



## CENTER FOR DMD RETREAT AGENDA, SEPT 16<sup>TH</sup>, 2010

### 9 AM **Welcome**

from the Directors Melissa Spencer, Carrie Miceli and Stan Nelson

### **Presentations by External advisory committee members**

9:10-9:40 Grace Pavlath, Ph.D. Professor of Pharmacology at Emory University: *"Potential Mechanisms of Muscle Pathology in Oculopharyngeal Muscular Dystrophy"*

9:50- 10:20 Glenn Walter, Ph.D. Professor of Physiology at The University of Florida: *"Looking Inside Dystrophic Muscle"*.

### **Core Director Presentations**

10:30-10:40 Core B Highthroughput Screening Core, PI Carrie Miceli

10:40-10:50 Core C Mouse Phenotyping and Imaging Core, PI Melissa Spencer

10:50-11:00 Core D: Bioinformatics Core, PI Stanley Nelson

11:00-11:15 Coffee Break

### **11:15-12:00 Trainee Presentations**

11:00-11:15- Genevieve Kendall,(collaboration between Miceli and Nelson Labs)  
*"High Throughput Screening Assays for the Identification of Small Molecules that Modulate Exon Skipping on the DMD Gene"*

11:20-11:35- Ekaterina Mohkonova (collaboration between Miceli, Nelson and Spencer Labs)  
*"In Vivo Testing of Small Molecules That Modulate Exon Skipping on the DMD Gene"*

11:40-11:55- Lily Chao (Totonoz Lab)  
*"The role of Nur77 in muscle metabolism and regeneration"*

12:00-1;00 **lunch and poster session**

### **Faculty Presentations**

1:00-1:20 April Pyle  
*"Development of Human Induced Pluripotent Stem Cells and Skeletal Muscle Derivatives as a Screening and Therapeutic Assessment Tool for Duchenne Muscular Dystrophy"*

1:25-1:45 Julio Vergara  
*"Characterization of the chloride conductance in mammalian skeletal muscle fibers"*

1:50-2:10 Eileen Fowler

*"Clinical outcome measurements in Duchenne muscular dystrophy"*

2:15-2:35-coffee break

2:40-3:00 Robb MacClellen

*"Epigenetics of muscle growth: Implications for Regeneration"*

3:05-3:25 Linda Baum

*"Novel approaches to regulating muscle cell glycosylation and function"*

3:30-3:50 Johan Holmberg (collaboration between Rachelle Crosbie and Linda Baum labs)

*"Small molecule high throughput screens for muscular dystrophy: In vivo evaluation of lead compounds"*

4:00-4:20 wine, beer and light snacks

4:30-4:50 Thomas Vondriska

*"How the Proteome Impacts the Genome, Causing the Heart to Fail"*

4:55-5:15 Yibin Wang

*"Novel Protein Phosphatases in Stress-Signal Regulation in Heart Muscle"*

5:20-5:40 Erik Anderson (Doug Black lab)

*"Modulation of SMN2 alternative pre-mRNA splicing by small molecule compounds"*

5:40-6:00 Ron Victor

*"NO control of blood flow in normal and DMD skeletal muscle"*

**THANK YOU FOR ATTENDING THE CDMD RETREAT. DRIVE SAFELY!**