The 8th International Collaborative Forum of Human Gene Therapy for Genetic Diseases

Date: Thursday, January 18th, 2018

Venue: The Jikei University School of Medicine, 3rd floor Auditorium in 1st Building
3-19-18, Nishishimbashi, Minato-ku, Tokyo, 105-8471

President: Torayuki Okuyama, MD, PhD
(Director of Center for Lysosomal Storage Diseases, and Department of Clinical Laboratory Medicine, National Center for Child Health and Development (NCCHD))

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Theme: Clinical Reseach on Gene Therapy for Inherited Metabolic Diseases

❖ Opening Remarks and Welcome Greeting

Katsunobu Kato (Minister of Health, Labour and Welfare)
Torayuki Okuyama (President of the 8th Forum)
Yoshikatsu Eto (Executive Chairperson of the Committee)

❖ Symposium 1: Progress in Clinical Research of Gene Therapy in Japan

Gradual improvements in the motor and cognitive function after gene therapy for patients with AADC deficiency : Karin Kojima (Jichi Medical University)
Stem cell gene therapy for primary immunodeficiencies in Japan : Masafumi Onodera (NCCHD)

❖ Symposium 2: Progress in Gene Therapy by pharmaceutical companies in Japan

Development and Application of Stealth RNA Vector : Mahito Nakanishi
(National Institute of Advanced Industrial Science and Technology (AIST) /TOKIWA-Bio, Inc.)
HGF plasmid gene therapy for the treatment of critical limb ischemia : Ei Yamada (AnGes,Inc.)

❖ Corporate Seminar

Takara Bio Inc.

❖ Symposium 3: Gene therapy in the world: clinical trial by pharmaceutical companies

bluebird bio, Inc. (Dr. Gary Fortin) **: Interim results from a Phase 2/3 Study of the Efficacy and Safety of Ex Vivo Lenti-D™ Gene Therapy for the Treatment of Cerebral Adrenoleukodystrophy**
Orchard Therapeutics (Dr. Jesus Garcia-Segovia) **: Ex-vivo lentiviral mediated gene therapy for the treatment of paediatric primary immunodeficiencies and Sanfilippo syndrome**
Spark Therapeutics (Dr. Daniel C. Chung) **: Investigational Gene Therapy for RPE65-Mediated Inherited Retinal Disease**
Pfizer Japan Inc. (Kazuhiro Kanmuri) **: Gene Therapy to Drive Transformative Medicine for Intractable Diseases –Pfizer's Prospects on Approaches and Challenges Ahead-**

❖ Symposium 4: Gene Therapy for Inherited Metabolic Diseases

Novel therapeutic approach for the treatment of inherited and metabolic diseases : Torayuki Okuyama (NCCHD)
Current status of gene therapy for inborn error of metabolism: Toya Ohashi (The Jikei University)
Gene Therapy for Methylmalonic Acidemia (MMA) and Related Disorders : Lessons from Patients and Mice : Charles P. Venditti (National Institutes of Health)
Strategies for Effectively Treating Complex Lysosomal Storage Diseases : Mark S. Sands (Washington University in St. Louis)
Gene therapy approaches for MPS diseases : Simon A. Jones (Manchester University)

❖ Special Lecture

TBD : Chester Whitely (University of Minnesota)

❖ Closing Remarks and Welcome Greeting

Yasufumi Kaneda (President of the JSGCT)
Toya Ohashi (President of the 9th Forum)