

Gaucher Disease: The Evolving Big Picture

Please join us for a 2-Part CE Satellite Symposium

Wednesday, February 7

11:45 AM – 12:45 PM

Grand Hall AB

Part II: Gaucher Disease Across the Lifespan

PROGRAM OVERVIEW:

Although Gaucher disease is one of the most widely known of the lysosomal diseases and treatments are available, there is still so much that needs to be addressed. This 2-part CE satellite session focuses on key issues with Gaucher disease (GD) that require ongoing discussion, research and attention. The goal is to raise awareness of the current challenges in GD diagnosis and lifelong management, and provide a forum for discussion for future changes.

PART I:

The **first part** addresses the role of inflammation in GD, the evolution of the natural history of GD including the need to re-define beyond types I, II and III, and how to manage neuroopathic GD, followed by a panel discussion.

PART II:

The **second part** of this session covers the challenges and opportunities for newborn screening (NBS), what to do after GD is identified on NBS, what discussions need to occur during management of GD during pregnancy, and concludes with a panel discussion.

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| 11:45 AM | Introduction | Uma Ramaswami |
| 11:50 AM | Patient Advocate and Carer Perspective | Tanya Collin-Histed |
| 12:00 PM | NBS for GD in the United States: Status and Needs Across the Patient's Lifetime | Amy Gaviglio |
| 12:10 PM | Challenges in Interpreting <i>GBA1</i> genotyping | Nahid Tayebi |
| 12:20 PM | Implications for Pregnancy, Gaps in Knowledge, Future Directions | Priya Kishnani |
| 12:30 PM | Wrap Up: Tying All the Pieces Together | Uma Ramaswami |
| 12:35 PM | Q&A and Discussion | Faculty |
| 12:45 PM | Adjourn | |

TARGET AUDIENCE:

This activity is intended for healthcare professionals involved in the screening, diagnosis, management, and treatment of lysosomal diseases affecting both children and adults, with research and clinically relevant information geared specifically to the following professionals: medical and clinical geneticists; genetic counselors; pediatricians; neurologists; psychologists; nurse practitioners; physician assistants; and patient advocates.

There is no additional fee to attend this satellite symposium.

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LEARNING OBJECTIVES:

1. Delineate the potential issues in diagnosing GD, follow-up testing, and initiation of therapy.
2. Discuss recent guidelines for identification and management of GD.
3. Outline appropriate strategies for management based on the patient's stage in life, including newborns, pediatrics, elderly patients and management during pregnancy.
4. Define the emerging treatments for Gaucher disease, differentiating for whom and when these treatments should be considered.
5. Elucidate the role of inflammation and the importance of biomarkers for monitoring therapies that address inflammation.
6. Identify biomarkers of neuronal disease and apply them in monitoring progression in patients.
7. Discuss the evolution of the natural history of GD and initiate discussions on redefining Gaucher disease beyond types I, II and III.
8. Define the evolving approach in managing type 3 Gaucher disease, including recent therapeutic advances.

FACULTY:



Uma Ramaswami, FRCPCH, MD (Chair)
Royal Free London
NHS Foundation Trust
London, United Kingdom



Nahid Tayebi, PhD
MGB, NHGRI, National Institutes of Health
Bethesda, MD, United States



Tanya Collin-Histed
International Gaucher Alliance
London, United Kingdom



Priya S. Kishnani, MD
Duke University Medical Center
Durham, NC, United States



Amy Gaviglio, MS, CGC
4ES Corporation
Centers for Disease Control and Prevention
Minneapolis, MN, United States



CE ACCREDITATION:

This activity is jointly provided by Postgraduate Institute for Medicine and Saterdalen & Associates, LLC.

Joint Accreditation Statement



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INTERPROFESSIONAL CONTINUING EDUCATION

In support of improving patient care, this activity has been planned and implemented by the Postgraduate Institute for Medicine and Saterdalen & Associates, LLC. Postgraduate Institute for Medicine is jointly accredited by the Accreditation Council for Continuing Medical Education (ACCME), the Accreditation Council for Pharmacy Education (ACPE), and the American Nurses Credentialing Center (ANCC), to provide continuing education for the healthcare team.

Physician Continuing Medical Education

The Postgraduate Institute for Medicine designates this live activity for a maximum of 2.0 *AMA PRA Category 1 Credit(s)*[™]. Physicians should claim only the credit commensurate with the extent of their participation in the activity.

Continuing Nursing Education

The maximum number of hours awarded for this Continuing Nursing Education activity is 2.0 contact hours.

Provider approved by the California Board of Registered Nursing, Provider Number 13485, for 2.0 contact hours.



CE CREDIT & FACULTY DISCLOSURES

Instructions to obtain CE credit or a Certificate of Attendance for this activity are available using the QR code. Faculty disclosures may also be downloaded at this link.

This activity is not available for CEU credits for Genetic Counselors.

Participants must attend both days to receive 2.0 hours of credit.

GRANT SUPPORT

This activity is supported by an independent educational grant from Takeda Pharmaceuticals, U.S.A., Inc.

Eligibility: Registration for *WORLDSymposium 2024* and an official name badge are required to attend this satellite symposium.

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