ERDERA

European Rare Diseases
Research Alliance

Making Europe a world-leader in rare diseases research and innovation

ERDERA Young Patients Training, Athens, 2025

ERDERA Training for Young Advocates for Rare Diseases, Paris, 2026

Co-funded by
the European Union

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Individually rare, collectively common



Defining the Invisible

European Union



< 5 in 10,000 individuals

United States



< 200,000 individuals total

Key Insight: The lack of a singular global definition creates barriers to recognition. These conditions are often termed 'Orphan Diseases'—overlooked and neglected.

Heterogeneity of Rare Diseases

8000+

distinct conditions fall under Rare Renal Disorders (RRDs) in Europe,
spanning nearly all paediatric kidney diagnoses

1

Clinical variability

Age of onset, severity, and disease trajectory differ widely even within the same diagnosis — many AYAs describe their condition as unpredictable and ever-changing.

2

Multisystem complexity

RDs frequently involve more than the kidneys — physical, cognitive, and psychosocial systems are affected together, demanding input from multiple specialities at once.

3

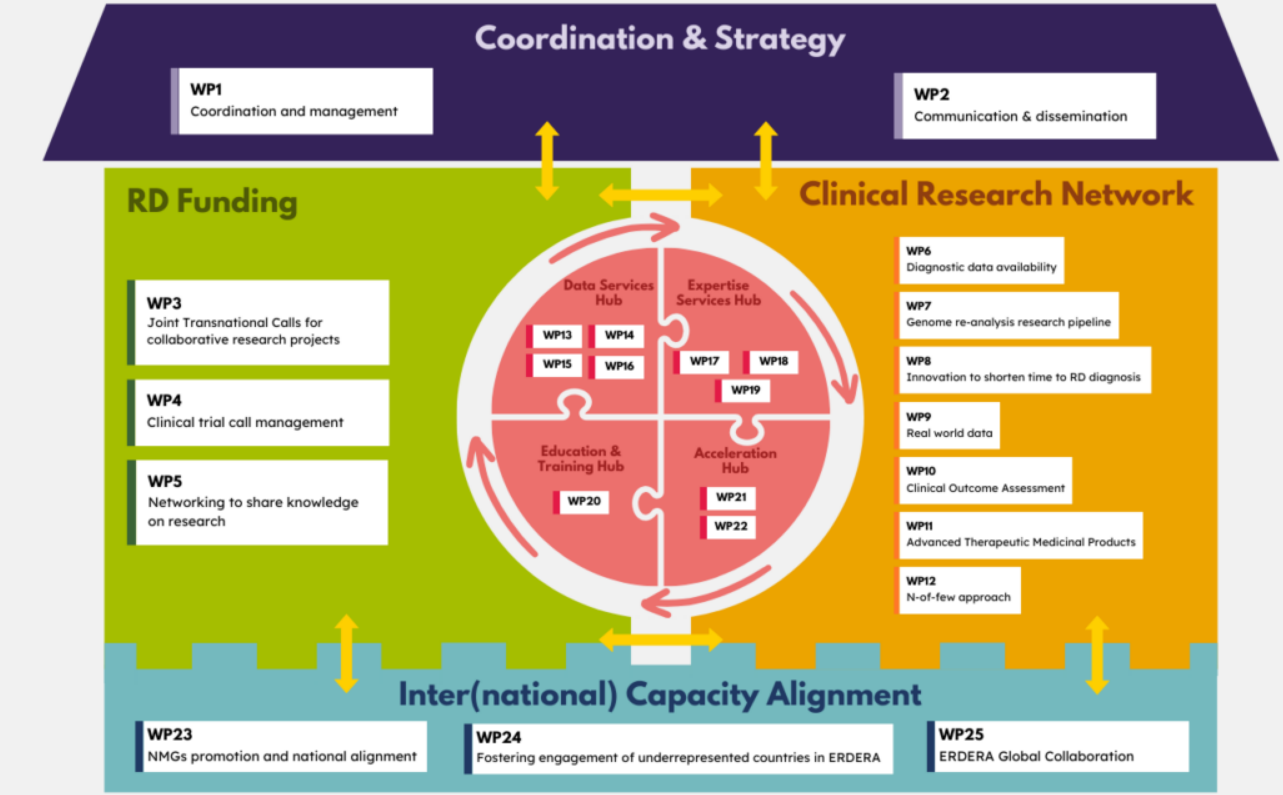
Diverse treatment pathways

Gene Therapy, Enzyme Replacement Therapies, transplantation, and alternative therapies each carry distinct burdens and timelines, so care and transition planning cannot follow a single template.

4

Rarity → thin adult expertise

Because each condition is individually rare, adult specialists with relevant experience are few and concentrated in a handful of tertiary centres.





WP13
Rare Diseases-Virtual Platform (RD-VP): Finding and accessing the data ecosystem

WP14
Data readiness services

WP15
Data sharing and analysis services

WP16
Knowledge bases and ontologies for RD research

WP17
Mentoring and consultancy

WP18
Regulatory support service

WP19
Methodological Support

WP20
Education and training in rare diseases research

WP21
Technology accelerator

WP22
Public-Private Collaboration Accelerator

Work package WP20 – Education and Training on RD Research

Subtask 20.1.2 Training workshops for paediatric patients

Subtask leader: TEDDY. Contributors: Institut IMAGINE, EURORDIS and conect4children (c4c)

This subtask aims to develop an annual, age-appropriate training programme for young people (12-18 years old) with rare diseases, equipping them with the knowledge and skills to actively engage in rare disease research.

ERDERA Young Patients Training so Far…



1. ERDERA – Young Patients Training, Athens 2-3 May 2025
2. Young patients' feedback from the 1st training
3. 2nd ERDERA Training for Young Advocates for Rare Diseases, Paris, 2026
4. Young patients' feedback from the 2nd training
5. Key takeaways for the 3rd edition

ERDERA – Young Patients Training

Athens, 2–3 May 2025

Preparation — TEDDY opened a call for applications and asked everyone to fill in a short test beforehand.

The test showed what people already knew, so the sessions could be built at the right level.

TEDDY also spoke with the speakers about what young patients wanted to learn and collected the consent and assent forms.

Who came — 23 young people from seven Young People's Advisory Groups: ANDO Portugal, eyeYPAG London, KIDS Albania, KIDS Bari, KIDS Rare Hellas, KIDS Umbria and RAiN Ireland.

85% were under 18; 54% had a rare disease

Format — YPAG presentations, expert lessons, interactive sessions

Explore the details of the first edition of the ERDERA Training through the information available on the TEDDY website: ERDERA – Young Patients Training, Athens 2-3 May 2025

ERDERA – Young Patients Training, Athens 2-3 May 2025

Topics covered:

- Rare and Genetic Diseases Specificities
- Patients' and Children's Rights
- Translational Research Challenges
- Shared Activities from the TEDDY KIDS Network
- Patient Engagement in Clinical Trials
- Orphan Medicinal Products & Pharmacovigilance

AGENDA

TRAINING SESSIONS

SPEAKERS

Success drivers of the event

EDUCATIONAL TRAINING

Educating young people is essential for incorporating their voices into the research and drug development process, communicating unmet priorities to stakeholders, and enabling them to participate in decisions regarding their health.

MUTUAL ENRICHMENT

Young participants shared their lived experiences, offering experts valuable insights into the real needs and priorities of rare disease patients.

Researchers and healthcare professionals provided young attendees with knowledge of medical and ethical topics, supporting their development of a deeper understanding of the research landscape.







AREAS FOR IMPROVEMENT

YOUNG PATIENTS' FEEDBACK



2nd ERDERA Training for Young Advocates for Rare Diseases

Paris, 23–25 April 2026

Preparation —TEDDY opened a call for applications.

Before the training, the young advocates answered a set of questions on rare diseases, genetics, consent and clinical trials so the second ERDERA Young Patient Training could be built at the right level.

TEDDY, Institut Imagine and EURORDIS met online to agree on the learning objectives and the experts who would lead the sessions

Who came —22 young advocates, 12–21 years old living with a rare disease from 14 organisations across Europe: Young People's Advisory Groups, national rare disease alliances, disease-specific patient associations, a research network and a patient expert group.

Format: participation across the three days: from listening to experts, to discussing with them, to working alongside them on the design of a paediatric study

Youth Participation

2nd ERDERA Training for Young Advocates for Rare Diseases

Representatives from Young Persons Advisory Groups (YPAGs) and patient organisations introduced their work

KIDS Bari Italy	**KIDS Albania** Albania	**KIDS Kosova** Kosovo	**POLPEDNET KIDS Warsaw** Poland
KIDS Rare Hellas Greece	**eyeYPAG** European YPAG for Eye Diseases	**ANDO Portugal** Bone Diseases Association, Portugal	**ADEE** Ectodermal Dysplasia Association, Spain
INVENTS PEG Patient Expert Group, INVENTS Project	**AEV ODV** Bladder Exstrophy-Epispadia, Italy	**RAiN Ireland** All Ireland Rare Diseases Research Network	**SMA Georgia** Spinal Muscular Atrophy Patient Community
Pro Rare Austria Alliance for Rare Diseases, Austria	**APPDH** ADHD Association, Portugal		

Their presentations highlighted the importance of peer exchange, patient representation, and youth engagement in shaping future research priorities.

Explore the details of the 2nd ERDERA Training for Young Advocates for Rare Diseases through the information available on the TEDDY website: Link

Summary reports of the three training days were published through dedicated news:

ERDERA Young Advocates Training Opens in Paris: Day 1 Focuses on Advocacy and Drug Development

ERDERA Young Advocates Training Continues in Paris with Focus on Clinical Research and Patient Engagement

ERDERA Young Advocates Training Concludes in Paris with Focus on Patient Rights and Research Partnership





ERDERA Training for Young Advocates for Rare Diseases

Topics Covered

- Understanding Clinical Trials, Protocols and Pharmacovigilance in Pediatrics
- Patient Engagement in Clinical Trials
- Rare and Genetic Diseases specificities
- Round Tables: How Can Patients Participate in Translational Research Projects?
- Introduction to INVENTS and the decision matrix
- What is a Patient Registry? Case discussion: Retrospective vs Prospective studies
- Patient's and Children's Rights
- Working session: Young Patients as Partners in Research

Click AGENDA

Click TRAINING SESSIONS

Click SPEAKERS

Young Patient's Feedback (Mentimiter)



Belonging and peer connection

- The strongest thing the young advocates took home was each other. Asked what they would take away from the training, "friends" was the most common answer:

"probably about the awesome people I met there"; "I met new friends and we talked about things related to research and clinical trials"

- They expressed relief being among people who already understand what is like living with a rare disease:

"I got to know better the other kids and the specifics of their disease"; "That I'm not the only one that feels alone and misunderstood"

- By the end, they were describing themselves as a group with a shared position, making claims on behalf of the group rather than at an individual level:

"our rights", "our role", "our priorities", "our own rare disease journey"

Young Patient's Feedback (Mentimiter)



Voice and rights

- Asked to explain what they did in Paris, participants described advocacy not as people who learned about advocacy, but as people who can do it:

"I learned how to speak up for the version of myself that people don't know."

"I learned a lot about my rights, how to advocate for myself and others."

"I studied to be able to advocate, meaning protect the right of the kids."

Young Patient's Feedback (Mentimiter)



Learning through active participation

- The taught sessions were praised for clarity, being eye-opening, and feeling comfortable in the room to ask questions and speak up:

"I felt comfortable asking anything I was curious about."

- The advocates developed a good relationship with the clinicians and experts delivering the sessions

- The participatory sessions were described as transformative:

"The best thing we did, interesting and important at the same time, really nice to work with the group."

"One of the most important workshops of this training."

"THE BEST"

"Very interactive"

Young Patient's Feedback (Mentimiter)

Areas for improvement

- **Density**- young advocates asked for a longer training to split the dense schedule and avoid listening for consecutive hours.
- **A group at different levels.** The same training sessions were described as "basic information" by some participants and as confusing by others. Participants proposed the remedy themselves: *"content tailoring"* and material sent out to read in advance.

Young Patient's Feedback (Mentimiter)

Looking into the future

Over three days at the second ERDERA Training for Young Advocates for Rare Diseases, participants moved from learning about rare disease research to seeing themselves as people who can actively contribute to and shape rare disease research:

"patients as partners"

"getting into research"

"kids at parliament"

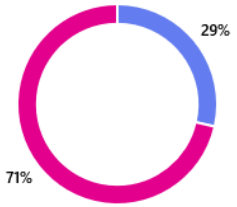
"a training on how to start making action in our own communities as young people".

YOUNG ADVOCATES' FEEDBACK (from second form)

1. How satisfied are you with the knowledge you gained from the training?

Più dettagli

Very satisfied	4
Satisfied	10
Neither satisfied nor dissatisfied	0
Dissatisfied	0
Very dissatisfied	0



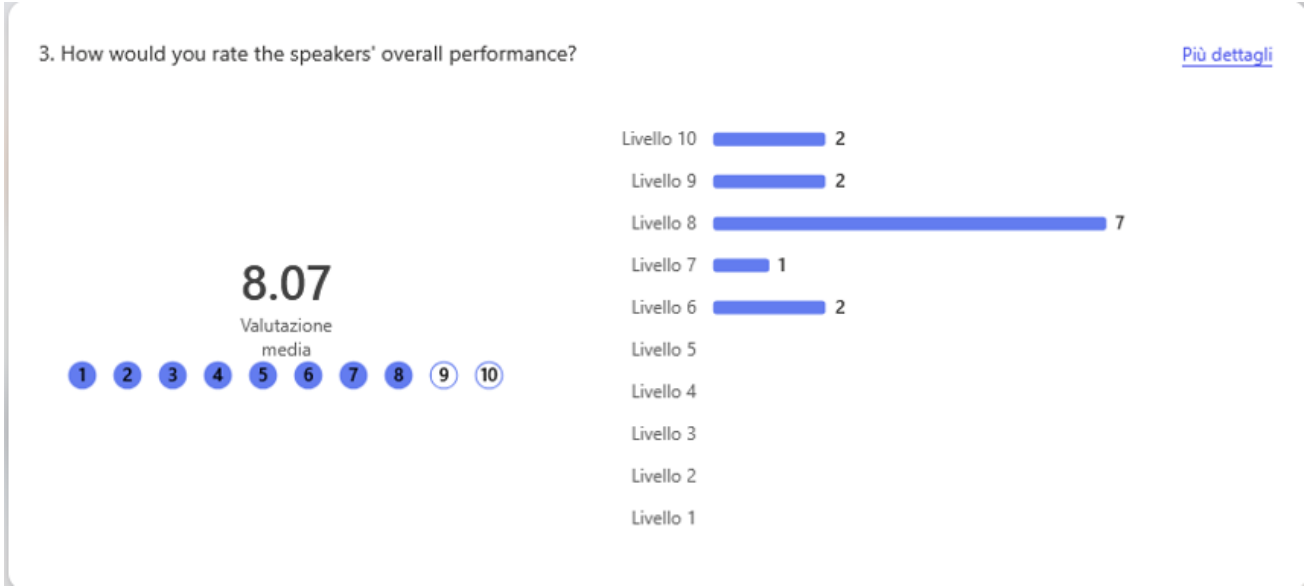
2. Do you feel you achieved your desired learning outcome?

Più dettagli

Yes	13
No	0
Not sure	1



YOUNG ADVOCATES' FEEDBACK (from second form)

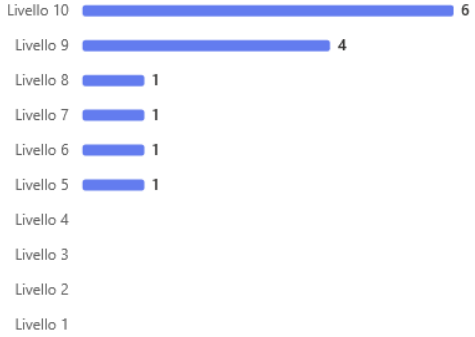


YOUNG ADVOCATES' FEEDBACK (from second form)

10. Did you get the support you needed from organizers before and during the training?

Più dettagli

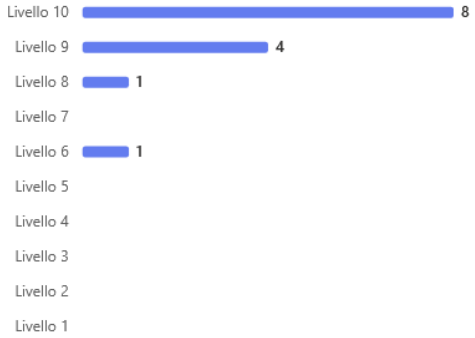




11. How likely are you to recommend this training to one of your peers?

Più dettagli





YOUNG ADVOCATES' FEEDBACK (from second form)

12. Do you have any additional comments or suggestions you would like to share with us? Help us improve!

4 Risposte

ID ↑	Nome	Risposte
1	anonymous	The time management could be improved
2	anonymous	Make sure to simplify to understand medical information more in the future and make it more interactive focusing on real life practices.
3	anonymous	I stayed in a separate accommodation
4	anonymous	The training was highly valuable, well-structured, and engaging. I particularly appreciated the participatory approach and the focus on including young people's voices in research. It created a safe and motivating environment for discussion and learning. Thank you to the organizers for the excellent support and coordination.

What the two training editions point to

Active participation. Both editions identify participatory sessions as the element that most contributes to learning.

Cooperation across groups. Young patients at the first ERDERA training called for more cross-group interaction

Uneven prior knowledge. Young patients arrive with different levels of understanding of the session topics. Participants asked for content tailoring and study materials to be sent in advance.

From belonging to a role. Across both editions, young patients moved from receiving information about rare disease research to asking how they could shape it: *“patients as partners”; “getting into research”; “kids at parliament”; “a training on how to start making action in our own communities as young people”.*

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European **Rare Diseases** Research Alliance

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