Preclinical safety assessments of gene therapy products

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Gene therapy can be defined as the introduction of nucleic acids into a host cell to result in the replacement or inactivation of a mutated gene, or to introduce something novel. The introduction of the nucleic acids can occur inside or outside of the body, often with the use of a viral vector, such as adeno-associated viruses (AAV). In the past several years, there has been a dramatic increase in the use of gene therapies to develop potential therapies for treating various diseases. However, there are multiple challenges associated with this relatively novel technique; such as antibody or cell mediated responses that limit efficacy, lack of data on the long term safety, and an evolving regulatory environment that establishes the requirements for drug development of gene therapies. This presentation will provide an overview of gene therapy, review the key aspects of the available nonclinical regulatory guidance, discuss things to consider when developing an nonclinical toxicology strategy, and examine case studies of nonclinical packages used for the development of novel therapies.
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