

**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

**AGENDA**

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*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.*

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

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**AGENDA (cont.)**

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

Efficacy Considerations

**Robert Temple, MD**  
Deputy Center Director for Clinical Science  
CDER, FDA

Ataluren Efficacy - Overview

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

Ataluren Efficacy Statistical  
Review

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

Ataluren Efficacy – Key  
Considerations

**Veneeta Tandon, PhD**

Evaluation of the Hypothesis of an  
“Inverted-U” Shaped Exposure-  
Response Relationship of Ataluren

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

Limitations of the Bioassays Used  
for Dystrophin in Studies 004 and  
007

**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

Nonclinical and In vitro  
Dystrophin Models

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

FDA Summary

**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

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**AGENDA (cont.)**

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

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