SUNDAY, OCTOBER 28, 2018 • NEW YORK, NY

COMPLIMENTARY CME SYMPOSIUM

Challenges in Disease Management for Type 1 Gaucher Disease

JOINTLY PROVIDED BY:

National Gaucher Foundation

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Panel Moderator

John D. Loike, PhD
Professor of Biology, Touro College and University System; Adjunct Professor, Columbia University College of Physicians and Surgeons and Columbia College
New York, NY
Keynote Speaker

LEARNING OBJECTIVES

Upon completion, participants should be able to:

1. Review updates to the recommendations for screening for GD
2. Identify patients at risk of GD through genetic counseling
3. Determine appropriate timing of genetic testing for GD
4. Differentiate clinical symptoms of GD1 over other subtypes
5. Evaluate changes in symptoms over the course of GD1
6. List the diagnostic tools critical for identification of GD1 symptoms
7. Identify early and late symptoms associated with GD1
8. Understand the implications of early treatment intervention for GD1 in children and adults
9. Describe outcomes of the latest clinical trials on early treatment initiation for GD1 in children and adults
10. Determine appropriate timing as to when to initiate GD1 therapy based on disease progression
11. Discuss the importance of newborn screening for GD
12. Implement steps to discuss with patients who are planning on having a baby
13. Discuss key points of GD1 in pregnancy
14. Develop a plan for parents with a newborn diagnosed with GD1
15. Assess cases for children and adults with GD1

Sunday, October 28, 2018
10:00 am - 4:00 pm

Registration and light breakfast
begin at 9:00 am

THE MEZZANINE HOTEL
55 Broadway
New York, NY 10006

AGENDA

- **Disease Management of GD1 in Adults:** *Determining Criteria for Initiation of Treatment*
- **Disease Management of GD1 in Children:** *Diagnosis, post-diagnosis Observation & Criteria for Initiation of Treatment*
- **Prevention of Late Onset Complications with Early initiation of Treatment in GD1:** *Examining Pre-Clinical and Clinical Evidence*
- **Gaucher Disease Management and Pregnancy**
- **Panel Discussion:** *Science, Benefits, Drawbacks and Ethics of Newborn Screening for GD (Moderated)*
- **Panel Discussion:** *Case Presentations of Challenging management problems in patients with GD1*

Keynote Address by Professor John D. Loike: Ethical and Social Questions About Screening and Pre-Implantation Genetic Diagnosis (PGD) as Related to the Special Sensitivities and Practices of Orthodox Jewry

CME Information

Statement of Need
Gaucher disease (GD) is one of the most common lysosomal storage disorders with an estimated incidence of 1:40,000-60,000 in the general population with the highest frequency seen among the Ashkenazi Jewish population (1:800).

The presentation of Gaucher's disease (GD) is extremely variable, with type 1 (GD1) being the most prevalent. Patients may present as early as infancy to as late as the eighth decade of life. In addition, there is substantial variability in organ involvement and disease progression. In general, patients with GD1 can present with hepatomegaly, splenomegaly, anemia, thrombocytopenia, and bone disease.

Once a GD1 diagnosis is suspected, confirmation is done through genetic testing. Due to GD1's high degree of clinical variability, it is difficult to predict disease progression and even more challenging to determine when to initiate treatment. Therefore, GD1 management requires an individually tailored multidisciplinary evaluation and monitoring. It is also important to counsel patients who have GD or are carriers about family planning.

This CME accredited session will provide expert insight into GD1 management including diagnosis, monitoring GD1 progression, early treatment initiation, counseling patients on family planning and the importance of prenatal and newborn screening. Clinicians will improve their knowledge of the variable presentation of GD1 in children, adolescents and adults in order to facilitate earlier diagnosis and determine appropriate management. Clinicians will understand that importance of genetic testing, prenatal and newborn screening. Clinicians will recognize the importance of counseling patients with GD1 who are planning on starting a family. Finally, expert faculty will discuss GD1 cases studies in children, adolescents, and adults.

Activity Overview
This live meeting will improve clinicians knowledge of the variable presentation of GD1 in children, adolescents and adults in order to facilitate earlier diagnosis and determine appropriate management. Clinicians will also discuss genetic testing and prenatal and newborn screening and review the importance of counseling patients with GD1 that are planning to start a family. Finally, expert faculty will discuss GD1 cases studies in children, adolescents, and adults.

Target Audience
This activity is intended for clinicians that care for patients with Gaucher disease in Gaucher specialized treatment centers, including primary care physicians, internists, gastroenterologists, neurologists, hematologists, hepatologists, geneticists, oncologists, orthopedists, pain specialists, cardiologists, pulmonologists, obstetricians/gynecologists, and pediatricians.

Accreditation/Designation Statements
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Participants must complete the evaluation and attestation within 60 days of participation.

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Complete your evaluations and click on the "Get Certificate" button to download your certificate!

Estimated Time to Complete This Activity: 4.5 hours

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