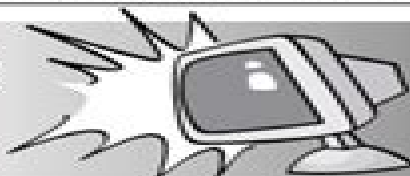




Exploring Gene Therapy - Answer Key



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 Exploring Gene Therapy Answers

Shasha Hu



Part Exploring Gene Therapy Answers:

The Ethics of Human Gene Therapy LeRoy Walters, Julie Gage Palmer, 1997 The authors of this absorbing new book describe the science of gene therapy in terms easily accessible to the non specialist and focus on the controversial ethical and public policy issues surrounding human interventions in human heredity After a brief survey of the structure and functions of DNA genes and cells Walters and Palmer discuss three major types of potential genetic intervention somatic cell gene therapy germ line gene therapy and genetic enhancements They start with the current techniques of gene addition using non reproductive somatic cells in an effort to cure or treat disease Next they address the technical problems and moral issues facing attempts to prevent disease through genetically modifying early human embryos or sperm and egg cells These changes would be passed on to future generations Chapter 4 in many ways the most original part of this volume confronts the issue of employing genetic means to improve human abilities and appearance Depending on the techniques employed such enhancements could affect not only the individuals receiving the intervention but their offspring as well Three types of genetic enhancements are considered physical alterations to improve size reduce the need for sleep and decelerate aging intellectual enhancements of memory and general cognitive ability and moral enhancements for control of violently aggressive behavior The authors maintain that genetic modifications should be evaluated individually rather than be condemned in principle or as a group The final chapter summarizes the public review process that human gene therapy proposals have been undergoing in the United States since 1990 Five appendices providing technical background information along with a complete list of questions raised in the national public review process supplement the discussion

Human Gene Therapy Eve K. Nichols, 1988 *Altered Fates, the Promise of Gene Therapy*, 1986 Recombinant DNA Research, 1984 **BMJ**, 2002 *Human Gene Therapy*, 1997 *Introduction to Reference Sources in the Health Sciences* Jeffrey T. Huber, Jo Anne Boorkman, Jean Blackwell, 2008 Lists several print resources and helps librarians to meet customers changing expectations for electronic versions of traditionally print reference sources reliable electronic only resources and resources that they can access from their home computers through freely available Web sites or through library licenses

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