

THE LATEST UPDATE ON METACHROMATIC LEUKODYSTROPHY

Screening, Diagnosis, and
Emerging Treatments to
Improve Quality of Life of Patients

Friday, October 14, 2022 | 12:30-2:00 PM ET Doors open
at 12:00 PM
Duke Energy Convention Center | Cincinnati, OH Rooms
200 & 205



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AGENDA

Symposium begins at 12:30 PM

FACULTY

Florian S. Eichler, MD (Moderator)

Associate Professor of Neurology
Massachusetts General Hospital
Harvard Medical School
Boston, MA

Laura A. Adang, MD, PhD

Assistant Professor of Child Neurology
Children's Hospital of Philadelphia
Philadelphia, PA

Rachel E. Hickey, MS, LCGC

Genetic Counselor, Care Coordinator of Leukodystrophy Clinic
Ann & Robert H. Lurie Children's Hospital of Chicago
Chicago, IL

TARGET AUDIENCE

Physicians, PAs, nurse practitioners, nurses and pharmacists specializing in genetics, metabolism, neurology, pediatrics, internal medicine and/or primary care.

FINANCIAL SUPPORT

Supported by an educational grant from Takeda Pharmaceuticals U.S.A., Inc.

LEARNING OBJECTIVES

At the end of this CME/CE activity, participants should be able to:

- Review the impact of diagnostic delay and misdiagnosis on patients with MLD.
- Indicate strategies for identifying patients with MLD at the pre-symptomatic phase of disease.
- Implement early screening for patients with suspected MLD based on symptoms and clinical presentations.
- Evaluate efficacy and safety of emerging therapies for MLD.

CREDIT INFORMATION

JOINTLY ACCREDITED PROVIDER



In support of improving patient care, CME Outfitters, LLC, 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.

PHYSICIANS (ACCME)

CME Outfitters, LLC, designates this live activity for a maximum of *1.5 AMA PRA Category 1 Credit(s)*[™]. Physicians should claim only the credit commensurate with the extent of their participation in the activity.

NURSES (ANCC)

This activity is designated for 1.5 contact hours.
Note to Nurse Practitioners: Nurse practitioners can apply for AMA PRA Category 1 Credit(s)[™] through the American Academy of Nurse Practitioners (AANP). AANP will accept AMA PRA Category 1 Credit(s)[™] from Jointly Accredited Organizations. Nurse practitioners can also apply for credit through their state boards.

CALIFORNIA RESIDENTS:

This continuing nursing education activity was approved by the California Board of Registered Nursing. CME Outfitters LLC's provider number is CEP15510.

Pharmacists (ACPE)

This application-based activity is approved for 1.5 contact hours (0.15 CEUs) of continuing pharmacy credit (JA0007185-0000-22-071-L01-P).

PAS (AAPA)



CME Outfitters, LLC, has been authorized by the American Academy of PAs (AAPA) to

award AAPA Category 1 CME credit for activities planned in accordance with AAPA CME Criteria. This activity is designated for 1.5 AAPA Category 1 CME credits. PAs should only claim credit commensurate with the extent of their participation.

ABIM MOC CREDIT



Successful completion of this CME activity, which includes participation in the evaluation component, enables the participant to earn up to 1.5 medical knowledge MOC points in the American Board of Internal Medicine's (ABIM) Maintenance of Certification (MOC) program. Participants will earn MOC points equivalent to the amount of CME credits claimed for the activity. It is the CME activity provider's responsibility to submit participant completion information to ACCME for the purpose of granting ABIM MOC credit.

ROYAL COLLEGE MOC

Through an agreement between the Accreditation Council for Continuing Medical Education and the Royal College of Physicians and Surgeons of Canada, medical practitioners participating in the Royal College MOC Program may record completion of accredited activities registered under the ACCME's "CME in Support of MOC" program in Section 3 of the Royal College's MOC Program.

MIPS IMPROVEMENT ACTIVITY

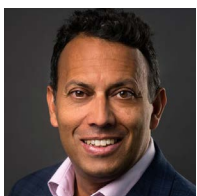
This activity counts towards MIPS Improvement Activity requirements under the Medicare Access and CHIP Reauthorization Act of 2015 (MACRA). Clinicians should submit their improvement activities by attestation via the CMS Quality Payment Program website.

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Florian S. Eichler, MD (Moderator)



Florian S. Eichler, MD, is an Associate Professor of Neurology at Massachusetts General Hospital (MGH) and Harvard Medical School. He is the Director of the Leukodystrophy Service, the Center for Rare Neurological Diseases at MGH, and the Mass General Brigham Neurogenetics and Gene Therapy Fellowship. He runs a laboratory at MGH that explores the relationship of mutant genes to specific biochemical defects and their

contribution to neurodegeneration. To develop novel treatments, his laboratory assesses the consequences of disease-causing genes. The laboratory identified two neurotoxic desoxysphingoid bases that accumulate in mutant transgenic mice and humans with hereditary sensory neuropathy type 1 (HSAN1). This work led to the first clinical trial of substrate supplementation therapy for patients with HSAN1 (NCT01733407). Dr Eichler is the principal investigator of several NIH-funded studies on neurogenetic disorders as well as a gene therapy trial of adrenoleukodystrophy (NCT01896102) that reported on first successful outcomes in the *New England Journal of Medicine*. For this work, he received the Martin Research Prize from MGH and the Herbert Pardes Clinical Excellence Award from the Clinical Research Forum.

Laura A. Adang, MD, PhD



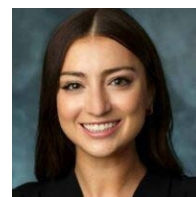
Dr. Laura Adang is an Assistant Professor of Child Neurology at the Children's Hospital of Philadelphia specializing in the care of children with leukodystrophies. Dr. Adang is a magna cum laude graduate of the University of Georgia's Foundation Fellowship scholarship program and a graduate of the Medical Scientist Training Program at the University of Virginia, where she received both her MD and PhD. Her graduate work in

the laboratory of Dean H. Kedes, MD, PhD characterized the immune evasion mechanisms of herpesvirus infections. Her work has been published in *NEJM*, *Cell*, and *Journal of Clinical Investigations* among others.

After graduating from the University of Virginia, she completed her pediatrics and child neurology residencies at the Children's Hospital of Philadelphia and the University of Pennsylvania. She completed additional fellowship training in multiple sclerosis and leukodystrophies and a Masters of Translational Research

at the Institute for Translational Medicine and Therapeutics (ITMAT) of the Perelman School of Medicine at the University of Pennsylvania. Her primary research focus is clinical trial readiness for leukodystrophies, including metachromatic leukodystrophy.

Rachel E. Hickey, MS, LCGC



Rachel received her Master of Science in Genetic Counseling from the University of South Carolina School of Medicine in May 2017 and was certified by the American Board of Genetic Counseling (ABGC) in August 2017. Rachel completed a genetic counseling fellowship in lysosomal storage diseases at Ann & Robert H. Lurie Children's Hospital, focusing on diagnosis, management, treatment, and newborn screening for this group

of complex diseases. She currently provides genetic counseling services in the pediatric setting, continuing to specialize in lysosomal storage diseases as well as general genetics. She serves as the clinic coordinator for the Leukodystrophy Clinic at Lurie Children's Hospital and is the primary coordinator for this center's Krabbe Disease and X-Linked Adrenoleukodystrophy Newborn Screening Programs.

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DISCLOSURE DECLARATIONS

It is the policy of CME Outfitters, LLC, to ensure independence, balance, objectivity, and scientific rigor and integrity in all of their CE activities. Faculty must disclose to the participants any relationships with commercial companies whose products or devices may be mentioned in faculty presentations, or with the commercial supporter of this CE activity. CME Outfitters, LLC, has evaluated, identified, and mitigated any potential conflicts of interest through a rigorous content validation procedure, use of evidence-based data/research, and a multidisciplinary peer review process. The following information is for participant information only. It is not assumed that these relationships will have a negative impact on the presentations.

Dr. Eichler reports the following financial relationships:

- **Advisory Board:** Bluebird Bio, Inc. and SwanBio Therapeutics, Inc.
- **Consultant:** Autobahn Therapeutics; Poxel S.A.; SwanBio Therapeutics, Inc.; Takeda Pharmaceuticals U.S.A., Inc.; Taysha Gene Therapies; and UpToDate, Inc.
- **Grants:** European Leukodystrophy Association; National Institute of Neurological Disorders and Stroke (NINDS); and Undiagnosed Disease Network (UDN)
- **Research Support:** Aspa Therapeutics; Bluebird Bio, Inc.; and Sio Gene Therapies
- **Speakers Bureau:** American Society for Gene and Cell Therapy (ASGCT) and PRIME Education, LLC
- **Stock Shareholder (ownership interest):** SwanBio Therapeutics, Inc.
- **Other Financial or Material Support:** UpToDate, Inc. Authorship Fees

Dr. Adang reports the following financial relationships:

- **Consultant:** MEGMA; Orchard Therapeutics; and Takeda Pharmaceuticals U.S.A., Inc.

- **Grants:** MSD Action Foundation; National Center for Advancing Translational Sciences (NCATS); National Institute of Neurological Disorders and Stroke (NINDS); and The Family Foundation

Ms. Hickey reports no financial relationships to disclose.

The following peer reviewer and CME Outfitters staff report no financial relationships:

- Jeffrey Helfand, DO (peer reviewer)
- Thomas Mitchell (planning committee)
- Kellie Busby, PharmD (planning committee)
- Susan H. Yarbrough, CHCP (planning committee)
- Sandra Caballero, PharmD (planning committee)
- Sharon Tordoff (planning committee)

Faculty of this CE activity may include discussions of products or devices that are not currently labeled for use by the FDA. The faculty have been informed of their responsibility to disclose to the audience if they will be discussing off-label or investigational uses (any uses not approved by the FDA) of products or devices.

Post-tests, credit request forms, and activity evaluations must be completed online (requires free account activation), and participants can print their certificate or statement of credit immediately (75% pass rate required). This website supports all browsers except Internet Explorer for Mac. For complete technical requirements and privacy policy, visit www.cmeoutfitters.com/privacy-and-confidentiality-policy.

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INSTRUCTIONS FOR INTERACTIVE TECHNOLOGY

Please use the iPads provided at your table to answer polling questions, view onsite presentations, and submit questions to the faculty. Please follow the instructions below:

1. Get Connected

Please connect to the CME Outfitters network with your mobile device or laptop. This network does not have a password. *Please note that this network is a local network and does NOT mean you are connected to the internet. Once you have joined the network, go to arraylearn.com. You are now connected to the symposium. All content and polling questions can be viewed on your device as long as you are connected to the above listed network.

2. Ask a Question to the Faculty

Please select the Ask Question tab below the slide viewer. Please include the faculty member's name if the question is specifically for them. Your question will be sent to the faculty for the question and answer portion of the session.

3. View and Take Notes on Presentation Slides

Please click on the Take Notes tab. If you did not enter your email address when you joined the meeting, you will be required to do so for note-taking. When the meeting is complete, all your notes will be emailed to you within 5 business days.

OBTAINING CREDIT

Learners must complete the posttest and evaluation on the iPads. CME Outfitters will email their certificates within 30 days to the email provided upon login to the digital array system.

DOWNLOADABLE RESOURCES

Downloadable resources will be available at www.cmeoutfitters.com/CNSresources

ENGAGE WITH US

Follow us on [Twitter @cmeoutfitters](https://twitter.com/cmeoutfitters) for announcements about upcoming CME opportunities, healthcare news, and more!

This seminar was staged in conjunction with the 51st Annual Meeting of the Child Neurology Society. A staging fee was paid to the CNS to present this session. CNS is not responsible for content or CME credit, nor does it endorse the sponsoring organization or its products and/or services.

