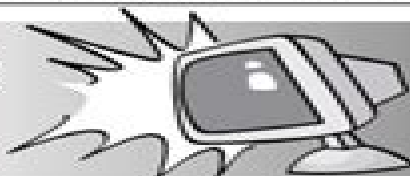




# Exploring Gene Therapy - Answer Key



Log on to: <http://gslc.genetics.utah.edu/units/genetherapy> 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?

*Adding a normally functioning copy of a mutated gene to help affected cells, tissues and organs work properly.*

2. What are the five criteria for a gene therapy candidate disease?

- 1. The condition must result from mutations in one or more genes.*
- 2. You must know which gene is involved and have an available DNA copy of that gene.*
- 3. You must know the biology of the disorder - which tissue is affected, the role of the protein encoded by the gene, and how mutations in the gene affect the protein product.*
- 4. Adding a normal copy of the gene must fix the problem.*
- 5. It must be possible to deliver the gene to cells of the affected tissue.*

3. Case Study: Is cystic fibrosis a good candidate for gene therapy? Why or why not?

*Yes, cystic fibrosis meets all of the criteria above.*

4. What are the hallmarks of successful gene delivery in gene therapy trials?

*Targeting the right cells, activating the gene, integrating the gene into the cells, avoiding harmful side effects.*

5. Explain the *in vivo* technique of delivering genes to a patient's cells.

*The vector carrying the gene is injected directly into the body. The *in vivo* approach happens inside the body.*

6. Explain the *ex vivo* technique of delivering genes to a patient's cells.

*Cells from the affected tissue are isolated and cultured in a Petri dish. The vector carrying the gene is introduced to the cells in the Petri dish. The *ex vivo* approach happens outside the body.*

# Part 1 Exploring Gene Therapy Answers

**A Loxley**



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