eYPAGnet

Benefits of involving patients and young people in paediatric clinical research

EnprEMA Annual Meeting

October 10, 2023

Steering Committee Experts

+ 50 years

of expertise in PPI

- **2006** First YPAG in the world
- **2012** Creation of eYPAGnet
- **2015** iCAN formed
- **2017** Recognition of EnprEMA
- **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)

Ethical Involvement and Engagement of Patients, Parents and YPAGs

Standardization of activities across countries and languages

- Best Practice Standards and Governance
- Standard CDA

eYPAGnet TODAY

Founder teams

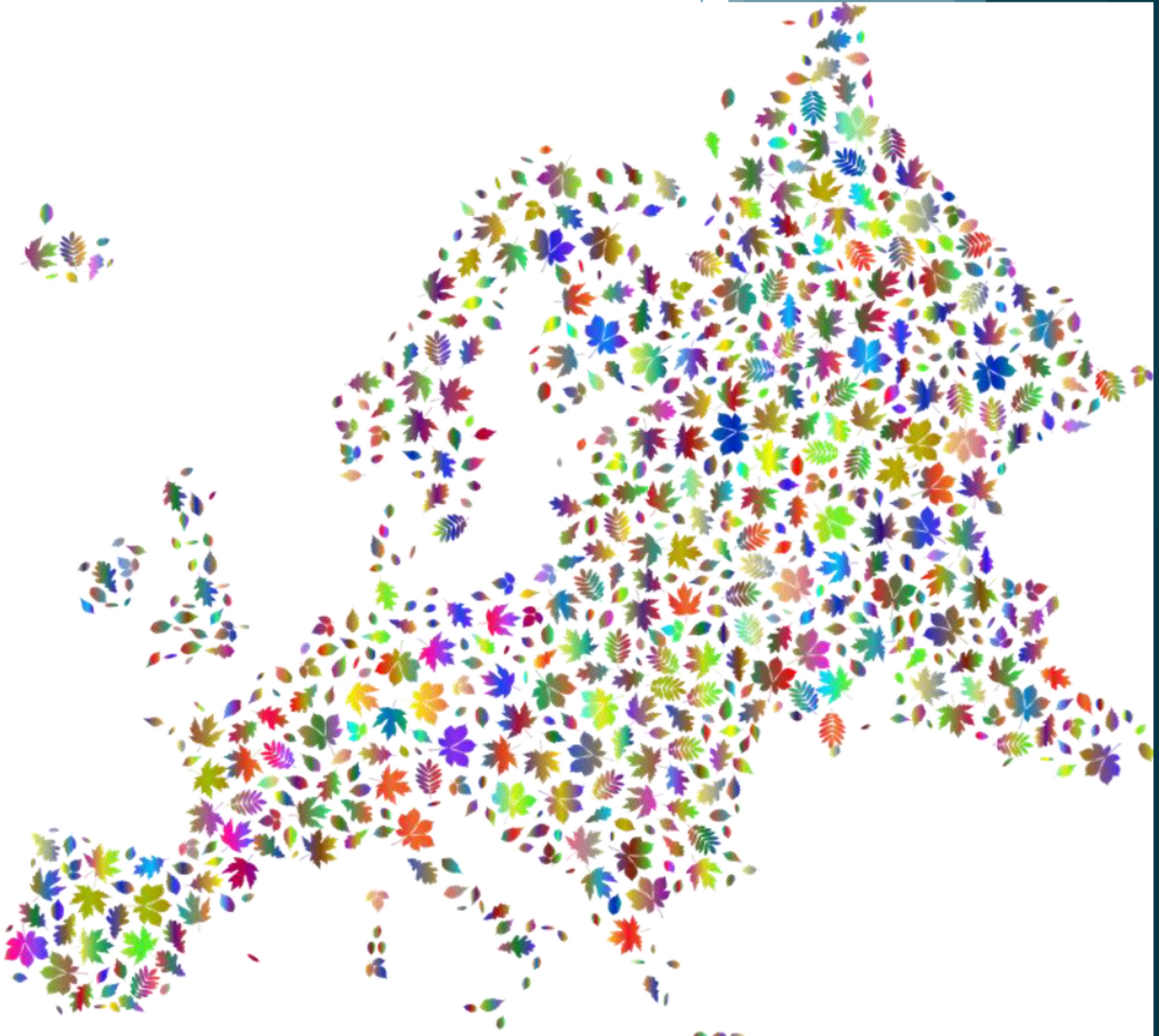
- Generation R
- Kids France
- ScotCRN
- Kids Barcelona

+ 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

Enpr-EMA
European network of paediatric research at the European Medicines Agency



Challenges

- Acceptability of formulation
- Clinical trial protocol:
    - Study design
    - Study endpoints relevant for Patients
    - Quality of life scales and PROMs/PREMs adapted to the disease
    - Options of digitalization and decentralization of clinical studies
    - Etc.
- Informed assent/consent documents
- Strategies of recruitment

Benefits on the ROI

Phase 1	Phase 2	Phase 3	Approval	Post-approval
50% Time reduction for the recruitment	**61 days** Time reduction of the study for one amendment, 90 days if this is a substantial amendment	**535 K** Cost of one amendment per study (141.000 $) + cost of 90 days for the approval	**20%** increasing the possibility of approval by the use of study design adapted to patients	**+ 25 M** Net present value

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

Research on PPI

+ 80 publications

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

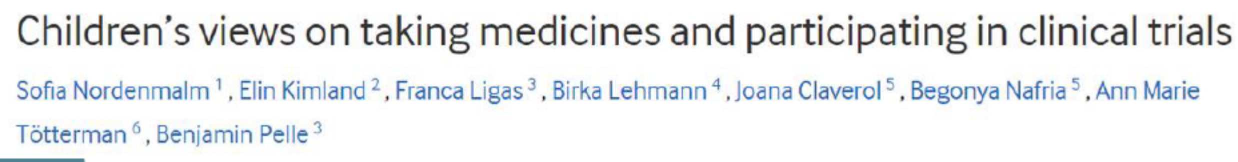
Preston *et al.*
Research Involvement and Engagement (2023) 9:61
https://doi.org/10.1186/s40900-023-00477-8

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]

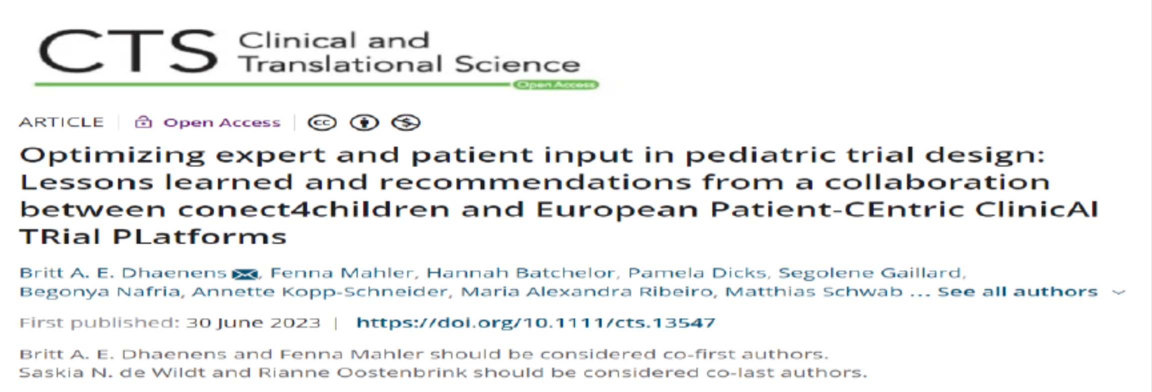


Children's views on taking medicines and participating in clinical trials

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



CTS Clinical and Translational Science

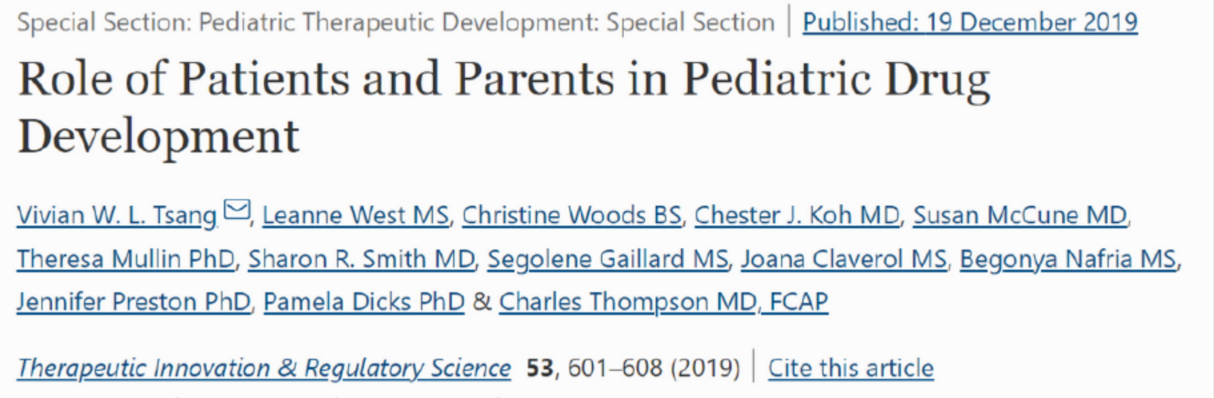
ARTICLE | Open Access

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.



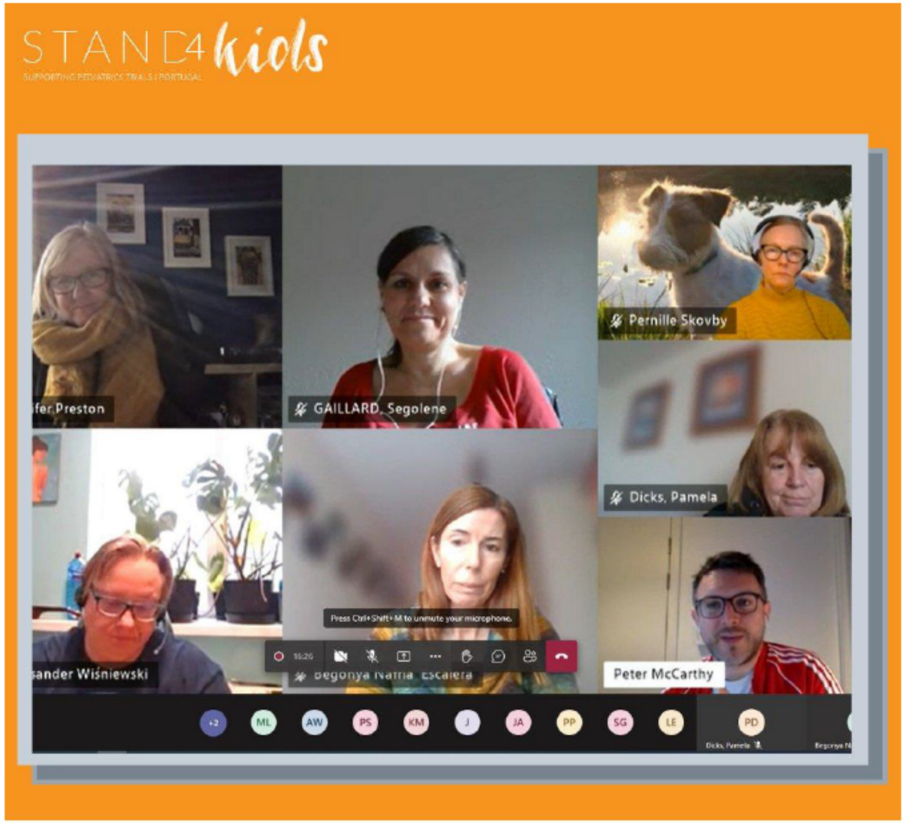
Special Section: Pediatric Therapeutic Development: Special Section | Published: 19 December 2019

Role of Patients and Parents in Pediatric Drug Development

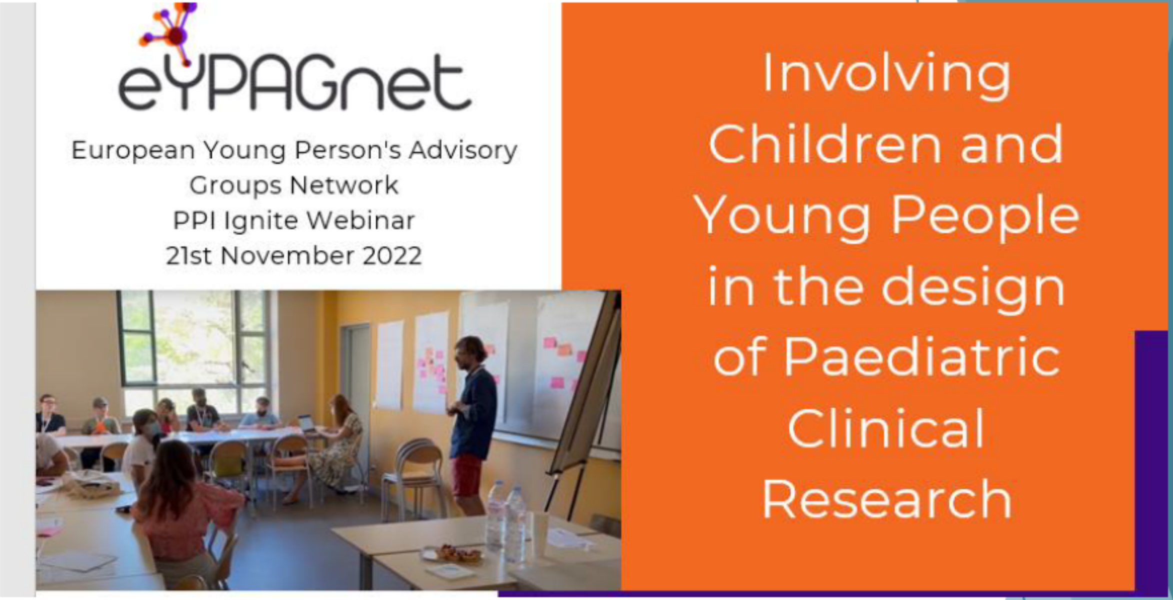
Vivian W. L. Tsang, Leanne West MS, Christine Woods BS, Chester J. Koh MD, Susan McCune MD, Theresa Mullin PhD, Sharon R. Smith MD, Segolene Gaillard MS, Joana Claverol MS, Begonya Nafria MS, Jennifer Preston PhD, Pamela Dicks PhD & Charles Thompson MD, FCAP

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

Collaboration and networking



conect4children 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

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!

info@eypagnet.eu