
Friday 12 July
The 5th International Forum of Lysosomal Storage Diseases PART II
Tokyo Prince Hotel

8:20-8:30 Opening Remarks

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

8:30-9:00 Keynote Lecture: Genome Science of Neurological Disease

Chair: Dr. Kaga Makiko (Tokyo Metropolitan Tobu Medical Center for children with developmental disabilities)

B1 Genome Science of Neurological Diseases

Prof. Tsuji Shoji (The University of Tokyo)

9:00-10:25 Work Shop I : Fabry Disease①

Chair: Prof. Kobayashi Hiroshi (Tokyo Jikei University)
Prof. Watanabe Yoriko (Kurume University)

B2-1 Clinical features of heterozygous Fabry disease

(9:00) Dr. Tsuboi Kazuya (Nagoya Central Hospital)

Invited Lecture 1

B2-2 Characterization of the plasma proteome of Fabry patients before & after enzyme replacement therapy and generation of iPS cells of Fabry disease

(9:20)

Prof. Yoo Han-Wook (Asan Medical Center)

9:45-10:25 Sponsored by Sanofi: Fabry Disease②

Invited Lecture 2

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

B3 Fabry Disease: Importance of Early & Effective Treatment

Prof. Desnick Robert J. (Icahn School of Medicine at Mount Sinai, NY)

10:25-10:40 BREAK

10:40-12:30 Work Shop I : Fabry Disease③

Chair: Prof. Narita Ichiei (Niigata University)
Prof. Desnick Robert J. (Icahn School of Medicine at Mount Sinai, NY)

B4-1 Cardiac manifestation and the effect of long-term enzyme replacement therapy in Japanese Fabry patients

(10:40)

Prof. Hongo Kenichi (Tokyo Jikei University)

B4-2 Glomerular and tubular impairment in the kidney of Fabry disease

(11:00)

Prof. Narita Ichiei (Niigata University)

B4-3 Comprehensive study on plasma lyso-Gb3 in Japanese Fabry patients

(11:20) Prof. Sakuraba Hitoshi (Meiji Pharmaceutical University)

B4-4 Mutation spectrum of α -Galactosidase A gene in Japanese patients with Fabry disease

(11:40)

Dr. Kobayashi Masahisa (Tokyo Jikei University)

B4-5 Future clinical and biochemical predictions of Fabry female disease by methylation studies of GLA gene

(12:00)

Dr. Hossein Arif Mohamed (Southern Tohoku Neuroscience Institute / Tokyo Jikei University)

12:20-12:30 **Discussion**12:40-13:20 **Sponsored by Dainippon-Sumitomo: Educational Lecture**

Chair: Prof. Sakai Norio (Osaka University)

B5 Insights in the heart and cardiovascular involvement in Fabry disease and long-term effects of treatment

Prof. Kampmann Christoph (University of Mainz)

13:30-14:10 **Sponsored by BioMarin: Lysosome disease^①****Invited Lecture 2**

Chair: Prof. Osawa Makiko (Tokyo Women's Medical University)

B6 Neuronal Ceroid Lipofuscinosis: New CNS Enzyme Therapy

Prof. Schulz Angela (University of Hamburg)

14:10-15:30 **Work Shop II: Gaucher disease**Chair: Prof. Ida Hiroyuki (Tokyo Jikei University)
Prof. Mistry Pramod (Yale University)**B7-1 Current Status of Treatment for Gaucher Disease in Japan**

(14:10) Prof. Ida Hiroyuki (Tokyo Jikei University)

B7-2 Pharmacological Chaperone Therapy for neuronopathic Gaucher disease

(14:30) Dr. Narita Aya (Tottori University)

Invited Lecture 3**B7-3 Clinical and Biochemical Insights of Gaucher disease**

(14:50) Prof. Mistry Pramod (Yale University)

15:20-15:25 Discussion

15:25-15:40 BREAK

15:40-17:10 Work ShopⅢ: Mucopolysaccharidosis

Chair: Prof. Suzuki Yasuyuki (Gifu University)
Prof. Yoo Han-Wook (Asan Medical Center)

Invited Lecture 4

B8-1 Unravelling the Role of Inflammation in Mucopolysaccharide Disease

(15:40) Pathogenesis

Prof. Bigger Brian (The University of Manchester)

B8-2 Mutation analysis of IDS gene among Japanese patients with Mucopolysaccharidosis type II

(16:10)

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

B8-3 Experience of enzyme replacement therapy for Mucopolysaccharidosis type VII

(16:30)

Prof. Hamazaki Takashi (Osaka City University)

B8-4 Hematopoietic stem cell transplantation for mucopolysaccharidosis

(16:50)

Prof. Yabe Hiromasa (Tokai University)

17:10-17:20 Discussion

17:20-18:30 Work ShopⅣ: Lysosome disease②

Chair: Prof. Okuyama Torayuki (NCCHD)
Prof. Hughes Derralynn (University college of London)

Invited Lecture 5

B9-1 Treatment of Fabry Disease with Galafold, an Oral Pharmacological Chaperone

(17:20) ~ New Paradigm in precision medicine ~

Prof. Hughes Derralynn (University college of London)

Invited Lecture 6

B9-2 Phase I/II clinical trial of intra-cerebroventricular enzyme replacement therapy for neuronopathic mucopolysaccharidosis type II

(18:00)

Prof. Okuyama Torayuki (National Center for Child Health and Development)

18:20-18:30 Discussion

19:00-21:00 Banquet