Gene Transfer Clinical Trials Monitoring

The monitoring studies performed in the X-SCID gene transfer clinical gene transfer trials can inform proposed monitoring plans for other gene transfer clinical trials.

- **Is it possible to obtain any useful information on sites of integration prior to infusion of *ex* vivo gene-modified cells?**

  - **If analyses are not performed either prior to or at the time of infusion, should cells be archived for future analyses? (i.e.- what useful information can be learned from analyses of archived samples?)**

Gene Transfer Clinical Trials Monitoring

- **How do the results of monitoring and analyses in this subject inform the formulation of monitoring plans in other trials?**
  - **What role does the nature of the disease have in the frequency and duration of monitoring?**
  - **What role does the phase and type of gene transfer clinical trial have in the frequency and duration of monitoring?**
  - **What role does the age of the subject have in the monitoring plan?**
  - **What role does the gene transfer procedure have in the monitoring plan?**
  - **What role do the above have in what samples should be collected and analyzed?**

Gene Transfer Clinical Trials Monitoring

- **If during the course of monitoring a subject a monoclonal expansion is demonstrated, how will this inform the future course of action for that research subject?**

  - **What role, if any, does the marker chosen to define the monoclonal expansion have in determining the future course of action for that research subject?**

    - **Cell surface marker defined monoclonal expansion (ex. Ig, TCR)**

    - **Molecular/genomic defined monoclonal expansion (ex. single insertion site in the monoclonal population)**