Gene Therapy: Nonclinical and Regulatory Strategy

Module 3 Abstract: Nonclinical Strategies for Gene Therapy Products to Support Regulatory Submission

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After decades as a therapeutic modality largely confined to the academic research environment, gene therapy has emerged in recent years as a rapidly expanding therapeutic modality in the biopharmaceutical industry. This interest in the field of gene therapy by industry has been enhanced by the recent success of approved therapies for curing genetic diseases such as ZOLGENSMA® for spinal muscular atrophy and LUXTURNA® for Leber congenital amaurosis. This presentation will discuss things to consider during nonclinical development and nonclinical to clinical translation of gene therapies. Topics that will be covered include regulatory guideline expectations, vector quality attributes, nonclinical study design, species selection, dose selection for toxicity studies, pre and post treatment immune assessment, use of animal models of disease for safety assessment, biodistribution, and assessment of genotoxicity risk. The presentation will culminate with several case studies in nonclinical to clinical translation.