Regulatory Aspects of Gene Therapy Products

(Today's thinking for Tomorrow's success)

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Human gene therapy is the use of normal genes or genetic material to replace or cancel out the "bad" or defective genes in a person's body that are responsible for a disease or medical problem. And we define that gene therapy is the administration of genetic material in order to modify or manipulate the expression of a gene product or to alter the biological properties of living cells for therapeutic use. Scientific progress over the past decade has led to the development of novel methods for the transfer of genetic material to cells. 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. Vectors used in gene therapy clinical trials are mainly retrovirus, adenovirus, naked plasmid, pox, vaccinia virus vector, etc. and around 1,000 cases of clinical trial are performed to assess the efficacy in the world. 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. Also, manufacturers of gene therapy products must test their products extensively and meet requirements for safety, purity and potency. The design, evaluation and regulation of gene therapies have demanded state-of-the art knowledge of the latest biotechnology to anticipate risks and devise methods to address them. Science-based regulations are satisfied with not only requirements for expert regulators, applicable laws, regulation and policy but also needs of manufacturers.