Patient and public involvement (PPI) in paediatric research within eYPAGnet

EnprEMA Annual Meeting

October 9, 2023

Agenda

- eYPAGnet and its expertise in PPI
- Core values of eYPAGnet
- Evolution of eYPAGnet
- Case study: multi-country advice
- Services and activities of the network
- Joining eYPAGnet

Steering Committee Experts

+ 50 years

of expertise in PPI

- **2006** First YPAG in the world
- **2012** Creation of eYPAGnet / Formalized by EnprEMA in **2017**
- **2015** iCAN formed
- **2018** Key partners in c4c

Jenny Preston
Senior PPI Engagement Manager,
Alder Hey Hospital, UK

Begonya Nafria
Patient Engagement in Research Coordinator
Barcelona Children's Hospital - Spain

Pamela Dicks PhD
Manager Children's Research Network
and Patient/Public Involvement Lead
NHS Scotland

Segolene Gaillard
Patient/Public Involvement Lead
Rare disease network manager
University Hospitals Lyon, France

European Network of experts in PPI

01

Professionals embedded in **Children's Hospitals** and **Clinical Research Facilities**

02

Alongside clinicians and research nurses conducting paediatric trials in **all paediatric specialties** including **rare diseases**

03

Direct link with **patients and parents** Collaboration with **patients associations**

04

Experience in all clinical trials phases, longitudinal studies, non-drug interventions and medical device research

European Network of experts in PPI

05

Leading experts in **developing and delivering bespoke PPI plans** that meet the needs of the client and that are consistent with eYPAGnet core values

06

Working with

- Parents and patients living with paediatric diseases
- YPAG's
- Patient Organisations
- National Paediatric Clinical Research Networks
- International Paediatric Clinical Research Networks: c4c-S
- Paediatric Hospital Services
- Paediatric Clinical Networks and Research Facilities
- Industry or academics partners

Core Values

Patients, Parents and Young Persons Involvement is **meaningful** to ALL stakeholders

Involvement leads to change and is **not tokenistic**

Impactful

Respectful of the needs of the patients, parents and young people

Core Values

Work to an agreed PPI Plan (**adapted methodology**)

Inclusion of an **Impact Assessment** (research, participants)

Standardization of activities across countries and languages

- Best Practice Standards and Governance
- Standard CDA

Core Values

Ethical Involvement and Engagement of Patients, Parents and Young People within YPAGs:

- Ensure patients are informed of actions implemented
- Reimbursement - fair market value
- Inclusive
- Supportive

eYPAGnet evolution

A network formed as a platform to share YPAG experiences and opportunities.

A network beyond YPAGs, involving dedicated groups of patients or parents to ensure that the right participants are involved in a meaningful way

- Respect
- Support
- Transparency
- Responsiveness
- Fairness of Opportunity
- Accountability
- Independence
- Partnership of “equals”

Young Persons Advisory Groups

- Group of children and young people that advocate for children involved in research.
- With **different experiences** of chronic and acute conditions and/or accessing healthcare.
- Meeting together regularly, **working in groups** and **providing advice** from a young person's perspective on a full range of activities including clinical trial design, recruitment methods and patient materials.
- **Receiving training** on research methods, clinical research, consent and assent, clinical trial design and governance.
- Meeting regularly therefore **responsive to requests**
- Provide **young persons perspective**.
- Having their own experiences of healthcare transferable to other diseases

Disease specific groups

- Patients or parents groups
- Disease and condition specific focus groups brought together for the **purpose of a project**
- Working with a facilitator to develop workshops to ensure **meaningful involvement** and that patient views are gathered
- **Lived experience** of physical and emotional symptoms and impact on family
- **Receiving training** on research methods, clinical research, drug discovery etc
- Partner in research
- Highly motivated

By permission of Stacey Hutchison, mum of Frankie

eYPAGnet today

Founder teams

- Generation R
- Kids France
- ScotCRN
- Kids Barcelona

+ 30 groups working with young people, patients and parents across Europe

14 countries

- Belgium
- Czech Republic
- Denmark
- Estonia
- Finland
- France
- Germany
- Ireland
- Italy
- Netherlands
- Poland
- Portugal
- Spain
- United Kingdom

EnprEMA
European network of paediatric research at the European Medicines Agency

Case study: multi-country advice

- **Study**: Academic European Platform Trial in rare diseases
- Service requested to the Single Point of Contact of Conect4Children
- **Countries involved in the project**: France + UK + Spain
- **Methodology**: Focus group discussion (teenager patients + parents): 9 families involved
- **Topics of the PPI activity**:
  1. Design acceptability
  2. Informed consent for a platform trial
  3. Schedule of assessment
  4. Acceptability of medical procedures

Case study: multi-country advice

What was the added value of eYPAGnet?

- Adapt the presentation provided by sponsor, to young people and families
- Design of the methodology
- Use of native language (English, French and Spanish)
- Translate material for participants
- Recruitment through local networks
- Facilitate and execute the activity
- Provide an adequate and “relaxed” atmosphere for the participants
- Main link with the patients and parents
- Report
- Request of changes made by the sponsor and ensure feedback to participants

Case study: multi-country advice

Some outcomes

- Acceptability of the platform trial design + waiting list.

Importance of providing to the patient the right information during the consent process: improve the health literacy of assent + consent.

- All patients and parents indicated that they would be willing to participate the observational period, but only if the visits for this study would be the same as those during regular clinical care → **Schedule of visits adapted**.
- Families expressed a strong preference for including a patient-reported outcome measure. → **Protocol adapted**
- **Country differences** about the check-ups for the trial. Diversity of preferences in regards the places of the follow up visits: satellite sites (near to the patient's home) vs. clinical trial site.

Case study: multi-country advice

CTS Clinical and Translational Science
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ARTICLE | Open Access

Optimizing expert and patient input in pediatric trial design: Lessons learned and recommendations from a collaboration between conect4children and European Patient-CEntric ClinicAl TRial PLatforms

Britt A. E. Dhaenens, Fenna Mahler, Hannah Batchelor, Pamela Dicks, Segolene Gaillard, Begonya Nafria, Annette Kopp-Schneider, Maria Alexandra Ribeiro, Matthias Schwab ... See all authors

First published: 30 June 2023 | https://doi.org/10.1111/cts.13547

Britt A. E. Dhaenens and Fenna Mahler should be considered co-first authors.
Saskia N. de Wildt and Rianne Oostenbrink should be considered co-last authors.

Some of our services

Study specific support

- Co-design of clinical trial protocols
- PROMS, PREMS, QoL…
- Development and review of patient documentation…
- etc…

Education and training of key stakeholders

- Co-creation (with children and families) educational resources
- Development of training for key stakeholders

PPI advice and coordination of patient and public involvement activities

- Development of patient and public involvement plans
- Execution of PPI plans

Reporting and measuring the impact of involvement

- Evaluation and impact assessments of involvement activities
- Access to co-created reporting tools

Bespoke PPI Plans

- **Advice on how to implement PPI within a project**

  => PPI plan tailored to the needs of the project

  - ✓ Which activities could be relevant at which stage of the project (conception, design, conduct, dissemination…etc)?
  - ✓ What type of participants are needed ? Disease focused, YPAGs, both?
  - ✓ What methodology should be applied for which activity?
  - ✓ What impact we foresee and how will we assess it? (impact on research and young people)

- **Facilitation of the PPI plan with disease groups and/or YPAGs**
  - ✓ Developing adequate workshop
  - ✓ Children and parent-centred methodology

Research on PPI

- Understand what ‘meaningful’ PPI means to children and young people
- Build best practice guidance on PPI in paediatrics (e.g. eYPAGnet tool kit…)
- Impact of PPI in paediatric clinical research: Impact on research and on young people. Build an evidence base on the impact of involvement on young people/patients
- Develop standard training for young people

Research on PPI

Review | Open Access | Published: 19 February 2022

Developing a More Tailored Approach to Patient and Public Involvement with Children and Families in Pediatric Clinical Research: Lessons Learned

J. Preston, B. Nafria, A. Ohmer, S. Gaillard, P. Dicks, L. West & M. A. Turner

Therapeutic Innovation & Regulatory Science **56**, 948–963 (2022) | Cite this article

2667 Accesses | **1** Citations | **57** Altmetric | Metrics

Preston *et al.*
Research Involvement and Engagement (2023) 9:61
https://doi.org/10.1186/s40900-023-00477-8

Research Involvement and Engagement

METHODOLOGY **Open Access**

Reporting involvement activities with children and young people in paediatric research: a framework analysis

Jennifer Preston[1*], Giovanni Biglino[2], Victoria Harbottle[3,4], Emma Dalrymple[5], Helen Stalford[6] and Michael W. Beresford[1,7]

Children's views on taking medicines and participating in clinical trials

Sofia Nordenmalm [1], Elin Kimland [2], Franca Ligas [3], Birka Lehmann [4], Joana Claverol [5], Begonya Nafria [5], Ann Marie Tötterman [6], Benjamin Pelle [3]

Involving children and young people in clinical research through the forum of a European Young Persons' Advisory Group: needs and challenges

Segolene Gaillard, Salma Malik, Jenny Preston, Begonya Nafria Escalera, Pamela Dicks, Nathalie Touil, Sandrine Mardirossian, Joana Claverol-Torres, Behrouz Kassaï

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Britt A. E. Dhaenens and Fenna Mahler should be considered co-first authors.
Saskia N. de Wildt and Rianne Oostenbrink should be considered co-last authors.

Special Section: Pediatric Therapeutic Development: Special Section | Published: 19 December 2019

Role of Patients and Parents in Pediatric Drug Development

Vivian W. L. Tsang, Leanne West MS, Christine Woods BS, Chester J. Koh MD, Susan McCune MD, Theresa Mullin PhD, Sharon R. Smith MD, Segolene Gaillard MS, Joana Claverol MS, Begonya Nafria MS, Jennifer Preston PhD, Pamela Dicks PhD & Charles Thompson MD, FCAP

Therapeutic Innovation & Regulatory Science **53**, 601–608 (2019) | Cite this article

International events with YPACs

Ican Summit – Barcelona 2016
Theme: Holistic health research

Ican Summit – Edinburgh 2018
Theme: Clinical trial design

Ican Summit – Lyon 2022
Theme: « Paediatric clinical research, therapeutic innovation and Rare Diseases »

Collaboration and networking

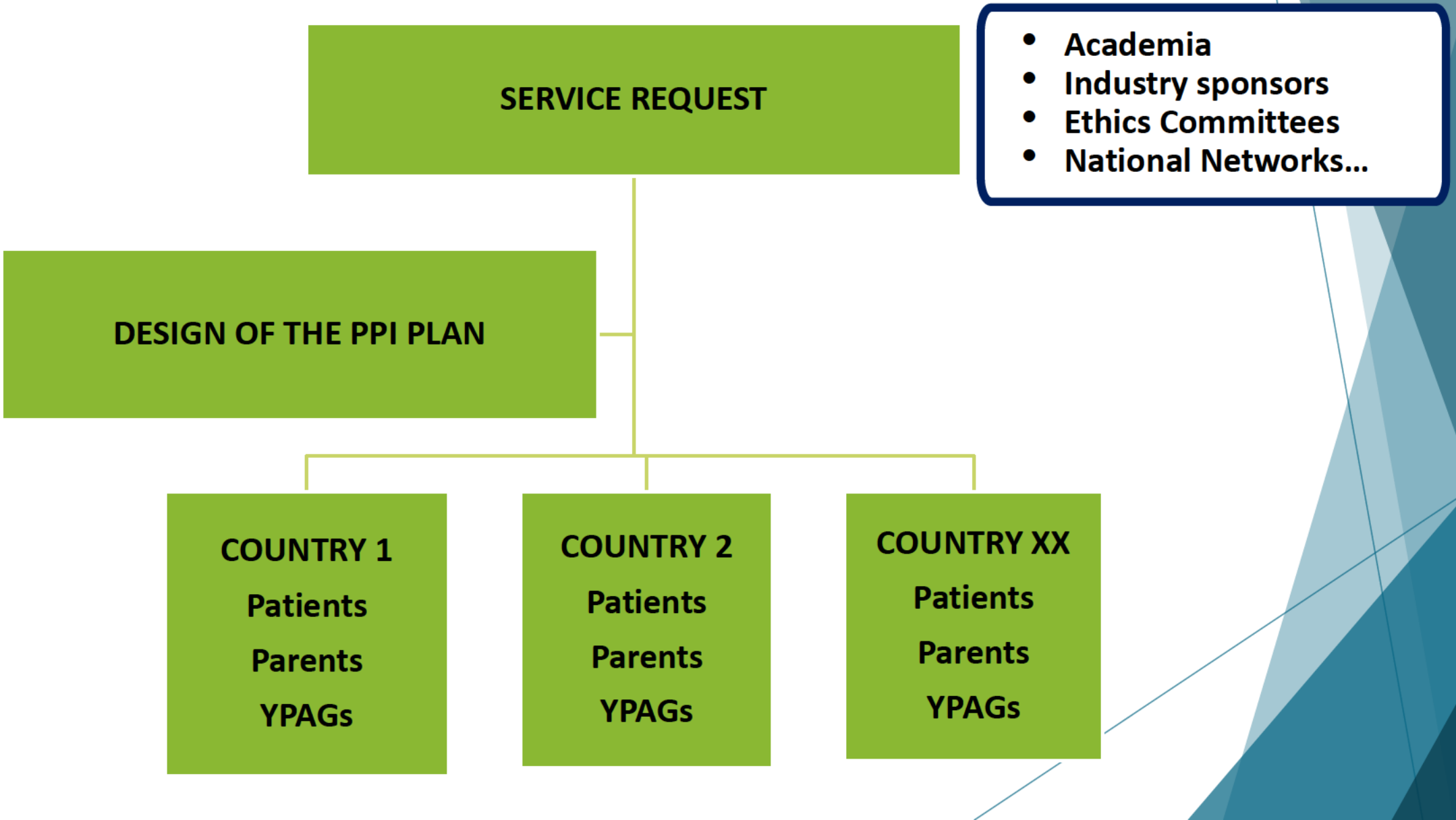
conect 4children · imi innovative medicines initiative · efpia · AIDFM CETERA

eYPAGnet

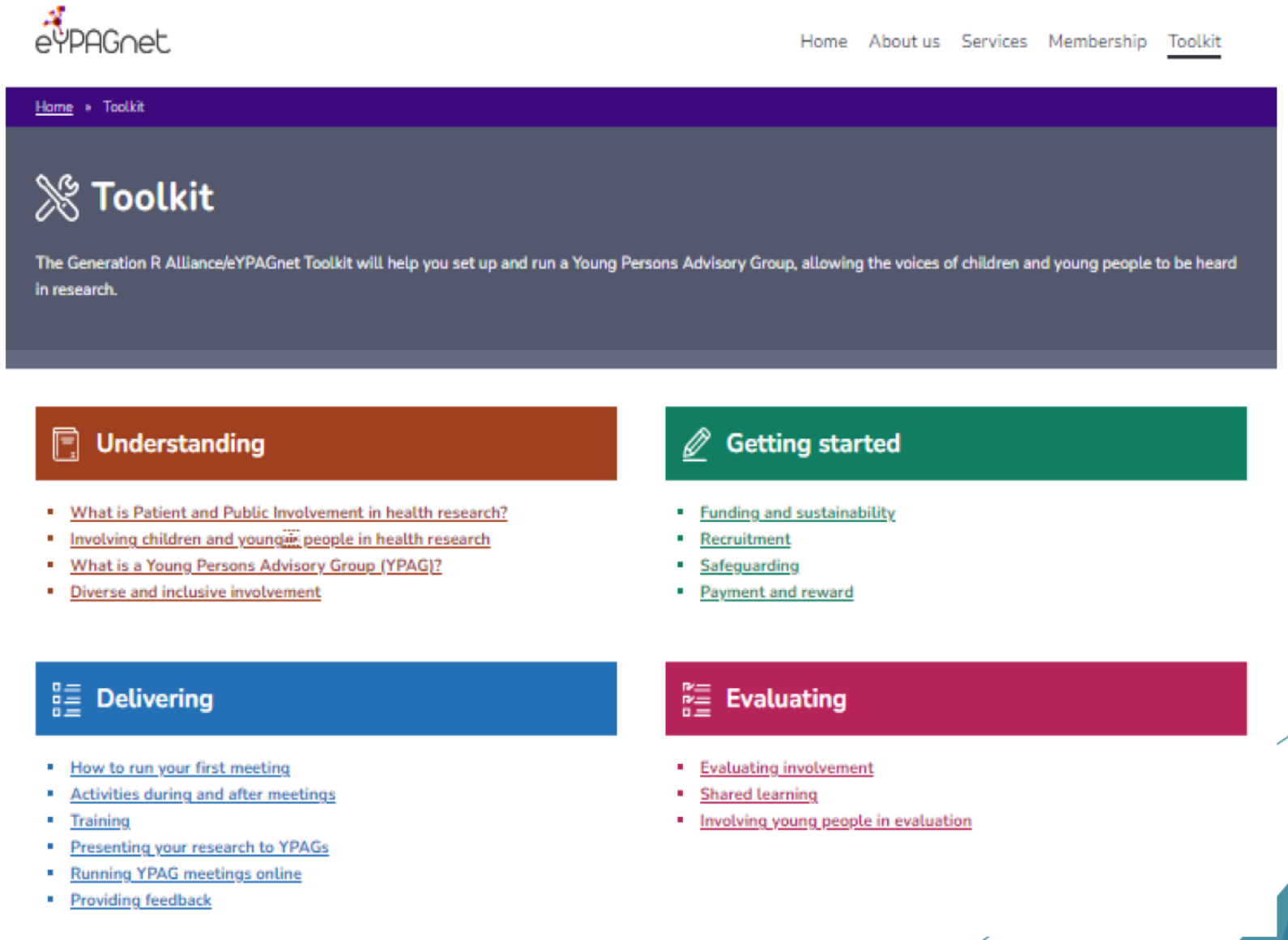
European Young Person's Advisory Groups Network
PPI Ignite Webinar
21st November 2022

Involving Children and Young People in the design of Paediatric Clinical Research

How do we work?



Supporting new experts …



eYPAGnet membership

https://eypagnet.eu/become-a-member/

Application form

Name of individual or organisation

Contact person (if different)

Address:

The future

- Continue to embed young people's involvement in clinical research
- Continue to grow the network & share best-practice
- Continue to collaborate with key stakeholders
- Continue research on PPI
- Build an evidence base on the impact of involvement
- Sustain the network through external funding

Respecting the rights of children and young people

Thank you!

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