

THE 25th ANNUAL MEETING

JSGCT2019

JAPAN SOCIETY OF GENE AND CELL THERAPY

Program & Abstracts

Date

July 21-23, 2019

Venue

**YASUDA AUDITORIUM and others,
THE UNIVERSITY OF TOKYO
HONGO CAMPUS**

7-3-1 Hongo Bunkyo-ku, Tokyo 113-8654, Japan

TOKYO



PROGRAM

JSGCT Chairman and President's Lecture (E)

Date: July 22, 2019, 11:20-12:00, Yasuda Auditorium

Chairperson: Shigetaka Asano

Tomoki Todo (*Chairman of Board of Directors, JSGCT, President, 25th Annual Meeting of JSGCT 2019, The Institute of Medical Science, The University of Tokyo*)

The dawn of the era for oncolytic virus therapy

Presidential Special Program (J)

Date: July 22, 2019, 15:10-17:10, Yasuda Auditorium

Chairpersons: Yoshikatsu Eto & Tatsuya Kondo

PSP-1. Hidemasa Oh (*Okayama University Hospital*)

Clinical translation of cell therapy for congenital heart disease

PSP-2. Jun Takahashi (*Center for iPS Cell Research and Application, Kyoto University*)

Stem cell-based therapy for Parkinson's disease

PSP-3. Osamu Honmou (*Sapporo Medical University*)

Intravenous injection of mesenchymal stem cells for spinal cord injury patients

PSP-4. Atsuhiko Kawamoto (*Foundation for Biomedical Research and Innovation at Kobe*)

Autologous CD34-positive cell therapy for critical limb ischemia

PSP-5. Hiroshi Shiku (*Mie University Graduate School of Medicine*)

Adoptive cell therapy with TCR-gene engineered T cells targeting NY-ESO-1

PSP-6. Yoshiaki Maruyama (*Pharmaceuticals and Medical Devices Agency*)

Experiences from PMDA – SAKIGAKE designation system for regenerative medical products

Special Lecture (E)

Date: July 22, 2019, 10:40-11:20, Yasuda Auditorium

Chairperson: Tomoki Todo

Hiroyuki Mano (*National Cancer Center*)

Cancer genomic medicine brought by molecularly targeted drugs

Special Symposium (E)

Date: July 22, 2019, 13:30-15:00, Yasuda Auditorium

Chairpersons: Hiroshi Fukuhara & Masatoshi Tagawa

SS-1. Robert L. Martuza (*Massachusetts General Hospital and Harvard Medical School*)

Synergistic cancer therapies utilizing genetically-engineered oncolytic herpes virus

SS-2. Chae-Ok Yun (*Hanyang University*)

Drug-conjugated polymeric micelle in combination with oncolytic adenovirus leads to synergistic antitumor effect

SS-3. Stephen J. Russell (*Department of Molecular Medicine, Mayo Clinic*)

Intravenous virotherapy: Inflaming tumors via the blood stream

Symposium I (E)
-Gene Therapy for Neurological Diseases-

Date: July 21, 2019, 13:30-15:10, Yasuda Auditorium
Chairpersons: Hideki Mochizuki & Shin-ichi Muramatsu

- S1-1. Satoru Tada (*Osaka University Graduate School of Medicine*)
βAPP processing drives gradual Tau pathology in an age-dependent amyloid rat model of Alzheimer's disease
- S1-2. Hitoshi Okazawa (*Tokyo Medical and Dental University*)
Gene therapy for spinocerebellar ataxia
- S1-3. Petra Kaufmann (*AveXis, Inc.*)
Recent advancements in gene therapy with a focus in amyotrophic lateral sclerosis and Rett Syndrome
- S1-4. Edwin J. Weeber (*PTC Therapeutics*)
Gene therapy approaches for the treatment of Angelman syndrome

Symposium II (E)
-Genetic Diseases-

Date: July 21, 2019, 15:10-16:50, Yasuda Auditorium
Chairpersons: Torayuki Okuyama & Toya Ohashi

- S2-1. Torayuki Okuyama (*National Center for Child Health and Development*)
Current status of gene therapy for monogenic disorders
- S2-2. Yuko Nitahara-Kasahara (*Nippon Medical School*)
Anti-inflammatory therapy using multipotent mesenchymal stromal cells for muscular dystrophy
- S2-3. Hiroshi Kobayashi (*The Jikei University School of Medicine*)
Gene therapy for inherited error of metabolism
- S2-4. Toru Uchiyama (*National Center for Child Health and Development*)
Progress of gene therapy for primary immunodeficiency
- S2-5. Hitoshi Osaka (*Jichi Medical University*)
Gene therapy of genetic neurological disorders

Symposium III (J)
-Regulatory Science-

Date: July 22, 2019, 09:00-10:40, Yasuda Auditorium
Chairpersons: Masafumi Onodera & Kenzaburo Tani

- S3-1. Masafumi Onodera (*National Center for Child Health and Development*)
Regulations of gene and cell therapy in Japan
- S3-2. Teruhide Yamaguchi (*Kanazawa Institute of Technology/ Nihon Pharmaceutical University*)
Current status of gene therapy in Japan - Regulatory issues
- S3-3. Megumi Kawamoto (*Pharmaceuticals and Medical Devices Agency*)
Points to consider in quality and preclinical aspects of gene therapy products
- S3-4. Yoji Sato (*National Institute of Health Sciences*)
The minimum consensus package for securing the quality and safety of human cell-based therapeutic products

Symposium IV (E)
-JCA & JSGCT joint Symposium-

*JCA: Japanese Cancer Association
Date: July 22, 2019, 09:00-10:40, Koshiba Hall
Chairpersons: Yasufumi Kaneda & Masanori Hatakeyama

- S4-1. Seiji Yano (*Kanazawa University*)
Mechanism of targeted drug resistance and its circumvention in lung cancer
- S4-2. Hiroyuki Seimiya (*The Cancer Chemotherapy Center of JFCR*)
Tankyrase and G-quadruplex as molecular targets for cancer therapy
- S4-3. Hiroshi Tazawa (*Okayama University Hospital*)
Development of diagnosis and treatment based on molecular pathogenesis of gastrointestinal cancer
- S4-4. Keisuke Nimura (*Osaka University School of Medicine*)
Elucidating a mechanism for RNA splicing driving human prostate cancer progression

Symposium V (E)
-Cardiovascular Diseases & Regenerative Medicine-

Date: July 23, 2019, 09:00-10:50, Yasuda Auditorium
Chairpersons: Katsuto Tamai & Yoshikazu Yonemitsu

- S5-1. Hironori Nakagami (*Osaka University Graduate School of Medicine*)
Physician-initiated clinical study of limb ulcers treated with a novel functional peptide, SR-0379
- S5-2. Katsuto Tamai (*Osaka University Graduate School of Medicine*)
HMGB increases ectoderm-derived mesenchymal stem/progenitor cells in the blood for necrotic tissue repair
- S5-3. Takumi Era (*Kumamoto University*)
Derivation, induction and gene manipulation in mesenchymal stem cell
- S5-4. Koichi Nakayama (*Saga University*)
Scaffold-free Bio-3D printing for solid organ fabrication
- S5-5. Yoshikazu Yonemitsu (*Kyushu University Graduate School of Pharmaceutical Sciences*)
A RNA gene drug DVC1-0101 based on recombinant Sendai virus to treat peripheral arterial disease

Symposium VI (E)
-Vector Technology Innovation towards Clinical Gene Therapy-

Date: July 23, 2019, 09:00-10:50, Sanjo Conference Hall
Chairpersons: Hiroyuki Mizuguchi & Takashi Okada

- S6-1. Susumu Uchiyama (*Graduate School of Engineering, Osaka University*)
Chemistry, manufacturing and control of biopharmaceuticals
- S6-2. Hirohiko Hohjoh (*National Institute of Neuroscience, NCNP*)
Allele expression vectors for assessment of disease-causing gene specific RNAi
- S6-3. Fuminori Sakurai (*Graduate School of Pharmaceutical Sciences, Osaka University*)
Roles of virus-associated RNAs in adenovirus infection and their applications for development of novel oncolytic adenoviruses
- S6-4. Yumiko Komatsu (*Kyoto University*)
RNA virus-based episomal vector system and its applications in gene and cell therapy

Symposium VII (E)

-Cancer-

Date: July 23, 2019, 12:30-14:20, Yasuda Auditorium
Chairpersons: Yasutomo Nasu & Masatoshi Tagawa

S7-1. Kah-Whye Peng (*Mayo Clinic*)

Theranostics guided oncolytic virotherapy

S7-2. Satoshi Serada (*Kochi University*)

Development of SOCS gene therapy for intractable cancers

S7-3. Kazuhiko Kurozumi (*Okayama University Graduate School of Medicine, Dentistry and Pharmaceutical Sciences*)

A phase I/II clinical trial of Ad-SGE-REIC for recurrent malignant glioma

S7-4. John Wood (*Tocagen Inc.*)

Toca 511 and Toca-FC was associated with long term survival and a potential immune cell signatures in a phase 1 study subset of recurrent High Grade Glioma patients.

S7-5. Kazunori Aoki (*National Cancer Center Research Institute*)

Clinical development of oncolytic adenoviruses targeting advanced pancreatic carcinoma

Symposium VIII (E)

-Genome Editing-

Date: July 23, 2019, 12:30-14:20, Sanjo Conference Hall
Chairpersons: Yumi Kanegae & Mahito Nakanishi

S8-1. Osamu Nureki (*The University of Tokyo*)

Structural basis for molecular mechanism of Cas9 and development of PAM-flex Cas9 variants

S8-2. Keiji Nishida (*Engineering Biology Research center, Kobe University*)

Potential of base editing technology for gene therapy

S8-3. Knut Woltjen (*Center for iPS Cell Research and Application, Kyoto University*)

Precision gene editing in human pluripotent stem cells

S8-4. Mahito Nakanishi (*AIST, TOKIWA-Bio, Inc.*)

Application of Stealth RNA vector to genome editing

10th Takara Bio Award Lecture (E)

Date: July 21, 2019, 17:05-17:20, Yasuda Auditorium
Chairperson: Tomoki Todo

Koji M. Nishiguchi (*Tohoku University Graduate School of Medicine*)

Retained plasticity and substantial recovery of rod-mediated visual acuity at the visual cortex in blind adult mice with retinal dystrophy

Plenary Session (E) (Abstracts PS-01~PS-04)

Date: July 21, 2019, 11:00-12:00, Yasuda Auditorium
Chairpersons: Toshiyoshii Fujiwara, & Takashi Shimada

PS-01. Extracellular vesicles involve in the abscopal effect induced by telomerase-specific oncolytic adenoviruses

Kakiuchi Y., Kuroda S., Kanaya N., Kumon K., Tsumura T., Kikuchi S., Nishizaki M., Kagawa S., Tazawa H., Urata Y., Fujiwara T.

PS-02. Induction of anti-tumor immunity by synthetic stem-loop RNA (sl-RNA) derived from Sendai virus RNA genome combined with a pyro drive jet injector

Nishikawa T., Suzuki A., Miki K., Yamashita K., Kaneda Y.

PS-03. Three-year efficacy and safety results from a Phase 1/2 clinical study for severe hemophilia A: Factor VIII activity levels following gene transfer with valoctocogene roxaparvovec

Fuchs H., Pasi J., Mitchell N., Lester W., Laffan M., Madan B., Rangarajan S., Symington E., Kim B., Yang X., Pierce G. F., Wong W. Y.

PS-04. A transcription-dependent approach to directed evolution of the AAV capsid identifies novel liver detargeted variants capable of enhanced neuronal transduction in mouse and non-human primate

Huang S. J., Adachi K., Baggett H. R., Song Z., Dissen G. A., Ojeda S. R., Nakai H.

Day 1: July 21, 2019

Oral Session I

Genetic Diseases (Abstracts OR-05~OR-09)

13:30~14:20, Sanjo Conference Hall
Chairpersons: Akihiro Kume & Toru Uchiyama

OR-05. Phenotypical correction of X-SCID mice after CRISPR/Cas9-mediated genome-editing therapy of hematopoietic stem cells

Byambaa S., Uosaki H., Hara H., Shibata H., Abe T., Nagao Y., Nureki O., Ohmori T., Hanazono Y.

OR-06. Establishment of a novel lentiviral gene transfer platform for the cure of hemoglobinopathies; Higher, safer, and cheaper

Ikawa Y., Breda L., Tanaka N., Ghiaccio V., Rivella S.

OR-07. Amelioration of ubiquitin-proteasome system and autophagy dysfunction by AAV-mediated gene therapy improved neuropathology and extended life span in murine model of Globoid Cell leukodystrophy

Lin D. S., Ho C. S., Chiang M. F.

OR-08. Development of the gene therapy by utilizing modified β -Hexosaminidase B for GM2 gangliosidosis

Ohnishi Y., Tsuji D., Kamori N., Muramatsu S., Itoh K.

OR-09. Efficient engraftment of genetically modified cells should be necessary to ameliorate CNS involvement of murine model of mucopolysaccharidosis type II by hematopoietic stem cell targeted gene therapy

Miwa S., Shimada Y., Higuchi T., Kobayashi H., Fukuda T., Watabe A. M., Ida H., Ohashi T.

Oral Session II
Cancer 1 (Abstracts OR-10~OR-14)

13:30~14:20, Koshiba Hall

Chairpersons: Kazunori Aoki & Toshiro Shirakawa

OR-10. Suicide gene cell therapy for malignant glioma using genome-edited human induced pluripotent stem cells

Toda M., Tamura R., Miyoshi H., Morimoto Y., Sampetean O., Saya H., Okano H., Yoshida K.

OR-11. An inhibitor for the lipid synthetic pathway increased oncolytic adenoviral replications in mesothelioma

Morinaga T., Kubo S., Shingyoji M., Tada Y., Shimada H., Hiroshima K., Tagawa M.

OR-12. Vasculostatin-armed oncolytic herpes virus decreased bevacizumab-induced glioma invasion by regulating CCN1 and AKT signaling pathway

Tomita Y., Kurozumi K., Fujii K., Ichikawa T., Matsumoto Y., Uneda A., Hattori Y., Shimizu T., Otani Y., Balveen K., Date I.

OR-13. PD-L1 blockade enhanced the therapeutic efficacy of the oncolytic virus-cancerpaturev in squamous carcinoma

Eissa I., Mukoyama N., Naoe Y., Matsumura S., Ichinose T., Bustos-Villalobos I., Morimoto T., Miyajima N., Tanaka M., Kasuya H.

OR-14. DNA immunization of tumor-associated antigen L6 cancer vaccines elicit anti-tumor immunity for cancer treatment

Liu S. J., Sher Y. P., Lin S. I., Cai K. M., Chen I. H.

Oral Session III
Vector 1 (Abstracts OR-15~OR-18)

14:20~15:00, Sanjo Conference Hall

Chairpersons: Yukihiko Hirai & Ken-ichiro Kosai

OR-15. Identification of novel AAV1 / AAV9 chimeric mutants exhibiting enhanced gene delivery in the central nervous system

Takahama K., Kimura M., Adachi K., Dissen G. A., Nakai H.

OR-16. In vivo imaging of transgene expression from a non-toxic herpes simplex virus vector

Miyagawa Y., Maruyama M., Kuroda S., Sakai A., Sato Y., Kinoh H., Yamamoto M., Cohen J. B., Glorioso J. C., Okada T.

OR-17. Development of microRNA-regulated oncolytic coxsackievirus for clinical trial.

Miyamoto S., Sagara M., Yang J., Soda Y., Miura Y., Shimizu H., Tani K.

OR-18. In vivo quantitative analysis of oncolytic virus-tumor kinetics

Ito H., Nock M., McLaughlin E., Fernandez S., Chiocca E. A., Nakashima H.

Oral Session IV
Cancer 2 (Abstract OR-19-OR-22)

14:20~15:00, Koshiba Hall

Chairpersons: Makoto Abei & Hideaki Tahara

OR-19. Development of human mesenchymal stem cells as tumor-homing cellular carriers for retroviral replicating vectors

Kubo S., Fujino H., Morinaga T., Tagawa M., Kasahara N.

OR-20. Intravenous application of Mesothelin-targeted oncolytic adenovirus in pancreatic cancer

Sato-Dahlman M., Miura Y., Hajeri P., Yoshida H., Jacobsen K., Yanagiba C., Yamamoto M.

OR-21. Anti-tumor and anti-metastatic effect of CD133-targeted oncolytic adenovirus in colorectal cancer.

Sato-Dahlman M., Huang J. L., Jacobsen K., Yamamoto M.

OR-22. Enhanced antitumor efficacy of midkine promoter-regulated oncolytic HSV-1 in slow-growing human liposarcoma

Taguchi S., Fukuhara H., Takeshima Y., Kakutani S., Naito A., Kume H., Todo T.

Oral Session V

Vector 2 (Abstracts OR-23~OR-26)

15:00~15:40, Sanjo Conference Hall

Chairpersons: Hiroyuki Nakai & Masashi Urabe

OR-23. Impaired production of an AAV VP1 unique region (VP1u) mutant provides new insight into the AAV vector biology

Adachi K., Nakai H.

OR-24. Identification of VP-interacting cellular proteins in AAV capsid assembly by proximity proteomics

Kollu S., Sairavi A., Song Z., Nakai H.

OR-25. AAV amplicon vectors with minimal virus-derived genetic material

Shiozawa Y., Adachi K., Tomono T., Miyagawa Y., Okada T.

OR-26. Viral vector platform development guidance based on process cost modeling

Shi M., Bohonak D., Patil R.

Oral Session VI

Cancer 3 (Abstracts OR-27~OR-30)

15:00~15:40, Koshiba Hall

Chairpersons: Hiroshi Tazawa & Eriko Uchida

OR-27. Triple combination immunotherapy using oncolytic herpes simplex virus type 1 (G47Δ), immune checkpoint inhibitor and focal adhesion kinase inhibitor is highly efficacious for pancreatic ductal adenocarcinoma

Yamada T., Iwai M., Tateishi R., Ijichi H., Sano M., Koike K., Todo T.

OR-28. MicroRNA regulation in malignant glioma can augment antitumor efficacy of oncolytic HSV-1 (G47Δ)

Kanayama S., Tanaka M., Iwai M., Chalise L., Todo T.

OR-29. Tumor suppressor p53-armed oncolytic adenovirus induces profound immunogenic cell death in neuroblastoma cells

Tani M., Tazawa H., Tanimoto T., Nouse H., Urata Y., Kagawa S., Noda T., Fujiwara T.

OR-30. A novel intraperitoneal theranostic virotherapy with Paclitaxel for peritoneal metastasis of gastric cancer

Ogawa T., Kikuchi S., Ishikawa W., Tazawa H., Tabuchi M., Kuroda S., Noma K., Nishizaki M., Kagawa S., Urata Y., Fujiwara T.

Oral Session VII
Vector 3 (Abstracts OR-31~OR-34)

15:40~16:20, Sanjo Conference Hall
Chairpersons: Junichi Mineno & Fuminori Sakurai

OR-31. Generation of novel cardiac specific AAV vectors by directed evolution in human iPSC derived cardiomyocytes

Kok C., Skelton R., Chong J., Alexander I., Lisowski L., Kizana E.

OR-32. Therapeutic efficacy of IL-12 expressing oncolytic HSV-1 with triple mutations for neck lymph node metastases in mouse tongue cancer models

Kurioka K., Uchihashi T., Sugauchi A., Iwai M., Kogo M., Todo T.

OR-33. Development of hydrodynamic gene therapy -Assessment of liver-targeted hydrodynamic gene delivery in large animal disease model

Kamimura K., Yokoo T., Kanefuji T., Suda T., Zhang G., Nichols T. C., Merricks E. P., Aoyagi Y., Terai S., Liu D.

OR-34. Methodologies for minimization of process- and product-related impurities in AAV gene therapy manufacturing

Baffi R., Calderaro C., Fouts E., Gold D., Sluzky V., Tenerowicz R.

Oral Session VIII
Cancer 4 (Abstracts OR-35~OR-38)

15:40~16:20, Koshiba Hall
Chairpersons: Akinobu Gotoh & Yozo Nakazawa

OR-35. Enhanced tumor accumulation of PEGylated liposomes by pretreatment with oncolytic reovirus in tumor-bearing mice

Sakurai F., Hirata S., Kurisu N., Ogawara K., Mizuguchi H.

OR-36. Augmented p53 expression induces nuclear factor-I and increases adenovirus replications in p53 wild-type mesothelioma

Tagawa M., Nguyễn T. T. T., Morinaga T., Hanazono M., Shingyoji M., Kubo S., Tada Y., Shimada H., Hiroshima K.

OR-37. Elimination of chemoresistant pancreatic ductal adenocarcinoma cells by telomerase-specific oncolytic virotherapy

Fushimi T., Tazawa H., Araki H., Nishiyama T., Kikuchi S., Kuroda S., Noma K., Yoshida R., Urata Y., Kagawa S., Fujiwara T.

OR-38. HSV-TK suicide gene therapy for glioblastoma using neural stem/progenitor cells derived from human induced pluripotent stem cells

Miyoshi H., Tamura R., Morimoto Y., Sampetean O., Iwasawa C., Mine Y., Narita M., Saya H., Yoshida K., Okano H., Toda M.

Oral Session IX
Neurologic Diseases (Abstracts OR-39~OR-41)

16:20~16:50, Sanjo Conference Hall
Chairpersons: Yasuhiro Ikeda & Shin'ichi Takeda

OR-39. The development of ErbB2-targeted gene therapy for Alzheimer's disease

Liao Y. F., Wang B. J., Chen Y. W., Wu P. Y.

OR-40. Improved gene transduction in non-human primate muscle with recombinant AAV following immunomodulation using dental pulp stem cells

Ishii A., Hayashita-Kinoh H., Guillermo P. H., Okada H., Takeda S., Okada T.

OR-41. Modulation of neurodegeneration by peripheral immune system in Niemann-Pick disease type C

Yasuda T., Watanabe N., Uchiyama T., Mochizuki H., Onodera M.

Day 2: July 22, 2019

Oral Session X

Basic Study 1 (Abstracts OR-42~OR-45)

09:00~09:40, Sanjo Conference Hall

Chairpersons: Yutaka Hanazono & Masato Yamamoto

OR-42. All-in-one adenovirus vectors expressing both highly multiplex double-nicking guide RNAs and Cas9 nickase for genome editing therapy

Nakanishi T., Tabata H., Nakamura M., Fuse R., Saito I.

OR-43. Development of an oligonucleotide delivery system based on AAV empty capsids.

Guillermo P. H., Masuda C., Hayashita-Kinoh H., Miyagawa Y., Okada T.

OR-44. Direct induction of naive-like human induced pluripotent stem cells (iPSCs) by a non-integrating measles virus vector

Liao J., Soda Y., Sugawara A., Miura Y., Hiramoto T., Tahara M., Takishima Y., Hijikata Y., Miyamoto S., Takeda M., Tani K.

OR-45. Recombinant fibronectin fragment, FCH296, is a substrate for the field of regenerative medicine using human ES/iPS cells

Otsuji T. G., Kato R., Tosaka Y., Okamoto S., Enoki T., Mineno J.

Oral Session XI

Basic Study 2 (Abstracts OR-46~OR-49)

09:40~10:20, Sanjo Conference Hall

Chairpersons: Koichi Miyake & Hiroaki Mizukami

OR-46. AdoptCell-NK: an upward compatible modality over CAR-T therapy against solid tumor

Harada Y., Yonemitsu Y.

OR-47. Dendritic cells loaded with survivin-FLIPr fusion protein provides antitumor immunity in mice

Chen H. W., Chiang C. Y., Shen K. Y., Chen Y. J., Wu C. C., Liu S. J.

OR-48. Apatite nanoparticle-encapsulated siRNA targeting tissue inhibitor of metalloprotease-1 for the treatment of hypertrophic scars and keloids

Aoki M., Matsumoto N., Okubo Y., Ogawa R., Yamamoto H., Takabe K., Okada T.

OR-49. Local administration of GITR agonistic antibody induces a stronger antitumor immunity than systemic delivery

Aoki K., Narumi K., Miyakawa R., Shibasaki C., Henmi M., Mizoguchi Y., Hiraoka N.

Oral Session XII
Cardiovascular Diseases (Abstracts OR-50~OR-53)

15:10~15:50, Sanjo Conference Hall
Chairpersons: Yui Harada & Hironori Nakagami

OR-50. Long term observation of alveolar bone regeneration by non-viral BMP gene transfer
Kawai M., Ohura K.

OR-51. Protection of cardiomyocytes against doxorubicin induced toxicity by transporter mediated efflux
Kok C., Skelton R., Rao R., Igoor S., Chong J., Kizana E.

OR-52. Serum levels of brain-derived neurotrophic factor in glaucoma patients
Igarashi T., Nakamoto K., Kobayashi M., Tobita Y., Takao K., Takahashi H., Okada T.

OR-53. Advanced therapy medicinal products (ATMPs) – Considerations for biosafety testing and product characterization
Ang E.

Oral Session XIII
Cancer 5 (Abstracts OR-54~OR-57)

15:50~16:30, Sanjo Conference Hall
Chairpersons: Hiroaki Ikeda & Yoshinobu Manome

OR-54. The use of a third generation oncolytic HSV-1 (G47delta) enhances the therapeutic efficacy of CTLA-4 blockade in a murine esophageal squamous cell carcinoma model.
Sugawara K., Iwai M., Tanaka M., Seto Y., Todo T.

OR-55. Pre-immunization of donor lymphocytes with G1TR agonistic antibody enhances antitumor immunity in autologous hematopoietic stem cell transplantation
Aoki K., Henmi M., Narumi K., Mizoguchi Y.

OR-56. Antitumor effect of intratumoral IL-7 and IL-12 expression by oncolytic vaccinia virus in poorly immunogenic murine lung cancer
Nakao S., Arai Y., Yamashita M., Tasaki M., Amino N., Kawase T., Nakatake M., Kurosaki H., Mori M., Takeuchi M., Nakamura T.

OR-57. Overexpression of SOCS3 mediated by adenovirus vector inhibited cell proliferation and increased chemosensitivity in triple-negative breast cancer cells in vitro and in vivo.
Narikiyo K., Kunimura N., Kitagawa K., Ishiko M., Kadowaki M., Sako R., Fujisawa M., Serada S., Naka T., Shirakawa T.

Oral Session XIV
Cancer 6 (Abstracts OR-58~OR-61)

16:30~17:10, Sanjo Conference Hall
Chairpersons: Toshihiko Okazaki & Toshihiko Wakabayashi

OR-58. LncRNA UCA1 is a potential biomarker of response to oncolytic vaccinia virotherapy and chemotherapy for ovarian cancer
Kosuke H., Kurosaki H., Nakatake M., Kuwano N., Ishii K., Kohno H., Ito M., Itamochi H., Oishi T., Harada T., Nakamura T.

OR-59. Podoplanin targeting chimeric antigen receptor T (CAR-T) cells based on cancer specific monoclonal antibody show tumor-specific cytotoxicity against glioblastoma.
Chalise L., Maeda S., Wakabayashi T., Natsume A., Todo T.

OR-60. Nicotine and stem cell's property for enzyme/prodrug-based therapy against glioma
Kenmochi H., Yamasaki T., Namba H.

OR-61. Preclinical safety studies of an oncolytic herpes simplex virus type I armed with interleukin-12
Iwai M., Fukuhara H., Tanaka M., Todo T.

□Best Poster ○Excellent Poster

Poster Session

Venue: Corridor of Yasuda Auditorium

Poster Session I

Basic Science (Abstracts: PO-62~PO-65)

Date: July 22, 2019, 17:20-17:40

Chairperson: Yuko Kasahara

PO-62. Molecular mechanisms of resistance to OxPhos inhibition in AML

Yang H., Saitoh K., Imoto J., Ikeo K., Miida T., Tabe Y.

PO-63. Gene therapy using modified adenovirus vector-mediated lysophosphatidylcholine acyltransferase overexpression for diabetes mellitus in a mouse model

Shimizu K., Ono M., Mikamoto T., Urayama Y., Nakanishi H., Terada T., Sakurai F., Mizuguchi H., Tomita K., Nishinaka T.

PO-64. Development of viral vector-producing amniotic mesenchymal stem cells for in situ cancer cell therapy

Yamazaki Y., Nitahara-Kasahara Y., Miyazaki K., Miyagawa Y., Okada T.

PO-65. Scalable stirred suspension culture for GMP/GCTP manufacturing of human induced pluripotent stem cells

Kohara J., Sakamoto M., Tahara K., Nukaya I., Mineno J.

Poster Session II

Neurologic Diseases (Abstracts: PO-66~PO-68)

Date: July 22, 2019, 17:20-17:35

Chairperson: Takanori Yamagata

PO-66. Pre-stroke delivery of anti-apoptotic factor survivin using adeno-associated virus rescued the prognosis after transient middle cerebral artery occlusion in rats

Sehara Y., Inaba T., Urabe T., Shimazaki K., Kawai K., Urabe M., Mizukami H.

PO-67. Determination of anti-adeno associated virus vector neutralizing antibody titers with a modified cell-based assay

Hayashita-Kinoh H., Nitahara-Kasahara Y., Kuraoka M., Guillermo P. H., Okada H., Ishii A., Takeda S., Okada T.

PO-68. Transplantation of human dental pulp stem cells attenuates brain injury after transient middle cerebral artery occlusion

Nito C., Sowa K., Sakamoto Y., Takahashi S., Arakawa M., Suda S., Nishiyama Y., Nakamura-Takahashi A., Nitahara-Kasahara Y., Kimura K., Okada T.

Poster Session III

Vector 1 (Abstracts: PO-69~PO-71)

Date: July 22, 2019, 17:20-17:35

Chairperson: Kenya Kamimura

PO-69. Oncolytic potential of an E4-deficient adenovirus that can recognize the stabilization of AU-rich element containing mRNA in cancer cells

Higashino F., Yanagawa-Matsuda A., Towfik-Alam M., Kitamura T.

PO-70. Enhancement of transduction efficiency of bornaviral vector by the modification of viral envelope glycoprotein

Sakai M., Komorizono R., Komastu Y., Makino A., Tomonaga K.

PO-71. Comparative stability analysis of recombinant AAV and wild-type AAV under various conditions to consider environmental effect assessment

Tomono T., Hirai Y., Chono H., Mineno J., Ishii A., Tamaoka A., Onodera M., Okada T.

**Poster Session IV
Vector 2 (Abstracts: PO-72~PO-74)**

Date: July 22, 2019, 17:20-17:35

Chairperson: Kei Adachi

PO-72. Production of neutralizing antibodies by systemically injected AAV-PHP.B completely blocks brain transduction by a second intravenous administration of AAV-PHP.B.

Konno A., Hirai H.

PO-73. Therapeutic activity of prodrug activator gene therapy with Toca 511 & 5-FC for lung cancer

Kushiya H., Hiraoka K., Suzuki T., Inoko K., Inagaki A., Nakamura T., Tsuchikawa T., Shichinohe T., Douglas J. J., Kasahara N., Hirano S.

PO-74. Analytical ultracentrifugation assays to characterize the purity of recombinant adeno-associated viral type 9 vectors (rAAV9)

Hirai Y., Tomono T., Miyagawa Y., Ishii A., Tamaoka A., Onodera M., Okada T.

**Poster Session V
Genetic Diseases (Abstracts: PO-75~PO-78)**

Date: July 22, 2019, 17:20-17:40

Chairperson: Hiroshi Kobayashi

PO-75. Treatment of peripheral neuropathy by intrathecal administration of AAV vector expressing α -Galactosidase A in murine Fabry model

Higuchi T., Shimada Y., Kobayashi H., Kato F., Ida H., Ohashi T.

PO-76. Treatment for murine mucopolysaccharidosis type ii model by ex vivo hematopoietic stem cells gene therapy with lentivirus vector

Higuchi T., Shimada Y., Kanegae Y., Fukuda T., Watabe A., Kobayashi H., Kato F., Ida H., Ohashi T.

PO-77. Gene therapy for a mouse model of Niemann-Pick disease type C

Kurokawa Y., Kouga T., Jimbo E., Yamagata T., Takayanagi Y., Onaka T., Muramatsu S., Osaka H.

PO-78. Suppression of in cellulo crystallization of NEU1 and application for gene therapy for NEU1 deficiency

Tsukimoto J., Horii Y., Nishioka S., Itoh K.

**Poster Session VI
Cancer 1 (Abstracts: PO-79~PO-81)**

Date: July 22, 2019, 17:20-17:35

Chairperson: Yasushi Soda

PO-79. Lentiviral vector mediated transfection of tumor suppressor genes.

Matsunaga W., Ichikawa M., Isibasi T., Gotoh A.

PO-80. MYOG promoter-controlled oncolytic adenovirus, AdMG-MYOGp(S)-E1-5/3F, kills rhabdomyosarcoma cells selectively

Yoshida H., Sato-Dahlman M., Hajeri P., Jacobsen K., Yamamoto M.

PO-81. A novel combination therapy using oxaliplatin and Coxsackievirus A11 against human colorectal cancer.

Ogata H., Wang B., Miyamoto S., Takishima Y., Sagara M., Tani K.

**Poster Session VII
Cancer 2 (Abstracts: PO-82~PO-85)**

Date: July 22, 2019, 17:20-17:40

Chairperson: Hideki Kasuya

PO-82. Anti-PD-1 antibody enhances the efficacy of oncolytic virus therapy in a mouse lung cancer model

Sakata Y., Iwai M., Ikeda N., Todo T.

PO-83. Combination therapy of cetuximab and CANERPATUREV suppresses human colorectal cancer growth

Matsumura S., Wu Z., Naoe Y., Eissa I., Tanaka M., Kasuya H.

PO-84. Combined effect of Lentiviral vector mediated gene transfer and cisplatin

Ichikawa M., Matsunaga W., Ishikawa T., Gotoh A.

○ **PO-85. Oncolytic virus therapy for renal cell carcinoma using herpes simplex virus type 1 armed with soluble ALK1**

Naito A., Fukuhara H., Uchihashi T., Iwai M., Watabe T., Miyazono K., Kume H., Todo T.

**Poster Session VIII
Cancer 3 (Abstracts: PO-86~PO-88)**

Date: July 22, 2019, 17:20-17:35

Chairperson: Yasunori Amaishi

PO-86. Preclinical evaluation of novel chondroitin sulfate polymer coated oncolytic measles virus therapy

Miura Y., Takishima Y., Miyamoto S., Yang J., Sagara M., Soda Y., Tani K.

PO-87. Bacteriophage-mediated systemic gene therapy of diffuse intrinsic pontine glioma through the blood-brain barrier

Yan W., Albahrani M., Carcaboso A., Suwan K., Hajitou A.

PO-88. Design of chimeric antigen receptor affect the characters of CAR-T cells

Amaishi Y., Okamoto S., Enoki T., Shiku H., Mineno J.

**Corporate Seminars
Evening Seminars**

►Day I

**Corporate Seminar-I
(Daiichi Sankyo Co., Ltd.)**

Date: July 21, 2019, 12:00-12:50, Yasuda Auditorium
Chairperson: Ryuichi Morishita
(Center of Medical Innovation and Translational Research School of Medicine, Osaka University)

Yuichi Oike (*Graduate School of Medical Sciences, Kumamoto University*)
Current status of pharmacotherapy for heart failure and potential new therapies

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**Corporate Seminar-II
(Kiko Tech Co., Ltd.)**

Date: July 21, 2019, 12:00-12:50, Sanjo Conference Hall
Chairperson: Makoto Otsu
(Kitasato University School of Medicine)

Shigeki Yagyu (*Kyoto Prefectural University of Medicine, Graduate School of Medical Science*)
Development of piggyBac transposon based CAR-T cell therapy - Driving CARs to the clinic for solid tumors

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**Corporate Seminar-III
(Novartis Pharma K. K.)**

Date: July 21, 2019, 12:00-12:50, Koshiba Hall
Chairperson: Hitoshi Osaka
(Jichi Medical University, Department of Pediatrics)

Petra Kaufmann (*AveXis, Inc.*)
The gene therapy for neurological diseases

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**Corporate Seminar-IV
(GE Healthcare Japan Corp.)**

Date: July 21, 2019, 12:00-12:50, Ito Hall
Chairperson: Joe Hirano
(Genomics & Cellular Research, GE Healthcare Life Sciences)

Kentaro Toriumi (*Cell & Gene Therapy Application, Life Sciences, GE Healthcare Japan Corp.*)
Future path to co-create scalable, cost-effective and safe viral vector development and production

**Evening Seminar I
(Nihon Pall Ltd.)**

Date: July 21, 2019, 18:20-19:00, Sanjo Conference Hall
Chairperson: Naohito Hariganeya
(Nihon Pall Ltd.)

Mark S. Szczypka (*Global Director, Process Development Services, Pall Biotech*)
Industrialization of gene therapy products using Pall's iCELLis Single-Use Bioreactor

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**Evening Seminar II
(Marken Japan Ltd.)**

Date: July 21, 2019, 18:20-19:00, Koshiba Hall
Chairperson: Yozo Nakazawa
(Shinshu University School of Medicine)

Minoru Mihara (*Marken Ltd.*)
Advanced therapy medical products end to end logistic

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►Day II

**Corporate Seminar-V
(AnGes, Inc.)**

Date: July 22, 2019, 12:00-12:50, Yasuda Auditorium
Chairperson: Yasufumi Kaneda
(Osaka University Graduate School of Medicine)

- 1. Ryuichi Morishita** (*Osaka University Graduate School of Medicine*)
Current status and future of Angiogenic gene therapy
 - 2. Hironori Nakagami** (*Osaka University Graduate School of Medicine*)
Research and development of vaccines for lifestyle diseases
-

**Corporate Seminar-VI
(Takara Bio Inc.)**

Date: July 22, 2019, 12:00-12:50, Sanjo Conference Hall
Chairperson: Junichi Mineno
(Takara Bio Inc.)

Takashi Okada (*Nippon Medical School*)
Innovation in AAV vector toward gene and cell-based therapies to treat neuromuscular diseases

**Corporate Seminar-VII
(Merck Limited Japan)**

Date: July 22, 2019, 12:00-12:50, Koshiba Hall

Chairperson: Sadao Ozawa
(Merck Limited Japan)

Patrick Barry (*BioReliance USA*)

Edmund Ang (*BioReliance Singapore*)

Optimizing manufacturing and biosafety testing timelines for your gene therapy product

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►Day III

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

Date: July 23, 2019, 11:00-11:50, Yasuda Auditorium

Chairperson: Tatsuya Kondo
(*Pharmaceuticals and Medical Devices Agency*)

Shigetaka Asano (*Professor Emeritus of the University of Tokyo &*

Professor Emeritus Guest Research Professor of Waseda University)

What and How to Proceed with Translational Research, Now?

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**Corporate Seminar-IX
(AnGes, Inc.)**

Date: July 23, 2019, 11:00-11:50, Sanjo Conference Hall

Chairperson: Yoshikatsu Eto
(*Advanced Clinical Research Center, Institute for Neurological Disorders*)

Masafumi Onodera (*National Center for Child Health and Development*)

To go with gene therapy smoothly in Japan - Problem and provision

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