RAISING AWARENESS: THE TEDDY KIDS' WAY

Inside the Lab: How New Treatments Are Developed

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What will we explore?

How a medicine goes from an idea in the laboratory to a treatment that can be used by children.

1. Medicine Development
2. Safety checks
3. Clinical Trials
4. Paediatric and rare disease research



INTRODUCTION

- *A Drug , A Vaccine, or any kind of treatment* we use today had to be tested before it became available.

- Scientists cannot just give a new drug to everyone, they must first check that it is **safe and works properly**.



How long does a scientific idea take to become a treatment a child can actually take?!

Shout out a number

Drug Development: From Discovery to Testing



Possible new treatments.
Only a few are ever tried with patients.

Tested in the laboratory, outside people, to learn as much as possible about new treatments side effects

Safety, benefits, efficacy and dose. tested with a few people, then hundreds, then thousands.

Regulators check the results and decide if it can go to patients

Medicines are carefully and continuously monitored after approval for safety

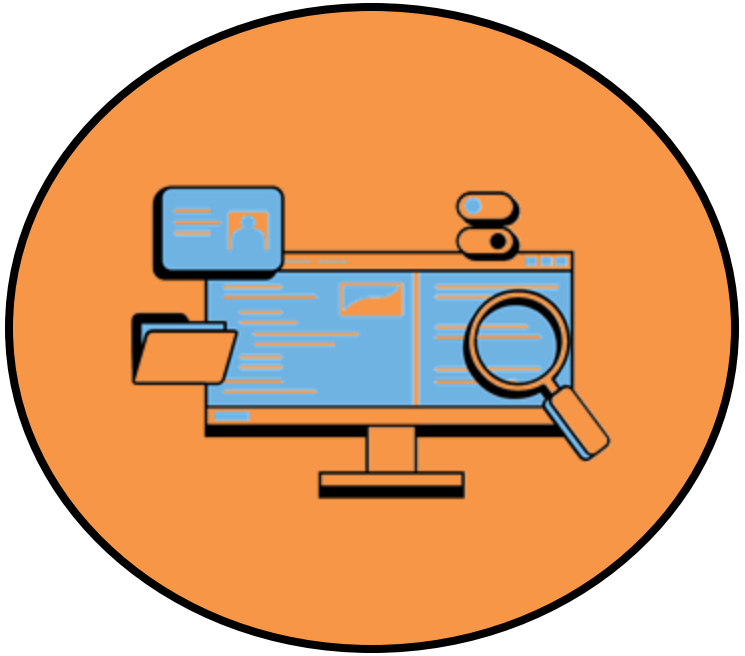
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The answer

- *New medical treatments take **10 to 15 years** to move from basic laboratory research to patient care in clinics.*

Types of Research Studies

Scientists can study medicines and diseases in different ways:



IN SILICO

Studies that use **computer models and simulations** to predict what might happen.



IN VITRO

Studies done **outside a living body**, for example using cells in a laboratory.



IN VIVO

Studies carried out **in a living organism** to understand how a medicine or disease behaves.

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The Safety Checks: Before the first patient



Every medicine begins as an idea.

It is first tested in the laboratory.

Tens of thousands of ideas are tested every year.

Only a small number are ever promising enough to be tested in people.

For most ideas, the journey ends in the laboratory.

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Basic Research and Discovery (Years 0–3)

- Scientists study diseases at a molecular or cellular level.
- They identify **biological targets** (like proteins or genes) linked to a disease.
- Researchers test thousands of compounds to see which ones interact with the target.
- These promising chemical or biological substances are called **lead compounds**.

Preclinical Testing (Years 3–5)

- Researchers test the lead compounds in the laboratory using cells and tissues (**in vitro**).
- They test the compounds in animals (**in vivo**) like mice or rats.
- These tests check if the treatment is toxic and if it works at all.
- Scientists apply for permission from regulatory agencies, like the U.S. Food and Drug Administration (FDA), to test on humans.

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Clinical Trials (Years 6–11)

Clinical trials happen in **three** main phases**:**

- **Phase 1:** Tests the treatment on a small group of 20 to 80 healthy volunteers to check safety and dosage.
- **Phase 2:** Tests 100 to 300 patients with the disease to see if the treatment works and find side effects.
- **Phase 3:** Tests 1,000 to 3,000 patients to confirm effectiveness, monitor side effects, and compare it to standard treatments.

Phase 4: Happens after the treatment is approved / Monitors long-term safety and effectiveness

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Regulatory Review and Approval (Years 11–12)

- Researchers submit all trial data to regulatory authorities.
- Experts review the data to ensure the treatment is safe and effective.
- If approved, the drug receives a license for public use.

What does clinical research try to find out?



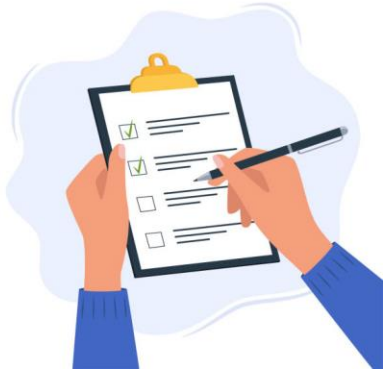
New Treatments

Finding new ways to treat a disease.



The Efficacy and Safety of Medicines

Does a medicine work, and is it safe?



Natural History

Improve the knowledge of the disease and its evolution over time



Origin of diseases

What causes a disease?

Every answer brings us one step closer to better care for children.

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Clinical Trials

- Before an investigational drug can be made available to the public, it must pass 3 phases

Phase I

First study in humans. Tests the drug's safety, dosage range, and side effects

Phase II

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

Phase III

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

An independent authority then reviews all the evidence to decide whether the medicine's quality, safety and benefits justify approving it, and for which patients. After approval, medicines are carefully and continuously monitored for safety

Why do we need Paediatric Research?

Children are not small adults!

A medicine for children needs to consider:



From babies to teenagers, one dose does NOT fit all!

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PEDIATRIC AGE UP TO DATE

- **For Drugs and Biologics:** The FDA generally defines the paediatric population as ranging from **birth to younger than 17 years old** (birth through 16 years of age).

- **For Medical Devices:** The FDA defines pediatric patients as persons **aged 21 or younger** at the time of diagnosis or treatment.



Why it takes so long

🧪 From an idea to a medicine: 10+ years

- A new medicine must pass through many stages of research and clinical testing before a doctor can prescribe it.
- Each stage checks something different: the time is not a delay. It is how problems are found before a medicine reaches patients.
- 10+ years is when everything goes well:

✓ Trials are completed as planned

✓ Results are reliable

✓ The medicine can be manufactured at scale

✓ Enough patients are recruited

🔬 **Why is it even harder for rare diseases?** Very few people have the condition. Trials are slower to fill, and less likely to be funded. This is why networks like TEDDY Network matter: they bring researchers, clinicians, patients, families and young people across many countries together to make trials for rare paediatric conditions possible.

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APPROVED RECENTLY

- On 2 June 2025, **Bosutinib** has been approved by the European Medicines Agency (EMA) for the treatment of children ≥ 6 years with Philadelphia chromosome-positive chronic myeloid leukemia (CML) in chronic phase.

- **Centanafadine:** Approved in July 2026 by FDA

Centanafadine (sold under the brand name **Simtriyo**) is a prescription medication approved to treat attention-deficit/hyperactivity disorder (ADHD) in adults and paediatric patients aged 6 years and older.

- The European Medicines Agency (EMA) recently adopted a positive opinion extending the use of Stelara (ustekinumab) for children **with Crohn's disease** who weight at least 40kg.

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Recap: There's more to Clinical Research than Lab Research

- It is common to think that clinical research occurs exclusively in labs where scientists perform experiments using test tubes and Petri dishes.

- Clinical Research is a field where we are testing new treatments, drugs, devices and therapies *in humans.*

  And while lab work can be one component, Clinical Research goes far beyond laboratory work.

- From the early stages of testing new drugs to large scale clinical trials, this dynamic and rewarding industry covers a wide range of studies focused on discovering new treatments that can save lives, transform lives, and improve human health.

Recap: There's more to Clinical Research than Lab Research

- Clinical research brings together professionals from many different backgrounds and roles. Researchers work across hospitals, universities, research institutions, pharmaceutical companies, Contract Research Organizations (CROs), and specialized vendors.
- They collaborate with doctors, nurses, pharmacists, data scientists, statisticians, patient advocates, project managers, regulatory specialists, and other healthcare professionals.
- Their work can involve designing and managing studies, collecting and analyzing data, ensuring patient safety, managing clinical trial operations, navigating regulatory requirements, and supporting the development of new treatments and therapies.

Together, these professionals help bring innovative treatments from the research stage to patients and improve health and quality of life worldwide.

- Doctors & Nurses
- Clinical Research Coordinators
- Data Scientists & Biostatisticians
- Clinical Trial Project Managers
- Regulatory Affairs Specialists
- Clinical Researcher Associates
- Pharmacovigilance & Patient Safety
- Patient Advocates
- Pharmaceutical Industry Professionals
- CRO & Vendor Specialists

The role of YPAGs

- But clinical research is not only about the professionals conducting the research, it is also about giving young patients and healthy youngers a voice in the process.

- Young people can play an important role in shaping clinical trials.

- Groups like YPAG help researchers design studies that are more suitable for young participants.

- They can give feedback on things like:

- Study materials (are they easy to understand?)
- Study design (is it practical and fair?)
- Study visits (are the number and length of visits manageable?)
- Recruitment & communication (are young people being reached and informed in the right way?)
- Patient experience (what could make participation more comfortable and accessible?)
- Study results (are the findings communicated in a way that young patients can understand?)

This helps make clinical trials more inclusive and effective.

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A Quick question:

What do you think is the biggest challenge in developing a medicine for a rare disease?

A. Finding enough patients
B. Knowing if the medicine is safe
C. Manufacturing the medicine
D. All of the above

THANK YOU!!



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