Gene Therapy: Nonclinical and Regulatory Strategy

Module 2 Abstract: Nonclinical Regulatory Considerations for Gene Therapy Product Development

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Efforts in the development of gene therapy products for the treatment of human diseases have increased exponentially over the past decades. While the potential benefits of gene therapies are conceptually apparent, the risks associated with these products following administration in patients are not as well understood. Thus, the transition of these gene therapy products to clinical trials requires comprehensive characterization of product risks and how they can be potentially mitigated. This presentation will provide a general overview of the existing regulatory framework of CBER/OTAT to guide nonclinical assessment of activity and potential risks of gene therapy products to enable administration in early-phase clinical trials.