Medical Biotechnology

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- **Medical biotechnology** is a diverse discipline dedicated to improving human health.
- It includes a variety of topics but mostly focuses on disease prevention, disease diagnosis, and disease treatment.

What is a disease?

- A **disease** is a condition that impairs normal bodily functioning.
- Examples of diseases include: Heart disease, Cancer, Diabetes, HIV/AIDS, Asthma.

Early Applications of Medical Biotechnology

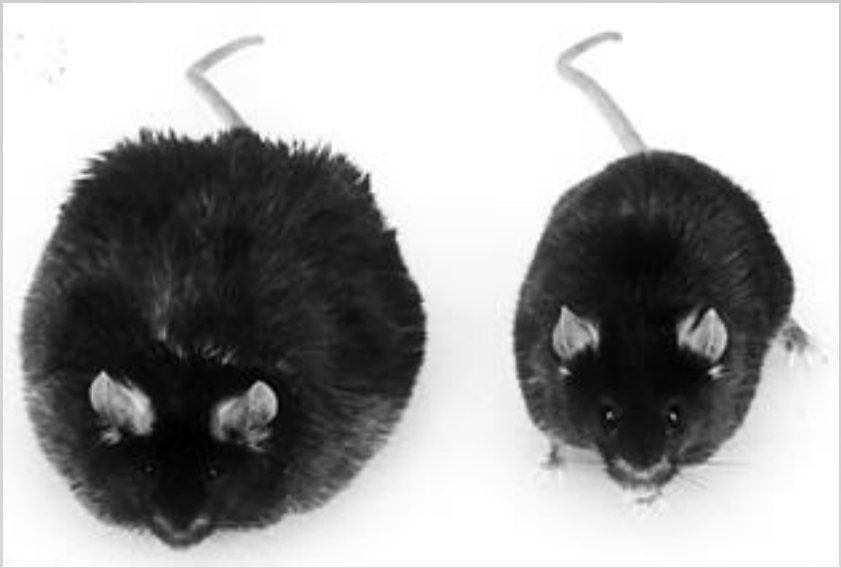
- Leeches were used to treat illnesses by “bloodletting”
- Leeches are now being studied again for their enzymes (in saliva that dissolve blood clots)

Gene conservation

- Many important genes are highly conserved across many different species
- Relation to human genes is based on DNA sequence similarity
- Related genes in different species are called **homologues**

Homologues

- Genes involved in human illness can be discovered in a **model organism** to learn about function
- Mice (pictured right) can become obese if they lack a single gene called *ob*
- *ob* codes for **leptin**, a protein hormone that helps regulate hunger



- Human homologue discovery reveals that genetics may influence weight disorders

Model Organisms

- **Model organisms** are nonhuman organisms that scientists use to study biological processes in experimental lab conditions.
- Examples include mice, rats, worms, fruit flies, & bacteria.
- Many human genetic disease occur in model organisms.

- Scientists use them to identify disease genes and test therapy approaches for **efficacy** (effectiveness) and safety before use in human clinical trials.

Relationships

The Human Genome Project shows that we share large numbers of genes with other organisms:

- About 31% related to yeast
- About 40% related to roundworms
- About 50% related to fruitfly
- About 90% related to mice

However, humans make many more proteins!

Pharmaceutical Industry

- The **Food and Drug Administration (FDA)** is a US regulatory agency that oversees food, drugs and devices. It also monitors clinical trials and approves new drugs that have succeeded.

- **Pharmaceutical companies** are those licensed to discover, develop, market, and distribute drugs.

- **Drugs** (according to the FDA) are defined as substances intended for use in diagnosis, cure, treatment, or prevention of disease.

Preclinical Testing

• The first step is to identify a potential drug candidate, called a **lead compound.**

•Once a lead compound has been identified in the lab, it begins years of testing to evaluate its safety and efficacy.

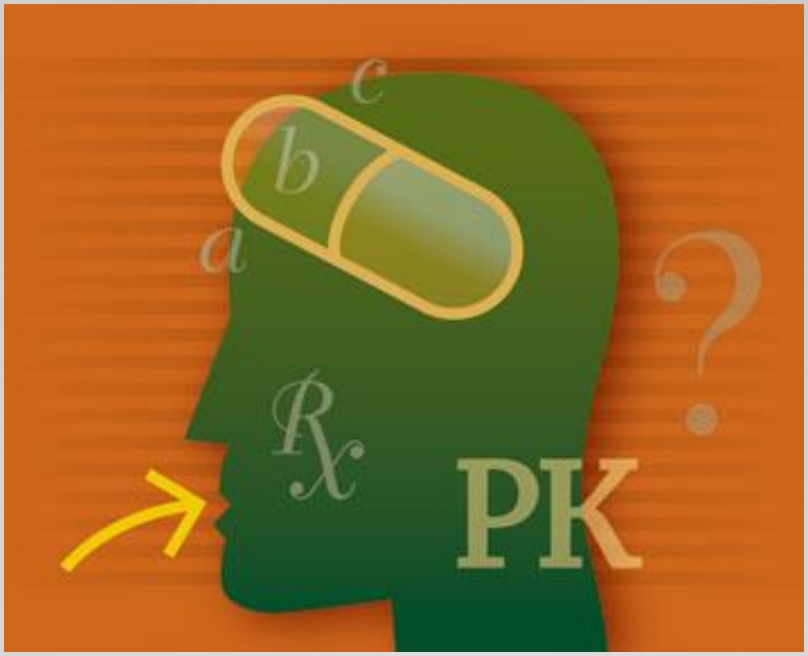
• *In vitro* studies are carried out to identify the way it interacts with its target.

• After that, tests on model organisms (animals that have the targeted disease) take place.

• This usually lasts around 6-7 years.

Preclinical Testing

• Many preclinical tests include **pharmacokinetics** - the study of how drugs move through living organisms.



• Four processes are examined in pharmacokinetic studies: *absorption*, *distribution*, *metabolism*, & *excretion*

• Other tests include **chemistry** tests on *purity*, *stability*, of product, as well as *dosing* and *administration* (tablet, injection, etc…)

Investigational New Drug (IND) Application

After preclinical testing is completed, a company files an IND application with the Food & Drug Administration (FDA) prior to any testing on humans.

This application includes:

- results of preclinical studies
- chemical structure & properties
- pharmacokinetics
- side effects
- clinical trial plan

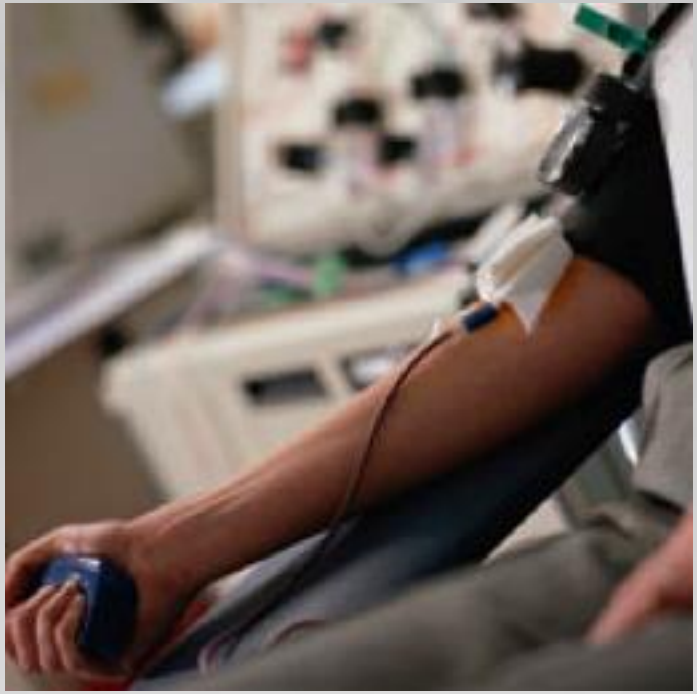
Clinical Trials

Clinical trials are a process of testing products prior to approval of a drug or treatment plan for widespread use in humans.

- Begin after the use of model organisms
- Most are classified into 3 phases: I, II, & III

Phase I

- Initial studies of drug in a small number (20 -80) of healthy volunteers to determine its safety
- Intended to determine safe dose range



- Pharmacokinetic studies determine dosing & limitations of use
- Take 6 months - 1 year to complete

Phase II

- Larger groups of people (100 - 300) with the disease are used to study efficacy
- Safety is further evaluated
- Side effects also studied
- Optimal dose strength and schedule (once or twice daily)
- Dose-response relationship evaluated to visualize drug performance
- Usually about 2 years to complete

Phase III

- Medicine is tested in large, randomized, controlled trials with larger numbers (1000 - 5000) of patient volunteers using

- Continued evaluation of efficacy & identification of side effects.

- Can take 1-4+ years (commonly 3)

New Drug Application (NDA)

- If findings from clinical trials prove safe and effective, then company files new drug application (NDA) with FDA
- Data must support that drug is more effective than **placebo** (a substance with no pharmacological effect) or drug offers alternative to existing medication

Phase IV/ Ongoing studies

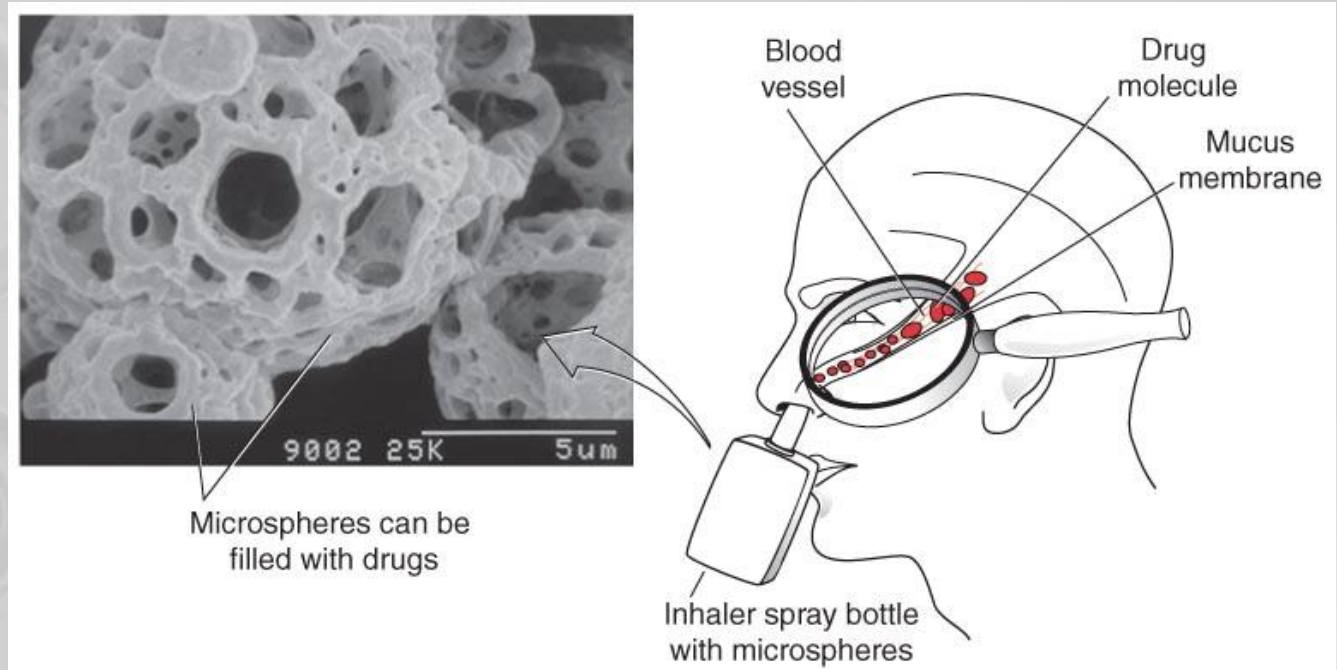
- Even after a product is on the market, studies & safety monitoring continue
- FDA requires periodic reports including cases of adverse events
- Continues for years
- Occasionally, new **indication** (intended use for drug) may be found

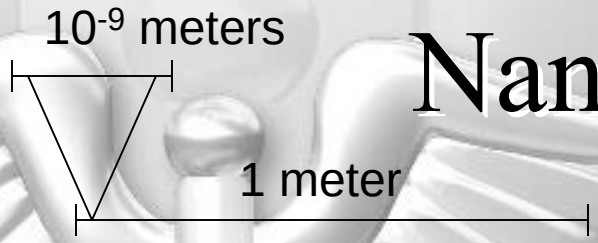
Biotechnology in medicine

Drug delivery

- Getting drug to target organs and tissue
- Microspheres

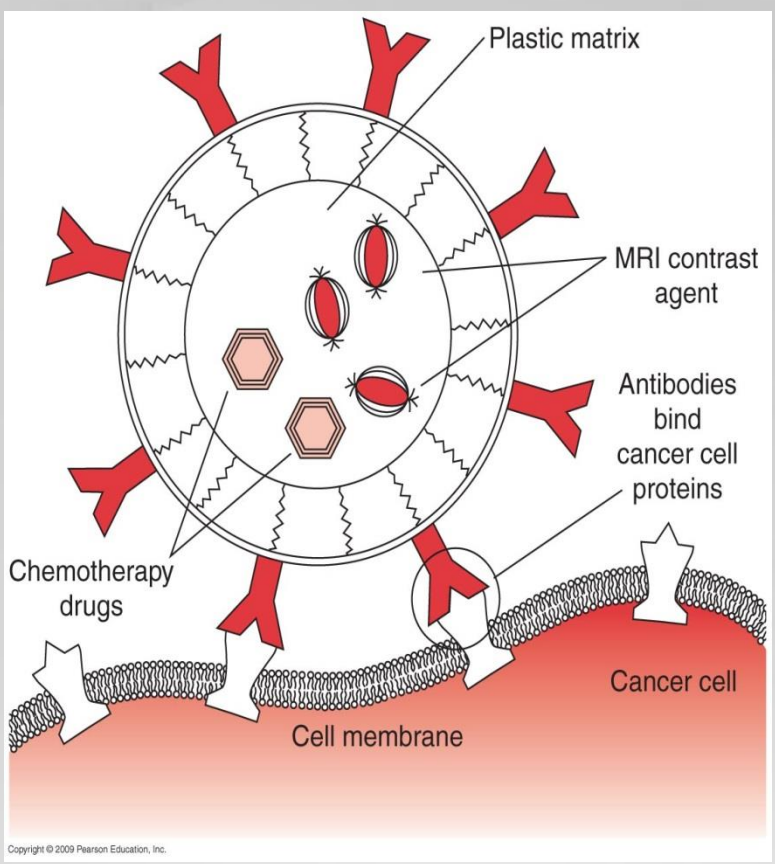
Insulin delivered as a powder through an inhaler





Nanomedicine

- Nanometer is one billionth of a meter
  - May be used for delivery of small sensors to target sites in body
  - Unclogging arteries
  - Detect and destroy cancer cells



Artificial blood

- Cell-free solutions containing molecules that can bind and transport oxygen like hemoglobin
- Benefits
  - Disease-free alternative to real blood
  - Constant supply
  - Universal donor type
- Disadvantages
  - Cannot perform all the functions of a red blood cell-only oxygen delivery