



## The 6<sup>th</sup> International Forum of Lysosomal Disorders

Program (March 30-April 1, 2023, Tokyo, Japan)

### Open Memorial Lecture on Top Medical Science

First Floor, The Auditorium, 2<sup>nd</sup> building The Jikei University

16:00 – 16:20 **Welcome Lecture**

Prof. Yoshikatsu Eto (President, the 6<sup>th</sup> International LSD Forum)

16:20-16:30 **Welcome Greeting**

Prof. Senya Matsufuji (President, Jikei University, Tokyo)

16:30-17:00 **Special Lecture I**

Chair: Prof. Yoshikatsu Eto (Sothern Tohoku Institute for Neuroscience/Jikei University, Tokyo)

Genetics, Genomics & Omics: Impact on Genetic Diseases"

Prof. Robert Desnick (Mount Sinai Medical Center, N.Y.)

17:00-17:30 **Special Lecture II**

Chair: Prof. Kimihiko. Oishi (Jikei University, Tokyo)

Advances in Gene Therapy for Lysosomal Diseases – Trials and Tribulations

Prof. Brian Bigger (Manchester University)

17:30 – 18:10 **Special Lecture III**

Chair: Prof. Torayuki Okuyama (National Institute for Child Health and Development, Tokyo)

The Golden Age of Rare Disease Treatment

Dr. Emil Kakkis ( CEO and President, Ultragenyx, USA)

18:10 – 18:40 **Special Lecture IV**

Chair: Prof. Toya Ohashi (Jikei University, Tokyo)

Life Science Development in Japan

Prof. Ryuichi Morishita ( Osaka University)

19:00– 21:00 **Welcome Party at the Prince Park Tower Tokyo, the 33th floor**

## **March 31 (Fri) Day 2**

### **The 6th International Forum of Lysosomal Storage Diseases PART II Tokyo Prince Hotel-Magnolia Hall**

8:30 – 8:40      Opening Remarks  
                    Prof. Yoshikatsu Eto (President, the 6<sup>th</sup> International LSD Forum)

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#### **Workshop 1 Fabry disease**

Chair: Prof. Robert J. Desnick (Mt Sinai Medical Center, NY) / Prof. Yoshikatsu Eto  
(Sothern Tohoku Institute for Neuroscience/Jikei University, Tokyo)

8:40 – 9:00

Chaperon therapy of Japanese Fabry patients  
                    Dr. Masahisa Kobayashi (Jikei University, Tokyo)

9:00 – 9:20      Clinical Phenotype and Genotype of Japanese Female Fabry Patients.  
                    Dr. Kazuya Tsuboi (Nagoya Central Hospital, Nagoya)

#### **Invited Lecture 1**

Chair: Prof. Yoshikatsu Eto

(Sothern Tohoku Institute for Neuroscience/Jikei University, Tokyo)

9:20 – 9:50      Fabry Disease: History, Subtypes and Treatment  
                    Prof. Robert J. Desnick (Mt. Sinai Medical Center, N.Y.)

9:50-10:00      Break

#### **Invited Lecture 2**

Chair: Prof. Hitoshi Sakuraba (Meiji Pharmaceutical University, Tokyo)

10:00 – 10:40    Biomarkers of Fabry disease  
                    Prof. Johannes MFG Aerts ( University of Leiden, Netherlands)

#### **Invited Lecture 3**

10:40 – 11:20    Chair: Dr. Ken Sakurai (Jikei University, Tokyo)  
                    Management of Fabry disease (Tentative)  
                    Prof. Robert J. Hopkin (Cincinnati Children Hospital, US)

#### **Invited Lecture 4**

11:20-12:00     Chair: Prof. Kenichi Hongo (Jikei University, Tokyo)  
                    Measuring cardiac involvement in Fabry disease and the use of AI  
                    Prof. James Moon ( Barts Heart Centre (BHC), UCL, UK)

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## Luncheon Seminar

Chair : Prof. Tsutomu Takahashi (Akita University, Akita)

12:10– 13:10

■ Japanese cases of LAL-D

Dr. Yuki Yamada (Osaka City General Hospital)

## Invited Lecture 5

■ Lysosomal Acid Lipase: Deficiency diseases, and roles in cholesterol metabolism and immune function

Prof. Gregory Grabowski (Cincinnati Children Hospital, USA)

## Invited Lecture 6

13:20-14:00

Chair: Prof. N. Sakai (Osaka University, Osaka)

Next-generation proteomics to dissect Fabry disease plasticity

Prof. Soumeiya Bekri (CHU, France)

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## Workshop 2 Gaucher Disease

### Invited Lecture 7

Chair: Prof. Kimitoshi Nakamura (Kumamoto University, Kumamoto)

14:00 – 14:30 Understanding of molecular and pathophysiology of GD3 patient with massive Lymphadenopathy

Prof. Han-Wook Yoo (Asan Medical Center, Korea)

Chair: Prof. Pramod Mistry (Yale University, New Haven)/

Dr. Kei Murayama(Chiba Children Hospital, Chiba)

14:30 – 14:50 Clinical Phenotypes of Japanese Gaucher Disease

Dr. Aya Narita (Tottori University, Tottori)

14:50 – 15:10 SRT treatment in Japanese Gaucher disease

Dr. Ken Sakurai (Jikei University, Tokyo)

15:10-15:40

Chair: Dr. Kei Murayama(Chiba Children Hospital, Chiba)

### Education Lecture 1

**Inflammation mechanisms of Gaucher disease, biomarkers and therapeutic targets.**

Prof. Pramod Mistry (Yale University, New Haven, US)

15:40-15:50

Coffee break

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## Workshop 3: Mucopolysaccharidosis

Chair: Prof. Robert Giugliani (Federal University of Rio Grande do Sul, Brazil)

Dr. Motomichi Kosuga (national Institute for Child Health and Development, Tokyo)

## Education Lecture 2

**Chair:** Dr. Motomichi Kosuga (national Institute for Child Health and Development, Tokyo)

15:50 – 16:20 MPS: overview

Prof. Roberto Giugliani (Federal University of Rio Grande do Sul, Brazil)

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16:20 – 16:40 Japanese MPS II Mutations and Phenotype of MPS

Dr. Motomichi Kosuga (National Center for Child Health and Development, Tokyo)

16:40 – 17:00 MPS VII and Nobel ERT

Prof. Takashi Hamazaki (Osaka Metropolitan University, Osaka)

17:00-17:10 Discussion

## Workshop 4: Novel Treatment for LSD

### Invited Lecture 8

**Chair:** Prof. Toshiyuki Seto (Osaka Metropolitan University, Osaka)

17:10– 17:50 Treatments for MPS: Past, Present and Future”

Prof. Paul Harnatz (UCSF, US)

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17:50 – 18:10 Nobel Enzyme Treatment of Pompe disease

Dr. Keiko Ishigaki (Tokyo Womens Medical University, Tokyo)

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### Invited Lecture 9

**Chair:** Prof. Toya Ohashi (Jikei University, Tokyo)

18:10– 18:50 Chaperon treatment of Fabry disease

Prof. Derralynn Hughes ( Royal Free Hospital London, UCL, UK)

19:00 –21:00 - **Banquet: Tokyo Prince Hotel (Sun Flower room)-**

## **April 1 (Sat) Day 3**

### **The 6th International Forum of Lysosomal Storage Diseases PART III Tokyo Prince Hotel-Magnolia Hall**

8:10-8:40

#### **Education Lecture 3**

**Chair:** Prof. Yoshikatsu Eto (Southern Tohoku Institute for Neuroscience/Jikei University, Tokyo)

Basic Pathology in Autophagy and Lysosomal Diseases

Prof. Norio Sakai (Osaka University, Osaka)

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#### **Workshop 5: CNS Treatment**

8:40-9:10

#### **Education Lecture 4**

**Chair.** Prof. Takashi Hamazaki (Osaka Metropolitan University, Osaka)

CNS treatment of MPS II

Prof. Torayuki Okuyama (Saitama University, Saitama)

9:10 –9:40

#### **Invited Lecture 10**

**Chair :** Prof. Hiroshi Kobayashi (Jikei University, Tokyo)

CNS treatment of MPS I, II

Prof. Roberto Giugliani (Federal University of Rio Grande do Sul, Brazil)

9:40 – 10:10

#### **Education Lecture 5**

**Chair:** Prof. Hitoshi Osaka (Jichi University School of Medicine, Tochigi)

CNS treatment of CLN2

Dr. Kaoru Eto (Tokyo Womens Medical University, Tokyo)

#### **Workshop 6: Gene Therapy for LSD**

**Chair:** Prof. Brian Bigger (Manchester University, UK)/

Prof. Masafumi Onodera (National Center for Child Health and Development, Tokyo)

10:10-10:30 Current Status of Gene Therapy in Japan

Prof. Masafumi Onodera (National Center for Child Health and Development, Tokyo)

10:30 –11:00 AAV gene therapy for Niemann Pick C1 and other diseases.

Prof. Takanori Yamagata (Jichi University, Tochigi)

11:00 –11:20 Gene Therapy for MPS II

Prof. Hiroshi Kobayashi (Jikei University, Tokyo)

## **Invited Lecture 11**

Chair : Prof. Takanori Yamagata (Jichi University, Tochigi)

11:20 –11:50 Stem Cell Gene Therapy for Childhood Dementias – A Sanfilippo Stor0079

Prof. Brian Bigger (Manchester University, UK)

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## **Luncheon Seminar**

12:00 –12:40

## **Invited Lecture 12**

Chair: Prof. Hiroyuki Ida (Jikei University, Tokyo)

A decade of eliglustat SRT transforming care of Gaucher disease

Prof. Pramod Mistry (Yale University, New Haven, US)

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## **Workshop 7: Newborn Screening of LSD**

### **Invited Lecture 13**

Chair: Prof. Kimihiko Oishi (Jikei University, Tokyo)

12:50 –13:30 Newborn screening of LSD In US

Dr. Jamie Mills (North Carolina US)

### **Invited Lecture 14:**

#### **Newborn screening in Asia**

Chair: Prof. Yin-Hsiu Chien (National Taiwan University, Taiwan)/

Prof. Shinichi Hirose (Fukuoka University, Fukuoka)

13:30- 14:00 Current situation in Taiwan

Prof. Yin-Hsiu Chien (National Taiwan University, Taiwan)

14:00 – 14:30 Current situation in Japan

Prof. Kimitoshi Nakamura (Kumamoto University, Kumamoto)

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14:30 –14:40 Closing Remarks

Prof. Yoshikatsu Eto (President, the 6<sup>th</sup> International LSD Forum)

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