TABLE 91e-4 TAKING HISTORY FROM SUBJECTS ENROLLED IN GENE TRANSFER STUDIES

Elements of History for Subjects Enrolled in Gene Transfer Trials
1. What vector was administered? Is it predominantly integrating (retroviral, lentiviral, herpesvirus [latency and reactivation]) or nonintegrating (plasmid, adenoviral, adeno-associated viral)?
2. What was the route of administration of the vector?
3. What was the target tissue?
4. What gene was transferred in? A disease-related gene? A marker?
5. Were there any adverse events noted after gene transfer?
Screening Questions for Long-Term Follow-Up in Gene Transfer Subjects[a]
1. Has a new malignancy been diagnosed?
2. Has a new neurologic/ophthalmologic disorder, or exacerbation of a preexisting disorder, been diagnosed?
3. Has a new autoimmune or rheumatologic disorder been diagnosed?
4. Has a new hematologic disorder been diagnosed?

[a]Factors influencing long-term risk include: integration of the vector into the genome, vector persistence without integration, and transgene-specific effects.

therapeutic modality in the twenty-first century. A central question to be addressed is the long-term safety of gene transfer, and regulatory agencies have mandated a 15-year follow-up for subjects enrolled in gene therapy trials (Table 91e-4). Realization of the therapeutic benefits of modern molecular medicine will depend on continued progress in gene transfer technology.