

Patients Expert Group (PEG) Meeting Report 27 June 2025

Last session before the summer break

1. Why we met

- **Show the first results** of the new questionnaire that checks what really matters to young people living with rare diseases.
- **Spot and fix gaps** (we noticed mental-health questions were missing).
- **Practise making choices** about new medicines through three short stories (“scenarios”).
- **Plan what happens next** after the holidays.

2. What we learned from the questionnaire (only 4 teens answered so far)

Topic	What you told us
Treatments today	Most take their treatment sometimes, but only half feel it works well enough.
Changing medicines	Half are open to a different medicine if it would work better.
Hospital stays	Half had been in hospital or for checks in the last 6 months.
Daily life	Half said the disease affects life “a lot.”
Who decides	Nearly everyone said decisions are made together with adults.
Top priorities	1) Being listened to and respected by health-care staff. 2) Feeling less pain and tiredness. 3) Treatments that really work and have fewer side-effects. 4) Getting a quick diagnosis and clear info. 5) Being able to go to school, hang out with friends and give opinions about care.

3. Mental health matters

Two people wrote that **mental health support is missing**, so we added a new block of questions such as:

- Do you sometimes feel sad, discouraged or stressed because of your illness?
- Do you have someone to talk to?
- Do you ever feel lonely?
- Would extra emotional support help?

4. Talking through the three “what-would-you-do?” stories

Story	Most common reactions
A. New drug that could help a lot, but no one knows long-term side-effects yet.	Most of you said you would wait for more safety info before trying it.
B. You want to try a new drug but your parents prefer to wait.	You felt it is unfair if parents block you completely; decisions should involve you, parents and doctor together.
C. A friend with the same disease can't decide.	Good advice: get reliable information, check pros & cons, talk to parents and doctors , then choose.

5. How you like decisions to be made

- **Final say:** Almost everyone wants a strong voice in choices about treatment.
- **Parents' role:** You want them at appointments for support, but still want doctors to speak directly to **you**.
- **Doctors:** When parents and doctors agree, you feel safer.
- **Risk:** Most prefer to play it safe unless the disease is life-threatening right now.
- **Research:** You'd rather read info together with family than scroll alone and risk bad websites.
- **Age to go alone to appointments:** Answers ranged from **13 – 16 years**, but many still like a parent there.
- **Mental-health chat:** Some can talk about it with parents; others might prefer a different adult or a therapist.

6. Extra point added

Your environment (weather, country, access to certain medicines) can also change how you feel and what care you get. We will keep that in mind.

7. What happens after summer

1. **Update the questionnaire** with the new mental-health and environment parts.
2. **Share it with more teens** across France to check if the results stay the same.
3. **Invite medicine regulators** (the people who approve drugs) to meet us in future sessions.

4. **Plan September-December meetings**—dates will be sent soon.
5. Keep working so that future studies, treatments and support programmes **match what matters to you**.

8. Quick reminders

- **Enjoy the holidays!** Use sunscreen, rest and have fun.
- The team will be back in touch at the end of August with the next meeting date and an updated survey link.

***Thanks for your ideas and honesty.
Your voices are shaping real changes!***