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

Cardiovascular and Renal Drugs Advisory Committee (CRDAC) Meeting
October 10, 2024

AGENDA

The Committee will discuss new drug application (NDA) 215244, for elamipretide hydrochloride injection, submitted by Stealth BioTherapeutics Inc., for the treatment of Barth Syndrome.

8:15 a.m.	Call to Order and Introduction of Committee	**Javed Butler, MD, MPH, MBA** Chairperson, CRDAC
8:20 a.m.	Conflict of Interest Statement	**LaToya Bonner, PharmD, MBA** Designated Federal Officer, CRDAC
8:25 a.m.	FDA Introductory Remarks	**Hylton Joffe, MD, MMSc** Director Office of Cardiology, Hematology, Endocrinology, and Nephrology (OCHEN) Office of New Drugs (OND), CDER, FDA
8:35 a.m.	**APPLICANT PRESENTATIONS**	**Stealth BioTherapeutics Inc.**
	Introduction	**Reenie McCarthy** Chief Executive Officer Stealth BioTherapeutics
	Urgency of Unmet Need and Benefit:Risk Tolerance	**Kate McCurdy, MBA** Chair, Board of Directors Barth Syndrome Foundation
	Disease and Natural History Cohort	**Hilary Vernon, MD, PhD** Professor of Medical Genetics Johns Hopkins University School of Medicine
	Safety and Efficacy	**Jim Carr, PharmD** Chief Clinical Development Officer Stealth BioTherapeutics
	Statistical Perspectives	**Janet Wittes, PhD** Wittes LLC

AGENDA (cont.)

	APPLICANT PRESENTATIONS (CONT.)	
	Expanded Access Program	**Kathryn Chatfield, MD, PhD** Associate Professor of Pediatrics-Cardiology Director, Cardiac Genetics Clinic Children's Hospital Colorado
	Closing Remarks	**Reenie McCarthy**
10:05 a.m.	Clarifying Questions to the Applicant	
10:35 a.m.	**BREAK**	
10:50 a.m.	**FDA PRESENTATIONS**	
	Efficacy Assessment of Elamipretide for Barth Syndrome	**Ann Punnoose, MD** Clinical Reviewer Division of Cardiology and Nephrology OCHEN, OND, CDER, FDA **Steven Bai, PhD** Biometrics Reviewer Division of Biometrics II Office of Biostatistics Office of Translational Sciences CDER, FDA
11:50 a.m.	Clarifying Questions to the FDA	
12:15 p.m.	**LUNCH**	
1:15 p.m.	**OPEN PUBLIC HEARING**	
2:15 p.m.	Clarifying Questions to the Applicant and FDA	
2:30 p.m.	Charge to the Committee	**Hylton Joffe, MD, MMSc**
2:35 p.m.	Questions to the Committee/Committee Discussion	
3:30 p.m.	**BREAK**	
3:45 p.m.	Questions to the Committee/Committee Discussion (cont.)	
5:30 p.m.	**ADJOURNMENT**	