

INVENTS Project – Patient Expert Group (PEG)

Online kick-off meeting – 24 January 2025, 16:01-17:35 (CET)

1 1. Attendance

Representatives	Organisation / Role
Vincent Montero	INSERM-OrphanDev – Pharmacist, rare-disease network lead
Yanis Mimouni	INSERM – Pharmacist, paediatric drug-development researcher
Dr Stephanie Gentile	INSERM – Public-health physician, patient-experience lead
Dr Vincent Damotte	INSERM – Scientific project manager, epidemiology & genetics
Viviana Giannuzzi	Fondazione Gianni Benzi / TEDDY Network – Regulatory science
Dr Emanuela Crea	TEDDY Network – Scientific secretary
Francesco Lapenna	TEDDY Bari YPAG – Youth-engagement coordinator

Patient participants **Valentina (12, NL/F); Teresa (18, IT); Charlotte (17, FR)**

Apologies: Donato Bonifazi (TEDDY Network) – last-minute commitment.

Recorder: Automatic MS Teams transcript

2 2. Agenda

1. Welcome & introductions
 2. Recap of INVENTS objectives
 3. Explainer: how medicines are discovered, trialled and authorised
 4. What “patient experience” means in this context
 5. Open discussion with adolescents:
 - Why their opinion matters for rare-disease trials & approvals
 - If / how they wish to give input
 - Practical and ethical preferences for collecting that input
 6. Next steps & closing
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3 3. Key discussions & outcomes

#	Topic	Discussion highlights	Conclusions / decisions
3.1	INVENTS overview	Team explained that INVENTS develops new trial-design and modelling methods for small paediatric rare-disease populations and wants regulators to accept them. Patient insight will be one of those methods.	Patients confirmed a basic grasp of the project aims.

#	Topic	Discussion highlights	Conclusions / decisions
3.2	Drug-development journey	Step-by-step walkthrough (discovery → pre-clinical → phases 1-3 → marketing authorisation → post-marketing pharmacovigilance). Emphasis on where patient input can shift a go/no-go decision.	Adolescents valued seeing where their voice “fits” in the lifecycle.
3.3	Defining “patient experience”	Facilitators illustrated experience as <i>how the medicine affects daily life, emotions, symptoms, trust in staff, etc.</i> Example: injection pain vs. blood-test improvements.	Group agreed mental-health and quality-of-life aspects must be measured, not just biological markers.
3.4	Will you give your opinion on which medicines should be developed?	Valentina & Charlotte: Yes ; Teresa: Unsure / No	Diversity of willingness noted; participation must remain voluntary.
3.5	Would you advise on <i>how</i> research is run (end-points, burden, etc.)?	Charlotte keen to discuss endpoints directly with developers; Valentina would “trust doctors but wants a say”.	Adolescents <i>can</i> contribute to endpoint selection if properly supported.
3.6	Preferred channels to give input	<ul style="list-style-type: none"> • Mobile/desktop app with pictorial body map and short questionnaires (Valentina) • Social-media-style tool usable on phones (Charlotte) • Must allow anonymity; some still fear identification even if data are anonymised. 	INVENTS team to scope a confidential, cross-platform digital diary/app prototype.
3.7	Who should speak for children?	Adolescents prefer peer representatives of the same age & condition over parents or doctors, who “don’t feel what we feel”.	Future consultations should prioritise peer-led or self-report models.
3.8	Ethical considerations	Confidentiality reaffirmed; recordings shared view-only; Miro board left open for asynchronous input.	All data to be kept confidential; assent/consent procedures to be detailed in next session.

4 4. Action items

#	Action	Responsible	Due
1	Circulate slide-deck, meeting recording (view-only) and Miro-board link.	Francesco Lapenna	29 Jan 2025
2	Keep Miro board open for at least 2 weeks for additional adolescent inputs.	All PEG facilitators	07 Feb 2025
3	Draft specification for confidential, dual-platform feedback app (incl. visual body-map).	V. Damotte & tech subgroup	Before next PEG
4	Prepare short briefing on data-privacy safeguards for minors.	S. Gentile	Next PEG

#	Action	Responsible	Due
5	Schedule next PEG virtual meeting (target late February).	Secretariat	05 Feb 2025

5 5. Decisions recorded

1. **Patient-feedback tool** will be digital, cross-platform, and designed with strong anonymity safeguards (exact format to be drafted).
 2. Peer-to-peer representation (same age/disease) is preferred; parent or clinician reporting only as fallback.
 3. Current Miro board remains the central asynchronous workspace until the app is tested.
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6 6. Next meeting

- **Proposed window:** last week of February 2025 (secretariat to poll availability).
- **Draft agenda:** review app concept, confirm ethical framework, continue co-design of patient-experience endpoints.