

empowering families to

# FACE IT! LIVE IT! CHANGE IT!

## Parent Project Muscular Dystrophy 2006 Annual Conference Agenda

FACE IT!

THURSDAY, JULY 13<sup>TH</sup>

### What is DMD and what is it going to take to overcome it

2:00–7:00 PM	Registration Open	
2:00–2:20 PM	Welcome and Story of the Journey .....	Pat Furlong
2:20–2:40 PM	Duchenne 101 .....	Steve Wilton, PhD
2:40–3:00 PM	Downstream Pathology .....	Andrew Hoey, PhD
3:00–3:20 PM	Sequencing the Gene—Why it is Important .....	Kevin Flanigan, MD
3:20–3:40 PM	Q & A and Break	
3:40–4:00 PM	Strategies for Treatments and Cure .....	Dominick Wells, PhD
4:00–4:45 PM	Developing Treatments for DMD—Drug Development .....	Frank Ollington, PhD
4:45–5:15 PM	FDA—What is Required and Why .....	James Bona, PhD
5:15–5:45 PM	TRIALS—What it Means to Participate, Questions You Should Ask .....	Katie Bushby, MD
5:45–6:05 PM	Santhera Pharmaceutical's Drug Development Program for DMD: Protease Inhibitors and SNT MC 17/idebenone .....	Thomas Meier, PhD
6:05–6:30 PM	Q & A	
8:00 PM	Welcome reception	

LIVE IT!

FRIDAY, JULY 14<sup>TH</sup>

### Managing Care

7:00–8:00 AM	Registration open / Continental Breakfast	
8:00–8:20 AM	Welcome .....	Pat Furlong
8:20–8:40 AM	Interdisciplinary CARE—Who Needs to be Involved and Why .....	Linda Cripe, MD
8:40–9:10 AM	Steroids .....	Doug Biggar, MD
9:10–9:40 AM	Exercise and Physical Therapy .....	Helen Posselt, PT
9:40–10:00 AM	Cardiac .....	Larry Markham, MD
10:00–10:20 AM	Breathing—Respiratory Management .....	Jonathon Finder, MD
10:20–10:40 AM	Q & A and Break	
10:40–11:10 AM	Behavior and DMD .....	James Poysky, PhD
11:10–11:30 AM	Nutrition and Supplements .....	Kirana Rao
11:30–11:50 AM	How Much Do you Say and When .....	Norb Weidner, MD
11:50–12:30 PM	Q & A	
12:30–1:30 PM	Lunch	
1:30–2:00 PM	Advocacy / Collaborations: A Panel Discussion .....	Debra Lappin
2:00–2:40 PM	Role of PPMD .....	Pat Furlong
2:40–3:00 PM	Break	

**LIVE IT!**  
FRIDAY, JULY 14<sup>TH</sup>

**FRIDAY BREAKOUT SESSIONS**

	ROOM 1	ROOM 2	ROOM 3	ROOM 4	ROOM 5
3:00–3:40 PM	<b>Fathers and DMD:</b> Moderated by Kevin Smith	<b>Physical Therapy / Stretching:</b> Helen Posselt, Laura Case	<b>From where I'm seated:</b> Moderated by Mary-Lou Weisman, Larry Weisman and Norb Weidner	<b>The Family role in Advocacy and Data Collection:</b> Debra Lappin, Ed Dougherty, Nick Manetto	<b>Steroids:</b> Doug Biggar, Brenda Wong
3:45–4:25 PM	<b>Behavior in DMD:</b> James Poysky	<b>Nutrition and Supplements:</b> Kirana Rao	<b>Creating Awareness through Press: Media Training:</b> What you need to know Sarah Durham	<b>Sequencing the Gene and Genetic Counseling:</b> Kevin Flanigan, Karin Dent	<b>Respiratory:</b> Jonathan Finder, George Emerson, Michelle Eagle
4:30–5:10 PM	<b>Behavior in DMD:</b> James Poysky	<b>Physical Therapy / Stretching:</b> Helen Posselt, Laura Case	<b>Getting Involved: Fundraising, Advocacy, Awareness:</b> Kimberly Galberaith	<b>Adolescence and DMD:</b> Kathi Kinnett	<b>Cardiac Care:</b> Linda Cripe, Andrew Hoey, Larry Markham

5:15–5:35 PM	<b>National Institute of Health (NIH)</b> .....	John Porter, PhD
5:35–5:55 PM	<b>National Institute of Health (NIH)</b> .....	Glen Nuckolls, PhD
5:55–6:15 PM	<b>Center for Disease Control (CDC)</b> .....	Kathy Mathews, MD
6:15–6:45 PM	<b>LIVING IT!</b> .....	Mary-Lou Weisman
6:45 PM	<b>Wrap Up and A Look at Tomorrow's Agenda</b> .....	Pat Furlong

**CHANGE IT!**  
SATURDAY, JULY 15<sup>TH</sup>

**Moving Research to New Levels**

7:15–8:30 AM	<i>Continental Breakfast</i>	
8:00–8:15 AM	<b>Overview of Research / The Pipeline</b> .....	Pat Furlong
8:20–8:40 AM	<b>Oligos—What is it, What Does It Mean For My Son, Where Are We Today?</b> .....	Dominick Wells, PhD
8:40–9:10 AM	<b>Chemistry of Oligos</b> .....	Steve Wilton, PhD
9:10–9:30 AM	<b>Clinical Aspects—Report on Single Muscle / Exon 51</b> .....	Kate Bushby, MD
9:30–9:45 AM	<b>Dystrophic Dog Studies Data</b> .....	Terry Partridge, PhD
9:45–10:15 AM	<i>Q &amp; A and Break</i>	
10:15–10:45 AM	<b>Therapeutics Overview Targets</b> .....	Lee Sweeney, PhD
10:45–11:20 AM	<b>PTC 124</b> .....	Langdon Miller, MD
11:20–11:40 AM	<b>IGF-1: Potential Existing Therapeutic Strategies</b> .....	Beth Barton, PhD
11:40–12:00 PM	<b>Myostatin: An Inhibitor of Muscle Growth with Clinical Applications to DMD</b> .....	Kathryn Wagner, MD, PhD
12:00–12:20 PM	<b>Anti-Inflammatory Agents</b> .....	Melissa Spencer, PhD
12:20–12:40 PM	<b>Antifibrotics and Nitric Oxide</b> .....	Andrew Hoey, PhD
12:40–1:30 PM	<i>Lunch Begins</i>	
1:00–1:30 PM	<i>Q &amp; A during lunch</i>	
1:40–2:00 PM	<b>Utrophin</b> .....	Kay Davies, PhD
2:00–2:20 PM	<b>P188</b> .....	Joseph Metzger, PhD
2:20–2:50 PM	<b>Status of Stem cells and DMD</b> .....	Terry Partridge, PhD
2:50–3:20 PM	<b>Status Viral Gene Therapy</b> .....	Kathy High, MD
3:20–3:40 PM	<b>MDX Mouse Clues—How They Adapt to Dystrophin Deficiency</b> .....	Brian Tseng, MD
3:40–4:00 PM	<i>Q &amp; A and Break</i>	
4:00–4:10 PM	<b>Industry and DMD</b> .....	Lee Sweeney, PhD
4:10–4:30 PM	<b>Prosensa (4:30–4:40 PM Q &amp; A)</b> .....	Gerard Platenburg, PhD
4:40–5:00 PM	<b>Ceptor (5:00–5:10 PM Q &amp; A)</b> .....	Terri Michele, MD
5:10–5:30 PM	<b>Initiation of the First Mini-dystrophin Clinical Trial for DMD:</b> <b>Asklepios BioPharmaceutical, Inc. (5:30–5:40 PM Q &amp; A)</b> .....	Scott McPhee, PhD
5:40–6:20 PM	<b>PTC Therapeutics—Project Catalyst Targets (6:20–6:30 PM Q &amp; A)</b> .....	Ellen Welch, PhD
6:30–7:00 PM	<i>Final Q &amp; A</i>	
7:45 PM	<i>Cocktails begin</i>	
8:15 PM	<i>Dinner</i>	

**SUNDAY,**  
JULY 16<sup>TH</sup>

8:00–10:00 AM	<i>Brunch</i> <i>This brunch is a great time for you to come and relax, connect with other families and process information before you leave.</i>
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