



## **Muscular Dystrophy Association**

*Workshop*

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### **Development Phase of The North American MD Clinical Trials Network**

June 11 & 12, 2004

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Westin La Paloma  
Tucson, Arizona

## ***WORKSHOP OBJECTIVE***

A well-designed, far-reaching network to conduct clinical trials in Duchenne and other muscular dystrophies is critical to advancing the development of the many new therapeutic strategies for these diseases. The establishment of such a clinical trial network will enhance the safety and efficiency of testing new therapies for muscular dystrophy and will permit greater opportunities for families to participate in clinical trials. This workshop will address issues relating to the development of such a clinical trials network.

**Development of the  
North American MD Clinical Trials Network**

Co-chairs: Susan T. Iannaccone, MD and Richard T. Moxley, MD

**THURSDAY, JUNE 10**

**6:00-7:00 pm**                    **OPENING RECEPTION**                    Cottonwood Room

**7:00-9:00 pm**                    **DINNER**

**FRIDAY, JUNE 11**

**7:15 - 8:00 am**                    **CONTINENTAL BREAKFAST**                    Terrace Foyer

**8:00-8:20 am**                    **Welcome and Overview of Workshop**                    Indigo Room  
- Susan Iannaccone, MD, Co-chair  
- Richard Moxley, MD, co-chair

**8:20-8:30 am**                    **DMD Networks and Databases**  
- Susan Iannaccone, MD - *Overview*

**8:30-8:40 am**                    - Richard Moxley, MD - *Introduction of Existing Databases/Data Networks*

**8:40-9:30 am**                    **CIDD: MDA Network to Study DMD**  
- Jerry Mendell, M.D. - *CIDD Structure and Major Findings*  
- Julaine Florence, PT, DPT; Wendy King, PT; Shree Pandya, PT, MS; Alan Pestronk, MD; Jerry Mendell, MD; John Kissel, MD and Richard Moxley, MD - *CIDD Challenges Related to Clinical Assessments, Data Management and Other Matters*  
- Discussion

**9:30-10:00 am**                    **CINRG: A Network to Study DMD**  
- Diana Escolar, MD - Structure and major findings  
- Eric Henricson, MPH; Diana Escolar, MD; ; Julaine Florence, PT, - Challenges  
- Discussion

**10:00-10:15 am**                    **BREAK**

**10:15-10:45 am**                    **MDSTARnet: A CDC-Funded Study of Epidemiology of DMD AND BMD**  
- Aileen Kenneson, PhD - Summary  
- Katherine Mathews, MD; Aileen Kenneson, PhD; Ed Brann, MD, MPH; Shree Pandya, PT, MS; Richard Moxley, MD - Challenges  
- Discussion

**10:45-11:00 am**                    **Utah Study**  
- Kevin Flanigan, MD - *Summary of NIH study of DMD*  
- Discussion

**11:00-11:15 am**                    **UC Davis Study**  
- Ted Abresch, MS - *Summary of NIH Study*  
- Discussion

**11:15-11:25 am**                    **Paul Wellstone Muscular Dystrophy Cooperative Research Centers (MDCRCs)**  
- Richard Lymn, PhD; Jerry Mendell, MD; Richard Moxley, MD- *Overview*  
- Discussion

**11:25-11:35 am**                    **Limb Girdle Muscular Dystrophy**  
- Jerry Mendell, MD - *Database and Therapeutic Trials*  
- Discussion

**11:35-11:50 am**                    **Muscle Study Group (MSG)**  
- John Kissel, MD; Wendy King, PT; Shree Pandya, PT; Jerry Mendell, MD; Richard Moxley, MD - *Overview of structure and projects*  
- Discussion

**11:50 am-12:05 pm**                    **Rare Diseases Network**  
- Giovanna Spinella- *Overview*  
- Discussion

**12:05-1:00 pm**                    **LUNCH**                    Verbena Room

**1:00-1:15 pm**                    **Overview of Challenges in Clinical Treatment of Duchenne Muscular Dystrophy**  
- Jerry Mendell, MD - *Challenges in the treatment of skeletal muscle manifestations*  
- Discussion

**1:15-1:30 pm**                    - Michael Sussman, MD - *Challenges in the management of musculoskeletal problems*  
- Discussion

**1:30-1:45 pm**                    - Jonathan Finder, MD; David Birnkrant, MD - *Challenges in the management of respiratory problems*  
- Discussion

**1:45-2:00 pm**                    - David Connuck, MD; Hugh Allen, MD; Jerry Mendell, MD, et al - *Challenges in the management of cardiomyopathy*  
- Discussion

**2:00-2:30 pm**                    **Industry Perspective**  
- Langdon Miller, MD (PTC Therapeutics); Sheila Mikhail (Asklepios); Cristina Csimma, MPH (Wyeth)  
- Discussion About and Review of Specific Questions by Asklepios, PTC Therapeutics and Wyeth

**2:30-2:45 pm**                    - Discussion

**FRIDAY, JUNE 11**

2:45-3:00 pm	<b>BREAK</b>	
3:00-5:00 pm	- Breakout Sessions 1. Natural History/Genetics 2. Medical Management 3. Therapeutics 4. Organization	
5:00-5:15 pm	<b>Natural History/Genetics Presentation</b>	
5:15-6:00 pm	- Discussion/Recommendations	
6:30 pm	<b>DINNER</b>	Verbena Room

**SATURDAY, JUNE 12**

7:15-8:00 am	<b>Continental Breakfast</b>	Terrace Foyer
8:00-8:15 am	<b>Medical Management Presentation</b>	
8:15-9:00 am	- Discussion/Recommendations	
9:00-9:15 am	<b>Therapeutics Group Presentation</b>	
9:15-10:00 am	- Discussion/Recommendations	
10:00-10:15 am	<b>BREAK</b>	
10:15-10:30 am	<b>Organization Group Presentation</b>	
10:30-11:00 am	- Discussion/Recommendations  <b>Plan of Action</b>	
11-11:15 am	- Group Co-Leaders- <i>Final Brief Summary of Recommendations</i>	
11:15-11:45 am	Discussion - <i>Next Steps</i>	
11:45-Noon	Discussion - <i>Future Meetings</i>	
NOON	<b>CLOSING LUNCHEON</b>	Verbena Room

## Participants

**Susan Iannacone, MD, Co-Chair**, University of Texas Southwestern Medical Center Scottish Rite Hospital, Dallas

**Richard Moxley, MD, Co-Chair**, University of Rochester Medical Center, Rochester, N.Y.

**Ted Abresch, MS**, Rehabilitation Research and Training Center in Neuromuscular Disease, Davis, CA

**Hugh Allen, MD**, Columbus Childrens Hospital, Columbus, OH

**David Birnkrant, MD**, Yale University, New Haven, CT

**Ed Brann, MD, MPH**, National Center on Birth Defects and Developmental Disabilities, Atlanta, GA

**Greg Carter, MD**, University of Washington. Seattle, WA

**Paula Clemens, MD**, University of Pittsburgh, Pittsburgh, PA

**David M. Connuck, MD**, University of Rochester, Rochester, N.Y.

**Christina Csimma**, Wyeth Pharmaceuticals, Collegeville, PA

**John Day, MD, PhD**, University of Minnesota, Minneapolis, MN

**Diana Escolar, MD**, Children's National Medical Center, Washington, DC

**Jonathan D. Finder, MD**, University of Pittsburgh, Pittsburgh, PA

**Kevin Flanigan, MD**, University of Utah, Salt Lake City, UT

**Julaine Florence PT, DPT**, Washington University, St. Louis, MO

**Michael Graves, MD**, University of California Los Angeles, Los Angeles, CA

**Jay J. Han, MD**, University of Washington, Seattle, WA

**Eric Henricson, MPH**, CINRG, Washington, DC

**R. Rodney Howell, MD**, Nat'l Institute for Child Health and Development, Bethesda, MD

**Aileen Kenneson, PhD**, Nat'l Center on Birth Defects and Developmental Disabilities, Atlanta, GA

**Wendy King, PT**, Ohio State University, Columbus, OH

**John T. Kissel, MD**, Ohio State University, Columbus, OH

**Robert Leshner, MD**, Children's Hospital, Richmond, VA

**Richard Lymn, PhD**, Nat'l Institute of Arthritis and Musculo-Skeletal and Skin Diseases, Bethesda, MD

**Katherine Mathews, MD**, University of Iowa, Iowa City, IA

**Jerry R. Mendell, MD**, Ohio State University, Columbus, OH

**Sheila Mikhail**, Asklepios, Chapel Hill, NC

**Langdon Miller**, PTC Therapeutics, South Plainfield, NJ

**Valerie Northcutt**, PTC Therapeutics, South Plainfield, NY

**Shree Pandya, MD**, University of Rochester, Rochester, NY

**Stuart Peltz, PhD**, PTC Therapeutics, South Plainfield, NJ

**Alan Pestronk, MD**, Washington University, St. Louis, MO

**Glenn Pierce, MD, PhD**, Avigen, Inc., Alameda, CA

**Melissa Spencer, PhD**, University of California Los Angeles, Los Angeles, CA

**Giovanna Spinella, MD**, Office of Rare Diseases, Bethesda, MD

**Lawrence Z. Stern, MD**, University of Arizona, Tucson, Arizona

**Michael Sussman, MD**, Shriners Hospital for Children, Portland, OR