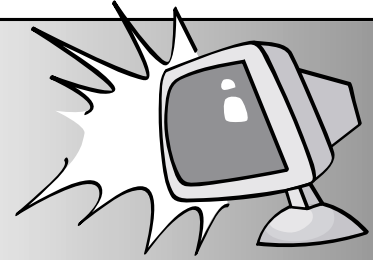




# Exploring Gene Therapy



**Log on to: <http://gslc.genetics.utah.edu/units/genethrapy> and explore this module to find the answers to the questions below.**

*Hint: the Search feature on this website may or may not help you find what you are looking for; it is best to go through the module to find the answers.*

**Questions:**

1. What is gene therapy?
2. What are the five criteria for a gene therapy candidate disease?
3. Case Study: Is cystic fibrosis a good candidate for gene therapy? Why or why not?
4. What are the hallmarks of successful gene delivery in gene therapy trials?
5. Explain the *in vivo* technique of delivering genes to a patient's cells.
6. Explain the *ex vivo* technique of delivering genes to a patient's cells.

7. What are vectors? What are the six different types of vectors contained in the Vector Toolbox?

*A vector is:*

*The six different types of vectors are:*

8. Go through the Vector Toolbox. Fill out the attached Vector Types Table.

9. Which vector would be the best choice for a cystic fibrosis gene therapy? Why?

10. Become the Space Doctor and design a gene therapy for one of the three given patients. *Fill out the following:*

Patient \_\_\_\_\_ Disease \_\_\_\_\_

Tissue targeted \_\_\_\_\_

Vector chosen (*consult the toolbox if you need a refresher*) \_\_\_\_\_

Was your therapy *in vivo* or *ex vivo*? \_\_\_\_\_

Was your therapy successful? \_\_\_\_\_

Why or why not? \_\_\_\_\_

List all of the vectors that worked for this gene therapy

\_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_

11. What are the challenges of gene therapy? *List and briefly describe each one:*

a. \_\_\_\_\_

\_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_

b. \_\_\_\_\_







\_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_

c. \_\_\_\_\_

\_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_

12. What are some issues involved in gene therapy?

# 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						