

**THE 22<sup>nd</sup> ANNUAL MEETING**

**JSGCT2016**

**JAPAN SOCIETY OF GENE AND CELL THERAPY**

**Program & Abstracts**

**Date**

**July 28-30, 2016**

**Venue**

**TORANOMON HILLS FORUM**

**Mori Tower 5<sup>th</sup> Floor, 1-23-3 Toranomom,  
Minato-ku, Tokyo 105-6305, Japan**

**TOKYO**



# PROGRAM

**Presidential Lecture (E)**

Date: July 29, 2016, 11:40-12:00, Hall-1  
 Chairperson: Yoshikatsu Eto

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

**Current treatment and gene therapy for lysosomal storage diseases**

**JSGCT Chairman's Lecture (E)**

Date: July 28, 2016, 14:00-14:30, Hall-1  
 Chairperson: Jun Yoshida

**Yasufumi Kaneda** (*Graduate School of Medicine, Osaka University, Chairman of JSGCT*)

**Mission of JSGCT for gene therapy prosperity in future**

**President's Special Program  
 with AMED, Ministry of Health, Labour and Welfare (J)**

Date: July 29, 2016 11:00-11:40, Hall-1  
 Chairperson: Toshiyoshi Fujiwara

**Makoto Suematsu** (*Japan Agency for Medical Research and Development*)

**AMED develops medical arts for patients with rare and undiagnosed diseases**

Chairperson: Shigetaka Asano

**Kazuhiko Mori** (*Ministry of Health, Labour and Welfare*)

**Current situation for encouraging realization of gene and cell therapy**

**Educational Lecture I (E)**

Date: July 28, 2016 14:30-15:10, Hall-1  
 Chairperson: Toya Ohashi

**Yoshikatsu Eto** (*Advanced Clinical Research Center, Institute of Neurological Disorders, Kanagawa*)

**Nobel treatment strategies including gene therapy for genetic neurological disorders**

**Educational Lecture II (E)**

Date: July 28, 2016 15:10-15:50, Hall-1  
 Chairpersons: Takashi Shimada

**Patrick Aubourg** (*INSERM, France*)

**Gene therapy for X-linked adrenoleukodystrophy and metachromatic leukodystrophy**

**Educational Lecture III (E)**

Date: July 29, 2016 13:00-13:40, Hall-1  
 Chairpersons: Yutaka Hanazono & Izumu Saito

**Kohei Miyazono** (*Graduate School of Medicine, The University of Tokyo*)

**TGF- $\beta$  signaling: Mechanisms of action and clinical application**

**Educational Lecture IV (E)**

Date: July 29, 2016 13:00-13:40, Hall-2  
 Chairpersons: Hiroshi Kobayashi & Eriko Uchida

**Naomichi Matsumoto** (*Yokohama City University Graduate School of Medicine*)

**Rare variants in human diseases**

**Special Lecture (E)**

Date: July 30 2016, 11:30-12:00, Hall-1  
 Chairperson: Takashi Okada

**Daniel Chung** (*Spark Therapeutics, Inc.*)

**Gene therapy for RPE65-mediated inherited retinal disease**

## Main Symposium (E)

Date: July 29, 2016, 9:50-11:00, Hall-1  
Chairpersons: Yasufumi Kaneda & Yasutomo Nasu

MS-1. Cynthia E. Dunbar (*NIH, ASGCT President*)

**Investigating stem cell biology and therapeutic potential in the rhesus macaque model**

MS-2. Robin R. Ali (*UCL Institute of Ophthalmology, ESGCT President*)

**Improved daylight vision in mice following gene therapy to increase the speed of rod photoreceptors**

## International Symposium (E)

Date: July 29, 2016 13:40-15:30, Hall-1  
Chairpersons: Noriyuki Kasahara & Masato Yamamoto

IS-1. Jeffrey Medin (*Medical College of Wisconsin, USA*)

**Modulation of the local tumor micro-environment by LV-mediated expression of IL-12 in primary AML cells**

IS-2. Harald Petry (*UniQure, The Netherlands*)

**Establishing gene therapy in the clinic: From bench to bedside**

IS-3. Michael C. Holmes (*Sangamo BioSciences, USA*)

**Genome editing in primary human cells and organs: Towards the goal of engineering genetic cures**

IS-4. Douglas J. Jolly (*Tocagen Inc., USA*)

**Ascending dose trials of a retroviral replicating vector (Toca 511) in patients with recurrent high-grade glioma: clinical update, molecular analyses, and proposed mechanism of action**

## Symposium I (E)

ICFHGTGD -JSGCT Joint Symposium

-Genetic Diseases-

\*ICFHGTGD: International Collaborative Forum of Human Gene Therapy for Genetic Disease

Date: July 28, 2016, 16:00-18:10, Hall-1

Chairpersons: Torayuki Okuyama & Masafumi Onodera

S1-1. Wuh-Liang Hwu (*National Taiwan University Hospital*)

**Gene therapy for AADC deficiency: toward a standard therapy**

S1-2. Hiroshi Kobayashi (*The Jikei University School of Medicine*)

**Ex-vivo gene therapy, as a promising approach for the treatment of central nervous system of Mucopolysaccharidosis type II murine model**

S1-3. Toru Uchiyama (*National Center for Child Health and Development*)

**Hematopoietic stem cell gene therapy for Wiskott-Aldrich syndrome**

S1-4. Takanori Yamagata (*Jichi Medical University*)

**Development and clinical application of gene therapy using AAV for child neurological diseases**

S1-5. Yasuhiro Ikeda (*Graduate School of Medical Sciences, Kyushu University*)

**Gene therapy for inherited retinal degenerative diseases**

## Symposium II (J)

-Regulatory Science-

Date: July 29, 2016, 8:30-9:50, Hall-1

Chairpersons: Akihiro Kume & Teruhide Yamaguchi

**S2-1. Yoshiro Meguro** (*Japan Agency for Medical Research and Development*)  
**AMED programs to promote innovative drug development**

**S2-2. Jun Matsumoto** (*Japan Agency for Medical Research and Development*)  
**AMED support for medical R&D projects**

**S2-3. Daisaku Sato** (*Pharmaceuticals and Medical Devices Agency*)  
**Regulatory perspective on cell and gene therapy products**

**S2-4. Hisashi Koike** (*Pharmaceuticals and Medical Devices Agency*)  
**Introduction of pharmaceutical affairs consultation on R&D strategy and its effective utilization**

### **Symposium III (E)**

**-Genome Editing-**

Date: July 29, 2016, 13:40-14:40, Hall-2

Chairpersons: Yumi Kanegae & Konosuke Mitani

**S3-1. Osamu Nureki** (*Graduate School of Science, The University of Tokyo*)  
**Structure-based development of genome-editing tool, CRISPR-Cas9 towards medical applications**

**S3-2. Akitsu Hotta** (*Center for iPS cell Research & Application (CiRA), Kyoto University*)  
**Genome editing gene therapy for Duchenne Muscular Dystrophy**

**S3-3. Konosuke Mitani** (*Saitama Medical University*)  
**Recent progress and remaining challenges in therapeutic genome editing**

### **Symposium IV (E)**

**-Cancer-**

Date: July 29, 2016, 15:40-17:40, Hall-1

Chairpersons: Kenzaburo Tani & Tomoki Todo

**S4-1. Toshiyoshi Fujiwara** (*Okayama University Graduate School of Medicine*)  
**Next-generation telomelysin: multifunctional telomerase-specific oncolytic adenovirus armed with the wild-type *p53* tumor suppressor gene**

**S4-2. Yasushi Ino** (*The Institute of Medical Science, The University of Tokyo*)  
**Clinical trials of oncolytic HSV-1 G47Δ for patients with glioblastoma**

**S4-3. Kerry Fisher** (*PsiOxus Therapeutics Limited, United Kingdom*)  
**Systemic delivery of Enadenotucirev, a group B oncolytic adenovirus for the treatment of cancer**

**S4-4. Swami Murugappan** (*Amgen Inc.*) **Kenzaburo Tani** (*The Institute of Medical Science, The University of Tokyo*)  
**Talimogene laherparepvec: Harnessing herpes virus for the treatment of advanced melanoma**

**S4-5. Kenzaburo Tani** (*The Institute of Medical Science, The University of Tokyo*)  
**Development of new oncolytic virotherapies for malignancies**

### **Symposium V (E)**

**-Cardiovascular Diseases-**

Date: July 30, 2016, 8:30-10:30, Hall-2

Chairpersons: Ryuichi Morishita & Yoshikazu Yonemitsu

**S5-1. Toyoaki Murohara** (*Nagoya University Graduate School of Medicine*)  
**Wnt11 gene therapy with adeno-associated virus 9 improves recovery from myocardial infarction**

**S5-2. Keiichi Fukuda** (*Keio University School of Medicine*)  
**Clinical application of human iPS cells for the treatment of patients with severe congestive heart failure**

**S5-3. Yoshikazu Yonemitsu** (*Graduate School of Medical Sciences, Kyushu University*)  
**Current status of clinical development of human FGF-2-expressing recombinant Sendai virus vector (DVC1-0101) to treat peripheral arterial disease**

**S5-4. Yukihiro Saito** (*Asahikawa Medical University*)  
**Gene therapy for lymphedema**

**S5-5. Hiroshi Koriyama** (*Graduate School of Medicine, Osaka University*)  
**The development of IL-17A vaccine**

**S5-6. Jun-ichi Suzuki** (*Department of Advanced Clinical Science and Therapeutics, The University of Tokyo*)  
**Pivotal roles of inflammatory factors in vascular disease and its specific regulation**

**Symposium VI (E)**  
JSRM-JSGCT Joint Symposium  
-Cell Therapy-

\*JSRM: The Japanese Society for Regenerative Medicine  
Date: July 30, 2016, 10:40-12:00, Hall-2  
Chairpersons: Tokiko Nagamura & Makoto Otsu

**S6-1. Osamu Honmou** (*Sapporo Medical University*)  
**Intravenous transplantation of autologous mesenchymal stem cells derived from bone marrow into stroke patients**

**S6-2. Taro Matsumoto** (*Nihon University School of Medicine*)  
**Dedifferentiated fat cells as a new cell source for therapeutic angiogenesis and regenerative medicine**

**S6-3. Tokiko Nagamura** (*The Institute of Medical Science, The University of Tokyo*)  
**Umbilical cord is a rich source of mesenchymal stromal cells for cell therapies**

**Symposium VII (E)**  
JCA-JSGCT Joint Symposium  
-Cancer Immunotherapy-

\*JCA: Japanese Cancer Association  
Date: July 30, 2016, 13:00-14:50, Hall-1  
Chairpersons: Keiya Ozawa & Koji Tamada

**S7-1. Koji Tamada** (*Yamaguchi University Graduate School of Medicine*)  
**New era of cancer immunotherapy begins: A role of gene-modified cell therapy**

**S7-2. Hiroshi Fujiwara** (*Ehime University Graduate School of Medicine*)  
**Clinical study of novel adoptive immunotherapy using gene-modified autologous lymphocytes to express WT1 specific T-cell receptor against myeloid leukemia**

**S7-3. Ken Omine** (*Jichi Medical University*)  
**Clinical development of CD19-CAR therapy in patients with B-cell malignancy**

**S7-4. Yutaka Kawakami** (*Keio University School of Medicine*)  
**Future perspectives of cancer immunotherapy: Towards personalized combination cancer immunotherapy**

**Symposium VIII (E)**  
-Neurological and Muscle Diseases-

Date: July 30, 2016, 13:00-14:50, Hall-2  
Chairpersons: Hideki Mochizuki & Shin-ichi Muramatsu

- S8-1. Krystof Bankewicz (*University of California*)  
**Surgical coverage of the putamen in Parkinson's disease with AAV2-AADC using MRI-guided convective delivery**
- S8-2. Hitoshi Okazawa (*Tokyo Medical and Dental University*)  
**Gene therapy to conquer spinocerebellar ataxia**
- S8-3. Shin Kwak (*Graduate School of Medicine, The University of Tokyo*)  
**Targeting therapy for sporadic ALS**
- S8-4. Masayuki Nakamori (*Graduate School of Medicine, Osaka University*)  
**Antisense oligonucleotide therapy for neurological and neuromuscular disorders**

**Technical Seminar (J)**  
**-AAV, Lentivirus Vector-**

Date: July 29, 2016, 8:30-9:10, Hall-2  
 Chairperson: Hiroyuki Miyoshi

- TS-1. Takashi Okada (*Nippon Medical School*)  
**Attractive characteristics of self-complementary AAV vectors and improved methods for efficient production**
- TS-2. Hiroyuki Miyoshi (*Keio University School of Medicine*)  
**Clinical use of lentiviral vectors**
- TS-3. Yumi Kanegae (*The Jikei University School of Medicine*)  
**The application of genome editing for gene therapy**

**The 2<sup>nd</sup> International Symposium of the Asia-Pacific Gene Therapy Consortium (E)**

(\*This Symposium is free of charge to attend but limited to those who have paid their JSGCT2016 Annual Meeting's registration fee.)

Date: July 30, 2016, 8:30-11:30, Hall-1  
 Chairpersons: Masatoshi Tagawa & Chae-Ok Yun

- AP-1. Ian E. Alexander (*Children's Medical Research Institute and the Sydney Children's Hospitals Network, Australia*)  
**AAV vectors as tools for liver-targeted gene therapy: efficacy and safety**
- AP-2. Wenlin Huang (*Sun Yat-Sen University Cancer Center, China*)  
**Multicenter randomized phase II/III clinical trial of a recombinant human endostatin adenovirus for cancer gene therapy**
- AP-3. Won Jong Kim (*Pohang University of Science and Technology (POSTECH), Korea*)  
**Smart nanoparticles for drug, gene and gas delivery**
- AP-4. Shin-ichi Muramatsu (*Jichi Medical University, Japan*)  
**Gene therapy for central nervous system disorders**

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**7<sup>th</sup> Takara Bio Award Lecture**

Date: July 29, 2016, 11:45-12:00, Hall-1  
 Chairperson: Yasufumi Kaneda

- Mitsuhiro Machitani (*Graduate School of Pharmaceutical Sciences, Osaka University*)  
**NF-κB promotes leaky expression of adenovirus genes in a replication-incompetent adenovirus vector**

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## Plenary Session (E) (Abstracts PS-1~PS-3)

Date: July 28, 2016, 11:00-11:45, Hall-1  
Chairpersons: Yoshiro Niitsu & Toshihiko Wakabayashi

### PS-1. Improved motor and cognitive function of Aromatic L-Amino acid Decarboxylase (AADC) deficient patients following gene therapy using the AAV2 vector

*Kojima K., Miyauchi A., Nakajima T., Asari-Ono S., Saga Y., Mizukami H., Sato T., Muramatsu S., Osaka H., Yamagata T.*

### PS-2. INVOSSA™ (TissueGene-C) in patients with osteoarthritis: A clinical study of phase III

*Cho J. J., Kim T. W., Min S. J., Suk K., Lee B.*

### PS-3. Systemic treatment of mesothelin-targeted oncolytic adenovirus eliminates pancreatic cancer

*Sato-Dahlman M., Miura Y., Hajeri P., Jacobsen K., Davydova J., Yamamoto M.*

## Day 1: July 28, 2016

### Oral Session I Neurologic Diseases-1 (Abstracts OR-4~OR-7)

14:30-15:10, Hall-2  
Chairpersons: Yasuhiro Ikeda & Shin'ichi Takeda

#### OR-4. Cell therapeutic approach using dental pulp stromal cells for Duchenne muscular dystrophy

*Nitahara-Kasahara Y., Kuraoka M., Hayashita-Kinoh H., Nakamura-Takahashi A., Masuda C., Imagawa K., Tachibana K., Takeda S., Okada T.*

#### OR-5. Systemic administration of rAAV9-microdystrophin with MSCs pre-treatment improves transgene expression and phenotype in CXMD<sub>1</sub>

*Hayashita-Kinoh H., Nitahara-Kasahara Y., Kuraoka M., Okada H., Imagawa K., Tachibana K., Takeda S., Okada T.*

#### OR-6. AAV-mediated efficient transduction of the nonhuman primate retina by surgical internal limiting membrane (ILM) peeling

*Takahashi K., Igarashi T., Miyake K., Kobayashi M., Yaguchi C., Iijima O., Yamazaki Y., Miyake N., Kameya S., Shimada T., Takahashi H., Okada T.*

#### OR-7. An approach to compare multiple adeno-associated virus (AAV) capsids side-by-side in a single eye following intravitreal injection

*Powers J. M., Adachi K., Sivyer B., Taylor W. R., Pennesi M. E., Nakai H.*

### Oral Session II Neurologic Diseases-2 (Abstracts OR-8~OR-10)

15:10-15:40, Hall-2  
Chairpersons: Shoji Tsuji & Takanori Yamagata

#### OR-8. Gene therapy for a mouse model of glucose transporter-1 deficiency syndrome.

*Nakamura S., Osaka H., Muramatsu S., Takino N., Aoki S., Jimbo E. F., Shimazaki K., Onaka T., Ohtsuki S., Terasaki T., Yamagata T.*

#### OR-9. Development of LNA Gapmer oligonucleotide based therapy for FTD/ALS caused by the C9ORF72 repeat expansion

*Aoki Y., Manzano R., Takeda S., Ohbayashi N., Fukuda M., Talbot K., Wood M. J. A.*

#### OR-10. Necdin prevents dopaminergic neurodegeneration in experimental Parkinson disease

*Mochizuki H., Yasuda T., Yoshikawa K.*



**Oral Session III**  
**Cancer-1 (Abstracts OR-11~OR-15)**

16:00-16:50, Hall-2

*Chairpersons: Yasushi Ino & Takafumi Nakamura*

**OR-11. Oncolytic herpes simplex virus type 1 G47 $\Delta$  is efficacious in mouse colon cancer models and induces immunogenic cell death**

*Abe S., Ino Y., Fukuhara H., Iwai M., Watanabe T., Todo T.*

**OR-12. Generation and evaluation of an oncolytic herpes simplex virus expressing an antibody as a therapeutic molecule**

*Ito H., Ino Y., Fukuhara H., Iwai M., Todo T.*

**OR-13. Retroviral replicating vector-mediated gene therapy induced anti-tumor immunity in an experimental glioma model**

*Inagaki A., Hiraoka K., Kato Y., Huang T. T., Kamijima S., Takahashi M., Matsumoto H., Hacke K., Kruse C. A., Ostertag D., Gruber H. E., Jolly D. J., Robbins J. M., Kasahara N.*

**OR-14. Analysis of tumor infiltrating cellular immune compartments in combination treatment of HF10 with anti-CTLA-4 antibody.**

*Tsuda H., Furui S., Chono H., Tanaka M., Ikeda H., Shiku H., Mineno J.*

**OR-15. Novel recombinant coxsackievirus B3 infection shows potent oncolytic activity against human non-small cell lung cancer and triple-negative breast cancer**

*Sagara M., Miyamoto S., Takishima Y., Shimizu H., Nakanishi Y., Tani K.*

**Oral Session IV**  
**Cancer-2 (Abstract OR-16-OR-19)**

16:50-17:30, Hall-2

*Chairpersons: Hideki Kasuya & Toshiro Shirakawa*

**OR-16. Inhibition of peritoneal dissemination of scirrhus gastric cancer by oncolytic adenovirus-mediated activation of tumor suppressor p53 and suppression of oncogenic receptor tyrosine kinases**

*Tazawa H., Hori N., Koujima T., Kuwada K., Tanimoto T., Kuroda S., Nishizaki M., Urata Y., Kagawa S., Fujiwara T.*

**OR-17. Impairment of KRAS-driven pancreatic cancer growth and invasion by tumor-specific oncolytic adenoviruses**

*Koujima T., Tazawa H., Kuwada K., Tanimoto T., Kuroda S., Kishimoto H., Nishizaki M., Urata Y., Kagawa S., Fujiwara T.*

**OR-18. Overexpression of SOCS-1 with adenoviral vector enhances anti tumor immunity of T-cell and inhibits tumor growth in ovarian cancer.**

*Nakagawa S., Serada S., Ueda Y., Yoshino K., Fujimoto M., Kimura T., Naka T.*

**OR-19. CXCL-2 gene-loaded HVJ-E suppressed mouse melanoma by increasing T-cell-mediated anti-tumor immunity through the accumulation of anti-tumorigenic neutrophils in tumor microenvironment.**

*Chang C. Y., Tai J. A., Kaneda Y.*

**Oral Session V**  
**Cancer-3 (Abstracts OR-20~OR-23)**

17:30-18:10, Hall-2

*Chairpersons: Junichi Mineno & Yozo Nakazawa*

**OR-20. CD269-targeted CAR-modified T cells showed promising therapeutic efficacy in an orthotopic mouse model of multiple myeloma.**

*Uchibori R., Teruya T., Ohmine K., Urabe M., Saga Y., Mizukami H., Mineno J., Ozawa K.*

**OR-21. Enhanced expression of anti-CD19 chimeric antigen receptor in piggyBac-modified engineered T cells**  
*Morita D., Tanaka M., Nishio N., Kawashima N., Okuno Y., Wilson M. H., Rooney C. M., Takahashi Y., Nakazawa Y.*

**OR-22. Intratumoral IFN- $\alpha$  gene transfer reduces trafficking of Tregs into tumor by inhibition of CCL17 expression in tumors**  
*Shibasaki C., Hashimoto H., Ueda R., Narumi K., Aoki K.*

**OR-23. Development of the novel oral cancer vaccine using *Bifidobacterium longum* displaying Wilms' tumor 1 protein**  
*Kitagawa K., Oda T., Araki A., Gono R., Saito H., Hashii Y., Katayama T., Shirakawa T.*

## Day 2: July 29, 2016

### Oral Session VI Vector-1 (Abstracts OR-24~OR-29)

9:10-10:10, Hall-2

*Chairpersons: Hiroaki Mizukami & Hiroyuki Nakai*

**OR-24. One-step nuclease-free generation of gene-repaired human iPS cells from IL2RG-defective fibroblasts**  
*Ohta N., Iwanaga Y., Ohtaka M., Nishimura K., Nakanishi M., Mitani K.*

**OR-25. Simple and effective ultracentrifugation-free large-scale purification of recombinant adeno-associated virus serotype 9 (rAAV9)**  
*Tomono T., Hirai Y., Okada H., Adachi K., Sakamoto S., Kawano Y., Chono H., Mineno J., Ishii A., Shimada T., Onodera M., Tamaoka A., Okada T.*

**OR-26. Assembly-activating protein is not required for the production of infectious AAV5 virions**  
*Earley L. F., Powers J., Adachi K., Meyer N. L., Xie Q., Chapman M. S., Nakai H.*

**OR-27. The 16 amino acid stretch of N-terminal region in AAV9 capsid is essential for persistence in blood circulation**  
*Adachi K., Nakai H.*

**OR-28. The systemic delivery of CXCR4-targeted conditionally replicative adenovirus with fiber and hexon modifications in pancreatic cancer**  
*Huang J. L., LaRocca C., Jacobsen K., Jensen E. H., Kaliberov S., Curiel D. T., Davydova J., Yamamoto M.*

**OR-29. Reovirus-mediated efficient killing of mouse cancer-associated fibroblasts**  
*Sakurai F., Kaminade T., Tachibana M., Mizuguchi H.*

### Oral Session VII Regenerative Medicine (Abstracts OR-30~OR-34)

10:10-11:00, Hall-2

*Chairpersons: Yukihiro Hirai & Hirotaka J. Okano*

**OR-30. The effect of autologous adipose-derived mesenchymal stem cell transplantation on vascular calcification in rats with adenine-induced kidney disease**  
*Yokote S., Yamanaka S., Tajiri S., Matsumoto K., Yokoo T.*

**OR-31. Alveolar bone regeneration therapy by BMP-2/7 double genes expression cassette vector with in vivo electroporation**  
*Kawai M., Ohura K.*

**OR-32. Middle ear mucosal regeneration by nasal mucosal epithelial cell sheets transplantation**  
*Yamamoto K., Morino T., Hama T., Yaguchi Y., Kojima H.*

**OR-33. Conditionally replicating adenovirus specifically eliminates pluripotent stem cell-derived tumorigenic cells without adverse effects to differentiated cardiomyocytes***Mitsui K., Ide K., Kosai K.***OR-34. Robust production of high purity endothelial cells from various human pluripotent stem cells***Yamamoto Y., Enoki T., Yamamizu K., Yamashita J. K., Mineno J.***Oral Session VIII  
Cancer-4 (Abstracts OR-35~OR-39)**

11:00-11:50, Hall-2

*Chairpersons: Kazunori Aoki & Kenichiro Kosai***OR-35. Monitoring and imaging tumor progression and tracking drug response in a preclinical mouse model***Huang P., Xu N., Takenaka F., Matsuura E., Watanabe M., Kumon H., Liu C., Nasu Y.***OR-36. Reovirus reduces the immunosuppressive activity of myeloid-derived suppressor cells via TLR3-dependent signaling pathway in tumor-bearing mice***Sakurai F., Katayama Y., Oya Y., Terasawa Y., Tachibana M., Kobiyama K., Ishii K., Akira S., Mizuguchi H.***OR-37. Upregulated lncRNA-UCA1 enhances therapeutic effect of oncolytic vaccinia virus in paclitaxel resistant ovarian cancer***Horita K., Kurosaki H., Nakatake M., Kuwano N., Ishii K., Parada R., Nomura M., Itamochi H., Oishi T., Harada T., Nakamura T.***OR-38. Peritoneal dissemination requires an Sp1-Dependent CXCR4/CXCL12 signaling axis and extracellular matrix-directed spheroid formation***Harada Y., Morodomi Y., Yonemitsu Y.***OR-39. A novel system to efficiently construct lentiviral vectors that identify and eliminate tumorigenic cells in pluripotent stem cells***Ide K., Mitsui K., Irie R., Matsushita Y., Ijichi N., Kosai K.***Oral Session IX  
Vector-2 (Abstracts OR-40~OR-45)**

14:40-15:40, Hall-2

*Chairpersons: Saki Kondo & Masashi Urabe***OR-40. Oncolytic adenovirus expressing IFN- $\alpha$  works synergistically with chemoradiation***Salzwedel A., LaRocca C., Ryvlin J., Han J., Davydova J., Yamamoto M.***OR-41. CD133-targeted oncolytic adenovirus exhibits anti-tumor effect by attacking colon cancer stem cells***Sato-Dahlman M., Miura Y., Huang J. L., Davydova J., Yamamoto M.***OR-42. A super gene expression system enhances the anti-glioma effects of adenovirus-mediated REIC/Dkk-3 gene therapy***Oka T., Kurozumi K., Shimazu Y., Ichikawa T., Ishida J., Otani Y., Shimizu T., Tomita Y., Sakaguchi M., Watanabe M., Nasu Y., Kumon H., Date I.***OR-43. Potent antitumor efficacy of oncolytic HSV-1 expressing interleukin 12 in cisplatin-resistant testicular cancer***Kakutani S., Fukuhara H., Taguchi S., Takeshima Y., Homma Y., Ino Y., Todo T.***OR-44. Development of platelet vector incorporating viral particles and anticancer cytokine for targeting tumor specific microenvironment***Nishikawa T., Li Y. T., Kaneda Y.*

**OR-45. Preclinical study for tumor-targeted and armed oncolytic vaccinia virus for systemic cancer virotherapy**

*Nakatake M., Kurosaki H., Yamane M., Horita K., Ishii K., Kuwano N., Hasegawa K., Fujiwara K., Nomura M., Parada R., Nakamura T.*

**Oral Session X  
Genetic Diseases-1 (Abstracts OR-46~OR-50)**

15:50-16:40, Hall-2

*Chairpersons: Koichi Miyake & Mahito Nakanishi*

**OR-46. Generation of high-performance pseudovirion for cancer immune therapy**

*Saga K., Kaneda Y.*

**OR-47. AAV-mediated gene therapy ameliorates autophagy dysfunction and neuropathy in CNS of globoid cell leukodystrophy mice**

*Lin D. S., Ho C. S., Liu H. L., Chiang M. F.*

**OR-48. Lentiviral mediated gene therapy & Zinc Finger Nuclease mediated gene editing for Krabbe disease**

*Kobayashi H., Izuka S., Fukuda T., Iwamoto T., Morita A., Ariga M., Shimada Y., Higuchi T., Ida H., Eto Y., Ohashi T.*

**OR-49. Ex vivo lentiviral gene therapy outperforms other conventional therapeutic strategies in murine model of mucopolysaccharidosis type II**

*Sato Y., Wada M., Wakabayashi T., Higuchi T., Shimada Y., Kobayashi H., Ida H., Ohashi T.*

**OR-50. Ex vivo genome editing therapy for Fabry disease**

*Kami D., Sakuma T., Yamamoto T., Gojo S.*

**Oral Session XI  
Genetic Diseases-2 (Abstracts OR-51~OR-56)**

16:40-17:40, Hall-2

*Chairpersons: Toshinao Kawai & Hiroyuki Nuno*

**OR-51. Assessment of the alveolar bone and tooth in lethal hypophosphatasia mice treated by rAAV8-TNALP-D10**

*Ikeue R., Takahashi A., Muramatsu T., Kasahara Y., Watanabe A., Shimada T., Okada T., Sato T.*

**OR-52. Therapeutic effects of scAAV8-mediated high dose expression of bone targeted alkaline phosphatase on the lethal hypophosphatasia mice**

*Nakamura-Takahashi A., Ikeue R., Nitahara-Kasahara Y., Watanabe A., Hirai Y., Kasahara M., Okada T.*

**OR-53. Follow-up lentiviral integration sites analysis in ADA-SCID clinical gene therapy**

*Wang W., Rivat C., Bartholomae C. C., Fronza R., Kutschera I., Rabenstein M., Paruzynski A., Kalle C., Deichmann A., Gaspar B., Schmidt M.*

**OR-54. Single cell-based vector tracing in the patients treated with stem cell gene therapy**

*Takahashi S., Yuka Igarashi, Uchiyama T., Watanabe N., Otsu M., Ariga T., Onodera M.*

**OR-55. Neonatal gene therapy for hemophilia B by a novel adenovirus vector showing reduction in the leaky expression of viral genes.**

*Iizuka S., Sakurai F., Tachibana M., Ohashi K., Mizuguchi H.*

**OR-56. Effective microdystrophin expression in non-human primate muscle with AAV type 8 vectors under immune suppression**

*Ishii A., Okada H., Hayashita-Kinoh H., Shin J. H., Okada T., Takeda S.*

**Poster Sessions**

□ Best Poster ○ Excellent Poster

**Poster Session I  
Acquired Diseases (Abstracts: PO-57~PO-62)**Date: July 29, 2016, 17:40-18:10, Poster & Exhibition Hall  
Chairperson: Hiroshi Tazawa**PO-57. Therapeutic effect of liver-targeted hydrodynamic delivery of MMP13 gene on liver fibrosis***Kamimura K., Abe H., Kobayashi Y., Yokoo T., Ohtsuka M., Miura H., Yamagiwa S., Terai S.***PO-58. Improvement of the efficiency in CRISPR/Cas9 system against Hepatitis B virus using adenovirus vector***Kondo S., Maekawa A., Suzuki M., Kanegae Y.***PO-59. Prevention of airway inflammation by simultaneous inhibition of NFκB and STST6 using chimeric decoy oligonucleotides in a mouse model of asthma***Miyake T., Miyake T., Hamada H., Morishita R.***PO-60. Study of erythroblastic-associated gene transduction by modified adenovirus vector for treatment of diabetes mellitus***Shimizu K., Okamoto M., Nishinaka T., Sakurai F., Mizuguchi H., Tomita K., Terada T.***PO-61. Knockdown of toll like receptor 4 signaling pathway ameliorates graft rejection in a mouse model of allograft transplantation***Hsieh J. L., Shen P. C., Chen S. Y.***PO-62. The development of candidate vaccine against MERS-CoV.***Koriyama H., Saito Y., Nakagami H., Morishita R.***Poster Session II  
Cancer-1 (Abstracts: PO-63~PO-68)**Date: July 29, 2016, 17:40-18:10, Poster & Exhibition Hall  
Chairperson: Yasushi Soda**PO-63. Cytoplasmic calcium increase via fusion with inactivated Sendai virus induces apoptosis in human multiple myeloma cells by downregulation of c-Myc oncogene***Jiang Y., Saga K., Kaneda Y.***PO-64. A therapeutic potential of a third generation oncolytic HSV-1 G47Δ for biliary tract cancer with peritoneal dissemination***Tateno Y., Ino Y., Iwai M., Shinozaki M., Todo T.***PO-65. The efficacy and safety of oncolytic HSV-1 (G47Δ) in mouse orthotopic esophageal cancer models***Yajima S., Ino Y., Fukuhara H., Iwai M., Seto Y., Todo T.***PO-66. Antitumor effect of oncolytic herpes simplex virus G47Δ in human head and neck cancers***Nagatomo T., Ino Y., Iwai M., Nishino H., Todo T.***PO-67. Antitumor efficacy of hTERT promoter regulated oncolytic herpes simplex viruses for human gastric cancer***Nakamori M., Kato T., Matsumura S., Tsuji T., Fukuhara H., Ino Y., Todo T., Yamaue H.***PO-68. RAMBO (rapid antiangiogenesis mediated by oncolytic virus) decreased bevacizumab-induced glioma cell invasion***Tomita Y., Kurozumi K., Shimizu T., Oka T., Ohtani Y., Ishida J., Kaur B., Ichikawa T., Date I.*

**Poster Session III**  
**Cancer-2 (Abstracts: PO-69~PO-74)**

Date: July 29, 2016, 17:40-18:10, Poster & Exhibition Hall  
Chairperson: Hiroshi Fukuhara

**PO-69. Chimeric antigen receptor T cells found to boost survival in mice with brain tumors**

*Natsume A., Ohno M., Kuramitsu S., Shiina S., Yamamichi A., Chalise L., Kato A., Ohka F., Kaneko M. K., Kato Y., Wakabayashi T.*

**PO-70. Ligand-based chimeric antigen receptor-modified T cells targeting CD116 for the treatment of acute myeloid leukemia**

*Nakano S., Morita D., Matsuda K., Ohnota H., Hongo K., Koike K., Nakazawa Y.*

**PO-71. Manufacture of chimeric antigen receptor (CAR) gene transduced T cells for clinical trials**

*Inoue N., Oshikawa T., Tanaka Y., Tadai Y., Tomura D., Ideno M., Nukaya I., Mineno J., Takesako K.*

**PO-72. Development of measuring *in vivo* cell kinetics of retrovirus-mediated gene engineered T cells in patients who received the T cell transfer**

*Tadai Y., Tanabe M., Tomura D., Takayama M., Kageyama S., Kato H., Shiku H., Takesako K., Mineno J.*

**PO-73. Genetically-engineered multilineage-differentiating stress-enduring (Muse) cells as cellular assassins against malignant gliomas**

*Namba H., Yamasaki T., Wakao S., Dezawa M.*

**PO-74. Multilineage-differentiating stress-enduring (Muse) cells migrate a long distance to the glioma in the mouse brain**

*Yamasaki T., Wakao S., Dezawa M., Namba H.*

**Poster Session IV**  
**Cancer-3 (Abstracts: PO-75~PO-81)**

Date: July 29, 2016, 17:40-18:15, Poster & Exhibition Hall  
Chairperson: Yoshinobu Manome

**PO-75. Prodrug activator gene therapy mediated by retroviral replicating vector (RRV) for pancreatic ductal adenocarcinoma**

*Hiraoka K., Inoko K., Inagaki A., Takahashi M., Kushibiki T., Hontani K., Nakamura T., Tsuchikawa T., Shichinohe T., Jolly D. J., Kasahara N., Hirano S.*

**PO-76. A tailor-made virotherapy with replicating retroviral replicating vectors on the basis of the cellular receptor expression**

*Kubo S., Mawatari-Furukawa Y., Sakagami R., Kasahara N.*

**PO-77. Novel polymer-coated oncolytic measles virus overcomes immune suppression and induces robust antitumor activity**

*Nosaki K., Takishima Y., Sagara M., Matsumura Y., Miyamoto S., Yang J., Matsuura K., Okazaki T., Inoue H., Nakanishi Y., Hamada K., Tani K.*

**PO-78. A novel viral therapy for human esophageal cancer using oncolytic enterovirus, echovirus 4.**

*Ogata H., Yasunari K., Inoue H., Miyamoto S., Takishima Y., Nakanishi Y., Tani K.*

**PO-79. A novel system strongly enhances hTERT promoter-dependent cancer-specific gene expression**

*Sadahira T., Watanabe M., Sakaguchi M., Ueki H., Kinoshita R., Nasu Y.*

**PO-80. Impact of Adenoviral Death Protein on sodium iodide symporter (NIS)-based iodine therapy and imaging for pancreatic cancer**

*Koodie L., Kawakami E., Eidenschink B., Sell J., LaRocca C., Jacobsen K., Ryvlin J., Tolosa E., Maxon D., Zapico M. F., Davydova J.*

**PO-81. Virus-guided fluorescent imaging of pancreatic cancer cells in the peritoneal cavity for an insight into roles of tumor-associated macrophage**

*Kuwada K., Kagawa S., Sakamoto S., Watanabe M., Kagawa T., Kikuchi S., Kuroda S., Yoshida R., Urata Y., Tazawa H., Fujiwara T.*

**Poster Session V  
Cancer-4 (Abstracts: PO-82~PO-87)**

Date: July 29, 2016, 17:40-18:10, Poster & Exhibition Hall

Chairperson: Yuji Heike

**PO-82. Development and preclinical evaluation of a novel RNA *trans*-splicing molecule for cancer gene therapy targeted to cancer-type organic anion transporting polypeptide 1B3**

*Harada M., Furihata T., Sun Y., Akita H., Anzai N., Koller U., Stierschneider A., Hofbauer J. P., Gruber C., Chiba K.*

**PO-83. Gene expression analysis of bevacizumab-induced glioma invasion**

*Shimizu T.*

**PO-84. Antiproliferative effect of gene therapy with SOCS-1 in esophageal squamous cell carcinoma**

*Sugase T., Takahashi T., Serada S., Nakatsuka R., Fujimoto M., Miyazaki Y., Makino T., Kurokawa Y., Yamasaki M., Nakajima K., Takiguchi S., Mori M., Doki Y., Naka T.*

**PO-85. Ex vivo expansion of human mesenchymal stem cells in combination with defined serum-free media and RetroNectin® for clinical use.**

*Tahara K., Yamaguchi S., Yamamoto F., Togashi K., Ideno M., Nukaya I., Takeda T., Yamagishi H., Mineno J.*

**PO-86. Role of P53 during T lymphopoiesis from human pluripotent stem cells**

*Kohara H., Jiyuan L., Miyamoto S., Nagai Y., Marumoto T., Tani K.*

**PO-87. MDM2 and MDM4 inhibitors in combination with adenoviruses up-regulating p53 produce synergistic anti-tumor effects on mesothelioma through augmenting apoptotic processes**

*Tagawa M., Kozono T., Qin Y., Ning X. R., Morinaga T., Kubo S., Shingyoji M., Sekine I., Tada Y., Tatsumi K., Shimada H., Hiroshima K.*

**Poster Session VI  
Cancer-5 (Abstracts: PO-88~PO-93)**

Date: July 29, 2016, 17:40-18:10, Poster & Exhibition Hall

Chairperson: Toshihiko Okazaki

**PO-88. Inhibition of hTERT-positive neuroblastoma malignant phenotype by hTERT-driven oncolytic adenovirus-mediated tumor suppressor p53 transactivation and oncogenic MYCN suppression.**

*Tanimoto T., Tazawa H., Koujima T., Kuwada K., Noso H., Oyama T., Urata Y., Kagawa S., Noda T., Fujiwara T.*

**PO-89. Evaluating the effect of 5FU on oncolytic adenoviral replication**

*Sell J., Oliviera A., Yamamoto M., Davydova J.*

**PO-90. Combination therapy of squamous cell carcinoma mouse models with PD-L1 immune checkpoint inhibitor and oncolytic herpes simplex virus HF10**

*Mukoyama N., Ichinose T., Luo C., Koide Y., Zhiwen W., Miyajima N., Morimoto D., Tanaka M., Fujimoto Y., Sone M., Koderia Y., Kasuya H.*

**PO-91. Systemic delivery strategy of oncolytic herpes simplex virus HF10 adsorbed on antigen-specific lymphocytes**

*Ichinose T., Luo C., Koide Y., Zhiwen W., Mukoyama N., Miyajima N., Morimoto D., Yamada S., Fujii T., Sugimoto H., Tanaka M., Koderia Y., Kasuya H.*

**PO-92. Combination treatment of human cancer xenograft models with the conventional chemotherapeutic drug and oncolytic herpes simplex virus HF10**

*Morimoto D., Luo C., Koide Y., Miyajima N., Mukoyama N., Zhiwen W., Ichinose T., Tanaka M., Fujimoto Y., Koderu Y., Kasuya H.*

**PO-93. Enhanced anti-tumoral activity of oncolytic herpes simplex virus HF10 with Cetuximab against human colorectal cancer**

*Wu Z., Ichinose T., Luo C., Yamada S., Fujii T., Sugimoto H., Miyajima N., Morimoto D., Mukoyama N., Nishikawa Y., Koide U., Kuwahara T., Koderu Y., Tanaka M., Kasuya H.*

**Poster Session VII**

**Genetic and Neurological Diseases (Abstracts: PO-94~PO-99)**

Date: July 29, 2016, 17:40-18:10, Poster & Exhibition Hall

Chairperson: Makoto Migita

**PO-94. Treatment for Fabry peripheral neuropathy with adeno-associated virus vector in murine Fabry disease model**

*Higuchi T., Shimada Y., Kobayashi H., Ohashi T.*

**PO-95. Effects of transplantation of human LCAT-secreting adipocytes on lipid metabolism in *lcat* deficient mice crossed with human *apoA-I* transgenic mice**

*Aoyagi Y., Kuroda M., Onitake A., Kirinashizawa M., Asada S., Saito Y., Tanaka S., Konno S., Tanio M., Yokote K., Saito Y., Bujo H., Aso M.*

**PO-96. Novel *ex vivo* gene manipulation technology of mature adipocytes for sustained protein replacement therapy of variety of intractable diseases**

*Asada S., Kuroda M., Aoyagi Y., Onitake A., Saito Y., Kirinashizawa M., Tanaka S., Konno S., Tanio M., Saito Y., Bujo H., Yokote K., Aso M.*

**PO-97. hnRNP A1 and A2 are potential targeted proteins for regulatory increasing of SMN synthesis in Spinal Muscular Atrophy.**

*Kashima T., Agawa-Ohta M., Kanegae Y., Yamada H.*

○ **PO-98. Generation of animal models of spinocerebellar ataxia via AAV9 vector-mediated gene transfer**

*Konno A., Matsuzaki Y., Hirai H.*

**PO-99. Neuroprotective effects of intravenous administration of dental pulp stem cells after ischemic-reperfusion injury**

*Nito C., Sowa K., Nakajima M., Ueda M., Nishiyama Y., Suda S., Takahashi A. N., Imagawa K., Tachibana K., Kasahara Y. N., Okada T., Kimura K.*

**Poster Session VIII**

**Vector (Abstracts: PO-100~PO-106)**

Date: July 29, 2016, 17:40-18:15, Poster & Exhibition Hall

Chairperson: Fuminori Sakurai

**PO-100. Generation of gene knock-in marmosets using CRISPR/Cas9 system**

*Yoshida T., Kishi N., Sasaki E., Okano H.*

**PO-101. Development of *in vitro* and *in vivo* evaluation systems for novel non-coding RNA, SINEUP, to enhance translation level of target genes as a nucleic acid medicine**

*Nitta K. R., Takahashi H., Nakano M., Suzuki A. M., Kozhuharova A., Sharma H., Kataoka Y., Zucchelli S., Gustincich S., Carninci P.*

**PO-102. Type I interferons impede short-hairpin RNA-mediated RNA interference *via* inhibition of dicer-mediated processing to small interfering RNA.**

*Machitani M., Sakurai F., Wakabayashi K., Tachibana M., Mizuguchi H.*



**PO-103. ssAAV vectors are able to pass through the BBB as well as scAAV**

*Miyake N., Miyake K., Yamamoto M., Shimada T., Okada T.*

**PO-104. Feasibility of large-scale rAAV production from suspension HEK293 cell line using flow electroporation system**

*Okada H., Tomono T., Adachi K., Hirai Y., Okada T.*

**PO-105. Stability comparison of recombinant serotype 2 adeno-associated virus vectors with wild-type viruses under different conditions relevant to gene therapy trials**

*Huang S., Kawano Y., Chono H., Okada T., Mineno J.*

**PO-106. Construction of One-stop GMP manufacturing facility**

*Nukaya I., Yoshioka H., Chono H., Shoudai T., Kidokoro T., Tomura D., Tanabe M., Inoue N., Inoue T., Mineno J.*

## Corporate Seminars Evening Seminar

### ►Day I

#### Corporate Seminar-I (SANOFI K.K.)

Date: July 28, 2016, 12:00-13:00, Hall-1

Chairperson: *Yoshikatsu Eto*

*(Advanced Clinical Research Center, Institute of Neurological Disorders)*

**Seng H. Cheng** (*SANOFI K.K.*)

**Emerging Therapeutic Concepts for Gaucher and Related Disease**

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#### Corporate Seminar-II (BioReliance)

Date: July 28, 2016, 12:00-13:00, Hall-2

Chairperson: *Sadao Ozawa*

*(BioReliance)*

**Susan Livingston** (*BioReliance*)

**Hidenori Meiseki** (*BioReliance*)

**Preparing for GMP manufacturing of Cell and Gene therapy clinical products - guidance and advice from manufacturing experts**

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#### Corporate Seminar-III (CellSeed Inc.)

Date: July 28, 2016, 12:00-13:00, Hall-3

Chairperson: *Katsumi Katayama*

*(CellSeed Inc.)*

**Nobuo Kanai** (*Tokyo Women's Medical University*)

**Hiromi Kojima** (*The Jikei University School of Medicine*)

**Moving forward toward the clinical use of cell-sheet regenerative medicine**

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#### Evening Seminar (Daicel Corporation)

Date: July 28, 2016, 18:10-18:40, Hall-2

Chairperson: *Yasufumi Kaneda*

*(Graduate School of Medicine, Osaka University)*

**Hironori Nakagami** (*Graduate School of Medicine, Osaka University*)

**Novel DNA vaccination by the needle-free injection system**

►Day II

**Corporate Seminar-IV  
(TAKARA BIO INC.)**

Date: July 29, 2016, 12:00-13:00, Hall-1  
Chairperson: Keiya Ozawa  
(The Institute of Medical Science, The University of Tokyo)

**Robert Hans Ingemar Andtbacka** (*Huntsman Cancer Institute, University of Utah*)  
**Cutting edge of oncolytic virus immunotherapy**

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**Corporate Seminar-V  
(Miltenyi Biotec)**

Date: July 29, 2016, 12:00-13:00, Hall-2  
Chairperson: Yoshikazu Yonemitsu  
(Kyushu University)

**Katharina Winnemöller** (*Miltenyi Biotec GmbH*)  
**Cell therapy manufacturing - making the future now MACS technology for CAR-T cells**

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**Corporate Seminar-VI  
(Sumitomo Dainippon Pharma Co., Ltd.)**

Date: July 29, 2016, 12:00-13:00, Hall-3  
Chairperson: Hiroyuki Ida  
(The Jikei University School of Medicine)

**Jeffrey A. Medin** (*Medical College of Wisconsin, USA*)  
**Lentivirus-mediated gene therapy for Fabry Disease**

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►Day III

**Corporate Seminar-VII  
(AnGes MG, Inc.)**

Date: July 30, 2016, 12:00-13:00, Hall-1  
Chairperson: Norio Sakai  
(Graduate School of Medicine, Osaka University)

**Torayuki Okuyama** (*National Center for Child Health and Development*)  
**Therapeutic advances in treatment of inherited rare diseases**

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**Corporate Seminar-VIII  
(Shire-Japan)**

Date: July 29, 2016, 12:00-13:00, Hall-2  
Chairperson: Hirokazu Iwamuro  
(Juntendo University Graduate School of Medicine)

**Vivian Choi** (*Discovery Therapeutics, Research & Nonclinical Development, Shire*)  
**Gene therapy: Emerging class of therapeutics with transformative potential in rare diseases**