Virtual Session Handout

Accredited CME/CNE Symposium

Thursday, February 11, 2021
12:00 – 1:00 PM EST

Critical Impact of Inflammation in Lysosomal Diseases: Unlocking Future Treatments and Improving Outcomes

Program Overview

The goal of this one-hour virtual satellite symposium to be held at WORLD*Symposium* 2021 will be to address the critical role of inflammation in lysosomal diseases, with a specific focus on inflammation in Gaucher disease, mucopolysaccharidosis type II (MPS II), and metachromatic leukodystrophy (MLD). Gaucher disease, MPS II, and MLD are three lysosomal diseases in which inflammation plays a significant role in disease progression and associated neurodegeneration. Given the role of inflammation in the development of these diseases, researchers have increasingly focused on reducing inflammation as a means of preventing their onset and slowing disease progression. This virtual satellite symposium will discuss current management of inflammation, ongoing research, and future options in Gaucher disease, MPS II, and MLD.

Agenda

12:00 PM	Welcome and introduction (Uma Ramaswami, Chair)
12:05 PM	Monitoring and management of inflammation in Gaucher disease: Challenges, options and useful tools (Ozlem Goker-Alpan)
12:20 PM	Managing MLD today and tomorrow: HSCT, ERT, gene therapy (Troy Lund)
12:35 PM	MPS: Current strategies, clinical trials, and hope for the future (Lynda Polgreen)
12:50 PM	Panel discussion and audience Q&A
1:00 PM	Adjourn

Target Audience

This educational activity has been designed to deliver up-to-date education to physicians, physician assistants, nurse practitioners, nurses, pharmacists, and other healthcare providers who manage patients with lysosomal diseases, specifically patients with MLD, Gaucher disease, and MPS II.

Learning Objectives

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

1. Delineate the connections among defective lysosomes, macromolecule storage, and inflammation in Gaucher disease, MPS II, and MLD.
2. Review the pathways through which chronic inflammation and neuroinflammation contribute to neurodegeneration.
3. Describe how inflammation contributes to neuronal death and the onset and progression of Gaucher disease, MPS II, and MLD.
4. Discuss the role of inflammation and its impact on current and future treatment strategies for Gaucher disease, MPS II, and MLD.

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Faculty

Uma Ramaswami, FRCPCH, MD (Chair)
Honorary Senior Research Associate
Genetics and Genomic Medicine Department
University College London
Consultant in Inherited Metabolic Disorders
Clinical Lead, Lysosomal Disorders Unit
Institute of Immunity and Transplantation
Royal Free London NHS Foundation Trust
London, United Kingdom

Dr. Uma Ramaswami is a Consultant in Inherited Metabolic Disorders and Clinical Lead for the Lysosomal Disorders Unit at the Royal Free Hospital, London. Uma has a special interest in clinical research relating to understanding of the natural history and disease progression of inherited metabolic disorders. Uma leads transition services for young patients with inherited metabolic disorders and is the National Clinical Lead for the UK Paediatric Familial Hypercholesterolaemia Register and Co-project lead for the European FH Register. Uma has been a principal investigator and co-investigator for many pivotal clinical trials for lysosomal disorders. She has over 100 peer reviewed and publications in metabolic medicine. Uma is a Lysosomal Disorders Expert Advisory Group member for NHS England, National Institute of Clinical Excellence (NICE) advisor, Communicating Editor for Journal of Inherited Metabolic Disorders (JIMD),teaching faculty at University College London, and an invited speaker at many National and International conferences, including lysosomal patient organisations led conferences.

Ozlem Goker-Alpan, MD
Founder and Chief Medical Officer
Lysosomal & Rare Disorders Research & Treatment Center
Fairfax, VA, USA

Dr. Ozlem Goker-Alpan is the founder and president of LDRTC, a nonprofit organization that focuses on Lysosomal Disorders and other Rare diseases. Dr. Goker-Alpan founded LDRTC in 2013 with a vision that quality care to individuals with Rare Diseases is provided by offering clinical care, clinical and translational research under one roof. LDRTC is a unique institution, and conducts a variety of clinical trials, investigator-initiated research protocols, and collaborative projects with academia, industry and patient advocacy organizations. Another goal that LDRTC undertakes is to bring patients, expert clinicians, researchers and industry together through education and collaboration. With that goal, LDRTC sponsored annual GRIDS symposium has covered many relevant topics in since 2014. Her passion for translational medicine and clinical genetics research has led to LDRTC's recognition in a short period of time. Dr. Goker-Alpan received her medical degree in 1990 from Marmara University School of Medicine in Istanbul, Turkey with the highest honors as first in her class. She trained in Pediatrics and then served as a Pediatric Chief Resident at SUNY at Stony Brook, New York. She completed her first fellowship in Clinical and Biochemical Genetics at the National Institutes of Health, Greater Washington Medical Genetics Program in 1999, and worked as an adjunct scientist at the National Child Health Institute. Her second fellowship focused on Lysosomal Storage Disorders and Gaucher disease at the Clinical Neuroscience Branch, NIMH. She coordinated the NIH Gaucher Clinic at the Medical Genetics Branch of the National Human Genome Research. As an established clinician and translational scientist in rare genetic and lysosomal storage disorders, her pursuit is to provide individualized care and treatment for patients with LSDs and rare diseases. Under her supervision, LDRCT has completed multiple scientific projects exploring immune pathways and lysosomal functions to develop new diagnostic and monitoring tools in LSDs and GBA-related parkinsonism. She serves on the scientific advisory boards of multiple pharmaceutical companies and patient advocacy organizations. Her continuing effort is to educate and train the new generation health care providers in Lysosomal Storage Disorders.

Troy Lund, MSMS, PhD, MD, FAAP
Associate Professor, University of Minnesota
Pediatric Blood and Marrow Transplant Program
Leukodystrophy Center of Excellence
Stem Cell Institute
Global Pediatrics
University of Minnesota Medical Center
Minneapolis, MN, USA

Dr. Troy Lund is an international expert on the use of cell and gene therapy for patients with inherited metabolic disorders and lysosomal storage disorders including adrenoleukodystrophy (ALD), metachromatic leukodystrophy (MLD), mucopolysaccharidosis type I (MPS I), and osteopetrosis (OP). Dr. Lund is a key opinion leader in all these areas. He has been consulting on various aspects of rare diseases and clinical research for more than 20 years. Dr. Lund's research at the University of Minnesota focuses on predicting the outcomes for patients undergoing blood and marrow transplantation. He also works to increase our understanding of the pathophysiological processes underlying inherited metabolic diseases in the lab and in the clinical settings. Dr. Lund has published extensively on various aspects of these rare diseases and made substantial contributions to the field with his work both in the clinic and the laboratory. He has more than 100 publications in peer-reviewed journals, including Blood, Biology of Blood and Marrow Transplantation, Stem Cells, Nature Reviews Clinical Oncology, and PLoS One. He has presented more than 100 abstracts and lectures at national and international meetings on a variety of topics.

Lynda Polgreen, MD, MS
Investigator, The Lundquist Institute at Harbor-UCLA
Associate Professor of Pediatrics
David Geffen School of Medicine at UCLA
Los Angeles, CA, USA

Dr. Lynda Polgreen is a pediatric endocrinologist who received her degree in Medicine from the Mount Sinai School of Medicine. She then completed her pediatric residency and postdoctoral fellowship in pediatric endocrinology at the University of Minnesota. She is currently an Investigator at The Lundquist Institute at Harbor-UCLA Medical Center, and an Associate Professor of Pediatrics at the David Geffen School of Medicine – UCLA. She is the director of the Center for Treatment of Rare Diseases at The Lundquist Institute and the chair of the National MPS Society Scientific Advisory Board. Dr. Polgreen's research primarily focuses on ways to improve the treatment and understanding of skeletal and joint pathology in the mucopolysaccharidoses (MPS). She is currently conducting clinical trials evaluating the role of inflammation and immune dysregulation in MPS disease pathology, and the effects of anti-inflammatory therapies.

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Disclosure of Conflicts of Interest

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The following CME/CE Co-Chairs and Speakers for WORLD*Symposium* have reported the following financial relationships or relationships they or their spouse/life partner have with commercial interests related to the content of this continuing education activity:

Faculty Name	Disclosure
Uma Ramaswami	Consulting Fees (e.g., advisory boards): Amicus Therapeutics, Takeda, Sanofi Contracted Research: Amicus Therapeutics, Takeda
Troy Lund	None
Ozlem Goker-Alpan	Consulting Fees (e.g., advisory boards): Sanofi Genzyme, Takeda, Amicus Therapeutics, Prevail, Freelite Fees for Non-CME/CE Services Received Directly from a Commercial Interest or their Agents (e.g., speakers' bureaus): Sanofi Genzyme, Takeda Contracted Research: Sanofi Genzyme, Protalix, Takeda, Amicus Therapeutics, Freelite, Sangamo
Lynda Polgreen	Consulting Fees (e.g., advisory boards): BioMarin, Sanofi-Genzyme, Pfizer-Therachon, Immusoft, Sangamo Fees for Non-CME/CE Services Received Directly from a Commercial Interest or their Agents (e.g., speakers' bureaus): Sanofi Genzyme Contracted Research: Takeda-Shire, BioMarin, Pfizer-Therachon

The planners and managers reported the following financial relationships or relationships they or their spouse/life partner have with commercial interests related to the content of this continuing education activity:

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Eligibility: Registration for WORLD*Symposium* 2021 is required to attend this satellite symposium.

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Fee Information: There is no fee for this educational activity.