Dear colleague,

please accept my invitation to join us for an informative and stimulating continuing medical education symposium entitled "Gaucher disease: Can we build a better roadmap for patient care?".

The goal of this CME symposium is to provide clinicians with new insights to navigate the complex challenges in managing patients with Gaucher disease. Despite very significant advances in disease understanding and treatment options, there are still a number of important issues that have not been resolved and which have become important research priorities.

These include:
- Explaining phenotypic heterogeneity including variability in organ involvement
- Defining the mechanisms underlying the relationships between GD and common clinically important complications and comorbidities, most notably Parkinson disease and malignancy
- Identifying and selecting biomarkers that are most useful for establishing a prognosis, predicting response to treatment, and monitoring the efficacy of therapy in patients with GD
- Developing approaches to support individualization of treatment for those patients with GD

The faculty and I very much look forward to welcoming you to this educational symposium on February 11th at 11:45 in room Windermere Ballroom Y at the Hyatt Regency Hotel, Orlando, Florida, USA.

Sincerely,

Prof. Atul Mehta
Symposium chairman

AGENDA

11:45 – 11:50 Welcome and introductory remarks
Atul Mehta (UK)

11:50 – 12:00 Biomarkers as a guide to understanding and effectively treating Gaucher disease
Priya Sunil Kishnani (USA)

12:00 – 12:10 Approaches to understanding genotype-phenotypic relationships in patients with Gaucher disease
Gregory A. Grabowski (USA)

12:10 – 12:20 Complications and comorbidities in Gaucher disease: Biologic pathways, prediction, and early markers of organ involvement
Derralynn Hughes (UK)

12:20 – 12:30 Rational individualization of therapy in an era of expanding options for the patient with Gaucher disease – ready yet?
Atul Mehta (UK)
All faculty

12:30 – 12:45 Questions and answers / Wrap-up

LEARNING OBJECTIVES

At the end of this activity participants will be able to:
- Describe the full spectrum of phenotypes in patients with different, subtypes of Gaucher disease
- Review factors beyond genotype with the potential to influence phenotype and risk for specific disease complications and comorbidities, and utility of biomarkers in establishing diagnoses
- Summarize what is known regarding relationships between genotypes and phenotypes in Gaucher disease
- Describe different treatments for Gaucher disease and factors with the potential to guide initial treatment selection and augmentation

TARGET AUDIENCE

This activity has been designed to meet the educational needs of healthcare professionals involved in the treatment of patients with lysosomal diseases, particularly those with Gaucher disease.

FACULTY

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Priya Sunil Kishnani
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Gregory A. Grabowski
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Derralynn Hughes
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