ERDERA

European Rare Diseases
Research Alliance

Legal and ethical considerations in paediatric research

FONDAZIONE
PER LA RICERCA FARMACOLOGICA
GIANNI BENZI
ONLUS

Silvia Torretta, PhD
Researcher

Co-funded by
the European Union

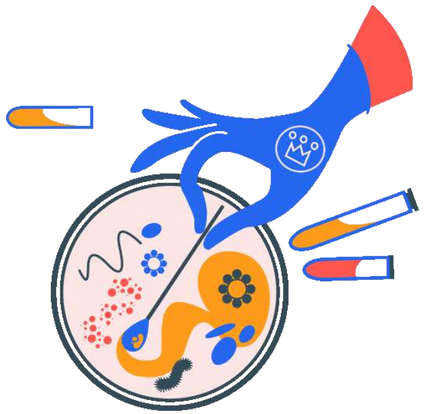
ERDERA has received funding from the European Union's Horizon Europe research and innovation programme under grant agreement N°101156595.
Views and opinions expressed are those of the author(s) only and do not necessarily reflect those of the European Union or any other granting authority, who cannot be held responsible for them.

Outline

1. What is **clinical research** and why it matters?
2. What means **ethics** in clinical research and why it’s so important?
3. Do young people have **rights** in the decisions about their health?
4. How **research** involving young people is made **safe, fair** and **respectful**

ERDERA European Rare Diseases Research Alliance

New Medicines Development and Testing Process



1

Discovery and Development

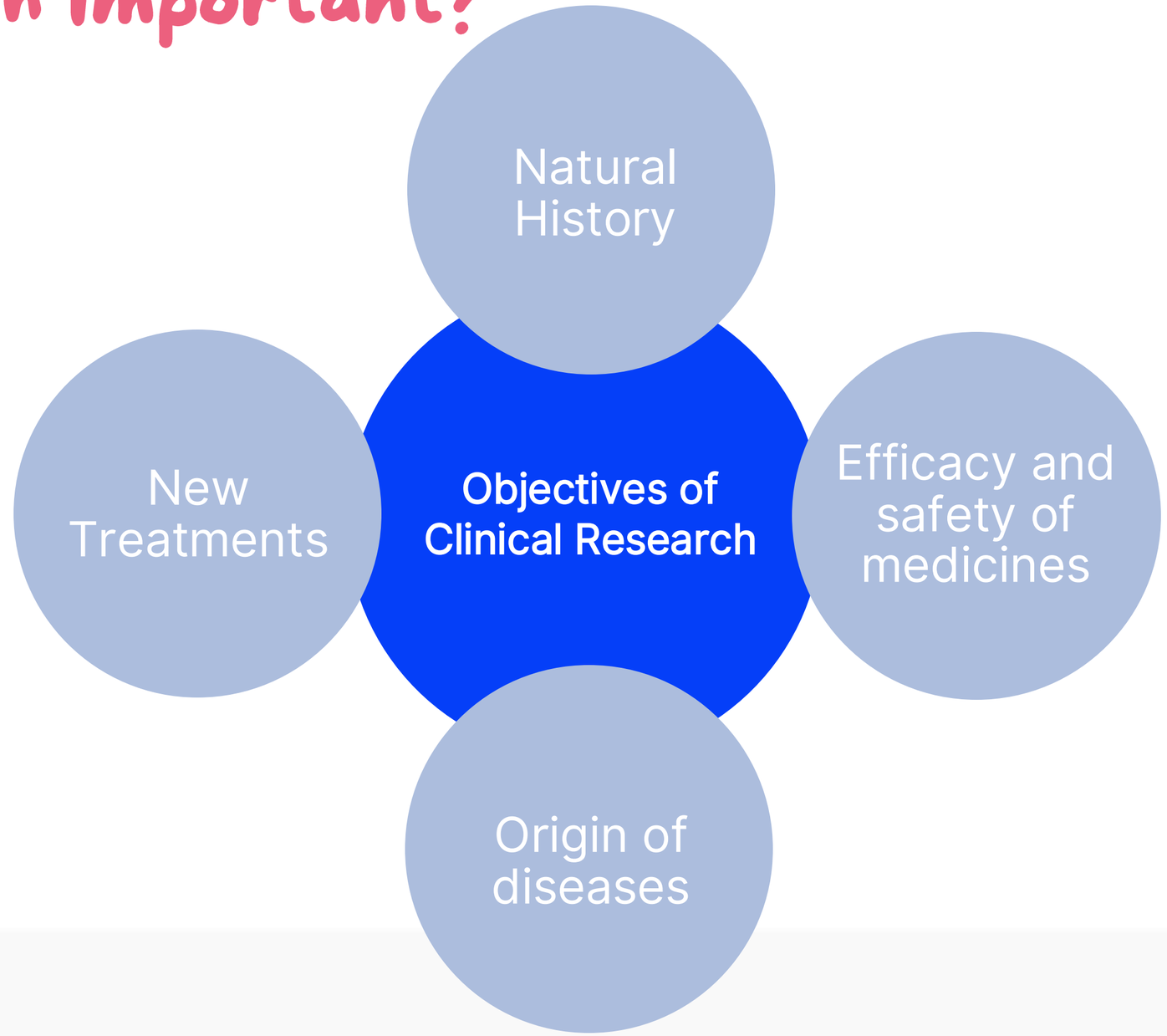
2

Clinical Research

3

Government Safety Review and Monitoring

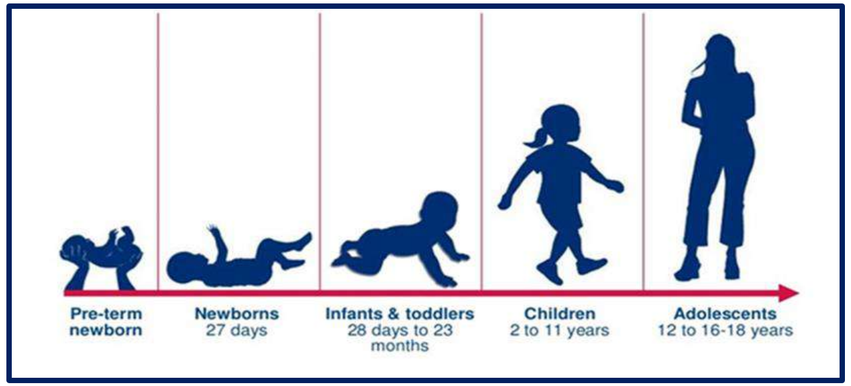
Why is Clinical Research Important?



ERDERA European Rare Diseases Research Alliance

Why do we need Paediatric Research?

When we talk about **paediatric medicines**, we refer to medicines that have to be used in **groups with very different characteristics**!



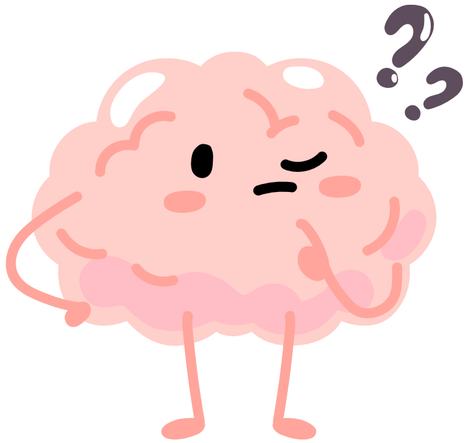
....Because children:

- ☑ are not small adults
- ☑ cannot take medicines like adults
- ☑ grow and change quickly
- ☑ respond to drugs differently

ERDERA European Rare Diseases Research Alliance

QUESTION TIME

What comes to mind when you hear 'ethics' in clinical research?

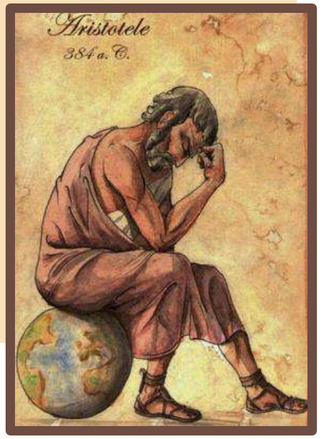


ERDERA European Rare Diseases Research Alliance

What is ETHICS

"Ethics is that branch of philosophy that studies the conduct of human beings and the criteria by which behaviour and choices are evaluated"

Aristotle



Hippocratic Oath (5th century BC)

Respect for human life, Respect for **patient privacy**, prohibition of intentionally **causing harm**

Nowadays

Still recited during doctors' graduation ceremonies as a **symbolic tradition**

History of unethical research

Human Experiments during the Nazi Era

The Tuskegee Study

◦ During the Second World War	**When?**	◦ from 1932 to 1972
◦ Prisoners in concentration camps	**Who?**	◦ 399 Afro-American men suffering from syphilis
◦ Horrific experiments (torture, forced administration of toxic substances)	**What?**	◦ Researchers observed the natural progression of the disease
◦ No consent was required ◦ Experiments caused suffering and death	**Lack of Respect for Human Rights**	◦ Participants were not informed of the true nature of the study and did not receive adequate treatment even after the treatment was available ☹

ERDERA European Rare Diseases Research Alliance

Need for ethics and laws in medical research

Informed consent, respect for human dignity

Respect for people, Benefit of the study, Justice

Human dignity

Nuremberg Code (1947)

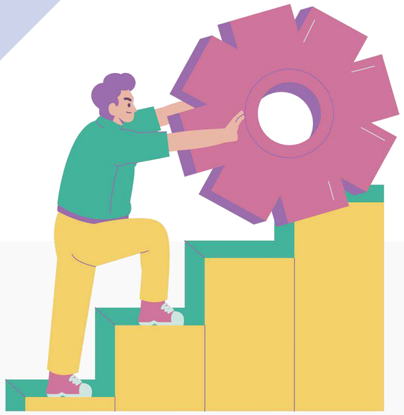
Declaration of Helsinki (1964)

Belmont Report (1979)

International Guidelines for Biomedical Research (1993)

Oviedo Convention (1997)

Singapore Declaration (2010)



ERDERA European Rare Diseases Research Alliance

Ethical Principles in Paediatric Research

Rules are even more important and special care is needed as children are more vulnerable

- **Respect for autonomy**
- **Beneficence (promote well-being)**
- **Non-maleficence (do not harm)**
- **Justice (fair distribution of risk, burden and benefits)**

Role of parents (legal representatives), Children and Healthcare Professionals

HEALTHCARE PROFESSIONALS
Central role in facilitating children and parents involvement in decisions

Decision-making process regarding children health

PARENTS – LEGAL REPRESENTATIVES
Natural, essential partners who provide an authorisation

CHILDREN
Rights holders who express their views once obtained an adapted information

ERDERA European Rare Diseases Research Alliance

What are Consent and Assent?

Consent

When **your parents or guardians**, after having been informed of all relevant aspects of the study, free and voluntary **agree to let you join a study**

Assent

When, in addition to the informed consent provided by your parents or guardians, **you agree to take part in the study, as you** are capable of forming your **own opinion** and understanding the information provided

What/who ensures that research is carried out ethically and legally?

CCMO Central Committee on Research Involving Human Subjects

AIFA AGENZIA ITALIANA DEL FARMACO

ΕΟΦ ΕΘΝΙΚΟΣ ΟΡΓΑΝΙΣΜΟΣ ΦΑΡΜΑΚΩΝ NATIONAL ORGANIZATION FOR MEDICINES (EOF)

Federal Institute for Drugs and Medical Devices

Medicines & Healthcare products Regulatory Agency

Rules: Laws, Guidelines

For example...

Clinical Trials Regulation (EU 536/2014)

General Data Protection Regulation (EU 679/2016)

Ethical considerations for clinical trials on medicinal products conducted with minors

Ethics Commettees

Independent groups of **experts**. They check that research is **fair, safe**, and **respectful** to participants

Competent Authorities

Government organisations that **authorise** and **monitor** research and medicines.
They make sure that clinical research **follows the law**

Personal Data Protection
How it works in the EU?

What's Personal Data?

Any information (written or image) that can help someone to **know who you are**

General Data Protection Regulation

A set of data protection **rules** that organisations have to follow and of **rights** people in the EU have when they share their personal data

- **Children**'s personal data should be especially protected
- Children and young people have exactly the **same rights as adults**

ERDERA European Rare Diseases Research Alliance

Data Protection Rights

Right to be informed

Right of access

Right to object

Right to data portability

Right to erasure

Right to rectification

Right to restrict processing

Right on automated decision making and profiling

ERDERA European Rare Diseases Research Alliance

Wrap-Up & Take Home Messages

Ethics = fairness, respect, protection (of rights, data confidentiality)

There are **rules** in clinical research!

Your voice matters in decisions about your health

You have rights!

Silvia Torretta, PhD

st@benzifoundation.org

FONDAZIONE
PER LA RICERCA FARMACOLOGICA
GIANNI BENZI
ONLUS

Questions & Reflections

What surprised you most?
What makes research feel ethical to you?
Any questions or thoughts?