



MUSCLE CELL BIOLOGY AND DISEASE ANNUAL SCIENTIFIC RETREAT
February 18, 2016

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

10:10- 10:30 Team Science Award 1 Andrea Hevener, Ph.D. and Karen Reue, Ph.D. “The Impact of Mitochondrial Fission-Fusion Dynamics on Muscle Metabolism.”

10:35- 10:55 Team Science Award 2 Bridgette Gomperts, Ph.D. and April Pyle Ph.D. “Modeling the Muscle Cell Niche in Duchenne Muscular Dystrophy Using Human Induced Pluripotent Stem Cells”

11:00- 11:10 Michael Hicks, Ph.D., CDMD-CureDuchenne Fellowship Award (Postdoc) “NCAM Progenitors Derived from Human Pluripotent Stem Cells (hPSCs) are not Developmentally Equivalent to Fetal or Adult Muscle Satellite Cells”

11:15-11:25 Kristen Stearns-Reider, Ph.D. (Postdoc) “Effects of Dystrophin Loss on the Biophysical Properties of Skeletal Muscle ECM”

11:30-11:40 Haibin Xi, Ph.D. (Postdoc) “Generation of Functional Somite Cells from Human Pluripotent Stem Cells for Deriving Skeletal Muscle Progenitor Cells”

11:45- 12:45 LUNCH

AFTERNOON SESSION: KEYNOTE SPEAKERS, TRAINEE SEMINARS, AND CLINICAL UPDATES

12:50- 1:10 **Carl Morris, Ph.D., Solid Biosciences, VP Research & Development “Drug Development in Duchenne Muscular Dystrophy”**

1:15- 1:35 **Glenn Walter, Ph.D. Professor, University of Florida “NMR at the Crossroads of Biomarker Discovery for DMD.” CDMD External Advisory Committee Member**

1:45-1:55 Derek Wang (Ph.D. Graduate Student) “Long-Term Therapy to Enhance Antisense Mediated Exon Skipping for DMD in the *mdx* Mouse”

2:00- 2:10 Andrew Jensen, M.D. (Resident, Orthopaedic Surgery) “The Role of PDGFR α + and PDGFR β + Cell Populations in Remodeling of the Rotator Cuff after Irreversible Injury in a Murine Model”

2:15- 2:25 Joana Capote (Ph.D. Graduate Student) “Osteopontin Ablation Ameliorates Muscular Dystrophy by Skewing Macrophage Polarization Towards a Pro-Regenerative State”

2:30-4:00 **Poster Session/Coffee Break**

4:00-4:10 Courtney Young, (Ph.D. Graduate Student) “A CRISPR/Cas9 Platform that Targets the Majority of DMD Patients Restores Dystrophin Function in hiPSC-Derived Muscle Cells”

4:20-4:40

Perry Shieh, M.D., Ph.D. "Update on Clinical Trials"

4:50- 5:15

Rachelle Crosbie-Watson, Ph.D., Education Liaison "Poster Finalists Present Elevator Pitch and Receive Awards"

5:15-6:15

SOCIAL HOUR WITH COCKTAILS AND APPETIZERS



Thank you for attending the Muscle Cell Biology and Disease Annual Scientific Retreat!