

FOOD AND DRUG ADMINISTRATION (FDA)
Center for Biologics Evaluation and Research (CBER)
74th Meeting of the Cellular, Tissue, and Gene Therapies Advisory Committee (CTGTAC)
May 12, 2023
DRAFT AGENDA

Meeting Topic:

The committee will meet in open session to discuss the biologics license application (BLA) 125781 from Sarepta Therapeutics, Inc. for delandistrogene moxeparvovec (SRP-9001). The applicant has requested an indication for the treatment of ambulatory patients with Duchenne Muscular Dystrophy (DMD) with a confirmed mutation in the DMD gene.

(EDT)	Presentation/Presenter	
9:00 a.m.	Opening Remarks: Call to Order and Welcome (5 min.)	Taby Ahsan, Ph.D. Acting Chair, CTGTAC Vice President, Cell Therapy Operations City of Hope Duarte, CA
9:05 a.m.	Administrative Announcements, Roll Call, Introduction of Committee, and Conflict of Interest Statement (20 min.)	Marie DeGregorio Designated Federal Officer, CTGTAC Division of Scientific Advisors and Consultants CBER, FDA
9:25 a.m.	FDA Opening Remarks (15 min.)	Celia Witten, Ph.D., M.D. Acting Director Office of Therapeutic Products (OTP), CBER, FDA
9:40 a.m.	Q&A (5 min.)	
9:45 a.m.	FDA Overview of BLA 125781, Application for Accelerated Approval of delandistrogene moxeparvovec (SRP-9001) (15 min.)	Rosa Sherafat-Kazemzadeh, M.D. Clinical Team Lead Office of Clinical Evaluation, Division of Clinical Evaluation and General Medicine OTP, CBER, FDA
10:00 a.m.	Q&A (5 min.)	
10:05 a.m.	BREAK (10 min.)	
10:15 a.m.	Sponsor Presentation (75 min.) Introduction Disease Background and Unmet Need	Sarepta Therapeutics, Inc. Patrick O'Malley Vice President, Regulatory Affairs Sarepta Therapeutics Jerry Mendell, M.D.

FOOD AND DRUG ADMINISTRATION (FDA)
Center for Biologics Evaluation and Research (CBER)
Cellular, Tissue, and Gene Therapies Advisory Committee (CTGTAC)
May 12, 2023
DRAFT AGENDA

	Evidence for Surrogacy	Curran-Peters Chair of Pediatric Research Professor of Neurology and Pediatrics Nationwide Children’s Hospital and The Ohio State University College of Medicine Columbus, Ohio Louise Rodino-Klapac, Ph.D. Executive Vice President, Head of R&D, and Chief Scientific Officer Sarepta Therapeutics
	Clinical Trial Results	Stefanie Mason, M.D. Senior Medical Director, Clinical Development Sarepta Therapeutics
	External Control Analyses	James Signorovitch, Ph.D. Co-Founder The Collaborative Trajectory Analysis Plan
	External Control Results	Craig M. McDonald, M.D. Director, Neuromuscular Disease Clinic University of California, Davis Children’s Hospital Study Chair, CINRG Duchenne Natural History Study
	Summary of Safety	Eddie Darton, M.D. Executive Medical Director, Safety Evaluation and Risk Management Sarepta Therapeutics
	Clinical Perspective	Craig M. McDonald, M.D. Director, Neuromuscular Disease Clinic University of California, Davis Children’s Hospital Study Chair, CINRG Duchenne Natural History Study
11:30 a.m.	Q&A (15 min.)	
11:45 p.m.	LUNCH (45 min.)	
12:30 p.m.	OPEN PUBLIC HEARING (60 min.)	

FOOD AND DRUG ADMINISTRATION (FDA)
Center for Biologics Evaluation and Research (CBER)
Cellular, Tissue, and Gene Therapies Advisory Committee (CTGTAC)
May 12, 2023
DRAFT AGENDA

1:30 p.m.	BREAK (10 min.)	
1:40 p.m.	FDA Presentations (75 min.)	<p>Clinical, Chemistry, Manufacturing, and Controls (CMC), Pharmacology/Toxicology, and Clinical Pharmacology Reviewers:</p> <p>Mike Singer, M.D., Ph.D. Clinical Reviewer Office of Clinical Evaluation, Division of Clinical Evaluation and General Medicine OTP, CBER, FDA</p> <p>Emmanuel Adu-Gyamfi, Ph.D. Chemistry Manufacturing and Controls (CMC) Reviewer Office of Gene Therapy (CMC), Division of Gene Therapy OTP, CBER, FDA</p> <p>Theresa Chen, Ph.D. Pharmacology/Toxicology Reviewer Office of Pharmacology and Toxicology, Division of Pharmacology and Toxicology OTP, CBER, FDA</p> <p>Xiaofei Wang, Ph.D. Clinical Pharmacology Reviewer Office of Clinical Evaluation, Division of Clinical Evaluation and General Medicine OTP, CBER, FDA</p>
2:55 p.m.	Q&A (15 min.)	
3:10 p.m.	BREAK (10 min.)	
3:20 p.m.	Committee Discussion, Voting, and Vote Explanation (2 hours, 35 min.)	
5:55 p.m.	Closing Remarks (5 min.)	Peter Marks, M.D., Ph.D. Director, CBER, FDA

FOOD AND DRUG ADMINISTRATION (FDA)
Center for Biologics Evaluation and Research (CBER)
Cellular, Tissue, and Gene Therapies Advisory Committee (CTGTAC)
May 12, 2023
DRAFT AGENDA

6:00 p.m.	ADJOURNMENT	Marie DeGregorio Designated Federal Officer, CTGTAC
-----------	--------------------	---