

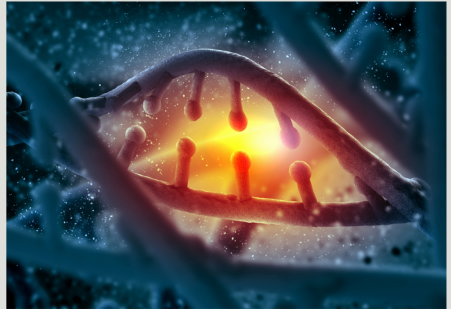
Molybdenum Cofactor Deficiency (MoCD): Recognition, Challenges, and Treatment

A CE-accredited lecture on MoCD for physicians and nurses

LEARNING OBJECTIVES

At the end of this educational event, participants will be able to:

- Describe MoCD as an autosomal recessive metabolic disorder and explain the function of enzymes whose activities are lost
- State the consequences of loss of Molybdenum Cofactor (MoCo)-dependent enzyme activities
- Explain the genetics of MoCD
- Recognize the presenting symptoms of MoCD, and be aware of the most common misdiagnoses
- Know the specific tests required to provide an accurate differential diagnosis of MoCD
- Describe new treatment options for patients with MoCD
- Recognize the importance of early diagnosis and intervention in MoCD, including the possibility of prenatal diagnosis



Agenda:

- 6:15 – 6:45am Breakfast*
- 6:45 – 7:00am Background and Overview of MoCD and Development of cPMP – Guenter Schwarz, PhD
- 7:00 – 7:15am Treatment with cPMP Present and Future – Alex Veldman, MD
- 7:15 – 7:30am Challenges of Urgent Treatment of MoCD – Judy Aschner, MD
- 7:30 – 7:45am Panel Discussion

Supported by an educational grant from
Alexion Pharmaceuticals

ALEXION

Tuesday, December 10, 2013
Empire Ballroom - 6:15am
Omni Shoreham Hotel, Washington, DC

PROGRAM OVERVIEW

Molybdenum cofactor deficiency (MoCD) is a rare, inherited metabolic disorder. In patients with this condition, the metabolic pathway for production of MoCo is disrupted, resulting in the simultaneous loss of all MoCo-dependent enzyme activities.

Patients usually present as neonates, with failure to thrive, refractory seizures, brain dysmorphologies, axial hypotonia, limb hypertonia, and feeding difficulties. Neuronal damage is severe and rapidly progressive as a result of accumulation of toxic levels of sulfite and related metabolites (i.e. S-sulfocysteine) in the brain.

Up until now, treatment options for MoCD have only been supportive, with most affected patients dying in early infancy. Cyclic pyranopterin monophosphate (cPMP) has shown



promise in an animal model, and has been recently used in human infants to reverse the metabolic and biochemical abnormalities with dramatic clinical improvement in some infants.

The possibility of intervening early in the disease process (ideally in the first hours or days of life) to modify patient outcomes increases the importance of early, accurate identification of MoCD by neonatologists.

CONTINUING EDUCATION

Physicians. The Meniscus Educational Institute designates this live activity for a maximum of *1.0 AMA PRA Category 1 Credits™*. Physicians should claim only the credit commensurate with the extent of their participation in the activity.

Nurses. This activity for 1.0 contact hour is provided by the Meniscus Educational Institute. The Meniscus Educational Institute is accredited as a provider of continuing nursing education by the American Nurses Credentialing Center's Commission on Accreditation. Provider approved by the California Board of Registered Nursing, Provider No. 13164, for 1.0 contact hour.

To successfully complete this activity and receive a credit certificate, participants are required to complete and submit the evaluation form. Statement of credit will be provided via e-mail within 3 weeks of completion date. There is no fee for participating in this activity.

To pre-register for this program please log onto www.NICUniversity.org



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*Continental breakfast available for first 250 to arrive.