

SUMMARY OF INTERNAL MEETING
[For Internal Purposes Only]

Submission: BLA 125781/0

Office: OTP

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Applicant: Sarepta Therapeutics, Inc.

Meeting Date/Time: Monday, May 15, 2023, from 10am-10:30am ET

FDA Participants

Rachel Duddy

Maureen DeMar

Nadia Whitt

Brian Stultz

Anthony Lorenzo

Varsha Garnepudi

Brendan Day

Christopher Jason

Carolyn Laurencot

Atul Bhattaram

Lilia Bi

Anna Kwilas

Emmanuel Adu-Gyamfi

Cong Wang

Tyree Newman

Lisa Stockbridge

Shiowjen Lee

Triet Tran

Anurag Sharma

Wei Liang

Meghna Alimchandani

Theresa Chen

Benjamin Cyge

Ramani Sista

Elin Cho

Sukyoung Sohn

Zhenzhen Xu

Heather Lombardi

Andrew Harmon

Kimberly Schultz

Abby Shearin

Iwen Wu

Christopher Saeui

Leila Hann

Tao Pan

Boguang Zhen

Lori Tull
Narayan Nair
Nicole Trudel
Mara Miller
Carolyn Renshaw
Natasha Thorne
John Scott
Maryna Eichelberger
Vishnu Sharma
Denise Gavin
Rong Rong
Rosa Sherafat
Xiaofei Wang
Lei Xu
Mike Singer
Hao Zhu
Larissa Lapteva
Melissa Mendoza
Christine Harman
Anita Richardson
Min Wu
Elizabeth Hart
Celia Witten
Nicole Trudel
Olivia Ma

Summary:

The purpose of this internal meeting was to discuss the outcome of the Cell, Tissue and Gene Therapy (CTGT) Advisory Committee (AC) meeting held on Friday, May 12, 2023, and to plan the next steps for this BLA. Clinical, Clinical Pharmacology and Biostatistics teams stated that the discussion of AC committee did not support that Sarepta's micro-dystrophin is reasonably likely to predict clinical benefit to be used as a surrogate endpoint for accelerated approval and that they continue recommending a Complete Response based on the available data. CMC noted that they stand behind Clinicals decision but that CMC concerns will be addressed prior to approval. Dr. Celia Witten would like to discuss the review team's decision with CBER Center Director, Dr. Peter Marks, before commenting on a path forward.

END