



CENTER FOR DMD, RETREAT AGENDA, NOV. 21<sup>st</sup>, 2013

9 AM **WELCOME**  
Co-Directors of the Center for Duchenne Muscular Dystrophy

**PILOT AND FEASIBILITY AWARD RECIPIENTS (10 min talks + 5 min Q and A)**

9:10- 9:25 **Jonathan Wanagat, M.D., Ph.D.**  
*“Single Cell Protein Measurement by LCM-ICP-MS”*

9:25- 9:40 **Holden Wu, Ph.D.**  
*“Using MRI to Characterize Fibrofatty Infiltration of Cardiac Muscle in DMD”*

9:40- 9:55 **Loren Fong, Ph.D.**  
*“Modulation of LMNA Splicing – A Potential New Approach to Treat Hutchinson Gilford Progeria Syndrome”*

9:55- 10:10 **Nancy Halnon, M.D.**  
*“Cardiopulmonary Exercise Testing for Duchenne Muscular Dystrophy”*

**10:10- 10:40 Q and A and COFFEE BREAK**

**CDMD FACULTY TALKS (15 min talks)**

10:40- 10:55 **April Pyle, Ph.D.**  
*“Reprogramming and Differentiation of Human Pluripotent Stem Cells to Skeletal Muscle”*

10:55- 11:10 **M. Carrie Miceli, Ph.D.**  
*“Targeting Muscle Calcium Signaling to Optimize DMD Anti-Sense Oligonucleotides Directed DMD Exon Skipping”*

11:10-11:25 **Melissa Spencer, Ph.D.**  
*“Osteopontin Plays a Role in Macrophage Plasticity in Dystrophic and Injured Muscle”*

11:25-11:40 **Rachelle Crosbie- Watson, Ph.D.**  
*“Translational Approaches for Sarcospan Therapeutics”*

**CLINICAL TRIAL UPDATES Nancy Halnon, M.D. - Moderator**

11:45- 12:00 **Subha Raman, M.D.**  
*“Work in Progress: Aldosterone Antagonism for DMD Heart Muscle Disease”*

**12:00-1:00 LUNCH**

## AFTERNOON SESSION

### CLINICAL TRIAL UPDATES CONTINUED Nancy Halnon, M.D. - Moderator

- 1:00- 1:20            **Perry Shieh, M.D., Ph.D.** -*“Ongoing DMD Trials at UCLA”*
- 1:20- 1:40            **Eileen Fowler, Ph.D.** -*“Biomechanical Analyses of Gait As an Outcome For DMD Clinical Trials”*
- 1:40- 2:00            **Mike Nelson, Ph.D.** -*“Update on PDE5 Inhibitor Trials”*
- 2:00- 2:20            **Stan Nelson, M.D.** - *“New Genetic Approaches to Diagnosis and Discovery”*

### 2:20- 3:45            **COFFEE BREAK/POSTER SESSION**

- 3:45- 4:15            **Glenn Walter, Ph.D. (External Advisory Committee Member)**  
*“Emerging Magnetic Resonance Biomarkers for Muscular Dystrophy”*

- 4:15- 4:20            **Rachelle Crosbie-Watson, Ph.D.**  
CDMD Awards for Excellence in Research, and Introduction of B.A.D Associates

### 4:20-5:00            **PANEL DISCUSSION REGARDING MULTIDISCIPLINARY CARE FOR DMD**

Christine Banderas, MA, CCLS Child Life Specialist, CDMD Clinic  
Paul Degrassi, Respiratory Therapist  
Anna Haddal, M.D., Pediatric Endocrinology  
Nancy Halnon, M.D., Moderator, Pediatric Cardiology, CDMD Clinical Liaison  
Stan Nelson, M.D., Genetics, CDMD Co-Director  
William Oppenheim, M.D., Orthopaedic Surgery  
Perry Shieh, M.D., Ph.D., Director of Pediatric Neuromuscular Clinic  
Keith Vinneour, Orthotist  
Hillary Zebberman, M.S.W., Social Work, CDMD Clinic

### 5:00-6:00            **SOCIAL HOUR WITH COCKTAILS / SNACKS**



## **SPEAKERS**

- ❖ Rachele Crosbie-Watson, Ph.D., Professor, Department of Integrative Biology and Physiology at UCLA, Department of Neurology
- ❖ Loren Fong, Ph.D., Professor, Department of Medicine - Cardiology at UCLA
- ❖ Eileen Fowler, Ph.D., Professor, Department of Orthopedic Surgery at UCLA
- ❖ Nancy Halnon, M.D., Assistant Clinical Professor, Department of Pediatric Cardiology at UCLA
- ❖ M. Carrie Miceli, Ph.D., Professor, Department of Microbiology, Immunology, and Molecular Genetics at UCLA
- ❖ Mike Nelson, Ph.D., Post Doctoral Researcher, Cedars Sinai Heart Institute
- ❖ Stan Nelson, M.D., Professor and Vice Chair, Department of Human Genetics at UCLA
- ❖ April Pyle, Ph.D., Assistant Professor, Department of Microbiology, Immunology, and Molecular Genetics at UCLA
- ❖ Subha Raman, M.D., Professor, Ohio State University Division of Cardiovascular Medicine
- ❖ Perry Shieh, M.D., Ph.D., Assistant Clinical Professor, Department of Neurology
- ❖ Melissa Spencer, Ph.D., Professor, Department of Neurology at UCLA
- ❖ Glenn Walter, Ph.D., Associate Professor, Department of Physiology and Functional Genomics, University of Florida Cancer Center
- ❖ Jonathan Wanagat, M.D., Ph.D., Assistant Professor, Department of Medicine – Geriatrics
- ❖ Holden Wu, Ph.D., Assistant Adjunct Professor, Department of Radiology