Cost-effectiveness analysis is not only a way to discipline the use of new technologies, but is also a way to acquire new medical knowledge. Epoetin for cancer anemia provides an example of both these uses.

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References

Gene therapy for X-linked chronic granulomatous disease

Patients with X-linked chronic granulomatous disease (CGD) associated with a defect in Gp91-phox suffer from a severe disease and exhibit the greatest morbidity and mortality of all CGD patients. In particular, they frequently undergo fungal and bacterial infections, which are mainly localized in subcutaneous tissues and are resistant to treatment. Stem cell transplantation is the only curative therapy available so far.

Gene transfer of Gp91-phox into hematopoietic stem cells and subsequent expression of the gene in mature phagocytes may be an attractive therapeutic alternative. However so far, long term studies have yielded disappointing results, mainly because of low transduction efficiencies.

In this issue, Bellantuono et al.1 examine the possible reasons for low titer amphotropic viral production associated with gene transfer of Gp91-phox into hematopoietic stem cells. These basic studies are crucial for development of therapeutic procedures. A number of studies on gene transfer into hematopoietic stem cells have recently appeared in this journal.2-5

References