

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

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

DRAFT 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

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DRAFT 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