Challenges in Disease Management for Type 1 Gaucher Disease
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 the 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
This activity has been planned and implemented in accordance with the accreditation requirements and policies of the Accreditation Council for Continuing Medical Education (ACCME) through the joint providership of Med-IQ and National Gaucher Foundation. Med-IQ is accredited by the ACCME to provide continuing medical education for physicians.

Physicians: Med-IQ is accredited by the Accreditation Council for Continuing Medical Education (ACCME) to provide continuing medical education for physicians.

Med-IQ designates this live activity for a maximum of 4.5 AMA PRA Category 1 Credit™. Physicians should claim only the credit commensurate with the extent of their participation in the activity.

Instructions to Receive Credit
To receive credit, read the introductory CME material, attend the meeting, and complete the evaluation and attestation online.

Participants must complete the evaluation and attestation within 60 days of participation.

To evaluate this activity, claim credit, and download your certificate, please visit: www.Med-IQ.com/a1162 and click on the “Get Credit” button. Log in using your Med-IQ username and password or create a new account. When prompted, choose your workshop location and use this access code: GAU-18

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

Estimated Time to Complete This Activity: 4.5 hours

Disclosure Policy
Med-IQ requires any person in a position to control the content of an educational activity to disclose all relevant financial relationships with any commercial interest. The ACCME defines “relevant financial relationships” as those in any amount occurring within the past 12 months, including those of a spouse/life partner, that could create a conflict of interest (COI). Individuals who refuse to disclose will not be permitted to contribute to this CME activity in any way. Med-IQ has policies in place that will identify and resolve COIs prior to this educational activity. Med-IQ also requires faculty to disclose discussions of investigational products or unlabeled/unapproved uses of drugs or devices regulated by the US Food and Drug Administration.
Disclosure Statement
The content of this activity has been peer reviewed and has been approved for compliance. The faculty and contributors have indicated the following financial relationships, which have been resolved through an established COI resolution process, and have stated that these reported relationships will not have any impact on their ability to give an unbiased presentation.

Manisha Balwani, MD: Consulting fees/advisory boards: Sanofi Genzyme
Debra-Lynn Day-Salvatore, MD, PhD, FAAP, FACMG: Consulting fees/advisory boards: Sanofi Genzyme, Shire; Fees received for promotional/non-CME activities: Sanofi Genzyme, Shire; Contracted research: Sanofi Genzyme
Ozlem Goker-Alpan, MD: has indicated no real or apparent conflicts.
Pradod K. Mistry, MD, PhD, FRCP: Consulting fees/advisory boards: Sanofi Genzyme; Fees received for promotional/non-CME activities: Sanofi Genzyme; Contracted research: Sanofi Genzyme
Ellen Sidransky, MD: has indicated no real or apparent conflicts.
Neal J. Weinreb, MD: Consulting fees/advisory boards: Aeglea BioTherapeutics, Inc., Alexion Pharmaceuticals, Pfizer, Inc., Sanofi Genzyme; Fees received for promotional/non-CME activities: Shire

The peer reviewers and activity planners have no financial relationships to disclose.

Statement of Evidence-Based Content
Educational activities that assist physicians in carrying out their professional responsibilities more effectively and efficiently are consistent with the ACCME definition of continuing medical education (CME). As an ACCME-accredited provider of CME, it is the policy of Med-IQ to review and ensure that all the content and any recommendations, treatments, and manners of practicing medicine in CME activities are scientifically based, valid, and relevant to the practice of medicine. Med-IQ is responsible for validating the content of the CME activities it provides. Specifically, (1) all recommendations addressing the medical care of patients must be based on evidence that is scientifically sound and recognized as such within the profession; (2) all scientific research referred to, reported, or used in CME in support or justification of a patient care recommendation must conform to generally accepted standards of experimental design, data collection, and analysis.

Med-IQ is not liable for any decision made or action taken in reliance upon the information provided through this activity. For questions or comments about this activity, please contact Med-IQ. Call (toll-free) 866 858 7434 or e-mail info@med-iq.com.

ADA Statement
Med-IQ fully complies with the legal requirements of the ADA and the rules and regulations thereof. If any participant in this educational activity is in need of accommodations, please contact Med-IQ at 443 543 5200.

Disclaimer
The information provided through this activity is for continuing education purposes only and is not meant to substitute for the independent medical judgment of a physician relative to diagnostic and treatment options of a specific patient’s medical condition.

Privacy and Confidentiality
Med-IQ is committed to honoring your privacy and protecting any personal information you choose to share with us. For detailed information about our privacy policy, please visit: www.med-iq.com/privacy-policy.html.

Acknowledgement of Commercial Support
This activity is supported by an educational grant from Pfizer, Inc.

© 2018 National Gaucher Foundation, Inc. All rights reserved.