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Safety and Efficacy Assessment in Cell and Gene Therapy Products : Today's Thinking for Tomorrow's Success

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Cell and gene therapies has greatly being developed from academia and industry, therefore regulatory authorities confronted by providing the necessary guidance documents to ensure quality, safety and efficacy, and by providing rapid review of the application.

Direct benefit of the many advances in biotechnology and other enabling technologies has been the practice of pharmaceutical toxicology, which has shifted from a ritualistic standards-based approach to a rational science based approach. While the key variables of selection of a testing model, doses, regimens, and endpoints have not changed; the tools to better design and evaluate these variables have changed. The International Conference on Harmonisation of technical requirement for registration of pharmaceuticals for human use (ICH) has provided standardization and flexibility in design of toxicity study, and proposed uniformity in content and format. The validation and acceptance of alternative methods, use of non-traditional animal models, development of noninvasive and minimally invasive technologies, are all expected to not only improve the predictive value of preclinical studies but also increase the safety knowledge base.

The design, evaluation and regulation of cell and gene therapies have demanded state-of-the art knowledge of the latest biotechnology to anticipate risks and devise methods to address them. Science-based regulation satisfy requirements an expert regulators, applicable laws, regulation and policy. Effective regulation requires that regulators recognize the careful balance of potential but unproven benefits against real or theoretical risks. Responsible regulation requires a commitment by regulators to surveillance and compliance activities, sensitivity to public concerns including safety and ethics, and the support for and facilitation of active and ongoing scientific exchange among academia, industry and regulatory groups. Regulations continually evolve as regulatory experience grows from the generation of increasingly more and better data.

Considerable progress has been made in the discovery and early clinical development of a variety of cell and gene therapy products. Success has been enabled by the availability, validation and implementation of new technologies not only for production processes, but also for preclinical safety testing and evaluation. New challenges will need to be overcome to ensure that products will also be successful in later clinical development and ultimately for marketing authorization. Authorities and institutions responsible for regulatory oversight have recognized the need for and updating of the necessary programs to support the continued progress in the field of cell and gene therapy products leading to safe and effective therapies. Clear regulatory pathways and requirements are critical to future successes.