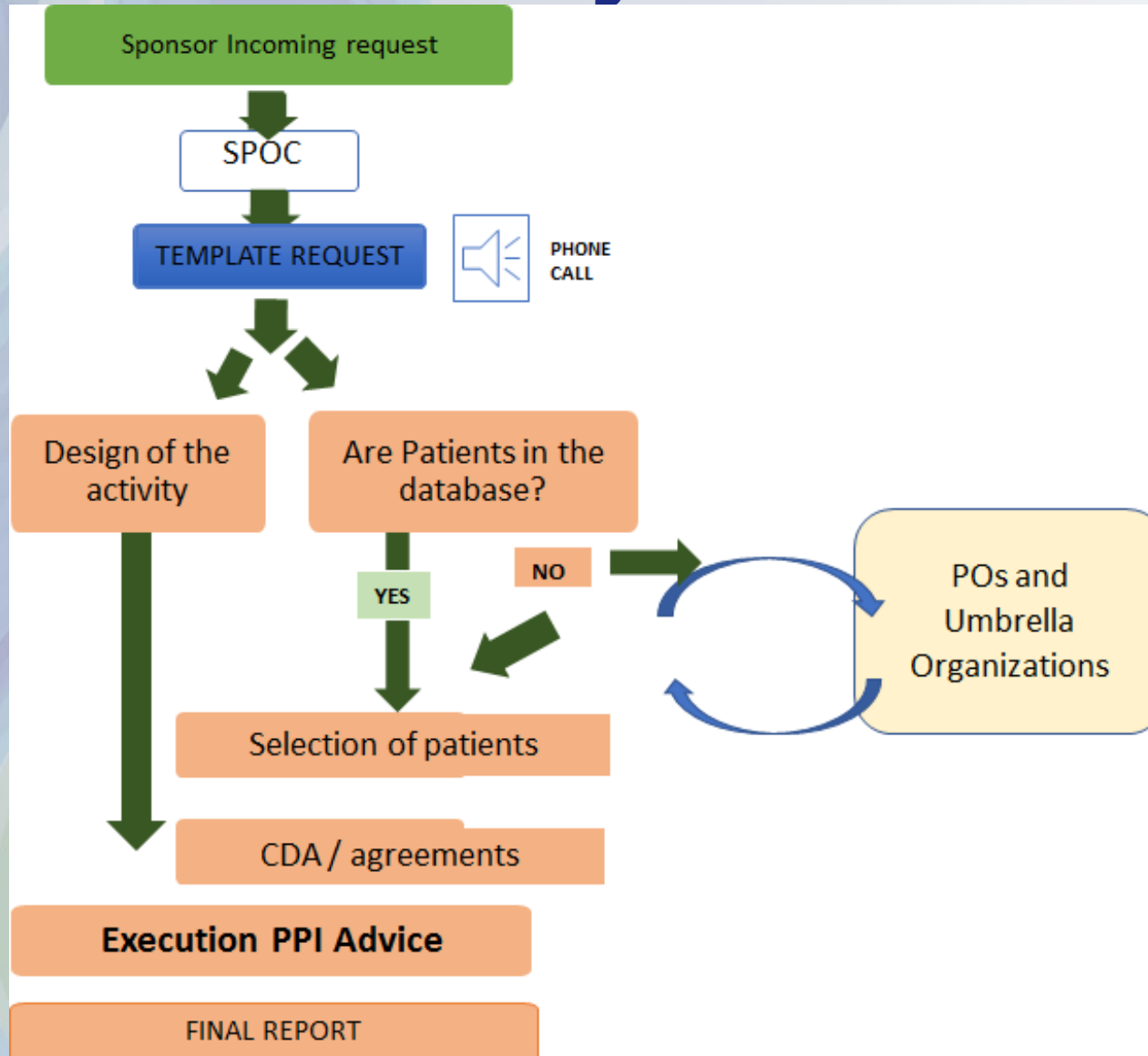
How...?

- Ensure:
 - Transparency and objectivity.
 - Special needs of the patients / family.
 - Means for the involvement (p.e. digital tools).
 - Alignment with ethics principles and the rights of the children.
 - Conflict of interest.
 - Compensation – reimbursement.
 - Choose the best and suitable methodology
 - Ask for advice of experts.

Methodology

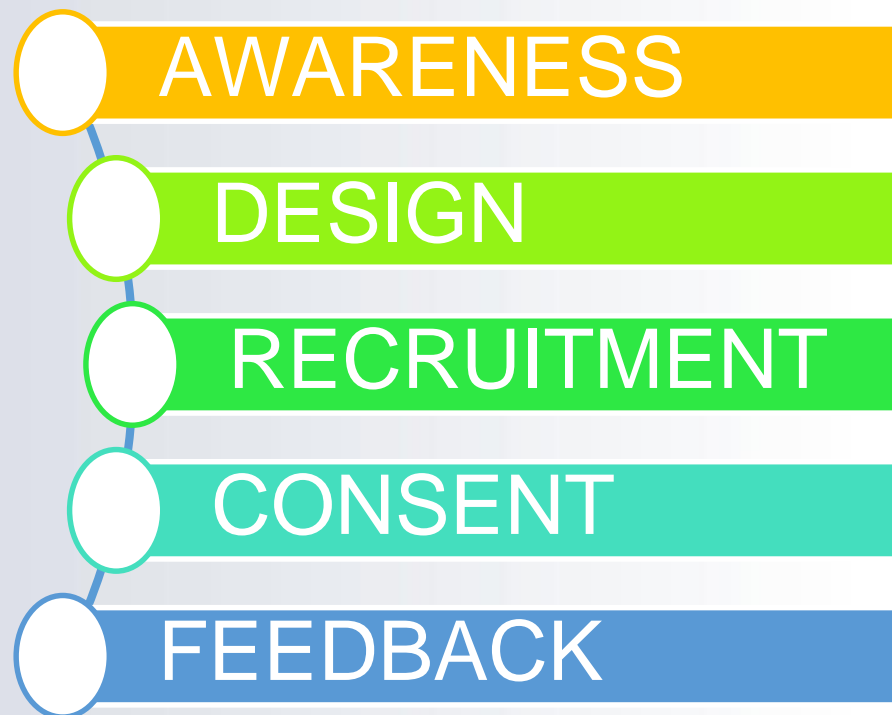
- Focus group
 - Questionnaire
 - Interview
 - Patient journey simulation
 - Advisory boards
 - Steering committee
 - Etc.
-
- **F2F - virtual - blended**

CYP & family involvement in c4c





**EVERYONE
WINS
WHEN YOU PUT
PATIENTS
FIRST**



PPI expert of your NH

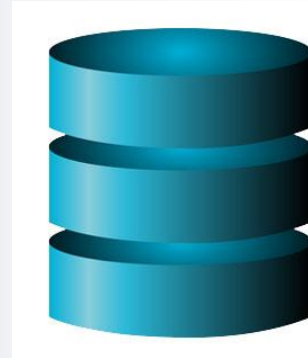
- One expert on PPI per country
- Support and liaison to involve patients
- Collaboration with POs and sites



Patient Expert Database

Registration open to:

- Carer of a young patient
- Adult patient
- Professional of a PO
- Facilitator of a YPAG



<https://conect4children.org/patient-and-public-involvement/>



conect4children (c4c): FROM THE PATIENT'S SIDE



0:01 / 2:14



Patient involvement in the academic trials of c4c

PPI Liaison

- What is EFCNI?
 - Overview of organisation
- Ongoing c4c feasibility trials:
 - cASperCF
 - TREOCAPA

EFCNI

The European voice of preterm children and their families

- First pan-European organisation to represent the interests of preterm and ill newborn infants and their families
- Combine forces of parents, healthcare experts, scientists → reducing preterm birth rates and improving outcomes
- Fields of activities
 - Preconception and maternal care
 - Treatment and care
 - Continuing care



A strong partnership with...

- | | |
|--|---|
| <ul style="list-style-type: none">• >100 national parent / patient organisations worldwide• >20 global healthcare societies• >30 European healthcare societies• >80 national healthcare societies | <ul style="list-style-type: none">• Supranational organisations (WHO, ...)• European institutions (EU parliament, commission, EMA,...)• G7 preparation-meetings |
|--|---|

TREOCAPA - Prophylactic **treatment** of the ductus **arteriosus** in **preterm** infants by **acetaminophen**

The study is being coordinated by INSERM in France and led by Nantes University Hospital. This study is a Phase II/III European multicentre double-blind randomised trial which will run in **66** sites in **17** European countries.

We test the primary hypothesis of whether prophylactic pharmacological intervention with acetaminophen (paracetamol), a drug with allegedly fewer adverse effects and efficacy for closure of ductus arteriosus, increases the survival without severe morbidity at postmenstrual age of 36 weeks in infants born extremely preterm.

Phase II - dose finding phase in order to assess the minimum effective dose regimen of acetaminophen for the closure of PDA for neonates with a gestational age less than 27 weeks. A maximum sample size of 30 patients will be enrolled (with 1/3 of patients with GA of 23-24 weeks, 1/3 of 25 weeks, and 1/3 of 26 weeks).

Phase III - placebo-controlled superiority trial of 548 infants of more than 27 weeks GA to increase survival without morbidity from 50% to 62%

This project has received funding from the Innovative Medicines Initiative 2 Joint Undertaking under Grant Agreement No 777389. The Joint Undertaking receives support from the European Union's Horizon 2020 research and innovation programme and EFPIA

- **Parent Advisory Board and further parent/patient representatives**
- Identification and selection of Trial Parent Advisory Board (PAB) members
- Management of PAB
- Coordination with c4c PPI-Team
- Creation of information material on the trial
- Research into further relevant patient/parent groups for TREOCAPA
- Coordination with further relevant patient/parent groups (if applicable)



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• **Providing input on trial materials**

- Providing input on Ethics proposal ✓
- Providing input for study protocol/design ✓
- Providing input on all relevant trial materials during the course of the trial
- Create and edit letter of consent for parents in the trial ✓
- Providing input for the development of the e-tool to perform follow-up
- Provide advice for parental adherence during follow-up

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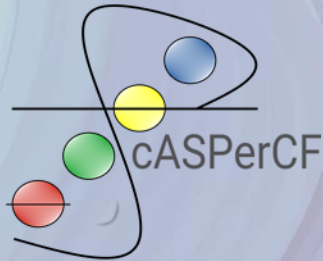
- **Involvement of EFCNI in the Steering Committee of the trial**

- Taking part in the Steering Committee calls/meetings
- Reviewing documents pertaining to the Steering Committee

- **Outreach and dissemination**

- Establishment of a communication strategy
- Dissemination via Social Media
- Identification of key stakeholders
- Translation and dissemination of trial results to patient/parent community, policy makers and general public

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cASPerCF - Finding the right dose of posaconazole for children and young people with CF and *Aspergillus* infection.

The study is being coordinated by the University of Exeter, UK, in collaboration with the Children's Hospital Bambino Gesù in Rome, Italy. This randomised, open label study will run in **35** centres in **12** European countries.

We aim to screen 1500 children and young people (between the ages of 8 and 18) for signs of *Aspergillus* infection in 18 months. 135 children and young people who were found to have an *Aspergillus* infection during the screening phase will be randomly allocated to either the treatment or control arm.

45

participants will receive **no treatment** but will continued to be monitored.

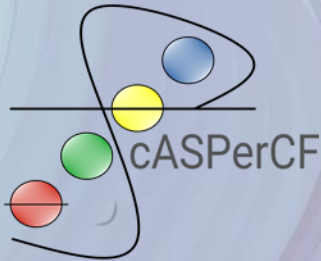
90

participants will receive the **posaconazole** treatment for three months.

We are interested to see if the posaconazole dose used gives sufficient levels in blood, and if it is able to clear *Aspergillus* infection, reduce inflammation, and stop lung function decline.

cASPerCF is a c4c project. This project has received funding from the Innovative Medicines Initiative 2 Joint Undertaking under grant agreement No 777389. The Joint Undertaking receives support from the European Union's Horizon 2020 research and innovation programme and EFPIA.





Patient and Public Involvement in the cASPerCF study.

Patient and Public Involvement (PPI) in cASPerCF can be summarised by three key pillars:

Educating patients and the public on the aims of the study.

Advocating for patients within the design and delivery of the study.

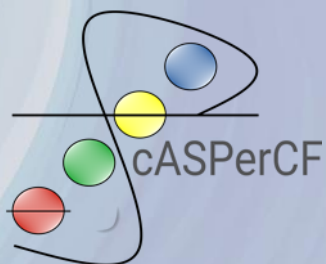
Disseminating progress and results of the study.

And we have a number of methods to complete these aims.

A Steering Committee, Focus Groups, Developing Accessible Trial Materials, Social Media and PPI lead involvement in Study Team Meetings.

cASPerCF is a c4c project. This project has received funding from the Innovative Medicines Initiative 2 Joint Undertaking under grant agreement No 777389. The Joint Undertaking receives support from the European Union's Horizon 2020 research and innovation programme and EFPIA.





Patient and Public Involvement in the cASPerCF study

Aims

Educating
Advocating
Disseminating



Methods

Steering Committee
Focus Groups
Accessible Trial Materials
Social Media
Study Team Meetings



Outcomes

Lay Protocol
Lay Protocol Graphics
Patient Information Sheet
Twitter Account
YouTube Video
Input and Review of Protocol Design
Considering Effects of Covid19
Dissemination of Results

This enables us to successfully involve, empower and listen to patients, and ensure that cASPerCF works best for everyone. **Follow us on twitter for more information: @cASPerCFstudy.**

cASPerCF is a c4c project. This project has received funding from the Innovative Medicines Initiative 2 Joint Undertaking under grant agreement No 777389. The Joint Undertaking receives support from the European Union's Horizon 2020 research and innovation programme and EFPIA.



Activity: Asmtha paediatric clinical trial

SUMMARY OF THE TRIAL

Aim of the trial: Find out if testing the MAS gene helps doctors to prescribe the best Stage 3 Defence medicine: Exhalin or Verabreath.

Design of the trial:

- 200 participants between 7 and 18 years old.
- Wash out period of 2 weeks. Patients will be able to use their inhalers.
- **Group 1:** test of the MAS gene.
 - If they have the gene= they take Verabreath
 - If they do not have the gene= they take Exhalin
- **Group 2:** not tested of MAS gene. Treatment: Exhalin

SUMMARY OF THE TRIAL

- One year of treatment
 - If the drug doesn't work doctors can change the treatment and patients can decide to not take part of the trial.
- Outcomes:
 - Effect school life. How many days of school have been missed because of ashtma.
 - Effect on ordinary life: confidential online questionnaire six times during the trial.
- Medical tests: half a day in hospital four times during the trial, to mave tests of their asthma.
- Thank you gift (20 € Amazon voucher) and travel expeses covered.

Questions for the discussion:

1. How clear is the aim of the clinical trial? Is there any missing information? What are the benefits/risks for patients?
2. What PPI activities could be carried out before the trial starts?
3. How clearly are the procedures during the trial explained?
4. What are your thoughts on the trial follow up/compensation? How could the patient feature more prominently?

Take home messages...

1. Make sure the **involvement of children and young people** in the drug development process **is feasible**.
2. **PPI needs to be an integrated part of the process** from the beginning
3. **Design, execution and evaluation** of every PPI activity is essential.
4. **Share your experiences of PPI (good and bad)** so everyone can learn 'how' to effectively involve patients and families in the research process
5. **Showing the added value of PPI** (in terms of outcomes/financially) is important for implementing points 2 and 3 below.

Promote a new research paradigm educating patients

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Educational Resources Library

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This Toolkit is intended as a living repository of educational materials to provide patients participating in clinical trials, their families and new YPAGs (Young Person Advisory Groups) with information to get started in patient and public involvement in the development of medicines.

In this section, patients and their families will find materials that will help them understand what a clinical trial is, what happens in a clinical trial, what ethical and legal implications entail and what role patients' (and their families) can play in the development of a clinical trial.

[Click the panel below to access the complete educational resource bank.](#)





Thank you!

ppi@conect4children.org

