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Regulatory Roadmap for Initiating a Gene Therapy Drug into Clinical Trials in the US

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Abstract

Exciting progress has been made in the development of gene therapy, and experimental research has brought forward novel treatment opportunities for viral vectors, DNA vectors, and gene-modified cell therapies. Clinical development of a gene therapy drug is challenging, requiring understanding of controlled manufacturing, relevant nonclinical pharmacology and safety studies, and clinical risk factors. For initiating clinical trials in the United States, regulatory requirements for investigational gene therapy drugs are more stringent than those with other investigational biologics (recombinant antibodies or recombinant proteins). This talk will highlight these requirements, including the following: 1) submissions to regulatory authorities, 2) manufacturing, and 3) non-clinical studies.

Biography

Dr. William Lee received his B.A. from Johns Hopkins University, and Ph.D. from Cornell University Graduate School of Medical Sciences. Dr. Lee has twenty years of research and industry experience. His focus is on gene therapy with retroviral vectors, adeno-associated viral vectors and DNA vectors. He spent 9 years at the gene therapy start-up firm, Viagene, Inc., followed by 2 years at Chiron. In 1999, he joined Cato Research, in Durham, North Carolina, where he is currently Vice President, Regulatory Affairs. His projects have included the design of Phase 1 and Phase 2 protocols for a gene therapy drug and interactions with the FDA and NIH/OBA. Currently, he manages projects involving regulatory strategy and submissions of investigational new drug applications and marketing applications for biologics and drugs.