



CENTER FOR DMD RETREAT AGENDA, SEPT 15TH, 2011

- 9 AM **Welcome**
Carrie Miceli, Ph.D. Co-director of the Center for Duchenne Muscular Dystrophy
- Pilot and Feasibility Award Recipients**
- 9:10-9:30 April Pyle, Ph.D. *Development of ips cells as pre-clinical screening tools for DMD.*
- 9:30-9:50 Eileen Fowler, Ph.D. *Outcome measures for clinical trials in DMD.*
- 9:50-10:10 Melissa Spencer, Ph.D. *Osteopontin as a Therapeutic Target for DMD.*
- 10:10-10:25 COFFEE BREAK
- Faculty Presentations**
- 10:30-10:50 Robert Damoiseaux Ph.D *Use of the Molecular Shared Screening Resource for HTS screening.*
- 10:50-11:10 Jonathan Wanagut M.D., Ph.D *Mitochondrial genetics and sarcopenia.*
- 11:10-11:30 Gail Thomas Ph.D. *Nitric Oxide regulation of muscle blood flow*
- 11:30-12:05 **Jeff Chamberlain Ph.D, External Advisory Committee Member**
University of Washington, Seattle
Viral Vector Mediated Gene Therapy for DMD.
- 12:05-1:00 Lunch**
- 1:00-1:20- Ron Victor M.D. *Clinical Trial of Sildenafil/Tadalafil in DMD and BMD.*
- Trainee Presentations**
- 1:25-1:40- Jamie Marshall *Compensatory Upregulation of Cytoskeletal Proteins in DMD.*
- 1:45-2:00- Genevieve Kendall *Identification of Enhancers of Exon Skipping for DMD.*
- Faculty Presentations**
- 2:00-2:20 Carmen Bertoni, Ph.D. *Gene Editing of the Dystrophin Gene for the Treatment of DMD Using Single-Stranded Oligodeoxynucleotides (ssODNs)*
- 2:20-2:40 COFFEE BREAK
- 2:45-3:05 Stan Nelson M.D. *Identification of Genetic Modifiers in DMD.*
- 3:10-3:45 **Kathryn Wagner M.D. Ph.D. (External Advisory Committee Member)**
Director, The Kennedy Krieger Institute, The Johns Hopkins School of Medicine.
Xenografts as animal models of muscular dystrophy.
- 3:50-4:20 **Panel Discussion regarding multidisciplinary care for DMD**
*Stan Nelson, MD (moderator),
Kathryn Wagner, M.D., Ph.D., Eileen Fowler, P.T., Ph.D. Nancy Halnon, M.D., Mariam Ischander, M.D., William Oppenheim, M.D., Ronald Victor, M.D., Hillary Zebberman, M.S.W.*
- 4:20-5:20 SOCIAL HOUR WITH COCKTAILS/SNACKS