THE 24th ANNUAL MEETING

JSGCT2018

JAPAN SOCIETY OF GENE AND CELL THERAPY

Program & Abstracts

Date
July 26-28, 2018

Venue
TORANOMON HILLS FORUM

Mori Tower 5 th Floor, 1-23-3 Toranomon,
Minato-ku, Tokyo 105-6305, Japan

TOKYO

Presidential Lecture (E)

Date: July 27, 2018, 11:40-12:00, Main Hall
Chairperson: Yoshikatsu Eto

Masafumi Onodera (*President of JSGCT2018*, *National Center for Child Health and Development*)
Gene and cell therapy, to take one step forward

JSGCT Chairman's Lecture (J)

Date: July 27, 2018, 11:10-11:40, Main Hall
Chairperson: Shigetaka Asano

Yasufumi Kaneda (*Chairman of JSGCT, Graduate School of Medicine, Osaka University*)
Japan should accelerate human gene therapy without delay

Presidential Special Program with AMED & PMDA (J)

Date: July 26, 2018, 11:10-12:00, Main Hall
Chairperson: Jun Yoshida

SP-1. Makoto Suematsu (*Japan Agency for Medical Research and Development*)
Mission of AMED: Empowering gene therapy for IRUD beyond

Chairperson: Takashi Shimada

SP-2. Tatsuya Kondo (*Pharmaceuticals and Medical Devices Agency*)
PMDA's initiatives based on regulatory science - In pursuit of developing gene therapies -

Educational Lecture (E)

Date: July 27, 2018, 13:00-13:40, Main Hall
Chairperson: Yoshiro Niitsu

EL-1. Osamu Nureki (*The University of Tokyo*)
Molecular mechanism of CRISPR and structure-based development of genome editing tool towards medical applications

EL-2. Ko Mitani (*Saitama Medical University*)
Genome editing for therapeutic applications

Special Symposium I (E)

Date: July 27, 2018, 9:50-11:10, Main Hall
Chairpersons: Masafumi Onodera & Tomoki Todo

SS1-1. Alessandro Aiuti (*San Raffaele Institute, Milan, Italy, ESGCT*)
Gene therapy for Wiskott-Aldrich syndrome

SS1-2. Guangping Gao (*University of Massachusetts Medical School, ASGCT President Elect*)
Human gene therapy - The road to today and beyond

Special Symposium II (J)

-Biosafety Regulations in Gene Therapy-

Date: July 27, 2018, 13:40-15:20, Main Hall
Chairpersons: Masafumi Onodera & Teruhide Yamaguchi

SS2-1. Eriko Uchida (*National Institute of Health Sciences*)
Environmental assessments and shedding studies for gene therapy products in the US and in the EU

SS2-2. Kazunobu Oyama (*Pharmaceuticals and Medical Devices Agency*)
Points to consider for environmental risk assessment of the use of living modified organisms; PMDA perspectives on type 1 use approval and type 2 use confirmation under "Cartagena Law"

International Symposium (E)

-Steps forward to Clinic in Gene and Cell Therapy of Genetic Diseases-

Date: July 27, 2018, 15:30-17:20, Main Hall

Chairpersons: Noriyuki Kasahara & Masato Yamamoto

IS-1. Daniel C. Chung (*Spark Therapeutics*)
Development of Luxturna™ (voretigene neparvovec-rzyl): Gene therapy for *RPE65* biallelic mutation associated inherited retinal disease

IS-2. Michele De Luca (*University of Modena and Reggio Emilia*)
YAP/TAZ-dependent depletion of human epidermal stem cells in junctional epidermolysis bullosa (JEB)

IS-3. Harry L. Malech (*National Institutes of Health*)
Lentivector gene therapy for X-linked chronic granulomatous disease.

Symposium I (E)

-JCA-JSGCT joint Symposium-

"All about CAR-T cell therapy: current status and future perspective"

*JCA: Japanese Cancer Association

Date: July 26, 2018, 15:10-17:00, Main Hall

Chairpersons: Keiya Ozawa & Koji Tamada

S1-1. Keiya Ozawa (*Jichi Medical University*)
Overview of CAR-T cell therapy

S1-2. Isabelle Rivière (*Michael G. Harris Cell Therapy and Cell Engineering Facility Center for Cell Engineering*)
CAR T cell therapy: The CD19 paradigm and beyond

S1-3. Naoki Hosen (*Osaka University Graduate School of Medicine*)
The activated conformation of integrin β7 is a novel multiple myeloma–specific target for CAR T-cell therapy

S1-4. Koji Tamada (*Yamaguchi University Graduate School of Medicine*)
A novel platform technology of CAR-T cell therapy for solid tumors

Symposium II (E)

-Genetic Diseases-

Date: July 26, 2018, 17:00-18:50, Main Hall

Chairpersons: Torayuki Okuyama & Toya Ohashi

S2-1. Shin'ichi Takeda (*National Center of Neurology and Psychiatry*)
Exon skipping therapy for muscular dystrophy

S2-2. Juan Ruiz (*Abeona Therapeutics, Inc.*)
Developing AAV gene therapies for rare genetic diseases

S2-3. Karin Kojima (*Jichi Medical University*)
Gene therapy for child neurological diseases: Effective for AADC deficiency, new challenges for Glut1-deficiency syndrome and potential for other diseases

S2-4. Alessandro Aiuti (*San Raffaele Institute, Milan, Italy*)
Genetic engineering of hematopoiesis to treat inherited diseases

Symposium III (J)

-Regulatory Science-

Date: July 27, 2018, 8:30-9:50, Main Hall

Chairpersons: Akihiro Kume & Eriko Uchida

S3-1. Takashi Ichikawa (*Ministry of Health, Labour and Welfare*)
Revised ethical guideline for clinical trials on gene therapy

S3-2. Teruhide Yamaguchi (*Kanazawa Institute of Technology/ Nihon Pharmaceutical University*)
Revision of guideline for gene therapy clinical study based on the novel technology of gene editing

S3-3. Megumi Kawamoto (*Pharmaceuticals and Medical Devices Agency*)
Points to consider in quality and preclinical aspects of gene therapy products

Symposium IV (J)

-JSGCT Working Group Symposium-

"The Advancement in Innovative Approaches for Next-generation Gene Therapy"

Date: July 27, 2018, 8:30-9:30, Hall-1

Chairpersons: Makoto Otsu & Fuminori Sakurai

S4-1. Satoshi Nagano (*Kagoshima University*)
First-in-human investigator-initiated clinical trial of survivin-respoinsive conditionally replicating adenovirus for treating bone and soft tissue sarcoma

S4-2. Kayoko Nakamura (*Shinshu University Hospital*)
Chimeric antigen receptor T cells redirecting EPHB4 for the treatment of human rhabdomyosarcoma

S4-3. Shinichiro Nakada (*Graduate School of Medicine, Osaka University*)
Precise gene editing by SNGD

Symposium V (E)

-Vector-

"GeneRide and HITI: Cutting Edge Technologies in Vectors and Genome Editing"

Date: July 27, 2018, 13:00-14:10, Hall-1

Chairpersons: Hiroyuki Nakai & Takashi Okada

S5-1. Nelson Chau (*LogicBio Therapeutics*)
GeneRideTM, a novel AAV-mediated strategy to treat pediatric indications

S5-2. Keiichiro Suzuki (*Osaka University*)
Genome-editing therapy via HITI (Homology-Independent Targeting Integration) method

Symposium VI (E)

-Genome Editing-

"Genome editing: Step to gene therapy"

Date: July 27, 2018, 14:10-15:20, Hall-1

Chairpersons: Akitsu Hotta & Yumi Kanegae

S6-1. Harry L. Malech (*National Institutes of Health*)
CRISPR-mediated correction of CD34+ hematopoietic stem cells from patients with chronic granulomatous disease

S6-2. Tsukasa Ohmori (*Jichi Medical University School of Medicine*)
Development of genome-editing treatment for hemophilia

S6-3. Akitsu Hotta (*CiRA, Kyoto University*)
Genome editing and iPS cell mediated gene therapy: how to overcome *in vivo* delivery and *ex vivo* immunorejection

Symposium VII (E)

-Regenerative Medicine-

"Interface between gene therapy and regenerative medicine"

Date: July 28, 2018, 8:30-10:15, Main Hall

Chairpersons: Katsuto Tamai & Toru Uchiyama

S7-1. Michele De Luca (*University of Modena and Reggio Emilia*)
Life-saving regeneration of the entire human epidermis by transgenic stem cells

S7-2. Katsuto Tamai (*Osaka University*)
Circulating mesenchymal stem cells: their function and possibility as a target of gene therapy.

S7-3. Takumi Era (*Institute of Molecular Embryology and Genetics, Kumamoto University*)
Origin and development of mesenchymal stem cells

S7-4. Tomoyuki Yamaguchi (*Institute of Medical Science, University of Tokyo*)
Interspecies organogenesis generates autologous functional islets

Symposium VIII (J)

-GCTP Marketing-

"Extracting issues toward Social Implementation of Cell & Gene therapy: What is proper price of the products?"

Date: July 28, 2018, 8:30-10:15, Hall-1

Chairpersons: Masanobu Kimura & Toshihiko Okazaki

S8-1. Hisako Hashimoto (*Foundation for Biomedical Research and Innovation at Kobe*)
Roadmap for industrialization of cell-based therapy from academia to clinical sites. Importance of the design of the cell manufacturing system by academia applicable to industrialization.

S8-2. Junichi Mineno (*TAKARA BIO INC.*)
Manufacturing of cellular and tissue-based products

S8-3. Tadashi Sameshima (*Terumo Corp.*)
Development of HeartSheet and current issue

S8-4. Yoji Sato (*National Institute of Health Sciences*)
Quality of biological raw materials for manufacturing of cell-based and gene therapy products

Symposium IX (E)

-Neurologic Diseases-

"Nervous system"

Date: July 28, 2018, 10:15-12:00, Main Hall

Chairpersons: Hideki Mochizuki & Shin-ichi Muramatsu

S9-1. Kohji Itoh (*Tokushima University*)
Gene therapy for GM2 gangliosidosis with CNS involvement

S9-2. Seiichi Nagano (*Osaka University*)
Gene therapy for ALS

S9-3. Atsumi Nitta (*University of Toyama*)
Effects of overexpression of TMEM-induced by AAV on the behavioral changes of the drug dependence and anxiety

S9-4. Takuro Horii (*Gunma University*)
Mouse modeling by genome and epigenome editing

Symposium X (E)

-Oncolytic Virus-

"For clinical implementation of oncolytic virotherapy"

Date: July 28, 2018, 10:15-12:00, Hall-1

Chairpersons: Toshiyoshi Fujiwara & Kenzaburo Tani

S10-1. Teruhide Yamaguchi (*Kanazawa Institute of Technology, Nihon Pharmaceutical University*)
Current issues on quality and safety of oncolytic virus products

S10-2. Shohei Miyamoto (*IMS, The University of Tokyo*)
The current preparative status of oncolytic coxsackievirotherapy toward clinical trial

S10-3. Hiroshi Tazawa (*Okayama Univerisity Hospital*)
Recent advances in clinical application of telomerase-specific oncolytic viruses

S10-4. Maki Tanaka (*Takara Bio Inc*)
Clinical evaluation of a novel oncolytic viral immunotherapy agent, canerpaturev (HF10)

S10-5. Tomoki Todo (*The Institute of Medical Science, The University of Tokyo*)
Clinical development of third generation oncolytic HSV-1 G47Δ

Symposium XI (E)

-Cardiovascular Diseases-

"Cardiovascular Gene & Cell Therapy and Genome Editing: a recent progress"

Date: July 28, 2018, 13:00-14:50, Main Hall

Chairpersons: Ryuichi Morishita & Yoshikazu Yonemitsu

S11-1. Yoshikazu Yonemitsu (*Kyushu University Graduate School of Pharmaceutical Sciences*)
Opening remarks: Recent progress of cardiovascular gene & cell therapy and gene editing

S11-2. Hidemasa Oh (*Okayama University Hospital*)
Cell therapy trials in congenital heart disease

S11-3. Yuichi Oike (*Kumamoto University*)
Identification of candidate genes for gene therapy against heart failure

S11-4. Hironori Nakagami (*Osaka University Graduate School of Medicine*)
Development of novel immunotherapy for cardiovascular diseases

S11-5. Haruhiko Morita (*EdiGENE Corporation*)
Frontline of gene editing application

S11-6. Ryuichi Morishita (*Osaka University Graduate School of Medicine*)
Closing remarks

Symposium XII (E)

-Cancer Gene Therapy-

"Gene medicine going into a translational phase"

Date: July 28, 2018, 13:00-14:50, Hall-1

Chairpersons: Yasutomo Nasu & Masatoshi Tagawa

S12-1. Nicholas A. Boyle (*Tocagen Inc.*)
Toca 511 & Toca FC: Evaluation of durable response rate in the post-resection setting and association with survival in patients with recurrent high grade glioma

S12-2. Toshihiro Nakajima (*Genomidea, Inc.*)
Clinical development of non-replicating oncolytic virus (HVJ-E/GEN0101) for the treatment of solid cancers

S12-3. Yuji Tada (*School of Medicine Chiba university*)
A phase1 clinical trial of inhibiting the HGF/c-Met pathway for malignant pleural mesothelioma with NK4 gene-expressing adenoviral vectors

S12-4. Kazuhiko Kurozumi (*Okayama University*)
A phase I/II clinical trial of Ad-SGE-REIC for malignant brain tumor

S12-5. Hitoshi Shiomi (*Momotaro Gene Inc.*)
The current status of clinical trials of Ad-SGE-REIC gene therapy

Presidential Evening Symposium I (E)

Date: July 26, 2018, 18:50-19:30, Main Hall
Chairperson: Masafumi Onodera

ES1-1. Christopher Dott (*Orchard Therapeutics*)
Orchard Therapeutics and its gene therapy programs

ES1-2. Tohru Hirose (*Novartis Pharma K.K.*)
Development of gene therapy related treatments in Novartis

Presidential Evening Symposium II (E)

Date: July 27, 2018, 18:00-18:20, Main Hall
Chairperson: Masafumi Onodera

ES2. Takayuki Imaeda (*Pfizer Inc.*)
Gene therapy (GTx): A path to deliver transformative medicine to patients

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9th Takara Bio Award Lecture

Date: July 26, 2018, 14:50-15:10, Main Hall
Chairperson: Yasufumi Kaneda

Fuminori Sakurai (*Osaka University Graduate School of Medicine*)
Neonatal gene therapy for hemophilia B by a novel adenovirus vector showing reduced leaky expression of viral genes

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

Date: July 26, 2018, 14:00-14:50, Main Hall
Chairpersons: Shin'ichi Takeda, & Toshihiko Wakabayashi

PS-1. Systemic treatment with mesothelin-targeted oncolytic adenovirus shows efficacy patient-derived xenograft of pancreatic cancer
Sato-Dahlman M., Miura Y., Hajeri P., Yoshida H., Jacobsen K., Yanagiba C., Yamamoto M.

PS-2. Systemic rAAV9-microdystrophin transduction with somatic stem cells pre-treatment improve transgene expression and their DMD phenotype in canine X-linked muscular dystrophy
Hayashita-Kinoh H., Nitahara-Kasahara Y., Kuraoka M., Okada H., Imagawa K., Hirato T., Takeda S., Okada T.

PS-3. Blastic transformation of retrovirus-transduced cells in X-CGD gene therapy.
Uchiyama T., Kawai T., Nakabayashi K., Ando Y., Watanabe N., Kato M., Kato K., Nishimura T., Nunoi H., Onodera M.

Day 1: July 26, 2018

Oral Session I
Basic Study I (Abstracts OR-4~OR-7)

15:10~15:46, Hall-1
Chairpersons: Koichi Miyake & Takafumi Nakamura

OR-4. Predictive biomarkers for cancer virotherapy with oncolytic vaccinia virus
Horita K., Kurosaki H., Nakatake M., Kuwano N., Ishii K., Kohno H., Ito M., Itamochi H., Oishi T., Harada T., Nakamura T.

OR-5. A Wee1 kinase inhibitor augments apoptosis and replications of adenoviruses in p53-deficient tumor cells
Morinaga T., Nguyen T. T. T., Zhong B., Kubo S., Shingyoji M., Tada Y., Tatsumi K., Shimada H., Hiroshima K., Tagawa M.

OR-6. Inhibitors for the MDM2-p53 interaction increase a DNA damage signal and augment replications of oncolytic adenoviruses in mesothelioma with the wild-type p53 genotype
Tagawa M., Nguyễn T. T. T., Morinaga T., Zhong B., Hanazono M., Shingyoji M., Kubo S., Tada Y., Tatsumi K., Shimada H., Hiroshima K.

OR-7. High efficient genome editing system using short-term and strong expression of Cas9
Nagamoto S., Maekawa A., Akimoto K., Kanegae Y.

Oral Session II
Basic Study II (Abstracts OR-8~OR-11)

15:46~16:22, Hall-1
Chairpersons: Yutaka Hanazono & Mahito Nakanishi

OR-8. Inactivated Sendai virus particles increase NK cell sensitivity of cancer cells via upregulating ICAM-1 expression on cancer cell surfaces
Nishikawa T., Li S., Kaneda Y.

OR-9. A high-throughput method of constructing and screening short synthetic gene regulatory elements
Ferrick-Kiddie E., Gupta A., Song Z., Galivo F., Grompe M., Nakai H.

OR-10. The efficacy of oncolytic HSV-1 (G47Δ) in mouse biliary tract cancer models
Tateno Y., Ino Y., Iwai M., Shinozaki M., Todo T.

OR-11. PiggyBac CD19 CAR T cells eradicate CNS leukemia by direct delivery into cerebral ventricle of xenograft mice model
Tanaka K., Kato I., Tanaka M., Morita D., Takahashi Y., Umeda K., Hiramatsu H., Adachi S., Nakazawa Y.

Oral Session III
Neurologic Diseases I (Abstracts OR-12~OR-15)

16:22~16:58, Hall-1
Chairpersons: Karin Kojima & Takanori Yokota

OR-12. Direct comparison between single-stranded and self-complementary type 9 AAV vector to treat adult MLD model mice by intravenous injection
Miyake N., Miyake K., Yamamoto M., Shimada T., Okada T.

OR-13. Efficiency of dental pulp-derived cell therapy for Duchenne muscular dystrophy treatment
Nitahara-Kasahara Y., Hayashita-Kinoh H., Kuraoka M., Oda Y., Imagawa K., Hirato T., Takeda S., Okada T.

OR-14. Immune response elicited by gene therapy using adeno-associated virus (AAV) vector for muscular dystrophy
Ishii A., Hayashita-Kinoh H., Okada H., Shin J. H., Okada T., Takeda S.

OR-15. Gene therapy for epileptic EL mice by intravascular administration of Adeno-associated virus
Oguro K., Shimazaki K., Yokota H., Tashiro T., Murashima Y., Kawai K., Muramatsu S.

Oral Session IV
Neurologic Diseases II (Abstract OR-16-OR-18)

16:58~17:25, Hall-1
Chairpersons: Yasuhiro Ikeda & Takanori Yamagata

OR-16. Human mesenchymal stem cells as cellular vehicles to deliver retroviral replicating vectors for cancer gene therapy
Kubo S., Takagi-Kimura M., Kasahara N.

OR-17. Psychosine-induced impairment of p62-KEAP1-NRF2 pathway underlying the pathogenesis of Globoid cell leukodystrophy
Lin D. S., Ho C. S., Chiang M. F.

OR-18. Newly developed xeno-free expansion medium for human mesenchymal stem cells.
Tosaka Y., Enoki T., Hatsuyama A., Okamoto S., Mineno J.

Oral Session V
Vector I (Abstracts OR-19~OR-21)

17:25~17:52, Hall-1
Chairpersons: Fuminori Sakurai & Masashi Urabe

OR-19. A proximity-based proteomics approach to identify cellular proteins interacting with AAV assembly activating protein (AAP)
Kollu S., Song Z., Earley L., Chang X., Kulik V., Nakai H.

OR-20. Intravitreal injection of AAV vector in cynomolgus monkeys affects neutralizing antibody titer against AAV in the serum
Igarashi T., Takahashi K., Miyake K., Kobayashi M., Yaguchi C., Miyake N., Kameya S., Takahashi H., Okada T.

OR-21. Preclinical toxicological evaluation in cynomolgus monkey with tumor-targeted and armed oncolytic vaccinia virus purified through manufacturing process
Kurosaki H., Okamura T., Ito M., Nakatake M., Kuwano N., Horita K., Ishii K., Kouno H., Yasutomi Y., Nakamura T.

Oral Session VI
Cardiovascular Diseases I (Abstracts OR-22~OR-24)

17:52~18:19, Hall-1
Chairpersons: Yozo Nakazawa & Yukihiro Saito

OR-22. Retained cortical plasticity and full recovery of rod-mediated visual acuity in blind adult mice with retinal dystrophy
Nishiguchi K. M.

OR-23. HMGB1 peptide ameliorates an intractable skin genetic disorder, dystrophic epidermolysis bullosa, by activating a unique subset of mesenchymal stem cells
Shimbo T., Yamazaki S., Kitayama T., Ouchi Y., Yamamoto R., Kikuchi Y., Bruckner-Tuderman L., Kaneda Y., Tamai K.

OR-24. Development of novel differentiation procedure and expansion medium for iPSC-derived endothelial cells.
Kudo Y., Tosaka Y., Enoki T., Kubo K., Yoshioka M., Ikuno T., Masumoto H., Yamashita J. K., Mineno J.

Oral Session VII
Cardiovascular Diseases II (Abstracts OR-25~OR-27)

18:19~18:46, Hall-1
Chairpersons: Hiroaki Mizukami & Hironori Nakagami

OR-25. AAV8-mediated gene expression *in vivo* using pig liver
Watano R., Ohmori T., Hishikawa S., Sakata A., Nishimura S., Mizukami H.

OR-26. Suppression of oncolytic adenovirus-mediated hepatotoxicity by liver-specific inhibition of NF-κB
Machitani M., Sakurai F., Wakabayashi K., Tachibana M., Kato N., Fujiwara T., Mizuguchi H.

OR-27. Reovirus ameliorates liver fibrosis following intravenous administration in mice ~Drug repurposing of reovirus for antifibrotic therapy~
Sakurai F., Inoue S., Kibe Y., Takayama K., Kobiyama K., Ishii K. J., Akira S., Mizuguchi H.

Day 2: July 27, 2018

Oral Session VIII
Cancer I (Abstracts OR-28~OR-32)

09:30~10:15, Hall-1
Chairpersons: Hiroshi Fukuhara & Hiroki Nanba

OR-28. Final total data of clinical trial using HF10 with gemcitabine and erlotinib against unresectable pancreatic cancer
Kasuya H., Hirooka Y., Naoe Y., Matsumura S., Bustos I., Ichinose T., Eissa I., Zhiwen W., Mukoyama N., Miyajima N., Morimoto D., Goto H., Tanaka M.

OR-29. CAR-T cells targeting GM-CSF receptors, generated by the piggyBac transposon system, are highly effective against acute myeloid leukemia *in vitro* and *in vivo*.
Hasegawa A., Narimatsu S., Tanaka M., Nagai M., Morita D., Saito S., Nakano S., Onozato T., Hayashi M., Matsuda K., Yanagisawa R., Nakamura K., Yagyu S., Ohnota H., Nakazawa Y.

OR-30. Therapeutic efficacy of oncolytic herpes simplex virus type 1 expressing an immunomodulatory molecule in mouse neuroblastoma and melanoma models
Suzuki S., Ino Y., Iwai M., Fukuhara H., Todo T.

OR-31. Overexpression of SOCS3 mediated by adenovirus vector in prostate cancer cells increased the sensitivity to lymphokine-activated killer cells in vitro and in vivo.
Kitagawa K., Yoneda T., Ishiko M., Kadowaki M., Otsuki N., Nibu K., Fujisawa M., Serada S., Naka T., Shirakawa T.

OR-32. Novel approach for systemic cancer therapy with oncolytic vaccinia virus through evading the host immune response.
Nakatake M., Kurosaki H., Nakamura T.

Oral Session IX
Cancer II (Abstracts OR-33~OR-37)

10:15~11:10, Hall-1
Chairpersons: Kazunori Aoki & Yasushi Ino

OR-33. Immunogenic potential of Telomelysin sensitizing gastrointestinal tumors to anti-PD-1 antibody
Kanaya N., Kuroda S., Kakiuchi Y., Morihiro T., Kubota T., Kikuchi S., Nishizaki M., Urata Y., Tazawa H., Kagawa S., Fujiwara T.

OR-34. Oncolytic adenovirus-mediated p53 overexpression induces profound immunogenic cell death in human pancreatic ductal adenocarcinoma cells
Araki H., Tazawa H., Ieda T., Fushimi T., Kuroda S., Yoshida R., Kishimoto H., Nishizaki M., Urata Y., Kagawa S., Fujiwara T.

OR-35. Attenuation of transforming growth factor-β-induced epithelial-mesenchymal transition by oncolytic virotherapy in human esophageal cancer
Masuda T., Tazawa H., Ieda T., Hashimoto Y., Tanabe S., Noma K., Urata Y., Kagawa S., Shirakawa Y., Fujiwara T.

OR-36. Intraperitoneal administration of fluorescence oncolytic adenovirus and paclitaxel is a novel theranostic strategy for the treatment of scirrhous gastric cancer with peritoneal metastasis
Ishikawa W., Kikuchi S., Tazawa H., Kuroda S., Noma K., Kishimoto H., Nishizaki M., Urata Y., Kagawa S., Fujiwara T.

OR-37. Induction of prostate cancer stem cell properties in mouse induced pluripotent stem cells via conditioned medium and tracking drug response
Huang P., Xu N., Watanabe M., Nasu Y.

Oral Session X
Vector II (Abstracts OR-38~OR-41)

15:30~16:06, Hall-1
Chairpersons: Ken-ichiro Kosai & Junichi Mineno

OR-38. Novel microRNA engineered coxsackievirus B3 for oncolytic virotherapy.
Yang J., Miyamoto S., Hirose L., Sagara M., Takishima Y., Shimizu H., Tani K.

OR-39. The optimization of site-specific gene knock-in using Cas9 ribonucleoprotein and AAV as a donor template.
Okamoto S., Maki I., Sugizaki M., Enoki T., Mineno J.

OR-40. GAIA-102: a new class NK cells manufactured in accordance with GMP/GCTP that can eliminate the solid tumors
Harada Y., Yonemitsu Y.

OR-41. Reovirus induces down-regulation of HIF-1α in the subcutaneous tumors following intravenous administration
Sakurai F., Hotani T., Mizuguchi H.

Oral Session XI
Genetic Diseases I (Abstracts OR-42~OR-45)

16:06~16:42, Hall-1
Chairpersons: Hiroshi Kobayashi & Toru Uchiyama

OR-42. Long-term follow-up assessment of the safety and efficacy of Invossa-K Inj. for Osteoarthritis
Cho J. J., Kim T. W., Kang S., Jung J. H., Lew S. H.

OR-43. Targeted genome editing of murine hematopoietic stem cells by CRISPR/Cas9
Byambaa S., Uosaki H., Hara H., Shibata H., Abe T., Nagao Y., Nureki O., Ohmori T., Hanazono Y.

OR-44. Development of adenovirus vectors expressing highly multiplex double-nicking guide RNAs for *in vivo* genome editong aiming clinical application
Nakanishi T., Maekawa A., Saito I.

OR-45. Successful in vivo genome editing of the OTC locus in a minigene system and a humanized mouse model
Amaya A. K., Ginn S. L., Liao S. H. Y., Zhu C., Lee M., Pickett H. A., Hallwirth C. V., Cunningham S. C., Logan G. J., Dilworth K., Lisowski L., Alexander I. E.

Oral Session XII
Genetic Diseases II (Abstracts OR-46~OR-48)

16:42~17:09, Hall-1
Chairpersons: Yukihiko Hirai & Izumu Saito

OR-46. Improvement of lethal hypophosphatasia in mice by high level expression of bone targeted alkaline phosphatase using self-complementary AAV8 vector
Nakamura-Takahashi A., Ikeue R., Nitahara-Kasahara Y., Watanabe A., Hirai Y., Okada T., Kasahara M.

OR-47. Distribution of retroviral vector in ADA-SCID patients treated with T cell- and stem cell-gene therapy.
Uchiyama T., Takahashi S., Edasawa K., Nakabayashi K., Watanabe N., Tamura E., Miura A., Kato M., Otsu M., Kawai T., Ariga T., Onodera M.

OR-48. Development of HSC mediated gene therapy using ACK2 as preconditioning
Miwa S., Shimada Y., Higuchi T., Kobayashi H., Ohashi T.

□Best Poster ○Excellent Poster

Poster Session

Poster Session I
Basic Science (Abstracts: PO-49~PO-52)

Date: July 27, 2018, 17:20-17:40, Poster & Exhibition Hall
Chairperson: Hideki Kasuya

PO-49. Does the expression level of STING correlate with susceptibility to oncolytic virus HF10 in human pancreatic cell lines?
Morimoto D., Naoe Y., Matsumura S., Ichinose T., Eissa I. R., Villalobos I. B., Miyajima N., Mukoyama N., Zhiwen W., Tanaka M., Kodera Y., Kasuya H.

PO-50. Involvement of cGAS and STING in sensitivity to oncolytic virus HF10 in breast cancer cell lines
Miyajima N., Naoe Y., Matsumura S., Bustos I., Ichinose T., Eissa I., Zhiwen W., Mukoyama N., Morimoto D., Tanaka M., Kasuya H.

PO-51. Enhancement of non-toxic HSV vector production and function by chemical compound treatment
Kuroda S., Miyagawa Y., Adachi K., Yamamoto M., Kinoh H., Cohen J. B., Glorioso J. C., Suzuki H., Yoshida H., Okada T.

PO-52. Molecular mechanisms of anti-leukemia efficacy of mitochondrial oxidative phosphorylation inhibition in AML cells
Yang H., Saitoh K., Imoto J., Ikeo K., Mogushi K., Hosoya M., Miida T., Ma H., Ruvolo V., Marszalek J. R., Konopleva M., Tabe Y.

Poster Session II
Basics Science and Neuronal Diseases (Abstracts: PO-53~PO-56)

Date: July 27, 2018, 17:20-17:40, Poster & Exhibition Hall
Chairperson: Noriko Miyake

PO-53. User-friendly analysis package of discovering transgene integration site by Next Generation Sequencing
Segawa H., Narukawa K., Kira S., Shimada M., Mineno J.

○ **PO-54. Anti-tumor effects of neutrophil activation by RIG-I pathway stimulation**
Chang C. Y., Tai J. A., Kaneda Y.

PO-55. The comparison of gene transduction of AAV-rh10 to AAV5 in gerbil hippocampus using different promoters
Sehara Y., Shimazaki K., Urabe M., Mizukami H.

PO-56. Generation of neuronal-like cells by the direct reprogramming method
Jinno J., Jin Y., Ikenaka K., Nagano S., Mochizuki H.

Poster Session III
Vector I (Abstracts: PO-57~PO-61)

Date: July 27, 2018, 17:20-17:45, Poster & Exhibition Hall
Chairperson: Kenya Kamimura

PO-57. Retracted.

PO-58. 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.

PO-59. New application of Borna disease virus vector to gene delivery
Sakai M., Ueda S., Daito T., Asada-Utsugi M., Komatsu Y., Kinoshita A., Maki T., Kuzuya A., Takahashi R., Makino A., Tomonaga K.

PO-60. Oncolytic virus therapy for breast cancer using a third generation oncolytic HSV-1 (G47Δ)
Hasegawa H., Ino Y., Iwai M., Todo T.

PO-61. Safety assessment of CRISPR-Cas9 genome editing for human gene therapy
Uchida E., Naito Y., Ono R., Hirabayashi Y., Sato Y., Inoue T.

Poster Session IV
Vector II (Abstracts: PO-62~PO-67)

Date: July 27, 2018, 17:20-17:50, Poster & Exhibition Hall
Chairperson: Yuji Heike

PO-62. Non-integrating measles virus vector is an outstanding gene transfer tool for establishing iPSCs from human T cells and hematopoietic progenitor cells
Liao J., Soda Y., Sugawara A., Miura Y., Hiramoto T., Kohara H., Hirose L., Takishima Y., Hijikata Y., Miyamoto S., Tahara M., Takeda M., Tani K.

PO-63. Large scale production of novel recombinant coxsacievirus B3 towards human clinical trial
Sagara M., Miyamoto S., Hara K., Miura Y., Hirose L., Takishima Y., Jia Y., Soda Y., Hijikata Y., Iwanaga A., Shimizu H., Tani K.

PO-64. Establishment of scalable production method for adenovirus type 5 vector using fixed-bed bioreactor
Ouchi M., Tsukihara T., Kidokoro T., Chono H., Mineno J.

PO-65. Human virus testing for biopharmaceuticals or regenerative medical products by using real-time PCR assay.
Shimomura M., Takashima R., Tomura D., Takayama M., Yoshizaki M., Uemori T., Sano M., Mineno J.

PO-66. Scalable and serum-free production of lentiviral vector in suspension culture system.
Inose H., Ouchi M., Utsunomiya Y., Tsukihara T., Chono H., Mineno J.

PO-67. Construction of the line clearance procedure for manufacturing gene and cell therapy products before/after operations.
Nakao M., Tahara K., Nukaya I., Mineno J.

Poster Session V
Cardiovascular Diseases (Abstracts: PO-68~PO-71)

Date: July 27, 2018, 17:20-17:40, Poster & Exhibition Hall
Chairperson: Yuko Kasahara

PO-68. siRNA embedded Chaperonin GroEL nanotubes for gene therapy applications
Hashim P. K.

PO-69. Therapeutic effect of lysophosphatidylcholine acyltransferase overexpression by modified adenovirus vector on type 2 diabetes mellitus
Shimizu K., Mikamoto T., Urayama Y., Nishinaka T., Sakurai F., Mizuguchi H., Tomita K., Terada T.

PO-70. Interleukin-10 gene transfer enhances neuroprotective effects of mesenchymal stem cells following transient focal cerebral ischemia
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.

○ **PO-71. Dental pulp stem cell overexpressing hepatocyte growth factor ameliorates blood-brain barrier permeability and promotes neuroprotection in a rat model of ischemic stroke.**
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.

Poster Session VI
Genetic Diseases (Abstracts: PO-72~PO-75)

Date: July 27, 2018, 17:20-17:40, Poster & Exhibition Hall
Chairperson: Toshinao Kawai

PO-72. Immune cell-mediated modulation of neurodegeneration in Niemann-Pick disease type C
Yasuda T., Watanabe N., Uchiyama T., Onodera M

PO-73. Improvement of peripheral neuropathy by intrathecal administration of adeno-associated virus vector expressing α-galactosidase A in murine Fabry model
Higuchi T., Shimada Y., Kobayashi H., Fukuda T., Kato F., Ida H., Ohashi T.

PO-74. Robust and high-efficient iPSC generation from non-mobilized peripheral CD34+ cells for disease modeling using auto-erasable Sendai virus vector
Okumura T., Horie Y., Kikuchi A., Lai C. Y., Okura H., Lin H. T., Ozaki F., Nishimura K., Ohtaka M., Nakanishi M., Otsu M.

PO-75. Selective inhibition of *KRAS* mutant hematopoietic progenitor cells
Lin H. T., Kubara K., Yamazaki K., Takagi M., Otsu M..

Poster Session VII
Cancer I (Abstracts: PO-76~PO-79)

Date: July 27, 2018, 17:20-17:40, Poster & Exhibition Hall
Chairperson: Atsushi Natsume

PO-76. Therapeutic efficacy of a third generation oncolytic HSV-1 (G47Δ) in gastric carcinoma
Sugawara K., Ino Y., Iwai M., Seto Y., Todo T.

PO-77. Anti-tumor monoclonal antibodies augment the therapeutic efficacy of oncolytic herpes simplex virus type 1 (G47Δ) through antitumor immune responses in mice
Nagatomo T., Ino Y., Iwai M., Nishino H., Todo T.

PO-78. Therapeutic efficacy of a third generation oncolytic HSV-1 G47Δ for pancreatic ductal adenocarcinoma
Yamada T., Ino Y., Iwai M., Koike K., Todo T.

PO-79. Therapeutic activity of prodrug activator gene therapy with Toca 511 for esophageal cancer
Suzuki T., Shichinohe T., Kushiya H., Umemoto K., Sato O., Inoko K., Inagaki A., Nakamura T., Hiraoka K., Tsuchikawa T., Jolly D. J., Kasahara N., Hirano S.

Poster Session VIII
Cancer II (Abstracts: PO-80~PO-84)

Date: July 27, 2018, 17:20-17:45, Poster & Exhibition Hall
Chairperson: Shuji Kubo

PO-80. CD133-targeted oncolytic adenovirus exhibits anti-tumor effect in colorectal cancers, and its combination with irradiation inhibits liver metastasis.
Sato-Dahlman M., Huang J. L., Jacobsen K., Yamamoto M.

PO-81. Use of oncolytic adenovirus expressing IFN alpha as a tool to improve IFN-based chemoradiation regimen to treat pancreatic cancer
Salzwedel A., LaRocca C., Han J., Davydova J., Yamamoto M.

PO-82. Preclinical studies for prodrug activator gene therapy by retroviral replicating vectors in CNS metastatic breast cancer
Inagaki A., Richardson A. M., Collins S. A., Hiraoka K., Kamijima S., Gruber H. E. , Jolly D. J., Merchan J., Slingerland J. M., Kasahara N.

PO-83. Assessments for prediction of bystander effect in HSV-tk/GCV gene therapy
Kenmochi H., Yamasaki T., Namba H.

PO-84. The synergistic effect of the combination therapy of PD-L1 immune checkpoint inhibitor and oncolytic herpes simplex virus HF10
Mukoyama N., Nassr E. I. R., Naoe Y., Ichinose T., Matsumura S., Villalobos I. B., Zhiwen W., Miyajima N., Morimoto D., Tanaka M., Fujimoto Y., Sone M., Kodera Y., Kasuya H.

Corporate Seminars
Evening Seminar

▶Day I

Corporate Seminar-I
(AnGes, Inc.)

Date: July 26, 2018, 12:00-13:00, Main Hall
Chairperson: Masafumi Onodera
(*National Center for Child Health and Development*)

Ryuichi Morishita (*Osaka University, Graduate School of Medicine*)
Collategen as first gene therapy drug in Japan to stimulate therapeutic angiogenesis

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Corporate Seminar-II
(Sanofi K. K.)

Date: July 26, 2018, 12:00-13:00, Hall-1
Chairperson: Fumio Endo
(*Kumamoto Ezuko Ryoiku Medical Center*)

Hideki Muramatsu (*Nagoya University Graduate School of Medicine*)
Newborn mass screening for severe combined immunodeficiency (SCID)

Torayuki Okuyama (*National Center for Child Health and Development*)
Early diagnosis for LSDs

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Corporate Seminar-III
(Gene Therapy Research Institution Co., Ltd.)

Date: July 26, 2018, 12:00-13:00, Hall-2
Chairperson: Shin-ichi Muramatsu
(*Jichi Medical University, The Institute of Medical Science, The University of Tokyo, Gene Therapy Research Institution Co., Ltd.*)

Shin Kwak (*Gene Therapy Research Institution Co., Ltd., The University of Tokyo, and Tokyo Medical University*)
Possible application of gene therapy to sporadic neurological diseases - ALS as a prototype -

▶Day II

Corporate Seminar-IV
(Miltenyi Biotec)

Date: July 27, 2018, 12:00-13:00, Main Hall
Chairperson: Keiya Ozawa
(*Jichi Medical University*)

Hiroshi Shiku（*Mie University Graduate School of Medicine*）
Adoptive cell therapy with gene-engineered T-cells - Specificity, construct, and cells –

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▸Day II

Corporate Seminar-V
(SHIMADZU CORPORATION)

Date: July 27, 2018, 12:00-13:00, Hall-1
Chairperson: Kenzaburo Tani
(*The Institute of Medical Science, The University of Tokyo*)

Shin Kawamata (*Research and Development Center for Cell Therapy, Foundation for Biomedical Research and Innovation*)
A new quality assurance method for cell manufacturing through in-process monitoring.

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Evening Seminar
(Takara Bio Inc.)

Date: July 27, 2018, 18:20-18:50, Main Hall
Chairperson: Junichi Mineno
(*Takara Bio Inc.*)

Masanobu Kimura (*Takara Bio Inc.*)
Current clinical development of gene and cellular therapy in Japan

▸Day III

Corporate Seminar-VI
(Takara Bio Inc.)

Date: July 28, 2018, 12:00-13:00, Main Hall
Chairperson: Junichi Mineno
(*Takara Bio Inc.*)

Keiya Ozawa (*Jichi Medical University*)
Gene therapy comes of age

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Corporate Seminar-VII
(AnGes, Inc.)

Date: July 28, 2018, 12:00-13:00, Hall-1
Chairperson: Yoshikatsu Eto
(*Advanced Clinical Research Center, Institute for Neurological Disorders*)

Torayuki Okuyama (*National Center for Child Health and Development*)
Novel therapeutic approach in lysosomal storage diseases