

THE 23rd ANNUAL MEETING

JSGCT2017

JAPAN SOCIETY OF GENE AND CELL THERAPY

Program & Abstracts

Date

July 20-22, 2017

Venue

OKAYAMA CONVENTION CENTER

14-1 Ekimoto-machi, Kita-ku, Okayama 700-0024, Japan

OKAYAMA



PROGRAM

Presidential Lecture (E)

Date: July 21, 2017, 11:30-12:00, Hall-A
Chairperson: Shigetaka Asano

Yasutomo Nasu (*Okayama University Graduate School of Medicine, Dentistry and Pharmaceutical Sciences*)
Role of academia for the development of gene therapy -Experience of prostate cancer for 20 years-

JSGCT Chairman's Lecture (E)

Date: July 21, 2017, 13:00-13:30, Hall-A
Chairperson: Yoshikatsu Eto

Yasufumi Kaneda (*Graduate School of Medicine, Osaka University, Chairman of JSGCT*)
Current status and future prospect of gene therapy in Japan

Special Lecture I (E)

Date: July 21, 2017, 11:00-11:30, Hall-A
Chairperson: Ryuichi Morishita

Hidemasa Oh (*Okayama University Hospital*)
Cell therapy trials in congenital heart disease

Special Lecture II (E)

Date: July 22, 2017, 11:00-11:30, Hall-A
Chairperson: Noriyuki Kasahara

Hiroshi Fukuhara (*Graduate School of Medicine, The University of Tokyo*)
A phase 1 study of an oncolytic HSV-1 G47Δ in patients with castration resistant prostate cancer

Special Lecture III (E)

Date: July 22, 2017, 11:30-12:00, Hall-A
Chairperson: Kohnosuke Mitani

Takashi Yamamoto (*Graduate School of Science, Hiroshima University*)
Advances in genome editing technology and its applications in biomedical fields

Special Symposium (E)

Date: July 21, 2017, 9:50-11:00, Hall-A
Chairpersons: Yasutomo Nasu & Masafumi Onodera

SS-1. Helen E. Heslop (*Baylor College of Medicine, ASGCT President*)
Adoptive transfer of virus specific T cells

SS-2. Hildegard Büning (*Institute of Experimental Hematology, Hannover Medical School, ESGCT President*)
Tailoring the Adeno-Associated Viral (AAV) vector system for gene therapy

Symposium I (E)

-Next Generation Cancer Immunotherapy using gene-modified T cells-

Date: July 20, 2017, 14:00-16:00, Hall-A
Chairpersons: Keiya Ozawa & Koji Tamada

S1-1. Keiya Ozawa (*The Institute of Medical Science, The University of Tokyo*)
Introduction: CD19-CAR-T gene therapy and beyond

S1-2. Ryosuke Uchibori (*Jichi Medical University*)
CAR-T therapy for multiple myeloma

S1-3. Yozo Nakazawa (*Shinshu University School of Medicine*)
Non-virally engineered CAR T cells for CD116-positive myeloid neoplasms

S1-4. Koji Tamada (*Yamaguchi University Graduate School of Medicine*)
Novel strategies of CAR-T cell therapy for solid tumors

S1-5. Shinichi Kageyama (*Mie University Graduate School of Medicine*)
Clinical trials of NY-ESO-1 and MAGE-A4 targeting TCR-gene transduced T cell transfer to patients with refractory solid tumors

Symposium II (E)
-Genetic Diseases Symposium-

Date: July 20, 2017, 16:10-18:10, Hall-A
Chairpersons: *Fumio Endo & Toya Ohashi*

S2-1. Torayuki Okuyama (*National Center for Child Health and Development*)
Progress in treatment and early diagnosis of inherited and metabolic diseases

S2-2. Toru Uchiyama (*National Center for Child Health and Development*)
Progress in gene therapy for primary immunodeficiencies

S2-3. Takashi Okada (*Nippon Medical School*)
AAV vector transduction strategy with immune-modulation to ameliorate muscular dystrophy

S2-4. Takanori Yamagata (*Jichi Medical University*)
Gene therapy for pediatric neurological diseases including AADC deficiency

S2-5. Hiroshi Kobayashi (*The Jikei University School of Medicine*)
Gene therapy for lysosomal storage diseases

Symposium III (J)
-Regulatory Science Symposium-

Date: July 21, 2017, 8:30-9:50, Hall-A
Chairpersons: *Akihiro Kume & Teruhide Yamaguchi*

S3-1. Eriko Uchida (*National Institute of Health Sciences*)
Update on ongoing revision of guideline for gene therapy clinical research focusing on genome editing technology

S3-2. Junichi Furuta (*Ministry of Health, Labour and Welfare*)
Revised ethical guidelines for medical research

S3-3. Yusuke Inoue (*The Institute of Medical Science, The University of Tokyo*)
The recent discussions on personal data regulation and clinical research governance

Symposium IV (J)
Regenerative Medicine - JSGCT Working Group Joint Symposium

Date: July 21, 2017, 13:30-15:30, Hall-A
Chairpersons: *Makoto Otsu & Fuminori Sakurai*

S4-1. Kaoru Mitsui (*Kagoshima University Graduate School of Medical and Dental Sciences*)
Viral vector-based innovative approaches to directly abolishing tumorigenic cells: Novel technologies for safer clinical application of pluripotent stem cell-based regenerative medicine

S4-2. Kazuo Takayama (*Graduate School of Pharmaceutical Sciences, Osaka University*)
Highly efficient biallelic genome editing of human ES/iPS cells using a CRISPR/Cas9 system

S4-3. Daisuke Morita (*Shinshu University School of Medicine*)
Chimeric antigen receptor (CAR) – T cell therapy using a non-viral gene transfer system

S4-4. Yoshimi Yashiro (*Center for iPS Cell Research and Application, Kyoto University*)
Relationship between advanced medicine and society as an example of regenerative medicine

S4-5. Keiji Itaka (*Tokyo Medical and Dental University*)
mRNA-based therapeutics for intractable diseases and regenerative medicine

Symposium V (E)

-Cancer Clinical Trial Symposium-

Date: July 21, 2017, 15:40-17:40, Hall-A

Chairpersons: Kenzaburo Tani & Tomoki Todo

S5-1. Tomoki Todo (*The Institute of Medical Science, The University of Tokyo*)
Clinical development of “Sakigake”-designated oncolytic herpes virus G47Δ

S5-2. Masatoshi Tagawa (*Chiba Cancer Center*)
Preclinical and clinical researches for malignant mesothelioma with gene medicine and drug repositioning

S5-3. Hiroshi Tazawa (*Okayama University Graduate School of Medicine, Dentistry and Pharmaceutical Sciences*)
Phase I/II study of a telomerase-specific oncolytic adenovirus OBP-301 in combination with radiotherapy in elderly esophageal cancer patients

S5-4. Ken-ichiro Kosai (*Kagoshima University Graduate School of Medical and Dental Sciences*)
The first-in-human investigator-initiated ICH-GCP clinical trial of oncolytic Surv.m-CRA towards an approval and the development of the next-generation oncolytic immunotherapies using m-CRA-based technology

S5-5. Noriyuki Kasahara (*University of Miami, USA*)
Clinical update: Long-term follow-up data from 126 patients with recurrent high-grade glioma in three phase 1 trials of Toca 511 and Toca FC, and justification for a phase 2/3

Symposium VI (E)

-Cardiovascular Diseases Symposium-

Date: July 21, 2017, 15:40-17:40, Hall-B

Chairpersons: Ryuichi Morishita & Yoshikazu Yonemitsu

S6-1. Shigeru Miyagawa (*Graduate School of Medicine, Osaka University*)
Building a new cellular treatment of severe heart failure

S6-2. Jun Fujita (*Keio University School of Medicine*)
Cardiac cell therapy using with induced pluripotent stem cells

S6-3. Yuji Shiba (*Institute for Biomedical Sciences*)
MHC-matched transplantation of iPS cell-derived cardiomyocytes regenerates non-human primate hearts

S6-4. Keishi Miyata (*Graduate School of Medical Sciences, Kumamoto University*)
ANGPTL2 : a novel therapeutic targeting gene in heart failure.

S6-5. Hironori Nakagami (*Graduate School of Medicine, Osaka University*)
Development of therapeutic vaccines for cardiovascular diseases

S6-6. Michiko Tanaka (*Graduate School of Pharmaceutical Sciences, Kyushu University*)
An update of clinical development for gene therapy with Sendai virus vector expressing the human FGF-2 gene (DVC1-0101) to treat peripheral arterial disease

Symposium VII (E)(J)
Genome Editing - JSGE Joint Symposium

Date: July 22, 2017, 8:30-10:30, Hall-A
Chairpersons: Yumi Kanegae & Takashi Yamamoto

- S7-1. **Takashi Yamamoto** (*Graduate School of Science, Hiroshima University*)
Genome editing technology and its applications in cultured cells and organisms
- S7-2. **Kohnosuke Mitani** (*Saitama Medical University*)
Somatic genome editing for gene therapy of inherited disorders
- S7-3. **Akitsu Hotta** (*Center for iPS Cell Research and Application, Kyoto University*)
Genome editing gene therapy for Duchenne muscular dystrophy
- S7-4. **Tsukasa Ohmori** (*Jichi Medical University*)
The cure of hemophilia B by CRISPR/Cas9-mediated genome editing
- S7-5. **Kaori Muto** (*The Institute of Medical Science, The University of Tokyo*)
Patient and public engagement in human genome editing

Symposium VIII (E)
-Neurologic Diseases Symposium-

Date: July 22, 2017, 13:00-15:00, Hall-A
Chairpersons: Shin-ichi Muramatsu & Takashi Okada

- S8-1. **Kinya Ishikawa** (*Tokyo Medical and Dental University*)
Research for developing fundamental therapies for spinocerebellar ataxias
- S8-2. **Yu Miyazaki** (*Graduate School of Medicine, Osaka University*)
AAV-based therapeutic delivery of miRNA in neurodegenerative diseases
- S8-3. **Yuji Yamanashi** (*The Institute of Medical Science, The University of Tokyo*)
A possible therapeutic approach aimed at enlarging neuromuscular junctions for neuromuscular disorders.
- S8-4. **Mariko Taniguchi-Ikeda** (*Kobe University Graduate School of Medicine*)
Antisense therapy for Fukuyama congenital muscular dystrophy
- S8-5. **Jodi A. Cook** (*Agilis Biotherapeutics*)
Gene therapy approaches for the treatment of Angelman syndrome

Symposium IX (E)
-Cancer Symposium-

Date: July 22, 2017, 13:00-15:00, Hall-B
Chairpersons: Masatoshi Tagawa & Toshihiko Wakabayashi

- S9-1. **Masato Yamamoto** (*University of Minnesota*)
Targeted adenoviruses at the level of infectivity: Development and application
- S9-2. **Takafumi Nakamura** (*Graduate School of Medical Sciences, Tottori University*)
Tumor-targeted and armed oncolytic vaccinia virus for systemic cancer virotherapy
- S9-3. **Kazunori Aoki** (*National Cancer Center Research Institute*)
Strong antitumor efficacy of a pancreatic tumor-targeting oncolytic adenovirus for neuroendocrine tumors

S9-4. Hideki Kasuya (*Nagoya University Graduate School of Medicine*)

Clinical trial data using HF10, oncolytic herpes simplex virus

S9-5. Toshiro Shirakawa (*Kobe University Graduate School of Science, Technology and Innovation*)

Development of combinational cancer immunotherapy comprising WT1 oral cancer vaccine and immune checkpoint inhibitor

S9-6. Yoshikazu Yonemitsu (*Graduate School of Pharmaceutical Sciences, Kyushu University*)

AdoptCell[®]-NK: a new class of highly active human natural killer cells for adoptive immunotherapy

Technical Seminar (J)

-Genome Editing, Lentiviral Vectors-

Date: July 21, 2017, 8:30-9:10, Hall-B

Chairperson: Hiroyuki Miyoshi

TS-1. Tetsushi Sakuma (*Graduate School of Science, Hiroshima University*)

Recent advances in genome editing technology

TS-2. Hiroyuki Miyoshi (*Keio University School of Medicine*)

Basics and clinical applications of lentiviral vectors

8th Takara Bio Award Lecture (E)

Date: July 20, 2017, 11:45-12:00, Hall-A

Chairperson: Yasufumi Kaneda

Takahito Sugase (*Kochi University*)

Suppressor of cytokine signaling - 1 gene therapy induces potent antitumor effect in patient-derived esophageal squamous cell carcinoma xenograft mice

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

Date: July 20, 2017, 11:00-11:45, Hall-A
Chairpersons: Hiroyuki Nakai & Takashi Shimada

PS-1. A novel single amino acid mutation to AAV9-PHP.B detargets the liver while retaining highly efficient brain neuron transduction

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

PS-2. A phase III clinical results of INVOSSA™ : A clues for the potential disease modifying OA drug

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

PS-3. Gene therapy for aromatic L-amino acid decarboxylase (AADC) deficiency: Indication for milder patients

Kojima K., Miyauchi A., Nakajima T., Saitoh S., Muramatsu S., Osaka H., Yamagata T.

Day 1: July 20, 2017

Oral Session I

Genetic Diseases-1 (Abstracts OR-4~OR-7)

14:40-15:20, Hall-B

Chairpersons: Hiroshi Kobayashi & Torayuki Okuyama

OR-4. Systemic rAAV9-microdystrophin transduction with MSCs pre-treatment of canine X-linked muscular dystrophy improves transgene expression and their DMD phenotype

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

OR-5. Dental pulp-derived cell therapy for Duchenne muscular dystrophy facilitates functional recovery

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

OR-6. Neonatal gene therapy for hemophilia B by a novel adenovirus vector showing sustained transgene expression via a reduction in the leaky expression of Ad genes

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

OR-7. rAAV8/9-mediated gene therapy for muscular dystrophy; Tacrolimus ameliorates immunoresponce in normal primates

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

Oral Session II

Genetic Diseases-2 (Abstracts OR-8~OR-11)

15:20-16:00, Hall-B

Chairpersons: Yozo Nakazawa & Makoto Otsu

OR-8. Alleviation of autophagy dysfunction and neuropathology by AAV-mediated gene therapy in Krabbe Disease

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

OR-9. Gene therapy for *Glut1*-deficient mouse using AAV vector with the human intrinsic GLUT1 promoter.

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

OR-10. Inducible KLF1 gene expression ameliorates hemoglobin switching in erythroid cells derived from Congenital dyserythropoietic anemia (CDA) patient specific iPS cells

Kohara H., Ogawa Y., Miyamoto S., Ogura H., Utsugisawa T., Kanno H., Tani K.

OR-11. Systemic delivery of HMGB1 ameliorates cutaneous and non-cutaneous manifestations in a dystrophic epidermolysis bullosa model mouse

Shimbo T., Yamazaki S., Wang X., Kikuchi Y., Bruckner-Tuderman L., Kaneda Y., Tamai K.

**Oral Session III
Cancer-1 (Abstracts OR-12~OR-15)**

16:10-16:50, Hall-B

Chairpersons: Kazunori Aoki & Hideki Kasuya

OR-12. Expanding the potential of oncolytic virus therapy by combining interleukin-12-expressing oncolytic herpes simplex virus type 1 and induced pluripotent stem cell-derived dendritic cells

Taguchi S., Fukuhara H., Cai S., Li X. K., Naito A., Kakutani S., Takeshima Y., Homma Y., Ino Y., Todo T.

OR-13. A fiber and hexon-modified infectivity-selective oncolytic adenovirus escapes liver sequestration after systemic administration in pancreatic cancer model.

Sato-Dahlman M., Miura Y., Huang J. L., Hajeri P., Yoshida H., Jacobsen K., Davydova J., Yamamoto M.

OR-14. Evaluation of manufacturing and quality control tests of T-cell receptor (TCR) gene modified cells derived from peripheral bloods of solid cancer patients in clinical trials

Inoue N., Nukaya I., Tadai Y., Tomura D., Kageyama S., Kato H., Watanabe T., Shiku H., Mineno J.

OR-15. Preclinical toxicological evaluation of tumor-targeted and armed oncolytic vaccinia virus in cynomolgus monkey

Kurosaki H., Okamura T., Nakatake M., Kuwano N., Horita K., Ishii K., Yasutomi Y., Nakamura T.

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

16:50-17:30, Hall-B

Chairpersons: Akinobu Gotoh & Yasushi Ino

OR-16. Suppressor of cytokine signaling-1 gene therapy improves radiosensitivity and enhances irradiation-induced DNA damage in esophageal squamous cell carcinoma

Sugase T., Takahashi T., Serada S., Hiramatsu K., Ohkawara T., Fujimoto M., Tanaka K., Miyazaki Y., Makino T., Kurokawa Y., Yamasaki M., Nakajima K., Takiguchi S., Mori M., Doki Y., Naka T.

OR-17. New treatment strategy for cervical cancer using CRISPR/CAS9 against human papillomavirus type 16/18 E6

Yoshida T., Saga Y., Sehara Y., Uchibori R., Urabe M., Kume A., Fujiwara H., Matsubara S., Mizukami H.

OR-18. A super gene expression system enhances the anti-glioma effects of adenovirus-mediated REIC/Dkk-3 gene therapy

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

OR-19. Reversal of MDR1-mediated chemoresistance in human osteosarcoma by p53-expressing oncolytic adenovirus

Sugiu K., Mochizuki Y., Tazawa H., Hasei J., Osaki S., Yamakawa Y., Omori T., Komatsubara T., Yoshida A., Fujiwara T., Kunisada T., Urata Y., Ozaki T., Fujiwara T.

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

17:30-18:10, Hall-B

Chairpersons: Yuji Heike & Hideaki Tahara

OR-20. T cells expressing CAR harboring GITR signaling domain exhibit prolonged in vivo survival and resistance to immunosuppression that lead to efficient tumor eradication

Wang L., Okamoto S., Amaishi Y., Seo N., Mineno J., Takesako K., Kato T., Shiku H.

OR-21. Tumor-targeting and CD/UPRT-armed oncolytic vaccinia virus enhanced their therapeutic efficacy by combination with prodrug 5-FC

Nakatake M., Kuwano N., Horita K., Ishii K., Sakamoto T., Kurosaki H., Nakamura T.

OR-22. CD133 infectivity-selective oncolytic adenovirus effectively targets radiation-resistant colorectal cancer cells

Huang J. L., Sato-Dahlman M., Miura Y., Shanley R., Jacobsen K., Salzwedel A., Madoff R., Davydova J., Yamamoto M.

OR-23. A MDM2 inhibitor augments cytotoxicity of oncolytic adenoviruses defective of E1B 55kDa molecules through enhancing DNA damages and apoptosis in mesothelioma

Tagawa M., Nguyen T. T. T., Morinaga T., Zhong B., Hanazono M., Kubo S., Shingyoji M., Tada Y., Tatsumi K., Shimada H., Hiroshima K.

Day 2: July 21, 2017

Oral Session VI
Vector-1 (Abstracts OR-24~OR-27)

09:10-09:50, Hall-B

Chairpersons: Hiroyuki Mizuguchi & Mahito Nakanishi

OR-24. A single amino acid in AAV capsids regulates the requirement of the assembly activating protein (AAP) for assembly

Kollu S., Earley L., Powers J., Song Z., Adachi K., Kelley H., Guo C., Cheng G., Nakai H.

OR-25. p53-armed telomerase-specific oncolytic adenovirus sensitizes human soft tissue sarcoma cells to ionizing radiation

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

OR-26. miR-27 inhibits adenovirus infection via the suppression of SNAP25 and TXN2 expressions

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

OR-27. Re-cutting is the major cause of the low efficiencies in single base mutation by genome-editing.

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

Oral Session VII
Vector-2 (Abstracts OR-28~OR-31)

09:50-10:30, Hall-B

Chairpersons: Masashi Urabe & Masato Yamamoto

OR-28. Immunogenic potential of telomerase-specific oncolytic adenovirus

Kanaya N., Kuroda S., Morihiro T., Kubota T., Tazawa H., Kikuchi S., Nishizaki M., Urata Y., Kagawa S., Fujiwara T.

OR-29. Development of flow electroporation-based suspension-adapted HEK293 transfection system toward rAAV production in GMP

Okada H., Tomono T., Adachi K., Hirai Y., Ouchi M., Utsunomiya Y., Chono H., Mineno J., Okada T.

OR-30. Transduction of anterior segment is not affected by vitrectomy before triple-mutated scAAV2 vector injection into vitreous

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

OR-31. Strong alpha cell preference of the AAV strains that best transduce human pancreatic islets in vitro

Song Z., Galivo F. H., Adachi K., Grompe M., Nakai H.

Oral Session VIII

Vector-3 (Abstracts OR-32~OR-35)

10:30-11:10, Hall-B

Chairpersons: Junichi Mineno & Takafumi Nakamura

OR-32. Production of AAV vectors in serum-free suspension culture system

Sakamoto S., Utsunomiya Y., Ouchi M., Inose H., Kaneseki T., Tsukihara T., Kidokoro T., Chono H., Mineno J.

OR-33. The utility of a recombinant measles virus in reprogramming with multiple human hematopoietic cells

Jiyuan L., Kohara H., Hiramoto T., Tahara M., Ogawa Y., Karasawa N., Sakamoto C., Takashima Y., Miyamoto S., Takeda M., Tani K.

OR-34. Elucidation of mechanism of reovirus-mediated down-regulation of hypoxia-inducible factor-1 α in tumor cells

Sakurai F., Hotani T., Mizuguchi H.

OR-35. Gene therapy for alveolar bone regeneration by BMP-2/7 heterodimer produced non-viral vector

Kawai M., Ohura K.

Oral Session IX

Cancer-4 (Abstracts OR-36~OR-39)

13:30-14:10, Hall-B

Chairpersons: Hiroshi Fukuhara & Toshiro Shirakawa

OR-36. iPS-derived neural stem cell transfected with HSV-TK gene using integration-free method as a feasible carrier for targeted delivery of phosphorylated ganciclovir into glioma cells

Yamasaki T., Kenmochi H., Namba H.

OR-37. Novel theranostic strategy with combination of fluorescence oncolytic adenovirus and chemotherapy for peritoneal metastasis of gastric cancer

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

OR-38. Combination therapy of PD-L1 immune checkpoint inhibitor and oncolytic herpes simplex virus HF10

Mukoyama N., Naoe Y., Ichinose T., Nassr E. I. R., Luo C., Villalobos I. B., Zhiwen W., Miyajima N., Morimoto D., Tanaka M., Fujimoto Y., Sone M., Kodera Y., Kasuya H.

OR-39. Use of oncolytic adenovirus expressing IFN- α to develop improved IFN therapy against pancreatic cancer

Salzwedel A., LaRocca C., Han J., Davydova J., Yamamoto M.

Oral Session X
Neurologic Diseases (Abstracts OR-40~OR-43)

14:10-14:50, Hall-B

Chairpersons: Shin'ichi Takeda & Takanori Yokota

OR-40. Life-long behavioral recovery and persistent expression of transgenes after gene therapy in a primate model of Parkinson disease

Sehara Y., Fujimoto K., Ikeguchi K., Katakai Y., Ono F., Takino N., Ito M., Ozawa K., Muramatsu S.

OR-41. Persistent transduction of CNS neurons by a non-toxic herpes simplex virus-based vector

Miyagawa Y., Verlengia G., Simonato M., Cohen J. B., Glorioso J. C.

OR-42. Anti-miR-351-5p rescues hippocampal neural stem cells from autophagic cell death

Park S., Woo H. N., Lee W. I., Lee H. J., Choi R., Kim J. H., Kim Y. K., Kim S. W., Lee H.

OR-43. Enhancement of gap junctional activity by iPS cell-derived neural stem cells in stem cell-based suicide gene therapy

Kenmochi H., Yamasaki T., Namba H.

Oral Session XI
Cardiovascular Diseases (Abstracts OR-44~OR-47)

14:50-15:30, Hall-B

Chairpersons: Yutaka Hanazono & Katsuto Tamai

OR-44. Systemic administration of HMGB1 peptide safely and specifically increases mesenchymal cells in human peripheral blood: outcomes from phase I clinical study

Tamai K., Yamazaki S., Wang X., Nishida M., Kikuchi Y., Sasaki E., Komurasaki Y., Kamei R., Okada A., Yao X., Matsumoto M., Shimbo T., Katayama I., Kaneda Y.

OR-45. Therapeutic efficacy of DNA vaccination against IL-17A in mouse autoimmune arthritis models

Ikeda Y., Nakagami H., Morishita R.

OR-46. Transplantation of mesenchymal stem cells overexpressing IL-10 by AAV1 vector in rat stroke model

Nakajima M., Nito C., Sowa K., Suda S., Nishiyama Y., Nakamura-Takahashi A., Nitahara-Kasahara Y., Imagawa K., Hirato T., Ueda M., Kimura K., Okada T.

OR-47. Effect of dental pulp stem cell overexpressing hepatocyte growth factor by adeno-associated virus vector in a rat stroke model

Sowa K., Nito C., Nakajima M., Suda S., Nishiyama Y., Nakamura-Takahashi A., Nitahara-Kasahara Y., Imagawa K., Hirato T., Ueda M., Kimura K., Okada T.

Day 3: July 22, 2017

Oral Session XII
Basic Science-1 (Abstracts OR-48~OR-51)

09:00-09:40, Hall-B

Chairpersons: Yukihiro Hirai & Hiroaki Mizukami

OR-48. Reduction of retinal vaso-obstruction by an adeno-associated virus expressing mTOR siRNA in a rat model of oxygen-induced retinopathy

Kim J. H., Lee S. H. S., Jung B. K., Park K., Lee J. Y., Lee H.

OR-49. High-throughput mapping of conformational epitopes of monoclonal and polyclonal antibodies against AAV capsids by immunoprecipitation (IP)-seq

Chang X. L., Adachi K., Nakai H.

OR-50. Development of safe and effective hydrodynamic gene therapy for liver diseases - Effects of fibrotic tissue on gene delivery efficiency

Kamimura K., Kobayashi Y., Abe H., Yokoo T., Ohtsuka M., Miura H., Zhang G., Liu D., Terai S.

OR-51. Development of *in vivo* evaluation systems for novel non-coding RNA, SINEUP, to enhance translation level of target genes as a nucleic acid medicine

Nitta K. R., Nakano M., Chinzei M., Suzuki A. M., Alka S., Goto T., Wrabel A. B., Takahashi H., Kataoka Y., Zucchelli S., Gustincich S., Carninci P.

Oral Session XIII

Basic Science-2 (Abstracts OR-52~OR-55)

09:40-10:20, Hall-B

Chairpersons: Nobuhiko Emi & Ken-ichiro Kosai

OR-52. Development of the novel miRNA regulated oncolytic coxsackievirus therapy.

Yang J., Miyamoto S., Kohara H., Yotsuya L., Sagara M., Takishima Y., Matsuura K., Shimizu H., Tani K.

OR-53. Adenovirus vector-induced hepatotoxicity during the early phase after systemic administration is attributed to inflammatory cytokine-induced leaky expression of adenovirus genes

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

OR-54. Promotion of adenovirus infection by a microRNA derived from adenovirus virus-associated RNA II.

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

OR-55. Artificial liver based on viral/transgene/feeder-free iPS cell-derived functional hepatocytes

Harada Y., Yonemitsu Y.

Oral Session XIV

Basic Science-3 (Abstracts OR-56~OR-59)

10:20-11:00, Hall-B

Chairpersons: Koichi Miyake & Hironori Nakagami

OR-56. A Wee1 kinase inhibitor increases apoptosis of replication-competent adenoviruses through induction of mitotic catastrophe and enhancement of viral replications

Morinaga T., Nguyen T. T. T., Zhong B., Kubo S., Shingyoji M., Tada Y., Tatsumi K., Shimada H., Hiroshima K., Tagawa M.

OR-57. Application of droplet digital PCR (ddPCR) for AAV vector titration and impurity evaluation

Furuta B., Yamaguchi T., Uchida E.

OR-58. The novel elimination system of HBV DNA using adenovirus vector expressing Cas9 and multiplex guide RNAs

Maekawa A., Suzuki M., Nagamoto S., Saito I., Kanegae Y.

OR-59. Thymidine kinase mutants offer a more potent safeguard system for iPS cells

Urabe M., Abe T., Uchibori R., Sehara Y., Saga Y., Kume A., Mizukami H., Hanazono Y., Ozawa K.

Poster Session

Poster Session I

Basic Science-4 (Abstracts: PO-60~PO-63)

Date: July 21, 2017, 17:40-18:10, Poster Hall
Chairperson: Eriko Uchida

PO-60. lncRNA UCA1 has the potential to be a biomarker of therapeutic response to oncolytic vaccinia virus in paclitaxel resistant ovarian cancer

Horita K., Kurosaki H., Nakatake M., Kuwano N., Ishii K., Itamochi H., Oishi T., Harada T., Nakamura T.

PO-61. Selective inhibition of HIV-1 replication and induction of cell death in latently infected cells by conditional expression of the *E. coli*-derived endonuclease MazF.

Okamoto M., Chono H., Hidaka A., Toyama M., Mineno J., Baba M.

PO-62. Genome editing efficiencies of high-fidelity Cas9 variants in various human cell types

Nitta H., Wada M., Harigai K., Noguchi A., Okushima N., Iwanaga Y., Saotome A., Mitani K.

PO-63. Efficient screening of genome-edited iPS cells using NGS

Amaishi Y., Aya K., Okamoto S., Tsujimoto Y., Enoki T., Mineno J.

Poster Session II

Vector-4 (Abstracts: PO-64~PO-67)

Date: July 21, 2017, 17:40-18:10, Poster Hall
Chairperson: Shuji Kubo

PO-64. Effect of culture conditions on efficient production of non-toxic herpes simplex virus-based vectors using a novel producer cell line

Kuroda S., Miyagawa Y., Adachi K., Yamamoto M., Cohen J. B., Glorioso J. C., Suzuki H., Uchida E., Okada T.

PO-65. Retroviral replicating vector - mediated prodrug activator gene therapy for osteosarcoma

Kubo S., Takagi-Kimura M., Kasahara N.

PO-66. Purification of AAV vectors both from culture supernatant and inside of host cells

Tsukihara T., Sakamoto S., Hashizume K., Inose H., Abe K., Kidokoro T., Chono H., Mineno J.

PO-67. Chromatography-based rAAV9 purification protocol with ultracentrifugation-free procedure

Tomono T., Hirai Y., Okada H., Miyagawa Y., Adachi K., Sakamoto S., Kawano Y., Chono H., Mineno J., Ishii A., Shimada T., Onodera M., Tamaoka A., Okada T.

Poster Session III

Cancer-5 (Abstracts: PO-68~PO-71)

Date: July 21, 2017, 17:40-18:10, Poster Hall
Chairperson: Atsushi Natsume

PO-68. Combination therapy using oncolytic HSV-1 G47Δ and anti-EGFR monoclonal antibody cetuximab for human head and neck cancers

Nagatomo T., Ino Y., Iwai M., Nishino H., Todo T.

PO-69. Telomerase-targeted oncolytic adenovirus induces autophagic cell death through E2F1-mediated suppression of MYCN in MYCN-amplified neuroblastoma

Tanimoto T., Tazawa H., Noso H., Oyama T., Urata Y., Kagawa S., Noda T., Fujiwara T.

PO-70. Chimeric antigen receptor T cells redirecting EPHB4 for the treatment of human rhabdomyosarcoma

Yagyu S., Nakamura K., Morita D., Kikuchi K., Iehara T., Hosoi H., Nakazawa Y.

PO-71. RAMBO (rapid antiangiogenesis mediated by oncolytic virus) decreased bevacizumab-induced glioma cell invasion

Tomita Y., Kurozumi K., Shimizu T., Oka T., Ohtani Y., Ishida J., Kaur B., Ichikawa T., Date I.

**Poster Session IV
Cancer-6 (Abstracts: PO-72~PO-75)**

Date: July 21, 2017, 17:40-18:10, Poster Hall
Chairperson: Toshihiko Okazaki

PO-72. Anti-glioma mechanism of the Ad-REIC vector with the super gene expression system

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

PO-73. Combination therapy against human pancreatic cancer with oncolytic herpes virus HF10 and abraxane

Wu Z., Ichinose T., Luo C., Naoe Y., Villalobos I. B., Yamada S., Fujii T., Sugimoto H., Miyajima N., Morimoto D., Mukoyama N., Nishikawa Y., Koide U., Kuwahara T., Koderia Y., Tanaka M., Kasuya H.

PO-74. Systemic therapeutic potential of oncolytic herpes simplex virus HF10 adsorbed on antigen-specific lymphocytes

Ichinose T., Naoe Y., Nassr E. I. R., Luo C., Villalobos I. B., Zhiwen W., Mukoyama N., Miyajima N., Morimoto D., Yamada S., Fujii T., Sugimoto H., Tanaka M., Koderia Y., Kasuya H.

PO-75. Potential of oncolytic HSV-1 (G47Δ) for treating biliary tract cancers

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

**Poster Session V
Cancer-7 (Abstracts: PO-76~PO-78)**

Date: July 21, 2017, 17:40-18:10, Poster Hall
Chairperson: Kazuhiko Kurozumi

PO-76. Therapeutic efficacy of an armed oncolytic herpes simplex virus type 1 with immunomodulatory functions

Suzuki S., Ino Y., Iwai M., Fukuhara H., Todo T.

PO-77. Oncolytic HSV-1 virus HF10 in combination with gemcitabine enhanced cytotoxic activity against pancreatic ductal adenocarcinoma cell *in vitro*.

Tsuda H., Kudo Y., Kamihagi K., Tanaka M., Mineno J.

PO-78. Therapeutic effect of a third generation oncolytic HSV-1 G47Δ for hepatocellular carcinoma

Yamada T., Ino Y., Iwai M., Koike K., Todo T.

**Poster Session VI
Genetic Diseases-3 (Abstracts: PO-79~PO-82)**

Date: July 21, 2017, 17:40-18:10, Poster Hall
Chairperson: Toshinao Kawai

PO-79. Improvement of peripheral neuropathy in murine Fabry model by intrathecal administration of serotype 9 Adeno-associated virus vector expressing α -galactosidase A

Higuchi T., Shimada Y., Kobayashi H., Fukuda T., Kaburagi H., Enomoto M., Kato F., Ida H., Ohashi T.

PO-80. Gene therapy and gene editing for lysosomal storage diseases

Kobayashi H., Izuka S., Ariga M., Fukuda T., Shimada Y., Higuchi T., Iwamoto T., Ponder K., Haskins M., Ohashi T.

PO-81. CRISPR/Cas9-mediated gene repair in B cells from XLA patient

Miura A.

PO-82. Reevaluation of the utility of induced pluripotent stem cells as a model for Wiskott Aldrich syndrome

Honda-Ozaki F., Lin H. T., Okumura T., Lai C. Y., Otsu M.

Poster Session VII

Neurologic & Other Diseases (Abstracts: PO-83~PO-85)

Date: July 21, 2017, 17:40-18:10, Poster Hall

Chairperson: Takanori Yamagata

PO-83. Why are scAAV9 vectors able to transduce the CNS but not ssAAV9? ; The difference between the ssAAV9 and scAAV9 vector in transduction of CNS by intravenous injection.

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

PO-84. The profiling of MicroRNA expression in rat hippocampal neural stem cells upon cell death following insulin withdrawal

Woo H. N., Lee W. I., Kang I. K., Park S., Lee H. J., Goo H., Kim J. H., Kim Y. K., Kim S. W., Lee H.

PO-85. Therapeutic effect of adenovirus vector-mediated lysophosphatidylcholine acyltransferase overexpression on obesity and type 2 diabetes mellitus

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

Corporate Seminars & Luncheon Seminar

►Day 1

Corporate Seminar I

(BioReliance)

Date: July 20, 2017, 12:00-13:00, Hall A

Chairperson: Sadao Ozawa

(Merck)

Antoine Heron (Merck)

Susan Livingston (BioReliance)

Guidance and advice for manufacturing and testing of gene and cell therapy clinical products (case studies and practical help from Manufacturing and Testing experts)

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Corporate Seminar II

(Oncolys BioPharma Inc.)

Date: July 20, 2017, 12:00-13:00, Hall B

Chairperson: Masafumi Onodera

(National Center for Child Health and Development)

Toward practical operation of “Cartagena Act” in gene therapy.

1) Takashi Kojima (National Cancer Center Hospital East)

From the view point of conducting investigator-initiated trials.

2) Yasuo Urata (Oncolys BioPharma Inc.)

From the view point of conducting sponsor-initiated trials.

3) Akihiro Kume (Jichi Medical University)

From the view point of translation between academia, industry and regulatory agencies.

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►Day 2

Corporate Seminar III

(TAKARA BIO INC.)

Date: July 21, 2017, 12:00-13:00, Hall A

Chairperson: Junichi Mineno

(TAKARA BIO INC.)

Shin Kaneko (Center for iPS Cell Research and Application, Kyoto University)

Development for regenerative T-cell immunotherapy

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Corporate Seminar IV

(AnGes, Inc.)

Date: July 21, 2017, 12:00-13:00, Hall B

Chairperson: Ryuichi Morishita

(Osaka University Graduate School of Medicine)

Hironori Nakagami (Osaka University Graduate School of Medicine)

Development of angiotensin II vaccine for hypertension

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►Day 3

Luncheon Seminar

Date: July 22, 2017, 12:00-12:40, Hall A

Chairperson: Yasutomo Nasu

(Okayama University Graduate School of Medicine, Dentistry and Pharmaceutical Sciences)

Mizuki Morita (Okayama University Hospital)

The role of biobank in the genome medicine.