FOOD AND DRUG ADMINISTRATION (FDA)
Center for Biologics Evaluation and Research (CBER)
78th Meeting of the Cellular, Tissue, and Gene Therapies Advisory Committee (CTGTAC)
July 29, 2026
DRAFT AGENDA

The committee will meet in open session to discuss and make recommendations on Biologics License Application (BLA) 125842, from Capricor, Inc., for deramiocel submitted for the treatment of cardiomyopathy in Duchenne muscular dystrophy.

Time EDT	Presentation/Presenter
9:30 a.m.	**Opening Remarks: Call to Order and Welcome (5 min)** Evan Snyder, MD, PhD, FAAP, Acting Chairperson, CTGTAC **Administrative Announcements, Roll Call, Introduction of Committee, Conflict of Interest Statement (10 min)** LCDR Cicely Reese, PharmD, Designated Federal Officer CBER, FDA
9:45 a.m.	**FDA Welcome (5 min)** **Karim Mikhail, BPharm, MSc** Acting Director, CBER Acting Director, Office of Therapeutic Products (OTP) CBER, FDA
9:50 a.m.	**FDA Introduction and Regulatory Overview (20 min including Q & A)** **Introductory Remarks and Regulatory Background of BLA** **Patroula Smpokou, MD, FACMG** Director, Division of Clinical Evaluation and General Medicine (DCEGM) Office of Clinical Evaluation (OCE) OTP, CBER, FDA **Q & A (5 min)**
10:10 a.m.	**Applicant Presentations (70 min including Q & A)** **Introduction** **Linda Marban, PhD** Chief Executive Officer and Director Capricor, Inc. **Deramiocel: Mechanism of Action & Preclinical Evidence of Efficacy** **Eduardo Marban, MD, PhD** Professor and Executive Director, Smidt Heart Institute Cedars-Sinai Health Sciences University

	Deramiocel Potency **Kristi Elliott, PhD** Chief Operating & Science Officer Capricor, Inc. **Statistical Overview and Methods** **Adam Sullivan, PhD** Senior Director of Biostatistics Capricor, Inc. **Skeletal Efficacy – Phase 2 and Phase 3 Placebo-Controlled Randomized Trials** **Craig McDonald, MD** Director MDA Clinics and Neuromuscular Research Laboratory UC Davis Health; Chair CINRG Duchenne Natural History Study **Cardiac Efficacy** **Jonathan Soslow, MD, MSCI** Director of Clinical Research, Pediatric Cardiology Co-Director, Duchenne Muscular Dystrophy Multispecialty Clinic Director of the Duchenne Muscular Dystrophy Cardiovascular Care Consortium (DMDCCC), Vanderbilt University Medical Center **Safety** **Michael Binks, MD** Chief Medical Officer Capricor, Inc. **Meaningfulness of Results** **Mindy Leffler** Patient Advocacy Capricor, Inc. **Conclusion** **Linda Marban, PhD** **Q & A (15 min)**
11:20 a.m.	**LUNCH (45 min)**
12:05 p.m.	**<u>Open Public Hearing</u> (90 min)**
1:35 p.m.	**BREAK (30 min)**

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2:05 p.m.	**<u>FDA Presentations</u> (70 min including Q & A)** **BLA 125842** **Erica Young, MD, MSCI** Clinical Reviewer, General Medicine Branch 4 OCE, DCEGM OTP, CBER, FDA **Tingting Zhou, PhD** Biostatistical Team Lead Division of Biostatistics Office of Biostatistics and Pharmacovigilance CBER, FDA **Prateek Shukla, MD** Acting Branch Chief, General Medicine Branch 3 OCE, DCEGM OTP, CBER, FDA **Q & A (15 min)**
3:15 p.m.	**<u>Committee Discussion</u> (90 min)** • Discussion Questions
4:45 p.m.	**<u>Closing Remarks</u> (5 min)** **Megha Kaushal, MD, MSc** Acting Deputy Director, OTP CBER, FDA
4:50 p.m.	**ADJOURNMENT**