ERDERA

European Rare Diseases
Research Alliance

Consent/assent, clinical study protocol, and lay summary

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Outline

1. What is a clinical trial?
   - Parties involved
   - Steps
   - Study protocol
   - Study report/ Lay summary
2. Informed consent-assent in clinical trials

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New medicines research & development process

Discovery

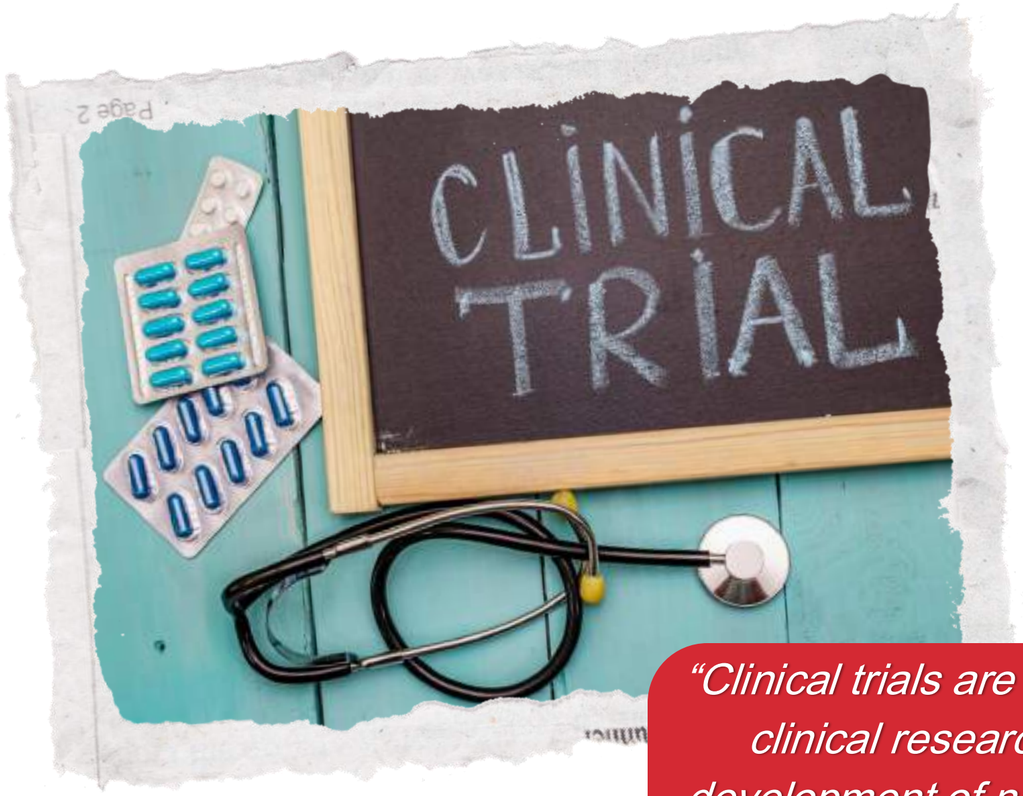
Non-clinical studies

Clinical Trials

Assessment

Vigilance

Clinical trial



A research study where participants are assigned to treatments or **interventions** (like new medicines or medical devices) that are **not part of normal medical practice**. These products are given as part of the study to determine their safety and efficacy.

"Clinical trials are a fundamental part of clinical research that support the development of new medicines or uses of existing medicines."

ICH E6 (R3) Guideline for good clinical practice (GCP)

Clinical studies **may not involve any treatment** at all. They just collect information.
In other cases, participants are just **observed while receiving normal medical treatment**.

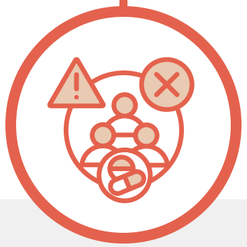
Clinical trial phases

Phase I

First study in humans. Tests the safe drug **dosages** on a **small group**

Focuses on the drug **efficacy** and further evaluates its **safety** in a **larger group** of patients with the targeted disease

Phase II



Phase III

Involves **large-scale** testing to confirm efficacy, monitor side effects, **compare** the drug to commonly used treatments

Research continues once the drug is on the market. Designated bodies **collect and analyse data** from patients to **monitor** side effects and confirm efficacy

Phase IV Follow-up

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Parties involved

- Healthy volunteers
- Patients

Study team

- Co-Investigators
- Study Coordinator
- Pharmacist
- Laboratory staff

- Investigational site (Hospitals)

Principal investigator

Is the person responsible for conducting a clinical trial at a clinical trial site

Sponsor

An individual, company, institution which takes responsibility for initiating, managing and financing the clinical trial

- Private bodies (Pharmaceutical companies, not-for-profit)

- Public institutions (Universities)

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Clinical trial steps

Design

- Clinical trial protocol

Clinical trial conduct

Analysis

Conclusion

Clinical trial protocol

- Describes the **objectives**
- Identifies the **population**
- Describes the **methodology**
- How the **treatment** will be administered

IMPORTANT

THE PROTOCOL MUST RECEIVE **APPROVAL** FROM THE **ETHICS COMMITTEE** AND **AUTHORISATION** FROM THE **NATIONAL COMPETENT AUTHORITY** BEFORE THE START OF THE TRIAL

Clinical trial design

Identify the target population

Recruitment **Screening** **Enrolment**

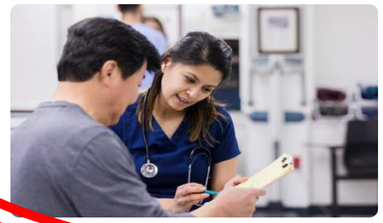
Inclusion/exclusion criteria

Clinical trial steps

Design

- Clinical trial protocol

- Informed consent



Clinical trial conduct

Analysis

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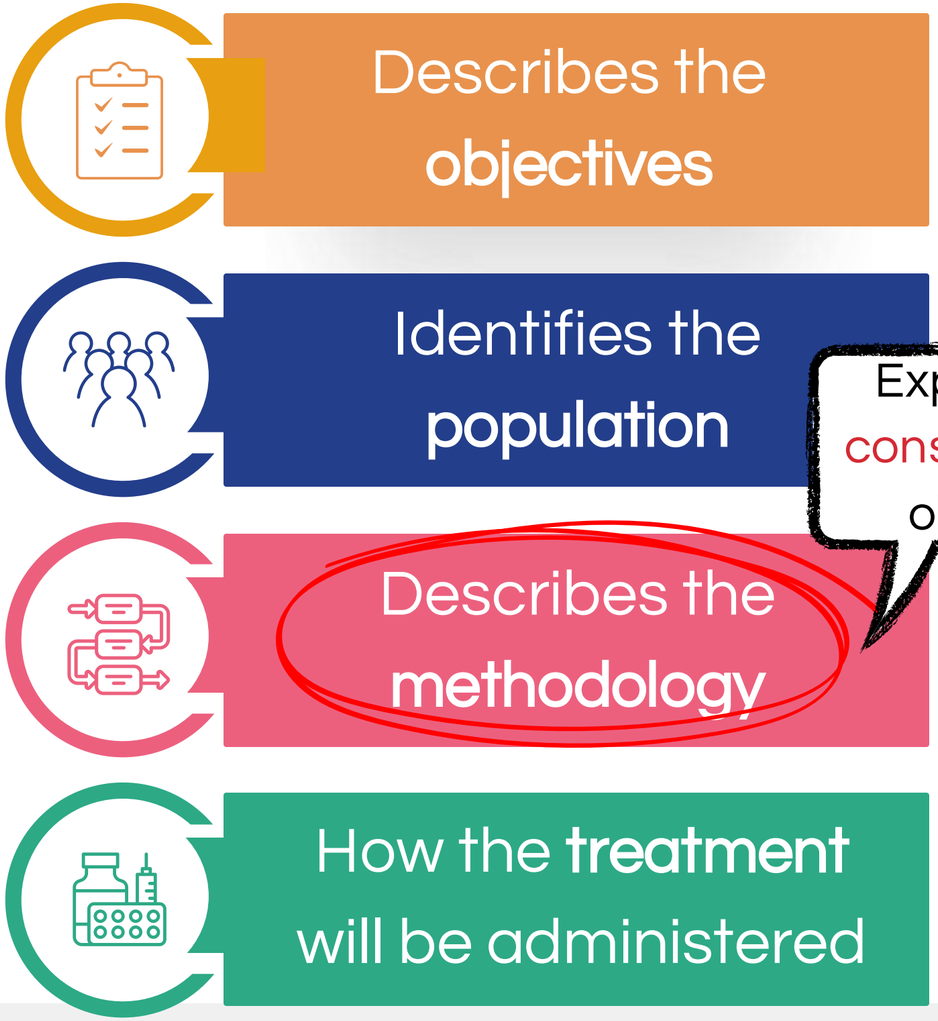
Clinical trial protocol



Informed consent

Regulation (EU) N° 536/2014, CTR

Informed consent means a subject's free and voluntary expression of his or her willingness to participate in a particular clinical trial, after having been informed of all aspects of the clinical trial that are relevant to the subject's decision to participate

OR

In case of minors and of incapacitated subjects, an authorisation from their legal representative to include them in the clinical trial

BUT

Children have the right to be fully informed about the trial through plain language and to decide whether or not to participate in the study.

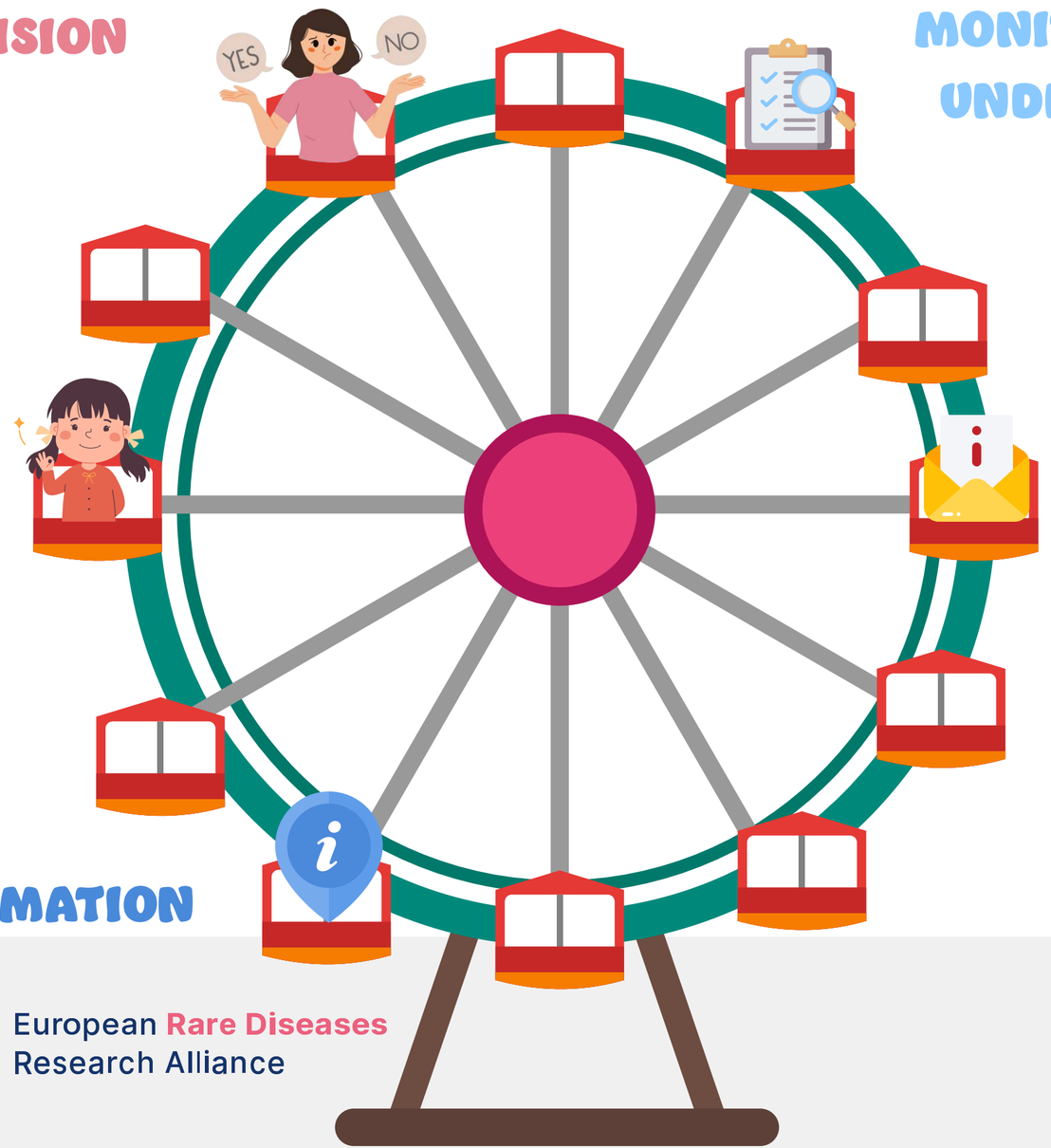
Informed consent

VOLUNTARY DECISION

MONITORING AND MAINTAINING THE UNDERSTANDING OF INFORMATION

UNDERSTANDING

AVAILABILITY OF NEW INFORMATION



INFORMATION

The informed consent is a **dynamic and continuous** process: it begins with subject recruitment but does not end with the subject's signature.

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Informed consent: how it looks like?

INFORMATION

All you need to know about the study

INFORMED CONSENT

To participate in the study/research

To process personal data

For any other purposes

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What consent should contain?

- Study duration
- Potential risks and benefits
- Treatment Procedures
- Participant's rights
- Any possible discomfort
- Right to withdraw consent
- Reasons for processing clearly stated
- Data confidentiality

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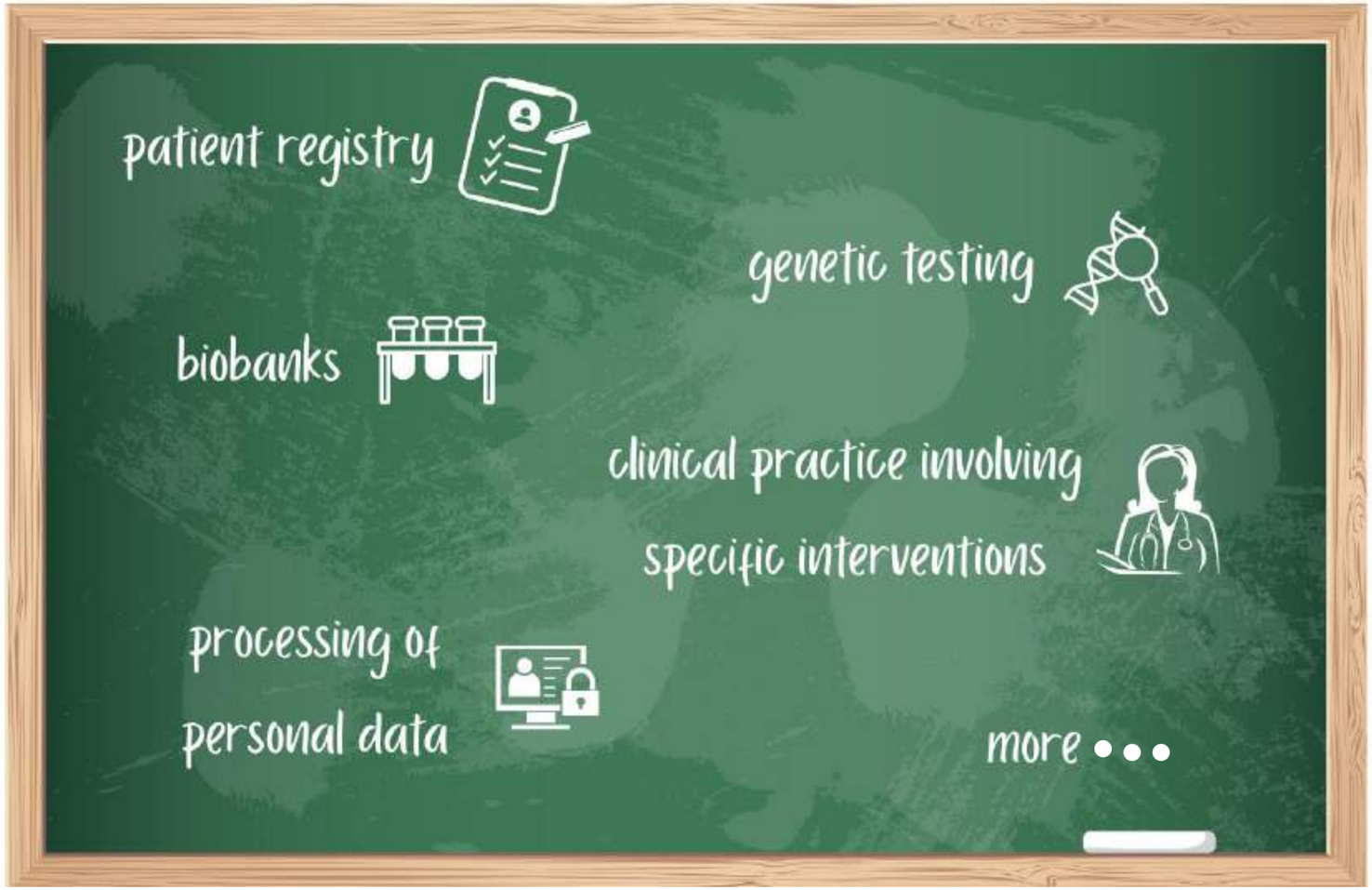
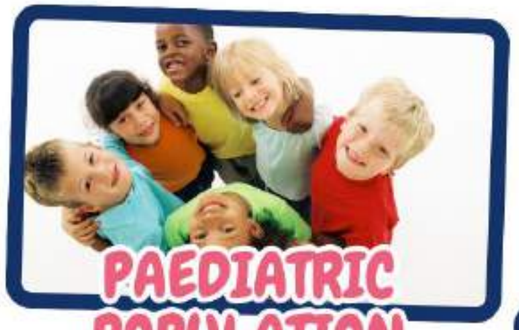
Informed consent

Other situations where it is required



... in addition to the informed consent provided by parents/legal representatives, **minors who are capable of forming their own opinion and assessing the information provided to them may also give their consent to participate in a clinical trial!**

Informed assent



PAEDIATRIC POPULATION

Children between birth and 18 years

MINOR

A person who has not reached the legally recognised age to give informed consent

LEGAL REPRESENTATIVE

Parents or the natural or legal person who has the power to give informed consent on behalf of an incapacitated person or minor

EMANCIPATED

Able to legally give consent under national law (vulnerability is considered)

Informed assent

Information adapted to the age and mental maturity of minors

Provided by staff members experienced in working with children

If a minor wishes to stop or not join the trial, the investigator must respect their decision.

Privacy information for minors should be explained clearly and in a child-friendly way

why personal data are required

what will be done with them

rights they have over their personal data

safeguards measures adopted to prevent unauthorised data access

How to collect assent

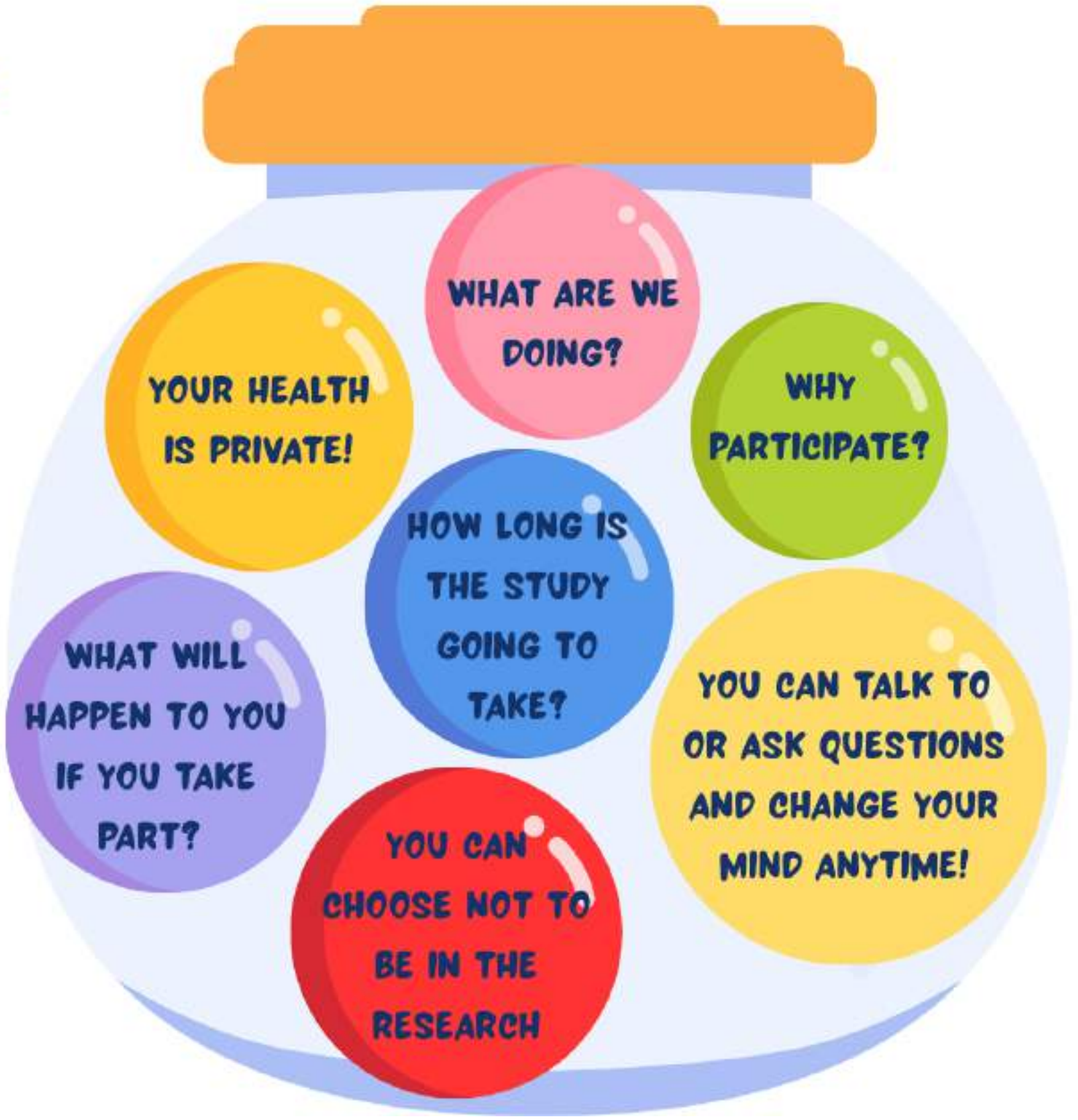
Age-appropriate information should be adapted to the language skills and understanding of the child

The use of visual strategies is encouraged (drawings, pictures, cartoons), but also other media and formats may be used

Where appropriate, the investigator should arrange for translation

Cultural differences should be considered

Informed assent contents



"Over the course of a clinical study, it may be necessary to reassess the assent of a child in recognition of their advancing age, evolving maturity and competency."

Informed assent

How children participate in the process

ASSENT

The **minor's declaration** of willingness to participate in a study with **legal value** according to the **national law** of EU member states

The child must consent to participate in a study

AGREEMENT

Highly recommended but **not required by law**

Investigators should ask for the child's agreement, even if it is not required by law

DISSENT

The explicit wish of a child, capable of expressing an opinion and evaluating information, to refuse participation in, or to withdraw from a study

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Minor's Agreement

A minor's **agreement alone** is **not enough** to join a clinical study; it must always be followed by the parents' or legal representative's consent.

Can you say NO if your parents say YES ?

Every effort should be made to understand and **respect differences of opinion between the minor and his/her parents/legal representative**

The minor's objections should be respected. The investigator should try to resolve differences, recognising the child's growing ability to make decisions like an adult

If, after trying to reach an agreement, there is still disagreement on participation, **the dissent of any of either party is decisive**

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Informed assent

Age groups

Newborns and infants (from birth to 2 years of age)

Only consent from parent(s)/ legal representative(s)

Pre-schoolers (2-5 years of age)

Only consent from parent(s)/ legal representative(s)

Schoolers (6-9 years of age)

Assent and consent from parent(s)/ legal representative(s)

Adolescents (10-18 years of age)

This group is treated differently across Member States....

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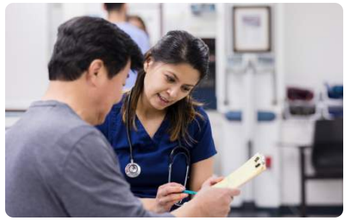
Clinical trial steps

Design

- Clinical trial protocol

- Informed consent/assent



Clinical trial conduct

Data from all subjects are COLLECTED, INSERTED into a clinical database and ANALYSED

Analysis

Conclusion

- Clinical study report
- Lay summary

Clinical study report

- purpose of the study
- number of participating subjects
- results of the study

Lay summary

AIMED AT A WIDER AUDIENCE

- STUDY PURPOSE AND RESULTS
- SHORT SENTENCES
- NON-PROMOTIONAL CONTENT
- SIMPLE, UNDERSTANDABLE LANGUAGE
- USE OF GRAPHICS AND FIGURES

PROTOCOL TEXT:
"PARTICIPANTS WILL UNDERGO A RANDOMISED, DOUBLE-BLIND, PLACEBO-CONTROLLED TRIAL WITH BI-WEEKLY SUBCUTANEOUS INJECTIONS OF THE INVESTIGATIONAL DRUG."

LAY SUMMARY:
"YOU WILL BE RANDOMLY PLACED INTO ONE OF TWO GROUPS. ONE GROUP WILL RECEIVE THE STUDY MEDICINE, AND THE OTHER WILL GET A PLACEBO (A DUMMY TREATMENT). YOU WON'T KNOW WHICH ONE YOU'RE GETTING. THE MEDICINE WILL BE GIVEN AS AN INJECTION EVERY TWO WEEKS."

Young patients should be involved in all clinical trials steps

Study protocol

Consent

INVOLVEMENT OF YOUNG PATIENTS

Study report

Lay summary

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