

NAME _____

DATE _____

This handout supplements the short film [Genes as Medicine](#).

1. True/False. Soon after a gene associated with childhood blindness was discovered in the 1990s, Drs. Bennett and Maguire were able to offer a gene therapy treatment for children. _____
2. Explain the reasoning or evidence you used to answer question 1.
3. In the film you met Molly, who has a form of childhood blindness called Leber amaurosis (also called Leber congenital amaurosis, or LCA). Her blindness is caused by _____
 - a. early exposure to intensely bright light.
 - b. an eye injury she received at a young age.
 - c. mutations in a gene that is necessary for maintaining sight.
 - d. a viral disease in her nervous system that moved into her eyes.
4. For many genetic diseases, children inherit the disease-causing mutations from their parents. What was the likely inheritance pattern in the case of Molly's form of childhood blindness? _____
 - a. Only females can have Leber amaurosis, so Molly must have inherited the genetic mutation only from her mother.
 - b. Molly became blind from new mutations that occurred in her eye as a baby.
 - c. Molly ended up with childhood blindness because both of her parents were blind when they were children.
 - d. Molly inherited a mutation in the same gene from each of her parents.
5. Which statements are reasons that eyes are a good target for clinical trials related to genetic medicine? _____
 - I. Eyes are easy to access.
 - II. Eyes are the least important of the five senses.
 - III. Eyes in humans are identical to eyes of other species used in animal models.
 - IV. One eye can be treated while the other can act as an experimental control.
 - a. I and II only
 - b. II and III only
 - c. III and IV only
 - d. I and IV only

6. The gene therapy technique designed by Drs. Bennett and Maguire to cure Molly's blindness involved using a virus as a vector to deliver the corrective gene into one of her eyes. What necessary step must be taken before viruses are used in this way? _____
 - a. Patients must first take flu medicine to help them avoid any flulike symptoms that the virus causes.
 - b. The virus's harmful genes and the ones the virus needs to replicate must be removed.
 - c. Patients like Molly must first receive a vaccine for the virus so that they don't get the disease that the virus carries.
 - d. Doctors must first snip out the defective gene from a patient's photoreceptor cells before using the virus to deliver the corrective gene.

7. Explain unique characteristics of viruses that make them useful tools for gene therapy.

8. As a final test of their technique to cure Leber amaurosis, Drs. Bennett and Maguire needed a large animal model that was similar to humans.
 - a. What animal did they choose?

 - b. List at least three reasons that this animal was a good model for testing a cure for childhood blindness.

9. Mutations to the *RPE65* gene can cause Leber amaurosis. Why is it a mistake to call *RPE65* "the Leber amaurosis gene"?

10. Sketch and annotate a diagram that shows how Molly ended up inheriting non-functional alleles for the gene associated with Leber amaurosis (Molly's form of childhood blindness).