NPO 法日本ライソゾーム病研究センター活動報告 2020-2021年

- 1. 研究会開催
- 1)国際協力遺伝病遺伝子治療フォーラム開催 2020年1月15-16日開催



2) 国際遺伝病若手育成のセミナー(NPO 法人日本ライソゾーム病センター主催)



- → DATE: 14:00-Sat. 25: ~13:00-Sun. 26, January, 2020

■ → VENUE : Awards Inn Hankyu--1-50-5, Qoj, Shinagawa-ku, Tokyo 140-0014 Japan <u>TEL:±</u>81-3-3775-6121--For Japanese: http://www.oursinn-hankyu.co.jp/ja/----e
For English: http://www-a.global.hankyu-hotel.com/oursinn-hankyu/e-

■ → Accommodation: Awards Inn-Hankyu (same as Seminar venue)

1-50-5, Qoj, Shinagawa-ku, Tokyo 140-0014 <u>Japan - TFL</u>:+81-3-3775-6121

<Awards Inn Hankvu>nnnnnnnnnnnnnnnnnnnn





Qimachi station (JR Keihin-Tohoku Line Rinkai Line Tokyu-Qimachi Line)

- · → JR Keihin-Tohoku Line⊕→0.1 minute walk from the central exit of Qimachi station↔ · → Bipkai Line⊕→0.1 minute walk from A2 exit of Qimachi station↔
- · → Tokyu-Qimachi Line→[3 minute walk from Qimachi station

<Steering Committee> ←

Director, · Advanced · Clinical · Research · Center, · Institute · of · Neuro · · ·

(Chairperson) @ Science Emeritus Professor, The Jikei University Masaki-Takayanagi⇔ Professor, Health Medical & Sports, Teikyo; Heisei University⇔ ⇔ Professor, Pediatrics, Research Center for Medical Science, The \leftrightarrow Jikei University Jorayuki; Okuyama Director, · LSD · Center, · National · Center · for · Child · Health · & · Development-(NCCHD)⊌ Professor, Pediatrics, Tohoku University Shigeo-Kure

Motomichi Kosuga + Head of Division of Medical Genetics, National Center for Child Health and Development (NCCHD) Hiroshi-Kobayashiri Associate Professor, Pediatrics, Research-Center-for-Medical-

Science, The Jikei University Shinichi-Hirose↔ Professor, Pediatrics, Fukuoka University Yoichi-Matsubarar² Director of the Research-Institute, National Center for Child Health-&-Development∈

Takanobu Otomoi Professor, Kawaseki Medical School, Japan Masafumi Onoderae Director of Department of Human Genetics, National Center

for Child Health and Development Mohammad-Arif-Principal Investigator, Advanced Clinical Research Center, Hossain^{c2} Institute of Neurological Disorders, Japan ^{c2}
David G. Warnock^{c2} Director of the Division of Nephrology and Professor of

Medicine and Physiology at the University of Alabama at Professor, Pediatrics & Medical Genetics, Asan Medical Center Han-Wook-Yoo⊖

Children's Hospital, University of Ulsan College of Medicine, Seoul, Korea

Yoshikatsu: Eto

Day-1:-Saturday-25-January

13:003	Reception-Opene ²
14:00-14:15⊖	Opening-Remarks-
	Yoshikatsu: Eto (Advanced Clinical Research Center, Japan)
14:15-14:40	Self-Introduction ^{e2}
14:40-15:00↔	Chair: Yoichi Matsubara (NCCHD, Japan) Co-sponsor: Sanofi K.K.
€	"Medical Genetics and Genomics for Pediatricians and Young
42	Investigators" ←
42	Han-Wook Yoo (Asan Medical Center, South Korea) ←
15:00-15:10↩	Q&Ar2
15:10-15:30↔	Chair: Toya Ohashi (The Jikei University, Japan)
€	"Gene-therapy-for-Immune-deficiency"
€	Masafumi Onodera (NCCHD, Japan)
15:30-15:40↔	Q&A _C J
15:40-15:55¢	Break [©]
15:55-16:15⊬	Chair : Shinichi-Hirose (Fukuoka-University, Japan) e
42	- "Autophagy-and-lysosomal-storage-disease."
42	Takanobu Otomo (Professor, Kawaseki Medical School, Japan)
16:15-16:25↩	- Q&Ae2
16:25-16:45↔	Chair : Masafumi Onodera (NCCHD, Japan)↔
42	"Update on gene therapy for lysosomal storage disorders" -
4	Hiorshi Kobayashi (The Jikei University, Japan)⊬
16:45-16:55⊍	Q&Ar ²
16:55-17:05↔	Breake ³
17:05-17:35↔	Special-Lecture-II- 0000000 Co-sponsor: Sanofi-K.K.
4	Chair: Shinichi-Hirose · (-Professor, Pediatrics, Fukuoka-University)
42	Title: TBD=
4	David G. Warnock (Professor, University of Alabama at Birmingham,
4	US) e
42	44
17:35-17:45↔	Q&A000000000000000000000000000000000000
17:45-18:00↔	Group-Photoe

18:00-19:00€ Dinne Case Presentation Moderator.:- ← Masaki-Takayanagi (Tejkyo Heisei-University, Japan) « Motomichi Kosuga (NCCHD, Japan) « Presentations Case (1) Case (3)

Day 2 : Sunday 26 January

08:30-08:40	Summary-of-the-1 st -day-
	Yoshikatsu: Eto (Advanced Clinical Res. Center, Japan)
08:50-09:10↔	Chair: · · · David · G. · Warnock (Professor, University of Alabama at
4	Birmingham, ·US) ←
44	"CNS-treatment of Genetic disease"
09:10-09:20	Torayuki-Okuyama (NCCHD, Japan)
	Q&Ar2
09:20-10:00	Special-Lecture-III0000000-00000000000-Co-sponsor: Sanofi-K.K.
4	Chair: Yoshikatsu:Eto (Advanced Clinical Res. Center, Japan)
e	"Renal-manifestations-in-Fabry-disease"
44	David G. Warnock (Professor, University of Alabama at Birmingham,
10:00-10:10	US) 44
	Q&A+2
10:10-10:25	Break ^{e3}
10:25-10:45	Chair: Hiroshi-Kobayashi-(The-Jikei-University,-Japan)-
4	"Future clinical and biochemical predictions of Fabry disease in
€	females by methylation studies of the GLA gene."
4	Mohammad · Arif · Hossain · (Advanced · Clinical · Res. · Center, · Japan)
10:45-10:55	Q&A- ↔
10:55-11:15↔	Chair: · · Han-Wook · Yoo · (_Asan · Medical · Center, · South · Korea) ←
44	"Long-term-outcome of a group of Japanese children with myelin-
4	oligodendrocyte glycoprotein encephalomyelitis without preventive
ei .	immunosuppressive-therapy."

3) 国際ライソゾーム病 Web カンファレンス

NPO 法人日本ライソゾーム病センター主催



- 2. 患者会共催或いは支援活動
- 1. 全国ファブリ病患者と家族の会 年間5回





- 2. 全国ポンペ病患者と家族の会 年間1回
- 3. 各種患者啓蒙活動並びに相談―東京クリニックで行つている。