

The 12th International Collaborative Forum of Human Gene Therapy for Genetic Disease

Expanding clinical application of gene therapy for genetic diseases

Date

Jan. 20 (Thu.), 2022 9:00–17:40 ※Hybrid Meeting

Venue

The Jikei University School of Medicine, Building 1 Hall

President

Takanori Yamagata (Professor, Department of Pediatrics, Center for Gene Therapy Research, Jichi Medical University School of Medicine)

Registration Fee

Regular: 3,000 JPY Company: 10,000 JPY Student: Admission free (with verification card)

※In regarding to the registration, please check above URL or ask to the Secretariat.

※Registration: Dec. 15, 2021 – Jan. 19, 2022



Time	Program
9:00~ 9:15	Opening Remarks Takanori Yamagata (President of the 12th GT Forum) Yoshikatsu Eto (Executive Chairperson of the Committee)
9:15~10:15	Clinical experience of AAV-based gene therapy 1) Onasemnogene abeparvovec (Zolgensma®) for Spinal Muscular Atrophy: Clinical and Real World Experience Sandra P. Reyna (Novartis Gene Therapies, USA) 2) Intracisternal AAV9-based Gene Therapy for Neuronopathic Lysosomal Storage Diseases: Experience from a Clinical Trial Program Marie-Laure Névoret (REGENXBIO, Inc., USA)
10:15~10:55	Special lecture Gene Therapy and Genome Editing for Human Genetic Diseases James M. Willson (University of Pennsylvania, USA)
11:00~11:40	Special lecture Advancing gene therapies for the brain Beverly L. Davidson (University of Pennsylvania, USA)
11:40~12:40	Gene therapy for Haemophilia 1) Clinical Application of Gene Therapy for Hemophilia: Experience with fidanacogene elaparvovec and giroctocogene fitelparvovec Gregory Di Russo (Pfizer Inc., USA) 2) Gene therapy of hemophilia A Kevin Eggan (BioMarin Pharmaceutical Inc., USA)
12:45~13:45	Luncheon Seminar Takara Bio's efforts to address gene therapy vectors Tatsuji Enoki (Takara Bio Inc., Japan)
13:55~14:25	Efforts on Gene and Cell Therapy Projects at AMED Michiko Takakura (AMED, Japan)
14:25~15:25	Gene therapy for AADC deficiency 1) Gene therapy for AADC deficiency in Japan -Improved motor and mental function of various phenotype patients Karin Kojima (Jichi Medical University, Japan) 2) Gene therapy for AADC deficiency in Taiwan Wuh-Liang Hwu (National Taiwan University Hospital, Taiwan)
15:40~16:40	Ex vivo Gene therapy 1) Hematopoietic stem cell gene therapy with a lentiviral gene delivery Naoya Uchida (The Institute of Medical Science, The University of Tokyo, Japan) 2) Orchard Therapeutics and its Gene Therapy Programs Christopher Dott (Orchard Therapeutics Ltd., UK)
16:40~17:20	Special lecture Hematopoietic stem cell gene therapy for inborn errors of immunity and metabolism Aiuti Alessandro (San Raffaele Telethon Institute for Gene Therapy, Italy)
17:20~17:40	Closing Remarks Ryuichi Morishita (President of the JSGCT)

[Organizing] International Collaborative Forum of Human Gene Therapy for Genetic Disease

[Support] Japan Society of Gene and Cell Therapy [Co-sponsored] NPO Japan Lysosomal Disease Research Center

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