

The 9th International Collaborative Forum of Human Gene Therapy for Genetic Disease

President | Toya Ohashi
Date | Thursday, January 17th, 2019
Venue | Auditorium, The Jikei University School of Medicine (Daigaku Ichigou-kan)
Location | 3-25-8, Nishi-Shimbashi, Minato-ku, Tokyo, 105-8461

Program

09:00-09:15 Opening Remarks and Welcome Greetings

President **Toya Ohashi** (The Jikei University School of Medicine)

09:15-10:15 Genome Editing Session

Chairs: **Yumi Kanegae** (The Jikei Univ. School of Medicine) & **Masafumi Onodera** (NCCHD)

1. **Molecular mechanism of CRISPR and structure-based development of genome editing tool toward medical applications**
Osamu Nureki (The University of Tokyo)
2. **Therapeutic potential of genome editing for genetic disorders**
Toru Uchiyama (NCCHD)

10:15-11:45 Foreign Biotech Companies I

Chairs: **Hiroshi Kobayashi** (The Jikei Univ. School of Medicine) & **Motomichi Kosuga** (NCCHD)

1. **Gene silencing in Huntington's disease**
Sander van Deventer (uniQure)
2. **Future Options for Patients with Fabry Disease: The Evolving Application of Lentiviral Gene Therapy**
Nerissa Kreher (Avrobio)

11:45-12:45 Corporate Seminar

Chair: **Hikaru Takakura** (Contract Service Business Development, TAKARA BIO INC.)

Gene transduced cell manufacturing and cell processing facility

Ikuei Nukaya (CDM Center Division 1, TAKARA BIO Inc.)

13:00-14:00 Novel Newborn Screening for Genetic Diseases

Chairs: **Torayuki Okuyama** (NCCHD) & **Akihiro Kume** (Jichi Medical University)

1. **Newborn Screening for Lysosomal Storage Diseases**
Torayuki Okuyama (NCCHD)
2. **Newborn Screening for X-linked Adrenoleukodystrophy**
Ryuichi Mashima (NCCHD)
3. **Newborn Screening for Spinal Muscular Atrophy**
Hisahide Nishino (Faculty of Rehabilitation Kobe Gakuin University)

14:00-15:00 Foreign Biotech Companies II

Chair: **Keiya Ozawa** (Jichi Medical University)

1. **Gene Therapy for Inherited Retinal Diseases**
Alison J. McVie Wylie (Sanofi)
2. **Preclinical assessment of valoctogene roxaparvovec (BMN270)**
Gordon Vehar (BioMarin)

15:00-15:30 Invited Lecture I

Chair: **Takanori Yamagata** (Jichi Medical University)

Regulation of Neural Crest Fate Specification for Regenerative Medicine

Ronald J. Parchem (Baylor College of Medicine)

15:30-15:40 Coffee Break I

15:40-16:40 Foreign Biotech Companies III

Chair: **Shin-ichi Muramatsu** (Jichi Medical University)

1. **AVXS-101 Gene Replacement Therapy in Spinal Muscular Atrophy**
Douglas M. Sproule (Avexis)
2. **AAV based Gene therapy for Hemophilia**
Friedrich Scheifflinger (Shire)

16:40-17:30 Invited Lecture II

Chair: **Toya Ohashi** (President of the 9th ICFHGTGD2019, The Jikei University School of Medicine)

"Title"

Luigi Naldini (San Raffaele Telethon Institute for Gene Therapy)

17:30-17:40 Coffee Break II

17:40-18:10 Evening Seminar

Chair: **Shin-ichi Takeda** (National Center of Neurology and Psychiatry)

Recent Advances of Lysosomal Storage Diseases-Toward to Gene Therapy

Yoshikatsu Eto (Advanced Clinical Research Center, Southern Tohoku Brain Research Center/Tokyo Jikei University School of Medicine)

18:10-18:30 Closing Remarks and Welcome Greetings from the President of JSGCT

Tomoki Todo (The Chairman of the Board of Directors of JSGCT, The Institute of Medical Science The University of Tokyo)

Yoshikatsu Eto (10th ICFHGTGD2020 President Elect)