

**Saturday 13 July**  
**The 5th International Forum of Lysosomal Storage Diseases PART III**  
**Tokyo Prince Hotel**

**8:20-8:40 Keynote Lecture: Educational Lecture**

Chair: Prof. Shimozawa Nobuyuki (Gifu University)

**C1 Basic Pathology for Lysosomal Diseases**  
Prof. Sakai Norio (Osaka University)

**8:40-10:35 Work Shop V: Pompe Disease**

Chair: Prof. Priya S. Kishnani (Division of Medical Genetics, Duke University)  
Prof. Fukuda Tokiko (Hamamatsu University School Of Medicine)

**C2-1 Juvenile onset type Pompe disease in Japanese**  
(8:40) Dr. Kobayashi Hiroshi (Tokyo Jikei University)

**C2-2 Adult patients with late-onset Pompe disease (LOPD)**  
(9:00) Dr. Ohya Yasushi (NCNP)

**C2-3 Characteristic skeletal muscle imaging patterns in Japanese patients with Pompe disease**  
(9:20) Dr. Ishigaki Keiko (Tokyo Women's Medical University)

**Invited Lecture 7 Sponsored by JCR Pharma**

**C2-4 Pompe Disease: Updates**  
(9:40) Prof. Priya S. Kishnani (Division of Medical Genetics, Duke University)

**10:20-10:35 BREAK**

**10:35-12:20 Work Shop VI: Lysosome disease<sup>③</sup>**

Chair: Prof. Bigger Brian (The University of Manchester)  
Prof. Ohashi Toya (Tokyo Jikei University)

**C3-1 AAV gene therapy for lysosomal diseases**  
(10:35) Dr. Sonoda Hiroyuki (JCR Pharma)

**Invited Lecture 8**

**C3-2 Novel Therapeutic Approaches to address unmet needs for Pompe Disease**  
(10:55) Dr. Pirozzi Gianluca (Sanofi)

**C3-3 Treatment of Fabry peripheral neuropathy by intrathecal gene therapy using AAV vector**  
(11:20) Prof. Ohashi Toya (Tokyo Jikei University)

---

---

## **Invited Lecture 9 Sponsored by Anges**

**C3-4** Haematopoietic stem cell gene therapy provides long-term correction of  
(11:40) **Mucopolysaccharidosis type II**  
Prof. Bigger Brian (The University of Manchester)

12:10-12:20 **Discussion**

12:30-13:10 **Sponsored by Sanofi-Genzyme:**

Chair: Prof. Takayanagi Masaki (Teikyo Heisei University)

## **Invited Lecture 10 (Educational Lecture)**

**C4** **Gaucher disease: educational seminar**  
Prof. Mistry Pramod (Yale University)

13:20-14:50 **Sponsored by Sanofi: Early Diagnosis for LSD by Newborn Screening**

Chair: Prof. Burton Barbara.K (Northwestern University)  
Prof. Fumio Endo (Kumamoto University)

## **Invited Lecture 11**

**C5-1** **Newborn Screening for Lysosomal Storage Disorders in the United States**  
(13:20) Prof. Burton Barbara.K (Northwestern University)

## **Invited Lecture 12**

**C5-2** **NBS for LSD: Current situation in Taiwan**  
(14:00) Dr. Chien Yin-Hsiu (National Taiwan University)

**C5-3** **The current situation and future prospects of Newborn Screening for Lysosomal Diseases in Japan**  
(14:30) Prof. Nakamura Kimitoshi (Kumamoto University)

14:50-15:00 **Closing Remarks**

Prof. Eto Yoshikatsu (President, the 5th International LSD Forum)