Sometimes its all in the Genes

Name\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_ Per\_\_\_\_

Part 1

1. Discuss why Nancy might or might not want to know the results of her blood test for CF.

2. Dr. Kwin told Nancy that she has "absolutely nothing to worry about." Although Nancy cannot get CF, is Dr. Kwin's statement entirely correct?

3. Did Dr. Kwin provide Nancy with enough information about cystic fibrosis and the test to make a good decision?

4. Should Nancy consent to the test? Provide the reasoning for your answer.

Part 2: Help Nancy finish answering Jake's questions.

1. The normal or good copy of the CF gene can be written shorthand as "F", and the mutant or bad copy of the CF gene can be represented as "f". Using this shorthand style, write out Nancy's genetic make-up for this gene.
2. What is the chance that Nancy passed on the CF allele to her baby?
3. What is the chance that Jake passed on the allele if he is a carrier?
4. What is the chance that their baby will have CF if they are both carriers?
5. If their first child has CF, what is the chance that their second child will have CF?
6. What is the chance that the baby will inherit CF if only Nancy is a carrier?

**Part 3**

1. How do mutant CFTR genes lead to thicker mucus in cystic fibrosis patients?
2. How would testing their unborn baby for CF help Nancy and Jake? Their baby?
3. What are their options if they find out their baby does have two bad CFTR genes?
4. Should they have the amniocentesis procedure? Provide your reasons for reaching this decision.

**Part 4**

1. The current therapies available to treat CF only treat the symptoms of the disorder. However, if gene therapy were to work, it could be considered a cure rather than a mere treatment of the symptoms. Explain why this could be the case.

2. "The successful use of gene therapy to cure SCID syndrome (2000) is hoped to be a permanent cure for those patients because a good copy of the problem gene was inserted into the patients' blood stem cells in the bone marrow . Once white blood cells enter the blood stream they have a limited life span, on the order of a few week to months. The blood stem cells are the cells that create more white blood cells to replace those that are lost. If the gene was only inserted into the circulating mature white blood cells, the patient would only be temporarily cured until those cells were used up or died." The current gene therapy approaches to cure CF involve inserting a functional CFTR gene into the mature epithelial cells of the lungs. In light of the preceding paragraph, do you think that this approach would be a "permanent" cure for CF? Explain your answer.

3. What level of risk should be acceptable for a patient involved in a clinical trial? In other words,

under what circumstances should Nancy feel comfortable enrolling Joshua in a gene therapy clinical trial?

4. In the current clinical trials for gene therapy treatments of CF, participants must be over 12, so Joshua could not be helped by the trials that are currently being run at this time. Why might there be an age restriction such as this? Is an age restriction such as this fair?

5. Should Joshua be enrolled in a clinical trial on cystic fibrosis gene therapy?