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**