

Patients Expert Group (PEG) Meeting Report 21 March 2025

Minutes

Why we met:

To talk about what young people think about joining clinical trials and computer-based (in silico) trials, and to start making a simple tool that collects patient experiences for medicine approval.

What we agreed as rules:

- Everything said in the meeting stays private.
- We listen respectfully to each other and speak clearly.
- We can share opinions without judgment.
- We keep our comments short.

Recap of Last Meeting

We confirmed key points from our last meeting:

- You want to help improve healthcare for people with rare diseases and ensure your voices are heard regarding your experiences with medical care.
- Getting a correct diagnosis can take a long time and feel frustrating.
- There are sometimes a feeling of being misunderstood by healthcare professionals, and an experience of dissatisfaction with certain treatments
- Medicines can affect emotions and sometimes cause worry about side effects.
- You have often conducted personal research to better understand your medical condition and explore additional treatment options.
 - Exercise methods like physiotherapy and Pilates help as part of care.

Clinical Trials

What is a clinical trial?

- A study where new medicines are tested on people to see if they work and are safe.

Your views:

- Main reasons for not joining the clinical trials include:
 - Current treatment works well.
 - Worry about unknown side effects.
 - Trials can take a lot of time away from school and life.
- You might consider joining a trial if current treatments stopped working.

In Silico (Computer) Trials

What is an in silico trial?

- Testing a medicine using computer programs instead of people.

Initial feelings:

- Skeptical about trusting results from a computer alone.
- Concerned that computers cannot show how a real person feels.

After explanation:

- Learned that in silico trials use real patient data and both math and statistical models.
- Models are checked against real life to make sure they work.
- Benefits include faster results, fewer people needed, and safer testing.
- You would trust in silico results more if combined with some real-person data.
- You want transparency about how accurate the computer models are.

Building a Patient Experience Tool**Goal:**

- Allow adolescent patients to share their experiences with medicines and to gather patient experiences, especially from young people, as part of the treatment authorization process.

Key points:

- It is important to include patients' experience in the approval of new treatments, noting that this is crucial for developing effective medications.
 - Young patients are often underrepresented in the process for providing such experience. Their insights are vital for developing a tool that will allow to capture the experience
- Many adolescents prefer anonymous online sharing over in-person meetings.
 - many might not want to participate due to fear or the desire to feel normal.
 - The role of patient associations in collecting experiences was discussed.
Patient associations are important, and for the two participants present, they were the channel through which they were able to join the meeting.
However, both young patients felt that being part of an association or actively participating in one can be challenging for someone their age. They explained that they are not always ready for that level of involvement, and seeing other patients can sometimes cause stress or anxiety. This is why, while associations can serve as a valuable communication channel, the way they collect patient experiences should be more anonymous and adapted to young people's needs.
- The idea of using social media or a customized app to collect feedback was suggested as a more anonymous and comfortable way for adolescents to share their experiences.
- This tool should be easy to use, with clear questions and reminders.
- It should protect privacy and verify users to keep data reliable.
- Features could include text, voice notes, emojis, and progress tracking.
- The "Patient Like Me" tool was briefly shown as an example that connects individuals with similar conditions, allowing them to share their experience and support each other.

NB PatientsLikeMe is an online platform created in 2004 that allows people living with health conditions to **share their experiences, track their symptoms and treatments, and connect with others facing similar challenges**. Users create a personal health profile with their conditions, symptoms, and treatments. They can track their progress and daily well-being (pain, mood, side effects, etc.). They

*share experiences and advice with other patients in the community. Data from the platform is anonymized and can be used to support **research** and **improve healthcare policies**. Researchers, pharmaceutical companies, and healthcare institutions can analyze aggregated data to:*

- *Better understand real-life treatment effects.*
- *Identify unmet needs.*
- *Support the design of clinical studies.*

*Patients are no longer just participants — they become **partners in the research process**.*

*Although originally designed for adults, the model of PatientsLikeMe could inspire tools tailored for **adolescents**, by Ensuring privacy and anonymity., Using accessible, friendly design (emojis, voice notes, avatars), Creating a safe, non-judgmental space to share experiences. PatientsLikeMe is like a mix between a **social network**, a **health tracking app**, and a **patient support forum** — empowering patients and helping improve care through shared knowledge.*

Importance of Patient Associations

- Among the different roles of patient associations, they provide support, help in sharing experiences and contribute to have this experience heard during the treatment authorization process. They advocate for patients' needs and perspectives.

Next Steps and Communication

- We will communicate through the INVENTS WhatsApp group for quick updates and planning.
- Think about what you want the tool to look like and how it should work before our next meeting.

Action Items:

- **Yanis** – Prepare a simple presentation about how in silico trials work for the next meeting.
- **Francesco** – Add Stephanie and Yanis first to the INVENTS WhatsApp group.
- **Stephanie and Francesco** – review and send these notes, and a short survey to participants.
- **Patients Expert Group Members** – Come up with at least three ideas for features and ways to share feedback in the new tool.

Next Meeting:

- 18 April 2025 from 17:00 to 18:30 CET Please note that one of the two participants will be on vacation and will not be able to attend the meeting on April 18.