

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?

