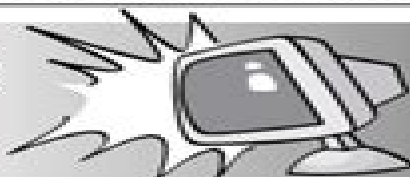




Exploring Gene Therapy - Answer Key



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

Islam M. Ghazi, Michael J. Cawley



Part 1 Exploring Gene Therapy Answers:

Embracing the Track of Phrase: An Emotional Symphony within **Part 1 Exploring Gene Therapy Answers**

In a world eaten by monitors and the ceaseless chatter of instant connection, the melodic elegance and mental symphony created by the written word usually fade in to the back ground, eclipsed by the relentless noise and interruptions that permeate our lives. However, situated within the pages of **Part 1 Exploring Gene Therapy Answers** an enchanting literary prize full of organic emotions, lies an immersive symphony waiting to be embraced. Crafted by a masterful composer of language, that fascinating masterpiece conducts visitors on a psychological trip, well unraveling the hidden songs and profound influence resonating within each carefully constructed phrase. Within the depths of this touching assessment, we can investigate the book is central harmonies, analyze its enthralling publishing style, and surrender ourselves to the profound resonance that echoes in the depths of readers souls.

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