ERDERA
European Rare Diseases
Research Alliance

Active Patient Involvement in Clinical Trials: PROMs, PREMs, and PISs

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What is a Clinical Trial?

A **clinical trial** is a research study conducted with **human participants** to evaluate the **safety**, **effectiveness**, and side effects of new treatments, medications, or medical devices.

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THE ACTIVE ROLE OF PATIENTS IN CLINICAL TRIALS

In the past, patients were seen as passive participants—they just followed instructions.

Today, clinical research involves patient engagement, where patients are treated as partners who share their experiences, priorities, and preferences during the entire trial process.

This shift allows researchers to better understand what matters most to patients, helping to improve treatments and care.

What is Patient Engagement?

Patient Engagement in clinical trials refers to the meaningful and active collaboration between patients or their caregivers, and researchers throughout the entire research process—far beyond simply enrolling or completing study procedures.

Key elements include:

Active Partnership

Co-Creation of Trial

Continuous Two-Way Dialogue

What is Patient Engagement?

Active Partnership

- Patients are treated as co-designers rather than passive “subjects.” They participate in defining research questions, selecting endpoints, and shaping study protocols.

Co-Creation of Trial Materials and Processes

- Patients review and help simplify informed-consent documents, questionnaires, and recruitment materials.
- They advise on visit schedules, location choices, and burden-reduction strategies to make participation feasible.

What is the role of the patient in a clinical trial?

The patient is an important part of the research. They share their thoughts, experiences, and feedback, which help researchers understand if the treatment works and how it affects them.

Why is patient involvement so important?

Only the patient can explain how they feel during the treatment, what side effects they notice, and whether the treatment improves their life.

How can I know if your voice matters in the study?

Most studies have ways for patients to share their feedback, like surveys or follow-up meetings, to make sure their opinions are heard.

Patient's Voice in Clinical Research

One important way to include patients' voices is through the use of **Patient Reported Experience Measures (PREMs)** and **Patient Reported Outcome Measures (PROMs).**

These **tools** help researchers **understand how patients feel about their care** and how their health is affected by treatments.

Let's explore these two important concepts further.

Patient's Voice in Clinical Research

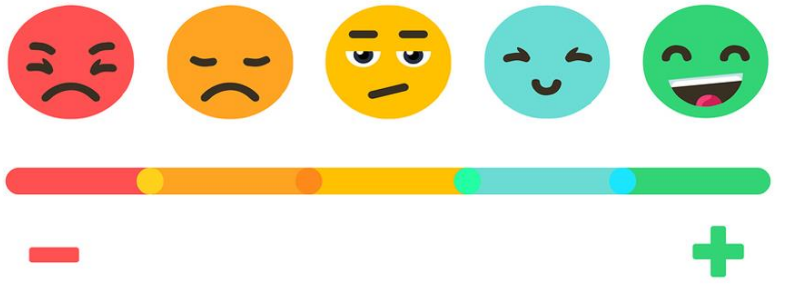
What are Patient-Reported Outcome Measures (PROMs)?

PROMs are standardized questionnaires used to collect information directly from patients about their health status, symptoms, emotional well-being, functional ability, and quality of life.

They are typically administered before, during, and after a treatment to assess changes over time from the patient's perspective.

Examples of what PROMs measure:

- Pain intensity
- Fatigue
- Anxiety or depression
- Physical and social functioning



Patient's Voice in Clinical Research

What are Patient-Reported Experience Measures (PREMs)?

PREMs assess a patient's experience with the healthcare process.

They capture how the patient felt during their care journey, including the quality of communication, involvement in decisions, and accessibility of services.

Examples of what PREMs assess:

- Was information about your treatment clear and understandable?
- Were you involved in making decisions about your care?
- Was it easy to access the healthcare services you needed?

Using PROMs and PREMs together offers a comprehensive view of the patient's journey:

- ✅ **PROMs show how patients are doing,**
- ✅ **PREMs show how patients feel about their care.**

How PROMs & PREMs Are Digitally Enabled?

Digital Data Collection

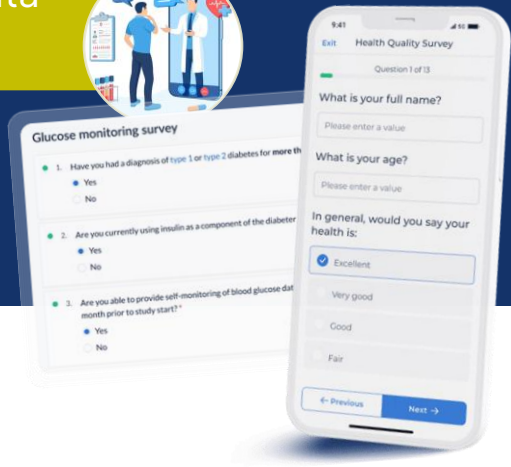
- **Web Platforms and Apps** Patients can complete PROMs and PREMs via secure online portals or mobile apps anytime, anywhere.
- **Wearables**: Devices like smartwatches or fitness trackers collect health data (e.g., activity, sleep) that can be combined with PROM data.

Integration with Electronic Health Records (EHRs)

- **Real-Time Data:** Data from PROMs and PREMs is automatically added to the patient's electronic health record (EHR), allowing doctors to view it immediately and make better decisions.

Real-Time Data Analysis

- **Better Decision-Making:** With real-time data, doctors can act quickly if they notice any changes in the patient's condition.



Why Combine PROMs & PREMs with Technology?

- Patients can answer online, at home or in the hospital
- Results are available immediately

Faster feedback

- Easier to collect answers from many people using apps, SMS, or e-mail

Reach more patients

- Monitor progress, not just one moment
- Useful for chronic conditions or recovery after surgery

Track over time

Benefits of Digitalization

✅ **Patient Engagement:**

Patients can actively participate in their health management by easily completing the questionnaires.

✅ **Better Health Management:**

Doctors can make faster and more personalized decisions based on the immediate availability of the data.

The Role of Young Patients in Clinical Research

Young patients play a crucial and growing role in clinical research. Their involvement not only enriches scientific understanding but also ensures that treatments are more effective, ethical, and truly responsive to their needs.

How Can Young Patients Contribute?

Direct participation in clinical trials-

By taking part in studies, children and adolescents provide essential data on how treatments work for young people.

Membership in advisory groups-

Young Persons' Advisory Groups (YPAGs) allow young people to review study designs, materials, and consent documents, ensuring they are clear, appropriate, and respectful.

Empowering Young Participants: Tools and Resources

Accessible educational materials
Comic books, animated videos, story-based guides, and visual aids help explain clinical research in a clear and engaging way.

Patient Information Sheets (PISs) are written documents provided to potential participants in a Clinical Trial. It clearly explains the purpose of the study, procedures, risks and benefits, and participant's rights.

Training
Programs that prepare youth for advisory or research roles.

Safe, inclusive feedback spaces
Youth forums, discussion groups, and surveys help gather honest input from young patients in a respectful way.

DEEP (DEferiprone Evaluation in Paediatrics)

The project aims to gather more information on the use of deferiprone in children with chronic iron overload.

A new liquid version of deferiprone, suitable for children, has been developed.

Once the studies are completed, a Paediatric Use Marketing Authorisation (PUMA) application will be submitted. Currently, two clinical trials and one observational study are ongoing to evaluate the safety and effectiveness of the medicine in children.

What is deferiprone?

Deferiprone is a medicine used to treat iron overload, a condition where the body has too much iron.

What is a Booklet ?

Imagine a booklet like a small book with lots of pages, full of pictures, drawings, and easy words to understand. It's a little book that helps you learn something in a simple and fun way. It’s often used to explain stories, important information, or how to do something.

DEEP (DEferiprone Evaluation in Paediatrics)

Booklet 0-6

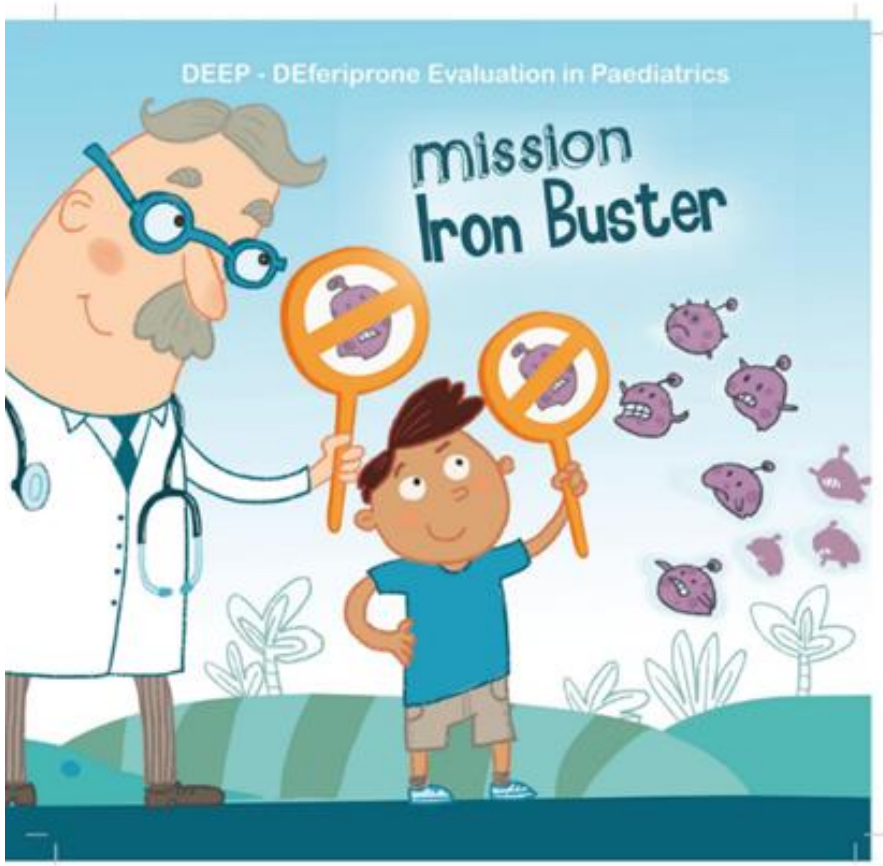
Created by


Jack and Sarah have got anemia.
This year they want to find out whether Doctor Ironless's new syrup works as well as the tablets do.
Once a week they go to the hospital.
Nora the nurse takes a sample of their blood.
Once a month,
they have an appointment with Doctor Ironless.
At home,
Sarah takes the syrup three times a day.
Jack takes
one tablet every day, dissolved in water.
At the end of the year, the doctor will find out whether he can give his new syrup to other children.
Do you want to be like Jack and Sarah?

DEEP - 2 EudraCT number 2012-000353-31

Multi-centre, randomised, open label, non-inferiority active-controlled trial to evaluate the efficacy and safety of deferiprone compared to deferasirox in paediatric patients from 1 month to less than 18 years of age affected by transfusion-dependent haemoglobinopathies. Funded by the European Commission under the Seventh Framework Programme for Research and Technological Development (FP7 Project - SP1 - Cooperation HEALTH-F4-2010-261483)

Version 2.0 Release date 30/09/2013

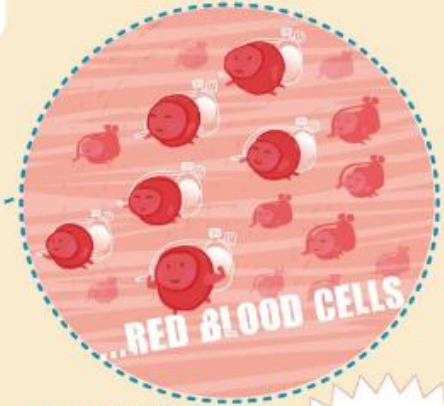

DEEP - DEferiprone Evaluation in Paediatrics
mission
Iron Buster

Now is your chance to ask Dr. Ironless

WHAT'S IN THE BLOOD?

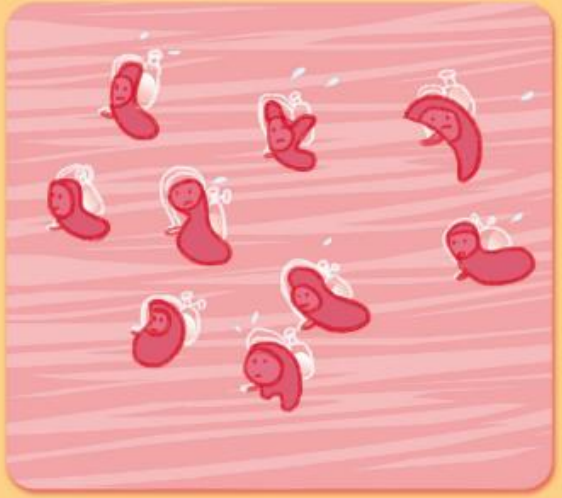
Goodness me! Do you mean to say you've never looked inside a drop of blood? Well, I'll show you then.

Inside each and every drop of blood are millions of little "soldiers". They are called...



They do a really important job: they are the ones who bring us **oxygen**, the fuel our body needs to work properly.

But when there aren't enough red blood cells and they're not feeling well, our bodies run out of fuel. All because of a family of diseases called...



ANEMIAS

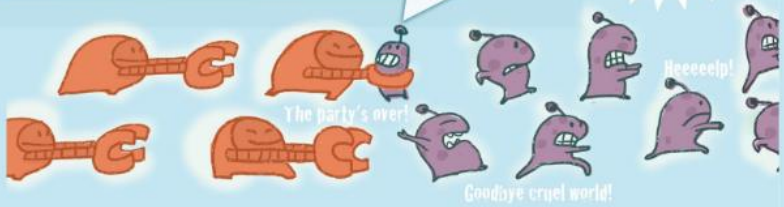
That is why every now and then you need to have a blood transfusion: to help your red blood cells to carry oxygen to every part of your body.

The problem is that every time you have a transfusion, the new blood brings with it a whole lot of **iron**

If you take it on its own, iron is very well-behaved.

But as soon as a whole lot of iron particles get together... **it spells trouble!**

So we need something to stop the iron, something that will get rid of it from your body. You need a... **medicine**



Scientists have invented various types of medicine to take the iron away

The old medicines which needed a needle and pump...

.... or else pills you had to swallow or tablets to dissolve in water...

... and now there's even a new syrup.

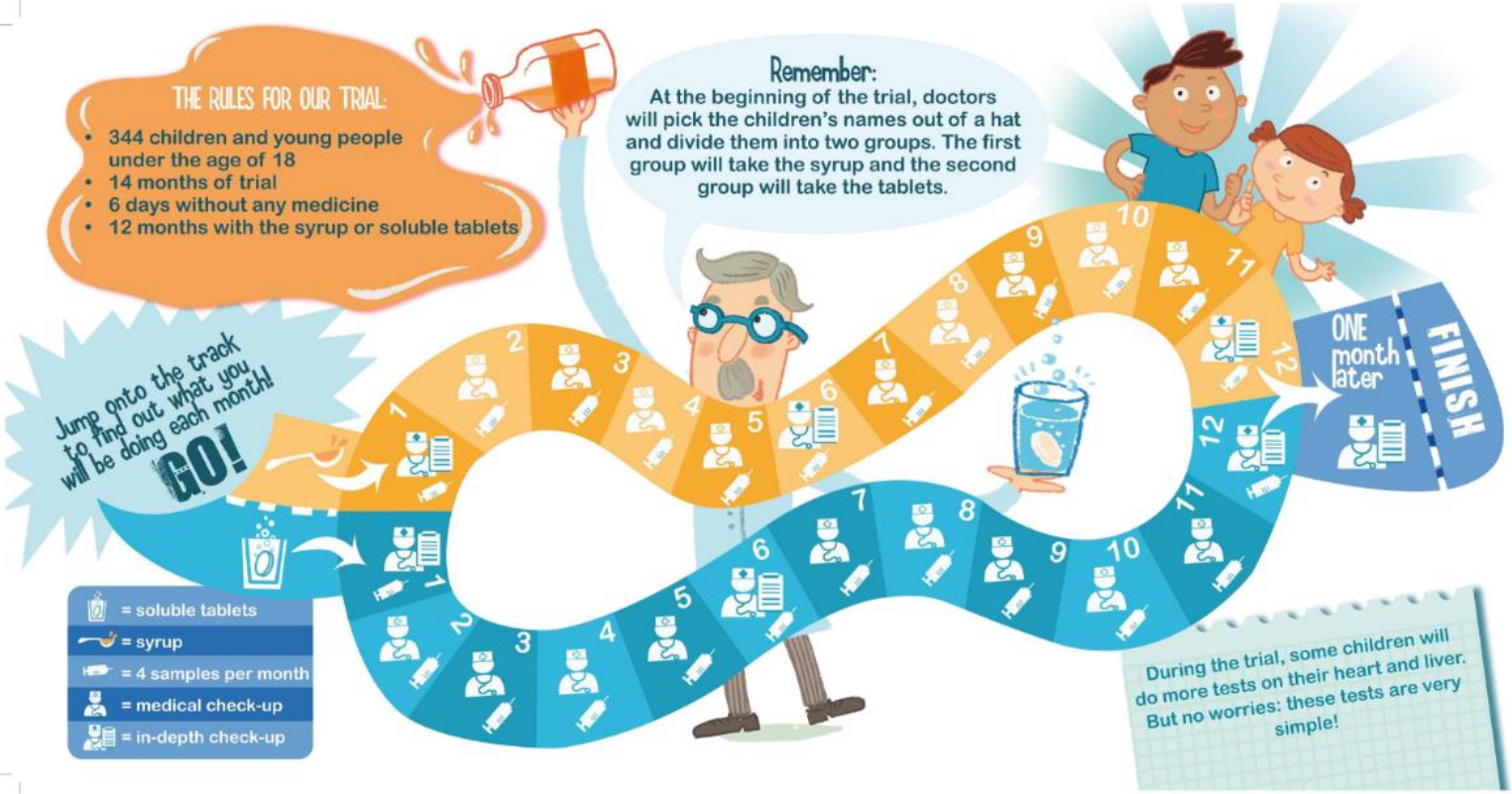
But, before we give any kids of your age this syrup, we have to carry out a... **TRIAL**

What's a trial?

It's a way of finding out whether the syrup works properly. If it works fine, in future all children just like you who need transfusions will be able to take it.

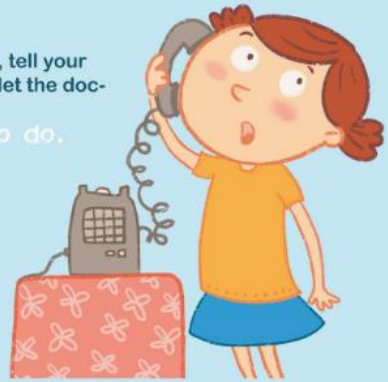
Do you want to help us out with our **trial**?
We would be so grateful. You'd be giving a great helping hand! Your doctor will take a look at you to check whether you can take part. But remember that the person who decides in the end is YOU. If you're frightened and don't feel like taking part, then just say so. No-one will be angry with you and the doctor will carry on giving you the old medicine.

But if you want to take part, take a look at the next page!


THE RULES FOR OUR TRIAL:
• 344 children and young people under the age of 18
• 14 months of trial
• 6 days without any medicine
• 12 months with the syrup or soluble tablets
Remember:
At the beginning of the trial, doctors will pick the children's names out of a hat and divide them into two groups. The first group will take the syrup and the second group will take the tablets.
Jump onto the track to find out what you will be doing each month!
GO!
ONE month later
FINISH
= soluble tablets
= syrup
= 4 samples per month
= medical check-up
= in-depth check-up
During the trial, some children will do more tests on their heart and liver. But no worries: these tests are very simple!

Sometimes, after taking the medicine, you might not feel great. That's quite **normal** – no need to be **afraid**.

If you feel anything **strange**, tell your parents straight away and let the doctor know.
He'll tell you what to do.



Now is your chance to ask Dr. Ironless

Test-tubes and lightning, what a lot of questions!

So can I relax?

Yes of course you can! Your doctor will keep an eye on you the whole time to make sure everything is OK. There's really nothing to be frightened of.

What if I change my mind and want to leave the Trial?

You can leave whenever you want. No-one will be angry with you and your doctor will carry on giving you the best treatment. Together with your doctor and your parents ... most of all the choice is up to YOU.

Who will know I'm taking part in this Trial?

No-one except for you, your family and your doctor.

SO WHAT DO I HAVE TO DO?

That's up to **you** to decide.

Your doctor and your parents think that this trial might help you, as well as lots of kids just like you.

In the whole country.

In all the countries around the **world**.

DEEP - DEferiprone Evaluation in Paediatrics

Funded by the European Commission under the 7th Framework Programme (FP7 Project - SP1 - Cooperation HEALTH-F4-2010-261483)

Study Title: Multi-centre, randomised, open label, non-inferiority active-controlled trial to evaluate the efficacy and safety of deferiprone compared to deferasirox in paediatric patients from 1 month to less than 18 years of age affected by transfusion-dependent haemoglobinopathies.

Clinical Study: DEEP-2

EudraCT number: 2012-000353-31

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What is a lay summary?

A lay summary is like telling someone about something in a very simple way, using easy words.

Lay Summary 6-12

DEEP-2

DEferiprone Evaluation in Paediatrics

Lay summary

What was DEEP-2 study about?

The DEEP-2 study was a **trial**, in other words, a way of finding out whether **deferiprone** is helpful and works properly for children with hereditary anaemias, such as thalassaemia or sickle cell anaemia. Let's look together at what the researchers found out about this medicine.

Why was the study needed?

Inside the blood are millions of little soldiers called **red blood cells**. They do an important job because they bring us **oxygen**, the fuel our body needs to work properly.

But if red blood cells don't work well, our bodies run out of fuel! All because of **hereditary anaemias**, which are a large family of **diseases present at birth** that affect the blood.

That is why you need to have a **blood transfusion**: to help your red blood cells to carry oxygen to every part of your body.

The problem is that every time you have a transfusion, the new blood brings with it **a lot of iron**. And when a whole lot of iron particles get together, it spells trouble!

So, we need a medicine that gets rid of extra iron from the body!

Which medicine was studied?

Researchers have invented different medicines to remove the extra iron: some medicines needed a needle and a pump, others were pills to swallow or tablets to dissolve in water. Among these was a medicine called **deferiprone**, which at first was used in people with thalassaemia but was not suitable for young children like you!

The DEEP-2 study looked at a new formulation of this medicine, a tasty **syrup** that was easy to use, to find out if it worked properly and was **suitable even for very young children** with thalassaemia and other anaemias.

What happened during the study?

393 children and young people under the age of 18 joined the study. They were from Albania, Cyprus, Egypt, Greece, Italy, Tunisia, and the United Kingdom.

These participants were divided into **two groups**: one group received **deferiprone** in syrup form, while the other group took **tablets** of a medicine called **deferasirox**, dissolved in water, for **one year**.

The researchers compared deferiprone with another medicine that is already known to be good for kids with hereditary anaemias to see if deferiprone works equally well.

How did the doctors know if the medicines works or not?

The children took some special tests. Thanks to these simple tests the doctors were able to check whether the two medicines were actually reducing the amount of **extra iron** in the **blood**, **heart** and **liver** of the participants and overall, that their bodies were working well.

These examinations were not painful nor dangerous and helped us to understand if the medicine is safe and works in children.

Sometimes the medicines can cause unwanted effects

What did we learn about Deferiprone?

All the medicines we take for their good effects can sometimes also affect other parts of our body and produce **unwanted effects**. Usually, these effects are not very dangerous, but it is important to investigate them to work out wheter the medicine is **safe**.

In DEEP-2, researchers studied the unwanted effects that happened when kids took the two medicines, **to see if deferiprone is as safe as deferasirox in children**.

Some of the participants did not feel well after taking the study medicines. Every time a participant felt something strange, **the doctors recorded it as an unwanted effect**.

During the study, both groups had a similar number of these unwanted effects. For example: some kids had experience with vomiting, nausea, diarrhoea or pain in the arms and legs. One of the main unwanted effects found in patients was a reduction in the number of **white blood cells** (these cells are very important for defending our body from infections).

So, then what happened?

Once the study was completed, **researchers compared the effect of the two drugs**. A total of 310 patients completed the treatment.

In the end, the study helped to show that deferiprone syrup, which is tastier and easier to swallow than the other types of medicines, can be very effective and useful for all young people and children with anaemias who need transfusions.

The DEEP-2 study also showed that some bad effects can happen; however, they are easy to manage and well known to your doctor. Therefore, **deferiprone is safe for children of every age**, from the very youngest to 18 year olds.

Why educational materials is important?

- ✅ Make complex information easier to understand
- ✅ Capture attention and interest
- ✅ Promote inclusion
- ✅ Increase awareness about health and research topics
- ✅ Help young people make informed decisions


THANK
YOU