

University of Miami Institutional Biosafety Committee

Human Gene Transfer Trial Submission Checklist

- 2 - 3 page scientific abstract with references that includes background of the disease, previous treatments a brief description of proposed treatment and its benefits.
 - A description of the product:
 - The derivation of the delivery vector system including the source (e.g., viral, bacterial, or plasmid vector); and modifications (e.g., deletions to attenuate or self-inactivate, encapsulation in any synthetic complex, changes to tropisms, etc.). Please reference any previous clinical experience with this vector or similar vectors.
 - The 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, and truncations). What are the regulatory elements contained in the construct?
 - Any other material to be used in preparation of the agent (vector and transgene) that will be administered to the human research subject (e.g., helper virus, packaging cell line, carrier particles).
 - The methods for replication-competent virus testing, if applicable.
 - The intended *ex vivo* or *in vivo* target cells and transduction efficiency.
 - The gene transfer agent delivery method.
- Investigator's brochure or Clinical protocol including tables, figures and relevant manuscripts
- All FDA correspondence associated with approval of the IND (approval, holds, SAEs associated with the product)