

**Food and Drug Administration
Cellular, Tissue and Gene Therapies Advisory Committee
Meeting #41, February 9, 2006**

Potency Measurements for Cellular and Gene Therapy Products

DRAFT QUESTIONS

Q1: Assay Design and Validation:

Assay validation is the characterization of assay performance that allows the significance of the values obtained in an assay to be evaluated. Biological assays are particularly prone to assay variability and therefore it is necessary to design and execute the assay to reduce variability as much as possible while providing a statistically valid measure of the reproducibility of potency estimates.

Q: Please discuss assay design schemes that would be necessary to successfully validate biological assays and allow accurate quantification and interpretation of the result obtained? Please consider in the context of C> products.

Q2: Correlation studies:

Considering the diversity and complexity of C> products, the inherent variability of biological assays, and challenges particular to C> products (e.g. time constraints) it may not be feasible to develop a single, quantitative potency assay for a product. For this reason, sponsors may develop a set of multiple assays (matrix) to identify a quantitative analytical assay that correlates with biological function.

Q: With this in mind, what data and study considerations are necessary (e.g. assay design, statistical analysis, controls, limitations) to demonstrate valid correlations? Please consider in the context of C> products.

Q3: Incorporating state of the art technologies:

OCTGT supports the use of scientifically valid tests for potency, and encourages the use of current state of the art technologies (e.g. Q-FACS, genomics, proteomics) in order to provide detailed information about the biological characteristics of a product.

Q: How can these approaches be adapted and/or implemented to get consistent results necessary for product characterization and potency measurement in particular? What types of reference materials are needed in order to achieve reproducible results? Please consider in the context of C> products.