Module 2: Thursday, February 17, 2022

Nonclinical Regulatory Considerations for Human Gene Therapies: An FDA/CBER Perspective

Unanswered Questions from the Live Q&A Module 2 Session / Responses provided by Dr. Sanduja:

Question #22 – Question on AAV and DRG –
Answer: Was answered, and to be covered by the next speaker in Module 3.

Question #23 – Do assays for analysis of NHP tissues/samples need to be GLP?
Answer: No

Question #26 – In vivo or in vitro assessment of immunogenicity requirements for AAV vectors?
Answer: From a preclinical perspective- assessment of immune response should be part of animal tox studies.

Question #27 – Are safety pharm such as hERG required? – this is in vitro screen for cardiovascular
Answer: No. For products with potential risks or cardiac indications – safety parameters, cardiac markers are to be assessed in the tox study.

Question #28 – Screening for immunogenicity before dosing to maximize GT exposure?
Answer: No

Question #29 – how long does it take to get an INTERACT – I think this was answered and also in the SOPP 8214 (FDA schedules meeting in 21 days and meeting is within 90 days of request receipt)
Answer: Was covered/answered.

Question #30 – General consensus on ELISPOT in tox studies?
Answer: The context of this Q is unclear- but in general- yes, to understand immune response to GT product.

Question #25 – Do hybrid pharmacology/tox studies need to be GLP compliant if in animal models?
Answer: Non-GLP may be acceptable; however, the study should be well controlled, nonbiased, with appropriate record keeping, etc.