

December 9, 2017

Gene Therapy Fixes Rare Skin Disease

Article-Based Observation: Q

Directions: Read the article "[Gene therapy fixes rare skin disease](#)" and then answer these questions:

1. Summarize the article by making brief statements defining "who, what, where, when and why."

2. What causes the genetic skin disease this boy has? Be specific.

3. Why are children with this disease sometimes referred to as "butterfly" children? What is the frequency and prognosis for patients with this disease in the United States?

4. What treatment did this boy receive prior to the stem cell and gene therapies, and did the treatment work?

5. How was new skin for the boy prepared in a lab?

6. How well developed was the gene therapy technique before it was tried on this boy? What was the outcome for the boy?

7. What are two competing theories for how skin cells are naturally replenished?

8. How were the boy's repaired cells monitored?

9. What do the results from tests of the boy's skin indicate about the two competing theories for how skin cells are naturally replenished?

10. What other questions do you still have after reading the article?