



**MUSCLE CELL BIOLOGY AND DISEASE ANNUAL
SCIENTIFIC RETREAT**

**AGENDA
NOV. 21st, 2014**



9:30 AM Registration and Coffee

10 AM **WELCOME** M. Carrie Miceli, Ph.D., Stanley Nelson, M.D., Melissa Spencer, Ph.D., Co-Directors of the Center for Duchenne Muscular Dystrophy at UCLA

MORNING SESSION: AWARD RECIPIENTS AND TRAINEE SEMINARS

10:05- 10:20 Courtney Young, Graduate Deans Scholar Award, NSF Predoctoral Fellow Award *"CRISPR/Cas9 Mediated Gene Editing to Restore the Reading Frame in Duchenne Muscular Dystrophy"* Mentor: Melissa Spencer, Ph.D.

10:20- 10:35 Michelle Parvatiyar, Ph.D., CureDuchenne Postdoctoral Fellow Award *"The Role of Sarcospan in the Cardiac Hypertrophic Response"* Mentor: Rachelle Crosbie-Watson, Ph.D.

10:35- 10:50 Brian McMorran, *"Unique and Specific Changes in Cell Surface Glycosylation During Myotube Differentiation"* Mentor: Linda Baum, M.D., Ph.D.

10:50- 11:05 Richard Wang, Ph.D., *"Wisdom from the Crowds: Inferences and Discoveries from the DuchenneConnect Registry"* Mentor: Stanley Nelson, M.D.

11:05- 11:20 Zhe Wang, *"Quantitative Left Ventricular Rotational Mechanics in Duchenne and Becker Muscular Dystrophy Patients"* Mentor: Daniel Ennis, Ph.D.

11:20- 1:00 POSTER SESSION AND LUNCH

AFTERNOON SESSION: KEYNOTE SPEAKER AND FACULTY SEMINARS

1:00- 1:40 **Jeffrey Chamberlain, Ph.D., *"Development of Gene Therapy Approaches for DMD/BMD"* McCaw Endowed Chair in Muscular Dystrophy, University of Washington School of Medicine**

1:40- 2:10 M. Carrie Miceli, Ph.D. *"How Does the CDMD Facilitate Translational Research at UCLA and Nationwide"*

2:10- 2:30 April Pyle, Ph.D. *"Human Pluripotent Stem Cell Approaches to DMD"*

2:30- 2:50 Linda Baum, M.D., Ph.D., *"Understanding the Role of Cell Surface Glycosylation in Muscle Cell Adhesion"*

2:50- 3:10 Melissa Spencer, Ph.D., *"Targeting Immunity and Inflammation to Combat Fibrosis and Improve Tolerance"*

3:10- 3:30 Rachelle Crosbie-Watson, Ph.D., *"Training the Next Generation of Muscular Dystrophy Scientists"*

3:30- 3:45 COFFEE BREAK

3:45- 4:00 Nancy Halnon, M.D., *"Introduction of Pediatric Neuromuscular Clinic Team and Update on Findings From the Eplerenone for Sub Clinical Cardiomyopathy in DMD Trial"*

4:00- 4:25 Stanley Nelson, M.D., *"New Diagnostic Approaches and Clinical Trial Pipeline for Duchenne"*

4:25- 5:00 Rachelle Crosbie-Watson, Ph.D., *"Poster Finalists Present Elevator Pitch and Receive Awards"*

5:00-6:00 SOCIAL HOUR WITH COCKTAILS AND APPETIZERS