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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 usin 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 <i>RPE65</i> gene can cause Leber amaurosis. Why is it a mistake to call <i>RPE65</i> "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).	

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