FOOD AND DRUG ADMINISTRATION (FDA)
Center for Drug Evaluation and Research (CDER)

Peripheral and Central Nervous System Drugs Advisory Committee (PCNS) Meeting
College Park Marriott Hotel and Conference Center, Chesapeake Ballroom
3501 University Blvd. East, Hyattsville, Maryland
April 25, 2016

DRAFT AGENDA

The committee will discuss new drug application (NDA) 206488, eteplirsen injection for intravenous infusion, sponsored by Sarepta Therapeutics, Inc., for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping.

8:00 a.m.	Call to Order and Introduction of Committee	**George C. Alexander, MD, MS** Chairperson, PCNS
8:05 a.m.	Conflict of Interest Statement	**Moon Hee V. Choi, PharmD** Designated Federal Officer, PCNS
8:10 a.m.	FDA Introductory Remarks	**Billy Dunn, MD** Director, Division of Neurology Products (DNP) Office of Drug Evaluation I (ODE-I) Office of New Drugs (OND) CDER, FDA
8:30 a.m.	**APPLICANT PRESENTATIONS**	**Sarepta Therapeutics, Inc.**
	Introduction	**Shamim Ruff, MSc** Senior Vice President Regulatory Affairs and Quality Sarepta Therapeutics, Inc.
	Disease Background and Natural History	**Eugenio Mercuri, MD, PhD** Professor of Pediatric Neurology Catholic University of the Sacred Heart
	Efficacy	**Edward M. Kaye, MD** Chief Medical Officer (Interim CEO) Sarepta Therapeutics, Inc.
	Safety	**Helen Eliopoulos, MD** Senior Medical Director Sarepta Therapeutics, Inc.
	Clinical Perspective	**Jerry Mendell, MD** Director, Center for Gene Therapy Professor of Pediatrics and Neurology Curran-Peters Chair of Pediatric Research Nationwide Children's Hospital
	Concluding Remarks	**Edward M. Kaye, MD**

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DRAFT AGENDA (cont.)

	APPLICANT GUEST SPEAKER PRESENTATION	
	Patient and Caregiver Reported Outcomes of Patients in Clinical Trials of Eteplirsen for Treatment of Duchenne	**Christine McSherry** Executive Director Jett Foundation
10:15 a.m.	Clarifying Questions	
10:30 a.m.	**BREAK**	
10:45 a.m.	**FDA PRESENTATIONS**	
	Issues to consider with External Control Studies	**Robert Temple, MD** Acting Deputy Director, ODE-I Deputy Center Director for Clinical Science CDER, FDA
	FDA Efficacy Review	**Ronald Farkas, MD, PhD** Clinical Team Leader Division of Neurology Products ODE-I, OND, CDER, FDA
		Ashutosh Rao, PhD Acting Chief Laboratory of Applied Biochemistry Division of Biotechnology Review & Research III Office of Biotechnology Products Office of Pharmaceutical Quality, CDER, FDA
	Concluding Remarks	**Eric Bastings, MD** Deputy Director Division of Neurology Products ODE-I, OND, CDER, FDA
	Center Director's Remarks	**Janet Woodcock, MD** Director CDER, FDA
12:45 p.m.	Clarifying Questions	
1:00 p.m.	**LUNCH**	
2:00 p.m.	Open Public Hearing	

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DRAFT AGENDA (cont.)

4:30 p.m. **BREAK**

4:45 p.m. Questions to the Committee/Committee Discussion

6:30 p.m. **ADJOURNMENT**