



CENTER FOR DMD, RETREAT AGENDA, NOV. 29TH, 2012

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 **Daniel Ennis, Ph.D.**
“Cardiac MRI evaluation of pediatric subjects with Becker/Duchenne muscular dystrophy.”

9:25-9:40 **Gail Thomas, Ph.D.**
“Screening of NO-donating compounds to alleviate functional muscle ischemia in mdx mice.”

9:45-10:00 **Daniel Cruz M.D., Ph.D**
“Harnessing the microenvironment to instruct innate immune responses in Duchenne muscular dystrophy.”

CDMD FACULTY TALKS (15 min talks + 5 min Q and A)

10:00-10:20 **Melissa Spencer, Ph.D.**
“Abnormal growth signaling pathways in limb girdle muscular dystrophy type 2A.”

10:20-10:35 Q and A and then COFFEE BREAK

10:35-10:55 **April Pyle, Ph.D.**
“Development and characterization of skeletal muscle cells derived from DMD-hiPSCs.”

10:55-11:15 **Linda Baum, M.D., Ph.D.**
“Characterization of the human skeletal muscle cell glycome and identification of glycan-related functions in membrane stabilization and extracellular matrix interactions.”

11:15-11:35 **Rachelle Crosbie-Watson, Ph.D.**
“Sarcospan: A small protein with big potential for Duchenne muscular dystrophy.”

11:35-11:55 **Stanley Nelson, M.D.**
“Genetic diagnostics and modifier genes in Duchenne muscular dystrophy.”

12:00-1:00 LUNCH

AFTERNOON SESSION

1:00-1:20 **Carrie Miceli, Ph.D.**
“Dantrolene enhances antisense-mediated exon skipping in human and mouse models of Duchenne muscular dystrophy.”

1:20-1:40 **Jeffrey Chamberlain Ph.D.** (External Advisory Committee Member) University of Washington.
“AAV vectors for gene therapy of muscular dystrophy.”

HUMAN SUBJECTS/CLINICAL TRIAL UPDATES

1:45-1:55 **Eileen Fowler, Ph.D.**-*“Update on Natural History Studies”*

1:55-2:05 **Ron Victor M.D.**-*“Update on PDE inhibitor Trials”*

2:05-2:15 **Glenn Walter, Ph.D.** *“Update on Imaging DMD”*

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

3:45-4:00 **Rachelle Crosbie-Watson, Ph.D.**
“Promoting DMD awareness through undergraduate science education.”

4:10-5:00 PANEL DISCUSSION REGARDING MULTIDISCIPLINARY CARE FOR DMD

- Nancy Halnon M.D., Moderator, Pediatric Cardiology
- Stan Nelson M.D., Genetics
- Dyan Salinas, RN Nurse, CDMD Clinic
- Perry Shieh M.D., Ph.D., Neuromuscular
- Jennifer Wallace-Hernandez, Physical Therapy
- Marlyn Woo, M.D., Pulmonary Medicine
- JJ Zaritsky, M.D., Orthopaedics
- Hillary Zebberman MSW Social work, CDMD Clinic

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

