

THE NOW & FUTURE OF RNA THERAPEUTICS

Awaji, Japan

Monday, June 19– Friday, June 23, 2023

Monday	5:30 pm – 8:00 pm	Welcome Remarks 1 Opening Keynote Session
Monday	8:00 pm – 10:00 pm	<i>Dinner & Sake Barrel Breaking</i>
Tuesday	9:30 am – 12:10 pm	2 RNA Targeting Mechanisms and Applications
Tuesday	Lunchtime	Parallel Mentoring Workshops for Graduate Students and Post Docs
Tuesday	2:00 pm – 6:15 pm	3 RNAs and Disease Targets or Drugs
Tuesday	7:15 pm – 9:30 pm	Poster Session
Tuesday	9:30 pm – 10:30 pm	<i>Social Hour</i>
Wednesday	9:30 am – 12:15 pm	4 Development of Small RNA (siRNA, LNA, PNA, ASO) Drugs
Wednesday	1:30 pm – 6:00 pm	<i>Excursion to Hemeji Castle</i>
Wednesday	7:00 pm – 9:40 pm	5 RNA Delivery
Thursday	9:30 am – 12:00 pm	6 Genome Editing
Thursday	2:00 pm – 4:45 pm	7 RNA Chemistry Closing Remarks
Thursday	6:30 pm – 9:30 pm	Cocktails and Banquet
Friday		<i>Departure</i>

Awaji Yumebutai Conference Center

Meeting venue: Main Hall, 2nd floor of the Conference Center
Poster session: Reception Hall B Foyer, 2nd floor of Conference Center
CSHA office: Room 202

*Breakfast**: Coccolare, 2nd floor of Grand Nikko Awaji
Dinner (June 19 - 21): Reception Hall B, 2nd floor of Conference Center
Lunch (June 20-22): Reception Hall B, 2nd floor of Conference Center

Cocktails (June 22): Cielo, 1st floor of Grand Nikko Awaji
Banquet (June 22): Stella, 1st floor of Grand Nikko Awaji

***Only available for guests staying at Grand Nikko Awaji**

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PROGRAM

MONDAY, June 19—5:30 PM

Welcome Remarks

CSH-Asia and Meeting Organizers

Marco Mangone

Arizona State University, USA

SESSION 1 KEYNOTE SESSION: RNA FROM BIRTH TO DEATH /
FROM BASIC TO TRANSLATIONAL

Chairperson: **Mikiko Siomi**, University of Tokyo, Tokyo, Japan

Introduction to the CSHA Ken-Ichi Arai auditing program

Hisao Masai [35'+10']

Presenter affiliation: Tokyo Metropolitan Institute of Medical Science,
Tokyo, Japan.

KEYNOTE SPEAKERS

**Molecular pathogenesis and potential therapy for RNA
modopathy**

Tsutomu Suzuki [35'+10']

Presenter affiliation: University of Tokyo, Tokyo, Japan.

1

**Nonsense-mediated mRNA decay is misregulated in Fragile X
Syndrome**

Lynne E. Maquat [35'+10']

Presenter affiliation: University of Rochester, Rochester, New York.

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MONDAY, June 19—8:00 PM

Dinner and Sake Barrel Breaking

TUESDAY, June 20—9:30 AM

SESSION 2 RNA TARGETING MECHANISMS AND APPLICATIONS

Chairpersons: **Yuanchao Xue**, Chinese Academy of Sciences, Beijing, China
Ligang Wu, Shanghai Institute of Biochemistry and Cell Biology, Shanghai, China

Mapping RNA interactome to annotate the molecular function of risk variants

Yuanchao Xue [20'+10']

Presenter affiliation: Institute of Biophysics, Chinese Academy of Sciences, Beijing, China.

3

Regulation of immune cell differentiation and activation by ASOs targeting mRNA structures recognized by Regnase-1-related RNases

Osamu Takeuchi [20'+10']

Presenter affiliation: Kyoto University, Kyoto, Japan.

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Break

gRNA dependent and gRNA independent off-target binding activities of dCas13

Huanhuan Feng, Zhixue Li, Hongdao Zhang, Ligang Wu [20'+10']

Presenter affiliation: Shanghai Institute of Biochemistry and Cell Biology, Shanghai, China.

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In vivo self-assembled small RNAs as a new generation of RNAi therapeutics

Chen-Yu Zhang [20'+10']

Presenter affiliation: Nanjing University, Nanjing, China.

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The piRNA pathway in Drosophila—How does the molecular machinery protect the genome from transposon invasion in the germline?

Mikiko C. Siomi [15'+5']

Presenter affiliation: The University of Tokyo, Tokyo, Japan.

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TUESDAY, June 20—Lunchtime

**Parallel Mentoring Workshops
for Graduate Students and Post Docs**

SESSION 3 RNAs AS DISEASE TARGETS OR DRUGS

Chairpersons: **Vinod Balachandran**, Memorial Sloan Kettering Cancer Center, New York, New York, USA
Jinzhong Lin, Fudan University, Shanghai, China

Pancreatic cancer—Exceptional survivors to RNA vaccines

Vinod P. Balachandran [20'+10']

Presenter affiliation: Memorial Sloan Kettering Cancer Center, New York, New York.

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KEYNOTE SPEAKER

Targeting RNA modification and RNA-RNA interaction for future therapies

Chuan He [35'+10']

Presenter affiliation: The University of Chicago, Chicago, Illinois.

9

RNA regulation in spermatogenesis and male infertility

Mo-Fang Liu [15'+5']

Presenter affiliation: Shanghai Institute of Biochemistry and Cell Biology, Shanghai, China.

10

Contribution and therapeutic implications of retroelement insertions in genetic diseases

Boxun Zhao, E. Alice Lee, Timothy W. Yu [15'+5']

Presenter affiliation: Boston Children's Hospital, Boston, Massachusetts.

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Break

Single-cell stereo-seq reveals induced progenitor cells involved in axolotl brain regeneration

Ying Gu [20'+10']

Presenter affiliation: BGI, Shenzhen, China.

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Splice modulating RNA targeting therapies for brain disorders: from treating many to treating one

Willeke M. van Roon-Mom [20'+10']

Presenter affiliation: Leiden University Medical Center, Leiden, the Netherlands.

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Transcriptome changes and new therapeutic targets during the initiation and progression of Duchenne Muscular Dystrophy in *C. elegans*

Heather C. Hrach, Shannon O'Brien, Hannah S. Steber, Jason Newbern, Alan Rawls, Marco Mangone [15'+5']
Presenter affiliation: The Biodesign Institute at Arizona State University, Tempe, Arizona.

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Nonsense-mediated RNA decay is a unique vulnerability of cancer cells with spliceosome gene mutations

Abigael Cheruiyot, Claudia Cabrera, Shan Li, Zheng Yang, Sridhar N. Srivatsan, Tanzir Ahmed, Yiwei Fu, Julie M. Bailis, Matthew J. Walter, Zhongsheng You [15'+5']
Presenter affiliation: Washington University in St. Louis, St. Louis, Missouri.

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mRNA selective regulation of translation nominates eIF3 as a drug target in cancer

Haoran Duan, Dieter A. Wolf [15'+5']
Presenter affiliation: Westlake University, Hangzhou, China.

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TUESDAY, June 20—7:15 PM

POSTER SESSION

Odd numbers present 7:15 pm – 8:15 pm

Even numbers present 8:15 pm – 9:30 pm

Reducing off-target effects of siRNA by introducing 2'-formamide modification in the seed region

Seongjin An, Yoshiaki Kobayashi, Kohei Nomura, Yasuaki Kimura, Hiroshi Abe, Dai Akase, Misako Aida, Kumiko Ui-Tei
Presenter affiliation: The University of Tokyo, Chiba, Japan.

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Characterization of circular RNAs in the regulation of inflammatory responses

Shuya Hiroki, Daisuke Ori, Taro Kawai
Presenter affiliation: Nara Institute of Science and Technology (NAIST), Ikoma, Japan.

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Synthetic circular mRNA engineering for cell-type-specific translational regulation

Shigetoshi Kameda, Hirohisa Ohno, Hiroki Tanaka, Hidetaka Akita, Hirohide Saito

Presenter affiliation: Center for iPS Cell Research and Application, Kyoto University, Kyoto, Japan.

19

DNA damage stress-induced translocation of mutant FUS proteins into cytosolic granules and screening for translocation inhibitors

Masahiro Nogami, Osamu Sano, Keiko Adachi-Tominari, Yoshika Hayakawa-Yano, Takako Furukawa, Hidehisa Iwata, Kazuhiro Ogi, Hideyuki Okano, Masato Yano

Presenter affiliation: Takeda Pharmaceutical Company Limited, Fujisawa, Japan.

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Development of single nucleotide mutation-distinguishable siRNA for PIK3CA (SNPD-siPIK3CA) and its effects on cancer cells

Toshinori Ohyama, Yoshiaki Kobayashi, Susumu Goyama, Kumiko Ui-Tei

Presenter affiliation: The University of Tokyo, Tokyo, Japan.

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Welcome to Join Zhejiang University RNA Medical Center

Hak Park, Xiaoning Sun, Xin Li

Presenter affiliation: Zhejiang University School of Medicine, Yiwu, Zhejiang, China.

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Identification of the factors required for nuclear retention of overexpressed mRNAs

Xiangchuan Wang, Hongjie Zhang

Presenter affiliation: University of Macau, Macau, China.

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Integrated database for RNA-targeting drug discovery

Chao Zeng, Michiaki Hamada

Presenter affiliation: Waseda University, Tokyo, Japan; National Institute of Advanced Industrial Science and Technology, Tokyo, Japan.

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DecoyFinder—Identification of decoy sequences in sets of homologous RNA sequences

Mingyi Zhu, Jeffrey Zuber, Zhen Tan, Gaurav Sharma, David H. Mathews

Presenter affiliation: University of Rochester Medical Center, Rochester, New York.

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Social Hour

WEDNESDAY, June 21—9:30 AM

SESSION 4 DEVELOPMENT OF SMALL RNA (siRNA, LNA, PNA, ASO) DRUGS

Chairperson: **Isabel Aznarez**, Stoke Therapeutics, Bedford, Massachusetts

A novel RNA-based approach to treat genetic diseases via upregulation of protein expression

Isabel Aznarez [20'+10']

Presenter affiliation: Stoke Therapeutics, Bedford, Massachusetts. 26

Development of new molecular technologies for siRNA therapeutics

Takeshi Wada [20'+10']

Presenter affiliation: Tokyo University of Science, Noda, Japan. 27

A framework for individualized splice-switching oligonucleotide therapy

Jinkuk Kim, Timothy Yu [15'+5']

Presenter affiliation: KAIST, Daejeon, South Korea. 28

Break

KEYNOTE SPEAKER

Designer DNA drug therapy for neurodegenerative disease

Don W. Cleveland [35'+10']

Presenter affiliation: University of California at San Diego, La Jolla, California. 29

Leveraging the REST-PTB axis to develop a therapeutic strategy for brain repair

Xiang-Dong Fu [15'+5']

Presenter affiliation: Westlake University, Hangzhou, China. 30

WEDNESDAY, June 21—1:30 PM

Excursion to Hemeji Castle

SESSION 5 RNA DELIVERY

Chairperson: **Zicai Liang**, Ribo Life Science Co. Ltd., Beijing, China

siRNA therapeutics as the third wave of modern pharmaceutical industry may profoundly change treatment of many chronic diseases

Zicai Liang, Sam Ma, Shan Gao, Li-Ming Gan, Hongyan Zhang
[20'+10']

Presenter affiliation: Ribo Life Science Co. Ltd., Beijing, China.

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An impact of helper lipids on extrahepatic delivery of nucleic acids based on ionizable cationic lipids

Hideyoshi Harashima, Mahmoud Younis, Seigo Kimura, Yusuke Sato
[20'+10']

Presenter affiliation: Hokkaido University, Sapporo, Japan.

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Revolutionary gene therapy—AAV9 delivers hope for SMA and ALS treatment through miRNA delivery

Jun-An Chen [15'+5']

Presenter affiliation: Academia Sinica, Taipei, Taiwan.

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Broadly protective COVID-19 mRNA vaccine—Beyond the spike protein

Renee Hajnik, Haitao Hu [15'+5']

Presenter affiliation: University of Texas Medical Branch, Galveston, Texas.

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Break

The potential role of mRNA decay in transcription

Hanae Sato, Robert H. Singer [15'+5']

Presenter affiliation: Kanazawa University, Kanazawa, Japan; Albert Einstein College of Medicine, Bronx, New York.

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Broad-spectrum mRNA vaccine candidates against SARS-Cov-2 variants

Jinzhong Lin [15'+5']

Presenter affiliation: Fudan Center for mRNA Translational Research, Shanghai, China.

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SESSION 6 GENOME EDITING

Chairperson: **Qiangfeng Cliff Zhang**, Tsinghua University, Beijing, China

SmrtNet: Predicting RNA-targeting small molecules using deep neural network

Yuhan Fei, Jiasheng Zhang, Pengfei Wang, Jianbo Ma, Zhuoer Dong, Zilin Cai, Qiangfeng C. Zhang [20'+10']

Presenter affiliation: Tsinghua University, Beijing, China; Tsinghua-Peking Center for Life Sciences, Beijing, China.

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Structure, function and engineering of the type III-E CRISPR nuclease-protease complex

Hiroshi Nishimasu [20'+10']

Presenter affiliation: University of Tokyo, Tokyo, Japan.

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Single-base precision Cas13 enables systematic silencing of oncogenic fusions

Wenxin Hu, Amit Kumar, Teresa Sadras, Joseph Trapani, Paul G. Ekert, Mohamed Fareh [15'+5']

Presenter affiliation: Peter MacCallum Cancer Centre, Melbourne, Australia; The University of Melbourne, Melbourne, Australia.

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Break

How CRISPR-Cas system defend against RNA bacteriophage

Jiuyu Wang, Liang Liu, Lilan You, Yanli Wang [20'+10']

Presenter affiliation: Institute of Biophysics, Chinese Academy of Sciences, Beijing, China.

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Targeting the oncogenic protein YB-1 in glioblastoma by RNA decoy oligonucleotides

Jin-Zhu Wang, Hong Zhu, Pu You, Hui Liu, Wei-Kang Wang, Liusong Yang, Zefeng Wang, Jingyi Hui [15'+5']

Presenter affiliation: Chinese Academy of Sciences, Shanghai, China.

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THURSDAY, June 22—2:00 PM

SESSION 7 RNA CHEMISTRY

Chairperson: Chengqi Yi, Peking University, Beijing, China

CRISPR-free programmable RNA pseudouridylation to suppress premature termination codons

Chengqi Yi [20'+10']

Presenter affiliation: Peking University, Beijing, China.

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Pum1 and Pum2 in embryonic stem cells are essential for neural spatial patterning and germ cell inhibition

Ting Lu, Shuo Shi [15'+5']

Presenter affiliation: Shanghai Advanced Institute of Immunochemical Studies, Shanghai, China.

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Centromere transcription and centromere RNAs—Their roles, regulation and separation of function during centromere establishment and maintenance

Jing Zhu, Hiroshi KIMURA, Karen Wing Yee YUEN [15'+5']

Presenter affiliation: The University of Hong Kong, Hong Kong.

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Break

High throughput discovery of ligandable RNA structures in the human transcriptome

Lee E. Vandivier, Luis Soares, Lu Han, Alexandra East-Seletsky, Helene Chamberlin, Lindsay Boudreau, Anthony Montibello, Alex Sexton, Herschel Mukherjee, Jennifer C. Petter, Dave Mauger, J. Craig Blain, Kathleen E. McGinness [15'+5']

Presenter affiliation: Arrakis Therapeutics, Waltham, Massachusetts.

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How the tRNA m⁵C methyltransferases-NSUN2 and NSUN6-recognize and catalyze mRNA substrates

Cai-Tao Li, Yuan-Yuan Zhang, Ru-Juan Liu [15'+5']

Presenter affiliation: ShanghaiTech University, Shanghai, China.

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Closing Remarks

Organizing Committee

THURSDAY, June 22—6:30 PM

COCKTAILS and BANQUET