

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

Peripheral and Central Nervous System Drugs Advisory Committee (PCNS) Meeting
Tommy Douglas Conference Center
10000 New Hampshire Avenue, Silver Spring, Maryland
September 28, 2017

DRAFT AGENDA

The committee will discuss new drug application (NDA) 200896, ataluren for oral suspension, sponsored by PTC Therapeutics, Inc., for the treatment of patients with dystrophinopathy due to a nonsense mutation in the dystrophin gene.

9:00 a.m.	Call to Order and Introduction of Committee	G. Caleb Alexander, MD, MS Chairperson, PCNS
9:05 a.m.	Conflict of Interest Statement	Moon Hee V. Choi, PharmD Designated Federal Officer, PCNS
9:10 a.m.	FDA Opening Remarks	Billy Dunn, MD Director, Division of Neurology Products (DNP) Office of Drug Evaluation I (ODE-I) Office of New Drugs (OND), CDER, FDA
9:30 a.m.	APPLICANT PRESENTATIONS	PTC Therapeutics, Inc.
	Introduction	Murad Husain, RPh, MS Senior Vice President, Global Regulatory Affairs PTC Therapeutics, Inc.
	DMD Natural History and Clinical Endpoints	Kevin Flanigan, MD Director, Center for Gene Therapy Nationwide Children's Hospital
	Ataluren Mechanism of Action	Ellen Welch, PhD Senior Vice President, Genetic Disorders PTC Therapeutics, Inc.
	Ataluren Efficacy and Safety	Joe McIntosh, MD Senior Vice President, Head of Clinical Development PTC Therapeutics, Inc.
	Clinical Perspective	Craig McDonald, MD Director, Neuromuscular Disease Clinics University of California, Davis Children's Hospital Study Chair, CINRG Duchenne Natural History Study
10:45 a.m.	Clarifying Questions	
11:00 a.m.	BREAK	

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DRAFT AGENDA (cont.)

11:15 a.m. **FDA PRESENTATIONS**

Efficacy Considerations

Robert Temple, MD
Acting Deputy Director
ODE-I, OND, CDER, FDA

Veneeta Tandon, PhD
Clinical Reviewer
DNP, ODE-I, OND, CDER, FDA

Xiang Ling, PhD
Senior Staff Fellow
Division of Biometrics I, Office of Biostatistics
Office of Translational Sciences (OTS), CDER, FDA

Venkatesh Atul Bhattaram, PhD
Senior Staff Fellow, Division of Pharmacometrics
Office of Clinical Pharmacology (OCP), OTS, CDER, FDA

Ashutosh Rao, PhD
Chief, Laboratory of Applied Biochemistry
Division of Biotechnology Review and Research III
Office of Biotechnology Products
Office of Pharmaceutical Quality, CDER, FDA

James L. Weaver, PhD
Research Pharmacologist
Division of Applied Regulatory Science
OCP, OTS, CDER, FDA

Nick Kozauer, MD
Clinical Team Leader
DNP, ODE-I, OND, CDER, FDA

12:30 p.m. Clarifying Questions

12:45 p.m. **LUNCH**

1:45 p.m. Open Public Hearing

3:15 p.m. Questions to the Committee/Committee Discussion

4:30 p.m. **ADJOURNMENT**