GENETICS
LESSON 12: GENETIC TECHNOLOGIES

Learning Outcomes

- **I can describe different technologies used to manipulate genes.**

Scientific Gene Manipulation

- in addition to using breeding practices to influence the genetic makeup of an individual or species, scientists can now directly modify or change the genetic code of an individual or species

1. Cloning
   - producing an identical copy of an **organism**
   - involves **replacing** the entire DNA within an egg with the DNA of the individual to be copied, and then **implanting** the new egg into a host mother

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

Dolly the sheep.

2. Gene Cloning
   - producing many identical copies of a specific gene in a **bacterial** host cell
   - these copies can then be used for **research** or for making **transgenic** organisms.

3. Transgenic Organisms
   - the introduction of a **foreign gene** into an organism to introduce a new **trait**
   - often done using **recombinant** DNA technologies (i.e., the method used for creating transgenic organisms) – using a **vector** (often a manipulated virus) to inject the new gene.
   - called **Genetically Modified Organisms** (GMO's)
     - Green fluorescent protein as a **tracer**
     - golden rice – higher **vitamin A** and **iron** content
     - tomatoes that can grow in **colder** climates
     - increased crop resistance to **herbicides** or infections
     - **insulin** producing bacteria
     - modifying milk production in goats to produce human **medical** products (growth hormones, anti-clotting factors, etc.)
     - more **compatible** pig organs for human transplant operations
     - https://www.youtube.com/watch?v=7TmcXYp8xu4

4. Reproductive Technologies
   - procedure used to **increase** the chance of becoming pregnant
   - artificial **insemination**

    - collection and concentration of **sperm** cells that are then placed into the female's **uterus**
    - also used in **domestic** animals
  - in vitro fertilization (i.e., test tube babies)
    - combining **sperm** and **egg** cells in glassware for fertilization, allowing the embryos to start to grow, then **implanting** the embryo(s) into the mother's uterus
  - intracytoplasmic sperm injection (form of in vitro fertilization)
    - directly **injecting** the sperm DNA into an egg for direct fertilization
    - embryo is **implanted** in mother's uterus

5. Gene Therapy
   - replacing a missing or **defective** gene with a healthy gene to cure a genetic disorder – this is **not** introducing genes from a different species
   - used to **increase** recognition of diseased cells to the **immune** system
   - new gene is **inserted** using a viral vector
   - first **approved** use in US
     - used to **treat** a 4 year old girl for ADA-SCID (a severe autoimmune deficiency disorder; called "**bubble** children")
       - https://www.uclahealth.org/ucla-pioneers-therapy-cure-for-children-with-bubble-baby-disease
       - SCID video https://youtu.be/B0NVTC8Fy3w
     - received injections every 2 months – achieved **temporary** success
   - since then, there have been many **clinical trials** and some FDA and European Medicines Agency (EMA) approvals for gene therapy treatment
     - **successful** treatment of ADA-SCID in multiple children
     - treatment of **hemophilia**
     - treatment of **sickle** cell disease
     - treatment of **spinal muscular atrophy** – loss of motor neurons leading to muscle wasting
     - treatment of simian **HIV** in monkeys
   - Potential Gene Therapy for Spinal Cord Injury without Side Effects
     - **Neuropathy** is the damage of nerves resulting in pain, numbness, tingling, and muscle dysfunction.
     - **Opioids** (morphine, oxycodone (OxyContin), hydrocodone (Vicodin), methadone) commonly prescribed for pain management.
       - adverse effects, including severe withdrawal and dependence
     - Gamma-aminobutyric acid (**GABA**) is a neurotransmitter that suppresses nerve impulses between neurons (pain receptors in this case).
     - **Injection** of GABA gene therapies (*gad65* and *vgat*) into **sciatic** injury in mouse spine successfully blocked pain signaling in mouse injured area lasting for at least **2.5 months** post-treatment.