# Program

# **Presidential Lecture (J)**

Date: Thu. July 24, 10:35-10:55, Room 1

Chair: Keiya Ozawa (Emeritus Professor, Jichi Medical University)

PL. Great Expectations: Advancing Global Gene and Cell Therapy from Japan

Takashi Okada (Center for Gene and Cell Therapy, The Institute of Medical Science, The University of Tokyo)

# **Invited Lecture 1 (J)**

Date: Wed. July 23, 16:20-16:50, Room 1

Chairs: Masahiro Toda (Dept. of Neurosurgery, Keio University) Hideki Mochizuki (Osaka Toneyama Medical Center)

IL1. Future Prospects of Regenerative Medicine, Cell Therapy, and Gene Therapy in Japan

Toshiharu Furukawa (Member of The House of Councillors. Law School and Medical School, Keio University / TMI Associates)

# **Invited Lecture 2 (J)**

Date: Fri. July 25, 11:15-11:45, Room 1

Chair: Akihiro Kume (Support Center for Clinical Investigation, Jichi Medical University)

IL2. Evaluation of value of gene and cellular therapy products, and the pharmaceutical regulation Daisaku Sato (Ministry of Health, Labour and Welfare)

# **Special Lecture 1 (E)**

Date: Wed. July 23, 12:15-12:55, Room 1

Chairs: Masafumi Onodera (Graduate School of Engineering, Osaka University)
Teruhide Yamaguchi (Nihon Pharmaceutical University/ National Institute of Health Sciences)

SL1. Beyond "One Disease at a Time": Platform Approaches for Rare Disease Gene Therapy/Gene Editing Clinical Trials

**Philip J. Brooks** (Division of Rare Diseases Research Innovation, National Center for Advancing Translational Sciences (NCATS), National Institutes of Health)

# Special Lecture 2 (E)

Date: Thu. July 24, 9:35-10:25, Room 1

Chairs: Hiroyuki Nakai (Dept. of Molecular and Medical Genetics, Oregon Health & Science University)
Toshihiko Okazaki (Cell Processing Center, OSAKA University, School of Medicine)

SL2. Redefining Viral Gene Therapy: Autonomous Parvovirus Capsids for Enhanced Delivery, Tropism, and Safety Sebastian Aguirre Kozlouski (*Platform Development, Carbon Biosciences*)

### Special Lecture 3 (E)

Date: Thu. July 24, 14:25-15:15, Room 1

Chair: Shin-ichi Muramatsu (Division of Neurological Gene Therapy, Center for Open Innovation, Jichi Medical University)

SL3. Immune reaction of AAV

J. Fraser Wright (Kriya Therapeutics®)

# **Special Lecture 4 (E)**

Date: Thu. July 24, 16:25-17:05, Room 1

Chairs: Yoshikatsu Eto (Advanced Clinical Research Center & Asian LSD Center, Institute of Neurological disorders)
Toya Ohashi (Development of Gene Therapy for Lysosomal Storage Diseases, The Jikei University School of
Medicine)

### SL4. AAV Gene Therapy for Rare Genetic Diseases

Guangping Gao (Li Weibo Institute for Rare Diseases Research, UMass Chan Medical School)

# Special Lecture 5 (E)

Date: Fri. July 25, 9:35-10:25, Room 1

Chairs: Jun-ichi Mineno (CDM Center, Takara Bio Inc.)

Masato Yamamoto (Basic and Translational Research, University of Minnesota)

SL5. in vivo CAR-T

Haig Aghajanian (Capstan Therapeutics)

# Special Lecture 6 (E)

Date: Thu. July 24, 12:45-13:25, Room 1

Chairs: En Kimura (Cell and Gene therapy Development, Astellas Pharma Inc.)

Takashi Okada (Division of Molecular and Medical Genetics, Contact for Gen

Takashi Okada (Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, The Institute of Medical Science, The University of Tokyo)

### SL6. Delivery and expression of mini- and micro-dystrophins for gene therapy of DMD

**Jeffrey S. Chamberlain** (Depts of Neurology, Biochemistry and Medicine, Wellstone Muscular Dystrophy Specialized Research Center, University of Washington)

# **Educational Lecture 1 (J)**

Date: Wed. July 23, 13:35-14:05, Room 1

Chairs: Yasufumi Kaneda (Center for infectious Disease Education and Research, The University of Osaka) Koji Nishida (Dept. of Ophthalmology, The University of Osaka)

#### EL1. Gene and cell therapy in the central nervous system

Hideyuki Okano (Keio University Regenerative Medicine Research Center)

# **Educational Lecture 2 (J)**

Date: Wed. July 23, 13:35-14:05, Room 2

Chairs: Yutaka Hanazono (Division of Regenerative Medicine, Center for Molecular Medicine, Jichi Medical University)
Ryuichi Morishita (Dept. of Clinical Gene Therapy, Osaka University Medical School)

### EL2. Advances in Neurodegenerative Disease Research Using iPS Cells and Gene Editing

Haruhisa Inoue (Kyoto University Center for iPS Cell Research and Application (CiRA) / RIKEN)

### **Educational Lecture 3 (J)**

Date: Fri. July 25, 10:35-11:05, Room 1

Chair: Kenzaburo Tani (Emeritus Professor, Kyushu University/ The Institute of Medical Science, The University of Tokyo)

### EL3. Oncolytic virus therapy using herpes simplex virus type 1

Tomoki Todo (The University of Tokyo Division of Innovative Cancer Therapy, The Institute of Medical Science)

### **Educational Lecture 4 (J)**

Date: Wed. July 23, 13:00-13:30, Room 1

Chairs: Shin-ichi Takeda (Dept. of Neuromuscular Research, National Institute of Neuroscience, National Center of Neurology and Psychiatry)

Torayuki Okuyama (Dept. of Clinical Genomics, Saitama Medical University)

#### EL4. Gene therapy for Fukuyama muscular dystrophy

Tatsushi Toda (NCNP / Dept. of Neurology, Graduate School of Medicine, University of Tokyo)

### Special Program 1 (J)

Date: Thu. July 24, 12:45-13:25, Room 2

Chair: Rika Tanaka (Dept. of Plastic Surgery, Juntendo University Hospital)

# SP1. Invent and Innovate-Towards Regenerative Medicine

Takanori Takebe (Institute of Science Tokyo Institute of Integrated Research / The University of Osaka Graduate School of Medicine / Cincinnati Children's Hospital Medical Center Division of Gastroenterology, Hepatology and Nutrition and Division of Developmental Biology / Cincinnati Children's Hospital Medical Center Center for Stem Cell and Organoid Medicine (CuSTOM) / Yokohama City University Communication Design Center)

Panelist: Karin Kojima (Dept. of Pediatrics, Jichi Medical University)

Satoshi Yamazaki (Institute of Medical Science, The Institute of Medical Science, The University of Tokyo)

### Special Program 2 (J)

Genesis Healthcare Co.

Date: Fri. July 25, 16:10-16:50, Room 2

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

#### SP2. Effects of Wine Tasting on Cognitive Function

Shinya Tasaki (Honorary president of ASI/ Honorary president of JSA)

# ASGCT/ESGCT/JSGCT Joint Symposium (E)

Date: Wed. July 23, 16:55-17:55, Room 1

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

Noriyuki Kasahara (Dept. of Neurological Surgery and Radiation Oncology, University of California, San Francisco)

- A. Genome Editing by Homology Directed Repair: An approach to go from Rare to Common
  Matthew Porteus (Definitive and Curative Medicine and Dept. of Pediatrics, Institute of Stem Cell Biology and Regenerative
  Medicine and Maternal-Child Health Research Institute at Stanford)
- E. Gene Therapy in Fanconi Anemia: Past, Present and Future

Juan A. Bueren (Division of Hematopoietic Innovative Therapies. Centro de Investigaciones Energéticas Medioambientales y
Tecnológicas and Centro de Investigación Biomédica en Red de Enfermedades Raras (CIEMAT / CIBERER),
Madrid 28040, Spain. / Advanced Therapies Unit, Instituto de Investigación Sanitaria Fundación Jiménez Díaz
(IIS-FJD, UAM), Madrid 28040, Spain.)

J. Multidisciplinary oncolytic virotherapy for esophageal cancer patients unfit for standard treatments

Toshiyoshi Fujiwara (Dept. of Gastrenterlogical Surgery, Okayama University Graduate School of Medicine, Dentistry, and

Pharmaceutical Sciences / Center for Innovative Clinical Medicine, Okayama University Hospital / Oncolys

BioPharma, Inc.)

### Takara Bio Research Award 2025 (J)

Date: Thu. July 24, 14:00-14:20, Room 1

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

Ryuichi Morishita (Dept. of Clinical Gene Therapy, Osaka University Medical School)
Kahori Shimizu (Laboratory of Biochemistry and Molecular Biology, Graduate School of Pharmaceutical Sciences, Osaka University)

# The Japanese Society for Virology Joint Program (J)

Exploration of Next-Generation Viral Vectors for Innovative Technologies

Date: Wed. July 23, 12:15-13:30, Room 2

Chairs: Kohnosuke Mitani (Research Center for Genome Medicine, Saitama Medical University) Keizo Tomonaga (Laboratory of RNA viruses, Institute for Life and Medical Sciences)

# JSVi-1. Biological characteristics of Chikungunya virus and its potential use as a viral vector

**Youichi Suzuki** (Osaka Medical and Pharmaceutical University Faculty of Medicine, Department of Microbiology and Infection Control)

- JSVi-2. Flaviviruses as Emerging Viral Vectors: Applications and Future Perspectives
  - Tomokazu Tamura (Kyushu University Faculty of Medical Sciences / Hokkaido University Faculty of Medicine / Hokkaido
    University Institute for Genetic Medicine / Hokkaido University Institute for Vaccine Research and
    Development (IVReD))
- JSVi-3. Application of recombinant rotaviruses for induction of mucosal immune responses to enteric pathogens

  Takahiro Kawagishi (The University of Osaka Department of Virology, Research Institute for Microbial Diseases / The University

  of Osaka Center for Advanced Modalities and DDS)
- JSVi-4. Oncolytic Activity of Blue Light-Controlled Measles Virus for Tongue Cancer and Melanoma Cell Lines
  Wang Yuying (The University of Tokyo Department of Microbiology, Graduate School of Medicine and Faculty of Medicine)

# The Japanese Society for Regenerative Medicine Joint Program (J)

JSGCT-JSRM Joint Symposium

Date: Wed. July 23, 14:10-15:10, Room 1

- Chairs: Masahiro Toda (Dept. of Neurosurgery, Keio University School of Medicine) Katsuto Tamai (The University of Osaka Graduate School of Medicine)
- RM-1. Development of iPSC-derived next generation T-cell therapy targeting refractory cancers Miki Ando (Juntendo University Faculty of Medicine Deaprtment of Hematology)
- RM-2. Improving CAR-T cell therapy by modulating cytokine signaling Yuki Kagoya (Keio University School of Medicine)
- RM-3. Clinical development of NK-like cells, GAIA-102, that can eliminate solid tumors Yoshikazu Yonemitsu (Kyushu University Graduate School of Pharmaceutical Sciences)

### The Japanese Society for Vaccinology Joint Program (J)

Frontline of accelerating next-generation vaccine development

Date: Wed. July 23, 14:10-15:10, Room 2

- Chairs: Takaji Wakita (Japan Institute for Health Security)
  Takafumi Nakamura (Division of Genomic Medicine, Faculty of Medicine, Tottori University)
- JSVa-1. Structural basis for the evolution of SARS-CoV-2 and vaccine-antigen design Takao Hashiguchi (Kyoto University Institute for Life and Medical Sciences)
- JSVa-2. Construction and evaluation of novel AAV vector vaccines aimed at effective immune induction

  Ken Sugo (The Institute of Medical Science, The University of Tokyo Division of Molecular and Medical Genetics, Center for Gene

  and Cell Therapy)
- JSVa-3. Next generation vaccine research and development using novel immune surrogate markers

  Yoshimasa Takahashi (Japan Institute for Health Security National Institute of Infectious Diseases Research Center for Vaccine

  Development)
- JSVa-4. Development of peptide vaccine using virus-derived epitope

  Hironori Nakagami (Osaka University Department of Health Development and Medicine, Osaka University Graduate School of Medicine)
- JSVa-5. Nucleic acid vaccines and adjuvants: science and design linked by novel modalities Ken Ishii (The University of Tokyo The Institute of Medical Science)

# The Japanese Society of Child Neurology Joint Program (J)

Advancing Gene Therapy for Pediatric Diseases: From Discovery to Clinical Translation

Date: Thu. July 24, 9:35-10:35, Room 2

- Chairs: Hitoshi Osaka (Dept. of Pediatrics, Jichi Medical School)

  Mariko Okubo (Dept. of Human Genetics, Nationl Center for Global health and Medicine, Research Institute)
- CN-1. Therapeutic Development Targeting Fukuyama Congenital Muscular Dystrophy Mariko Ikeda (Kochi University School of medicine Department of Pediatrics)
- CN-2. Genomic analysis of rare diseases in pediatric neurology: exploring therapeutic approaches Takeshi Yoshida (Kyoto University Graduate School of Medicine Department of Pediatrics)

#### CN-3. Current status and challenges of gene therapy for childhood-onset neuromuscular disease

Keiko Ishigaki (Tokyo Women's Medical University School of Medicine Department of Pediatrics)

# Japanese Society for Genetic Counseling Joint Program (J)

The intersection between Gene & Cell Therapy and genetic medicine/genetic counseling.

Date: Fri. July 25, 9:35-10:35, Room 2

Chairs: Reiko Arakawa (Dept. of Genomic Medicine, National Center for Global Health and Medicine)

Atsushi Watanabe (Divison of Clinical Genetics/Support Center for Medical Genetics, Kanazawa University Hospital)

### GC-1. What genome researchers hope to achieve with gene-cell therapy.

**Noriko Miyake** (National Institute of Global Health and Medicine, Japan Institute for Health Security Department of Human Genetics)

#### GC-2. Gene and Cell Therapy and Genetic counseling

**Masayoshi Nakakuni** (National Center for Child Health and Development Gene & Cell Therapy Promotion Center / Center for Clinical Research and Development)

### GC-3. The Basic Plan under the Act on Promotion of Genome Medicine

**Kaori Muto** (Department of Public Policy, The Institute of Medical Science, The University of Tokyo / Laboratory of Biomedical Ethics and Co-design, RIKEN Center for Integrative Medical Sciences)

# **Nucleic Acids Therapeutics Society of Japan Joint Program (J)**

Date: Fri. July 25, 10:40-11:50, Room 2

Chairs: Takanori Yokota (NucleoTIDE and PepTIDE Drug Discovery Center, Institute of Science Tokyo) Kumiko Ui-Tei (NucleoTIDE and PepTIDE Drug Discovery Center, Institute of Science Tokyo)

### NA-1. Development of Nucleic Acid Therapeutics Based on RNA Editing

**Masatora Fukuda** (Fukuoka University Department of Chemistry, Faculty of Science / Fukuoka University Graduate School of Science)

### NA-2. Development of a Gene Expression Control Technology by RNA hacking

Yousuke Katsuda (Kumamoto University Faculty of Advanced Science and Technology / StapleBio Inc.)

### NA-3. Chemistry based mRNA design for application to gene and cell therapy

Hiroshi Abe (Department of Chemistry, Graduate School of Science, Nagoya University.)

### NA-4. Development of Nucleic Acid-Based Therapeutics for Fukuyama Congenital Muscular Dystrophy

Mariko Ikeda (Kochi University School of medicine Department of Pediatrics)

# The Japanese Society for Genome Editing Joint Program (J)

Genome Editing and the Future of Next-Generation Medicine

Date: Fri. July 25, 15:50-16:50, Room 1

Chairs: Hironori Nakagami (University of Osaka Graduate School of Medicine)
Tsukasa Ohmori (Center for Gene Therapy Research, Jichi Medical University)

### GE-1. Genome Editing and the Future of Next-Generation Medicine: Prospects and Challenges

Mashimo Tomoji (University of Tokyo Institute of Medical Science Division of Animal Genetics)

### GE-2. TIGR-Tas: Modular RNA-guided systems with Nop domain-containing proteins

Saito Makoto (RIKEN Pioneering Research Institute)

#### GE-3. In vivo delivery of CRISPR/Cas based on lipid nanoparticles

Yusuke Sato (Hokkaido University Faculty of Pharmaceutical Sciences)

### GE-4. Trends in the development of genome editing products and their quality and safety assessment

Takao Inoue (National Institute of Health Sciences Division of Molecular Target and Gene Therapy Products)

# Symposium 1 (J)

Neurology

Date: Wed. July 23, 16:55-17:55, Room 2

Chairs: Chikako Nito (Laboratory for Clinical Research, Nihon Medical School)
Seiichi Nagano (Dept. of Neurology, Osaka University, Graduate School of Medicine)

- S1-1. AAV-Mediated Gene Therapy and Genome Editing for Hereditary Hearing Loss Kazusaku Kamiya (Department of Otorhinolaryngology, Juntendo University)
- S1-2. A Compact GAD67 promoter enables inhibitory neuron-specific gene therapy for epilepsy
  Hirokazu Hirai (Gunma University Department of Neurophysiology & Neural Repair / Gunma University Viral Vector Core)
- S1-3. Development of vascular smooth muscle cells-directed adeno-associated virus vector
  Yoshihide Sehara (Jichi Medical University Division of Genetic Therapeutics, Center for Molecular Medicine / Jichi Medical
  University Internal Medicine Division (Neurology), Neuroscience Center)

# Symposium 2 (J)

Oncolytic virotherapy

Date: Thu. July 24, 14:25-15:25, Room 2

Chairs: Ken-ichiro Kosai (Dept. of Gene Therapy and Regenerative Medicine, Kagoshima University) Hiroshi Fukuhara (Dept. of Urology, Kyorin University School of Medicine)

- S2-1. Oncolytic virus therapy using G47∆ as the first line treatment for patients with prostate cancer Hiroshi Fukuhara (Kyorin University School of Medicine Department of Urology)
- S2-2. Development of next-generation oncolytic vaccinia virus

  Takafumi Nakamura (Tottori University Faculty of Medicine, Division of genomic Medicine)
- S2-3. Research and development of oncolytic virotherapy using measles virus Chieko Kai (*Teikyo University Advanced Comprehensive Research Organization*)
- S2-4. Basic, non-clinical, and clinical development of oncolytic adenovirus Surv.m-CRAs

Kenichiro Kosai (Kagoshima University Graduate School of Medical and Dental Sciences. Department of Gene Therapy and
Regenerative Medicine / Kagoshima University Graduate School of Medical and Dental Sciences. South Kyushu
Center for Innovative Medical Research and Application / Kagoshima University Graduate School of Medical
and Dental Sciences. Center for Innovative Therapy Research and Application / Kagoshima University Hospital.
Translational Research Center / Surv BioPharma Inc. (Kagoshima University Certified Start-Up Company))

### Symposium 3 (J)

Endocrine and metabolic disease

Date: Thu. July 24, 16:25-17:25, Room 2

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

Toru Uchiyama (Division of Molecular Pathogenesis, National Center for Child Health and Development)

- S3-1. AAV Vector-Mediated In Vivo Gene Therapy for Inherited Metabolic Diseases

  Kazuhiro Muramatsu (Jichi Medical University Department of Pediatrics / Jichi Medical University Center for Gene Therapy research)
- S3-2. Research and Development of gene therapy for Mucopolysaccharidosis type II

  Hiroshi Kobayashi (The Jikei University School Of Medicine Division of Gene Therapy, Research Center for Medical Sciences)
- S3-3. Current status and future perspectives of siRNA-based nucleic acid therapeutics for endocrine and metabolic diseases Efforts toward realizing personalized medicine to address unmet medical needs Kumiko Ui-Tei (NucleoTIDE and PepTIDE Drug Discovery Center, INSTITUTE OF SCIENCE TOKYO)

# Symposium 4 (J)

Vector Development (AAV & HBoV)

Date: Fri. July 25, 9:35-10:35, Room 3

Chairs: Hiroaki Mizukami (Division of Genetic Therapeutics, Center for Molecular Medicine, Jichi Medical University) Hiroyuki Mizuguchi (Graduate School of Pharmaceutical Sciences, Osaka University)

- S4-1. Adeno-associated virus-mediated cell type-specific gene expression for mapping neuronal circuit architecture Kaoru Seiriki (*The University of Osaka Graduate School of Pharmaceutical Sciences*)
- S4-2. The development of next-generation AAV vector system

  Kenji Ohba (Jichi Medical University Center for Molecular Medicine, Division of Genetic Therapeutics)
- S4-3. System and infrastructure development for gene therapy and vaccine development via parvovirus vectors

  Yuji Tsunekawa (Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, The Institute of Medical Science,

  The University of Tokyo)

# Symposium 5 (J)

Ophthalmology

Date: Fri. July 25, 10:40-11:40, Room 3

Chairs: Tsutomu Igarashi (Dept. of Ophthalmology, Nippon Medical School Chiba Hokusoh Hospital)
Yasuhiro Ikeda (Dept. of Medicine of Sensory and Motor Organs, Ophthalmology, University of Miyazaki)

- **S5-1.** Regenerative medicine for the corneal epithelium

  Yoshinori Oie (Department of Ophthalmology, Graduate School of Medicine, Osaka University)
- **S5-2.** Development of cultured human corneal endothelial cell transplantation for bullous keratopathy Morio Ueno (Kyoto Prefectural University of Medicine Department of Ophthalmology)
- S5-3. Ocular Gene Therapy: Social Implementation of Gene Therapy for Inherited Retinal Dystrophy
  Kaoru Fujinami (National Institute of Sensory Organs, NHO Tokyo Medical Center Laboratory of Visual Physiology, Division of
  Vision Research / UCL Institute of Ophthalmology, University College London Genetics / Keio University School
  of Medicine Department of Ophthalmology / Graduate School of Science, The University of Tokyo Department of
  Biological Sciences)
- S5-4. Frontiers in the Development of Gene Therapy for Vision Restoration
  Yusaku Katada (Keio University School of Medicine Department of Ophthalmology / Restore Vision Inc.)

# Symposium 6 (J/E)

Duchenne muscular dystrophy and rare diseases

Date: Fri. July 25, 13:45-14:45, Room 1

- Chairs: Takanori Yamagata (Tochigi Rehabilitation Center, Jichi Medical University)
  Yoshitsugu Aoki (Dept. of Molecular Therapy, National Institute of Neuroscience, National Center of Neurology and Psychiatry)
- **S6-1.** Molecular pathomechanism and therapeutic strategy for muscular dystrophy-dystroglycanopathy Motoi Kanagawa (Ehime University Graduate School of Medicine)
- S6-2. Research on therapeutic strategy for musculocontractural Ehlers-Danlos syndrome
  Tomoki Kosho (Shinshu University School of Medicine Department of Medical Genetics / Shinshu University Hospital Center for
  Medical Genetics / Shinshu University School of Medicine Division of Clinical Sequencing / Shinshu University
  Research Center for Supports to Advanced Science / Shinshu University Hospital BioBank Shinshu)
- S6-3. Roche's reflections on preparing for a future with gene therapy in Duchenne muscular dystrophy Alexander P. Murphy (Roche Products Ltd.)

# Symposium 7 (J)

Cancer gene therapy

Date: Fri. July 25, 13:45-14:45, Room 2

Chairs: Yozo Nakazawa (Dept. of Pediatric, Shinshu University School of Medicine)
Yoshikazu Yonemitsu (Kyushu University Graduate School of Pharmaceutical Sciences)

- S7-1. Development of CAR gene-modified allogeneic γδ-T cells for the treatment of refractory solid cancers
  Hiroshi Fujiwara (Mie University Department of Personalized Cancer Immunotherapy, Mie University Graduate School of
  Medicine)
- S7-2. Potential for New Technologies to Boost Cell Expansion and Persistence in CAR-T Therapy
  Ken Ohmine (Jichi Medical University Division of Hematology, Department of Medicine / Jichi Medical University Center for Gene
  Therapy Research)
- S7-3. Academic drug development of non-viral CAR-T cells
  Yozo Nakazawa (Shinshu University School of Medicine Department of Pediatrics)

# Symposium 8 (J)

Young Investigators Session

Date: Fri. July 25, 13:45-14:45, Room 3

Chairs: Fuminori Sakurai (Dept. of Pharmacy, Kindai University)

Takenori Yamamoto (Division of Molecular Target and Gene Therapy Products, National Institute of Health Sciences)

- S8-1. Efforts Toward the Clinical Application of CAR-T Cell Therapy for Gynecologic Malignancies Manaka Shinagawa (Shinshu University Department of Obstetrics and Gynecology)
- S8-2. Iron metabolism and therapeutic strategies for SENDA/BPAN

  Kiwako Tsukida (Jichi Medical University Center For Gene Therapy Research / Jichi Medical university Department of Pediatrics)
- S8-3. In Vivo Genome Editing Therapy for Congenital Diseases

  Tomoki Togashi (Jichi Medical University School of Medicine Department of Biochemistry)
- S8-4. Approaches to quality evaluation of mRNA therapeutics
  Takenori Yamamoto (National Institute of Health Sciences Division of Molecular Target and Gene Therapy Products)

### Symposium 9 (J)

Regulatory Science

Date: Fri. July 25, 15:50-16:50, Room 3

Chairs: Eriko Uchida (Division of Molecular Target and Gene Therapy Products, National Institute of Health Sciences)
Akihiro Kume (Support Center for Clinical Investigation, Jichi Medical University)

- S9-1. Amendments to the Act on the Safety of Regenerative Medicine and the Efforts of the Ministry of Health, Labour and Welfare
  Hideyuki Hakui (Ministry of Health, Labour and Welfare Health Policy Bureau, Research and Development Policy Division)
- S9-2. The Patent Application Trends Survey in FY2024 "mRNA medicines"

  Matsuo Eriko (Japan Patent Office Medical Science Division of Patent Examination Department (Chemistry, Life Science and Material Science))
- S9-3. New Trends in Ethics Regarding Clinical Research on Gene and Cell Therapy

  Yoichi Yamamoto (The University of Osaka Hospital Academic Clinical Research Center, Department of Medical Innovation)

# Plenary Session (E or J)

Date: Wed. July 23, 10:15-11:05, Room 1

Chairs: Makoto Otsu (Kitasato University School of allied Health Science) Yumi Kanegae (AnGes, Inc.)

PS1. Development of an AAV Capsid Engineering Method Using In Silico Directed Evolution Hiroaki Ono (International Institute for Integrative Sleep Medicine, University of Tsukuba Shi lab)

- PS2. Adeno Associated Virus (AAV) and polymer complexes enabling tumor targeting delivery and its therapeutic efficacy
  - **Nozomi Matsudaira** (Institute of Science Tokyo Department of Life Science and Technology / Institute of Science Tokyo Laboratory for Chemistry and Life Science)
- PS3. Development of adaptive cancer immunotherapy by NGS-based identification of neoantigen-specific T cell receptor genes
  - **Hidetoshi Sumimoto** (Shiga University of Medical Science Department of Medical Oncology / Shiga University of Medical Science Cancer Center / Shiga University of Medical Science Center for Advanced Medicine against Cancer)
- $PS4. \quad Development \ of \ an \ SRG \ system \ for \ selective \ control \ of \ CAR-T \ cell \ kinetics$ 
  - Ryosuke Uchibori (Jichi Medical University Center For Gene Therapy Research)

# **Oral Session**

### Oral Session 1 (E or J)

Cancer

Date: Wed. July 23, 12:15-13:35, Room 3

Chairs: Hiroshi Tazawa (Dept. of Gastrenterlogical Surgery, Okayama University.)
Tomoyuki Nishikawa (The Center of Medical Innovation and Translation Research, Graduate School of Medicine,
Osaka University)

- O1-1. Engineered hematopoietic progenitor cells for robust anti-cancer responses Richard Koya (University of Chicago)
- O1-2. Investigator-initiated phase I trial of an oligonucleotide therapeutic targeting long noncoding RNA TUG 1 for recurrent glioblastoma
  Saito Ryuta (Nagoya University Graduate School of Medicine Department of Neurosurgery)
- O1-3. Efficacy of HSV-TK/GCV system suicide gene therapy using SHED expressing modified HSV-TK against Glioblastoma Stem Cell

Zhitong Liu (Hamamatsu University School of Medicine Department of Neurological Surgery)

- O1-4. Combination therapy of an oral cancer vaccine using Bifidobacterium expressing Wilms' tumor 1 antigen and an immune checkpoint inhibitor suppressed tumor growth in a pancreatic cancer mouse model

  Taiki Yamazaki (Graduate School, Kobe University Science, Technology and Innovation)
- O1-5. Autophagy inhibition enhances the anti-tumor effect of p53-armed oncolytic virotherapy by activating MHC-I expression and apoptosis induction in pancreatic cancer

  Yosuke Takahashi (Okayama University Graduate School of Medicine, Dentistry and Pharmaceutical Sciences Department of

  Gastroenterological Surgery)
- O1-6. Targeting myeloid-rich glioma with oncolytic HSV

  Hiroshi Nakashima (Mass General Brigham Hospital and Harvard Medical School Neurosurgery)
- O1-7. Preclinical study of the antitumor effects of fusogenic oncolytic vaccinia virus in mouse models of bladder cancer Yuri Koyama (Tottori University Faculty of Medicine Department of Surgery, Division of Urology / Tottori University Faculty of Medicine Division of genomic Medicine)
- O1-8. CDX2 promoter-controlled oncolytic adenovirus suppresses tumor growth and liver metastasis of colorectal cancer

  Naohiko Nakamaura (University of Minnesota Surgery)
- O1-9. Cell-cell fusion and immunostimulatory cytokines significantly impact immunotherapeutic potential of oncolytic vaccinia virus.

  Motomu Nakatake (Faculty of Medicine Tottori University Division of Genomic Medicine)
- O1-10. Efficient cytotoxic effects of reovirus on tumor cells undergoing epithelial-mesenchymal transition
  Fuminori Sakurai (Kindai University Faculty of Pharmacy / Osaka University Graduate School of Pharmaceutical Sciences)

### Oral Session 2 (E or J)

Vector Development

Date: Wed. July 23, 13:40-15:10, Room 3

- Chairs: Yoshihide Sehara (Jichi Medical University Division of Genetic Therapeutics, Center for Molecular Medicine)
  Saki Matsushima(The Jikei University School of Medicine Division of Gene Therapy, Research Center for Medical Sciences)
  - Takashi Higuchi (Division of Gene Therapy, Research Center for Medical Sciences, The Jikei University School of Medicine)
- O2-1. Development of a High-Purity circRNA Manufacturing Process

Wenjing Yuan (Shenzhen Yuanxing Gene-tech Co.,LTD.)

O2-2. Efficient Neuronal Transduction of AAV2-derived CereAAV.YN Vector in Cynomolgus Macaque Brain without Liver Transduction by Intravenous Injection.

Hiroaki Nariki (TAKARA BIO INC. CDM Center 3)

- O2-3. Versatile cell/tissue-specific delivery of AAV9 with no capsid engineering at all Junichi Takagi (The University of Osaka Institute for Protein Research)
- O2-4. Receptor targeting of adeno-associated virus vectors using a bispecific antibody Yusuke Shiozawa (Nippon Medical School Laboratory of Molecular Analysis)
- O2-5. Anti-tumor effect of a conditionally-replicative adenovirus vector containing CD44ECD-synNotch-HIF-3 $\alpha$ 4 fusion gene in a mouse bladder cancer xenograft model.

Ruhan A (Kobe University Graduate School of Science, Technology and Innovation)

O2-6. Overcoming Challenges in Rapidly Assessing AAV-Mediated Gene Delivery to Renal Tubules by Slow Retrograde Renal Pelvis Injection of Miniprep Vectors

Anusha Sairavi (Oregon Health and Science University Molecular and Medical Genetics)

O2-7. Engineering of Bocavirus Capsids by Peptide Insertion to Develop Highly Efficient Next-generation Viral Vectors

Yumi Sano (Heidelberg University Medical Faculty Department of Infectious Diseases / Virology / Heidelberg University Faculty of Engineering Sciences Molecular Biotechnology)

O2-8. Structural analysis of recombinant adeno-associated virus using hydrogen/deuterium exchange mass spectrometry

Tomohiko Ikeda (Osaka University engineering)

O2-9. Adeno-Associated Virus Self-Assembled with Tannic Acid and Phenylboronic Acid Polymers to Evade Neutralizing Antibodies and Reduce Adverse Events

Hiroaki Kinoh (Innovation Center of Nanomedicine Kataoka Kinoh lab)

O2-10. The heating strategy of sgRNA enhances the efficiency of RNP-loaded lipid nanoparticles
Rina Shimizu (Hokkaido University Laboratory for Molecular Design of Pharmaceutics, Graduated School of Pharmaceutical

O2-11. Regulatory science research for chain-length distribution analysis of mRNA using capillary gel electrophoresis

Takenori Yamamoto (National Institute of Health Sciences Division of Molecular Target and Gene Therapy Products)

# Oral Session 3 (E or J)

Preclinical Study

Date: Wed. July 23, 16:55-17:55, Room 3

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

Akiko Ishii (Iwaki National Hospital ·)

- O3-1. Hematopoietic stem cell gene therapy for Pompe disease using lentiviral vector carrying next-generation enzyme Yohta Shimada (The Jikei University School of Medicine Division of Gene Therapy, Research Center for Medical Sciences)
- O3-2. Overcoming Key Barriers in Effective AAV-Mediated Kidney Gene Delivery in Non-Human Primates Through Renal Pelvis Injection

Ranjan Das (Oregon Health and Science University Department of Molecular and Medical Genetics)

- O3-3. Development of CRISPR/Cas9 in vivo therapeutic gene editing for Charcot-Marie-Tooth 1A (CMT1A) Jae young Lee (Ajou University School of Medicine Department of Anatomy)
- O3-4. Therapeutic genome editing for Pelizaeus-Merzbacher disease targeting intronic enhancer elements Heng Li (National Center of Neurology and Psychiatry Institute of Neurosicence)
- O3-5. Gene therapy for a mouse model of Niemann-Pick disease type C1 by systemic delivery of CereAAV mutant Toru Yasuda (National Center for Child Health and Development Department of Human Genetics)
- O3-6. In vivo HB-EGF/ HGF gene therapy to protect and regenerate residual β cells in type 1 diabetes

  Eriko Matsuda (Kagoshima University Department of Gene Therapy and Regenerative Medicine, Graduate School of Medical and

  Dental Sciences)
- O3-7. Elucidating mechanism of attenuation of vaccine efficacy via type I IFN signaling by intranasally administered adenovirus vectors

Hayato Nakatani (Osaka University Graduate school of Pharmaceutical Sciences)

# Oral Session 4 (E or J)

Cell Therapy

Date: Thu. July 24, 9:35-10:35, Room 3

Chairs: Ryosuke Uchibori (Jichi Medical University Center For Gene Therapy Research)
Yosuke Morodomi (R&D Laboratory for Innovative Biotherapeutics Science, Kyushu University)

O4-1. Semi-automated Manufacturing of PiggyBac CAR-T cells Targeting the EPH Family and Optimization of T cell Metabolic Fitness

Konomi Morita (Shinshu University Graduate School of Interdisciplinary Medical and Engineering Sciences Division of Biomedical Engineering, Department of Life Science and Medical Engineering, Pediatrics Medicine Laboratory / A-SEEDS Co., Ltd. Research and Development Department)

O4-2. Development of manufacturing methods using an automated cell processing equipment in closed systems for practical application of hematopoietic stem cell ex vivo gene therapy

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

O4-3. Enhancing Mesothelin CAR T Cell Therapy for Pancreatic Cancer with an Oncolytic Herpes Virus Boosting CAR Target Antigen Expression

Mona Alhussein Aboalela (Nagoya University Graduate School of Medicine cancer immunotherapy research center / Nagoya
University Graduate School of Medicine Department of Gastroenterological surgery / Faculty of
medicine, Zagazig University Medical Microbiology and Immunology)

O4-4. Simplification of the short period operation for T-cell production (Spo-T) method for CAR-T cells using RetroNectin in G-Rex Bioreactor.

Izumi Maki (Takara Bio Inc. CDM Center 3)

O4-5. Enhancing γδ T Cell Expansion and Memory Phenotypes Using Glucocorticoid-Related Compounds for Adoptive Cell Therapy Against Cancer

Chiung-Hui Liu (National Chung Hsing University College of Medicine, Doctoral Program in Tissue Engineering and Regenerative Medicine / National Chung Hsing University Department of Post-Baccalaureate Medicine)

O4-6. Generation of Spinal Cord Organoids for Transplantation Using Fluid Dynamics

**Keiko Imamura** (Kyoto University Center for iPS Cell Research and Application (CiRA) / RIKEN BioResource Research Center (BRC) / RIKEN Center for Advanced Intelligence Project (AIP))

O4-7. Establishment of organoids using gelatin-alginate enriched with liver-derived extracellular-matrix particles for organ repair

Tanveer A Mir (King Faisal Specialist Hospital and Research Centre Transplant Research and Innovation Department)

### Oral Session 5 (E or J)

Basic Science

Date: Thu. July 24, 14:25-15:25, Room 3

Chairs: Shigeki Yagyu (Innovative Research & Liaison Organization, Shinshu University / A-SEEDS Co., Ltd.)
Noriko Miyake (National Institute of Global Health and Medicine, Japan Institute for Health Security Department of Human Genetics)

O5-1. An AAP-Dependent-to-Independent Capsid Assembly Switch is exclusively induced by a single conserved F-Y mutation at the 5-fold interface

Anusha Sairavi (Oregon Health and Science University Molecular and Medical Genetics)

O5-2. In vivo liver-targeted genome editing by type I-E CRISPR Cas3 using AAV vector

**Takahiro Sato** (JIchi Medical University School of Medicine Department of Biochemistry / Jichi Medical University School of Medicine Department of Surgery, Division of Gastroenterological, General and Transplant Surgery)

O5-3. In Vivo Gene Therapy for Transthyretin Amyloidosis via CRISPR/Cas3

Saeko Ishida (Institute of Medical Science, The University of Tokyo Division of Animal Genetics)

O5-4. A red light-responsive photoswitch for deep tissue optogenetics

**Takahiro Nakajima** (Kanagawa Institute of Industrial Science and Technology "photo-switch therapeutics" project / Graduate School of Arts and Sciences, The University of Tokyo Department of Life Sciences)

O5-5. hsa\_circ\_0004781 Promotes Pancreatic Ductal Adenocarcinoma Progression through miR-9-5p and miR-338-3p Sponge Activity and Functional Validation with Synthesized Circular RNA

Kun-Lin Lee (Taipei Medical University Medical Laboratory Department)

- O5-6. Universal Base Editing Therapy for Hemophilia B via Induction of Gain-of-Function Variant
  Nemekhbayar Baatartsogt (Jichi Medical University Department of Biochemistry / Jichi Medical University Center for Gene
  Therapy Research)
- O5-7. IL-10RA Regulates IDO Expression in Shaping Lymphocyte Immune Responses

  Tzong-Shyuan Tai (Chang Gung Memorial Hospital Department of Medical Research and Development)

# Poster Session

# Poster Session 1 (E or J)

Date: Thu. July 24, 10:55-11:35, ExhibitionRoom

- P1-1. Exon skipping with TiD-X genome editing for Duchenne muscular dystrophy Miyu Asari (Science Tokyo Sch. of Life Sci. and Tech)
- P1-2. Stem cell-based gene therapy for malignant glioma using genome-edited human induced pluripotent stem cells
  Ryota Tamura (Keio University School of Medicine Department of Neurosurgery)
- P1-3. Impact of Cryopreservation on the Metabolism of Highly Activated NK-like Cells (GAIA-102)

  Kenta Ishimoto (Faculty of Pharmaceutical Sciences, Kyushu University R&D Laboratory for Innovative Biotherapeutics)
- P1-4. A stable manufacturing method for CAR-T cells that resolves donor dependent variations Megumi Kishimoto (AGC Inc. Technology General Division)
- P1-5. A simple CAR-T cell manufacturing process using RetroNectin in G-Rex Bioreactor. Sachiko Okamoto (Takara Bio Inc. CDM Center 3)
- P1-6. Comparison of the characteristics of human mesenchymal stem cells as oncolytic viruse carrier cells

  Makoto Sukegawa (Nippon Medical School Department of Biochemistry and Molecular Biology / Nippon Medical School

  Musashikosugi Hospital Department of Gastrointestinal Surgery / Nippon Medical School Department of

  Surgery)
- P1-7. Development of Photoactivatable Prime editing technology

  Takahiro Otabe (Kanagawa Institute of Industrial Science and Technology Photo-switch therapeutics project / The University of

  Tokyo Graduate School of Arts and Sciences)
- P1-8. Antitumor effects of DAMPs released from reovirus-infected tumor cells

  Fuminori Sakurai (Kindai University Faculty of Pharmacy / Graduate School of Pharmaceutical Sciences, Osaka University)
- P1-9. Elucidating pathogenic genes of hematopoietic dysfunction in PNH using patient-derived iPS cells Jiyuan Liao (The University of Tokyo The Institute of Medical Science)
- P1-10. Elucidation of the Antisense Oligonucleotide Delivery Mechanism Mediated by Inflammatory Cells
  Norio Motohashi (National Center of Neurology and Psychiatry Department of Molecular Therapy, National Institute of
  Neuroscience)
- P1-11. Development of dosing protocol to reduce the required dose of rAAV using adult stem cells

  Hiromi Hayashita-Kinoh (The University of Tokyo Division of Molecular and Medical Sciences, Center for Gene and Cell Therapy,

  The Institute of Medical Science)
- P1-12. Withdraw
- P1-13. Withdraw
- P1-14. The analysis of replication mechanism for developing novel AAV vector production system Yuri Ofusa (Jichi Medical University Center for Molecular Medicine, Division of Genetic Therapeutics)
- P1-15. Glycosylation analysis of recombinant adeno-associated virus Yuki Yamaguchi (Osaka University Graduate School of Engineering)
- P1-16. Evaluation of particle number and size distribution changes in lentiviral vector against freeze-thaw Mana Yamasaki (Osaka University Graduate School of Engineering)
- P1-17. Adeno-associated virus (AAV)-loaded ternary complex with neutralizing antibody evasion ability for liver parenchymal cell targeting and potential hemophilia therapy

  Yuto Honda (Institute of Sicence Tokyo Institute of Integrated Research / Institute of Sicence Tokyo School of Life Science and

  Technology / Innovation Center of NanoMedicine)

- P1-18. Improvement of gene-engineered oncolytic mammalian orthoreovirus utilizing reverse genetics technology Yuta Kanai (Research Institute for Microbial DiseasesOsaka University, Osaka University Department of Virology)
- P1-19. A Case Study for Understanding Impact of Nuclease Elimination on Bioprocess Cost and Downstream AAV5 Quality Using 3M<sup>TM</sup> Harvest RC Chromatographic Clarifier

Kazunori Senga (Solventum Japan Innovation Inc, Purification and Filtration Business)

- P1-20. Development of AAV9 purification materials utilizing novel peptide affinity ligands
  Hiroki Takahashi (FUJIFILM Corporation Bio Science & Engineering Laboratories)
- P1-21. Manufacturing and quality assessment of AAV vectors by newly established HAT cell line Susumu Uchiyama (The University of Osaka Graduate School of Engineering)
- P1-22. Development of a novel lentivirus affinity purification method using the Tim4 ligand Yuki Marutani (FUJIFILM Corporation Bio Science & Engineering Laboratory)
- P1-23. Evaluation of Ready-to-Use Lipid Nanoparticles for ex-vivo mRNA and DNA delivery in primary human T cells Sayako Umetani (FUJIFILM Corporation Bio Science & Engineering Laboratory)
- P1-24. Tissue-specific transgene expression by intraperitoneal administration of non-toxic herpes simplex virus-based vectors into neonatal mice

Yoshitaka Miyagawa (Nippon Medical School Department of Biochemistry and Molecular Biology)

P1-25. Process Development for AAV Vector Manufacturing with All-in-One Plasmid and Analysis of Cost Saving Impact by Single Transfection System

Shunsuke Saito (Synplogen Co., Ltd. Medical Business Unit)

- P1-26. Analytical characterization of full and empty AAV capsids of AAV8 and AAV9
  Youji Fujita (TOSOH Analysis and Research Center Co.,Ltd. Tokyo Division)
- P1-27. Development of lentiviral vector with high titer and improved safety.

  Izumi Maki (Takara Bio Inc. CDM Center 3)
- P1-28. Oncolytic Activity of Blue Light-Controlled Measles Virus for Tongue Cancer and Melanoma Cell Lines
  Wang Yuying (The University of Tokyo Department of Microbiology, Graduate School of Medicine and Faculty of Medicine)
- P1-29. Production of AAV vectors by large-scale PCR amplification

  Rinji Akada (Yamaguchi University Graduate School of Sciences and Technology for Innovation / Helix Extension Inc.)
- P1-30. Quality and functional evaluation of rAAV5 derived from AAV stable Producer cell line Yusuke Mori (FUJIFILM Corporation Bio Science & Engineering Laboratories)
- P1-31. Optimization of Activation, Culture Conditions, and Promoter Activity for CAR-T/NK Cell Manufacturing from Peripheral and Cord Blood Using Lentiviral Vectors

Naokazu Nakamura (The Institute of Medical Science, The University of Tokyo Division of Molecular and Medical Genetivs, Center for Gene and Cell Therapy / Graduate School of Medicine, Kyoto University Department of Hematology)

- P1-32. Less Invasive Light Irradiation Method for Inner Ear Gene Therapy based on Photoswitch Technology
  Masao Noda (Jichi Medical University Department of Otolaryngology and Head and Neck Surgery / Jichi Medical University
  Division of Neurological Gene Therapy)
- P1-33. Development of Mutated Ligand-Based CAR-T Cells Targeting the CD123/CD131 Complex for Acute Myeloid Leukemia

Aiko Hasegawa (Shinshu University School of Medicine Department of Pediatrics)

- P1-34. Evaluation of Telomerase-specific Oncolytic Adenovirus Infection to Spinal Tumors for Therapeutic Use Ryo Takatori (Okayama University Hospital Orthopaedic Surgery)
- P1-35. Double-stand break-free gene correction in IEI

**Shoichiro Tateishi** (National Defense Medical College / Department of Pediatrics / National Center for Child Health and Development / Department of Human Genetics)

P1-36. Comparison of in vivo dynamics between human amniotic and bone marrow derived mesenchymal stem cells administered into peritoneal dissemination pancreatic cancer cell model mice

Yoshiyuki Yamazaki (Nippon Medical School Department of Biochemistry and Molecular Biology)

P1-37. Development of a Novel T cell Activation reagent using Rabbit anti-CD28 antibody to prevent T cell Exhaustion Mayu Yamaguchi (FUJIFILM Corporation Bio Science & Engineering Laboratory)

# P1-38. Notch ligand-based supplements to maintain undifferentiated T cells and CAR-T cells

Nao Yamazaki (FUJIFILM Coporation BioScience & Engineering Laboratories)

#### P1-39. Cell-based CRISPR-Cas9 delivery system for muscular dystrophy gene therapy

**Uikyu Bang** (Kyoto University Graduate School of Medicine)

### P1-40. Potential for enhanced oncolytic HSV-1 therapy via SHED as a vehicle against malignant gliomas

**Satoru Kida** (Hamamatsu University School of Medicine Department of Neurosurgery)

#### P1-41. Development of a Novel Oncolytic Adenovirus for Neuroblastoma

**Ryo Ikushima** (Kyoto Prefectural University of Medicine Department of Pediatrics)

#### P1-42. Construction of gene therapy system for alveolar bone regeneration

Mariko Yamamoto (Kansai Women's College Department of Health Science / Kyoto University)

# P1-43. Tumor-Specific Adaptive Immunity Induced by NK-like cell GAIA-102 Depends on Host NK Cell-Derived IFN-γ via CCR5 Signaling

Situo Zheng (Graduate School of Pharmaceutical Sciences, Kyushu University R&D Laboratory for Innovative Biotherapeutics)

### P1-44. Preclinical study of rAAV-mediated gene therapy for xeroderma pigmentosum group A

Mizuho Takahashi (The Institute of Medical Science, The University of Tokyo Division of Molecular and Medical Genetics Center for Gene & Cell Therapy / Graduated School of Medicine, Nippon Medical School Department of Neurological Science)

# P1-45. Triple gene delivery of dopamine-synthesizing enzymes into the striatal neurons alleviated motor symptoms in a mouse model of tyrosine hydroxylase deficiency

**Yo Sato** (Institute of Science Tokyo School of Life Science and Technology)

# P1-46. Cerebroprotective effect of human amniotic mesenchymal stromal cell-derived exosomes in a mouse model of cerebral ischemia.

Moeko Saito (Nippon Medical School Laboratory for Clinical Research, Collaborative Research Center)

# P1-47. Pre-apheresis red blood cell distribution width (RDW) predicts prognosis after chimeric antigen receptor T (CAR-T) cell therapy

Nakamura Naokazu (Graduate School of Medicine, Kyoto University Department of Hematology / The Institute of Medical Science,
The University of Tokyo Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy)

# P1-48. Increased relative eosinophil counts portend neck edema after chimeric antigen receptor (CAR)-T cell therapy (NEC)

Naokazu Nakamura (Graduate School of Medicine, Kyoto University Department of Hematology / The Institute of Medical Science,
The University of Tokyo Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy)

# P1-49. Study on a sensitive detection of off-target genome editing by Wild-type blocking PCR

Takuma Yamashita (National Institute of Health Sciences Division of Molecular Target and Gene Therepy)

# P1-50. Utilisation of Next Generations Sequencing (NGS) in an ICH Q5A R2 Compliant QC Testing Program and Virus Vectors and Cell-Based Therapies

**Hiroyuki Urano** (LunaPath Institute of Toxicologic Pathology Co., Ltd. Grobal Business Development / Showa University Graduate School of Medicine)

### Poster Session 2 (E or J)

Date: Fri. July 25, 13:00-13:40, ExhibitionRoom

#### P2-1. Effects of cellular uptake pathway on the efficacy of Adenovirus vector-based vaccine

Rika Onishi (Osaka University Pharmaceutical Sciences)

### P2-2. Development of CRISPR-Cas13 system-expressing adenovirus vector.

Yuki Osakada (Osaka University Graduate School of Pharmaceutical Sciences)

# P2-3. Establishment of a novel human amniotic epithelial-derived cell line, HAT, for AAV vector production

Yugo Hirai (Chitose Laboratory Corp. Tech & Biz Development Div. / Manufacturing Technology Association of Biologics)

### P2-4. Charge Detection Mass Spectrometry-Based Evaluation of Critical Quality Attributes for Recombinant Adeno-Associated Virus.

Ryoji Nakatsuka (Osaka university Department of Biotechnology, Graduate School of Engineering / Shimadzu Corporation
Technology Research Laboratory / Osaka University Shimadzu Analytical Innovation Research Laboratories)

### P2-5. Efforts to supply clinical AAV vectors for academia and startups

**Toshie Kuwahara** (*U-Medico Inc. Manufacturing Business Headquarters*)

### P2-6. Analysis of glycosylation on AAV capsids toward gene therapy

Mikako Wada (The Institute of Medical Science, The University of Tokyo Division of Molecular and Medical Genetics Center for Gene and Cell Therapy)

### P2-7. Optimization of upstream process for lentiviral vector production in accordance with GMP guidelines

**Yukage Kobari** (The Institute of Medical Science, The University of Tokyo Division of Molecular and Medical Genetics, Center for Gene & Cell Therapy)

# P2-8. Plasmid design and large-scale ultracentrifugation for production of high-purity recombinant adeno-associated virus

**Mikako Wada** (The Institute of Medical Science, The University of Tokyo Division of Molecular and Medical Genetics Center for Gene and Cell Therapy)

# P2-9. Elucidation of encapsidated genome size effect on physicochemical and biological properties of adeno-associated virus vector

Kiichi Hirohata (The University of Osaka Department of Biotechnology, Graduate School of Engineering)

# P2-10. CRISPR-Cas9 RNP delivery systems comprising tannic acid and fine-tuned boronic acid-conjugated polymers for tissue-selective in vivo genome editing

**Toshizumi Chino** (Institute of Science Tokyo Department of Life Science and Technology / Institute of Science Tokyo Laboratry of Chemistry and Life Science)

### P2-11. Lab-Scale Purification Method of Adeno-Associated Vector Using Chloroform

Yoshihide Schara (Jichi Medical University Division of Genetic Therapeutics, Center for Molecular Medicine)

#### P2-12. Investigation of process parameters for high production of rAAV vectors by transfection

**Kyoko Masumi** (Kobe University Graduate School of Science, Technology and Innovation)

### P2-13. Development of a novel purification method for AAV vectors using tangential flow filtration

**Yuji Tsunekawa** (The Institute of Medical Science, The University of Tokyo, Center for Gene & Cell Therapy Division of Molecular and Medical Genetics)

### P2-14. Development of AAV vector productivity improvement technology by codon optimization

**Jumpei Hayakawa** (The Institute of Medical Science, The University of Tokyo Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, / MicroBiopharm Japan)

### P2-15. Investigation of human amnion epithelial-derived cells for the establishment of high AAV vector-producing cells

**Mikako Wada** (The Institute of Medical Science, The University of Tokyo Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy)

# P2-16. Characterization on Adeno-Associated Virus Vectors by Anion Exchange Chromatography Based Multi-Method Assav

Zhuolun Yang (Osaka University Engineering School, Biotechnology Department)

# P2-17. Improving AAV Purity and Yield Using Multicolumn Countercurrent Solvent Gradient Purification (MCSGP)

Ryosuke Takahashi (YMC CO., LTD.)

# P2-18. p53-expressing oncolytic virus-transduced dendritic cell vaccines augment the anti-tumor effect of oncolytic virotherapy by activating anti-tumor immune responses

Noahiro Okada (Okayama University Department of Gastroenterological Surgery)

# P2-19. Efficacy of IL-10 gene therapy by adeno-associated virus vectors in experimental autoimmune encephalomyelitis Hiroto Kodera (Graduate School of Medicine, Nippon Medical School Department of Neurology / Center for Gene and Cell

Therapy, the Institute of Medical Science, the University of Tokyo Division of Molecular and Medical Genetics)

# P2-20. A Blood-Brain Barrier-Penetrating Hematopoietic Stem Cell Gene Therapy Approach for GM1 Gangliosidosis Toshiki Tsunogai (Jikei University School of Medicine Department of Pediatrics)

# P2-21. Development of triple-regulated conditionally replicating adenovirus for effective and safer treatment of peritoneal carcinomatosis

Yuya Nishikawaji (Kagoshima University Graduate School of Medicine and Dental Sciences Department of Gene Therapy and Regenerative Medicine)

#### P2-22. Brain Tumor Therapy with STING Agonist and Oncolytic Vaccinia Virus

**Yuhei Takido** (Nagoya University Graduate School of Medicine Department of Neurosurgery / Nagoya University Graduate School of Medicine Cancer Immune Therapy Research Center)

P2-23. Preclinical study of fusogenic vaccinia virus armed with immunostimulatory genes in orthotopic pancreatic cancer models

Hajime Kurosaki (Tottori University Faculty of Medicine Division of Genomic Medicine)

P2-24. Inhibiting an invasion-promoting factor to overcome anti-angiogenic therapy resistance in glioblastoma

Yasushi Soda (The Institute of Medical Science, The University of Tokyo Division of Molecular and Medical Genetics / The Salk Institute)

P2-25. Study of to Restore Gene Therapy of Dermatan 4-O-Sulfotransferase-1 Expression in Muscle Contracture Type Ehlers-Danlos Syndrome by Dermatan sulfate biotynthetic enzyme Expression

Yuko Kasahara (The Institute of Medical Science, The university of Tokyo Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy)

P2-26. Neuroprotective effects of amniotic membrane-derived mesenchymal stem cell transplantation in a rat model of cerebral ischemia-reperfusion injury

Chikako Nito (Nippon Medical School Collaborative Research Center)

P2-27. AAV-mediated Delivery of USP48-targted shRNA Effectively Rescues the Cognitive Impairments in a Transgenic Mouse Model of Alzheimer's Disease

Yung-Feng Liao (Academia Sinica ICOB)

P2-28. Correction of β-Globin Gene Mutation Using Prime Editing in Human Embryonic Kidney 293T Cells

**Tiwaporn Nualkaew** (School of Allied Health Sciences, Walailak University, Nakhon Si Thammarat, Thailand / Hematology and Transfusion Science Research Center, Walailak University, Nakhon Si Thammarat, Thailand)

P2-29. Enhancing BCMA-Targeted CAR-T Cell Therapy for Multiple Myeloma: Overcoming receptor BCMA Shedding with a Novel Secretory Peptide Approach

Sheetal Sheetal (Department of Biosciences Jamia Millia Islamia / Multidisciplinary Centre for advanced research and studies

Jamia Millia Islamia / MicroCrispr Pvt. Ltd. Research and development / Department of Biotechnology Jamia

Millia Islamia)

P2-30. Intraperitoneal administration of the CD133-targeted oncolytic adenovirus suppresses peritoneal metastases of colon cancer in mouse xenograft model

Mizuho Sato-Dahlman (University of Minnesota Surgery)

P2-31. Sealing Amniotic Fluid Leak in Previable Gestations with a Novel Intraamniotic Fibrin and Platelet Therapy: A Multicentric Prospective Case Series

Sowmya H Rajashekar (Santasa Fertility Pvt Ltd Reproductive Medicine)

P2-32. Loss of polr1c leads to photoreceptors degeneration in zebrafish

Zulvikar Syambani Ulhaq (National Research and Innovation Agency Republic of Indonesia)

P2-33. Withdraw

P2-34. Development of a Short-Term Manufacturing Method, Spo-T for TCR-T Cells Using Retroviral Vectors Kaho Takeichi (Takara Bio Incorporated CDM Center 3)

P2-35. Genetic Insights into Vasomotor Symptoms: A Genome-Wide Association Study and Polygenic Risk Score Analysis Using Data from the Taiwan Biobank

Airu Hsieh (Tamkang University Department of Statistics)

P2-36. Infrastructure Development for Academia GMP Production of Adeno-Associated Virus (AAV) Vectors

**Hitomi Ikeda** (The Institute of Medical Science, The University of Tokyo Division of Molecular and Medical Genetics, Center for Gene & Cell Therapy)

P2-37. Optimization of synthetic riboswitches that function in mammalian cells

Yohei Yokobayashi (Okinawa Institute of Science and Technology Graduate University Nucleic Acid Chemistry and Engineering Unit)

P2-38. A Robust and Accurate Lentiviral Provirus Copy Number Quantification Method Using qPCR Yoshinori Tanaka (TAKARA BIO INC. CDM Center 3)

#### P2-39. Functional Enhancement Amino Acid Substitution Sites in Coagulation Factor VIII

**Yuji Kashiwakura** (Jichi Medical University Department of Biochemistry / Jichi Medical University Center for Gene Therapy Research)

P2-40. Influences of variations in stoichiometry and deamidation ratio of adeno-associated viral vector protein on transduction efficiency

**Takahiro Maruno** (U-Medico Inc. Manufacturing Business Headquarters / The University of Osaka Graduate School of Engineering)

#### P2-41. Optimization of AAV capsids and promoters for marmoset cerebral cortical cell types

Yasunori Matsuzaki (Gunma University Graduate School of Medicine / Gunma University Initiative for Advanced Research Viral Vector Core)

#### P2-42. Development of Novel Nonwoven Cell Culture Substrates for Virus Vector Production

Kouhei Sasaki (Japan Vilene Company, Ltd. Central Reserch Laboratory)

# P2-43. Development of educational program on the development & production of gene therapy products in collaboration between Japan Society of Gene and Cell Therapy and BRET

**Kazuhisa Uchida** (Kobe University Graduate Shcool of Science, Technology and Innovation / Biologics center for research and training)

### P2-44. A novel oncolytic viral therapy for human chemotherapy-resistant colorectal cancer

**Ogata Hisanobu** (Kyushu University Hospital Clinical Education Center)

### P2-45. Development of novel gene-modified coxsackievirus therapy for enhanced anti-tumour effect

Ryuki Kusakabe (The Jikei University School of Medicine Division of Oncology)

P2-46. Withdraw

### Late Breaking Abstract (Poster) (J/E)

LP1. CDP-ribitol prodrug treatment ameliorates ISPD-deficient muscular dystrophy
Motoi Kanagawa (Department of Cell Biology and Molecular Medicine, Ehime University Graduate School of Medicine)

# LP2. CRISPR/Cas9 Engineering of 3D Genome Structural Variants: Unveiling Predictive Models of Oncogene Activation in Cancer Genomes

Zhichao Xu (Department of Biochemistry and Molecular Biology McGovern Medical School, UT Health Houston)

# LP3. Optimizing Recombinant AAV Vector Research: The Role of Centrifugation in Purification and Characterization Soji Murayama (Beckman Coulter Life Sciences)

# LP4. Temporal requirement of dystroglycan glycosylation during brain development and rescue of severe cortical dysplasia via gene delivery in the fetal stage

**Atsushi Sudo** (Department of Neurology, Graduate School of Medicine, The University of Tokyo / Division of Molecular Brain Science, Kobe University Graduate School of Medicine)

# LP5. Highly Efficient Engineering Of Difficult-to-Transfect Immune Cells Using MaxCyte® Electroporation Peter Gee (MaxCyte, Inc.)

### LP6. Development and efficacy evaluation of novel vaccines using AAV vector-exosome complexes

**Ken Sugo** (Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, The Institute of Medical Science, The University of Tokyo)

# LP7. Development of a tightly regulated, rapid, and reversible ON/OFF gene expression system and its application in neuroscience research

Takahiko Matsuda (Graduate School of Science, University of Hyogo)

# LP8. Development of a novel cancer therapy using immunostimulatory oncolytic coxsackievirus B3 Shohei Miyamoto (Division of Oncology, Research Center for Medical Sciences, The Jikei University School of Medicine)

# LP9. Identification and evaluation of drug candidates that enhance recombinant AAV-mediated transgene expression using a newly engineered cell line screening system.

Guillermo Posadas-Herrera (Division of Molecular and Medical Genetics, The Institute of Medical Science, University of Tokyo)

# LP10. Advancing AAV Capsid Engineering for Targeted Gene Therapy Using PackGene's π-Icosa Platform Derrick Zhong (PackGene Biotech)

- LP11. Enhancing AAV Production Efficiency through Plasmid Modification and Dual-Plasmid Systems

  Derrick Zhong (PackGene Biotech)
- LP12. Chemical Synthesis of Chimeric Antigen Receptor-Modified Natural Killer Cells with Novel Antibody Modification Platform "AJICAP®"
  Shinsuke Segawa (AJINOMOTO CO., INC.)

# Reports on the ESGCT Spring School 2025 (E)

Date: Thu. July 24, 12:45-13:10, Room 3

Chairs: Shin-ichi Muramatsu (Division of Neurological Gene Therapy, Center for Open Innovation, Jichi Medical University)
Juan A. Bueren (CIEMAT and Biomedical Network Centre for Research on Rare Diseases, CIBERER)

- ESS-1. Takahiro Sato (Dept. of Biochemistry Jichi Medical University School of Medicine)
- **ESS-2. Mizuho Takahashi** (Division of Molecular and Medical Genetics Center for Gene & Cell Therapy, The Institute of Medical Science, The University of Tokyo)

# Corporate Seminar

# **Morning Seminar 1**

(Mitsui Chemicals, Inc.)

Date: Thu. July 24, 8:45-9:30, Room 1

Chair: Akihiro Okabe (Mitsui Chemicals, Inc.)

MS1-1. Introduction of Cell Culture Equipment Developed with Functional Materials for High Productivity Shoko Matsumura (Mitsui Chemicals, Inc.)

MS1-2. Mass Culture System Using Tidal Culture with Nonwoven Scaffold Materials Shunmei Chiba (FullStem Co., Ltd.)

# **Morning Seminar 2**

(U-Medico Inc.)

Date: Thu. July 24, 8:45-9:30, Room 2

Chair: Tsukasa Ohmori (Dept. of Biochemistry, Jichi Medical University School of Medicine)

MS2. The Present Landscape and Future Perspective of Process Development and Quality Analysis in Gene Therapy Susumu Uchiyama (Dept. of Biotechnology, Graduate School of Engineering, The University of Osaka)

# **Morning Seminar 3**

(Sartorius Japan K.K.)

Date: Thu. July 24, 8:45-9:30, Room 3

Chair: Yusuke Maruyama (Sartorius Japan K.K.)

MS3. Understanding the Pathophysiology of Ehlers-Danlos Syndrome: Foundations and Future Prospects for Gene Therapy

**Yuko Kasahara** (Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, The Institute of Medical Science, The University of Tokyo)

# **Morning Seminar 4**

(Revvity, Inc.)

Date: Fri. July 25, 8:45-9:30, Room 1

Chair: Ryuichi Uozumi (Technology and Licensing, Revvity, Inc.)

MS4. Enhancing AAV Production Efficiency: A Dual Approach to DoE Optimization and Economic Feasibility Analysis

Igor Alves Mancilla (Process Development, Revvity Gene Delivery, Revvity, Inc.)

### **Morning Seminar 5**

Initiatives for Further Advancement of Gene Therapy Using AAV Vectors (Unchained Labs)

Date: Fri. July 25, 8:45-9:30, Room 2

Chair: Takuma Iwasaki (Field Application Scientist, Unchained Labs)

MS5-1. Exploration of Efficient AAV Vector Production Strategies

**Mikako Wada** (Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, The Institute of Medical Science, The University of Tokyo)

MS5-2. AAV vectors: expansion of research and standardization

Hiroaki Mizukami (Division of Genetic Therapeutics, Center for Molecular Medicine, Jichi Medical University)

# **Morning Seminar 6**

AAV-based oral active immunotherapy and vaccines

(La Vita Co.)

Date: Fri. July 25, 8:45-9:30, Room 3

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

Shin-ichi Muramatsu (Center for Open Innovation, Jichi Medical University)

MS6-1. Development of active immunotherapy and vaccines for Alzheimer's disease

Takeshi Tabira (Graduate School of Medicine, Juntendo University)

MS6-2. Gene transfer into target cells using modified viral vectors

Yasunari Matsuzaka (Department of Microbiology and Immunology, Showa Medical University)

### **Luncheon Seminar 1**

(AnGes Inc.)

Date: Wed. July 23, 11:10-12:10, Room 1

Chair: Ryuichi Morishita (Dept. of Clinical Gene Therapy, Osaka University Medical School)

LS1. Current Status and Issues of Gene Therapy and Nucleic Acid Drugs in Japan

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

#### **Luncheon Seminar 2**

The innovation imperative: driving the evolution of genomic medicine (Cytiva)

Date: Wed. July 23, 11:10-12:10, Room 2

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

LS2-1. Marc Bisschops (Genomic Medicine, Cytiva)

LS2-2. Peiqing Zhang (Genomic Medicine, Cytiva)

### **Luncheon Seminar 3**

(Tosoh Corporation)

Date: Wed. July 23, 11:10-12:10, Room 3

Chair: Yuji Tsunekawa (Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, The Institute of Medical Science, The University of Tokyo)

LS3-1. Solid-state nanopore sensors for characterizing AAV vectors

Makusu Tsutsui (SANKEN, The University of Osaka)

LS3-2. Advanced Chromatographic Techniques for Purification and Characterization of Full Capsid AAV Vectors

Kouhei Yoshida (Tosoh Corporation Life Science Research Laboratory)

#### **Luncheon Seminar 4**

(KANEKA CORPORATION)

Date: Thu. July 24, 11:40-12:40, Room 1

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

LS4-1. Translational Research on Amnion MSCs for Muscular Dystrophy: From Bench to Bedside

**Yuko Kasahara** (Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, The Institute of Medical Science, The University of Tokyo)

LS4-2. Enhancing the Safety of AAV Gene Therapy for DMD: Development of a Dose-Reduction Protocol Using AMSC Combination Therapy

**Hiromi Hayashita-Kinoh** (Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, The Institute of Medical Science, The University of Tokyo)

# Program

### **Luncheon Seminar 5**

(Takara Bio Inc.)

Date: Thu. July 24, 11:40-12:40, Room 2

Chair: Maki Tanaka (TAKARA BIO INC.)

### LS5. Gene Therapy for Genetic Disorders Using Engineered AAV Capsid

**Toru Uchiyama** (Division of Molecular Pathogenesis, Dept. of Human Genetics, National Center for Child Health and Development)

#### **Luncheon Seminar 6**

(Miltenyi Biotec)

Date: Thu. July 24, 11:40-12:40, Room 3

Chair: Hiroshi Fujiwara (Dept. of Personalized Cancer Immunotherapy, Mie University Graduate School of Medicine)

#### LS6. R&D for GAIA-102, a novel NK-like phenotype that can eliminate solid tumors

Yoshikazu Yonemitsu (R&D Laboratory for Innovative Biotherapeutics Science, Kyushu University Graduate School of Pharmaceutical Sciences / GAIA BioMedicine, Inc.)

#### **Luncheon Seminar 7**

Importance of Manufacturing Process and Quality Analysis thorough Development of AAV-based Gene Therapy Product
(Synplogen Co., Ltd. / TAIYO PHARMA TECH CO., LTD.)

Date: Fri. July 25, 11:55-12:55, Room 1

Chair: Naoyuki Yamada (Synplogen Co., Ltd.)

### LS7-1. AAV vector manufacturing and quality control for first in human study

Susumu Uchiyama (Osaka University Graduate School of Engineering)

# LS7-2. Gene Therapy Bio Foundry; Integrated Value Chain from DNA Design & Synthesis to Development of Manufacturing Process & Analytical Testing Method

Kumiko Otsuki (Synplogen Co., Ltd.)

### **Luncheon Seminar 8**

(ROHTO Pharmaceutical Co.,Ltd.)

Date: Fri. July 25, 11:55-12:55, Room 2

Chair: Mahito Nakanishi (TOKIWA Bio Inc.)

#### LS8-1. Development and Application of Stealth RNA Vector

Mahito Nakanishi (TOKIWA Bio Inc.)

#### LS8-2. Progress in direct reprogramming research and its medical prospects

Atsushi Suzuki (Medical Institute of Bioregulation, Kyushu University)

### **Luncheon Seminar 9-1**

(PackGene Biotech INC.)

Date: Fri. July 25, 11:50-12:20, Room 3

Chairs: Shunjun Liu (Accelerate Bio Inc.)
Tomoki Echigoya (Accelerate Bio Inc.)

# LS9-1. Current Challenges and Our Approaches to Developing Best-in-Class rAAV Manufacturing Processes for Large-scale GMP Production

Liying Yang (PackGene)

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### **Luncheon Seminar 9-2**

(Merck Ltd.)

Date: Fri. July 25, 12:25-12:55, Room 3

Chair: Sadao Ozawa (Merck Ltd.)

LS9-2. Application study of Salt Tolerant Benzonase and AAV production Yosuke Imamura (Merck Ltd.)

### Afternoon Seminar 1

(QIAGEN K.K.)

Date: Wed. July 23, 15:15-16:15, Room 1

Chair: Yasuyuki Miyazaki (QIAGEN K.K.)

AS1-1. Quantitative and Digital PCR - Past, Present and the Future

Mikael Kubista (Czech Academy of Science & Precision BioAnalytics, Sweden)

AS1-2. Verification of an AAV Vector Genome Quantification Method and DNA Extraction Efficiency for Biodistribution Studies Using Digital PCR

Yurie Okawa (Shin Nippon Biomedical Laboratories, Ltd.)

## **Afternoon Seminar 2**

Advancing Cell Therapy and Viral Vector Manufacturing (Thermo Fisher Scientific)

Date: Wed. July 23, 15:15-16:15, Room 2

Chair: Shin Kawamata (Cyto-Facto Inc.)

AS2-1.Advancing the Manufacturing of "my iPS" Cells Using a Closed Automated Cell Processing System Masayoshi Tsukahara (Research and Development Center, CiRA Foundation)

AS2-2. Addressing challenges in viral vector production: Innovations to accelerate development, improve scalability, and reduce cost of goods

Natasha Serzedello (Thermo Fisher Scientific)

AS2-3. Platformable AAV virus vector purification solution

Haruna Tomotake (Bioproduction Division, Life Technologies Japan Ltd.)

# **Afternoon Seminar 3**

Advancing Cell and Gene Therapies Through Non-Viral Engineering Modalities (MaxCyte, Inc., PHC Corporation)

Date: Wed. July 23, 15:15-16:15, Room 3

Chair: Lucy Yang (MaxCyte, Inc.)

AS3-1. Masahisa Ohishi (MaxCyte, Inc.)

AS3-2. Peter Gee (MaxCyte, Inc.)

# **Afternoon Seminar 4**

(FUKOKU CO., LTD)

Date: Thu. July 24, 15:35-16:20, Room 1

Chair: Yui Harada (Graduate School of Pharmaceutical Sciences, Kyushu University)

AS4. Efforts Toward GMP-Compliant AAV Vector Manufacturing in Academia

Mikako Wada (The Institute of Medical Science, The University of Tokyo Division of Molecular and Medical Genetics Center for Gene and Cell Therapy)

### **Afternoon Seminar 5**

(KM Biologics Co., Ltd.)

Date: Thu. July 24, 15:35-16:20, Room 2

Chair: Fumio Endo (Kumamoto-Ezuko Medical Center for the Severely Disabled)

AS5. Importance of Newborn Screening for Therapeutic Strategy with Gene Therapy and Stem Cell Transplantation Shirou Matsumoto (Dept. of Neonatology, Kumamoto University Hospital)

#### **Afternoon Seminar 6**

(Bio-Rad Laboratories, Inc.)

Date: Thu. July 24, 15:35-16:20, Room 3

Chair: Shin Adachi (Bio-Rad Laboratories K.K.)

AS6-1. The Impact of Droplet Digital PCR on Gene and Cell Therapy Development

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

AS6-2. Application of ddPCR in Quality Evaluation of Gene Therapy Products: Considerations for Ensuring Measurement Stability

Takenori Yamamoto (Division of Molecular Target and Gene Therapy Products, National Institute of Health Sciences)

### Afternoon Seminar 7

Advancing the Frontier of Gene Therapy with AAV (AJINOMOTO CO., INC)

Date: Fri. July 25, 14:55-15:40, Room 1

Chair: Susumu Uchiyama (Dept. of Biotechnology, Graduate School of Engineering, The University of Osaka)

AS7-1. Towards the Development and Provision of Optimal Media for AAV Takahiro Katayama (AJINOMOTO CO., INC)

AS7-2. Fueling the Future of AAV Gene Therapies with Manufacturing Innovation

Rachael Hardison (Forge Biologics)

#### **Afternoon Seminar 8**

4D-150 for wet AMD and DME: a targeted and transformative backbone therapy empowered by directed molecular evolution

(4D Molecular Therapeutics, Inc.)

Date: Fri. July 25, 14:55-15:40, Room 2

Chair: Noriyuki Kasahara (Dept. of Neurological Surgery and Radiation Oncology, University of California, San Francisco / 4D Molecular Therapeutics, Inc.)

AS8. 4D-150 for wet AMD and DME: a targeted and transformative backbone therapy empowered by directed molecular evolution

David Kirn (4D Molecular Therapeutics, Inc.)

### Afternoon Seminar 9

(ASAHI KASEI LIFE SCIENCE CORPORATION)

Date: Fri. July 25, 14:55-15:40, Room 3

Chair: Hiroki Fukutomi (ASAHI KASEI LIFE SCIENCE CORPORATION)

AS9. Utilization of tangential flow filtration filters in AAV clarification process

 $\textbf{Ryosuke Koide} \ (\textit{ASAHI KASEI LIFE SCIENCE CORPORATION})$ 

#### **Afternoon Seminar 10**

(Sartorius Stedim Group)

Date: Thu. July 24, 16:25-17:10, Room 3

Chair: Ryosuke Fujimori (Sartorius Stedim Group)

AS10. Exploring the advantages of lipid-based gene delivery for CAR-T and CAR-NK cell therapy Ian Villamagna (Sartorius Stedim Group)

# **Evening Seminar 1**

(JCR Pharmaceuticals Co., Ltd.)

Date: Wed. July 23, 18:00-18:45, Room 1

Chair: Kenichi Kashimada (Division of Endocrinology and Metabolism, National Center for Child Health and Development)

ES1. Gene therapy with blood-brain barrier-penetrating AAV

Hiroyuki Sonoda (JCR Pharmaceuticals Co., Ltd.)

### **Evening Seminar 2**

Glycans and Gene/Cell Therapy (VectorBuilder Inc.)

Date: Wed. July 23, 18:00-18:45, Room 2

Chair: Masafumi Onodera (Graduate School of Engineering, Osaka University)

ES2. Advancing AAV production in combination with our large-scale AAV capsid evolution and high-throughput antibody screening

Miho Matakatsu (VectorBuilder Japan Inc.)

### **Evening Seminar 3**

Glycans and Gene / Cell Therapy (The IT Lab Co.,Ltd.)

Date: Wed. July 23, 18:00-18:45, Room 3

Chair: Takashi Okada (Center for Gene and Cell Therapy, The Institute of Medical Science, The University of Tokyo)

ES3-1. Algal lectins open up new technological developments-Applications in medicine of highly accurate glycan recognition ability-

Kanji Hori (Hiroshima University, The IT Lab)

ES3-2. Analysis of AAV Capsid Surface Glycosylation for Development of a Purification Strategy

Mikako Wada (The Institute of Medical Science, The University of Tokyo Division of Molecular and Medical Genetics Center for Gene and Cell Therapy)

### **Evening Seminar 4**

(MediRidge Co.,Ltd. / TEIJIN REGENET CO., LTD.)

Date: Thu. July 24, 17:35-18:20, Room 1

Chair: Yozo Nakazawa (Dept. of Pediatric, Shinshu University School of Medicine)

ES4. Development trends and prospects for CAR-T therapy

Shigeki Yagyu (Innovative Research & Liaison Organization, Shinshu University / A-SEEDS Co., Ltd.)

# **Evening Seminar 5**

(Terumo BCT, Inc.)

Date: Thu. July 24, 17:35-18:20, Room 2

Chair: Masahiro Kino-oka (Dept. of Biotechnology, Graduate School of Engineering, The University of Osaka)

ES5. Collaboration between CiRA Foundation and Terumo BCT: toward automation of iPSC expansion and differentiation

Masayoshi Tsukahara (CiRA Foundation)

# **Evening Seminar 6**

A novel approach to developing cell therapies (FUJIFILM Wako Pure Chemical Corporation)

Date: Thu. July 24, 17:35-18:20, Room 3

Chair: Takashi Torashima (FUJIFILM Wako Pure Chemical Corporation)

ES6-1. Shinichi Hashimoto (Bio Science & Engineering Laboratories, FUJIFILM Corporation)

ES6-2. Yuki Kagoya (Division of Tumor Immunology, Institute for Advanced Medical Research, Keio University School of Medicine)