



Duchenne Platform Trial Stakeholder Meeting

September 9, 2019

Sheraton Silver Spring – Silver Spring, Md.

Meeting Objectives:

- Discuss the planned master protocol and its rationale with key stakeholders
- Obtain feedback, build consensus and seek active community engagement
- Delineate next steps and timelines

AGENDA

7:30 am	Registration and Continental Breakfast <i>Cypress Ball Room</i>
8:30-9 am	Welcome and Introduction <i>Abby Bronson, MBA, Parent Project Muscular Dystrophy</i> <i>Ed Connor, MD, MBE, Institute for Advanced Clinical Trials (I-ACT) for Children</i> <i>Jane Larkindale, DPhil, Critical Path Institute</i>
9-9:30 am	Opening Remarks <i>Janet Woodcock, MD, U.S. Food and Drug Administration</i> <i>Billy Dunn, MD, U.S. Food and Drug Administration</i>
9:30-10 am	The DPT Protocol in Practice <i>Scott Berry, PhD, Berry Consultants</i>
10-10:30 am	Overview of the DPT Proposed Protocol <i>Deborah Ascheim, MD, d2a ltd</i>
10:30-10:45 am	Q & A
10:45-11 am	BREAK

11 am-Noon	<p>PANEL Discussion 1: Protocol Objectives and Eligibility</p> <p><i>Moderators: Abby Bronson, Scott Berry</i></p> <p><u>Panelists:</u> Joanne Donovan, MD, PhD, Catabasis Pharmaceuticals Michael Panzara, MD, MPH, Wave Life Sciences Hank Mayer, MD, Children’s Hospital of Philadelphia Paula Clemens, MD, University of Pittsburgh Gretchen Egner, Parent/Advocate</p>
Noon-12:45 pm	LUNCH
12:45-1:45 pm	<p>PANEL Discussion 2: Endpoints and Schedule of Events</p> <p><i>Moderators: Deborah Ascheim, Jane Larkindale</i></p> <p><u>Panelists:</u> Craig McDonald, MD, University of California, Davis Tina Duong, MPT, Stanford University Linda Cripe, MD, Nationwide Children’s Hospital Jodi Wolff, PhD, Santhera Roxana Dregghici, MD, Roche Beth Bumgarner, Parent/Advocate</p>
1:45-2:45 pm	<p>PANEL Discussion 3: Operations and Governance</p> <p><i>Moderators: Jane Larkindale, Deborah Ascheim</i></p> <p><u>Panelists:</u> Laura Lee Johnson, PhD, U.S. Food and Drug Administration Marianne Chase, BA, Massachusetts General Hospital Russell Butterfield, MD, PhD, University of Utah Karla Kramer, Parent/Advocate</p>
2:45-3 pm	BREAK
3-4 pm	<p>Q&A - Parent Commentary and Advocacy Perspective</p> <p><i>Moderator: Pat Furlong, Parent Project Muscular Dystrophy</i></p>
4-4:15 pm	<p>Key Take-Aways from Panel Discussions and Next Steps</p> <p><i>Deborah Ascheim</i></p>
4:15-4:30 pm	<p>Closing Remarks</p> <p><i>Abby Bronson Ed Connor Jane Larkindale</i></p>