Points to Consider: Institutional Biosafety Committee (IBC) Review of Human Gene Transfer Protocols

Responsibilities of institutions and IBCs are articulated in Section IV-B of the *NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules* (*NIH Guidelines*). Institutions and IBCs subject to the *NIH Guidelines* are expected to establish policies and procedures to ensure that the research is conducted in full conformity with the provisions of the *NIH Guidelines*. Though any required documentation is at the discretion of the institution and IBC, the following information may be useful in reviewing human gene transfer protocols:

- A scientific abstract.
- The proposed clinical protocol, including tables, figures, and any relevant publications.
- Summary of preclinical studies conducted in support of the proposed clinical trial or reference to the specific section of the protocol providing this information.
- Product description, for instance:
    - Derivation of the delivery vector system including the source (e.g., viral, bacterial, plasmid), associated modifications (i.e., deletions to attenuate or self-inactivate, encapsulation in any synthetic complex, changes to tropisms), and previous clinical experience with the system
    - Genetic content of the transgene or nucleic acid delivered, including the species source of the sequence, and whether any modifications have been made (e.g., mutations, deletions, truncations)
    - Any other material to be used in preparation of the agent (vector and transgene) to be administered to research participants (e.g., helper virus, packaging cell line, carrier particles)
    - Methods for replication-competent virus testing
    - Intended *ex vivo* or *in vivo* target cells and transduction efficiency
    - Gene transfer agent delivery method