The 7th International Forum of Lysosomal Disorders

第７回国際ライソゾーム病フォーラム

会 場

東京慈恵会医科大学 2号館1階講堂
Tokyo Jikei University School : Main Auditorium

日 時

２０２４年 July １２（Fri） ９：００－１８：４０
July １３（Sat）９：００－１３：１０

Organized by Interantional LSD Forum Commettee
President：Prof. T. Okuyama（Saitama Medical Univ.）・Vice President：Prof. H. Kobayashi（Jikei Univ.）
Honorary President：Prof. Y. Eto（Southern Tohoku Institute for Neuroscience/Jikei Univ.）

［共催］NPO法人　Japan LSD Research Center, Saitama Medical University・
厚生労働省難治性疾患政策研究事業「ライソゾーム病・ペルオキシソーム病」研究班（奥山班）・全国ファブリ病患者と家族の会
［参加費］医療関係者 5,000円・製薬会社関係者 20,000円・学生/大学院生/患者会 無料・マスコミ関係者 無料

参加登録はこちら

July 12（Fri）

- Opening greeting: Prof. T. Okuyama (Saitama Medical Univ.)
- Lysosomal diseases: an Overview: Prof. Y. Eto (Southern Tohoku Inst. for Neuroscience/Jikei Univ.)
- Current Status of Gene Therapy for Intractable Genetics Diseases: Prof. M. Onodera (NCCHD. Japan)
- Gene Therapy for Lysosomal Storage Diseases: Prof. H. Kobayashi (Jikei Univ.)
- An Overview of Progress in Gene Therapy for Mucopolysaccharidosis Diseases: Prof. C. Whitley (Minnesota Univ. USA) (Web)
- REKLAIM, a novel Phase Ib Clinical Trial of FBX-101 (AAVrh10.GALC) Intravenously administered after UCBT for Krabbe Disease: Prof. M. Escolar (CMO ForgeBiologics and Adjunct Professor of Pediatrics, University of Pittsburgh, USA)
- Luncheon seminar : Pharmacological chaperones – past, present and future: J. P. Castelli (Amicus Therapeutics, Inc.)
- Hematopoietic Stem Cell Gene Therapy for Hurler Syndrome: interim skeletal, neurological and systemic outcomes: Prof. M. E. Bernardo (TIGET, Italy)
- Accelerating Medicines Partnership Bespoke Gene Therapy Consortium for Rare Disorders: Prof. S. Tomatsu (Nemours Children's Health, Wilmington, DE)
- Lysosomes: Fascinating organelles involved in health and disease: Prof. P. Saftig (Kiel Univ. Germany) (Web)
- Intracerebroventricular enzyme replacement therapy in patients with neuronopathic mucopolysaccharidosis type II: Prof. T. Okuyama (Saitama Medical Univ.)
- Clinical Experience with CNS-Targeting Enzyme Replacement Therapy for Neuropathic MPS: Prof. T. Hamazaki (Osaka Metropolitan Univ.)
- Leveraging physiology & engineering for efficient enzyme delivery to the brain and other body tissues: the application of tividenofusp alfa (DNL310) for MPS II: Prof. R. G. Thorne (Denali Therapeutics & Univ. Of Minnesota, USA)
- Heparan sulfate in cerebrospinal fluid, a primary disease activity biomarker, is predictive of clinical benefit in patients with MPS IIIA treated with UX111, an in vivo AAV gene therapy: Dr. H. A. Lau (Ultragenyx, USA)
- Development of hematopoietic stem cell gene therapy for mucopolysaccharidosis type II: Dr. Y. Shimada (Jikei Univ.)
- Gene Therapy approaches with adeno-associated virus(AAV) vectors: Prof. H. Buening (Hannover Univ. Germany) (Web)

July 13（Sat）

- Gene therapy for patients with Nieman-Pick disease type C: Assoc. Prof. K. Kojima (Jichi Univ.)
- Nobel Enzyme Replacement Therapy for Fabry disease: Prof. D. Hughes (The University College London, UK)
- Central Nervous System Enzyme Replacement Therapy (ERT) in Mucopolysaccharidosis type II.: Prof. N. Guffon (Reference Center of Inherited Metabolic Disorders- Hospices Civils of Lyon, HFME Hospital, France)
- TBD: Dr. H. Sonoda (JCR Pharmaceuticals)
- In Utero Enzyme Replacement Therapy for Lysosomal Storage Disorders - The PEARL Trial:Prof. T. MacKenzie (UCSF, US) (Web)
- Gene therapy for autophagy disease:Prof. K. Muramatsu (Jichi Univ.)
- Luncheon seminar: Morbidity and mortality of ASMD patients. Meaning of early diagnosis and treatment: Dr. E. Mengel (Principle investigator, founder and CEO of the SphinCS GmbH & SphinCS Lyso non-profit UG.) (Web)

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