GENE THERAPY DISCUSSION QUESTIONS

- **Neutralizing antibodies (NAbs)**
  - Methodologies and parameters for inclusion/exclusion
  - Options/possibilities if a patient screen fails due to Nabs – other trials with other serotypes?
  - Risk of developing antibodies while waiting to participate (placebo controlled study, siblings)

- **Potential for virus shedding** and any needed precautions following gene therapy

- **Consequences of choices** (by opting in, what is the patient opting out of? Other future trials? Could a patient get a different vector if the trial he enroll in doesn’t work?)

- **Length of efficacy** - Do we really expect the effect to last forever, or might we reasonably expect it to wear off over time? What is known? What is unknown?

- **Informed consent** – Are all of these issues discussed?