Name

Date

Vector Types

	RETROVIRUS 	ADENOVIRUS 	ADENO-ASSOCIATED VIRUS 	HERPES SIMPLEX VIRUS 	LIPOSOME 	NAKED DNA 
How the vector carries genetic material	In the form of RNA rather than DNA. The most famous is Human Immunodeficiency Virus (HIV), which causes AIDS.	Double-stranded DNA . The common cold virus.	Single-stranded DNA	Single-stranded DNA	Plasmid DNA packaged into miniature lipid –based pockets that can fuse to the cell's own membranes, The DNA is released and transported into the nucleus	Plasmid DNA molecule all by itself that can be taken up by some cells, and transported into the nucleus
Maximum length of DNA that can be inserted into vector	8, 000 base pairs	7,500 base pairs	5,000 base pairs	20,000 base pairs		
ADVANTAGES	+ Infects only dividing cells	+ Infects both dividing and non-dividing cells very effectively + Possible to target specific cell types by engineering proteins on the virus surface to recognize special proteins on the target cell's surface	+ Does not cause illness in humans + Infects a wide range of dividing and non-dividing cell types very effectively + Need the assistance of a "helper" virus to replicate themselves inside cells + Possible to target specific cell types by engineering proteins on the virus surface to recognize special proteins on the target cell's surface + Integrates into the host cell's genome; 95% of the time, it will integrate into a specific region on Chromosome 19, greatly reducing the chance that integration will disrupt the function of other genes in the cell + Typically will not cause an immune response	+ Infects cells of the nervous system + Will not integrate into the host cell's genome, but is a circular piece of DNA that replicates when the cell divides; will stay in the cell's nucleus for a long time + Will not disrupt the function of other genes in the host cell	+ Will not generate an immune response + Better suited for *ex vivo* gene therapy approaches	+ Will not generate an immune response + Generally not toxic
DISADVANTAGES	- Integrates into the host cell's genome in random locations; might integrate into a place where it disrupts another gene - Can cause an immune response	- Will not integrate into the host cell's genome; the cell will discard the virus and gene activation will be lost - Can cause an immune response	- 5% chance of integrating and disrupting the function of other genes in the cell	- Can cause an immune response in the patient	- Not specific for any cell type - Enter, cells far less effectively than viruses - Will not integrate into the host cell's genome	- Not specific for any cell type - Enter, cells far less effectively than viruses - Will not integrate into the host cell's genome - Unstable in most body tissues

vector selector

You are a researcher at Advanced Bio Delivery Systems (ABDS), a company that supplies vectors for use in gene therapy. The marketing department has come to you for assistance. They would like to sell the vectors made by ABDS to biotech companies pursuing specific gene therapies. It is your job to research the disorders these biotech companies are targeting and decide on a vector that has potential to work. You will be presenting your findings to the marketing department for approval. There may be more than one vector possibility, so your job is to choose what you feel is the best one and defend your position. Your company stands to make a considerable profit if you are able to "sell" their vector for use in research.

Some things to consider:

- What are important characteristics of the disease being investigated (genetic defect, body system affected…)?
- Will the gene fit into the vector?
- What types of cells would the therapy need to target (dividing/non-dividing)?
- What will happen to the genetic material once it is inserted into the cells?
- Does gene expression need to last a long time?
- What kinds of safety issues need to be considered before recommending a vector type?

Situation I

The ABDS marketing department has identified NFS Co. as a biotech company that is developing gene therapies. NFS Co. is focusing on neurofibromatosis type 1 which is caused by a mutation in the *NF1* gene (8,454 base pairs) on chromosome 17. This disorder causes tumors to grow on nerves, skin changes and bone deformities. NFS Co. is looking for a vector that could target non-dividing nerve cells and insert a transgene stably into the chromosome.

Prepare a sales pitch to suggest a vector.

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Situation 2

The ABDS marketing department has identified CellMart as a biotech company that is developing gene therapies. CellMart is searching for a vector to treat hemophilia, a bleeding disorder caused by a deficiency in one of the blood clotting factors. This results in excessive bleeding. CellMart intends to isolate blood stem cells, which divide frequently, and introduce the vector *ex vivo*. The gene they plan to use is 7,350 base pairs long.

Prepare a sales pitch to suggest a vector.

vector selector

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Situation 3

The ABDS marketing department has identified Oma, Inc. as a biotech company that is developing gene therapies. Oma Inc. would like to target non-small cell lung cancer (75% of all lung cancers) with gene therapy. They need a vector to introduce *p53*, a tumor suppressor gene (2,150 base pairs long), directly into the tumor. The vector must target the lung cells directly and the potential immune response must be considered.

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vector selector

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Situation 4

The ABDS marketing department has identified SCID-BE-GONE (SBG), Inc. as a biotech company that is developing gene therapies. SBG is interested in developing a delivery system that will insert a good *ADA* gene (1,498 base pairs long) in T-cells of patients who have SCID (severe combined immune deficiency). SBG plans to harvest T-cells from a patient and use an *ex-vivo* technique to insert a functional *ADA* gene. As the transfected cells begin to produce the ADA protein, they will be retuned to the patient. The company is having trouble finding a vector to deliver their gene.

Prepare a sales pitch to suggest a vector.

vector selector

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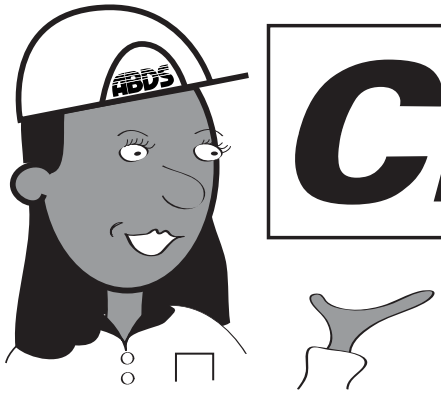
Situation 5

The ABDS marketing department has identified CF, Inc. (CFI) as a biotech company that is developing gene therapies. CFI is interested in developing a delivery system that will insert a good *CFTR* gene in the lung cells of patients who have cystic fibrosis. The *CFTR* gene (4,443 base pairs long) codes for the protein that controls ion transfer across cell membranes. CFI is developing an *in vivo* system to deliver the functional gene copy. Challenges that face CFI are getting the vector to the lung cells and dealing with the immune response mounted as the vector enters the lungs.



Prepare a sales pitch to suggest a vector.

Vector Selector: Assessment Rubric

CATEGORY	4	3	2	1
Amount of information on disease	Important characteristics of the disease are addressed and described in detail.	Most of the important characteristics of the disease are addressed and described.	Important characteristics of the disease are addressed and described marginally.	Only one characteristic of the disease is addressed.
Amount of information on vector	Important characteristics of the vector are addressed and described in detail.	Most of the important characteristics of the vector are addressed and described.	Important characteristics of the vector are addressed and described marginally.	Only one characteristic of the vector is addressed.
Targeting issues	Vector chosen will bind to the target cells. Description of targeting strategy is detailed.	Vector chosen will bind to the target cells. Description of targeting strategy is somewhat vague.	Vector chosen will bind to the target cells. Description of targeting strategy is incomplete.	Vector chosen will not bind to the target cells.
Integration of genetic material	Includes detailed description of what will happen to the DNA.	Description of what will happen to the DNA is correct, but described in less detail	Description of what will happen to the DNA is lacking some information.	Description of what will happen to the DNA is unclear or incorrect.
Vector Choice	Provides ideas that support choice of vector.	Provides ideas that support choice of vector.	Provides one idea that supports choice of vector.	No ideas presented that support choice of vector.

Name ______________________ Date __________

Vector Types

	RETROVIRUS	ADENOVIRUS	ADENO-ASSOCIATED VIRUS	HERPES SIMPLEX VIRUS	LIPOSOME	NAKED DNA
How the vector carries genetic material						
Maximum length of DNA that can be inserted into vector						
ADVANTAGES						
DISADVANTAGES						