**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**
**FINAL AGENDA**

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***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.*

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| (EDT) | Presentation/Presenter | |
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| 9:00 a.m. | **Opening Remarks: Call to Order and Welcome** (5 min.) | **Taby Ahsan, Ph.D.**<br>Acting Chair, CTGTAC<br>Vice President, Cell Therapy Operations<br>City of Hope<br>Duarte, CA |
| 9:05 a.m. | **Administrative Announcements, Roll Call, Introduction of Committee, and Conflict of Interest Statement** (20 min.) | **Marie DeGregorio**<br>Designated Federal Officer, CTGTAC<br>Division of Scientific Advisors and Consultants<br>CBER, FDA |
| 9:25 a.m. | **FDA Opening Remarks** (15 min.) | **Celia Witten, Ph.D., M.D.**<br>Deputy Director, CBER, FDA and<br>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.**<br>Clinical Team Lead<br>Office of Clinical Evaluation, Division of Clinical Evaluation and General Medicine<br>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.)<br><br>Introduction | **Sarepta Therapeutics, Inc.**<br><br>**Patrick O'Malley**<br>Vice President, Regulatory Affairs<br>Sarepta Therapeutics |

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

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| 12:30 p.m. | **OPEN PUBLIC HEARING** (60 min.) | |
| 1:30 p.m. | **BREAK** (10 min.) | |
| 1:40 p.m. | **FDA Presentations** (75 min.) | Clinical, Chemistry, Manufacturing, and Controls (CMC), Pharmacology/Toxicology, and Clinical Pharmacology Reviewers:<br><br>**Mike Singer, M.D., Ph.D.**<br>Clinical Reviewer<br>Office of Clinical Evaluation, Division of Clinical Evaluation and General Medicine<br>OTP, CBER, FDA<br><br>**Emmanuel Adu-Gyamfi, Ph.D.**<br>Chemistry Manufacturing and Controls (CMC) Reviewer<br>Office of Gene Therapy (CMC), Division of Gene Therapy<br>OTP, CBER, FDA<br><br>**Theresa Chen, Ph.D.**<br>Pharmacology/Toxicology Reviewer<br>Office of Pharmacology and Toxicology, Division of Pharmacology and Toxicology<br>OTP, CBER, FDA<br><br>**Xiaofei Wang, Ph.D.**<br>Clinical Pharmacology Reviewer<br>Office of Clinical Evaluation, Division of Clinical Evaluation and General Medicine<br>OTP, CBER, FDA |
| 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.** |

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| | | Director, CBER, FDA |
| 6:00 p.m. | **ADJOURNMENT** | **Marie DeGregorio**<br>Designated Federal Officer, CTGTAC |