



UNIT FIVE -THALASSEMIA QUESTION

TEACHER GUIDE

You are a doctor who wants to treat a patient for thalassemia. Thalassemia is a genetic disease caused by a mutation in hemoglobin that disrupts the molecule's ability to carry and deliver oxygen in the body. You want to try to treat your patient with iPS cells. Describe how you would obtain or produce the iPS cells. Include the original cell type and transfection method and why you have chosen these methods. Also describe how the iPS cells would be treated before transplantation.

Answer-

The student may either choose to make patient specific iPS cells or acquire cells from an iPS bank.

There are multiple possible answers to the question on origin of cell type.

Some possible answers to cell type

1. Skin cells as these are easy to acquire
2. An adult stem cell type, because it may be easier to make into iPS cells
3. Stomach or liver cells because these are less likely to turn cancerous

For transfection technique a student should ideally say without using c-Myc and using a method that won't integrate viral DNA into the host DNA such as plasmids, adenovirus, or small molecules. In reality using all four factors with a retrovirus would be unacceptable because the risk of developing cancer would be too great. However, as these methods are more efficient than the "safer" methods. There is no specific right answer to this question as no method for iPS delivers the exact desired results. That is a point of this exercise. Right now there isn't a good enough way to do what the question is asking.

If the student decides to use patient specific iPS cells they must use homologous recombination to replace the mutant gene with a normal gene.

Both patient specific iPS cells and iPS cells from a bank would need to be differentiated into hematopoietic stem cells before they are transplanted into the patient.