invents

# How innovation can improve paediatric clinical research, children's health and wellbeing

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TEDDY - European Network of Excellence for Paediatric Research

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INVENTS has received funding from the European Union's Horizon Europe Research and Innovation program under grant agreement 101136365.

Funded by the Swiss State Secretariat for Education, Research and Innovation (SERI).

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# What does innovation mean?

Innovation means finding new and better ways to do things.

# …and in paediatric research?

Doctors and scientists discover new treatments, tools, and ideas to improve kids' health.

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# How does innovation improve paediatric health care?

By using **modern technology and smart ideas**, we can make **tests, monitoring and treatments safer, faster, and more comfortable for kids**.

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# The “Main Characters” of the innovation

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# Innovation

Digital Health Technologies (DHTs)

Advanced therapy medicinal products (ATMPs)

Innovative Trial Designs

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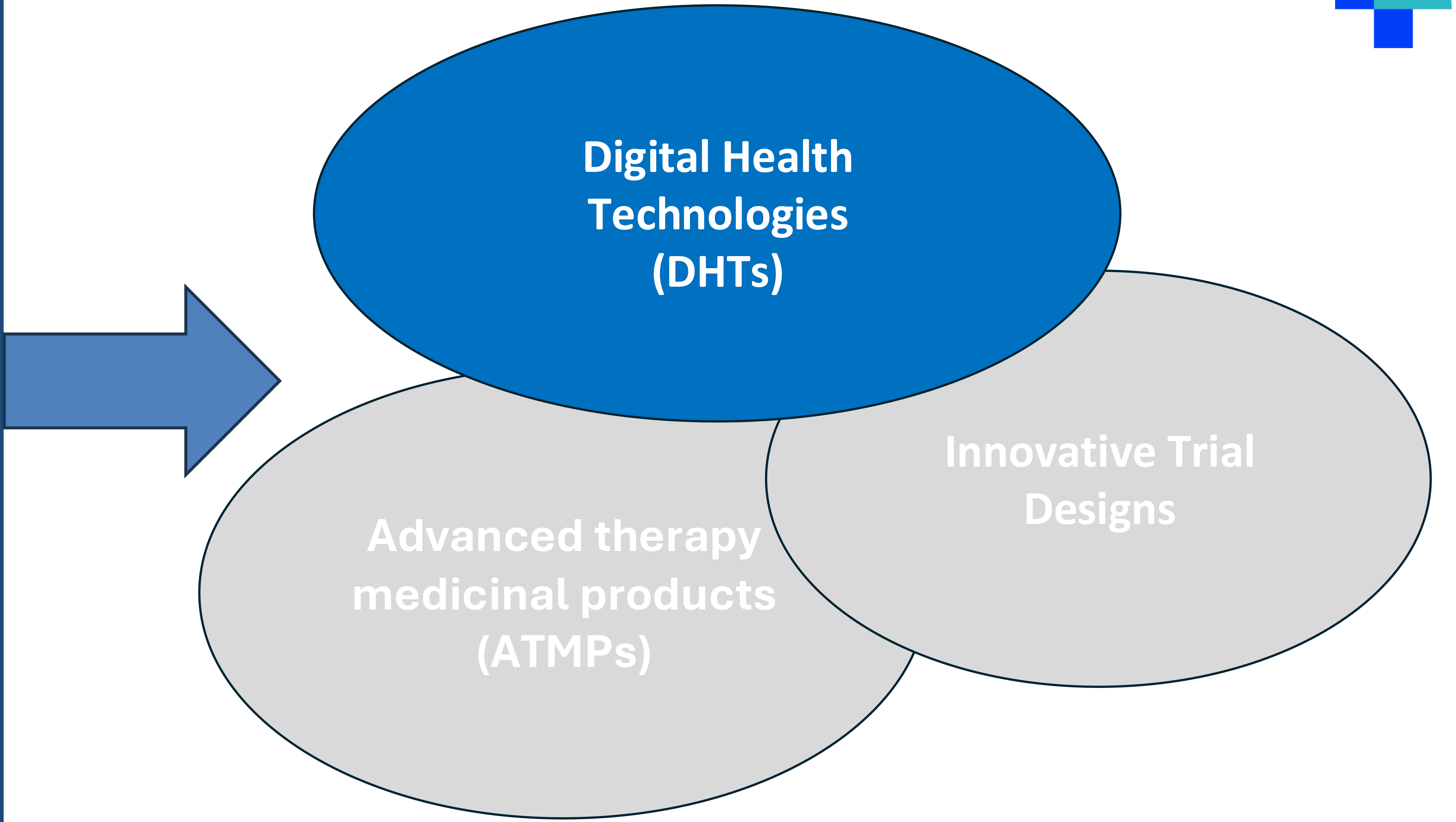
# Innovation





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# Digital Health

Digital Health Technologies (DHTs)

Refers to tools and services that use information and communication technology to improve prevention, diagnosis, treatment, monitoring and management of health and lifestyle.

Which types of digital health tools do you know?

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# Digital health technologies (DHTs)

Digital Health Technologies (DHTs)

- Mobile Health
- Telemedicine
- Wearable Devices
- Artificial Intelligence (AI)
- Electronic Health Records (EHRs), Consent (eConsent) and Patient-Reported Outcomes (ePRO)


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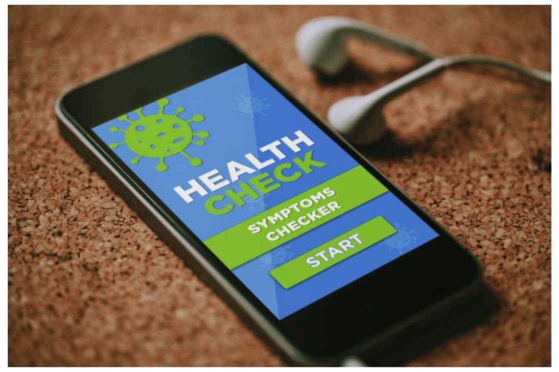
# Mobile Health

Use of mobile devices and applications to support health and wellness.

**Health management apps** support individuals in monitoring their own health conditions.

### Symptom Checkers




### Medication Reminders


**Healthy Living & Wellness apps** include nutrition-tracking apps, sleep apps, stress-management apps that help us live healthier lives.



Digital Health Technologies (DHTs)

# Telemedicine

Remote consultation for medical, diagnostic and treatment-related services that allow patients to communicate with healthcare providers via video, phone, or chat.


This technology Increases access to care, especially for individuals in rural areas or those with mobility issues.

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# Wearable devices

Electronic devices worn on the body, close to or on the surface of the skin, that detect, analyze, and transmit information such as vital signs, and/or ambient data on real time basis.


Provides continuous health data, promoting proactive health management and early detection of issues.

These data can be shared with doctors without the child to visit the hospital as often.

Digital Health Technologies (DHTs)

# Artificial Intelligence (AI)

The technology that enables machines to mimic human thinking and learning. It allows computers and systems to analyse data, solve problems, make decisions, and improve over time by processing large amounts of data.

AI will play an increasingly central role in transforming clinical trials into faster, more patient-centric, and data-driven processes, leading to faster trial completion and quicker access to drugs.

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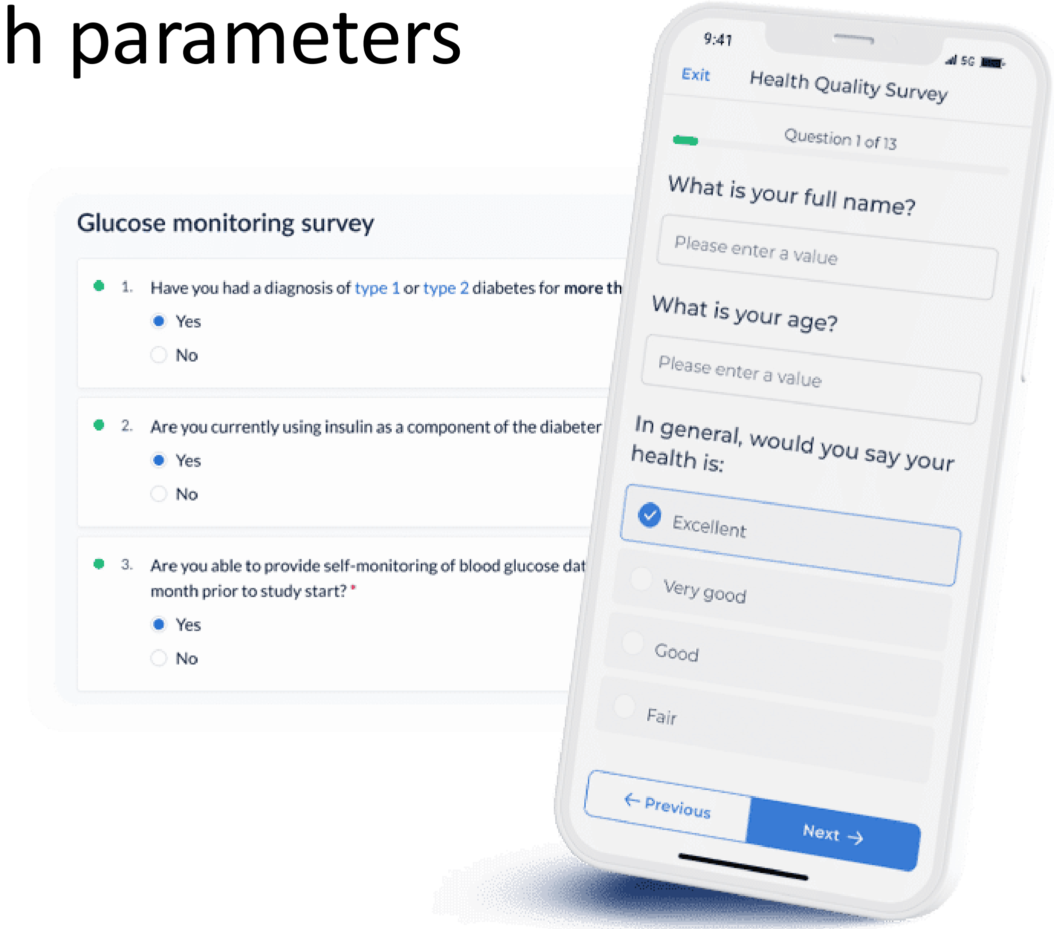
# Electronic Health Records (EHRs)

An EHR is a digital version of a patient’s paper chart.


# Electronic Patient-Reported Outcomes (ePRO)

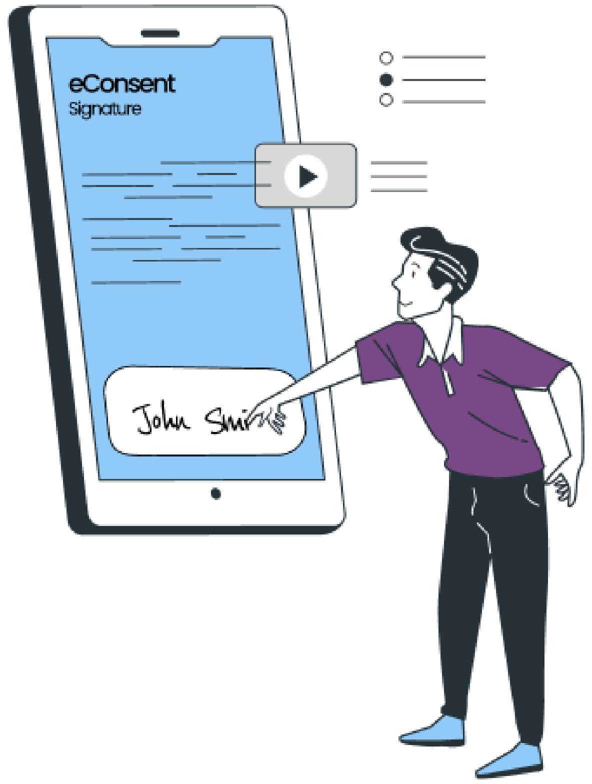
ePRO is the digital capture of patient-reported data on their health parameters




Digital Health Technologies (DHTs)

# Electronic Consent (eConsent)

eConsent is a digital process that enables patients to provide their informed consent




These technologies enhance healthcare efficiency, quality, and patient involvement

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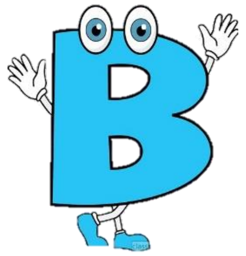
https://www.youtube.com/watch?v=nEahhnk3VvM

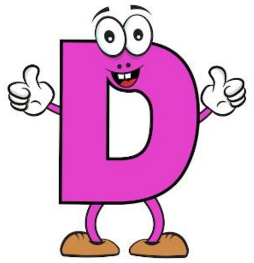
https://www.youtube.com/watch?v=Gtl-cuwwqXQ

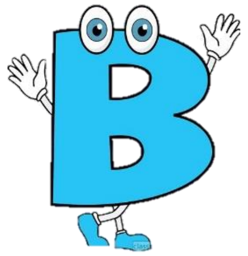

https://www.youtube.com/watch?v=nEahhnk3VvM

**What is Mobile Health?**
A) Medical research platform
B) Use of mobile devices to support health practices
C) Traditional health care system
D) Health services only for rural areas



**What type of data can wearable devices typically collect?**
A) Heart rate and physical activity levels
B) Blood sugar levels
C) Sleep patterns
D) All of the above

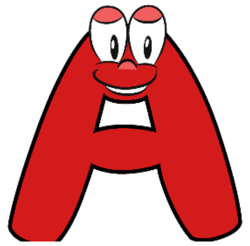




**What is one major benefit of Telemedicine technology?**
A) They replace doctors entirely
B) Improve access to healthcare, especially in remote areas
C) Increase hospital wait times
D) Require a high level of physical fitness to use

**What is Artificial Intelligence?**
A) A software that works like a calculator
B) An advanced computer program with no practical applications
C) The simulation of human intelligence by machines
D) Technology used only for playing games

**What is electronic consent (eConsent)?**
A) Digital process of obtaining patient consent for clinical studies
B) Consent given verbally without documentation
C) A paper form for patient consent
D) Consent given only through a laptop



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# Innovation

Digital Health Technologies (DHTs)

Innovative Trial Designs

Advanced therapy medicinal products (ATMPs)

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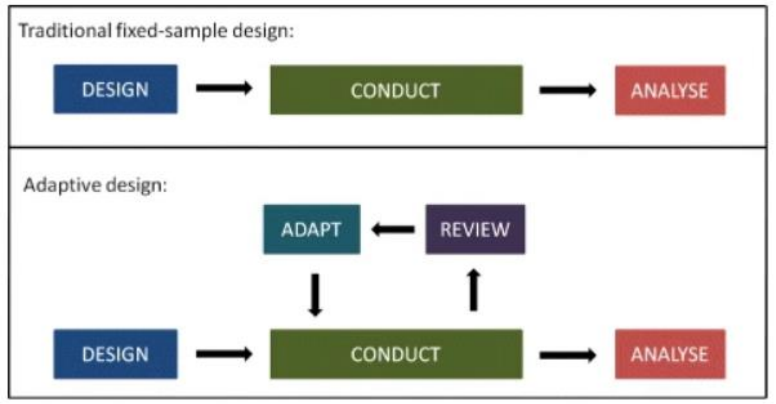
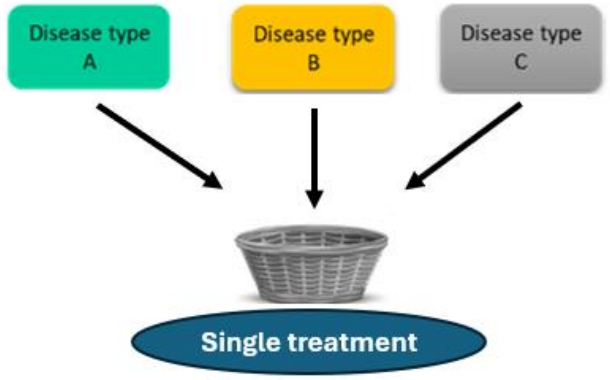
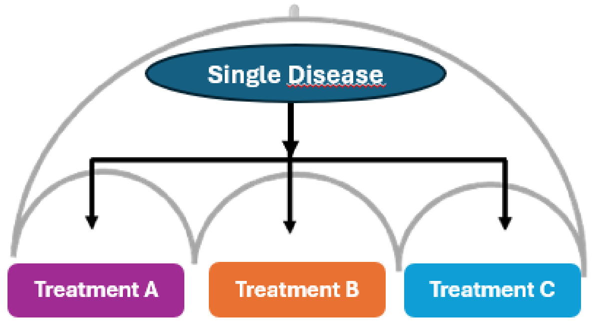
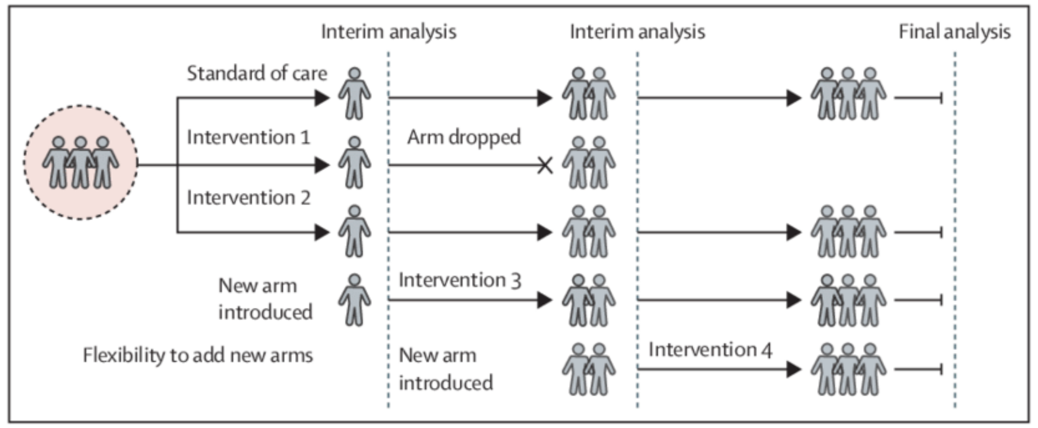
# Innovative Trial Designs

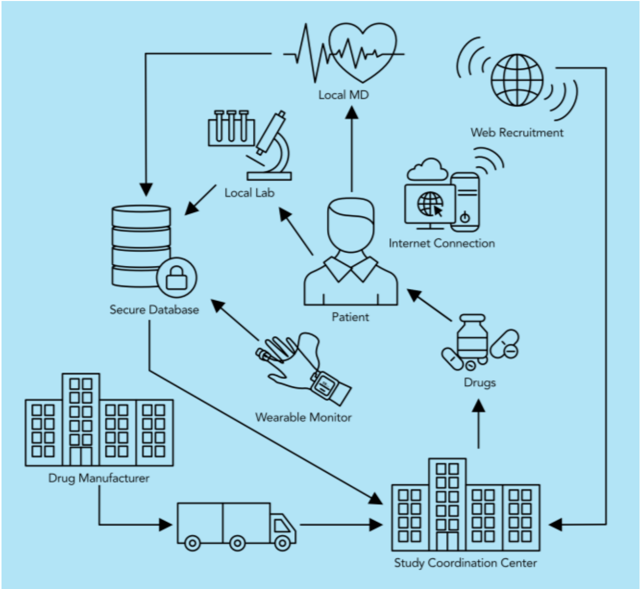
Innovative trial designs are advanced methods for structuring clinical trials, using approaches that can adapt to real-time data, test multiple treatments or conditions simultaneously, or leverage digital tools to broaden patient access.





Innovative
Trial Designs



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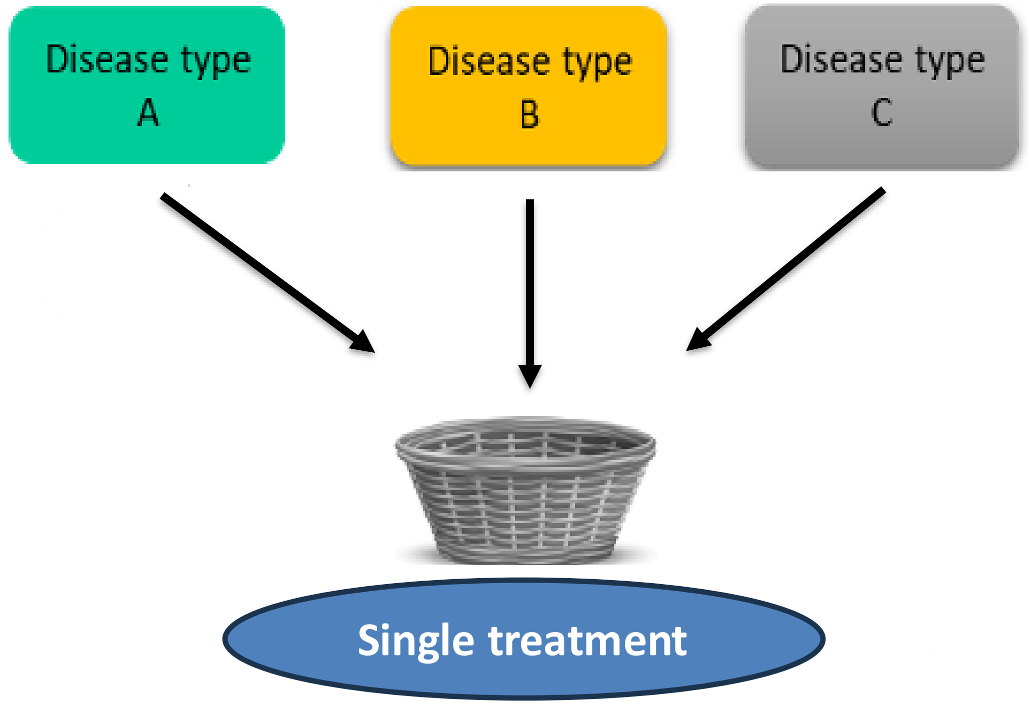
# Basket trials

These trials test a single treatment across different diseases that share a common molecular or genetic marker.

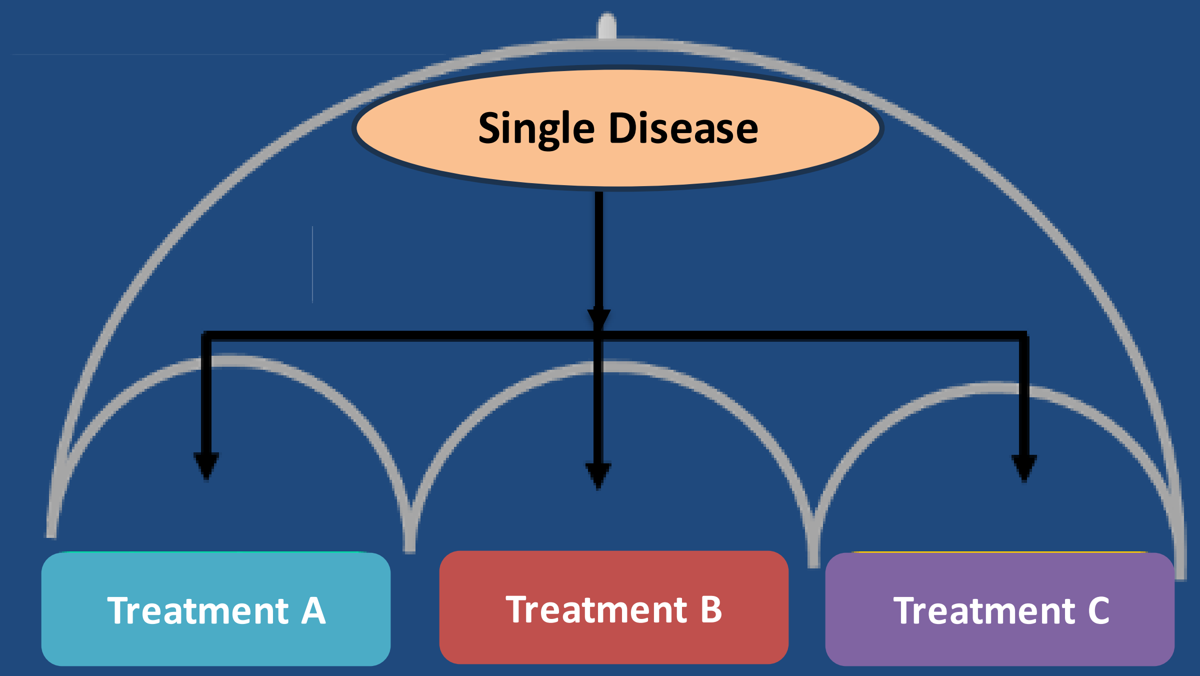



This design helps to quickly determine if a treatment is effective in multiple conditions with similar characteristics.

Innovative Trial Designs

# Umbrella Trials

These trials evaluate multiple treatments within a single disease.




This design is useful to test different treatments tailored to specific patient based on genetic profiles, allowing personalized therapy approaches to be tested simultaneously.

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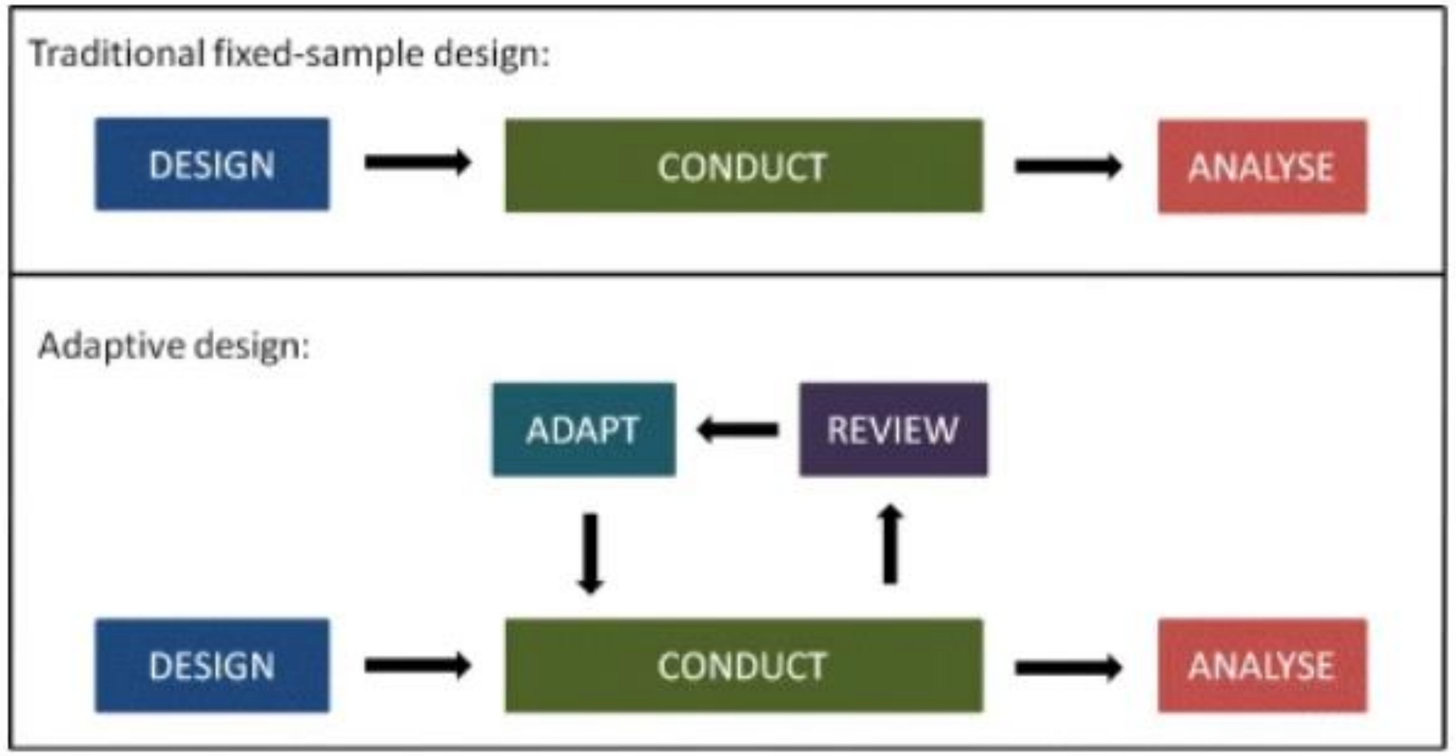
# Adaptive Trials

These trials allow modifications to aspects like sample size, treatment arms, or patient eligibility based on interim results without compromising trial integrity.

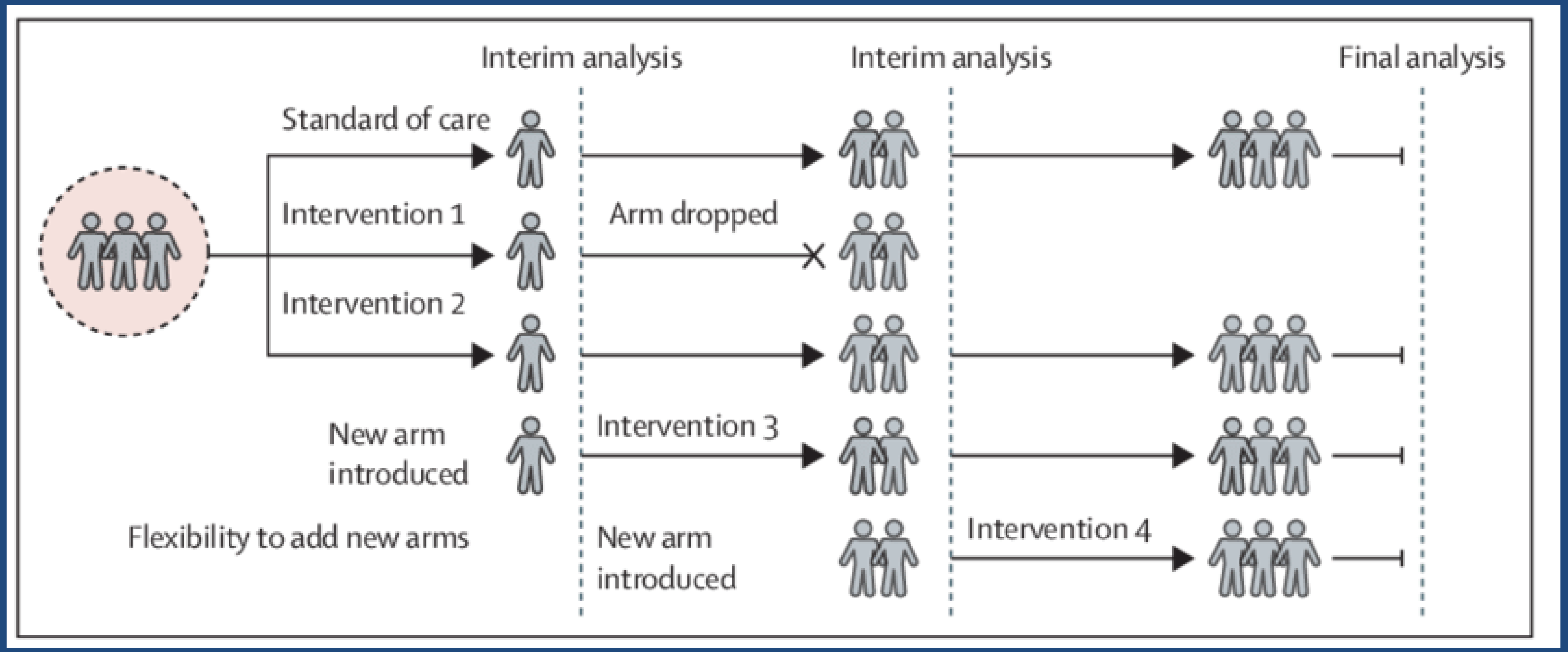



This design allows for higher flexibility that can lead to faster, more efficient studies by focusing on the most promising outcomes.

**Innovative Trial Designs**

# Platform Trials

In platform trials, multiple treatments for a single disease are tested within a single trial.




New treatments can be added, or ineffective ones dropped, making it a more efficient time-saving method for studying multiple therapies simultaneously

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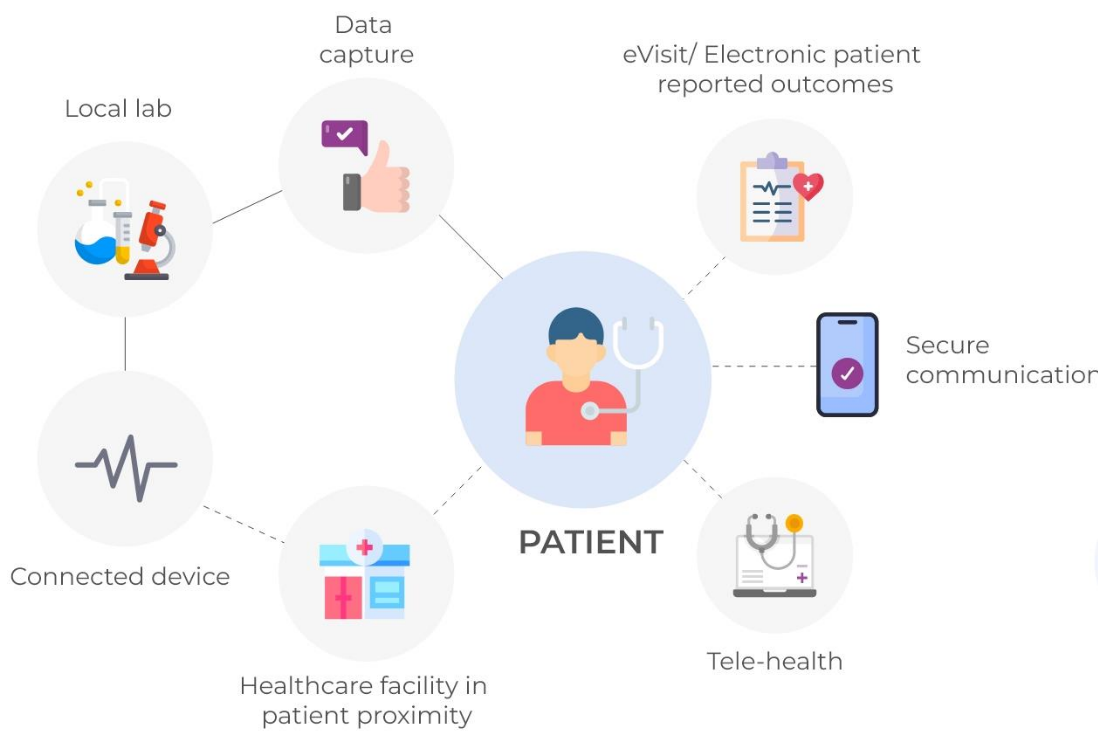
# Decentralised Clinical Trials (DCTs)

Decentralised clinical trials, or DCTs, allows for the conduction of trial activities at, or in the vicinity of, the participant's home, rather than at a traditional clinical site.

Digital Health Implementation makes possible to recruit, assess, and monitor trial participants without them having to leave their homes or their local healthcare environment.




Innovat
ive
Trial
Designs

DCTs redu e
participar avelling to
the investigator site

Allows for the inclusion of participants who live further away

Reduce the burden of participating in clinical trials

Facilitating patient recruitment.

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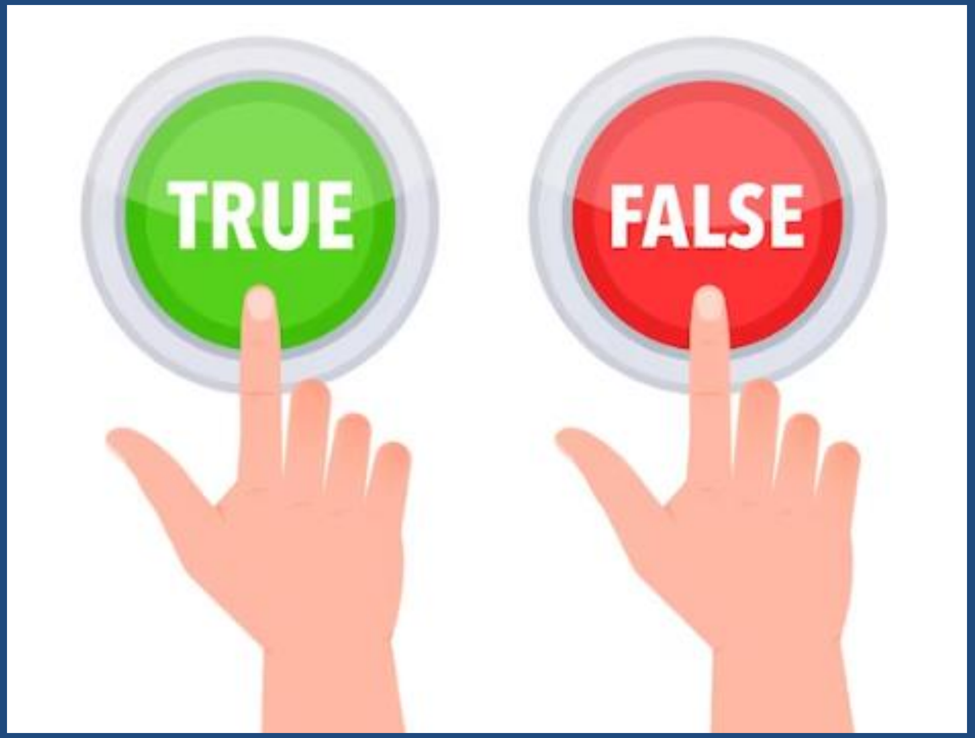


**1. Adaptive trial designs allow modifications to the study parameters based on interim results.**


**2. In basket trials, patients with the same disease are treated with different drugs to test efficacy.**

*(Basket trials treat patients with different diseases with the same drug.)*


**3. Decentralized clinical trials allow participants to complete many trial activities from their homes, reducing the need for frequent visits to clinical sites.**


**4. Umbrella trials test multiple drugs on multiple diseases within a single clinical trial framework.**

*(Umbrella trials test multiple drugs on different subtypes of the same disease.)*


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Innovative Trial Designs

Advanced therapy medicinal products (ATMPs)

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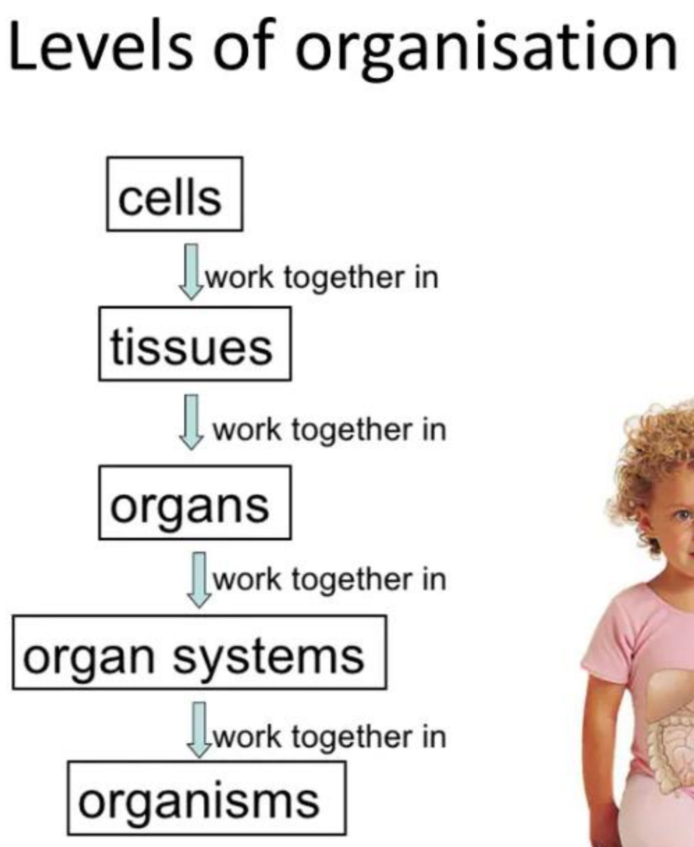
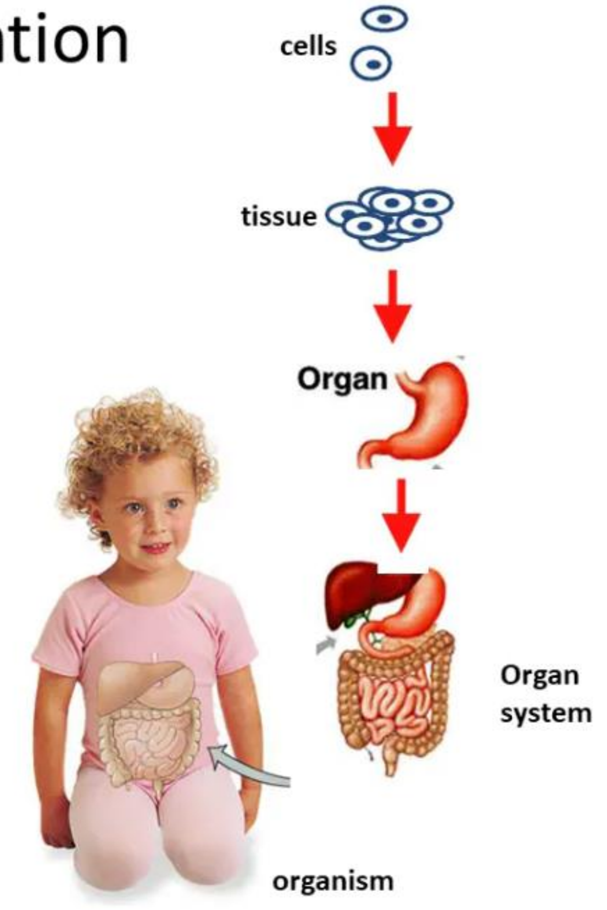
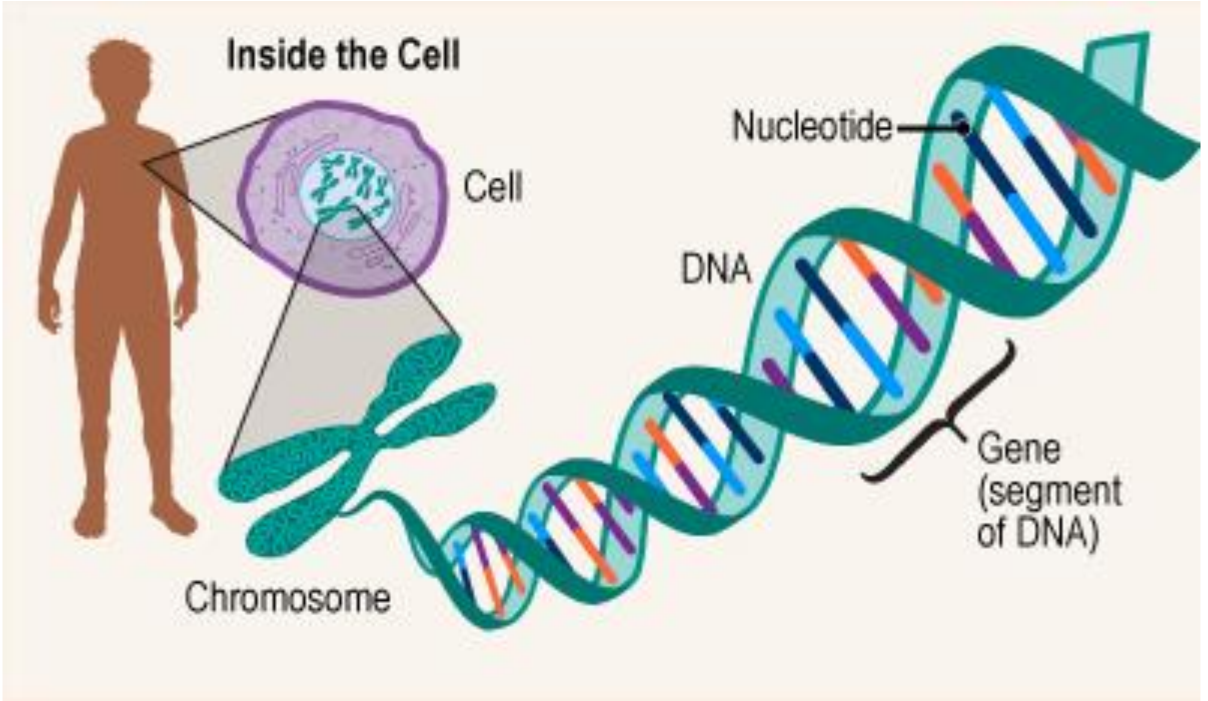
# Advanced therapy medicinal products (ATMPs)

ATMPs are a category of innovative therapies for human use based on genes, cells or tissues. They are used especially for treating rare or genetic diseases.




Advanced therapy medicinal products (ATMPs)

Do you know what a gene, cell, and tissue are?

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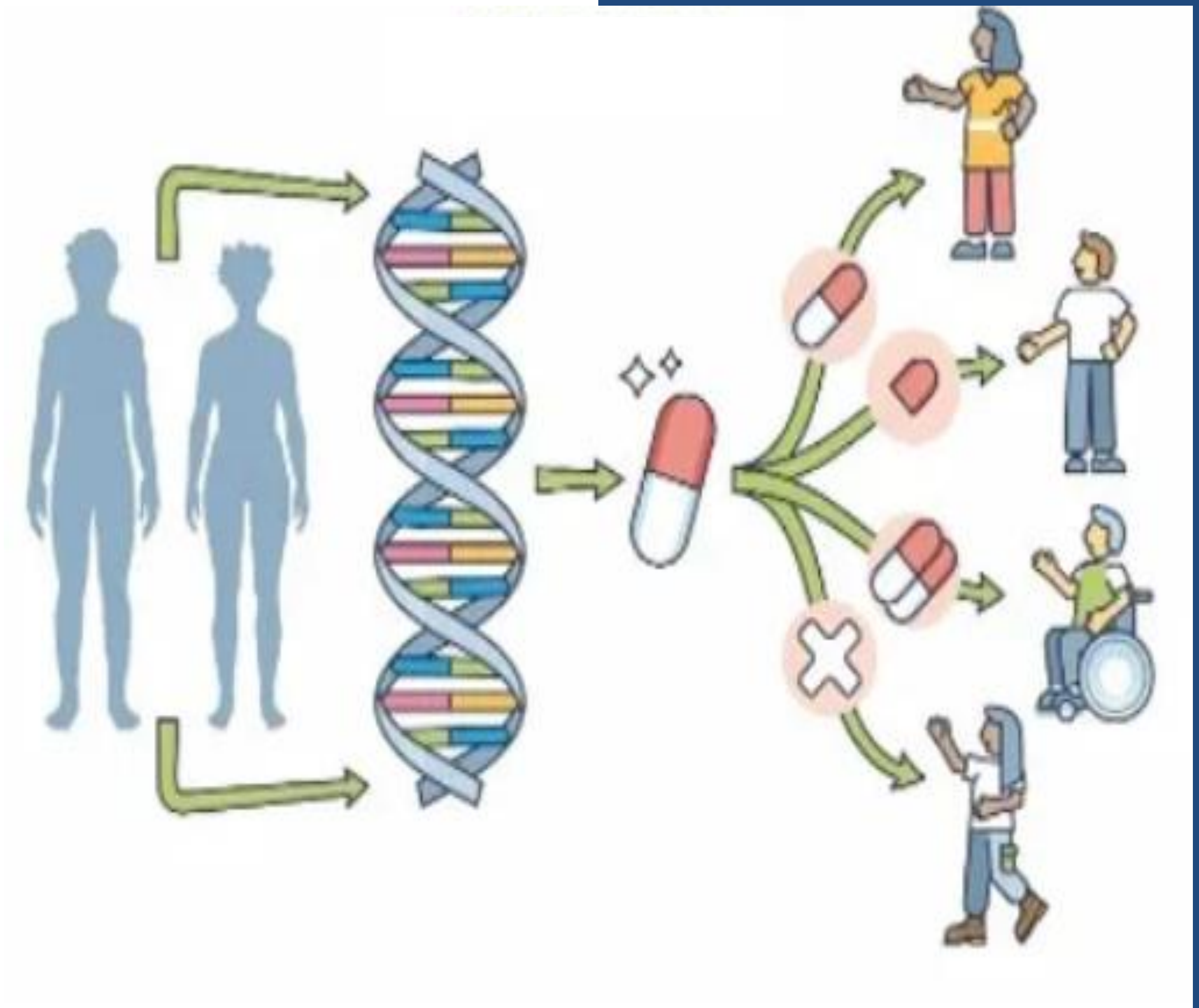
# Advanced therapy medicinal products (ATMPs)

ATMPs are mainly used to treat rare diseases especially in paediatric population.
Almost 70% of rare diseases have genetic origin and a paediatric onset.

ATMPs by targeting specific genes can potentially cure or alleviate the symptoms of genetic disorders where traditional treatments may be ineffective.

ATMPs enable personalized medicine, an innovative approach in healthcare that tailors treatment to each patient's unique characteristics.

This allows for more precise, effective, and targeted interventions, optimizing medical outcomes and minimizing potential side effects.



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1. ATMPs are innovative therapies that include gene therapy, **cell therapy** and tissue-engineered products.

2. One primary benefit of ATMPs is their potential to treat diseases that currently do not have **effective treatments**.

3. Gene therapy, a type of ATMP, works by introducing, removing, or altering **genetic materials** within a patient's cells.

4. ATMPs are mainly used to treat **rare diseases** especially in paediatric population.

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# Innovation

# Key Takeaways

Enhance access to care and patient involvement and allows for real-time health monitoring

Allows for personalized medicine to have precise, effective, and targeted interventions

Makes clinical research faster, more efficient, patient-centric, and data-driven

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**Innovation in paediatric clinical research leads to healthier and happier kids, who can live better lives and grow up strong and well.**


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