### The 21st Annual Meeting of Japan Society of Gene Therapy

#### **Presidential Lecture**

Date: July 24, 2015, 14:30-15:00, Hall 1

Chairperson: Fumio Endo (Kumamoto University)

Ryuichi Morishita (Department of Clinical Gene Therapy, Osaka University, Japan)

New Era in Gene Therapu: Japan as rising sun in gene therapy

### **Special Lecture I**

Date: July 24, 2015, 8:40-9:25, Hall 1

Chairperson: Shigetaka Asano (Waseda University)

Michel Sadelain (Memorial Sloan Kettering Cancer Center)

CAR therapy: the CD19 paradigm

### **Special Lecture II**

Date: July 24, 2015, 11:00-11:50, Hall 1

Chairperson: Hiromi Kumon (Niimi College)

Shizuo Akira (WPI Immunology Frontier Research Center, Osaka University)

Regnase-1, a ribonuclease involved in the inflammatory and immune responses

### **Special Lecture III**

Date: July 25, 2015, 10:30-11:20, Hall 1

Chairperson: Izumu Saito (The University of Tokyo)

Tamotsu Yoshimori (Graduate School of Medicine, Osaka University)

Autophagy: The Intracellular Self-Degradation System Fighting against Diseases

### Special Lecture IV

Date: July 25, 2015, 12:30-13:00, Hall 1

Chairperson: Yoshiro Niitsu (Sapporo medical University)

Nathalie Cartier-Lacave (Université Paris Descartes)

Gene therapy strategy for Alzheimer's disease: the cholesterol Connection

### **Memorial Lecture**

Date: July 26, 2015, 12:40-13:10, Hall 1

Chairperson: Ryuichi Morishita (Osaka University)

Shimon Sakaguchi (Osaka University Immunology Frontier Research Center)

Targeting Human Regulatory T Cells for Controlling Immune Responses

#### **Educational Lecture I**

Date: July 24, 2015, 9:35-10:05, Hall 1

Chairperson: Kohnosuke Mitani (Saitama Medical University)

Takashi Nagasawa (Kyoto University)

The microenvironmental niches for hematopoietic stem and progenitor cells in bone marrow

#### **Educational Lecture II**

Date: July 24, 2015, 10:05-10:35, Hall 1

Chairperson: Toshihiko Wakabayashi (Nagoya University)

Yoshiki Sawa (Osaka University)

Translational research of autologous stem cell-sheet transplantation therapy for treating cardiomyopathy: A "proof-of-concept" clinical trial

### **Educational Lecture III**

Date: July 24, 2015, 10:10-10:40, Hall 2

Chairperson: Hideaki Tahara (The University of Tokyo)

Koji Kawakami (Kyoto University Graduate School of Medicine and Public Health)
Real World Data and Clinical/Pharmaco Epidemiology in Japan

### Presidential Special Program I

Japan Strategy for HealthCare Innovation: Gene Therapy as Next Generation of Innovative Drugs

Date: July 24, 2015, 15:10-16:40, Hall 1

Chairpersons: Ryuichi Morishita (Osaka University) & Yasufumi Kaneda (Osaka University)

Yasutoshi Nishimura (State Minister of Cabinet Office (3rd Abe Cabinet))

Makoto Suematsu (Japan Agency for Medical Research and Development)

Kazuhiko Mori (Ministry of Health, Labour and Welfare, Government of Japan)

### Presidential Special Program II

New Law for Regenerative Medicine & Gene Therapy

Date: July 25, 2015, 16:20-17:20, Hall 2

Chairpersons: Ryuichi Morishita (Osaka University) & Masafumi Onodera (National Center for Child Health and Development)

Kenichi Kimura (Ministry of Health, Labour and Welfare, the Government of Japan)

**Panelists** 

Yoshikazu Yonemitsu (Kyushu University)

Katsuto Tamai (Osaka University)

Shigeru Miyagawa (Osaka University)

#### Symposium I

### Towards clinical translation of virus therapy for malignancies

Date: July 24, 2015, 13:00-14:30, Hall 1

Chairpersons: Kenzaburo Tani (The University of Tokyo) & Masatoshi Tagawa (Chiba Cancer Center Research Institute)

Chieko Kai (Institute of Medical Science, The University of Tokyo)

An oncolytic modified measles virus is a novel tool for cancer treatment

Ken-ichiro Kosai (Department of Gene Therapy and Regenerative Medicine / Center for Innovative Therapy Research and Application, Kagoshima University Graduate School of Medical and Dental Sciences)

Development of the original survivin-responsive conditionally replicating adenovirus toward the investigator-initiated GCP clinical trial

Masato Yamamoto (University of Minnesota)

Advance in Oncolytic Adenoviruses for Targeting

**Takafumi Nakamura** (Department of Biomedical Science, Graduate School of Medical Sciences, Tottori University)

Tumor-specific, replication-competent vaccinia virus for systemic oncolytic virotherapy

Shohei Miyamoto (The Institute of Medical Science, The University of Tokyo)

Development of the novel oncolytic coxsackievirus therapy for the clinical trial.

### Symposium II

### Medical care and gene therapy for genetic diseases, its present and future

Date: July 24, 2015, 13:00-14:30, Hall 2

Chairpersons: Torayuki Okuyama (National Center for Child Health and Development) &

Toya Ohashi (The Jikei University School of Medicine)

**Hiroshi Kobayashi** (Division of Gene Therapy, Research Center for Medical Sciences, Department of Pediatrics The Jikei University School of Medicine)

Medical Care and Gene therapy for genetic diseases, its present and future: Lysosomal Storage Disorders (LSDs)

Motomichi Kosuga (Division of Medical Genetics, National Center for Child Health and Development) Newborn screening for genetic diseases

Toshinao Kawai (Department of Human Genetics, National Center for Child Health and Development) Clinical trials of hematopoietic stem cell gene therapy for monogenic diseases

### Symposium III

Basic research and clinical development of gene-modified T cell therapy against canser

Date: July 24, 2015, 13:00-14:30, Hall 3

Chairpersons: Hiroaki Ikeda (Mie University Gradyate School of Medicine) &

Kouji Tamada (Yamaguchi University Graduate School of Medicine)

Koji Tamada (Department of Immunology Yamaguchi University Graduate School of Medicine)
Next generation CAR-T cells endowed with a superior survival and migration potential

Yozo Nakazawa (Department of Pediatrics, Shinshu University School of Medicine)

CANCER IMMUNOTHERAPY USING CHIMERIC ANTIGEN RECEPTORS: ADVANCES, ISSUES, AND FUTURE DIRECTIONS

Hiroaki Ikeda (Department of Immuno-Gene Therapy, Mie University Graduate School of Medicine) Clinical development of TCR gene-therapy for patients with epithelial cancer and leukemia

**Hiroshi Fujiwara** (Department of Hematology, Clinical Immunology and Infectious Disease, Ehime University Graduate School of Medicine)

Development of antileukemia adoptive immunotherapy using gene-modified T cells to express leukemia antigen-specific T-cell receptors

Miki Ando (Division of Stem Cell Therapy, Center for Stem Cell Biology and Regenerative Medicine, The Institute of Medical Science, The University of Tokyo (IMSUT))

Induced Pluripotent Stem Cell-Derived Rejuvenated T-Cell Therapy with Suicide Gene-based Safeguard System

### Symposium IV

### iPS cell research and vector technology

Date: July 25, 2015, 9:00-10:30, Hall 1

Chairpersons: Hiroyuki Mizuguchi (Osaka University) & Yoshikazu Yonemitsu (Kyushu University)

**Yoshikazu Yonemitsu** (R&D Laboratoty for Innovative Biotherapeutics)

Overview: recent advances on the vector technology of iPS cell research

Hiroyuki Mizuguchi (Graduate School of Pharmaceutical Sciences, Osaka University, Osaka, Japan. / Graduate School of Medicine, Osaka University, Osaka, Japan. / Global Center for Medical Engineering and Informatics, Osaka University, Osaka, Japan. / National Institutes of)

Use of adenovirus vectors for stem cell research

Noemi Fusaki (Keio University School of Medicine)

Efficient generation of footprint-free patient-specific iPS cells and the application for drug screening

Masavo Takahashi (RIKEN)

The iPS cell generation with a plasmid vector and application of iPS-derived retinal cells

### Symposium V

### Progress of cancer gene therapy and oncolytic virus therapy

Date: July 26, 2015, 10:00-11:30, Hall 2

Chairpersons: Yasutomo Nasu (Okayama University Graduate School of Medicine) & Tomoki Todo (The University of Tokyo)

Toshiyoshi Fujiwara (Okayama University Graduate School)

Phase I/II trial of endoscopic intratumoral administration of OBP-301, a novel telomerase-specific oncolytic virus, with radiation in esophageal cancer patients

Hiroshi Fukuhara (Department of Urology, Graduate School of Medicine, The University of Tokyo)
A phase 1 study of a third-generation oncolytic HSV-1 G47Δ in patients with castration resistant

Noriyuki Kasahara (Department of Cell Biology & Pathology, University of Miami)

Retroviral Replicating Vectors for Prodrug Activator Gene Therapy of Recurrent High-Grade Glioma: Translational Studies & Clinical Update

Yasutomo Nasu (Department of Urology, Okayama University Graduate School of Medicine, Dentistry and Pharmaceutical Sciences)

DEVELOPMENT OF CANCER GENE THERAPY USING REDUCED EXPRESSION IN IMMORTAL-IZED CELLS (REIC/DKK-3) GENE

Hiroshi Shiku (Department of Immuno-Gene Therapy Mie University Gradyate School of Medicine) Adoptive immunotherapy with antigen receptor gene-modified lymphocytes

### Symposium VI

Recent advances in the development of oncolytic viruses and genome editing technology

Date: July 26, 2015, 10:10-11:30, Hall 3

Chairpersons: Hiroaki Uchida (The University of Tokyo) & Fuminori Sakurai (Osaka University)

**Hiroaki Uchida** (Division of Bioengineering, Advanced Clinical Research Center, The Institute of Medical Science, The University of Tokyo)

Exploration of cancer-targeting antibodies using an HSV-based screening system for development of novel HSV vectors fully retargeted to cancer cells

Fuminori Sakurai (Graduate School of Pharmaceutical Sciences, Osaka University, Osaka, Japan)

Development of a potent oncolytic adenovirus via regulation of dicer-mediated processing of virus-associated RNAs

Takashi Yamamoto (Department of Mathematical and Life Sciences, Hiroshima University) Genome editing with site-specific nucleases

## **International Symposium Hot Topics in Clinical Gene Therapy**

Date: July 25, 2015, 13:10-14:00, Hall 1

Chairpersons: Yoshikatsu Eto (Institute for Neurologicl Disorders / Tokyo Jikei University School of Medicine) & Akinobu Gotoh (Hyogo College of Medicine)

Vijay Samant (Vical Incorporated)
Clinical Advances in DNA Vaccines

Barrie J. Carter (Biomarin Pharmaceutical) Gene Therapy for Hemophilia

Date: July 25, 2015, 14:00-14:50, Hall 1

Chairpersons: Keiya Ozawa (The University of Tokyo) & Hiroshi Shiku (Mie University Granduate School of Medicine)

Robert M. Kotin (National Heart, Lung, and Blood Institute, Bethesda, MD, USA / Voyager Therapeutics,

Cambridge, MA, USA / University of Massachusetts Medical School, Worcester, MA, USA)

Progress report on systemic delivery of rAAV9-U7smOPT vector in the golden retriever muscular dystrophy canine model of Duchenne muscular dystrophy

Katherine High (Spark Therapeutics)

Update on clinical development of AAV-mediated gene therapy for a rare form of inherited blindness

Date: July 25, 2015, 14:50-15:40, Hall 1

Chairpersons: Hideki Mochizuki (Osaka University) &

Shuichi Kaneko (Kanazawa University Graduate School of Medical Sciences)

Shin-ichi Muramatsu (Jichi Medical University / The Institute of Medical Science, The University of Tokyo / Gene Therapy Research Institution, Co., Ltd.)

Gene therapy for Neurodegenerative Diseases

Gabor Veres (bluebirdbio Inc)

Gene Therapy for Hemoglobinopathies with Autologous Hematopoietic Stem Transduced Ex vivo with LentiGlobin Lentiviral Vector

### Joint Symposium I

Current Topics of gene therapy using iPS cells

Date: July 25, 2015, 12:30-14:00, Hall 2

Joint: The Japanese Society of Regenerative Medicine

Chairperson: Yonehiro Kanemura (National Hospital Organization)

Akitsu Hotta (Center for iPS Cell Research & Application (CiRA), Kyoto University / iCeMS, Kyoto University) Efficient gene correction of Duchunne muscular dystrophy mutation in iPS cells by TALENs and CRISPR-Cas9

Hiroyuki Miyoshi (Department of Physiology, Keio University School of Medicine) The Use of Suicide Genes in iPSCs for Safety and Cancer Therapy

### Joint Symposium II Frontier of vascular biology

Date: July 25, 2015, 14:10-15:40, Hall 2

Joint: Japanese Vascular Biology and Medicine Organization

Chairpersons: Nobuyuki Takakura (Osaka University) &

Kensuke Egashira (Kyushu University Graduate School of Medical Sciences)

Shigetomo Fukuhara (Department of Cell Biology, National Cerebral and Cardiovascular Center Research Institute) Dynamic regulation of endothelial barrier function

Tetsuya Matoba (Department of Cardiovascular Medicine, Graduate School of Medical Sciences, Kyushu University) Nanoparticle-mediated Drug Delivery System for Atherosclerotic Cardiovascular Disease

Nobuyuki Takakura (Department of Signal Transduction, Research Institute for Microbial Diseases, Osaka University) Molecular mechanism of arterial-venous alignment and its biological significance

Yoshikazu Nakaoka (Department of Cardiovascular Medicine, Osaka University Graduate School of Medicine) Interleukin-6/interleukin-21 signaling axis in the pathogenesis of pulmonary arterial hypertension

### Joint Symposium III

Integration of cutting-edge neuroscience with forefront gene therapy

Date: July 26, 2015, 8:30-10:00, Hall 3 **Joint: Japan Neuroscience Society** 

Chairpersons: Hideki Mochizuki (Osaka University) & Hirokazu Hirai (Grunma University Graguate school of Medicine)

Masahiko Takada (Primate Research Institute, Kyoto University)

Protection against MPTP-induced parkinsonian insults by calbindin recruitment into nigrostriatal dopamine neurons using recombinant viral vectors

Takanori Yokota (Department of Neurology and Neurological Science, Tokyo Medical and Dental University)
DNA/RNA heteroduplex oligonucleotide for highly efficient gene silencing

Hideyuki Okano (Department of Phsiology Keio University School of Medicine) Regenerative Medicine and Diseases Modeling using iPS cell technologies

### Regulatory Science Symposium Regulation of Gene Therapy in Prospect

Date: July 25, 2015, 16:20-17:20, Hall 3

Chairpersons: Takashi Shimada (Nippon Medical School) & Akihiro Kume (Pharmaceuticals and Medical Devices Agency)

Teruhide Yamaguchi (Nihon Phamaceutical University)

Revision of Japanese Gene Therapy Guidelines

**Panelists** 

**Tomoki Todo** (division of Innovative Cancer Therapy, and Department of Surgical Neuro · Oncology The Institute of Medical Science, The University of Tokyo)

Toshio Miyata (Health and Global Policy Institute)

## Technical Seminar Basics of gene transfer vectors

Date: July 26, 2015, 8:30-10:00, Hall 3

Chairpersons: Hiroyuki Mizuguchi (Osaka University) & Masashi Urabe (Jichi Medical University)

Hiroyuki Mizuguchi (Graduate School of Pharmaceutical Sciences, Osaka University, Osaka, Japan / Graduate School of Medicine, Osaka University, Osaka, Japan / Global Center for Medical Engineering and Informatics, Osaka University, Osaka, Japan / National Institutes of Biomedical Innovation, Health and Nutrition, Osaka, Japan)

Adenovirus vectors

Hiroyuki Miyoshi (Department of Physiology, Keio University School of Medicine) Basics of Lentiviral Vectors

Masashi Urabe (Division of Genetic Therapeutics, Jichi Medical University)

How to make and use adeno-associated virus vectors ---from the basics to applications---

**Tetsushi Sakuma** (Department of Mathematical and Life Sciences, Graduate School of Science, Hiroshima University)

Vectors for TALEN- and CRISPR/Cas9-mediated genome editing

### Young Session Challenge to Incurable Disease

Date: July 24, 2015, 8:40-10:10, Hall 3

Chairpersons: Yoshiaki Taniyama (Osaka University) & Hironori Nakagami (Osaka University)

Shoji Sanada (Research and Development Division, Health Policy Bureau, Ministry of Health, Labour and Welfare) Advanced Medical Care System and Clinical Trials as Strategies for Establishment of Innovative Medical Drugs and Technologies in Japan

Yukihiro Saito (Division of Vascular Surgery, Asahikawa Medical University)

Therapeutic Lymphangiogenesis for Lymphedema by Gene Therapy of Hepatocyte Growth Factor Plasmid DNA.

Masayuki Endo (Osaka University / The Children's Hospital of Philadelphia)

Developmental Stage and Delivery mode Determines Distribution and Duration of Gene Expression after Intra-Utero Gene Transfer Using Lentiviral Vectors

Hironori Nakagami (Osaka University)

Physicians-initiated clinical trial for skin ulcer using novel functional peptide

### The 1st International Symposium of the Asia-Pacific Gene Therapy Consortium

### **Memorial Symposium**

Date: July 25, 2015, 15:50-17:20, Hall 1 Chairperson: Yasufumi Kneda (Osaka University)

Nathalie Cartier-Lacave (Université Paris Descartes)

An Overview on the Gene Therapy Trials for Leukodystrophies that We are Running

Katherine High (Spark Therapeutics)

Gene therapy in 2015: Clinical successes spark a renaissance

**Keiya Ozawa** (Center for Gene & Cell Therapy (CGCT) and The Advanced Clinical Research Center, The Institute of Medical Science, The University of Tokyo/ Division of Immuno-Gene & Cell Therapy (Takara Bio), Jichi Medical University)

RECENT TRENDS OF GENE THERAPY IN JAPAN

### The 1st International Symposium for Asia-Pacific Gene Therapy Consortium

Date: July 26, 2015, 8:30-11:30, Hall 1

Chairpersons: Masatoshi Tagawa (Chiba Cancer Center Research Institute) & Chae-Ok Yun (Hanyang University) Keynote

Yasufumi Kaneda (Division of Gene Therapy Science, Graduate School of Medicine, Osaka University)

Wen Ye (State Key Laboratory of Oncology in South China, Collaborative Innovation Center of Cancer Medicine, Sun Yat-sen University Cancer Center, Guangzhou, China)

Multicenter Randomized Phase II Clinical Trial of a Recombinant Human Endostatin Adenovirus in Patients with Advanced Head and Neck Carcinoma

Ding-Gang Li (Beijing Haidian Hospital, China)

Early Efficacy Study of Gemcitabine Plus Intraperitoneal Cisplatin in Combination With Ad-p53 Plus regional Thermotherapy in Advanced Pancreatic Cancer With Ascites

Chae-Ok Yun (Department of Bioengineering, College of Engineering, Hanyang University, Seoul, Korea)
Using a magnetic field to redirect an oncolytic adenovirus complexed with iron oxide augments gene therapy efficacy

Dario Campana (NATIONAL UNIVERSITY OF SINGAPORE)

Advances in cancer therapy with genetically-modified immune cells

Paul Gregorevic (NHMRC R.D. Wright Biomedical Fellow)

Using Gene Therapy Technology To Study And Treat Skeletal Muscle - Related Disorders

Masatoshi Tagawa (Division of Pathology and Cell Therapy, Chiba Cancer Center Research Institute) A therapeutic strategy for malignant mesothelioma with targeting the genetic abnormality

### The 21st Annual Meeting of Japan Society of Gene Therapy

### 6<sup>th</sup> Takara Bio Award Lecture

Date: July 24, 2015, 10:35-10:50, Hall 1

Chairperson: Yasufumi Kaneda (Osaka University)

Tomoyuki Nishikawa (Division of Gene Therapy Science, Graduate School of Medicine, Osaka University)
Systemic Administration of Platelets Incorporating Inactivated Sendai Virus Eradicates Melanoma in Mice.

### Plenary Session (Abstracts PS-1~PS-6)

Date: July 24, 2015, 8:40-10:10, Hall 2

Chairpersons: Toshiyoshi Fujiwara (Okayama University) & Sumio Sugano (The University of Tokyo)

PS-1. Ultrasound-mediated transient modulation of blood-brain interface in adult common marmoset to induce brain pathology with rAAV

Okada H., Ishibashi H., Masuda C., Hayashita-Kinoh H., Chiyo T., Nitahara-Kasahara Y., Endo-Takahashi Y., Kato K., Negishi Y., Takeda S., Okada T.

PS-2. ENGINEERED INTEGRATION-FREE iPS CELLS USING ARTIFICIAL CHROMOSOME VECTORS FOR HEMOPHILIA A THERAPY

Kurosaki H., Yakura Y., Hiratsuka M., Takehara S., Yoshino T., Kazuki Y., Nakamura T., Oshimura M.

PS-3. Mesenchymal stromal cells ameliorate progressive phenotype of Duchenne muscular dystrophy in dog

Kasahara Y., Kinoh H., Kuraoka M., Chiyo T., Okada H., Tsumita N., Imagawa K., Tachibana K., Takeda S., Okada T.

PS-4. Oncolytic herpes simplex virus type 1 mutant HF10 in combination with anti-PD-1 antibody enhanced systemic anti-tumor effect.

Tsuda H., Furui S., Okamoto S., Inoue K., Chono H., Tanaka M., Ikeda H., Shiku H., Mineno J.

PS-5. Three-dimensional imaging of multiple AAV vector distributions in the non-human primate brain

Adachi K., Liu Z., Kroenke C., Dissen G., Ojeda S., Nakai H.

PS-6. The development of IL-17 vaccine.

Koriyama H., Nakagami H., Morishita R.

### Day 2: July 25, 2015

### Young Investigator Award Session (Abstracts YIA-7~YIA-12)

12:30-13:30, Hall 3

Chairpersons: Shin'ichi Takeda (National Center of Neurology and Psychiatry) & Masato Nakanishi (National Institute of Advanced Industrial Science and Technology)

YIA-7. Gene therapy to rescue lethal hypophosphatasia model mice by adeno-associated virus-mediated muscle transduction of bone-targeted alkaline phosphatase

Nakamura A., Miyake K., Watanabe A., Hirai Y., Miyake N., Iijima O., Adachi K., Kinoshita H., Noguchi T., Abe S., Shimada T., Okada T.

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

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

YIA-9. Newly developed measles virus vector simultaneously transfer multiple genes into hematopoietic cells and induce ground state like pluripotent stem cells.

Hiramoto T., Tahara M., Sakamoto C., Ono H., Kohara H., Takeda M., Tani K.

YIA-10. A potential of a third generation oncolytic HSV-1 G47 $\Delta$  as a new therapeutic agent for biliary tract cancer

Tateno Y., Ino Y., Iwai M., Shinozaki M., Todo T.

YIA-11. Striking differences in the mode of capsid assembly between adeno-associated virus serotypes 2 and 5

Earley L., Powers J., Adachi K., Meyer N., Xie Q., Chapman M., Nakai H.

YIA-12. Selective Blockade of Periostin Exon-17 Ameliorates Cardiac Performance in Acute Myocardial Infarction

Sanada F., Taniyama Y., Muratsu J., Ikeda-Iwabu Y., Morishita R.

### Oral Session I (Abstracts OR-13~OR-17)

9:00-9:50, Hall 2

Chairpersons: Takashi Okada (Nippon Medical School) & Yoshiaki Taniyama (Osaka University)

OR-13. Development of novel RNA drugs for cancer therapy *Liu L.* 

OR-14. Manufacture of MAGE-A4-specific TCR gene modified T cells in a cell processing center from patients and healthy donors

Fukushima K., Inoue N., Tanabe M., Tomura D., Nukaya I., Kageyama S., Ikeda H., Shiku H., Mineno J.

OR-15. Reovirus mediates reduction in immunosuppressive acitivity of myeloid-derived suppressor cells via TLR3-dependent signaling pathway in tumor-bearing mice

Katayama Y., Oya Y., Terasawa Y., Tachibana M., Kobiyama K., Ishii K., Akira S., Mizuguchi H., Sakurai F.

## OR-16. Conditionally replicating adenovirus kills tumorigenic pluripotent stem cells *Mitsui K., Ide K., Takayama A., Wada T., Irie R., Wang Y., Kosai K.*

## OR-17. Type I IFN Gene Delivery Suppresses Regulatory T Cells within Tumors Hashimoto H., Ueda R., Narumi K., Aoki K.

### Oral Session II (Abstracts OR-18~OR-21)

9:00-9:40, Hall 3

Chairpersons: Haruhiko Kijima (Osaka University) & Masaaki Mizuno (Nagoya University Hospital Center)

#### OR-18. NMJ-enlarging therapy for neuromuscular diseases

Arimura S., Okada T., Tezuka T., Chiyo T., Kasahara Y., Yoshimura T., Motomura M., Yoshida N., Beeson D., Takeda S., Yamanashi Y.

## OR-19. INVOSSA™ (TissueGene-C) IN PATIENTS WITH OSTEOARTHRITIS: A clinical study of phase II in US

Cho J., Kim T., Park Y., Noh M., Lee K., Lee B.

## OR-20. FEASIBLE GENE TRANSDUCTION IN NON-HUMAN PRIMATE MUSCLE WITH RECONBINANT AAV FOLLOWING IMMUNOMODULATION

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

## OR-21. Generation and Evaluation of an Oncolytic Herpes Simplex Virus Expressing a Therapeutic Antibody

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

### Oral Session III (Abstracts OR-22~OR-26)

9:50-10:40, Hall 2

Chairpersons: Yasushi Inao (The University of Tokyo) & Tomoyuki Nishikawa (Osaka University)

## OR-22. Therapeutic efficacy of IL-12-expressing oncolytic herpes simplex virus type 1 in mouse melanoma models

Higuchi A., Ino Y., Fukuhara H., Iwai M., Todo T.

# OR-23. TREATMENT OF A MOUSE ORTHOTOPIC ESOPHAGEAL SQUAMOUSE CELL CARCINOMA MODEL USING G47 $\Delta$ , A THIRD GENERATION ONCOLYTIC HSV-1

Yajima S., Ino Y., Fukuhara H., Iwai M., Seto Y., Todo T.

### OR-24. Retroviral replicating vector (RRV)-mediated prodrug activator gene therapy for pancreatic cancer

Inoko K., Hiraoka K., Inagaki A., Takano H., Sato S., Takahashi M., Nakamura T., Tsuchikawa T., Shichinohe T., Gruber H., Jolly D., Kasahara N., Hirano S.

## OR-25. Therapeutic efficacy of retroviral replicating vectors expressing thymidine kinase prodrug activator genes in experimental models of human glioma

Inagaki A., Hiraoka K., Kamijima S., Sato M., Bogan B., Logg C. R., Gruber H. E., Robbins J. M., Lin A. H., Jolly D. J., Kasahara N.

OR-26. Coxsackievirus A11 causes potent oncolytic activity and immunogenic cell death in human non-small cell lung cancer cells

Kuroda M., Inoue H., Sagara M., Miyamoto S., Ogata H., Nakano Y., Wang B., Yamada K., Shimizu H., Nakanishi Y., Tani K.

### Oral Session IV (Abstracts OR-27~OR-31)

9:50-10:40, Hall 3

Chairpersons: Norio Sakai (Osaka University) & Kotaro Saga (Osaka University)

OR-27. A Gene Therapy Clinical Study of a Patient with Chronic Granulomatous Disease

Kawai T., Goto F., Nakazawa Y., Uchiyama T., Watanabe N., Yagita M., Igarashi Y., Mizukami T., Nunoi

H., Onodera M.

- OR-28. Perinatal Gjb2 gene transfer rescues hearing in a mouse model of hereditary deafness *Ikeda K., Kamiya K., Iizuka T., Minowa O.*
- OR-29. Successful treatment of neonatal metachromatic leukodystrophy model mice by intravenous injection of self-complementary AAV type 9 vector expressing ASA Miyake N., Miyake K., Yamamoto M., Shimada T., Okada T.
- OR-30. Long-term efficacy of factor IX gene expression following AAV8-mediated liver transduction in macaques

Mizukami H., Ohmori T., Uchibori R., Tsukahara T., Saga Y., Urabe M., Kume A., Sakata Y., Ozawa K.

OR-31. Tumor-associated antigen gene-loading polyplex micelle: a promising platform of anti-tumor DNA vaccine

Cui L., Furugaki K., Osada K., Kataoka K., Nakano K.

### Oral Session V (Abstracts OR-32~OR-35)

10:40-11:20, Hall 2

Chairpersons: Kaku Nakano (Kyushu University) & Yui Harada (Kyushu University)

- OR-32. Novel Gene Delivery System in Combination with Pyrotechnic Needle-free Injection System *Nakagami H.*
- OR-33. Cationic Nanocarriers Induce Cell Necrosis Through Impairment of Na<sup>+</sup>/K<sup>+</sup>-ATPase and Cause Subsequent Inflammatory Response

  Wei X.
- OR-34. Type I IFN signaling induced by systemically administrated adenovirus vector promotes the antigen-specific mucosal immunity.

Hemmi M., Tachibana M., Shoji M., Sakurai F., Kawabata K., Kobiyama K., Ishii K., Akira S., Mizuguchi H.

## OR-35. Angiotensin II type 1 receptor antagonist improves lipoprotein (a) induced atherosclerosis via integrin $\alpha V\beta 3$ pathway suppression

Muratsu J., Taniyama Y., Iwabayashi M., Sanada F., Ikeda-Iwabu Y., Otsu R., Shibata K., Brule M., Rakugi H., Morishita R.

### Oral Session VI (Abstracts OR-36~OR-39)

10:40-11:20, Hall 3

Chairpersons: Kazunori Aoki (National Cancer Center Research Institute) & Hiroaki Kinoh (Innovation Center of Nano Medicine)

OR-36. NF-κB mediates leaky expression of adenovirus genes in a replication-incompetent adenovirus vector

Machitani M., Sakurai F., Tachibana M., Mizuguchi H.

- OR-37. Oncolytic virotherapy using genetically engineered mammalian reoviruses Kanai Y., Kawagishi T., Matsuura Y., Kobayashi T.
- OR-38. Influence of virus production methods on genotoxic potential of gene transfer vectors Takahashi S., Uchiyama T., Masafumi O.
- OR-39. Enhancing tumor specificity and therapeutic index of oncolytic vaccinia virus through deletions of both VGF and O1 protein genes

Nakatake M., Yamane M., Parada R., Horita K., Okazaki M., Hasegawa K., Miyara A., Fujiwara K., Kurosaki H., Nakamura T.

### Day 1: July 24, 2015

#### Poster Session I-1 (Abstracts PO-40~PO-44)

18:00-19:00, Poster

Chairperson: Yukihiko Hirai (Nippon Medical School)

 $PO-40. \ The \ large-scale \ purification \ of \ rAAV1 \ from \ the \ serum-free \ cultured \ medium \ by \ ion-exchange \ and \ gel-filtration \ chromatography-steps \ with \ ultracentrifugation-free \ technique$ 

Tomono T., Hirai Y., Okada H., Adachi K., Chiyo T., Ishii A., Shimada T., Onodera M., Tamaoka A., Okada T.

PO-41. Regulatory science of viral vector for gene therapy.

Igarashi Y., Uchiyama T., Uchida E., Onodera M.

- PO-42. Oncolytic Adenovirus expressing IFN as a tool to eliminate pancreatic cancer stem cells Oliveira A. R., LaRocca C. J., Davydova J., Yamamoto M.
- PO-43. Suppression of adenovirus vector-induced hepatotoxicity at the early phase via suppression of leaky expression of adenovirus genes

Shimizu K., Sakurai F., Iizuka S., Tachibana M., Nishinaka T., Terada T., Mizuguchi H.

### PO-44. Engineering of CD19-CAR-T Cells from B-NHL Patients in combination with RetroNectin/OKT3 Stimulation.

Tahara K., Chono H., Nukaya I., Mineno J., Uehara E., Tsukahara T., Ohmine K., Ozawa K., Takesako K.

### Poster Session I-2 (Abstracts PO-45~PO-49)

18:00-19:00, Poster

Chairperson: Toshiro Shirakawa (Kobe University)

## PO-45. ONCOLYTIC VIRUS THERAPY USING RECOMBINANT HSV-1 FOR NONSEMINOMA TESTICULAR CANCER

Kakutani S., Fukuhara H., Taguchi S., Takeshima Y., Homma Y., Ino Y., Todo T.

- PO-46. Single-cell-state culture of human pluripotent stem cells increases transfection efficiency Nii T., Kohara H., Tani K.
- PO-47. Vicious cycle of inflammation via humoral factor in macrophages and adipocytes is inhibited by HGF

Ohtsu R., Taniyama Y., Kusunoki H., Sanada F., Muratsu J., Rakugi H., Morishita R.

- PO-48. Viral/transgene/feeder-free iPS cell-based functional hepatocytes Harada Y., Yonemitsu Y.
- PO-49. Re-evaluation of thymidine kinase mutants as a safety switch for iPS cells Urabe M., Abe T., Uchibori R., Tsukahara T., Kume A., Mizukami H., Hanazono Y., Ozawa K.

### Poster Session I-3 (Abstracts PO-50~PO-54)

18:00-19:00, Poster

Chairperson: Naoyuki Sato (Osaka University)

- PO-50. Combinational prodrug activator gene therapy using retroviral replicating vectors derived from amphotropic murine leukemia virus and gibbon ape leukemia virus *Kubo S., Takagi-Kimura M., Kasahara N.*
- PO-51. Best CAR design based on the analyses of ligand-independent activation of CD19-CAR T cells

Chono H., Amaishi Y., Pei Z., Okamoto S., Mineno J.

PO-52. Quantitative analysis of virus-associated RNAI expression following transduction with a replication-incompetent adenovirus vector *in vitro* and *in vivo*.

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

PO-53. An intranuclear RNA virus-based episomal vector system for long-term stem cell modification

Tomonaga K., Makino A., Holditch S., Lu B., Ikeda Y.

#### PO-54. Evaluation of transduction properties of an adenovirus vector in neonatal mice

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

### Poster Session I-4 (Abstracts PO-55~PO-59)

18:00-19:00, Poster

Chairperson: Nobuhiko Emi (Fujita Hospital University)

### PO-55. COMBINATION THERAPY AGAINST HUMAN COLORECTAL CANCER WITH THE EPI-DERMAL GROWTH FACTOR RECEPTOR INHIBITOR CETUXIMAB AND ONCOLYTIC HERPES SIMPLEX VIRUS HF10

Wu Z., Ichinose T., Luo C., Tan G., Yamada S., Fujii T., Sugimoto H., Nishikawa Y., Hotta Y., Koide U., Fukuda S., Kuwahara T., Kodera Y., Tanaka M., Kasuya H.

### PO-56. RADIOSENSITIZATION OF BONE AND SOFT TISSUE SARCOMA CELLS BY TUMOR-SPE-CIFIC ONCOLYTIC ADENOVIRUS

Omori T., Sugiu K., Yamakawa Y., Hasei J., Tazawa H., Osaki S., Sasaki T., Fujiwara T., Kunisada T., Urata Y., Ozaki T., Fujiwara T.

PO-57. Heat shock protein 90 inhibitors augment endogenous p53 in p53 wild-type tumors but suppress Ad-mediated over-expressed exogenous p53 levels

Tagawa M., Okamoto S., Morinaga T., Kozono T., Kubo S., Shingyoji M., Sekine I., Tada Y., Tatsumi K., Shimada H., Hiroshima K.

PO-58. Preclinical evaluation of tumor-specific oncolytic virotherapy against human colorectal cancer cells with different KRAS/BRAF mutation and microsatellite stability

Tamura S., Tazawa H., Hori N., Koujima T., Kikuchi S., Kuroda S., Kishimoto H., Nagasaka T., Nishizaki M., Urata Y., Kagawa S., Fujiwara T.

PO-59. Preclinical evaluation of tumor-specific oncolytic virotherapy against pancreatic cancer cells with different invasion ability

Koujima T., Tazawa H., Hori N., Tamura S., Kuroda S., Kishimoto H., Nishizaki M., Urata Y., Kagawa S., Fujiwara T.

### Poster Session I-5 (Abstracts PO-60~PO-63)

18:00-19:00, Poster

Chairperson: Makoto Abei (University of Tsukuba)

- PO-60. Repeated whole cell vaccine using oncolytic virus against mouse colon cancer model *Yamano T., Kubo S., Yano A., Okamura H., Tomita N.*
- PO-61. Combination treatment of human head and neck cancer xenograft models with cetuximab and oncolytic herpes simplex virus HF10

Koide Y., Zhiwen W., Hotta Y., Ichinose T., Luo C., Tanaka M., Fujimoto Y., Kodera Y., Kasuya H.

PO-62. Effective Systemic Treatment with Fiber-redesigned Oncolytic Adenovirus in Pancreatic Cancer *in vivo* Model

Miura Y., Sato M., Masato Y.

PO-63. Cancer-selective apoptosis in human multiple myeloma cells through downregulation of c-Myc oncogene by inactivated Sendai virus (HVJ envelope)

Jiang Y.

### Poster Session I-6 (Abstracts PO-64~PO-67)

18:00-19:00, Poster

Chairperson: Hiroyuki Nunoi (University of Miyazaki)

PO-64. High efficiency lentiviral gene transfer to pluripotent stem cell allows disease modeling and phenotypic correction of late-onset Pompe disease iPSC

Sato Y., Kobayashi H., Eto Y., Ida H., Ohashi T.

PO-65. AAV-mediated Gene Therapy Efficiently Mitigates Cerebellar Neurodegeneration in Globoid Cell Leukodystrophy Mice

Lin D., Wang T., Chiang M., Liu H., Lee A., Hsiao C., Ho C.

PO-66. Characterization of domains within ZFP809 essential for nuclear localization and gene silencing

Ichida Y., Utsunomiya Y., Sato T., Onodera M.

PO-67. CRISPR/Cas9-mediated gene knockout of NANOG and NANOGP8 leads to low malignant potential of prostate cancer cells

Kawamura N., Nimura K., Nagano H., Nonomura N., Kaneda Y.

### Poster Session I-7 (Abstracts PO-68~PO-72)

18:00-19:00, Poster

Chairperson: Kenji Nakano (Kyushu University)

PO-68. Blockade of CRE activity represses intimal hyperplasia via decrease of vascular smooth muscle cell proliferation and migration

Uchida D., Saito Y., Azuma N.

- PO-69. The Effect of Fibrosis on Gene Delivery Efficiency of Hydrodynamics-based Gene Delivery Kamimura K., Abe H., Kobayashi Y., Ohtsuka M., Miura H., Yokoo T., Suda T., Tsuchida M., Liu D., Terai S.
- PO-70. Gene Therapy for alveolar bone regeneration using *in vivo* electroporation *Kawai M., Kataoka Y., Ohura K., Yamamoto T.*
- PO-71. Development of Novel Decoy-Coated Balloon Catheter: Prevention of Neointimal Formation After Angioplasty Using NF-kappaB Decoy Oligodeoxynucleotide-Coated Balloon Catheter in Rabbit Restenosis Model

Miyake T., Miyake T., Hamada H., Morishita R.

PO-72. Sonoporation-mediated gene transfer for acute kidney injury.

Ishida R., Kami D., Adachi T., Yuhei K., Tetsuro K., Tamagaki K., Gojo S.

### Day 2: July 25, 2015

### Poster Session II-1 (Abstracts PO-73~PO-76)

17:20-18:20, Poster

Chairperson: Yumi Kanegae (Jikei University School of Medicine)

PO-73. An efficient construction of lentiviral vectors that identify and eliminate tumorigenic cells in pluripotent stem cells

Ide K., Mitsui K., Matsushita Y., Kosai K.

PO-74. Triple Targeting of Regular and TDEC Angiogenesis, and Hyperinvasiveness: A Breakthrough on Resistance to Anti-angiogenic Therapies in Glioblastoma

Soda Y., Myskiw C., Soda M., Ke E., Kesari S., Verma I.

- PO-75. BubR1 insufficiency reduces atherosclerosis in apolipoprotein E-deficient mice *Tanaka S.*
- PO-76. Estimation of Vector Copy Number States by Droplet Digital PCR Lin H., Otsu M., Nakauchi H.

### Poster Session II-2 (Abstracts PO-77~PO-80)

17:20-18:20, Poster

Chairperson: Eriko Uchida (National Institute of Helth Sciences)

PO-77. Enhanced transduction and antitumor efficiency of fiber-modified, CD133-targeted oncolytic adenovirus in colon cancer

Sato M., Miura Y., Davydova J., Yamamoto M.

PO-78. Inhibitory effects of adenovirus neutralizing antibodies against different adenoviral capsid proteins on transduction with an adenovirus vector

Tomita K., Sakurai F., Machitani M., Tachibana M., Mizuguchi H.

PO-79. An Oncolytic Adenovirus Employing the Cox2 Promoter Demonstrates Selective Replication in Human Esophageal Adenocarcinoma Specimens

LaRocca C. J., Oliveira A. R., Andrade R., Davydova J., Yamamoto M.

PO-80. A super gene expression system enhances the anti-glioma effects of adenovirus-mediated REIC/Dkk-3 gene therapy

Oka T., Kurozumi K., Ichikawa T., Shimazu Y., Otani Y., Shimizu T., Sakaguchi M., Watanabe M., Nasu Y., Kumon H., Date I.

### Poster Session II-3 (Abstracts PO-81~PO-84)

17:20-18:20, Poster

Chairperson: Makoto Otsu (The University of Tokyo)

PO-81. Scalable production of AAV vector by baculovirus expression system for clinical use Enoki T., Kawano Y., Sakamoto S., Takakura H., Mineno J., Okada T., Ohmori T., Mizukami H., Ozawa K., Sakata Y. PO-82. Simple and scalable serotype-independent purification method of AAV vector Kawano Y., Huang S., Enoki T., Takakura H., Kitagawa M., Mineno J.

PO-83. Enhancing anti-tumor immune response of HF10 virotherapy through HSV amplicon carrying cancer therapeutic gene

Luo C., Shibata K., Goshima F., Wu Z., Hotta Y., Ichinose T., Koide Y., Tanaka M., Kodera Y., Kasuya H.

PO-84. Nonviral gene therapeutic vector designed with water-soluble nanodiamond

Kojima H., Nakae Y., Terashima T., Isoda S., Tanaka S., Katagi M., Ogawa N., Okano J.

### Poster Session II-4 (Abstracts PO-85~PO-90)

17:20-18:20, Poster

Chairperson: Teruhiko Yoshida (National Cancer Center Research Institute)

PO-85. Preclinical evaluation of telomerase-specific p53 tumor suppressor gene overexpression in human scirrhous gastric cancer cells with different p53 status

Hori N., Tazawa H., Nishizaki M., Watanabe M., Tamura S., Koujima T., Kuroda S., Urata Y., Kagawa S., Fujiwara T.

PO-86. Effects of chemotherapy and radiation on replication of interferon-expressing adenovirus in pancreatic cancer cells

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

PO-87. Reovirus-mediated lysis of cancer-associated fibroblasts.

Kaminade T., Sakurai F., Tachibana M., Mizuguchi H.

PO-88. Analysis of combination therapy of the adenovirus vector carrying REIC/Dkk-3 (Ad-REIC) and the integrin antagonist cilengitide

Shimizu T., Shimazu Y., Kurozumi K., Ichikawa T., Fujii K., Onishi M., Ishida J., Oka T., Ohtani Y., Watanabe M., Nasu Y., Kumon H., Date I.

PO-89. VIRUS-GUIDED FLUORESCENCE IMAGING OF INTRAPERITONEAL FREE GASTRIC CANCER CELLS.

Watanabe M., Kagawa S., Kuwata K., Hori N., Kikuchi S., Kuroda S., Kishimoto H., Nishizaki M., Tazawa H., Urata Y., Fujiwara T.

PO-90. COMBINATION WITH CHEMOTHERAPY ENHANCES THE ANTITUMOR EFFECT OF ON-COLYTIC HERPES SIMPLEX VIRUS TYPE 1 AGAINST COLORECTAL CANCER

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

### Poster Session II-5 (Abstracts PO-91~PO-96)

17:20-18:20, Poster

Chairperson: Hiroaki Mizukami (Jichi Medical University)

 $PO\text{-}91\text{.}\ G47\Delta$  is effective and safe in treating advanced mouse tongue tumor model

Uchihashi T., Ino Y., Nakahara H., Hukuhara H., Iwai M., Kogo M., Todo T.

PO-92. Ad-SOCS inhibited the cell growth and increased the sensitivity to LAK cell therapy of human prostate cancer cell line

Saito H., Ando S., Kitagawa K., Nakanishi A., Naka T., Serada S., Shirakawa T.

## PO-93. TUMOR TARGETING STRATEGY OF ONCOLYTIC HERPES SIMPLEX VIRUS USING HITCHHIKING ON ANTIGEN-SPECIFIC LYMPHOCYTES

Ichinose T., Kanzaki A., Luo C., Hotta Y., Koide Y., Wu Z., Fukuda S., Kuwahara T., Yamada S., Fujii T., Sugimoto H., Tanaka M., Kodera Y., Kasuya H.

PO-94. Nucleic acid vaccine elicits anti-tumor immunity by targeting the mutant Kras in a spontaneous lung cancer model

Weng T., Huang C., Lai M.

PO-95. DC-Dependent efficient prevention of spontaneous neuroblastoma in MYCN transgenic mice Kawakubo N., Harada Y., Souzaki R., Kinoshita Y., Taguchi T., Yonemitsu Y.

### Poster Session II-6 (Abstracts PO-96~PO-99)

17:20-18:20, Poster

Chairperson: Hirokazu Miyake (Nippon Medical School)

PO-96. Tumor Specific and Infectivity Enhanced Adenovirus Progresses Radioiodine Theranostics to Non-Thyroid Cancer

Eidenschink B., Sell J., LaRocca C. J., Jacobsen K., Trujilo M., Frenandez-Zapico M., Morris J., Yamamoto M., Davydova J.

PO-97. CRISPR/Cas9-mediated targeted insertion of a safety switch in an extragenic safe harbor of induced pluripotent stem cells

Kimura Y., Kanemura Y., Shofuda T., Onodera M., Oda M., Nakamori M., Nakano T., Mochizuki H.

PO-98. Identification of host factors required for enhancing replication and spread of oncolytic vaccinia virus

Horita K., Nakatake M., Goto I., Yamane M., Okazaki M., Parada R., Kurosaki H., Nakamura T.

PO-99. Embryo-fetal distribution of beperminogene perplasmid following single intramuscular administration to pregnant rats during the early embryonic stages

Komatsuno T., Miyake T., Oguro M., Yoshida K., Tomioka H., Ihara S., Nakazawa T., Morishita R.

### Poster Session II-7 (Abstracts PO-100~PO-103)

17:20-18:20. Poster

Chairperson: Jun Watanabe (Nippon Medical School Hospital)

PO-100. Improved transduction of canine X-linked muscular dystrophy with rAAV9- microdystrophin by MSCs pretreatment

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

PO-101. Protection of the retina in rat retinal ischemic injury model by triple mutant AAV2-mediated BDNF transduction

Kobayashi M., Igarashi T., Miyake K., Miyake N., Iijima O., Nakamoto K., Hirai Y., Takahashi H., Okada T.

PS-102. Co-delivery of EGFR and HSV-tk by LCMV-pseudotyped bicistronic lentiviral vectors to enhance therapeutic gene distribution for glioblastoma treatment

Hossain J. A., Riecken K., Fehse B., Miletic H.

PO-103. Periostin C-terminus is involved in tumor-stromal interactions in triple negative breast cancer.

Ikeda-Iwabu Y., Taniyama Y., Morishita R.

### **Corporate Seminars**

Day 1: July 24, 2015

### Luncheon Seminar I (Astellas Pharma Inc./Nippon Boehringer Ingelheim Co., Ltd.)

12:00-12:50, Hall 1

Chairperson: Ryuichi Morishita (Osaka University)

Hypertension and Cognitive Impairment; Roles of Cross-Talk of Renin Angiotensin System and  $PPAR_{\gamma}$ 

Masatsugu Horiuchi (Department of Molecular Cardiovascular Biology and Pharmacology, Ehime University Graduate School of Medicine)

# Luncheon Seminar II (TAKARA BIO INC.)

12:00-12:50, Hall 2

Chairperson: Junichi Mineno (TAKARA BIO INC.)

Development in AAV vector-based technology and its application to the gene and cell therapy for neuromuscular diseases

Takashi Okada (Department of Biochemistry and Molecular Biology, Nippon Medical School)

Luncheon Seminar III (AnGes MG, Inc.)

12:00-12:50, Hall 3

Chairperson: Keiya Ozawa (The University of Tokyo)

**Clinical Advances in DNA Vaccines** 

Vijay Samant (Vical Inc.)

Day 2: July 25, 2015

Luncheon Seminar IV (AnGes MG, Inc.)

11:30-12:20, Hall 1

Chairperson: Yoshikatsu Eto (Institute for Neurological Disorders)

The Current Status and Prospects for Gene Therapy

Barrie J. Carter (BioMarin Pharmaceutical Inc.)

### Luncheon Seminar V (Nippon Boehringer Ingelheim Co., Ltd. / Eli Lilly Japan K.K.)

11:30-12:20, Hall 2

Chairperson: Ryuichi Morishita (Osaka University)

Genetic association study as a tool to identify and validate the drug target for metabolic diseases *Kazuo Hara (Tokyo Medical University)* 

### Luncheon Seminar VI (Mitsubishi Tanabe Pharma Corporation)

11:30-12:20, Hall 3

Chairperson: Nobuyuki Takakura (Osaka University)

#### New insight into pathophysiology towards diabetes treatment

Yukio Tanizawa (Division of Endocrinology, Metabolism, Hematological Sciences and Therapeutics Yamaguchi University Graduate School of Medicine)

Day 3: July 26, 2015

# Luncheon Seminar VII (Novartis Pharma K.K.)

11:40-12:30, Hall 1

Chairperson: Hideki Mochizuki (Osaka University)

### "Current and future treatment for Parkinson disease" Gene Therapy vs Cell Therapy

Shinichi Muramatsu (Division of Neurology, Jichi Medical University Project Professor, Center for Gene & Cell Therapy, The Institute of Medical Science, The University of Tokyo)

# Luncheon Seminar VIII (tella, Inc.)

11:40-12:30, Hall 2

Chairperson: Yoshikazu Yonemitsu (Kyushu University)

#### Update on oncolytic virus therapy

Tomoki Todo (Division of Innovative Cancer Therapy, and Department of Surgical Neuro · Oncology The Institute of Medical Science, The University of Tokyo)

# Luncheon Seminar IX (BioReliance, K.K.)

11:40-12:30, Hall 3

Chairperson: Makoto Kitano (Sigma-Aldrich Japan G.K.)

Cell and Gene Therapy Manufacturing and Biosafety Testing- Strategies and Advice to Facilitate Release to the Clinic and Market

Pamela Hamill (Development Services, BioReliance UK)

Day 2: July 25, 2015

## Coffee Break Seminar (DAIICHI SANKYO COMPANY, LIMITED)

Recent progress of treatment in cerebrovascular disease

14:10-15:40, Hall 3

Chairperson: Ryuichi Morishita (Osaka University)

#### CB-1. Topics of neuroendovascular therapy

Nobuyuki Sakai (Department of Neurosurgery, Kobe City Medical Center General Hospital, Japan)

#### CB-2. Pharmacotherapy for acute ischemic stroke

Kazuo Kitagawa (Department of Neurology, Tokyo Women's Medical University, Japan).

### CB-3. Cell therapy for stroke patients

Akihiko Taguchi (Department of Regenerative Medicine Research, Institute of Biomedical Research and Innovation, Japan)