FDA U.S. FOOD & DRUG ADMINISTRATION
ONCOLOGY CENTER OF EXCELLENCE

Product Development in Hemophilia Public Workshop

Hashtag: #OCEhemophilia18

Co-chaired by:
Center for Biologics Evaluation and Research
Center for Drug Evaluation and Research

FDA White Oak Campus – Great Room
10903 New Hampshire Avenue, Silver Spring, MD 20993
December 6, 2018
8:30 am to 4:30 pm

AGENDA		
Time	**Topics**	**Speakers**
8:00 – 8:30 am	**Registration – Great Room Reception Area**	
Welcome and Opening Remarks		
8:30 – 8:35 am	Welcome and Opening Remarks	Peter Marks
8:35 – 8:45 am	FDA 101	Albert Deisseroth
8:45 to 8:55 am	CBER Points of Interest	Jay Lozier
8:55 - 9:05 am	CDER Points of Interest	Laurel Menapace
Session 1: Overview of Product Development in Hemophilia Moderator: Lori Ehrlich (CDER)		
9:05 - 9:25 am	Overview of Product Development in Hemophilia	Margaret Ragni (University of Pittsburgh)
9:25 – 9:40 am	**BREAK**	
Session 2: Clinical Endpoints Moderator: Najat Bouchkouj (CBER)		
9:40 - 10:00 am	Synthesis and Physiology of Coagulation Factors: Effect on Clinical Hemostasis	Robert Montgomery (BloodCenter of Wisconsin)
10:00 - 10:20 am	Joint Bleeding and Hemarthrosis	Marilyn Manco-Johnson (University of Colorado)
10:20 – 10:40 am	Panel Discussion	Session Speakers with Laurel Menapace, Lori Ehrlich, Najat Bouchkouj & Poornima Sharma

Session 3: Overview of Patient-Reported Outcomes (PROs) Moderator: Laurel Menapace (CDER)		
10:40 – 10:50 am	COA Staff Perspective	Elektra Papadopoulos
10:50 – 11:10 am	PRO Instruments in Hemophilia	Christine Kempton (Emory University)
11:10 – 11:30 am (5 min each)	Patient Perspective	Christopher Templin Shelby Smoak George Stone Miriam Goldstein
11:30 - 11:40 am	Clinician Perspective	Christine Guelcher (Children's National Health System)
11:40 – 11:50 pm	Reviewer Perspective	Laurel Menapace, Lori Ehrlich, Virginia Kwitkowski & Bellinda King-Kallimanis
11:50 – 12:15 pm	Panel Discussion	Session Speakers & FDA representatives
12:15 – 1:15 pm	**LUNCH**	
Session 4: Factor Activity as a Surrogate Endpoint Moderator: Mikhail Ovanesov (CBER)		
1:15 – 1:35 pm	Analytical Perspective: Methodology and Reference Standards	Elaine Gray (UK Medicines and Healthcare Products Regulatory Agency)
1:35 – 1:55 pm	Clinical Lab Perspective: Replacement Therapy vs. Gene Therapy	Steven Pipe (University of Michigan)
1:55 – 2:25 pm	Panel Discussion: Factor Activity Assay Discrepancies in Clinical Trials	Session Speakers & Panelists: Kenneth Friedman (BloodCenter of Wisconsin), Johannes Dodt (Paul-Ehrlich-Institut, Germany) & Richard Marlar (University of New Mexico)
2:25 – 2:35 pm	**BREAK**	
Session 5: Clinical Trial Design Moderator: Jay Lozier (CBER)		
2:35 – 2:50 pm	Duration of Gene Therapy Response	Amy Shapiro (Indiana Hemophilia and Thrombosis Center)
2:50 – 3:05 pm	Adolescent Liver Development	Stacey Huppert (Cincinnati Children's Hospital)
3:05 – 3:20 pm	Tumorigenesis with AAV Gene Transfer	Mark Sands (Washington University, St Louis)
3:20 – 3:35 pm	Surveillance for Hepatocellular Carcinoma in Humans	Theo Heller (National Institute of Diabetes and Digestive and Kidney Disease (NIDDK), NIH
3:35 – 4:00 pm	Panel Discussion	Session speakers & Jay Lozier, Bindu George
Closing Session		
4:00 – 4:30 pm	Wrap Up	Ann Farrell & Jay Lozier
4:30 pm	**ADJOURN**	

Program Committee		
CBER	**CDER**	**Office of Patient Affairs**
Bindu George	Ann Farrell	Andrea Furia-Helms
Jay Lozier	Al Deisseroth	Susan Chittooran
Najat Bouchkouj	Lori Ehrlich	Lauren Spicher
Mikhail Ovanesov	Laurel Menapace	Salina Miller
Poornima Sharma	Bellinda Kallimanis	
Megha Kaushal	Virginia Kwitkowski	
	Joan Todd	
	Tony Cossentino	
	Dianne Spillman	
	Valerie Vashio	