

**SUMMARY OF INTERNAL MEETING**  
**[For Internal Purposes Only]**

Submission: BLA 125781/0

Office: OTP

Product: delandistrogene moxeparvovec-rokl

Applicant: Sarepta Therapeutics, Inc.

Meeting Date/Time: Thursday, May 18, 2023, from 3:30pm-4pm ET

**FDA Participants**

Rachel Duddy

Andrey Sarafanov

Carolyn Renshaw

Nicole Trudel

Christine Harman

Andrew Harmon

Brendan Day

Shiowjen Lee

Kimberly Schultz

Atul Bhattaram

Olivia Ma

Benjamin Cyge

Min Wu

Wei Liang

Cong Wang

Varsha Garnepudi

Rosa Sherafat

Maureen DeMar

Elin Cho

Carolyn Laurencot

Theresa Chen

Brian Stultz

Sukyoung Sohn

Carrie Mampilly

Anurag Sharma

Ramani Sista

Christopher Saeui

Lei Xu

Lilia Bi

Peter Marks

Nadia Whitt

Natasha Thorne

Larissa Lapteva

Anna Kwilas

Xiaofei Wang

Hao Zhu

Andrew Byrnes  
Tyree Newman  
Lori Tull  
Leila Hann  
Iwen Wu  
Meghna Alimchandani  
Christopher Jason  
John Scott  
Denise Gavin  
Anthony Lorenzo  
Celia Witten  
Meghna Alimchandani  
Anita Richardson  
Sandhya Sanduja  
Maryna Eichelberger  
Dennis Cato  
Tyree Newman  
Heather Lombardi  
Steven Oh  
Lea Carrington  
Vishnu Sharma  
Mike Singer  
Triet Tran  
Carolyn Laurencot  
Anurag Sharma

**Summary:**

Dr. Peter Marks expressed that he has given this application a fair amount of thought and that there are some major issues on data the applicant presented, issues on how the applicant responded to the questions, issues with the external controls and issues on the explanation of the results of their 6–7-year-old population.

Dr. Marks concludes gene therapy may have an effect on the 4-5 years of age population based on totality of information and that the biomarker of micro-dystrophin expression may be able to predict clinical benefit. Therefore, he would like to move forward with an Accelerated Approval (AA) for that age range based on available data and exercising regulatory flexibility. However, there is not any evidence that the biomarker of micro-dystrophin expression may be able to predict clinical benefit in the 6–7-year-old age range. Limiting the indication in a narrow patient population also minimize the potential of disrupting the ongoing Phase 3 trial, which is intended to serve as the confirmatory study if SRP-9001 is approved through Accelerated Approval pathway.

If the outcome from the ongoing confirmatory trial is negative, FDA would have to withdraw the AA. If it is positive, depending on the final data analyses, the label may be expanded to include additional age group(s).

Leila Hann commented on the difference in Process A and Process B and using Process A to justify AA in 4–5-year-olds. CMC commented that there is no analytical comparability.

Dr. Marks reiterated that the review team should write their memos as they feel appropriate. The review team will need to move forward to revise the proposed label.

Clinical recommended an updated indication of “Treatment of ambulatory pediatric patients aged 4 to 5 years with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the *DMD* gene.”

Clinical team also recommended that Section 14 of the label focus on Study 102 Part 1 study results and Study 103 Cohort 1. Clinical also recommended not including the data comparing SRP-9001 with the external control. Dr. Marks agreed with the team.

The questions regarding Process A versus Process B can be described in the review memos.

The review team will meet with Dr. Marks and Dr. Witten tomorrow, Friday, May 19th at 9:30am to discuss an updated timeline for the final review memos, SBRA and labeling. Dr. Peter Marks agreed to discuss the updated timeline and indication with the Applicant after the internal meeting tomorrow morning.

END