



Pediatric Patients Expert Group

June, 26th
Online Meeting

<https://invents-he.eu/>



INVENTS has received funding from the European Union's Horizon Europe Research and Innovation program under grant agreement 101136365.



Funded by the Swiss State Secretariat for Education, Research and Innovation (SERI).



Funded by the UKRI Innovative UK under their Horizon Europe Guarantee scheme.

Ground Rules for a Teen Focus Group

Speaking Rules

- **Respect and Active Listening** – Everyone should listen without interrupting and respect each other's opinions, even if they disagree.
- **Fair Speaking Time** – Everyone gets a chance to talk. If you've spoken a lot, let others have a turn.
- **Speak Freely, No Judgment** – This is a safe space. You can share your thoughts without fear of being judged.
- **Keep It Short and Clear** – Try to be brief so everyone has time to express themselves.
- **It's a Discussion, Not a Debate** – We're here to share experiences, not to convince others.





Ground Rules for a Teen Focus Group

Confidentiality Rules

- **What's Said Here, Stays Here** – What we talk about in this group should not be shared outside.
- **Anonymity is Key** – If any thoughts from this session are shared later (in a report, for example), they will remain anonymous.
- **Recording for Content Analysis Only** – The session will be recorded, but only for content analysis. The recordings will be deleted once the project is over.
- **Session Summaries for Accuracy** – At the end of each session, we will provide you with a summary of the key points discussed to ensure we understood you correctly.
- **Respect Personal Information** – Don't share personal details about others outside this group.



Today's Discussion






- Over these five sessions, we've learned a lot from each other. You've shared what it's really like to live with a rare disease — not just the medical side, but also what you feel, what you find hard, and what helps you.
- Together, we've talked about what matters most to you, what makes you feel more included, and what kinds of support really make a difference.
- Your ideas will help us build better tools and projects that actually meet your needs — not just what adults or health professional *think* you need. You've shown that your voice matters, and we'll make sure it's heard in future decisions.





Today's session: What did we learn together?

- Thanks to your ideas and feedback, we improved the questionnaire for adolescents with rare diseases.
 We added an important part that was missing: **mental health and emotional well-being.**
-  **What we'll talk about today:**
- What the first results tell us
- How your voices helped shape the final version
- What this tool will be used for (regulators, researchers, and care teams)
- And... what comes next: testing it with a larger group of adolescents to confirm what really matters.
-  **You made this possible. Let's build the next step together!**





Description of the Tool

The tool presented contains 13 items, organized into two main sections:

Treatment Profile and Current Experience (Questions 1–6)

These questions address treatment frequency, perceived effectiveness, openness to change, recent hospitalizations, impact on daily life, and the adolescent's role in decision-making.

What Matters Most (Questions 7–12)

- Six domains are explored: relationship with healthcare professionals, experience of illness, treatments/interventions, care pathway, daily life/autonomy, and participation/empowerment.
- For each domain, adolescents are asked to **rank** four items from most to least important.

What matters most?





Summary of Responses (n = 4)

Theme	Key Findings
Current treatment	3/4 adolescents take their treatment “sometimes”; none take it daily
Perceived effectiveness	2/4 report their treatment “helps a bit, but I wish it worked better.”
Willingness to change	2/4 are open to changing if the new treatment “works better.”
Hospitalizations	2/4 had a hospital follow-up or stay in the past 6 months.
Daily impact	2/4 say the disease affects their daily life “a lot.”
Shared decision-making	3/4 say “we decide together” with adults.



Top Priorities (Average Rankings)



1-Relationship with Healthcare Providers

- ① “Trust the information I’m given”
- ② “Be listened to and taken seriously”

2-Experience of the Disease

- ① “Feel less pain”
- ② “Feel less tired”

3-Treatments & Interventions

- ① “Have a treatment that really works”
- ② “Avoid annoying side effects”

4-Care Pathway

- ① “Get a diagnosis quickly”
- ② “Doctors communicate well with each other”

5-Daily Life & Autonomy

- ① “Be able to go to school or do activities like others”
- ② “Go out and see my friends”

6-Participation & Empowerment

- ① “Be listened to when deciding my care”
- ② “Give my opinion about treatments”



Emerging Need (Open Question):



Two respondents highlighted the need for **mental health support** adapted to the challenges of living with a chronic condition



Key Takeaways



- **Shared decision-making** is already present but could benefit from deeper trust and listening.
- **Pain, fatigue, and treatment effectiveness** remain core concerns.
- **School and social participation** are top priorities for quality of life.
- **Mental health support** is a clear unmet need that should be addressed.



New Structure of the Questionnaire



New =Section 2: to explore the adolescent's subjective experience and support needs;

1-Your Treatment and Life with the Disease
(existing questions to keep)

Are you currently taking any treatment?
Is your treatment working well?
Would you be willing to try a new treatment?
Have you been hospitalized recently?
Does your condition affect your daily life?
Who makes the treatment decisions?

2. Your Emotional Experience and Mental Health

Goal: make the mental-health dimension and its subjective impact explicit.

Do you sometimes feel sad, discouraged, or stressed because of your illness?

Yes, often Sometimes Rarely Never

Do you have someone to talk to when you're not feeling well?

Yes Sometimes No

Have you ever felt that no one understands what you are going through?

Yes, often Sometimes No

Do you feel isolated or alone because of your illness?

Yes No

Would you like to have more psychological or emotional support?

Yes No I don't know





New Structure of the Questionnaire

3. What Matters Most to You (Please rank the following themes in order of importance to you — for example, from 1 = most important to 8 = less important)

Section 3: to allow the adolescent to express whether it is a priority for them

A. Relationship with Healthcare Professionals (existing items)

- Being listened to
- Being respected
- Receiving clear explanations

B. Living with the Disease (daily experience)

- Being able to do everyday activities
- Feeling less alone (new item)
- Feeling better mentally / less stressed (new item)
- Managing fatigue and pain

C. Treatments and Care Pathway (existing items)

- Access to new treatments
- Less side effects
- Fewer hospital visits

D. Social Life and Independence (existing items)

- Going to school or university
- Seeing friends
- Doing activities without help





New Structure of the Questionnaire

(We know that living with a rare disease isn't always easy — and sometimes, you have to make decisions about your treatments, your well-being, or what's best for you.

Today, we'd like to talk with you about something really important:

What kinds of risks would *you* be willing to take for a treatment that could work better?

Some treatments can help more, but they may also come with more side effects or uncertainties. We want to understand **how you feel about that** — and also how your parents' opinions might influence your choices.

There are no right or wrong answers.

You can share as much or as little as you want.

Everything you say will help us create better care and better support for people your age.

Let's talk, let's listen, and let's learn from each other





New Structure of the Questionnaire

(We know that living with a rare disease isn't always easy — and sometimes, you have to make decisions about your treatments, your well-being, or what's best for you.

Today, we'd like to talk with you about something really important:

What kinds of risks would *you* be willing to take for a treatment that could work better?

Some treatments can help more, but they may also come with more side effects or uncertainties. We want to understand **how you feel about that** — and also how your parents' opinions might influence your choices.





New Structure of the Questionnaire

“Have you ever had to make a decision about your treatment? Can you tell us how it went and who was involved?”



INVENTS has received funding from the European Union's Horizon Europe Research and Innovation program under grant agreement 101136365.



Funded by the Swiss State Secretariat for Education, Research and Innovation (SERI).



Funded by the UKRI Innovative UK under their Horizon Europe Guarantee scheme.

What's Next? 🤔



Thanks to your help, we were able to design a better questionnaire that really reflects what matters most to young people living with a rare disease.



Now comes the next step:

We're going to share this questionnaire with a larger group of adolescents across France to check if the priorities and feelings you helped identify are shared by others too.

This will help us make sure the tool is solid, clear, and useful — and that your voices count in the way treatments and support are imagined for young people like you.





 **"The purpose of this questionnaire is to better understand what really matters to adolescents living with rare diseases, so that future treatments, support programs, and healthcare decisions truly reflect their needs and priorities."**

◆ **For Regulatory Authorities**

- Contextualized benefit-risk assessment (based on what matters most to young patients)
- Prioritization of approval criteria (e.g., impact on school, pain, or mental health)
- Integration into patient advisory boards (ethical and participatory deliberations)

◆ **For Pharmaceutical Industry**

- Design of patient-centered clinical trials (relevant endpoints based on young patient priorities)
- Selection of preferred drug forms (e.g., oral, patches, etc.)
- Market access strategies that highlight perceived young patient benefits

◆ **Cross-cutting Utility**

- Identifies unmet needs (e.g., lack of mental health support, social isolation)
- Reinforcement of the legitimacy of patient-centered data in health technology assessment





 **For our last focus group before the summer...**

We'd like to explore a new topic with you:

 **How does it feel to decide about a treatment that's new and not yet well known?**

Together, we'll talk about:

- uncertainty,
- choices,
- risks...

and most importantly, **what YOU think!**





"What Would You Do? Choices and Risks with a New Treatment"

● Scenario A – Fast results, but unknown risks

Hope & hesitation

Your current treatment works a little, but not enough.

A new medicine could really improve your daily life (less fatigue, more energy...), but no one knows yet if there are any long-term side effects.

You would be one of the first to try it.

Discussion questions:

How do you feel about this choice?

Would you rather take a chance for quick improvement, or wait until there's more information? 





What Would You Do? Choices and Risks with a New Treatment

● Scenario B – Your parents have doubts

😞 Conflict & family pressure

Your doctor thinks a new treatment could help you.

There are some risks, but you're ready to give it a try.

But your parents are worried — they're afraid of side effects and would prefer to wait.

Discussion questions:

Would you go ahead with the treatment anyway?

How would you feel if your opinion was different from your parents'?

Do you think your parents could influence your decision?





What Would You Do? Choices and Risks with a New Treatment

● Scenario C – A friend who's unsure

🤔 Doubt & connection

Someone your age, in the same situation, is hesitating.

They say:

“What if I regret it? What if it doesn't work? But at the same time, I really want to feel better.”

They don't know what to do.

Discussion questions:

What would you say to them?

Can you relate to how they feel?

Have you ever felt the same way?








And You – Do You Feel Free to Decide?

When your doctor suggests a new treatment...

Me First 🚀	With My Parents 👨👩
I want to have the final say.”	We talk and decide together
“I know my body best.”	Their opinion reassures me.”
If the treatment helps me, I’m willing to try even if they hesitate	They handle the medical details; I trust their judgment.
I’m ready to take a risk now rather than wait.	I prefer to wait until they feel it’s safe enough
I do my own research online	We read information and decide as a family
“I’d like the doctor to speak directly to me.”	I want my parents present in every appointment.

-  = Totally agree
-  = Kind of agree
-  = Not at all

What if your opinion is different from your parents'?

What would help you feel like you're really part of the decision?





We wish you a wonderful summer! 🌞

Thank you so much for everything you shared and for being part of this adventure with us.

We'll see you after the holidays – maybe in a slightly different way – but always with the same goal: making sure your voice stays part of the project





Thank you for your participation

Follow us on [LinkedIn](#)



INVENTS has received funding from the European Union's Horizon Europe Research and Innovation program under grant agreement 101136365.



Funded by the Swiss State Secretariat for Education, Research and Innovation (SERI).



Funded by the UKRI Innovative UK under their Horizon Europe Guarantee scheme.

