

Program

Chairman's Lecture (J)

Date: Jul. 16, 11:20-11:50, Room 1 (501+502)

Chair: *Yoshikatsu Eto (Advanced Clinical Research Center, Southern Tohoku Institute for Neuroscience)*

CL. Future direction of JSCGT

Ryuichi Morishita (*Department of Clinical Gene Therapy, Graduate School of Medicine, Osaka University*)

Presidential Lecture (J)

Chairman's Lecture

Date: Jul. 17, 14:10-14:40, Room 1 (501+502)

Chair: *Yoshikazu Yonemitsu (R&D Laboratory for Innovative Biotherapeutics Science, Graduate School of Pharmaceutical Sciences, Kyushu University)*

PL. Gene therapy using the migratory activity of neural stem cells

Masahiro Toda (*Department of Neurosurgery, Keio University School of Medicine*)

Special Lecture 1 (E)

Date: Jul. 16, 13:10-14:00, Room 1 (501+502)

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

SL1. Neural stem cell delivery of an oncolytic adenovirus in malignant glioma patients

Maciej S. Lesniak (*Department of Neurological Surgery, Northwestern University Feinberg School of Medicine*)

Special Lecture 2 (J)

Date: Jul. 17, 14:40-15:30, Room 1 (501+502)

Chair: *Masafumi Onodera (Gene & Cell Therapy Promotion Center)*

SL2. Governmental commitment to create the "Innovation Ecosystem" of gene and cell therapy in Japan

Toshiharu Furukawa (*Members of the House of Councillors, Department of Surgery, Keio University School of Medicine*)

Special Lecture 3 (J)

Date: Jul. 18, 12:30-13:20, Room 1 (501+502)

Chair: *Toya Ohashi (The Jikei University School of Nursing)*

SL3. Development of Regenerative Medicine for the CNS Disorders: Challenges Using Genome Editing and Ex vivo Gene Therapy

Hideyuki Okano (*Keio University*)

Educational lecture 1 (E)

Date: Jul. 16, 15:50-16:20, Room 1 (501+502)

Chair: *Masato Yamamoto (Department of Surgery, University of Minnesota)*

EL1. Lesson from first clinical trial of oncolytic HSV therapy in recurrent glioblastoma and future development

Hiroshi Nakashima (*Brigham and Women's Hospital, and Harvard Medical School*)

Educational lecture 2 (J)

Date: Jul. 17, 17:20-17:50, Room 1 (501+502)

Chair: *Masahiro Toda (Department of Neurosurgery, Keio University School of Medicine)*

EL2. Deep phenotyping of disease and therapeutic cells using AI

Kazuhiro Sakurada (*Keio University School of Medicine*)

Educational Lecture 3 (J)

Date: Jul. 18, 10:40-11:10, Room 1 (501+502)

Chair: Takafumi Nakamura (Division of genomic Medicine, Tottori University Faculty of Medicine)

EL3. Clinical development of oncolytic herpes viruses

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

ASGCT/ESGCT/JSGCT Joint Symposium (E)

Date: Jul. 16, 16:30-18:00, Room 1 (501+502)

Chairs: Takafumi Nakamura (Division of genomic Medicine, Tottori University Faculty of Medicine)

Noriyuki Kasahara (Departments of Neurological Surgery and Radiation Oncology University of California)

JS-1. Recent advances in the treatment of primary immunodeficiencies

Juan A. Bueren (Director of the Biomedical Innovation Unit. CIEMAT/National Institute for Rare Diseases (CIBERER))

JS-2. Lessons Learned from Thirty Years of Clinical AAV Gene Therapy

Terence R Flotte (Department of Pediatrics, Horae Gene Therapy Center and Microbiology and Physiological Systems (MaPS) University of Massachusetts Chan Medical School)

JS-3. Recent Progress in Cardiovascular Gene Therapy

Ryuichi Morishita (Department of Clinical Gene Therapy, Graduate School of Medicine, Osaka University)

TAKARA BIO Research Award 2024 (J)

Date: Jul. 17, 13:40-14:00, Room 1 (501+502)

Chair: Ryuichi Morishita (Chairman of the Board of Director, The Japan Society of Gene and Cell Therapy / Department of Clinical Gene Therapy, Graduate School of Medicine, Osaka University)

Development of brain cell type-specific adeno-associated virus vectors that penetrate the primate blood-brain barrier

Hirokazu Hirai (Department of Neurophysiology & Neural Repair, Gunma University Graduate School of Medicine / Viral Vector Core, Gunma University Initiative for Advanced Research (GIAR))

The Japanese Society for Regenerative Medicine Joint Program (J)

Date: Jul. 16, 14:10-15:40, Room 1 (501+502)

Chairs: Takashi Okada (Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, IMSUT, The University of Tokyo)

Masayo Takahashi (VC Cell Therapy Inc., Kobe City Eye Hospital, Ritsumeikan University)

RM-1. Establishing a Consortium for Academia-driven Drug Discovery and Commercialization of Non-viral CAR-T Cells

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

RM-2. iPSC-derived next-generation T cell therapy for cervical cancer

Miki Ando (Department of Hematology, Juntendo University School of Medicine)

RM-3. Development of the gene replacement therapy for the treatment of spinal muscular atrophy (SMA)

Toru Hirose (Novartis Pharma KK)

RM-4. Development of dosing protocol to reduce the required dose of rAAV using mesenchymal stem cells

Hiroimi Hayashita-Kinoh (Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, IMSUT, The University of Tokyo)

RM-5. Sustainable regenerative medicine

Masayo Takahashi (VC Cell Therapy Inc., Kobe City Eye Hospital, Ritsumeikan University)

The Japanese Society of Child Neurology Joint Program (J)

Date: Jul. 17, 10:00-11:30, Room 2 (303)

*Chairs: Hitoshi Osaka (Department of Pediatrics, Jichi Medical University)
Gaku Yamanaka (Department of Pediatrics and Adolescent Medicine, Tokyo Medical University)*

CN-1. New therapeutic management strategies for patients with spinal muscular atrophy
Tomokazu Kimizu (*Osaka Women's and Children's Hospital*)

CN-2. Splice-switching antisense oligonucleotides for treatment of genetic disorders
Tomonari Awaya (*Kyoto University Graduate School of Medicine, Center for Anatomical, Pathological, and Forensic Medical Researches*)

CN-3. Current status of neuronal cell therapy for Dravet syndrome
Kiyoshi Egawa (*Division of Child Maternal and Female medicine, Hokkaido University Hospita*)

CN-4. Gene therapy development for child neurological diseases using AAV vectors
Karin Kojima (*Department of Pediatrics, Jichi Medical University*)

The Japanese Society for Genome Editing Joint Program (J)

Date: Jul. 18, 13:30-15:00, Room 1 (501+502)

*Chairs: Tomoji Mashimo (Division of Animal Genetics, Laboratory Animal Research Center, The Institute of Medical Science, The University of Tokyo)
Yumi Kanegae (Jikei University of Medicine)*

GE-1. Development of genome-editing treatment for inherited disorders
Yuji Kashiwakura (*Department of Biochemistry, Jichi Medical University*)

GE-2. Development of genome editing tools to introduce disease protective variants
Atsushi Hoshino (*Department of Cardiovascular Medicine Department of Nephrology, Kyoto Prefectural University of Medicine*)

GE-3. Bridge RNAs direct programmable recombination of target and donor DNA
Masahiro Hiraizumi (*Factory of Engineering, The University of Tokyo*)

GE-4. Optical manipulation of the genome
Moritoshi Sato (*Graduate School of Arts and Sciences, The University of Tokyo*)

Symposium 1 (J)

Regulatory sciences

Date: Jul. 16, 14:10-15:40, Room 2 (303)

*Chairs: Akihiro Kume (RPM Co.,Ltd)
Eriko Uchida (National Institute of Health Sciences)*

S1-1. Point-to-Consider for mRNA
Teruhide Yamaguchi (*Nihon Pharmaceutical University*)

S1-2. Point to consider for viral safety strategy of gene therapeutic products in light of ICH Q5AR2
Akira Sakurai (*Pharmaceuticals and Medical devices Agency*)

S1-3. ICH S12 (Nonclinical Biodistribution Considerations for Gene Therapy Products)
Yuto Takishima (*Pharmaceuticals and Medical devices Agency*)

S1-4. PMDA Science Board Subcommittee on Cell and Gene Therapy Products Produced in vivo
Akihiro Kume (*RPM Co.,Ltd*)

Symposium 2 (J)

Cancer gene therapy 1

Date: Jul. 16, 16:35-18:05, Room 2 (303)

*Chairs: Shigeki Yagyū (Shinshu University, Innovative Research & Liaison Organization)
Kazunori Aoki (National Cancer Center Research Institute)*

S2-1. Cell therapy for hematological malignancy - Bench to bedside at National University of Singapore -
Noriko Shimasaki (*Nagoya University Hospital Children's Cancer Center Lecturer of hospital*)

S2-2. CAR-T cell therapy for solid tumorsShigeki Yagyu (*Shinshu University, Innovative Research & Liaison Organization*)**S2-3. Two New Strategies to Improve the Limitations of Cellular Therapy for Solid Tumors**Hideki Kasuya (*Cancer Immune Therapy Research Center, International Medical Education, Nagoya University, Graduate*)**S2-4. Development of receptor-retargeted oncolytic HSV with syncytial mutations (RRsyn-oHSV)**Takuma Suzuki (*Tokyo University of Pharmacy and Life Sciences*)**Symposium 3 (E or J)**

Oncolytic virus

Date: Jul. 17, 8:30-10:00, Room 1 (501+502)

*Chairs: Hiroshi Fukuhara (Urology, Kyorin University)**Masato Yamamoto (Department of Surgery, University of Minnesota)***S3-1. Reovirus-mediated alteration of tumor microenvironment**Fuminori Sakurai (*Graduate School of Pharmaceutical Sciences, Osaka University*)**S3-2. An investigator-initiated clinical trial of third generation oncolytic virus armed with IL-12 against malignant melanoma**Ryuhei Okuyama (*Department of Dermatology, Shinshu University School of Medicine*)**S3-3. Crestostimogene grenadenorepvec: Pivotal results from CORE-001 and Phase 3 BOND-003 trials for the treatment of high risk, BCG-unresponsive Non-muscle Invasive Blad**Kasturi Vijay (*CG Oncology*)**S3-4. Advancements and Challenges in Oncolytic Virus Therapy: A Clinical Perspective.**Takashi Kojima (*National Cancer Center Hospital East, Gastrointestinal Oncology*)**Symposium 4 (E or J)**

Vector development

Date: Jul. 17, 10:00-11:30, Room 1 (501+502)

*Chairs: Hiroyuki Nakai (Oregon Health & Science University)**Hiroyuki Mizuguchi (Graduate School and School of Pharmaceutical Sciences, Osaka University)***S4-1. Capsid Engineering to Feasibilize Adenoviral Retargeting**David Curiel (*Department of Radiation Oncology, Groningen University, The Netherlands*)**S4-2. Dawning era of genomic medicines: Strategies for non-viral vectors targeting organs beyond the liver**Gaurav Sahay (*Department of Pharmaceutical Sciences, Oregon State University*)**S4-3. Development of AAV vectors for gene therapy for brain diseases**Hirokazu Hirai (*Gunma University Graduate School of Medicine*)**Symposium 5 (J)**

Cancer gene therapy 2

Date: Jul. 17, 15:40-17:10, Room 1 (501+502)

*Chairs: Yozo Nakazawa (Department of Pediatrics, Shinshu University School of Medicine)**koji Tamada (Research Institute for Cell Design Medical Science, Yamaguchi University)***S5-1. Real-world experience of CD19 CAR-T cell therapy for ALL in Japan**Itaru Kato (*Kyoto University Hospital*)**S5-2. CAR-T therapy for AML**Shoji Saito (*Shinshu University School of Medicine*)**S5-3. Current status and challenges of CAR-T therapy for hematological malignancies**Koji Kato (*Department of Medicine and Biosystemic Science, Kyushu University, School of Medicine*)**S5-4. Development of new gene-modified cellular immunotherapy for hematological malignancy**Naoki Hosen (*Department of Hematology and Oncology, Osaka University Graduate School of Medicine*)

Symposium 6 (E or J)

Neuromuscular disorders

Date: Jul. 17, 8:30-10:00, Room 2 (303)

*Chairs: Shin-ichi Muramatsu (Division of Neurological Gene Therapy, Center for Open Innovation, Jichi Medical University)
Takanori Yamagata (Department of Pediatrics, Tochigi Prefectural Rehabilitation Center)*

S6-1. Development of gene therapy for MECP2 gene-related diseases by regulating MECP2 protein expression
Eriko Jimbo (*Department of Pediatrics, Jichi Medical University*)

S6-2. Development of gene therapy for a megalencephalic leukoencephalopathy with subcortical cysts
Kenji Tanaka (*Division of Brain Sciences, Keio University School of Medicine*)

S6-3. Developing NeuroD1 gene therapy to treat neurological disorders
Gong Chen (*Co-Founder, Board Director Chief Scientific Advisor NeuExcell Therapeutics, Director Brain Repair Center Jinan University*)

S6-4. Gene Therapy for Neuromuscular Disorders in Astellas
Eiji Yoshimi (*Astellas Pharma Inc.*)

Symposium 7 (J)

Young investigators session

Date: Jul. 17, 16:20-17:50, Room 2 (303)

*Chairs: Fuminori Sakurai (Graduate School of Pharmaceutical Sciences, Osaka University)
Kenya Kamimura (Department of General Medicine, Niigata University School of Medicine)*

S7-1. Engineering the cancer-targeted oncolytic adenovirus with fiber modification
Mizuho Sato-Dahlman (*Department of Surgery, University of Minnesota, Minneapolis, MN*)

S7-2. Regulation of the immune system by nucleic acid medicine targeting mRNA stem-loop structures
Osamu Takeuchi (*Department of Medical Chemistry, Graduate School of Medicine, Kyoto University*)

S7-3. Development of basic technology for the advancement of mRNA vaccines and viral vector vaccines
Yasuo Yoshika (*Research Institute for Microbial Diseases, Osaka University*)

S7-4. Development of genome editing methods to induce gene therapy via homology directed repair.
Daisuke Matsumoto (*Graduate School of Biomedical and Health Sciences, Hiroshima University*)

Symposium 8 (J)

Genetic diseases

Date: Jul. 18, 9:00-10:30, Room 1 (501+502)

*Chairs: Hiroshi Kobayashi (Division of Gene Therapy, Research Center for Medical Sciences, The Jikei University School of Medicine)
Toru Uchiyama (Division of Molecular Pathogenesis, Department of Human Genetics, National Center for Child Health and Development)*

S8-1. Hematopoietic stem cell-targeted gene therapy in sickle cell disease
Naoya Uchida (*National Heart, Lung and Blood Institute, National Institutes of Health*)

S8-2. Lentiviral gene therapy targeting hematopoietic stem cells for Pompe disease
Yota Shimada (*Division of Gene Therapy, Research Center for Medical Sciences, The Jikei University School of Medicine*)

S8-3. Development of Novel Gene Therapy for Neurological Genetic Disorders
Kazuhiro Muramatsu (*Jichi Medical University*)

S8-4. Advances in Gene Therapy for Inborn Errors of Immunity
Toru Uchiyama (*Division of Molecular Pathogenesis, Department of Human Genetics, National Center for Child Health and Development*)

Symposium 9 (E or J)

Regenerative medicine & Cardiovascular diseases

Date: Jul. 18, 9:00-10:30, Room 2 (303)

*Chairs: Yasuhiro Ikeda (Department of Ophthalmology, Faculty of Medicine, University of Miyazaki)
Hironori Nakagami (Department of Health Development and Medicine, Graduate school of Medicine, Osaka University)*

S9-1. Gene augmentation therapy for inherited retinal dystrophy

Kazushige Tsunoda (Tokyo Medical Center)

S9-2. Establishment of visual restoration technology utilizing chimeric rhodopsin

Toshihide Kurihara (Department of Ophthalmology, Keio University School of Medicine)

S9-3.

Seitaro Nomura (Department of Cardiovascular Medicine, The University of Tokyo Hospital)

S9-4. Exploring the Mechanism of Therapeutic Effects of Mesenchymal Stem Cells in Ischemic Stroke

Munehisa Shimamura (Dept. of Gene & Stem Cell Regenerative Therapy, Osaka Univ. Grad. School of Medicine)

S9-5. Results from clinical trials show evidence of activity for a single intra-myocardial administration of Mesenchymal Precursor Cells in ischemic Heart Failure with reduced Ejection Fraction (HFrEF) patients that also have high inflammation

Michael Schuster (Mesoblast Inc.)

Plenary Session (E or J)

Date: Jul. 16, 10:10-11:10, Room 1 (501+502)

Chairs: Makoto Otsu (Kitasato University School of Allied Health Sciences)

Katsuto Tamai (Osaka University Graduate School of Medicine)

PS-1. Identification and mechanistic understanding of a novel AAV capsid exhibiting selective and enhanced liver transduction

Pratheepa Rajagopal (Oregon Health and Science University)

PS-2. Small-dose in vivo gene therapy for CNS symptoms of lysosomal disease mediated by blood-brain barrier-penetrating enzymes

Saki Matsushima (The Jikei University School of Medicine)

PS-3. Restoration of MHC expression by epigenetic modulators facilitates the efficacy of oncolytic virotherapy combined with dendritic cell vaccine in pancreatic cancer

Kanto Suemori (Department of Gastroenterological Surgery, Okayama University Graduate School of Medicine)

PS-4. Optimization of Gene Knock-in Technology Targeting the Liver by a Single AAV Vector

Tomoki Togashi (Kanazawa University)

Panel Discussion (E or J)

Cell therapy

Date: Jul. 16, 10:25-11:10, Room 2 (303)

Chairs: Ko Mitani (Saitama Medical University)

Akinobu Gotoh (Department of Education for Medical Research Base, Hyogo Medical University)

PD-1. Therapeutic Effects of Adipose-Derived Mesenchymal Stem Cells for the Treatment of Patients with Knee Osteoarthritis

Minseo Kang ((College of Pharmacy and Research Institute of Pharmaceutical Sciences, Seoul National University)

PD-2. Generation of natural killer (NK) cells from chimeric antigen receptor (CAR) -transduced human induced pluripotent stem (iPS) cells engineered with NK function-promoting genes for solid cancer therapy

Masashi Yamada (Healios K.K)

PD-3. Novel cell therapy CAR-DC suppresses the growth of heterogenous solid tumors through antigen spreading

Yoshinori Naoe (Nagoya University Graduate School of Medicine, Cancer Immune Therapy Center)

Oral Session

Oral Session 1 (E or J)

Viral vectors 1

Date: Jul. 16, 10:20-11:10, Room 3 (304)

Chairs: Yasushi Soda (Division of Molecular and Medical Genetics, Center for Gene & Cell Therapy, The Institute of Medical Science, The University of Tokyo)
Kazuhiko Kurozumi (Department of Neurosurgery, Hamamatsu University School of Medicine)

- O1-1. Method-development of rAAV producing host cell by several environmental stresses.**
Masahiro Baba (AGC Inc.)
- O1-2. Improvement of productivity and quality of rAAV in HEK293 cell by ethanol addition.**
Keita Shimizu (AGC Inc.)
- O1-3. Challenge of developing the academic infrastructure for GMP-compliant manufacturing of viral vectors**
Toshihiko Okazaki (Medical Center for Translational Research Osaka University Hospital)
- O1-4. Anti-tumor effect of a conditionally-replicative adenovirus vector containing CD44ECD-synNotch-HIF-3 α 4 fusion gene in a mouse bladder cancer xenograft model.**
Ruhan A (Kobe University)
- O1-5. Improvement of gene delivery efficiency into the mouse brain through amino acid substitution of CereAAV**
Sayuri Ueda (Takara bio inc.)

Oral Session 2 (E or J)

Genetic diseases

Date: Jul. 16, 14:00-14:50, Room 3 (304)

Chairs: Yohta Shimada (Division of Gene Therapy, Research Center for Medical Sciences, The Jikei University School of Medicine)
Naoya Uchida (National Institutes of Health)

- O2-1. Engineered Coagulation Factor VIII with Improved Activity and Secretion for Hemophilia A Gene Therapy**
Yuji Kashiwakura (Department of Biochemistry, Jichi Medical University)
- O2-2. Gene therapy for a mouse model of Niemann-Pick disease type C1 by systemic delivery of AAV2 mutant**
Toru Yasuda (National Center for Child Health and Development)
- O2-3. Cochlear gene therapy for genetic hearing loss**
Hidekane Yoshimura (Department of Otorhinolaryngology - Head and Neck Surgery, Shinshu University School of Medicine)
- O2-4. Safety test of gene therapy drug for hypophosphatasia using non-human primates**
Dongwei Zhao (Department of Gene Therapy, Nippon Medical School)
- O2-5. Enhancing Gene Delivery to Renal Tubules and Podocytes Through Context-Specific Selection of AAV Capsids**
Ranjan Das (Oregon Health and Science University School of Medicine)

Oral Session 3 (E or J)

Cancer 1

Date: Jul. 16, 14:50-15:40, Room 3 (304)

Chairs: Hideki Kasuya (Nagoya University Graduate School of Medicine, Cancer immune therapy research center)
Tetsuro Sasada (Kanagawa Cancer Center Research Institute)

- O3-1. STEAP1-targeting CAR-T cells combined with extracellular matrix-targeted interleukin-12 for advanced prostate cancer**
Koichi Sasaki (Department of Bioengineering, Imperial College London)
- O3-2. The prediction for therapeutic effects of oncolytic adenovirus via serum extracellular vesicles**
Shunya Hanzawa (Department of Gastroenterological Surgery, Okayama University Graduate School of Medicine)

O3-3. DEVELOPMENT OF A NOVEL ONCOLYTIC ADENOVIRUS CONTROLLED BY CDX2 PROMOTER FOR ESOPHAGEAL ADENOCARCINOMANaohiko Nakamura (*University of Minnesota*)**O3-4. Suicide gene therapy against brain tumor using stem cells from human exfoliated deciduous teeth (SHED) expressing HSV1-TK**Kazuhiko Kurozumi (*Department of Neurosurgery, Hamamatsu University School of Medicine*)**O3-5. Fusogenic oncolytic vaccinia virus dramatically changes the tumor immune microenvironment through inducing cell-cell fusion and delivering multiple immune modulators.**Motomu Nakatake (*Division of Genomic Medicine, Graduate School of Medical Sciences, Tottori University*)**Oral Session 4 (J)**

Gene editing, other gene transfer technologies, and stem cells

Date: Jul. 16, 16:35-17:15, Room 3 (304)

Chairs: Masashi Urabe (*Division of Genetic Therapeutics, Jichi Medical University*)Noriko Miyake (*Department of Biochemistry and Molecular Biology, Nippon Medical School*)**O4-1. PCR products with partial ITR sequence could be packaged into AAV empty capsids for long-term expression in vivo**Hiromi Kinoh (*Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, IMSUT, The University of Tokyo*)**O4-2. Development of adenovirus vector introducing whole expression unit of normal gene in safe harbor region aiming at therapy of inherited metabolic disorder**Tomoko Nakanishi (*Juntendo University*)**O4-3. Evaluation of ex vivo gene editing efficiency in human primary T cells by mRNA-LNP**Sayako Umetani (*BioScience & Engineering Laboratory, Fujifilm Corporation*)**O4-4. Evaluation of gene expressions and immune responses induced by DNA/mRNA vaccination using PJI (Pyro-drive Jet Injector) in mouse model**Tomoyuki Nishikawa (*Department of Device Application for Molecular Therapeutics, Graduate School of Medicine, Osaka University*)**Oral Session 5 (E or J)**

Regulatory Science, Immune/Inflammation regulation

Date: Jul. 16, 17:15-17:55, Room 3 (304)

Chairs: Toshihiko Okazaki (*Osaka University Hospital, Medical Center for Translational and Clinical Research*)Toshinao Kawai (*Division of Immunology, National Center for Child Health and Development*)**O5-1. Apoptotic peptide targeting pro-inflammatory microglia ameliorates inflammation in neonatal hypoxic ischemic encephalopathy**Rika Zen (*Department of Obstetrics and Gynecology, Shiga University of Medical Science*)**O5-2. The role of bone marrow-derived inducible microglia-like cells in regulating proinflammatory/anti-inflammatory microglia balance in chronic ischemic stroke**Bach Ngoc Nguyen (*Department of Neurology, Shiga University of Medical Science*)**O5-3. Development of novel cell-free analysis software to predict off-target mutation sites by genome editing**Takuma Yamashita (*Division of Molecular Target and Gene Therapy Products, National Institute of Health Sciences*)**O5-4. Study for Stable Genome Titer Measurement of AAV Vectors Using Digital PCR**Takenori Yamamoto (*Division of Molecular Target and Gene Therapy Products, National Institute of Health Sciences*)**Oral Session 6 (E or J)**

Viral vectors 2

Date: Jul. 17, 08:30-09:30, Room 3 (304)

Chairs: Ken-ichiro Kosai (*Kagoshima University Graduate School of Medical and Dental Sciences*)Hiroaki Mizukami (*Div. Genetic Therapeutics Center for Molecular Medicine Jichi Medical University*)**O6-1. A Novel AAV2 Mutant SonuAAV Expressing mGjb2 can Improve Hearing in Adult Gjb2-deficient Mice by Cochlear Perilymph Administration**Yoshinori Tanaka (*Takara Bio Inc.*)

- O6-2. The development of next-generation molecule-specific AAV vector for Gene Therapy**
Kenji Oba (*Division of Genetic Therapeutics, Center for Molecular Medicine, Jichi Medical University*)
- O6-3. Unraveling the Process of AAV Capsid Assembly through Molecular Dynamics Simulations**
Anusha Sairavi (*Oregon Health and Science University*)
- O6-4. Purification of the functional recombinant adeno-associated virus with zonal ultracentrifugation for manufacturing of gene therapy**
Mikako Wada (*Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, The Institute of Medical Science, The University of Tokyo*)
- O6-5. Development of one-step establishment method for adeno-associated virus (AAV) producer cell and high production technology**
Yusuke Mori (*Fujifilm Corporation Fujifilm Corporation*)
- O6-6. Immunological mechanism of intranasal adenovirus vaccine-induced effects via IL-1 signaling**
Rika Onishi (*Grad. Sch. of Pharm. Sci., Osaka Univ.*)

Oral Session 7 (E or J)

Cancer 2

Date: Jul. 17, 09:35-10:15, Room 3 (304)

Chairs: Shuji Kubo (Laboratory of Molecular and Genetic Therapeutics, Institute for Advanced Medical Sciences, Hyogo Medical University)

Hiroaki Ikeda (Department of Oncology, Nagasaki University Graduate School of Biomedical Sciences)

- O7-1. Stem cell therapy has efficacy for leptomeningeal metastasis of solid cancers**
Yohei Kitamura (*Department of Neurosurgery, Keio University School of Medicine*)
- O7-2. The identification of the role of tumor microenvironment in oncolytic virotherapy**
Yoshihiro Otani (*Department of Neurological Surgery, Okayama University Graduate School of Medicine*)
- O7-3. The Utility of Human Mesenchymal Stem Cells as Carriers of Retroviral Replicating Vectors for Intraperitoneal Virotherapy in Peritoneally Disseminated Tumor Models**
Shuji Kubo (*Laboratory of Molecular and Genetic Therapeutics, Institute for Advanced Medical Sciences, Hyogo Medical University*)
- O7-4. Regulation of cytokine transgene expression by an optimal promoter is crucial to safe and effective oncolytic virus immunotherapy**
Yuya Nishikawaji (*Department of Gene Therapy and Regenerative Medicine, Kagoshima University Graduate School of Medical and Dental Sciences*)

Oral Session 8 (E or J)

Neurologic, ophthalmic, Cardiovascular and musculo-skeletal diseases

Date: Jul. 17, 10:20-11:30, Room 3 (304)

Chairs: Seiichi Nagano (Department of Neurotherapeutics, Osaka University Graduate School of Medicine)

Koich Miyake (Department of Gene Therapy, Nippon Medical School)

- O8-1. Suppression of liver-specific expression of phospholipase A2G6 ameliorates abnormal glucose and lipid metabolism**
Kahori Shimizu (*Graduate School and School of Pharmaceutical Sciences, Osaka University*)
- O8-2. In vivo gene therapy for striated muscle laminopathy**
Mariko Okubo (*Sorbonne Université, Inserm, Institut de Myologie, Centre de Recherche en Myologie, Paris, France*)
- O8-3. Development of safer inner ear gene therapy based on photoswitch technology**
Masao Noda (*Department of Otolaryngology and Head and Neck Surgery, Jichi Medical University, Tochigi, Japan*)
- O8-4. Therapeutic effects of amnion derived-mesenchymal stromal cells on cardiomyopathy in animal models with Duchenne muscular dystrophy**
Yuko Kasahara (*Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, IMSUT, The University of Tokyo*)
- O8-5. Development of dosing protocol to reduce the required dose of rAAV using adult stem cells**
Hiromi Kinou (*NHO Iwaki National Hospital*)

- O8-6. RNAi-mediated Downregulation of USP48 Alleviates Amyloidopathy and Cognitive Impairments in an APP/PS1 Transgenic Mouse Model of Alzheimer's Disease**
Yung-Feng Liao (*Academia Sinica*)
- O8-7. Thorough correction of CNS and PNS pathology in Globoid cell leukodystrophy by AAV-mediated gene therapy**
Dar-Shong Lin (*MacKay Memorial Hospital*)

Oral Session 9 (J)

Basic Science 1

Date: Jul. 17, 16:20-17:50, Room 3 (304)

Chairs: Tsukasa Ohmori (Department Biochemistry, Jichi Medical University)
Yutaka Hanazono (Jichi Medical University)
Yuko Kasahara (The Institute of Medical Science, The University of Tokyo)

- O9-1. Chemical regulation of transgene expression by synthetic riboswitches**
Yohei Yokobayashi (*Okinawa Institute of Science and Technology*)
- O9-2. Development of a novel drug for AAV vector infection enhancement**
Junpei Hayakawa (*Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, IMSUT, The University of Tokyo*)
- O9-3. Artificial U-to-C RNA editing using DYW domain homologous gene from hornworts.**
Toshifumi Tsukahara (*Japan Advanced Institute of Science and Technology*)
- O9-4. Development of highly efficient HDR technology by using artificially damaged donor DNA**
Shogo Yamamoto (*Osaka University*)
- O9-5. The challenge for the radical treatment of interstitial cystitis using transplantation of human deciduous dental pulp stem cells.**
Shunichi Kajioka (*International University of Health and Welfare*)
- O9-6. Studying for abnormal phenotype and drug development in ganglioside-accumulated disease**
Takumi Era (*Dept of Cell modulation, Institute of Molecular Embryology and Genetics, Kumamoto University*)
- O9-7. Development of CAR-T manufacturing method in the short-term production method (Spo-T) using an automated cultivation device.**
jun Kohara (*Takara Bio Inc.*)
- O9-8. Novel CAR-T manufacturing process: Integration of cell processing isolator and automated cell processing in a closed system**
Michiko Takahashi (*Takara Bio Inc.*)

Oral Session 10 (E or J)

Cell therapy, Regenerative medicine

Date: Jul. 18, 13:10-13:50, Room 2 (303)

Chairs: Junichi Mineno (Takara Bio Inc.)
Ryosuke Uchibori (Division of gene and cell therapy for intractable diseases, Jichi Medical University)

- O10-1. NK-like Cell GAIA-102 Induces Tumor-Specific Immune Response Against Solid Tumors via Host IFN- γ Dependency**
Situo Zheng (*R&D Laboratory for Innovative Biotherapeutics Kyushu university*)
- O10-2. Elucidation of the contribution of monocytes and TNF- α in the enhancement of NK-like cell (GAIA-102) proliferation**
Zhuyue Yuan (*R&D Laboratory for Innovative Biotherapeutics Kyushu university*)
- O10-3. Neuroprotective Effects of Genome-Edited Human iPS Cell-Derived Neural Stem/Progenitor Cells on Traumatic Brain Injury**
Ryota Tamura (*Department of Neurosurgery, Keio University School of Medicine*)
- O10-4. Morphometric change in alveolar bone tissues after bmp gene transfer to periodontal tissues**
Mariko Yamamoto (*Kansai Women's Collage*)

Oral Session 11 (E or J)

Basic Science 2

Date: Jul. 18, 13:50-15:00, Room 2 (303)

Chairs: Hiromi Hayashita-Kinoh (Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, IMSUT, The University of Tokyo)
Yuji Tsunekawa (Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, IMSUT, The University of Tokyo)

O11-1. Genetic Engineering Optimization of Suicide Gene CD for Cell Therapy Using In silico Prediction Calculations
 Masahiro Yo (*Keio University School of Medicine*)

O11-2. CRISPR/Cas9 library screening for adeno-associated virus vector production enhancers
 Yoshitaka Miyagawa (*Department of Biochemistry and Molecular Biology, Nippon Medical School*)

O11-3. Relationship between viral protein stoichiometry and transduction efficiency of adeno-associated virus vector
 Takahiro Maruno (*U-Medico Inc.*)

O11-4. Chloroform purification of adeno-associated virus vector
 Yoshihide Sehara (*Division of Genetic Therapeutics, Center for Molecular Medicine, Jichi Medical University*)

O11-5. Development standard particle candidate for quantifying subvisible particles in adeno-associate viral vector
 Takaaki Kurinomaru (*U-Medico Inc.*)

O11-6. Development of oncolytic virus therapy using Coxsackievirus B3 for gastric cancer
 Yuki Sakashita (*The Jikei University School of Medicine*)

O11-7. Process development and scale-up of rAAV manufacturing in suspension-based system with single-use technologies
 Frank Agbogbo (*Forge Biologics*)

Oral Session 12 (E or J)

Viral vectors 3

Date: Jul. 18, 09:00-10:00, Room 3 (304)

Chairs: Toshiro Shirakawa (Kobe University Graduate School of Science, Technology and Innovation)
Takashi Higuchi (The Jikei university school of Medicine)

O12-1. Development of anti-HER2-JAK/STAT-CAR-T therapy using short period operation for T-cell production (Spo-T) method
 Yasunori Amaishi (*Technology Development Center, Takara Bio Inc.*)

O12-2. The expanded potential of optically controllable mononegavirus vectors
 Wang Yuying (*The Department of Microbiology, Graduate School of Medicine and Faculty of Medicine, The University of Tokyo*)

O12-3. Development of New Virus Vector Vaccine using Vexosomes
 Yasunari Matsuzaka (*Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, IMSUT, The University of Tokyo*)

O12-4. Novel human amnion-derived cell lines, HAT, for high-yield AAV production
 Yu-Hsin Chang (*Chitose Laboratory Corp.*)

O12-5. Selective anti-tumorigenic activity of AAVs carrying miR inversely-dependent genome-editing system
 Toru Kondo (*Hokkaido University*)

O12-6. Development of a method for producing AAV vectors with fewer empty particles
 Mitsuko Fukuhara (*U-Medico Inc.*)

Oral Session 13 (E or J)

Viral vectors 4

Date: Jul. 18, 10:10-11:10, Room 3 (304)

Chairs: Sachiko Okamoto (Technology Development Center, Takara Bio Inc.)
Yoshihide Sehara (Division of Genetic Therapeutics, Center for Molecular Medicine, Jichi Medical University)

O13-1. Generation of an expression regulation system based on CRISPR activation for non-cytotoxic herpes simplex virus vector
 Yasunobu Maruoka (*Department of Biochemistry and Molecular Biology, Nippon Medical School*)

O13-2. Adeno-associated virus (AAV) vector modification for increased AAV yield and safetyCliff Aquino (*Forge Biologics*)**O13-3. Development of a simple method for producing herpes simplex virus (HSV) amplicon vectors**Fumio Maeda (*National Institute of Advanced Industrial Science and Technology*)**O13-4. Effects of adding herpesvirus-derived helper genes on AAV vector production**Yuya Nishimura (*Synplogen Co., Ltd.*)**O13-5. Histone deacetylase inhibitor improves anticancer potential of fusogenic oncolytic vaccinia virus by enhancing the cell-cell fusion.**Motomu Nakatake (*Division of genomic Medicine, Tottori University Faculty of Medicine*)**O13-6. Standard verification of novel viral vector vaccines for effective immune induction**Ken Sugo (*Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, IMSUT, The University of Tokyo*)**Oral Session 14 (E or J)**

Viral vectors 5

Date: Jul. 18, 13:20-14:10, Room 3 (304)

Chairs: Yasunari Matsuzaka (*Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, The Institute of Medical Science, The University of Tokyo*)Saki Matsushima (*Division of Gene Therapy, Research Center for Medical Sciences, The Jikei University School of Medicine*)**O14-1. Optimization of manufacturing conditions for lentiviral vectors in accordance with GMP guidelines**Yukage Kobari (*Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, IMSUT, The University of Tokyo*)**O14-2. Development of microglia-targeted AAV vectors that maintain high specificity and efficiency over long periods of time**Ryo Aoki (*Department of Neurophysiology & Neural Repair, Gunma University Graduate School of Medicine*)**O14-3. Development of a replication-incompetent rotavirus vector**Tomohiro Kotaki (*Research Institute for Microbial Diseases, Osaka University*)**O14-4. Development of CAR-T cells that co-express Dominant negative TGF β R II and chimeric cytokine receptors.**Izumi Maki (*Technology Development Center, Takara Bio Inc.*)**O14-5. Amplifying AAV vector transduction efficacy with transgenic cell-driven compound discovery**Guillermo Posadas-Herrera (*The Institute of Medical Science, Tokyo University, Center for Gene and Cell Therapy*)**Oral Session 15 (J)**

Cancer 3

Date: Jul. 18, 14:10-15:00, Room 3 (304)

Chairs: Hiroshi Tazawa (*Center for Innovative Clinical Medicine, Okayama University Hospital*)Tomoyuki Nishikawa (*Osaka University, Graduate School of Medicine*)**O15-1. Development of novel DC vaccine loaded with tumor cell lysate by using the membrane transporter polymer.**Yuri Fujioka (*Kobe University Graduate School of Science, Technology and Innovation, Division of Advanced Medical Science*)**O15-2. The efficacy and safety of minimally invasive administration of triple mutated HSV-1 for oral and esophageal duplication cancer**Seitaro Hio (*Department of Oral and Maxillofacial Surgery, Graduate School of Dentistry, Osaka University*)**O15-3. Development of piggyBac transposon mediated ligand-based EGFR CAR-T cells for the treatment of gynecological malignancies**Manaka Shinagawa (*Department of Obstetrics & Gynecology, Shinshu University School of Medicine*)**O15-4. p53-armed oncolytic virus sensitizes murine colorectal cancer cells to p53-transduced dendritic cell vaccine by inducing MHC-bound p53 epitope expression**Naohiro Okada (*Department of Gastroenterological Surgery, Okayama University Graduate School of Medicine*)**O15-5. Analysis of in vivo dynamics of retroviral vector-producing human amniotic mesenchymal stem cells administered to tumor-bearing mice model**Yoshiyuki Yamazaki (*Department of Biochemistry and Molecular Biology, Nippon Medical School*)

Corporate Seminar

Luncheon Seminar 1

(JASC Inc.)

Date: Jul. 16, 12:00-13:00, Room 1 (501+502)

Chair: *Yoshikazu Yonemitsu (R&D Laboratory for Innovative Biotherapeutics Science, Graduate School of Pharmaceutical Sciences, Kyushu University)*

LS1. Therapeutic Effects of Adipose-Derived Mesenchymal Stem Cells for the Treatment of Patients with Knee Osteoarthritis

Jangik Ike Lee (*College of Pharmacy and Research Institute of Pharmaceutical Sciences, Seoul National University, Seoul, Republic of Korea*)

Luncheon Seminar 2

(Eisai Co., Ltd.)

Date: Jul. 16, 12:00-13:00, Room 2 (303)

Chair: *Kazuhiko Kurozumi (Department of Neurosurgery, Hamamatsu University School of Medicine)*

LS2-1. Translational Research for Brain Tumor Treatment- Drug Repositioning and Gene Therapy-

Ryo Tamura (*Department of Neurosurgery, Keio University School of Medicine*)

LS2-2. Precision Neurosurgery using gamma oscillation regularity analysis

Yosuke Sato (*Brain Function Analysis & Digital Medicine Research Institute, Showa University*)

Luncheon Seminar 3

(Miltenyi Biotec K.K.)

Date: Jul. 16, 12:00-13:00, Room 3 (304)

Chair: *Toru Uchiyama (Division of Molecular Pathogenesis, Department of Human Genetics, National Center for Child Health and Development)*

LS3. Basic research for practical application of hematopoietic stem cell ex vivo gene therapy for lysosomal storage disease

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

Saki Matsushima (*Division of Gene Therapy, Research Center for Medical Sciences, The Jikei University School of Medicine*)

Luncheon Seminar 4

(MaxCyte, Kiko Tech Co., Ltd.)

Date: Jul. 17, 11:40-12:40, Room 1 (501+502)

Chair: *Toru Uchiyama (Division of Molecular Pathogenesis, Department of Human Genetics, National Center for Child Health and Development)*

LS4. Development of a non-viral gene delivery platform for CAR T manufacturing

Steven A. Feldman (*Site Head/Scientific Director, Laboratory for Cell and Gene Medicine, Stanford University School of Medicine*)

Luncheon Seminar 5

(Takara Bio Inc.)

Date: Jul. 17, 11:40-12:40, Room 2 (303)

Chair: *Tatsuji Enoki (Takara Bio Inc.)*

LS5. Development of AAV vectors targeting inner ear and gene therapy for hearing loss

Kazusaku Kamiya (*Department of Otorhinolaryngology, Juntendo University Faculty of Medicine*)

Luncheon Seminar 6

(Cytiva, Global Life Sciences Technologies Japan K.K.)

Date: Jul. 17, 11:40-12:40, Room 3 (304)

Chair: Naohito Hariganeya (Sales Specialist, Genomic Medicine Viral Vector Workflows, Cytiva)

LS6. Re-think AAV gene therapies: Bridging research to clinical and commercial manufacturing with next-generation technologies

Peiqing Zhang (Strategic Technology Partnership Leader APAC, Cytiva)

Luncheon Seminar 7

(AnGes, Inc.)

Date: Jul. 18, 11:20-12:20, Room 1 (501+502)

Chair: Ryuichi Morishita (Department of Clinical Gene Therapy, Graduate School of Medicine, Osaka University)

LS7. Lonafarnib, drug for Hereditary Progeria (Hutchinson-Gilford Progeria syndrome)

Hironori Nakagami (Department of Health Development and Medicine, Graduate school of Medicine, Osaka University)

Luncheon Seminar 8

(Tosoh Corporation)

Date: Jul. 18, 11:20-12:20, Room 2 (303)

Chair: Ohmori Toshitaka (Tosoh corporation)

LS8. Overview of CGT in China-marketing, technology and regulatory

Jia Guodong (OBiO Technology)

Luncheon Seminar 9

(Ricoh Company, Ltd.)

Date: Jul. 18, 11:20-12:20, Room 3 (304)

Chair: Yasuji Kitabatake (Department of Pediatrics, Osaka University Graduate School of Medicine)

LS9-1. Pioneering mRNA therapy through CRISPR-Cas3 genome editing technology

Tomoji Mashimo (Division of Animal Genetics, Laboratory Animal Research Center, The Institute of Medical Science, The University of Tokyo)

LS9-2. Overview of our service and activities to support drug discovery using high-quality mRNA

Motoki Azuma (Elixirgen Scientific Japan, Inc.)

Afternoon Seminar 1

Breakthroughs in AAV Gene Therapy

(VectorBuilder Japan Inc.)

Date: Jul. 16, 15:50-16:30, Room 2 (303)

Chair: Miho Matakatsu (VectorBuilder Japan Inc.)

AS1-1. Development of capsid-modified adeno-associated virus (AAV) targeting hereditary hearing loss

Kazusaku Kamiya (Department of Otorhinolaryngology, Juntendo University Faculty of Medicine)

AS1-2. miniVec™ plasmid: VectorBuilder's miniaturized backbone, providing remarkable efficacy, safety, and manufacturability for cell and gene therapies

Miho Matakatsu (VectorBuilder Japan Inc.)

Afternoon Seminar 2

(Revvity, Inc.)

Date: Jul. 16, 15:50-16:30, Room 3 (304)

Chair: Ryuichi Uozumi (Revvity, Inc.)

AS2. Next generation evolved AAV vectors for retinal gene transfer

Yvonne Berghöfer-Hochheimer (Head of Alliance and Program Management, Revvity Gene Delivery GmbH/Munich, Germany)

Afternoon Seminar 3

(Bio-Rad Laboratories K.K.)

Date: Jul. 17, 15:30-16:10, Room 2 (303)

Chair: Mieko Shiwa (Bio-Rad Laboratories K.K.)

AS3-1. Effectiveness of Droplet Digital PCR in Nucleic Acid Quantitation

Yukinori Yatsuda (*Bio-Rad Laboratories K.K.*)

AS3-2. Development of Analytical Testing Method Using Droplet Digital PCR for Gene Therapy Drug Product

Shunsuke Saito (*Synplogen Co., Ltd.*)

Afternoon Seminar 4

(Genesis Healthcare Co.)

Date: Jul. 17, 15:30-16:10, Room 3 (304)

AS4. Innovative genomics platform using generative AI

Michel Mommejat (*Genesis Healthcare Co.*)