Gene Therapy Liaison Meeting
FDA and ASGCT
September 13, 2018, 1 – 4 p.m.
Sheraton Silver Spring Hotel
8777 Georgia Avenue, Silver Spring, MD

AGENDA

Co-Chairs: Maritza McIntyre, PhD, ASGCT Clinical Trials and Regulatory Affairs Committee Chair
Donald Kohn, MD, ASGCT Government Relations Committee

Time	Agenda Topic	Speaker
1:00 – 1:10	Call to Order, Introduction of Agenda and Meeting Participants	ASGCT: Maritza McIntyre, PhD Director, CBER: Peter Marks, MD, PhD
1:10 – 1:30	Presentation 1: Replication competent lentivirus (RCL) and replication competent retrovirus (RCR) testing of drug product	ASGCT Member: Kenneth Cornetta, MD
1:30 – 1:40	Discussion	
1:40 – 2:00	Presentation 2: Testing methods and depth recommendation for off-target analysis of gene editing technologies	ASGCT Member: J. Keith Joung, MD, PhD
2:00 – 2:10	Discussion	
2:10 – 2:20	Break	
2:20 – 2:40	Presentation 3: Presented by FDA CBER RMAT designation update regarding alternative clinical data/trial designs and eligibility of gene therapies with durable modification of cells	Director, OTAT: Wilson Bryan, MD
2:40 – 2:50	Discussion	
2:50 – 3:10	Presentation 4: Manufacturing considerations • Appropriate manufacturing controls for genetic/biological starting materials and process intermediates (i.e., plasmids, producer viruses) • Suitability of certain cell lines for manufacturing specific vector classes and recommendations to address risk	ASGCT Member: John T. Gray, PhD
3:10 – 3:20	Discussion	
3:20 – 3:40	Presentation 5: Data from long-term follow-up for persistent vs. transient gene therapy product classes on critical safety parameters	ASGCT Members: RNA Vectors: Helen Heslop, MD DNA Vectors: Ronald Crystal, MD
3:40 – 3:50	Discussion	
3:50 – 4:00	Closing Remarks	ASGCT: Donald Kohn, MD