ERDERA

European Rare Diseases
Research Alliance

Designing Paediatric Multinational Clinical Trials with YPAGs

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Co-funded by
the European Union

ERDERA has received funding from the European Union's Horizon Europe research and innovation programme under grant agreement N°101156595.
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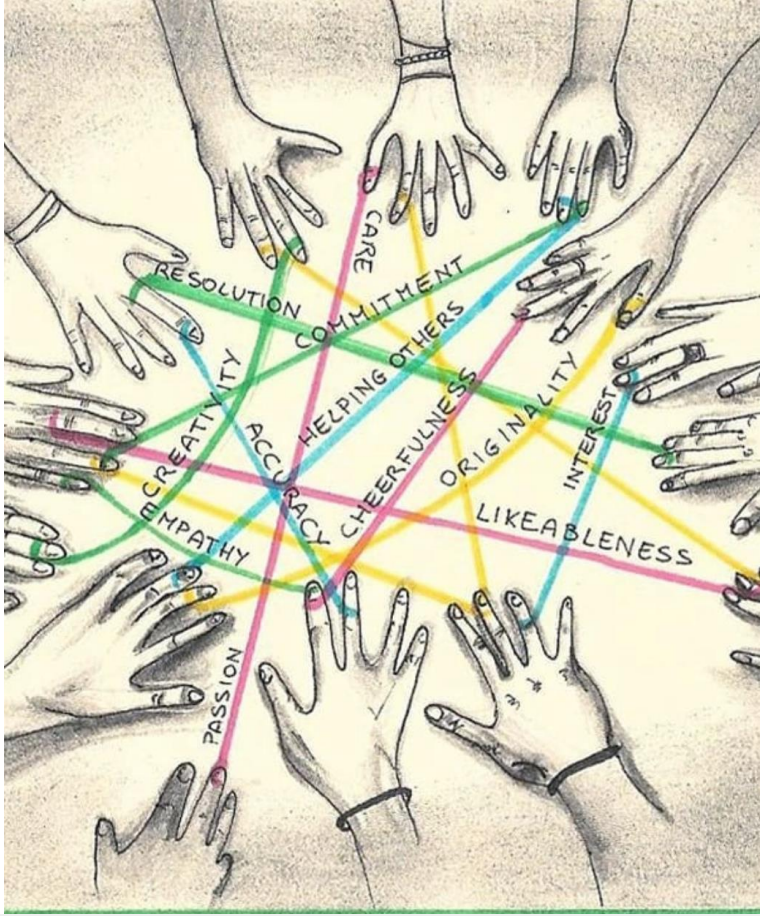
Introduction

How a clinical study conducted in many countries is designed **with** young people, not only **for** them.

Team: clinical study team, researchers, doctors and YPAGs



Main Points list

How YPAGs improve clinical studies

Real life example – MODERATO study

MODERATO study – Informative Booklets/Assent Forms

Why your voice matters

How YPAGs improve clinical studies

Involvement of YPAGs make a difference and improve clinical studies by:

- Making patient information sheet easier to read and understand
- Suggesting better formats (videos, apps, comics)
- Improving how visits are scheduled
- Saying what really matters to young people

Before

- Long time during hospital visits
- Consent form and Information sheet with complicated words
- Long Boring printable materials/no pictures

After

- Reducing visits/assessments time
- Understandable language according to age range
- Nice, colorful printable materials with cartoons

Real life example- MODERATO study

Real life example - MODERATO study

- Paediatric: 3-18 years old
- Multinational: Albania, Italy, Poland, Spain, UK

- To see if the atropine eyedrops are safe and work well for a period of 24 month treatment, in slowing the progression of myopia in children and adolescents from 3-18 years old.

MODERATO study logo has been designed from YPAGs during ICAN Summit 2024 in Bari

Real life example - MODERATO study

Feedback from YPAG

General comments:

- The length of the assessments should not exceed half a day :
  - especially for children under 12 years of age.
  - breaks during hospital visits/ If late morning, provide lunch for the family.

- Incentives to be offered (a small gift at enrolment - e.g a waterbottle)

Real life example - MODERATO study

Feedback from YPAG

Specific comments on the Informative booklets (3-5; 6-11; 12-17 EU/ 12-15 UK)

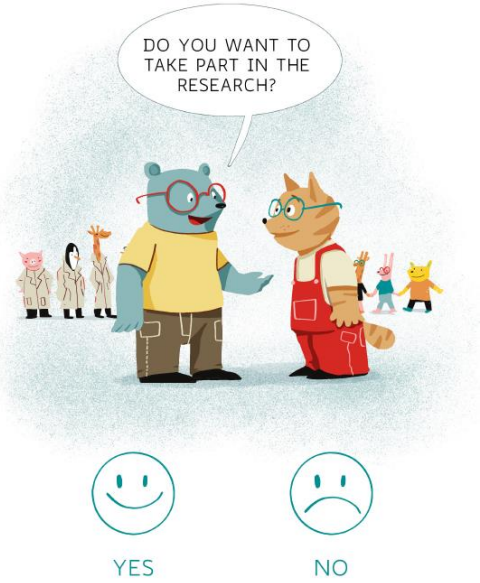
'Colourful, but the colours should be brighter – the palette is very "adult" and muted'

'Lots of words need changing, because this is not how children and parents talk' 'A title should be added'

'This cartoon prompted a big discussion about ethnicity, diversity and inclusion. At least one character should be black and have curly/Afro hair. If there is a person in authority, like an optician, they should be black or Asian and should have Afro hair or wear a Hijab'

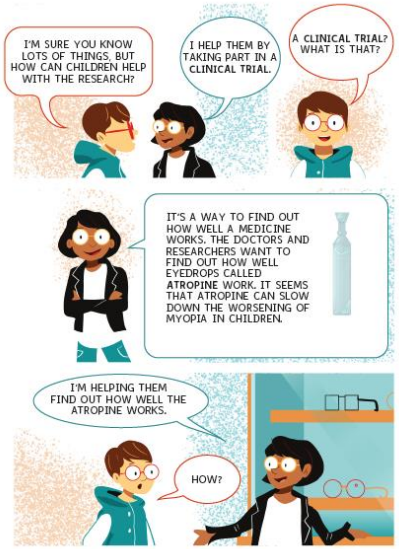
MODERATO study – Informative Booklets/Assent Form

INFORMATIVE BOOKLET/ ASSENT FORM **3-5 years**



MODERATO study – Informative Booklets/Assent Form

INFORMATIVE BOOKLET/ASSENT FORM **6-11 years**



MODERATO study – Informative Booklets/Assent Form

INFORMATIVE BOOKLET/ASSENT FORM **12-17 years EU/ 12-15 years UK**

MODERATO
clinical trial

STUDY ON ATROPINE TO PREVENT WORSENING OF MYOPIA IN CHILDREN AND YOUNG PEOPLE

Study Visits

All visits will happen at the clinic. In total you will come to the clinic six or seven times for check-ups. During the study period, you will talk to the study doctor over the phone 3 times, who will also monitor your health remotely.

During the first hospital visit you will be screened to see if you meet the requirements to participate in the study. You will be asked questions about yourself, your medical condition(s) and your medical history, other medicines you take and recent treatments.

You will fill out 2 questionnaires about your vision and 1 questionnaire about eyedrops acceptability. If you are a girl, you will take a pregnancy test.

If the results meet the requirements some eye tests will be done, and your glasses prescription will be checked. You will be randomly assigned to one of the groups and given a code that will identify you. The drops will be given to you for the first time and the study doctor will give you the eyedrop vials and the instructions to use them at home until the next visit.

Depending on the number of the study visit the following will be done:

A – Screening to see if you meet the requirements to participate in the study
B – Medical history review including any current medications or recent procedures
C – Questionnaires 1 and 2 about your vision
D – Questionnaire 3 about eyedrops acceptability
E – pregnancy test if you are a girl (atropine may harm an unborn baby)
F – Glasses prescription check
G – Eye tests
H – Eyedrops provided

	A	B	C	D	E	F	G	H
VISIT 1 – Day 1	✓	✓	✓		✓	✓	✓	✓
VISIT 2 – After 3 months		✓		✓	✓	✓	✓	✓
VISIT 3 – After 6 months		✓	✓	✓	✓	✓	✓	✓
VISIT 3 B								
VISIT 4 After 12 months		✓	✓	✓	✓	✓	✓	✓
VISIT 5 – After 18 months		✓		✓	✓	✓	✓	✓
VISIT 6 – After 24 months		✓	✓	✓	✓	✓	✓	

MODERATO clinical trial
ASSENT FORM for adolescent 12 – 15 years old
Identification code EU CT 2023-510439-13-00
Study title: Randomized, double-blind, multiple-dose, placebo-controlled parallel-group, adaptive study to evaluate the efficacy of atropine to treat the progression of myopia in children and adolescents from 3 to less than 18 years of age.
UK Version 1.0 15.08.2024

Assent Form

I have been invited to participate in the MODERATO clinical trial.

- ☐ I received clear information and explanations about this clinical trial.
- ☐ It was clearly explained to me what kind of trial it is and why it's being done. I understand the methods, rules, expected benefits, possible risks, and difficulties of taking part in this trial.
- ☐ I had the chance to ask questions to the trial investigator and I received clear answers to all my questions.
- ☐ I had enough time to think about all the information and discuss it with my family.
- ☐ I know that participation is fully voluntary. I know that I can decide at any given time to withdraw without giving any reason. Nothing will change about the help I get from doctors, nurses, hospitals, and other places where I get help for my condition.
- ☐ I understand that the trial investigator can stop the trial at any time, either permanently or for a while.
- ☐ I was told that if any new information shows it's not best for me to continue with the trial, I will be informed.
- ☐ I was told that the results of the trial will be shared with groups of scientists, but my name will be kept private.
- ☐ I have received a copy of this consent form.

I HEREBY DECLARE THAT

- ☐ YES ☐ NO – I am willing to take part in the trial.
- ☐ YES ☐ NO – I want to be told all new health information, even if it's surprising.
- ☐ YES ☐ NO – I want to be told only the health information that can help me take care of myself or make better choices.

Why your voice matters

You help make research and clinical studies safer, kinder and more fair

You speak from real experience

You help little participants feel more confident to be part of the trials

ERDERA

European **Rare Diseases** Research Alliance

Thank you!

You help researchers see through your eyes!

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