## 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)	Торіс	Speaker(s)
10:00 - 10:15	Opening Remarks	<u>Speakers:</u> 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	<u>Speakers:</u> 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), Christin Marra, MD (University of Washington), Jennifer Lyons, MD (Biogen)
12:50 - 13:20	Break (lunch)	interior (charterior) of trasmington, seminer Lyons, the (Diogen)

14:15 - 14:55   Discussion and Q&A: Selection of Control Groups for PML Clinical Trials   Moderator: Virginia Sheikh, MD (FDA/CDER/Division of Antivir Panelists: Joseph Berger, MD (University of Pennsylvania), Farrah Mateen, J PhD (Massachusetts General Hospital), Kiran Thakur, MD (Colun University), Guillaume Martin-Blondel, MD, PhD (University of Toulouse), Gloria von Geldern, MD (University of Washington), I Porter, MD (Cellevolve)     14:55 - 15:05   Break     Trial Design		ctives		
13:45 - 13:55   Patient Perspective   Speaker: Luca Isabella     Selection of Control Groups   Selection of Control Groups for PML Clinical Trials   Speaker: Paul Lee, MD, PhD (FDA/CDER/Division of Neurology     14:15 - 14:55   Discussion and Q&A: Selection of Control Groups for PML Clinical Trials   Moderator: Virginia Sheikh, MD (FDA/CDER/Division of Antivir PML Clinical Trials     14:15 - 14:55   Discussion and Q&A: Selection of Control Groups for PML Clinical Trials   Moderator: Virginia Sheikh, MD (FDA/CDER/Division of Antivir Panelists: Joseph Berger, MD (University of Pennsylvania), Farrah Mateen, I PhD (Massachusetts General Hospital), Kiran Thakur, MD (Colun University), Guillaume Martin-Blondel, MD, PhD (University of Toulouse), Gloria von Geldern, MD (University of Washington), I Porter, MD (Cellevolve)     14:55 - 15:05   Break     Trial Design   Speakers: Irene Cortese, MD (NIH/NINDS) & Ken Cheung, PhD (Columbia University)     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:   Panelists:   Moderator: Laura Baldassari, MD, MHS (FDA)		Speaker: Joan Ohayon, MSN, CRNP, MSCN (NIH/NINDS)	Patient Focused Drug Development for PML	13:20 - 13:35
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     14:15 - 14:55   Discussion and Q&A: Selection of Control Groups for PML Clinical Trials   Moderator: Virginia Sheikh, MD (FDA/CDER/Division of Antivir PML Clinical Trials     14:15 - 14:55   Discussion and Q&A: Selection of Control Groups for PML Clinical Trials   Moderator: Virginia Sheikh, MD (FDA/CDER/Division of Antivir Panelists: Joseph Berger, MD (University of Pennsylvania), Farrah Mateen, J PhD (Massachusetts General Hospital), Kiran Thakur, MD (Colum University), Guillaume Martin-Blondel, MD, PhD (University of Toulouse), Gloria von Geldern, MD (University of Washington), I Porter, MD (Cellevolve)     14:55 - 15:05   Break     Trial Design   Speakers: Irene Cortese, MD (NIH/NINDS) & Ken Cheung, PhD (Columbia University)     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)     15:25 - 16:10   Discussion and Q&A: Clinical Trial Designs for PML Treatments   Panelists:		Speaker: Suzanne Tobin	Patient Perspective	13:35 - 13:45
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