Continuing Medical Education examination

Successes and risks of gene therapy in primary immunodeficiencies

Instructions for category 1 Continuing Medical Education credit

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Learning objectives: "Successes and risks of gene therapy in primary immunodeficiencies"

1. To discuss the actual and theoretical advantages of gene therapy in primary immunodeficiencies.
2. To review the progress of gene therapy as a therapeutic option for primary immunodeficiencies.
3. To understand the risk of insertional mutagenesis and leukemia in patients receiving hematopoeitic stem cells transduced with a retroviral vector.

CME items

Question 1. Gene therapy has been shown to provide immune restoration in patients with which of the following?

A. hyper-IgM syndrome
B. Wiskott-Aldrich syndrome
C. XSCID
D. X-linked agammaglobulinemia

Question 2. Improved immunoreconstitution was observed in ADA patients enrolled in a recent gene therapy trial. Differences from previous approaches include which of the following?

A. use of cyto-reductive regimen
B. continued use of PEG-ADA
C. use of an adenoviral vector
D. close clinical follow-up

Question 3. XSCID is a good candidate disease for gene therapy for which of the following reasons?

A. The disease gene is only expressed in T cells.
B. The target hematopoietic stem cells can be transduced ex vivo.
C. Immune reaction against transduced cells is very likely to occur.
D. Overexpression of the γ-c chain protein is not possible.

Question 4. Which of the following statements is correct?

A. Insertional mutagenesis always results in malignancy.
B. Insertional mutagenesis may occur when retroviral vectors are used.
C. Insertional mutagenesis has not caused cancer in clinical trials.
D. Insertional mutagenesis locations in the genome are fully predictable.