AP Biology Review

Unit 1:

- Chemical Bonds

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- Covalent bonds: share valence electrons
 - Nonpolar/polar
 - Ionic bond: transfer of charges

• IONIC BONDS ARE STRONGER

- Property of Water
 - Polar: Oxygen is more electronegativity
 - Hydrogen bonds: Hydrogen of one, to negative Oxygen
 - Water can form 4
 - Cohesion: Water to water (surface tension)
 - Adhesion: water to another surface
 - Transpiration: how water moves up plants. Water to water; water to xylem
 - Specific heat: the amount of heat necessary to change substance by 1*C. Water has high specific heat, so temp is stable
 - Insulation/Density: Ice is less dense than water
 - Universal solvent
 - Hydrophilic- water soluble
 - Hydrophobic- not soluble in water
 - o pH: 0-14
 - acids, base, buffer, carbonic acids
 - Dehydration synthesis, Hydrolysis
- Biological Molecules

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- All organic molecules have Carbon. Why?
 - Has 4 valence electrons; can form up to 4 covalent bonds
 - Single, double, and triple covalent bonds possible
 - Can form large molecules
 - Backbone can be chained, rings, or branched
 - ISOMERS: molecules with same chemical formula, but different atom arrangement==diff. properties

Function	al Group	Class Name	Examples	Characteristics
- ОН	hydroxyl	alcohols	ethanol, glycerol, sugars	polar hydrophilic
- с ^{#0} ОН	carboxyl	carboxylic acids	aceticacid, aminoacids, fattyacids, sugars	polar, hydrophilic, weakacld
- N H	amino	amines	aminoacids	polar, hydrophilic, weakbase
0 	, phosphate	organic phosphates	DNA, ATP, phospholipids	polar, hydrophilic, acid
0 = - C -	carbonyl	ketones	acetone, sugars	polar, hydrophilic
0 -С-н	carbonyl	aldehydes	formaldehyde, sugars	polar, hydrophilic
н - с – н	methyl	_	fatty acids, oils, waxes	nonpolar, hydrophobic

- Functional Groups:
- Macromolecules!
 - Carbohydrates : monosaccharides, disaccharides, polysaccharides, glycosidic linkages

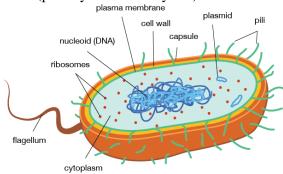
- Main functions of polysaccharides: energy storage and structural support
 - Energy storing
 - Starch (plants)
 - Glycogen (animals)
 - o Structural support
 - Cellulose
 - Chitin
- Proteins: amino acids, peptides, polypeptides, peptide bonds
 - Primary structure, secondary, tertiary, quaternary
 - Alpha helix, beta pleated sheets
 - Protein shape ; denaturation
 - Denatration caused by heat, pH change, others
- Nucleotides: DNA and RNA, nucleotides, phosphodiester bonds, hydrogen bonds, etc.
 - Nucleotides: nitrogenous base, pentose sugar (deoxyribose or ribose), phosphate group
 - Purines (double ring): A, G
 - Pyrimidines (single ring): C, U, T
- Lipids (not technically a macromolecule, b/c can't form polymers)
 - All hydrophobic
 - Fats (triglycerides) made off one glycerol and three fatty acids
 - Fatty acids=nonpolar/hydrophobic
 - Saturated fatty acid (no double links, solid, animals)
 - Unsaturated (double links, liquid, plants)
 - Function:

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- Energy storage, insulation/protection of organs
- Phospholipid membranes
- Steroids (cholesterol, estrogen, testosterone)
- REMEMBER: Change structure changes function

Unit 2

- Cells (prokaryote vs. eukaryotes)



Organelle	Animal/Plant	Function	l		
Rough ER	Both	Protein synthesis for secretion using ribosome, hydrogen			, hydrogen
C			and steroid hormo		
Smooth ER	Both	Regulates and release calcium ion and processing of toxins			
Nucleus	Both		hereditary information		
		chromati	'n		-
Plasma Membrane	Both	Made of	phospholipids encl	oses membrane	
Ribosome	Both	Makes p	rotein by translating	g mRNA, bound to	o ER/nuclear
		envelope	e or free in cytoplas	m	
Golgi apparatus	Both	Synthesi	s, modification, sor	ting, and secretion	of cell products,
		namely p	proteins		
Lysosome	Animal	Digests a	and recycles macro	molecules by hydr	olysis
Mitochondria	Both	Cellular	respiration and AT	P generation	
Peroxisome	Both	Metaboli	izes hydrogen peroz	xide	
Microvilli	Both	Increase	cell surface area fo	or more efficiency	
Cytoskeleton	Both	Reinforc	e cell shape, cell m	ovement; microfil	aments,
		intermed	liate filaments, mici	rotubules	
		Property	Microtubules	Microfilaments (Actin Filaments)	Intermediate Filaments
		Structure	Hollow tubes; wall consists of 13 columns of tubulin molecules	Two intertwined strands of actin	Fibrous proteins supercoiled into thicker cables
		Diameter	25 nm with 15-nm lumen	7 nm	8–12 nm
		Protein subunits	Tubulin, consisting of α-tubulin and β-tubulin	Actin	One of several different proteins of the keratin family, depending on cell type
		Main functions	Maintenance of cell shape (compression-resisting "girders") Cell motility (as in cilia or flagella) Chromosome movements in cell division Organelle movements	Maintenance of cell shape (tension-bearing elements) Changes in cell shape Muscle contraction Cytoplasmic streaming Cell motility (as in pseudopodia) Cell division (cleavage furrow formation	Maintenance of cell shape (tension-bearing elements) Anchorage of nucleus and certain other organelles Formation of nuclear lamina
Centromere	Both		ell microtubules are	e initiated, contain	centrioles in A,
Centrioles	Animals	but not in		an aall dissiatan	
			on of spindle fiber f		nodo of
Flagellum	Animal, some plant	microtub	animal cells (sperm	is), for transport, in	liade of
	sperm	Iniciolul	Jules		
Chloroplast	Plant	Photosyr	othesis		
Central Vacuole	Plant	~	und in older plant c	alle storage and h	reakdown of
Central Vacuole	1 Iani		ydrolysis of macror	•	
		membrai	-	noiceules. Enclose	d by the tonoplast
Plasmodesmata	Plant			· connect cytoplas	m of adjacent cells
		Channels through cell walls; connect cytoplasm of adjacent cells Protection, structural integrity of plant cell			in or adjucent cents
Cell wall	Planis				
Cell wall Extracellular Matrix	Plants Animal				ells
Extracellular Matrix	Animal	Tight Ju	nctions: cell membr		ells
		Tight Jun fused=w	nctions: cell membr atertight	rane of neighbor co	
		Tight Jun fused=w Desmoso	nctions: cell membr atertight omes: fasten adjace	rane of neighbor co	
		Tight Jun fused=w Desmoso cells into	nctions: cell membr atertight	rane of neighbor contracted and the set of neighbor contracted and the set of	ke rivets, fasten

Membrane Mosaic: (more unsaturated fats means more flexibility)

- Phospholipid= the membrane base
- Proteins
 - o transport channel
 - receptors for ligands (signal molecules)
 - peripheral proteins (hang loosely from membrane surface, like G protein)
- Carbohydrates=cell-cell recognition ; crucial for immune response and tissue differentiation
- Selective permeability
 - Nonpolar molecules easily pass
 - Ions and polar molecules struggle b/c hydrophilic. Require facilitated transport. Require transport proteins
 - Work by:
 - Provide channel so molecule can pass
 - Bind loosely to molecule and carry through membrane
 - Water move in aquaporins
 - Active transport (move against concentration gradient) (use energy aka ATP)
 - Ex. Sodium-potassium pump
 - Creates a membrane potential which is restored to resting by a chemical force
 - (ion conc. Gradient) and a voltage gradient, together "electrochemical gradient
- Exocytosis/endocytosis

Tonicity

- Isotonic: balanced on both sides, water travel at same rate
- In hypertonic cell, cell lose water b/c more solute in water around the cell. Cell shrivel
- In hypotonic cell, cell enter water faster than leaves b/c fewer solutes in water surrounding than in cell

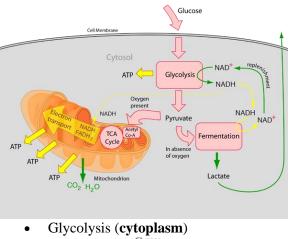
Unit 3

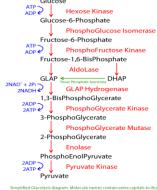
Enzyme: macromolecules that are biological catalust

- Work by lowering the activation energy of a reaction w/o changing the free energy change of reaction
 - Reactant of enzymatic reaction "substrate"
 - Enzyme most important has ACTIVE SITE (has a shape specifically complementary to substrate). Enzyme+substrate= enzyme-substrate complex
 - Enzyme shape/activity affected by pH, temp, etc. b/c made up of proteins
 - Cofactors: nonprotein helpers of reaction, like metal ions etc.
 - If organic "coenzyme": vitamins ex.
 - Competitive inhibitor: occupy active site and prevent activity
 - Noncompetitive inhibitor: change shape of active site by binding to another site nearby and changing enzyme shape
 - Enzyme regulator mostly bind to allosteric site (specific binding site not the active site)
 - Inhibition: feedback inhibition (product inhibits reaction by binding to allosteric site). Allow more efficiency by turning pathway off when end product is too much

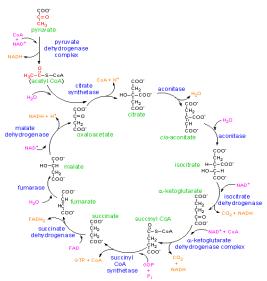
Metabolism: the totality of an organism's chemical reaction

- Catabolic pathway: release of energy b/c of breakdown of complex molecules
- Anabolic pathway: consume energy to build
- Energy: ability to do work. Kinetic, potential, chemical
- Laws of Thermodynamics:
 - First law: energy of universe is constant, and energy can only be converted; not created/destroyed
 - Second law: every energy transformation increases the entropy (disorder) of the universe
- Free energy: the part of a system's energy that is able to perform work when the temperature is constant
 - Exergonic: release energy; $\Delta G < 0$
 - Endergonic: absorb energy; $\triangle G > 0$
- Energy coupling: use exergonic process to drive endergonic process (ATP production).
- Cellular Respiration

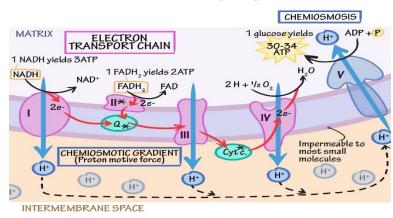




• Citric Acid/Krebs Cycle (in Matrix of Mitochondria, Cytoplasm in Prokaryotes)

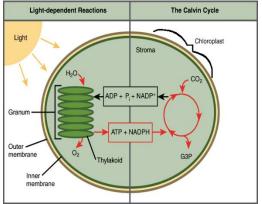


- Function is to provide electrons to fuel oxidative phosphorylation
- Oxidative Phosphorylation •

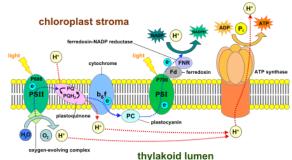


Oxidative Phosphorylation

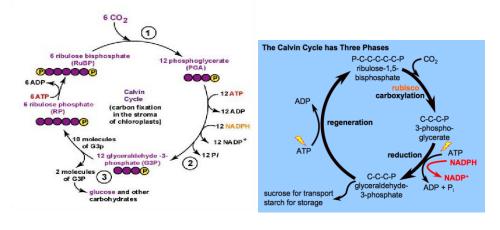
- Coenzyme q "ubiquinone" V= "ATP synthase"
- Photosynthesis •



• Light Dependent Reaction (across thylakoid membrane)



- (cyclic photophosphorylation): synthesis of ATP coupled to electron transport activated by Photosystem I solely. Photolysis of water doesn't occur
- (noncyclic photophosphorylation): both PSII and PSI. photolysis occurs
- Light Independent Reaction (Calvin Cycle) (in stroma of chloroplast) (takes two turns for 1 glucose



Unit 4

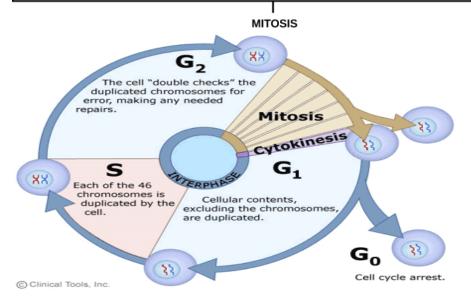
Cell Communication: Reception, Transduction, Response, Ligands, Receptors

- Intracellular receptors : found within the plasma membrane; hydrophobic ligands b/c the signal molecule must be able to cross plasmam membrane
- Plasma membrane receptor: bind to water-soluble ligands
- G protein-coupled receptor: ligand binds to G protein receptor; G protein is activated with GTP; G protein binds to specific enzyme and activates
- Receptor tyrosine kinase: ligand binds and causes dimer conform which causes ATP to become ADP, activates phosphorylated tyrosine
- Ligand-gated ion channels: open and close to regulate specific ions' flow, opens when ligand is attached
- Phospohorylation cascade, protein kinases, protein phosphatase (remove phosphate group from kinase and turn off response), second messengers (Ca2+, cAMP)
- Receptors depend on shape of binding site, so temp. pH and environment matter

Feedback: Positive and Negative, examples

Prophase	Prometaphase	Metaphase	Anaphase	Telophase	Cytokinesis
 Chromosomes condense and become visible Spindle fibers emerge from the centrosomes Nuclear envelope breaks down Nucleolus disappears 	 Chromosomes continue to condense Kinetochores appear at the centromeres Mitotic spindle microtubules attach to kinetochores Centrosomes move toward opposite poles 	 Mitotic spindle is fully developed, centrosomes are at opposite poles of the cell Chromosomes are lined up at the metaphase plate Each sister chromatid is attached to a spindle fiber originating from opposite poles 	 Cohesin proteins binding the sister chromatids together break down Sister chromatids (now called chromosomes) are pulled toward opposite poles Non-kinetochore spindle fibers lengthen, elongating the cell 	 Chromosomes arrive at opposite poles and begin to decondense Nuclear envelope material surrounds each set of chromosomes The mitotic spindle breaks down 	 Animal cells: a cleavage furrow separates the daughter cells Plant cells: a cell plate separates the daughter cells

Cell Cycle: Apoptosis, stages,



Density-dependent inhibition: cell too crowded so stop divided

Anchorage deppendncy: cell eeds to bind to some substratam to divide

Unit 5:

Meiosis: (chiasmata; chiasma is the point of contact, the physical link, between two chromatids belonging to homologous chromosomes. Kinetochore: a complex of proteins associated with the centromere of a chromosome during cell division, to which the microtubules of the spindle attach)

	Stage	Event	Outcome
INTERPHASE	S phase	Nuclear envelope Centrosomes (with centriole pairs) Chromatin	Chromosomes are duplicated during interphase. The resulting sister chromatids are held together at the centromere. The centrosomes are also duplicated.
	Prophase I	Sister Chiasmata chromatids Tetrad	Chromosomes condense, and the nuclear envelope fragments. Homologous chromosomes bind firmly together along their length, forming a tetrad. Chiasmata form between non-sister chromatids. Crossing over occurs at the chiasmata. Spindle fibers emerge from the centrosomes.
_	Prometaphase I	Centromere (with kinetochore)	Homologous chromosomes are attached to spindle microtubules at the fused kinetochore shared by the sister chromatids. Chromosomes continue to condense, and the nuclear envelope completely disappears.
MEIOSIS	Metaphase I	Microtubule attached to kinetochore	Homologous chromosomes randomly assemble at the metaphase plate, where they have been maneuvered into place by the microtubules.
	Anaphase I	Sister chromatids remain attached	Spindle microtubules pull the homologous chromosomes apart. The sister chromatids are still attached at the centromere.
	Telophase I and Cytokinesis	Cleavage	Sister chromatids arrive at the poles of the cell and begin to decondense. A nuclear envelope forms around each nucleus and the cytoplasm is divided by a cleavage furrow. The result is two haploid cells. Each cell contains one duplicated copy of each homologous chromosome pair.
	Prophase II		Sister chromatids condense. A new spindle begins to form. The nuclear envelope starts to fragment.
	Prometaphase II		The nuclear envelope disappears, and the spindle fibers engage the individual kinetochores on the sister chromatids.
MEIOSIS II	Metaphase II		Sister chromatids line up at the metaphase plate.
	Anaphase II	Sister chromatids separate	Sister chromatids are pulled apart by the shortening of the kinetochore microtubules. Non-kinetochore microtubules lengthen the cell.
	Telophase II and Cytokinesis	Haploid daughter cells	Chromosomes arrive at the poles of the cell and decondense. Nuclear envelopes surround the four nuclei. Cleavage furrows divide the two cells into four haploid cells.

Mendelian Genetics: genes, alleles, gametes, locus, asexual reproduction, clone, sexual reproduction, life cycle, somatic cells, karyotype, homologous chromsomes, sex chromosomes, autosomes (chromosomes that aren't X or Y), fertilization, zygote, diploid, heterozygoys, genotype, phenotype, testcross, monohybrid cross, dihybrid cross, complete dominance, codominance, incomplete dominance (pink flowers), pedigree. Parental generation (both true bred), F1 generation (both mixed), then F2 (mixed)

Recessively inherited disorder: cystic fibrosis (mutation in an allele that codes for cell membrane rotein for transport Cl ions), tay-sachs disease (allele codes for dysfunctional enzyme that is unable to break down certain lipids in brain), sickle cell (allele code for mutated hemoglobin protein)

Lethal dominant alleles (require only one copy of allele in order for disorder to express (Huntington's disease

Multiple alleles: gene has more than two alleles like blood types

Pleiotropy: one gene has multiple phenotyphic effects

Epistasis: one gene at one locus affects another gene at another locus

Polygenic inheritance: two or more genes add to one phenotype

Crossing over: prophase 1

Independent assortment of chromosomes: metaphase I, homologous chromsomes pair up randomly on the metaphase plate

Random fertilization:

Mendelian laws:

- Alternative versions of genes account for variations in inherited characteristics among offspring, b/c alleles
- For each character every organism inherits one allele from each parent
- Dominant and recessive alleles
- Law of Segregation: alleles separate when gamete form
- Law of independent assortment: anaphase I, alleles will segregate independently during gamete formation

Phenotype (Blood type)	Genotype
Туре А	I ^A I ^A or I ^A i
Туре В	I ^B I ^B or I ^B i
Type AB	$\mathbf{I}^{A}\mathbf{I}^{B}$
Type O	11

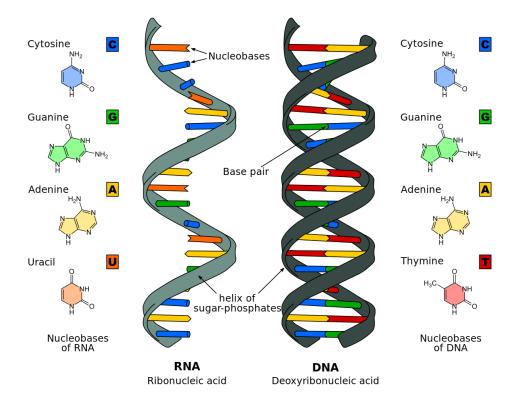
Chromosomal Inheritance: genes have specific location on chromosome. And this affects inheritance

- Sex linked gene, x-inactivation, methylation, barr body, linked genes, genetic recombination, parentaly types and recombinants, crossing over, linkage map, map unit=1% recombination, nondisjunction (meiosis I and II), aneuploidy (faulty number of chromosomes), trisomic, monosomic, polyploidy (more than normal sets of chromosomes like 3n instead of 2n),
- Aneuploidy is more dangerous.
 - \circ Kinds of chromosomal mutations:
 - Deletion: part of the chromosome is missing
 - Duplication: part of the chromosome is repeated
 - Inversion: chromosome fragment breaks off and rejoins but backwards
 - Translocation: deleted chromosome fragment joins to a nonhomologous chromosome
- The closer two genes are, the less likely to recombine
- Genomic imprinting: during gamete formation, and result in the silencing of particular alleles of certain genes
- Disorders:
 - Duchenne muscular dystrophy (x-linked disorder; gradual weakening of muscles, rarely live past 20)
 - Hemophelia (x-linked; blood that can't clot b/c the protein for blood clotting isn't there)
 - Down syndrome (aneuploidy resulting in extra trisomy chromosome 21)
 - Klinefelter (aneuploidy of male possessing extra X (XXY))
 - Turner syndrome (monosomic with female just XO. Female just sterile and taller than normal)
- Most organisms, as long as have 1 Y, its male
- Most sex-linked disorders are x-linked. Most y-linked have only to do with male organ developents, etc.

Environment effect on phenotype: phenotypic plasticity (when individuals with same genotype express different phenotype b/c of different environments, like flowers and soil pH, seasonal fur color, sex determination in snakes.

Unit 6:

Griffith, Hershey and Chase, Watson and Crick, X-ray crystallography, double helix,



replication (semiconservative): origins of replication, replication bubble, DNA polymerase, leading strand (5'3'), lagging strand (3'5'), Okazaki fragments, DNA ligase, mismatch repair, nucleotide exision repare, nucleases, telomeres

- DNA+protein= chromatin
 - o Euchromatin: extended and lose form, for transcription
 - Heterochromatin: more condensed chromatin, not able to transcribe
- Gene expression, transcription, mRNA, RNA processing, translation, template strands, codons
- One gene-one polypeptide hypothesis, each gene code for a polypeptide, a protein
- Template strand, complementary for the original DNA strand, triples=codons (written in 5'3' direction)
- Genetic code is redundant, more than 1 codon can code for an amino acid
- Promoter, terminator, transcription unit (the stretch of DNA that is transcribed into an RNA molecule)
- Initiation, transcription factors (bind to RNA polymerase to promoter and initiate transcription)
 - RNA polymerase II+transcription factors= (transription initiation complex)
 - o Elongation : RNA polymerase moves along, unwinding and adds RNA base
 - Termination: transcribes termination
- 5'cap and polyA tail added to RNA, and export mRNA from nucleus protect mRNA from degradation)
- RNA splicing, introns (spliced out), exons (glued together by spliceosome), small nuclear RNA (snRNA; catalyzes the excision of introns, etc.), ribozyme (RNA serving catalytic role), tRNA, anticodon, rRNA, P-A-E sites,
- Point mutation (alterations of just one base of a gene); nucleotide-pair substitution (replacement of one nucleotide and its partner with another pair of nucleotides

- Missense (substitution that enables the codon to still code for an amino acid); nonsense (substitution causes regular amino acid codon into a stop codon)
- Insertion and deletion cause frameshift mutations. Mutagens; interact with DNA in ways that cause mutations
- Operons: operator (controls access of RNA polymerase to the gene), promoter (where the RNA polymerase attaches), genes of the operon (the entire stretch of DNA required for all the enzymes produced by the operon)
- Regulatory genes (produce repressor proteins that may bind to the operator site
- Kinds of operons; repressible operon (normally on but can be inhibited; anabolic, building an essential organic molecule); if the organic molecule being produced by the operon is provided to the cell, act as a corepressor, bind to repressor protein and activate it.
 - Inducible operon; normally off but can be activated on, requires inducer to bind and inactivate the repressor protein (ex. Lac operon)
- Differential gene expression: the expression of different genes by cells with the same genome
- DNA methylation (add methyl group to DNA and make more tightly packaged, thus reduce gene expression)
- Histone acetylation (acetyl group added to amino acid, and thus make chromatin less tightly packed, encourage transcription)
 - Epigenetic inheritance, transcription initiation, enhancer regions, activators
 - \circ $\,$ Enhancer regions, DNA far from gene, bind to promoter regions by proteins "activators" $\,$
 - microRNA, small interfering RNA; bind to mRNA and degrade mRNA to prevent gene expression
- Zygote undergoing transformation: cell division, cell differentiation, morphogenesis (gives and organism its shape)

Stuff:

- Cytoplasmic determinants (maternal substances in the egg that influecnt the course of early development)
- Cell-cell signals, like growth factors, which influence neighboring cells "induction"
- Determination, is the series of events that lead to observable differences
- Pattern formation, sets uo body plan as a result of cytoplasmic determinants, inductive signals
- Homeotuc genes- master control genes that control pattern formation

Cancer, oncogenes (genes cause cancer); proto-oncogenes (code normal cell growth)

Tumor-suppresor genes; p53 genes-which activate p21 gene halts cell cycle by binding to cyclein kinases; apoptosis

Mutations: changes in genotype can mean changes in phenotype b/c of changes in gene expression

Biotechnology: genetic engineering, biotechnology, recombinant DNA, gene cloning, restriction enzymes, restriction sites, restriction fragments, sticky ends, DNA ligase, cloning vectors, nucleic acid hybridization, probe, PCR, gel electrophoresis, restriction fragment length polymorphisms

1. Gene expression- the physical expression/appearance/effect that a gene plays on the larger organism.

Gene Expression Steps	Molecules Involved: What molecules and proteins are involved in this step?	Summary: What happens during this step?
Transcription	RNA Polymerase, DNA, RNA nucleotides	RNA Polymerase enzyme transcribes DNA into RNA using RNA nucleotides. Base pairings go G-C and A-U. RNA transcription occurs in the 5'3' direction.
RNA Splicing	Spliceosome, intron/exon, mRNA	Pre-mRNA contains segments of noncoding genes (introns). Using chemical markers, spliceosomes remove intron segments and stick exons back together.
mRNA Transport	Cytoplasm, mRNA, nucleus	The edited mRNA is transported out of the nucleus into the cytoplasm through nuclear pores. These mRNA molecules will be used to create polypeptides.
Translation	Ribosome, rRNA, mRNA, amino acids, tRNA	Using the mRNA as a guide, tRNA amino acids to form polypeptides using anticodons. Each amino acid is coded for by multiple tri-codon combinations. The mRNA is read through by the ribosome, which is composed of rRNA sequences with proteins.
Protein Processing	Cytoplasm, endoplasmic reticulum, golgi system	Completed polypeptide chain folds into 3-D functional protein. This occurs in either the cytoplasm or endoplasmic reticulum. Proteins often finish folding in the golgi system.

3.

Genetic Medicine	Short Summary	Detailed Description
CRISPR- Cas9	CRISPR-Cas9 utilizes genetic markers to cut out DNA segments.	CRISPR-Cas9 is most often used to knock out a gene so that it is not expressed, a s well as edit/correct disease-causing mutations. The technology is currently being used on sickle cell disease and cystic fibrosis.
Gene Therapy	Gene therapy inserts properly-functioning segments of DNA or genes to repair disease causing ones.	Gene therapy utilizes a variety methods of inserting genes, most commonly using viral vectors. By mimicking the cellular receptors and markers found on cell-infecting viruses, gene therapy is able to insert therapeutic genes into the cell's genome.
Gene Switches	Gene switches utilizes the already present switch	Gene switches turn on and off genes by placing or taking out certain targeted regulatory

	sequences to turn off and on certain gene expressions.	proteins. Gene switches are currently being experimented on sickle cell disease.
Exon Skipping	Exon skipping changes how pre-mRNA is cut and which gene sequences get cut out as introns and which are kept as exons.	Exon skipping changes how the primary RNA transcript of a gene with a disease-causing mutation is spliced, removing the mutation from the final mRNA molecule. Currently being tested on Duchenne muscle dystrophy.
RNA interference	RNA interference involves small RNA segments that target various mRNAs for destruction, reducing the expression of certain genes.	Scientists synthesize short RNA segments with a sequence complementary to that of a target sequence. This double strand is then cut into smaller pieces, and incorporated into silencing complexes, which cleave the mRNA, preventing expression. Huntington's disease.
Small Molecule Drug	Utilizes drugs of low molecular weight, which interact with disease- causing proteins.	Block protein translation and transcription pathways. Used in cystic fibrosis treatments.

4.

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Name of Disease	What are the key characteristics of the disease and whom does it affect? How can the featured technologies be utilized as a cure.		
Leber Congenital Amaurosis	 -eye disorder affecting the retina. Mostly affects young infants. Sluggish pupillary responses, severe acute vision and photophobia. - caused by mutation in the CRX or IMPDH1 genes. Autosomal dominant pattern of inheritance - possible cure in gene therapy 		
Sickle Cell Disease	 Disorder caused by protein misfolds in the red blood cell hemoglobin Mutation in gene, inherited. Possible cure in stem cell transplant and gene switches 		
Duchenne Muscular Dystrophy	 Progressive form of muscle dystrophy Genetic mutation resulting in protein misfold of dystrophin Possible cure in exon skipping of 51 and RNA splicing 		
Huntington's Disease	 Progressive breakdown of nerve cells in brain Caused by inherited defect in autosomal gene Possible cure in RNA interference 		
Cystic Fibrosis	 Hereditary disease that creates mucus that clogs lungs and obstructs the pancreas Defect in the CFTR gene that makes the protein responsible for the movement of salt and water in and out of body cell's 		

•	Possible cure in small molecule drug
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 The step we would want to target would be the transcription process, as it is the gene expression step that occurs most upstream of all other affected processes. Therefore, the best treatment would be gene therapy. Gene therapy utilizes functional genes which are inserted into the patient's genome. Another alternative treatment would be CRISPR-Cas9. However, CRISPR would just cut out the mutated genes, without replacing the necessary genes for protein expression.