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

Genetic Metabolic Diseases Advisory Committee (GeMDAC) Meeting
August 2, 2024

AGENDA

The Committee will discuss new drug application 214927, for arimoclomol, submitted by Zevra Denmark A/S, for the treatment of adults and pediatric patients 2 years of age and older with Niemann-Pick disease type C.

9:00 a.m.	Call to Order and Introduction of Committee	**Robert Alexander, MD** Acting Chairperson, GeMDAC
9:10 a.m.	Conflict of Interest Statement	**Moon Hee V. Choi, PharmD** Designated Federal Officer, GeMDAC
9:15 a.m.	FDA Initial Remarks	**Patrizia Cavazzoni, MD** Director CDER, FDA
9:20 a.m.	FDA Opening Remarks	**Catherine Pilgrim-Grayson, MD, MPH** Acting Director Division of Rare Diseases and Medical Genetics (DRDMG) Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine (ORPURM) Office of New Drugs (OND), CDER, FDA
9:30 a.m.	**APPLICANT PRESENTATIONS**	**Zevra Therapeutics**
	Introduction	**Louise Himmelstrup** Vice President (VP), Regulatory Affairs Zevra Therapeutics
	Clinical Background on Niemann-Pick Type C	**Marc Patterson, MD** Professor of Neurology, Pediatrics and Medical Genetics Emeritus Chair Division of Child and Adolescent Neurology Mayo Clinic, Rochester, MN
	Pivotal Efficacy	**Dan Gallo, PhD** Senior VP, Medical Affairs and Advocacy Zevra Therapeutics
	Confirmatory Evidence of Effectiveness	**Travis Mickle, PhD** Co-Founder, Senior Advisor Zevra Therapeutics

AGENDA (cont.)

APPLICANT PRESENTATIONS (CONT.)

	Safety	**Christine í Dali, MD** VP, Group Clinical Science Zevra Therapeutics
	Clinical Perspective	**Kristina Julich, MD** Assistant Professor Department of Neurology Chief, Pediatric Neurogenetics Center University of Texas at Austin
11:00 a.m.	Clarifying Questions to the Applicant	
11:20 a.m.	**LUNCH**	
12:20 p.m.	**FDA PRESENTATIONS**	
	Overview of the Clinical Program	**Maura RZ Ruzhnikov, MD, FACMG** Clinical Reviewer DRDMG, ORPURM, OND, CDER, FDA
	Primary Efficacy Results in Pivotal Trial	**Wonyul Lee, PhD** Senior Staff Fellow Division of Biometrics IV Office of Biostatistics Office of Translational Sciences (OTS) CDER, FDA
	NPCCSS: Measurement Considerations	**Naomi Knoble, PhD** Associate Director Division of Clinical Outcome Assessment Office of Drug Evaluation Sciences OND, CDER, FDA
	Additional Data: Nonclinical	**Shawna L. Weis, PhD** Lead Pharmacologist (Acting) Division of Pharmacology/Toxicology for Rare Diseases, Pediatrics, Urologic and Reproductive Medicine ORPURM, OND, CDER, FDA

AGENDA (cont.)

	FDA PRESENTATIONS (CONT.)	
	Additional Data: Clinical Pharmacology	**Sydney Stern, PhD** Pharmacokineticist Division of Translational and Precision Medicine Office of Clinical Pharmacology OTS, CDER, FDA
	Additional Clinical Data and Summary	**Maura RZ Ruzhnikov, MD, FACMG**
1:50 p.m.	Clarifying Questions to the FDA	
2:10 p.m.	**BREAK**	
2:25 p.m.	**OPEN PUBLIC HEARING**	
3:25 p.m.	Charge to the Committee	**Catherine Pilgrim-Grayson, MD, MPH**
3:40 p.m.	Questions to the Committee/Committee Discussion	
6:00 p.m.	**ADJOURNMENT**	