



**MUSCLE CELL BIOLOGY AND DISEASE ANNUAL SCIENTIFIC RETREAT**  
**Tuesday November 29<sup>th</sup> 9:30AM- 6:30PM**  
**Luskin Conference Center, Optimist Room**

9:00-9:50 Registration and Coffee

**MORNING SESSION**

*Host: Michelle Parvatiyar, Ph.D.*

*Moderators: Elizabeth Gibbs Ph.D, Cynthia Shu, Haibin Xi, Ph.D. Michael Hicks, Ph.D.*

9:50-10:00 Welcome Rachele Crosbie-Watson

10:05-10:15 Austin Nakano, Ph.D. Pilot and Feasibility Awardee  
*“Developing iPSC-derived Cardiomyocyte as a Model System for Evaluating Therapeutic Intervention of DMD Cardiomyopathy”*

10:20- 10:30 Shantanu Joshi, Ph.D. Pilot and Feasibility Awardee  
*“Brain Imaging Signatures in Duchenne Muscular Dystrophy and their Correlates With Cognitive Function”*

10:35- 10:45 Sandra Loo, Ph.D. Pilot and Feasibility Awardee  
*“Behavioral, Cognitive, and Neurophysiological Phenotypes in Duchenne Muscular Dystrophy”*

10:50-11:00 Florian Barthelemy, Ph.D., Post-Doctoral Fellow  
*“Can we Promote Exon Skipping in DMD Patient Cells Using Calcium Modulators?”*

11:10-11:40 Armando Villalta, Ph.D., Assistant Professor, University of California, Irvine  
*“Immune Regulation in Muscular Dystrophy”*

11:45- 12:15 Jeff Chamberlain, Ph.D., External Advisory Committee Member, Professor, University of Washington  
*“Approaches for Gene Therapy of DMD”*

**12:25-1:25 LUNCH**

**AFTERNOON SESSION**

*Host: Kristen Stearns-Reider, PT, Ph.D.*

*Moderators: Brian McMorran, Derek Wang, Courtney Young, Florian Barthelemy, Ph.D.*

1:25-1:50 Roy Doumani, JD, Executive Director, Business of Science Center UCLA  
*“ Exploring Entrepreneurship”*

- 1:55- 2:25 Ellen Welch, Ph.D., Head, Genetic Disorders, PTC Therapeutics  
*"Making Sense of Nonsense: Ataluran for the Treatment of Genetic Disorders Caused by Nonsense Mutations"*
- 2:30-2:50 Rachelle Crosbie-Watson, Ph.D.-CDMD Education Liaison and T32 Director  
*"Muscle UP: A comprehensive Training Program for Undergraduates, Pre-doc and Postdoctoral Fellows"*
- 3:00- 3:10 Ayelet Dar Ph.D. Post-doctoral Fellow  
*"Distinct Subsets of PDGFR $\beta$ + Progenitor Cells Promote Sequential Myogenic and Degenerative Remodeling after Chronic Muscle Injury"*
- 3:15- 3:25 Matthew Hakimi, M.D.  
*"Exercise-induced Vasospasm in Skeletal Muscle of Patients with Becker Muscular Dystrophy Lacking Sarcolemmal nNOS"*
- 3:30- 3:40 Patrick Magrath, Pre-doctoral Student  
*"Towards MRI-Based Quantification of Cardiomyocyte Performance in Duchenne Muscular Dystrophy"*
- 3:45- 5:00 **Poster Session/Judging/ Coffee Break**
- 5:00-5:05 Courtney Young and Cynthia Shu  
*"Bruin Allies for Duchenne Update"*
- 5:10-5:20 **Poster Awards**
- 5:20-6:30 **SOCIAL HOUR WITH COCKTAILS AND APPETIZERS**

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