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YOUNG PATIENTS AS PARTNERS IN RESEARCH

An Interactive Working Session
ERDERA 2nd Young Patients Training

Imagine Institute, Paris – France
25/04/2026

Co-funded by the European Union

ERDERA European Rare Diseases Research Alliance

ERDERA has received funding from the European Union's Horizon Europe research and innovation programme under grant agreement N°101156595.
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HOW WE CHANGE THE WAY RESEARCH IS DONE

ACTIVITY	FORMAT
Icebreaker: The Web of Words	Whole group
INVENTS PEG Testimonials	Talk-Show
Anatomy of a Clinical Trial Protocol	Presentation
Where Can YOU Make a Difference?	Interactive mapping
Small-Group Co-creation Exercise	3 groups
Plenary Restitution & Wrap-up	All

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ICEBREAKER: THE WEB OF WORDS

THE ACTIVITY

We try to face each other.

Each person states their name and **one word** that comes to mind when they hear "**Clinical Research**" then throws the ball to another participant while holding onto the end of the string.

That participant repeats the process, throwing the ball to someone else across the room.

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WHY WE HAVE DONE THIS

Research is never done in isolation. It requires a network of connected individuals — researchers, doctors, and most importantly, patients.

THIS WEB VISUALISES THE PARTNERSHIP WE ARE BUILDING TODAY.

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INVENTS Patients Expert Group Testimonials

INVENTS has received funding from the European Union's Horizon Europe Research and Innovation programme under grant agreement 101136365. This work is further supported by the Swiss State Secretariat for Education, Research and Innovation (SERI) and by the UKRI Innovative UK under their Horizon Europe Guarantee scheme.

INVENTS PUTS YOUNG PATIENTS AT THE HEART OF RARE DISEASE RESEARCH

THE INVENTS PROJECT

(Innovative designs, extrapolation, simulation methods and evidence-tools for rare diseases addressing regulatory needs) is a 5-year Horizon Europe project (2024–2029) coordinated by Inserm. It aims to improve clinical trial methods for rare and paediatric diseases, so that patients can access effective treatments faster.

WORK PACKAGE 6 & THE PEG

Work Package 6 is dedicated to ethical, legal and patient engagement issues. At its core is the **Patient Expert Group (PEG)**: a group of young patients with rare diseases who were trained, consulted, and actively involved in shaping the innovative methods

> *Patient perspectives will be incorporated into a quantitative criterion reflecting patients' experiences to authorities.*
>
> — INVENTS, WP6

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YOUNG EXPERTS — THEIR STORY, IN THEIR OWN OWN WORDS

What was your biggest surprise when you you joined the PEG?

How did your involvement change the way you see medical research?

What would you say to a young patient patient who is hesitating to get involved? involved?

What is the one thing you are most proud of from your time in INVENTS?

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Anatomy of a Clinical Trial Protocol

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A CLINICAL TRIAL PROTOCOL IS A BLUEPRINT — AND EVERY BLUEPRINT HAS PLACES WHERE PATIENTS CAN LEAVE THEIR MARK

A clinical trial protocol is the detailed plan that describes how a study will be conducted.

It is the document that researchers, regulators, and ethics committees review before a single patient is enrolled.

UNDERSTANDING ITS STRUCTURE IS THE FIRST STEP TO INFLUENCING IT.

1. **STUDY RATIONALE & OBJECTIVES**
   Why the study is needed and what it aims to prove
2. **STUDY DESIGN**
   Type of trial (Randomised Controlled Trial, open-label, etc.), duration, phases
3. **ELIGIBILITY CRITERIA**
   Who can and cannot participate
4. **INTERVENTIONS**
   Treatment, dose, route of administration, comparator
5. **OUTCOME MEASURES**
   What will be measured to assess success (endpoints)
6. **PARTICIPANT EXPERIENCE**
   Visits, procedures, burden, consent and assent
7. **SAFETY & ETHICS**
   Monitoring, stopping rules, rights of participants

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Where Can YOU Make a Difference?

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PATIENTS DON'T JUST PARTICIPATE IN TRIALS — THEY THEY CAN SHAPE EVERY PART OF THEM

Not all parts of a protocol are equally open to patient influence. Some are highly technical and driven by regulatory requirements.

Others are deeply human; and that is exactly where the voice of young patients is most powerful and most needed.

PROTOCOL SECTION SECTION	LEVEL OF PATIENT INFLUENCE	WHY IT MATTERS
Study Rationale & Objectives	★★★★★ HIGH	Patients define what questions matter most
Study Design	★★★ MEDIUM	Patients can flag burdensome or unrealistic designs
Eligibility Criteria	★★★★ HIGH	Patients identify exclusions that are too restrictive
Interventions	★★★ MEDIUM	Patients flag unacceptable routes/frequencies
Outcome Measures	★★★★★ HIGH	Patients define what "getting better" means to them
Participant Experience	★★★★★ HIGH	Patients know the real burden of participation
Safety & Ethics	★★★★ HIGH	Patients define acceptable risk and rights

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BEFORE ANY STUDY BEGINS — DOES IT ASK THE RIGHT QUESTION?

The study rationale is where everything starts.

If the question being asked does not reflect what patients actually need, the whole study is built on the wrong foundation.

TRIGGER QUESTIONS FOR PARTICIPANTS

1. "If you could ask researchers ONE question about your disease that no one has answered yet, what would it be?"
2. "Have you ever been through a treatment that worked medically, medically, but made your daily life much harder? What would would you have wanted researchers to measure instead?" instead?"
3. "Who do you think should decide which diseases get studied studied first, researchers, companies, or patients? Why?"

WHO GETS IN — AND WHO GETS LEFT OUT — IS A A DECISION PATIENTS CAN CHALLENGE

Eligibility criteria determine who can participate in a trial. They are often written by researchers and can unintentionally exclude patients who would benefit most.

Study design determines the structure of the trial — and some designs are far more burdensome than others.

TRIGGER QUESTIONS FOR PARTICIPANTS

1. "Have you or someone you know ever been told you couldn't join a clinical trial? How did that feel? Do you know why?"
2. "If a trial required you to come to hospital every week for 2 years, would you join? What would make it more manageable?"
3. "Some trials compare a new treatment against a placebo (no (no treatment). If you were very sick, how would you feel about feel about being in the placebo group? What would you want to want to know before agreeing?"

"GETTING BETTER" MEANS DIFFERENT THINGS TO TO DIFFERENT PEOPLE — PATIENTS DEFINE WHAT WHAT COUNTS

Outcome measures — also called endpoints — are what a trial measures to determine if a treatment works. Traditionally, these have been defined by researchers and clinicians.

But what researchers measure is not always what matters most to patients.

TRIGGER QUESTIONS FOR PARTICIPANTS

1. **"Think about a treatment you have received. What changed in your life that made you feel it was working, and was that what the doctors were measuring?"**
2. **"If you were designing a trial for your disease, what would you measure to know if a treatment was truly helping? School attendance? Energy levels? Pain? Social life?"**
3. **"Have you ever heard of a drug being approved because it it worked in a clinical trial, but patients said it didn't really help really help them in real life? Why do you think that happens?" happens?"**

THE REAL COST OF PARTICIPATING IN A TRIAL IS PAID BY THE THE PATIENT — NOT THE RESEARCHER

The participant experience section covers everything from the number of hospital visits, to blood draws, to questionnaires, to travel requirements.

For children and adolescents with rare diseases, this burden can be enormous — and it is often underestimated by researchers.

TRIGGER QUESTIONS FOR PARTICIPANTS

1. **"Think about the most difficult part of being a patient, not the disease itself, but the experience of being treated. What was hardest? Hospital visits? Missing school? Side effects? Uncertainty?"**
2. **"If you were in a clinical trial, what would make you want to stay in it until the end? And what would make you want to drop out?"**
3. **"Researchers often say a study is 'low burden.' What does burden burden mean to you as a young patient? Is it the same as what a what a researcher might think?"**

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CO-CREATION

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FROM REFLECTION TO ACTION: THE CO-CREATION EXERCISE

Everything you have just reflected on — the questions that matter, who gets included, what gets measured, what the experience feels like — is exactly what you will now put into practice.

This step will be led by **Nicole Chemaly (APHP), Yanis Mimouni (C-Path), and Mark Turner (c4c) and Donato Bonifazi (Teddy Network)**. You will be divided into three groups to co-create the outline of a paediatric clinical study.

CO-CREATION EXERCISE STRUCTURE

ELEMENT	WHAT YOUR GROUP WILL DEFINE
Disease & Rationale	What disease? What question does the study answer?
Study Design	What type of trial? How long?
Who Participates	Age range, eligibility, inclusion/exclusion
What Is Measured	Primary and secondary endpoints — from a patient perspective
Patient Experience	Visits, burden, consent/assent, communication

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THE CO-CREATION EXERCISE: HOW TO BUILD YOUR PROTOCOL

1

THE PATIENT PERSONA

Before writing the protocol, you must know who it is for. You have Persona Cards, each with with a name, an age, and an outline of their daily struggles with the rare disease.

USE MARKERS & LARGE PAPER

2

THE PROTOCOL CANVAS

Move to the large Protocol Canvas Bord. Discuss each of the 5 protocol elements (Design, Eligibility, Outcomes, etc.). Write your group's decisions on sticky notes and place them on the corresponding sections of the canvas.

USE STICKY NOTES & CANVAS

3

THE PITCH

Review your completed canvas. Does it address the needs of the persona you drew in Step 1? Select a spokesperson from your group to present your patient-centred protocol outline to the rest of the room.

PRESENT TO THE ROOM

Group 1: Bones, Joints & Movement
Group 2: Blood, Vessels & Organs
Group 3: Nerves, Eyes & Skin

Small-Group Co-creation Exercise

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Wrap-up

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YOUNG PATIENTS ARE NOT THE SUBJECT OF RESEARCH — THEY ARE ITS CO-AUTHORS

FIRST

Patient involvement is not symbolic. Young patients can contribute meaningfully to trial design and scientific frameworks; when given proper training and genuine partnership.

SECOND

Every part of a clinical trial protocol has a place for the patient voice. The most powerful contributions come from those who know what it means to live with a rare disease every day.

THIRD

The best clinical studies are co-designed. What you have done today is not a simulation. It is a rehearsal for the kind of collaborative, patient-centred research that the field needs more of.