Making Patient Engagement Meaningful: Lessons from Children and Young People in Paediatric Research

ACT-EU Multi-stakeholder Platform Advisory Group Meeting

17th Sep 2026

Donato Bonifazi, TEDDY Network Chair

email dbonifazi@teddynetwork.net

www.teddynetwork.net

TEDDY NETWORK
European Network of Excellence for Paediatric Clinical Research

The model adopted by the TEDDY NETWORK

Objective

Promote a shift in the perspective that places the empowerment of children and adolescents at the center, recognizing their active role in decisions that affect their health, and valuing their experiences and specific knowledge.

The involvement of children in health and biomedical research is a progressive process, aligned with the development of their capacities, and involves different levels of participation:

1. **To be informed**

2. **To express an opinion**

3. **To influence decisions**

TEDDY Charter

TEDDY Model: KIDS-YPAG Groups to Promote Children's Participation in Decision-Making Processes

01 Strong motivation

02 Scientific contribution on par with experts

03 Appropriate educational tools (cartoons, videos)

04 Engagement with institutions and external stakeholders

The first **YPAG** from **TEDDY** was established in 2017. To date, 10 groups are members of the **TEDDY KIDS Network** and are also part of **iCAN** (international Children Advisory Network).

Our Statutes provide for an **ADVISORY GROUP – TEDDY KIDS**, one member of which is part of the **TEDDY Board of Directors**.

Ongoing European funded projects involving TEDDY as a Partner, and its TEDDY KIDS Network

Involvement of children and young patients (CYP)

Building an integrated European research ecosystem to advance rare disease research, accelerate new treatments, and integrate patient-centred solutions across Europe.

Building an ecosystem to support the development of dedicated paediatric medical devices specifically tailored to address unmet clinical needs in rare paediatric conditions.

Developing innovative methodology and advanced clinical trial designs with regulatory relevance to optimise drug development for paediatric and rare diseases.

ERDERA Project

ERDERA
European Rare Diseases Research Alliance

1st ERDERA Training, 2-3 May 2025, Athens

Click here to discover
Speakers list
AGENDA
TRAINING SESSIONS

2nd ERDERA Training, 23 - 25 April 2026, Paris

Click here to discover
Speakers list
AGENDA
TRAINING SESSIONS

TEDDY NETWORK

INVENTS Project

Within the project activities, TEDDY aims to recruit and engage patients in order to promote informed decision-making and active participation.

01 **Action plan**

02 **PEG development**

03 **Education &Training**

04 **participation in decision-making processes**

TRAINING

The young patients training process began with a two-day meeting in Bari and continued via online sessions, leading to key capacity-building and decision-making milestones:

- **Education & Capacity Building**: Completed 1st cycle, with the second in person meeting during ERDERA Training in Paris (April 2026).
- **Decision-Making & Strategy**: evaluated Task 6.3 decision-making matrix, and integrated PEG contributions across Work Packages.

Pediatric expert patient group (PEG) – kick-off in-person meeting – Bari (Nov 2024)

OrphaDev4kids Project

The Patient Expert Committee (PEC) contributes to identifying therapeutic needs and provides support in the preparation of the documentation required for clinical trial submissions.

Pediatric expert patient group – kick-off-meeting – University of Bologna – March 2025

The review of the survey by the PEC contributed to the future development of a medical device for patients with congenital heart disease.

Example of a questionnaire administered to the PEC for the validation of the survey developed for patients.

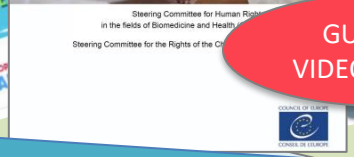
Orphadev4kids

TEDDY KIDs Activities & Tools

AWARENESS ACTIONS among KEY STAKEHOLDERS

VIDEO

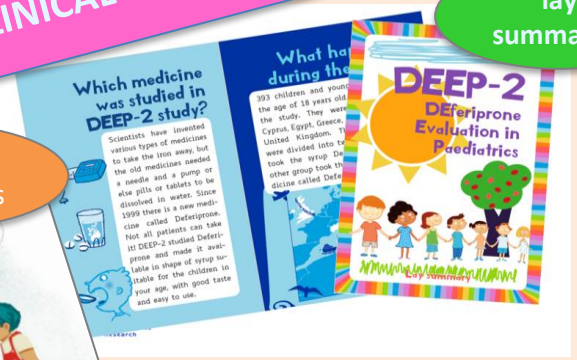
Participation of children in the decision-making process on matters regarding their health



GUIDE and VIDEO with CoE

AGE-TAILORED MATERIALS for CLINICAL TRIALS

lay summaries



consent/ assent forms

CONFERENCES and ROUND-TABLE discussions

AGE-TAILORED animated VIDEOS on clinical research

To children

To teenagers

World Orphan Drug Conference

Amsterdam, October 27, 2025

A 12-year-old patient with juvenile idiopathic arthritis (JIA), shared her experience and feedback on participating in the INVENTS paediatric Patients Expert Group

Council of Europe (CoE)

CoE Conference on the European Strategy for Children's Rights, Strasbourg, April 2-4, 2025

CoE Guide to the participation of children in decision-making processes regarding their health

The Council of Europe (CoE), with the direct contribution of European children and adolescents—including TEDDY KIDS—developed a guide and a video created by children for children, aimed at promoting their participation in health-related decision-making within its strategic plan.

GAPP (GAbapentin in Paediatric Pain)

Multinational Trial sponsored by TEDDY Network

GAPP GAbapentin in Paediatric Pain

Toward new therapies to challenge chronic pain in children

Multi-center, non-profit Phase III, **single-arm, adaptive, prospective cohort study** to evaluate the pharmacokinetics, efficacy and safety of gabapentin liquid formulation in children **from 3 months to less than 18 years of age** experiencing moderate to severe chronic neuropathic, nociplastic or mixed pain.

Study Objectives

The primary objective of the study is to evaluate the effectiveness of gabapentin in reducing chronic pain intensity in paediatric patients.

Secondary objectives include:

Assessing the safety and tolerability of gabapentin in children and adolescents.

Characterising the pharmacokinetic profile of gabapentin across different paediatric age groups.

Evaluating the impact of treatment on quality of life, sleep and daily functioning.

Assessing treatment acceptability and overall patient and caregiver satisfaction.

TEDDY NETWORK

FAST-EU Submission: 11th September 2026

Applying the TEDDY Model: The GAPP Study Experience

01 Strong motivation

To drive high engagement from the start in the creation of the **Patient Advisory Board** (PAB), TEDDY launched a targeted **Call for Interest** across TEDDY social media platforms and official website, alongside direct outreach to the **TEDDY KIDS Network**.

02 Scientific contribution on par with experts

TEDDY organised four **dedicated PAB workshops** during the study preparatory phase, bringing PAB members together with clinical experts to contribute to study design, clinical protocol review, consent/assent forms, patient diaries, and communication materials.

03 Appropriate educational tools (cartoons, videos)

Original scientific content: authentic clinical protocols, aligned with SPIRIT-C guidelines, were used to assess clarity and patient burden.

Empowerment: tailored visual tools and a technical glossary supported informed participation.

Interactive feedback: Mentimeter enabled real-time participant input, combining scientific rigour with patient empowerment.

04 Engagement with institutions and external stakeholders

- Patient representation is embedded in the **PAB and SSC (Study Steering Committee)**, with a young patient representative joining **quarterly SSC meetings** and contributing to trial decisions.
- A **GAPP Roundtable** at the **TEDDY General Assembly & Scientific Meeting** brings together young PAB members, the **SSC and DSMB** to discuss the study and patient involvement.

Practical Considerations for Meaningful Engagement:

A roadmap for creating, coordinating, and sustaining YPAGs/PABs over time

Governance & Structure

- Establish clear roles, responsibilities, and decision pathways.
- Connect Young Patient Advisory Boards (PABs) directly to research teams and stakeholders.
- Navigate ethical and legal frameworks.
- Define explicit scope and formalise terms of reference.

Representativeness

- Proactively ensure gender balance and diversity across ages, conditions, and geographies.
- Avoid over-relying on the same highly active families.
- Ensure equitable participation opportunities.

Sustainability

- Provide continuous training and appropriate participant recognition.
- Address unmet needs and ethical barriers.
- Embed engagement costs directly into project budgets.
- Adapt models flexibly to each research context.

Common principles can be shared across initiatives, but the engagement model must be tailored by assessing unmet needs, target population specifics, and ethical/legal frameworks, overcoming key barriers that currently limit youth representativeness in patient organisations and institutions.

TEDDY NETWORK

Demonstrating the Tangible Impact and Value of Youth Engagement

Formal Recognition

Explicitly recognize young patients advocates through co-authorship in publications, dissemination and advisory roles, aligning with regulatory guidelines to transition from symbolic consultation to research partnership.

Documenting Tangible Changes

Move beyond anecdotal evidence by tracking concrete modifications made to clinical trial protocols, consent/assent forms, and recruitment strategies.

Simple Impact Indicators

Implement straightforward metrics to monitor feedback uptake, participation frequency, and the quality of PAB/YPAG scientific advisory activities over time.

Institutional & Funder Support

Demonstrating clear value is essential to secure long-term institutional backing and dedicated funding for meaningful patients engagement activities.

TEDDY NETWORK

Structured Paediatric Engagement as a Benchmark for European Clinical Trials

Operationalizing the TEDDY Charter to advance ACT EU Obj. 4 across the lifespan

Obj. 4: Engage stakeholders to deliver **patient-oriented development, integrating patients' voices across the lifecycle**.

FROM PRINCIPLE TO FRAMEWORK

Translating patient-centred research into **meaningful, structured, and sustainable youth participation** requires overcoming unique ethical and regulatory challenges.

TEDDY "Paediatric patients' engagement and empowerment" Charter

A SCALABLE EUROPEAN BENCHMARK

By addressing paediatric complexities (balancing protection with autonomy), the TEDDY methodologies serve as a **standardised benchmark** directly applicable to adult research.

Transferable governance & decision-influencing roles for multi-stakeholder engagement.

DISCUSSION POINT

How can the ACT EU MSP leverage paediatric patients engagement learnings to structure continuous patient involvement across the entire lifespan?

TEDDY NETWORK