Considerations for Progressive Multifocal Leukoencephalopathy (PML) Clinical Trial Designs FDA Workshop
Agenda
September 21, 2021
10:00– 16:15 (10:00 am – 4:15 pm) Eastern Standard Time (EST)

Time (EST)	Topic	Speaker(s)
10:00 - 10:15	Opening Remarks	Speakers: Debra Birnkrant, MD (FDA/CDER/Division of Antivirals) & Virginia Sheikh, MD (FDA/CDER/Division of Antivirals)
Background		
10:15 - 10:30	JC Virus Virology and PML Pathogenesis	Speaker: Eugene Major, PhD (NIH/NINDS)
10:30 - 10:50	PML Drug Development History, Current Standard-of-Care, and PML Therapeutic Landscape	Speaker: David Clifford, MD (Washington University in St. Louis)
10:50 - 11:05	Clinical Outcomes among PML Patient Populations	Speaker: Bryan Smith, MD (NIH/NINDS)
11:05 - 11:15	***Break***	
Trial Endpoints		
11:15 - 11:25	Considerations for Trial Endpoints	Speaker: Virginia Sheikh, MD (FDA/CDER/Division of Antivirals)
11:25 - 11:35	PML Disability Outcome Measures	Speaker: Laura Baldassari, MD, MHS (FDA/CDER/Division of Neurology 2)
11:35 - 11:45	Brain Imaging in PML	Speaker: Mike Wattjes, MD (Hannover Medical School, Germany)
11:45 – 12.05	Evaluating the Potential for JCV DNA in Cerebrospinal Fluid as an Endpoint in Clinical Trials for PML Product Development	Speakers: Irene Cortese, MD (NIH/NINDS) & Gina Norato, Sc.M (NIH/NINDS) & Paola Cinque, MD, PhD (San Raffaele Scientific Institute)
12:05 - 12:50	Discussion and Q&A: Potential Endpoints in PML Clinical Trials	Moderator: Patrick Harrington, PhD (FDA/CDER/Division of Antivirals) Panelists: Igor Koralnik, MD (Northwestern University), Roland Martin, MD (University of Zurich), Clemens Warnke, MD (University of Cologne), Serena Spudich, MD (Yale), Avindra Nath, MD (NIH/NINDS), Christina Marra, MD (University of Washington), Jennifer Lyons, MD (Biogen)
12:50 – 13:20	***Break (lunch)***	

Patient Perspectives		
13:20 - 13:35	Patient Focused Drug Development for PML	Speaker: Joan Ohayon**,** MSN, CRNP, MSCN (NIH/NINDS)
13:35 - 13:45	Patient Perspective	Speaker: Suzanne Tobin
13:45 - 13:55	Patient Perspective	Speaker: Luca Isabella
Selection of Control Groups		
13:55 - 14:15	Selection of Control Groups for PML Clinical Trials	Speaker: Paul Lee, MD, PhD (FDA/CDER/Division of Neurology 2)
14:15 - 14:55	Discussion and Q&A: Selection of Control Groups for PML Clinical Trials	Moderator: Virginia Sheikh, MD (FDA/CDER/Division of Antivirals) Panelists: Joseph Berger, MD (University of Pennsylvania), Farrah Mateen, MD, PhD (Massachusetts General Hospital), Kiran Thakur, MD (Columbia University), Guillaume Martin-Blondel, MD, PhD (University of Toulouse), Gloria von Geldern, MD (University of Washington), Derrell Porter, MD (Cellevolve)
14:55 – 15:05	***Break***	
Trial Design		
15:05 - 15:25	Potential Clinical Trial Designs for PML Treatments	Speakers: Irene Cortese, MD (NIH/NINDS) & Ken Cheung, PhD (Columbia University)
15:25 - 16:10	Discussion and Q&A: Clinical Trial Designs for PML Treatments	Moderator: Laura Baldassari, MD, MHS (FDA) Panelists: Walter Royal, III, MD (Morehouse School of Medicine), David Clifford, MD (Washington University in St. Louis), Lori Dodd, PhD (NIH/NIAID), Andrew Goodman, MD (University of Rochester), Sabrina Tan, MD (Beth Israel Deaconess Medical Center), Paola Cinque, MD, PhD (San Raffaele Scientific Institute), NgocDiep Le, MD, PhD (NeoImmune Tech)
16:10 -16:15	Closing Remarks	Speaker: Virginia Sheikh, MD (FDA/CDER/Division of Antivirals)
16:15	Adjourn	