

-The 24th Annual Meeting of JSGCT2018-
Venue: Toranomon Hills Forum, Tokyo

Time Table

(E: English Presentation, J: Japanese Presentation)

Day I Thursday, July 26

- 10:50~11:00 **Opening Remarks: President Masafumi Onodera**
- 11:10~12:00 **Presidential Special Program with AMED & PMDA (J)** *Chairperson: Jun Yoshida*
- Makoto Suematsu** (Japan Agency for Medical Research and Development)
“Mission of AMED: Empowering gene therapy for IRUD beyond”
- Chairperson: Takashi Shimada*
- Tatsuya Kondo** (Pharmaceuticals and Medical Devices Agency)
“PMDA’s initiatives based on regulatory science - In pursuit of developing gene therapies -”
- 12:00~13:00 Corporate Seminar I (AnGes, Inc.) *Chairperson: Masafumi Onodera*
(National Center for Child Health and Development)
- Ryuichi Morishita** (Osaka University, Graduate School of Medicine)
“Collategen as first gene therapy drug in Japan to stimulate therapeutic angiogenesis”
- Corporate Seminar II (Sanofi K. K.) *Chairperson: Fumio Endo*
(Kumamoto Ezuko Ryoiku Medical Center)
- Hideki Muramatsu** (Nagoya University Graduate School of Medicine)
“Newborn mass screening for severe combined immunodeficiency (SCID)”
- Torayuki Okuyama** (National Center for Child Health and Development)
“Early diagnosis for LSDs”
- Corporate Seminar III (Gene Therapy Research Institution Co., Ltd.) *Chairperson: Shin-ichi Muramatsu*
(Jichi Medical University, The Institute of Medical Science, The University of Tokyo, Gene Therapy Research Institution Co., Ltd.)
- Shin Kwak** (Gene Therapy Research Institution Co., Ltd., The University of Tokyo, and Tokyo Medical University)
“Possible application of gene therapy to sporadic neurological diseases - ALS as a prototype -”
- 13:00~14:00 General Assembly
- 14:00~14:50 **Plenary Session (PS-1~PS-3) (E)** *Chairpersons: Shin'ichi Takeda, & Toshihiko Wakabayashi*
- 14:50~15:10 **9th Takara Bio Award Lecture** *Chairperson: Yasufumi Kaneda*
- Fuminori Sakurai** (Osaka University Graduate School of Medicine)
“Neonatal gene therapy for hemophilia B by a novel adenovirus vector showing reduced leaky expression of viral genes”
- 15:10~17:00 **Symposium-I: -JCA-JSGCT joint Symposium “All about CAR-T cell therapy: current status and future perspective”- (E)** *Chairpersons: Keiya Ozawa & Koji Tamada*
- Keiya Ozawa** (Jichi Medical University)
“Overview of CAR-T cell therapy”
- Isabelle Rivière** (Michael G. Harris Cell Therapy and Cell Engineering Facility Center for Cell Engineering)
“CAR T cell therapy: The CD19 Paradigm and beyond”
- Naoki Hosen** (Osaka University Graduate School of Medicine)
“The activated conformation of integrin β7 is a novel multiple myeloma-specific target for CAR T-cell therapy”
- Koji Tamada** (Yamaguchi University Graduate School of Medicine)
“A novel platform technology of CAR-T cell therapy for solid tumors”
- 17:00~18:50 **Symposium II: -Genetic Diseases- (E)** *Chairpersons: Torayuki Okuyama & Toya Ohashi*
- Shin'ichi Takeda** (National Center of Neurology and Psychiatry)
“Exon skipping therapy for muscular dystrophy”
- Juan Ruiz** (Abeona Therapeutics, Inc.)
“Developing AAV gene therapies for rare genetic diseases”
- Karin Kojima** (Jichi Medical University)
“Gene therapy for child neurological diseases: Effective for AADC deficiency, new challenges for Glut1-deficiency syndrome and potential for other diseases”
- Alessandro Aiuti** (San Raffaele Institute, Milan, Italy)
“Genetic engineering of hematopoiesis to treat inherited diseases”

15:10~15:46 Oral Session I: “Basic Study I” (OR-4~OR-7) *Chairpersons: Koichi Miyake & Takafumi Nakamura*
 15:46~16:22 Oral Session II: “Basic Study II” (OR-8~OR-11) *Chairpersons: Yutaka Hanazono & Mahito Nakanishi*
 16:22~16:58 Oral Session III: “Neurologic Diseases I” (OR-12~OR-15) *Chairpersons: Karin Kojima & Takanori Yokota*
 16:58~17:25 Oral Session IV: “Neurologic Diseases II” (OR-16~OR-18) *Chairpersons: Yasuhiro Ikeda & Takanori Yamagata*
 17:25~17:52 Oral Session V: “Vector I” (OR-19~OR-21) *Chairpersons: Fuminori Sakurai & Masashi Urabe*
 17:52~18:19 Oral Session VI: “Cardiovascular Diseases I” (OR-22~OR-24) *Chairpersons: Yozo Nakazawa & Yukihiro Saito*
 18:19~18:46 Oral Session VII: “Cardiovascular Diseases II” (OR-25~OR-27) *Chairpersons: Hiroaki Mizukami & Hironori Nakagami*

18:50~19:30 **Presidential Evening Symposium I (E)** *Chairperson: Masafumi Onodera*

Christopher Dott (Orchard Therapeutics)
 “Orchard therapeutics and its gene therapy programs”

Tohru Hirose (Novartis Pharma K.K.)
 “Development of gene therapy related treatments in Novartis”

19:30~20:30 **Get-Together**

Day II Friday, July 27

08:30~09:50 **Symposium-III: -Regulatory Science- (J)** *Chairpersons: Akihiro Kume & Eriko Uchida*

Takashi Ichikawa (Ministry of Health, Labour and Welfare)
 “Revised ethical guideline for clinical trials on gene therapy”

Teruhide Yamaguchi (Kanazawa Institute of Technology/ Nihon Pharmaceutical University)
 “Revision of guideline for gene therapy clinical study based on the novel technology of gene editing”

Megumi Kawamoto (Pharmaceuticals and Medical Devices Agency)
 “Points to consider in quality and preclinical aspects of gene therapy products”

08:30~09:30 **Symposium IV: -JSGCT Working Group Symposium “The Advancement in Innovative Approaches for Next-generation Gene Therapy”- (J)**

Chairpersons: Makoto Otsu & Fuminori Sakurai

Satoshi Nagano (Kagoshima University)
 “First-in-human investigator-initiated clinical trial of survivin-responsive conditionally replicating adenovirus for treating bone and soft tissue sarcoma”

Kayoko Nakamura (Shinshu University Hospital)
 “Chimeric antigen receptor T cells redirecting EPHB4 for the treatment of human rhabdomyosarcoma”

Shinichiro Nakada (Graduate School of Medicine, Osaka University)
 “Precise gene editing by SNGD”

09:30~10:15 Oral Session VIII: “Cancer I” (OR-28~OR-32)

Chairpersons: Hiroshi Fukuhara & Hiroki Nanba

10:15~11:10 Oral Session IX: “Cancer II” (OR-33~OR-37)

Chairpersons: Kazunori Aoki & Yasushi Ino

09:50~11:10 **Special Symposium I (E)**

Chairpersons: Masafumi Onodera & Tomoki Todo

Alessandro Aiuti (San Raffaele Institute, Milan, Italy, ESGCT)
 “Gene therapy for Wiskott-Aldrich syndrome”

Guangping Gao (University of Massachusetts Medical School, ASGCT President Elect)
 “Human gene therapy - The road to today and beyond”

11:10~11:40 **JSGCT Chairman’s Lecture (J)**

Chairperson: Shigetaka Asano

Yasufumi Kaneda (Chairman of JSGCT, Graduate School of Medicine, Osaka University)
 “Japan should accelerate human gene therapy without delay”

11:40~12:00 **Presidential Lecture (E)**

Chairperson: Yoshikatsu Eto

Masafumi Onodera (President of JSGCT2018, National Center for Child Health and Development)
 “Gene and cell therapy, to take one step forward”

12:00~13:00 Corporate Seminar IV (Miltenyi Biotec)

*Chairperson: Keiya Ozawa
 (Jichi Medical University)*

Hiroshi Shiku (Mie University Graduate School of Medicine)
 “Adoptive cell therapy with gene-engineered T-cells - Specificity, construct, and cells -”

Corporate Seminar V (SHIMADZU CORPORATION)

*Chairperson: Kenzaburo Tani
 (The Institute of Medical Science, The University of Tokyo)*

Shin Kawamata (Research and Development Center for Cell Therapy, Foundation for Biomedical Research and Innovation)
 “A new quality assurance method for cell manufacturing through in-process monitoring”

- 13:00~13:40 **Educational Lecture (E)** *Chairpersons: Yoshiro Niitsu*
- Osamu Nureki** (The University of Tokyo)
“Molecular mechanism of CRISPR and structure-based development of genome editing tool towards medical applications”
- Ko Mitani** (Saitama Medical University)
“Genome editing for therapeutic applications”
- 13:00~14:10 **Symposium V: -Vector “GeneRide and HITI: Cutting Edge Technologies in Vectors and Genome Editing”- (E)**
Chairpersons: Hiroyuki Nakai & Takashi Okada
- Nelson Chau** (LogicBio Therapeutics)
“GeneRide™, a novel AAV-mediated strategy to treat pediatric indications”
- Keiichiro Suzuki** (Osaka University)
“Genome-editing therapy via HITI (Homology-Independent Targeting Integration) method”
- 13:40~15:20 **Special Symposium II: -Biosafety Regulations in Gene Therapy- (J)**
Chairpersons: Masafumi Onodera & Teruhide Yamaguchi
- Eriko Uchida** (National Institute of Health Sciences)
“Environmental assessments and shedding studies for gene therapy products in the US and in the EU”
- Kazunobu Oyama** (Pharmaceuticals and Medical Devices Agency)
“Points to consider for environmental risk assessment of the use of living modified organisms; PMDA perspectives on type 1 use approval and type 2 use confirmation under “Cartagena Law””
- 14:10~15:20 **Symposium-VI: -Genome Editing “Genome editing: Step to gene therapy”- (E)**
Chairpersons: Akitsu Hotta & Yumi Kanegae
- Harry L. Malech** (National Institutes of Health)
“CRISPR-mediated correction of CD34+ hematopoietic stem cells from patients with chronic granulomatous disease”
- Tsukasa Ohmori** (Jichi Medical University School of Medicine)
“Development of genome-editing treatment for hemophilia”
- Akitsu Hotta** (CiRA, Kyoto University)
“Genome editing and iPS cell mediated gene therapy: how to overcome *in vivo* delivery and *ex vivo* immunorejection”
- 15:30~17:20 **International Symposium -Steps forward to Clinic in Gene and Cell Therapy of Genetic Diseases- (E)**
Chairpersons: Noriyuki Kasahara & Masato Yamamoto
- Daniel C. Chung** (Spark Therapeutics)
“Development of Luxturna™ (voretigene neparvovec-rzyl): Gene therapy for *RPE65* biallelic mutation associated inherited retinal disease”
- Michele De Luca** (University of Modena and Reggio Emilia)
“YAP/TAZ-dependent depletion of human epidermal stem cells in junctional epidermolysis bullosa”
- Harry L. Malech** (National Institutes of Health)
“Lentivector gene therapy for X-linked chronic granulomatous disease.”
- 15:30~16:06 Oral Session X: “Vector II” (OR-38~OR-41) *Chairpersons: Ken-ichiro Kosai & Junichi Mineno*
- 16:06~16:42 Oral Session XI: “Genetic Diseases I” (OR-42~OR-45) *Chairpersons: Hiroshi Kobayashi & Toru Uchiyama*
- 16:42~17:09 Oral Session XII: “Genetic Diseases II” (OR-46~OR-48) *Chairpersons: Yukihiko Hirai & Izumu Saito*
- 17:20~17:50 **Poster Discussion: (PO-49~PO-84)**
- Poster Session I: “Basic Science” (PO-49~PO-52) *Chairperson: Hideki Kasuya*
- Poster Session II: “Basics Science and Neuronal Diseases” (PO-53~PO-56) *Chairperson: Noriko Miyake*
- Poster Session III: “Vector I” (PO-57~PO-61) *Chairperson: Kenya Kamimura*
- Poster Session IV: “Vector II” (PO-62~PO-67) *Chairperson: Yuji Heike*
- Poster Session V: “Cardiovascular Diseases” (PO-68~PO-71) *Chairperson: Yuko Kasahara*
- Poster Session VI: “Genetic Diseases” (PO-72~PO-75) *Chairperson: Toshinao Kawai*
- Poster Session VII: “Cancer I” (PO-76~PO-79) *Chairperson: Atsushi Natsume*
- Poster Session VIII: “Cancer II” (PO-80~PO-84) *Chairperson: Shuji Kubo*
- 18:00~18:20 **Presidential Evening Symposium II** *Chairperson: Masafumi Onodera*
- Takayuki Imaeda** (Pfizer Inc.)
“Gene therapy (GTx): A path to deliver transformative medicine to patients”
- 18:20~18:50 Evening Seminar (Takara Bio Inc.) *Chairperson: Junichi Mineno*
(Takara Bio Inc.)
- Masanobu Kimura** (Takara Bio Inc.)
“Current clinical development of gene and cellular therapy in Japan”
- 19:00~20:50 **Welcome Reception**

Day III Saturday, July 28

- 08:30~10:15 **Symposium-VII: -Regenerative Medicine “Interface between gene therapy and regenerative medicine”- (E)**
Chairpersons: Katsuto Tamai & Toru Uchiyama
- Michele De Luca** (University of Modena and Reggio Emilia)
“Life-saving regeneration of the entire human epidermis by transgenic stem cells”
- Katsuto Tamai** (Osaka University)
“Circulating mesenchymal stem cells: their function and possibility as a target of gene therapy.”
- Takumi Era** (Institute of Molecular Embryology and Genetics, Kumamoto University)
“Origin and development of mesenchymal stem cells”
- Tomoyuki Yamaguchi** (Institute of Medical Science, University of Tokyo)
“Interspecies organogenesis generates autologous functional islets”
- 08:30~10:15 **Symposium-VIII: -GCTP Marketing “Extracting issues toward Social Implementation of Cell & Gene therapy: What is proper price of the products ?”- (J)**
Chairpersons: Masanobu Kimura & Toshihiko Okazaki
- Hisako Hashimoto** (Foundation for Biomedical Research and Innovation at Kobe)
“Roadmap for industrialization of cell-based therapy from academia to clinical sites. Importance of the design of the cell manufacturing system by academia applicable to industrialization.”
- Junichi Mineno** (TAKARA BIO INC.)
“Manufacturing of cellular and tissue-based products”
- Tadashi Sameshima** (Terumo Corp.)
“Development of HeartSheet and current issue”
- Yoji Sato** (National Institute of Health Sciences)
“Quality of biological raw materials for manufacturing of cell-based and gene therapy products”
- 10:15~12:00 **Symposium-IX: -Neurologic Diseases “Nervous system”- (E)**
Chairpersons: Hideki Mochizuki & Shin-ichi Muramatsu
- Kohji Itoh** (Tokushima University)
“Gene therapy for GM2 gangliosidosis with CNS involvement”
- Seiichi Nagano** (Osaka University)
“Gene therapy for ALS”
- Atsumi Nitta** (University of Toyama)
“Effects of overexpression of TMEM-induced by AAV on the behavioral changes of the drug dependence and anxiety”
- Takuro Horii** (Gunma University)
“Mouse modeling by genome and epigenome editing”
- 10:15~12:00 **Symposium-X: -Oncolytic Virus “For clinical implementation of oncolytic virotherapy”- (E)**
Chairpersons: Toshiyoshi Fujiwara & Kenzaburo Tani
- Teruhide Yamaguchi** (Kanazawa Institute of Technology, Nihon Pharmaceutical University)
“Current issues on quality and safety of oncolytic virus products”
- Shohei Miyamoto** (IMS, The University of Tokyo)
“The current preparative status of oncolytic coxsackievirotherapy toward clinical trial”
- Hiroshi Tazawa** (Okayama University Hospital)
“Recent advances in clinical application of telomerase-specific oncolytic viruses”
- Maki Tanaka** (Takara Bio Inc)
“Clinical evaluation of a novel oncolytic viral immunotherapy agent, Canerpaturev (HF10)”
- Tomoki Todo** (The Institute of Medical Science, The University of Tokyo)
“Clinical development of third generation oncolytic HSV-1 G47Δ”
- 12:00~13:00 Corporate Seminar VI (Takara Bio Inc.) *Chairperson: Junichi Mineno (Takara Bio Inc.)*
- Keiya Ozawa** (Jichi Medical University)
“Gene therapy comes of age”
- Corporate Seminar VII (AnGes, Inc.) *Chairperson: Yoshikatsu Eto (Advanced Clinical Research Center, Institute for Neurological Disorders)*
- Torayuki Okuyama** (National Center for Child Health and Development)
“Novel therapeutic approach in lysosomal storage diseases”
- 13:00~14:50 **Symposium-XI: -Cardiovascular Diseases “Cardiovascular Gene & Cell Therapy and Genome Editing: a recent progress”- (E)**
Chairpersons: Ryuichi Morishita & Yoshikazu Yonemitsu
- Yoshikazu Yonemitsu** (Kyushu University Graduate School of Pharmaceutical Sciences)
“Opening remarks: Recent progress of cardiovascular gene & cell therapy and gene editing”
- Hidemasa Oh** (Okayama University Hospital)
“Cell therapy trials in congenital heart disease”
- Yuichi Oike** (Kumamoto University)
“Identification of candidate genes for gene therapy against heart failure”
- Hironori Nakagami** (Osaka University Graduate School of Medicine)
“Development of novel immunotherapy for cardiovascular diseases”

Haruhiko Morita (EdiGENE Corporation)

“Frontline of gene editing application”

Ryuichi Morishita (Osaka University Graduate School of Medicine)

“Closing remarks”

13:00~14:50 **Symposium-XII: -Cancer Gene Therapy “Gene medicine going into a translational phase”- (E)**

Chairpersons: Yasutomo Nasu & Masatoshi Tagawa

Nicholas A. Boyle (Tocagen Inc.,)

“Toca 511 & Toca FC: Evaluation of durable response rate in the post-resection setting and association with survival in patients with recurrent high grade glioma”

Toshihiro Nakajima (Genomidea, Inc.)

“Clinical development of non-replicating oncolytic virus (HVJ-E/GEN0101) for the treatment of solid cancers”

Yuji Tada (School of Medicine Chiba university)

“A phase I clinical trial of inhibiting the HGF/c-Met pathway for malignant pleural mesothelioma with NK4 gene-expressing adenoviral vectors”

Kazuhiko Kurozumi (Okayama University)

“A phase I/II clinical trial of Ad-SGE-REIC for malignant brain tumor”

Hitoshi Shiomi (Momotaro Gene Inc.,)

“The current status of clinical trials of Ad-SGE-REIC gene therapy”

14:50~15:00 **Closing Remarks: President Masafumi Onodera &**

Welcome Greetings from the President-Elect Tomoki Todo