Primary disciplinary learning goal

- To understand the processes involved in these three technologies, and the fact that research is proceeding at an ever-increasing rate.
    - Gene therapy- put functional genes into cells with non fuctio0nal genes
        - Remove he good genes- use restriction enzymes
        - Make copies – use pcr
    - A gene gun- shoots genes into cells
    - Liposomes-
        - Put dna in a fatty sphere
        - The sphee then joins with the fats in a cell membrane
        - The dna gets into the cell wall
    - There are 0 fda approved gene therapies
        - The work is underway
            - Faulty immune system
            - A type of blindness
            - Leukemia and myeloma
            - HIV
            - Heart disease
            - Hemophilia
            - High blood fat
    - Cloning- clones consist of individuals that are genetically identical to one another.
        - Phenotype: the genotype interacting with environment
        - That cell is genetically identical to all of your

- To understand the potential that these technologies have in the treatment – and perhaps cure – of human diseases and disorders.
- To appreciate ethical issues related to the misuse of these technologies.
- To appreciate that ethical issues in medicine have a long history in the U.S., as in the infamous Tuskegee study on syphilis.

- Cell theory: all living things are made of one or more cells. Thus, many biologists do not consider viruses to be living things – viruses aren't cells.
- Cells are 'bags of life' separated from the outside world. An outer membrane (made of fats and proteins) acts as a barrier with 'gates.' Not all materials can enter or leave through the gates in the membrane – in other words, these gates are selective.
- Within cells, different structures, called organelles, perform different tasks.
- Ribosomes are the sites of protein synthesis (where mRNA codons and tRNA carrying amino acids come together in the process of translation).
- Mitochondria produce energy by respiration: glucose + oxygen -> carbon dioxide + ATP. We can think of ATP as energy in storage (a bit like a battery). Useable energy is released when ATP -> ADP (i.e., when ATP loses one of its phosphate groups – *tri*phosphate becomes *di*phosphate).
- Chloroplasts (in plants only) produce sugars by photosynthesis: carbon dioxide + water + sunlight -> sugars + oxygen.
- Note how the equation for photosynthesis is almost the mirror image of that for respiration. What might that mean at an ecological level?
- Cells with a distinct nucleus and organelles surrounded by membranes are called eukaryotes. Cells without, such as bacteria, are called prokaryotes.
- Eukaryotes and prokaryotes differ in many aspects of how they 'earn a living.' Eukaryotes are like slow, fancy cadillacs whereas prokaryotes are more like speedy, stripped-down race cars.
- Plant, animal and bacterial cells are linked in a complex web of ecological interactions involving respiration and photosynthesis.
- How can the diverse phenotypes of cells in a living body be produced when those cells have the same genotypes? It all depends on which genes are turned on, when and where.
- The goal is to replace a defective gene with a functional copy, and so cure – well, at least treat – a genetic disease. But, would the offspring of such a cured individual be guaranteed a disease-free life? Not necessarily, but do you know why?
- Remember our ISICU strategy? First, *I*solate a functional gene, then *C*opy it and finally *U*se it by putting it into an affected person.
- Fatty blobs (liposomes) and gene guns can be used to put DNA into cells.
- Viruses are also used as so-called vectors to 'infect' human cells carrying defective genes with functional copies. That's what parasitic viruses normally do – inject genetic material (their own, of course) into host cells.

- But the process isn't fool-proof. For example, people may mount an immune response against the virus, destroying both it and the good genes. Or, the virus may place its payload of genes into the human DNA but in the wrong place. This might cause cancer (why?).
- Overall, current successes for gene therapy are quite limited, but there is promise for the future. But, could all human diseases ultimately be fixed by gene therapy? And if not, why?

ASK YOURSELF … As clever genetic technologies mature (and they surely will), should society support causing genetic changes to DNA that can be inherited across generations?

STEM CELLS

- Stem cells have the potential to develop into any kind (or almost any kind) of mature cell type. Not surprisingly, embryonic cells seem to be the most useful stem cells, called totipotent stem cells. Of course, embryonic cells have to develop into every kind of cell present in the adult body. Pluripotent stem cells are less versatile, and committed stem cells are the least versatile.
- If stem cells can be coaxed to develop into specific cell types, then we may be able to devise better treatments for such conditions as Parkinson's disease (a brain disorder), spinal cord injury and Type I diabetes (and many more).
- There's lots of experimental work going on. One troubling observation is that stem cells sometimes develop into tumors rather than the desired cell type.
- One success story so far (it made it into all of the media) involves the growth of a windpipe (or trachea, connecting the throat to the lungs) using stem cells taken from a person's bone marrow.
- Is it ethical to destroy a very young embryo in order to get the most valuable (totipotent) stem cells, even if doing so benefits others? Do the ends justify the means? What if such embryos would be destroyed anyway?
- The Bush administration put restrictions on federally-funded research on stem cells in the U.S. (such research was restricted, but not banned). Many scientists and some politicians feared that the U.S. might lose its edge if stem cell research shifted abroad to countries with fewer regulations. Some States revolted, e.g., California, with its stem cell initiative.
- With the advent of the Obama administration, restrictions on the use of embryonic stem cells were relaxed somewhat – which isn't to say that regulations on and legal challenges to their use in research aren't continuing.