



**Congenital Muscular Dystrophy
Information Day
The Children's Hospital of Philadelphia
Abramson Research Building, Room 123
Saturday, July 23rd, 2011**

2011 Congenital Muscular Dystrophy Family and Professional Conference – July 23, 2011

The Children's Hospital of Philadelphia

Neuromuscular Program

Division of Neurology

CME Course Credits: 6.5 AMA PRA Category 1 Credits™

Overview

Congenital muscular dystrophies (CMDs) are a genetically and clinically complex group of disorders that, together however, contribute significantly to early childhood neuromuscular morbidity. There has been significant progress in the last few years in the identification of new genes and pathophysiological mechanisms. This new knowledge allows for better diagnosis, but also increases the complexity of the diagnostic process. At the same time, new therapeutic avenues are explored and the first clinical trial will soon start in this group of conditions. Very recently, a group of international experts (including the course director, Carsten Bonnemann, M.D.) has worked out and published a set of standards of care for this group of disorders, as well as a catalog of diagnostic guidelines and algorithms. There is a wealth of new information to pass on to the medical community. This one-day educational conference will enhance participant's skills in recognizing patients with these conditions, allow them to arrive at a proper diagnosis and significantly improve their medical management skills when dealing with patients affected by such a condition. In addition, the activity will provide knowledge about emerging treatments, advances and clinical trials in the study of congenital muscular dystrophy.

Target Audience

This conference is intended to enhance the knowledge and clinical practice skills of pediatricians and primary care providers, practitioners in Developmental Pediatrics, pediatric neurologists, neurologists, PMR physicians, pulmonologists, nurse practitioners, nurses, and physical, occupational and speech therapists.

Learning Objectives

Upon completion of this conference, participants will be able to:

1. Recognize and list the five (5) main subtypes of congenital muscular dystrophy: collagen VI myopathy, LAMA2def CMD, alpha dystroglycanopathies, SEPN1 related myopathy and LMNA related-CMD.
2. Discuss the key drivers of morbidity and mortality and key elements to providing optimal medical management.
3. Identify key therapeutic targets in the CMDs based upon underlying genetic mutations and drivers of disease pathogenesis.

Accreditation Statement

The Children's Hospital of Philadelphia is accredited by the Accreditation Council for Continuing Medical Education (ACCME) to provide continuing education for physicians.

AMA Credit Designation Statement

The Children's Hospital of Philadelphia designates this live activity for a maximum of **6.5 AMA PRA Category 1 Credit(s)™**. Physicians should claim only the credit commensurate with the extent of their participation in the activity.

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Agenda

8:00-8:30AM	Breakfast, Registration and Introduction			
8:30 a.m.	CMD Therapeutic Targets	Carsten Bonnemann, M.D.		
9:10 a.m.	Key Note Lecture: Developing Therapies for the CMDs- Perspectives from the Lab Bench	Mahasweta Girgenrath, Ph.D.		
9:40 a.m.	CMD Clinical Trials	Anne Rutkowski, M.D.		
10:10 a.m.	Q &A: CMD Therapy Panel	Moderator: Eunice Kim, M.B.A. Carsten Bonnemann, M.D., Mahasweta Girgenrath, Ph.D., Anne Rutkowski, M.D.		
10:45 a.m.	Break			
11:00 a.m.	Key Note Lecture: Developing Therapies in CMD- the Biotech Sector and Rare Disease	Jasbir S. Seehra, Ph.D.		
11:30 a.m.	CMD Medical Management Overview	Anne Rutkowski, M.D.		
12:00 Noon	Lunch, Group Photo			
1:00 p.m.	CMD Clinical Best Practices	Meganne Leach, M.S.N., CRNP		
1:15p.m.	CMD Diagnostic Overview	Carsten Bonnemann, M.D.		
2:00 p.m.	Ultrasound Teaching Course: Using Ultrasound to Guide Congenital Muscular Dystrophy Diagnosis	Carsten Bonnemann, M.D.		
3:00 p.m. Breakout Session #1	Perspectives for Newly Diagnosed Families Panel: A. Rutkowski, C. Bonnemann, D. Hoban-Smith, S. Sklaroff-Van Hook	Nutrition and CMD: Tubes, Supplements, Dehydration and Constipation Presenter: D. DiVito	Breathing and CMD: How Do We Track How the Lungs are Doing? Presenters: H. Sawnani, H. Mayer	CMDIR and CMD BioBank: Purpose, Function and Link to CMDIR Presenters: D. Berlin, A. Merton
3:30 p.m.	Break			
3:45 p.m. Breakout Session #2	CMD Care Guidelines and Medical Management, Q &A: C. Bonnemann, D. Hoban-Smith, S. Sklaroff-Van Hook, A. Leach	Cognitively Disabled CMD Experiences: Moderator: A. Huntington	CMDIR and CMD BioBank: Purpose, Function Presenters: D. Berlin, A. Merton	Transition to Adulthood in Pulmonary Care: Presenters: H. Mayer, H. Sawnani
4:15 p.m.	Concluding Remarks	Carsten Bonnemann, M.D.		
4:30 p.m.	Adjourn			

Course Directors

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