

Abstracts of papers presented
at the 2025 Cold Spring Harbor Asia Conference

THE NOW AND FUTURE OF RNA THERAPEUTICS

June 9–June 13, 2025

Arranged by

Xiang-Dong Fu, *Westlake University*

Mofang Liu, *Shanghai Institute of Biochemistry & Cell Biology, CAS*

Marco Mangone, *Arizona State University*

Lynne Maquat, *University of Rochester Medical Center*

Mikiko Siomi, *Tokyo University*



Cold Spring Harbor Conferences Asia
Cold Spring Harbor Laboratory



THE NOW AND FUTURE OF RNA THERAPEUTICS

Monday, June 9 – Friday, June 13, 2025

Monday	7:00 pm	Opening Remarks 1 Keynote Session
Tuesday	9:00 am	2 RNA Targeting Mechanisms and Applications
Tuesday	2:00 pm	Poster Session
Tuesday	3:00 pm	<i>Chinese Tea and Beer Tasting</i>
Tuesday	7:00 pm	3 RNAs as Disease Targets or Drugs
Wednesday	9:00 am	4 Development of Small RNA (siRNA, LNA, PNA, ASO) Drugs
Wednesday	11:30 am	Meet an Editor
Wednesday	1:30 pm	<i>Visit to Old Suzhou*</i>
Wednesday	7:00 pm	5 RNA Delivery
Thursday	9:00 am	6 Genome Editing
Thursday	2:00 pm	7 RNA Chemistry I
Thursday	5:00 pm	<i>Cocktails and Banquet</i>
Friday	9:00 am	8 RNA Chemistry II

Oral presentation sessions are located in the CSHA Auditorium
Poster session and Chinese Tea & Beer Tasting are in the Lake Front Hall.
Cocktail social hour is held outside in the Suz Garden.

Old Suzhou visits depart from the CSHA lobby

**optional tour requires additional fee.*

Meal locations and times are as follows:

Lunch: Main Cafeteria 12:00pm - 1:30pm

Dinner: Main Cafeteria 6:00pm - 7:30pm

Banquet: Suz Garden 6:00pm

More information will be available at CSHA office.

(Map at the end of this abstract book)

PROGRAM

MONDAY, June 9—7:00 PM

OPENING REMARKS The Organizing Committee

SESSION 1 KEYNOTE SESSION

Introduction by: Mikiko Siomi, The University of Tokyo, Tokyo, Japan

Immune regulation via the control of mRNA decay

Osamu Takeuchi [35'+10']

Presenter affiliation: Kyoto University, Kyoto, Japan.

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Introduction by: Lynne Maquat, University of Rochester Medical Center,
New York, New York, USA

Translating translation in neurodegeneration

Giovanna Mallucci [35'+10']

Presenter affiliation: Altos Labs, Cambridge, United Kingdom.

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TUESDAY, June 10—9:00 AM

SESSION 2 RNA TARGETING MECHANISMS AND APPLICATIONS

Chairpersons: **Ligang Wu**, Shanghai Institute of Biochemistry and Cell
Biology, Chinese Academy of Sciences, Shanghai, China
Jinzhong Lin, Fudan University, Shanghai, China

Chemically modified single-stranded Ago2-processed interfering RNA mediates efficient gene silencing in vivo

Hongdao Zhang, Shiqi Gao, Zhixue Li, Ligang Wu [20'+10']

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

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Functional insights into viral tRNA-like structures and their application in mRNA design

Guoliang Lu, Ye Li, Liming Wan, Yuchen Chen, Jinzhong Lin [20'+10']

Presenter affiliation: Fudan University, Shanghai, China.

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Morphological stress granule profiling among RNA-binding proteins for identifying distinctive features and localization patterns

Patrick Louis L. Hilario, Yuma Hanai, Katsutomo Okamura [10'+5']

Presenter affiliation: Nara Institute of Science and Technology, Ikoma City, Nara, Japan.

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PB-scope—Contrastive learning of dynamic processing body formation reveals undefined mechanisms of approved compounds

Dexin Shen, Qionghua Zhu, Xiquan Pang, Liang Fang, Wei Chen, Tatsuhisa Tsuboi [10'+5']

Presenter affiliation: Tsinghua University, Shenzhen, China.

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Break

Chairperson: **Yuanchao Xue**, Chinese Academy of Sciences, Beijing, China

Iterative chemoresistance induction combined with enriched RIC-seq uncovers IncATRIG-mediated DNA damage protection in glioma

Yuanchao Xue [20'+10']

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

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Harnessing miRNAs in RNA-based therapeutics: Strategies, standards, and successes

Marco Mangone [20'+10']

Presenter affiliation: The Biodesign Institute at Arizona State University, Tempe, Arizona.

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Molecular mechanisms of hypothermia-mediated RNA editing and neuroprotection

Shuhan Shen, Qiaojin Lin [10'+5']

Presenter affiliation: The Hong Kong University of Science and Technology (Guangzhou), Guangzhou, China.

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POSTER SESSION

PNPase localizes bound mRNA to mitochondrial surface and increases protein synthesis

Lan Cheng, Tatsuhisa Tsuboi

Presenter affiliation: Tsinghua University, Shenzhen, China.

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Structural based optimization of a dual-antigen mRNA vaccine confers broad-spectrum protection against henipaviruses

Binhe Du, Jing Lu, Jinzhong Lin

Presenter affiliation: Fudan University, Shanghai, China.

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BH3 domain-mediated mRNA therapy synergistically enhance antitumor immunity with killing T cells

Jiayan Fu, Yongfeng Jin

Presenter affiliation: College of Life Sciences, Zhejiang University, Hangzhou, China.

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A universal strategy of anti-tumor mRNA vaccine by harnessing “off-the-shelf” immunity

Jiayan Fu, Yongfeng Jin

Presenter affiliation: College of Life Sciences, Zhejiang University, Hangzhou, China.

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Decoding transcription start sites from 5' single-cell RNA-seq reveals a diverse initiation landscape across human tissues

Tan Wu, Huijuan Xu, Qiheng Qian, Cheng Peng, Jingfa Xiao, Yajing Hao

Presenter affiliation: Beijing Institute of Genomics, Chinese Academy of Sciences/China National Center for Bioinformation, Beijing, China.

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Study mRNA translation initiation mechanism with an *in vitro* ePURE system

Li Li, Jing Yao, Xi Lv, Jing H. Zhao, Yan Liu, Ye Li, Zhao Xue, Zhao L. Yu, Yu C. Gu, Jin Z. Lin

Presenter affiliation: Fudan University, Shanghai, China.

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MIWI/piRNAs are programmed to enter the nucleus to drive continuous meiotic progression of male germ cells

Zhi-Tong Li, Mo-Fang Liu

Presenter affiliation: Hangzhou Institute for Advanced Study, UCAS, Hangzhou, China; Shanghai Institute of Biochemistry and Cell Biology, UCAS, Shanghai, China.

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NSun7 regulates sperm flagellum assembly in a manner independent of RNA:m5C modification

Jing Li, Wen-Yu Zhu, Zhen Wang, Lin Lin, Ru-Juan Liu

Presenter affiliation: ShanghaiTech University, Shanghai, China.

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HSP90 α -mediated TDRD9 stabilization is essential for male fertility

Duo Pan, Mo-fang Liu

Presenter affiliation: Hangzhou Institute for Advanced Study, UCAS, Hangzhou, China; Shanghai Institute of Biochemistry and Cell Biology, CAS, Shanghai, China.

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***In vitro* reconstitution of human mRNA translation for studies of mammalian translation regulation**

Li Li, Jing Yao, Xi Lv, Jinghua Zhao, Yan Liu, Ye Li, Zhao Xue, Zhaoli Yu, Yuchen Gu, Jinzhong Lin

Presenter affiliation: Fudan University, Shanghai, China.

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siRNA potency reinforcement through 5'-end uridine rational designs based on hsAGO2-MID domain binding pocket

Yuqi Yao, Xinming Qi, Jin Ren, Jinbiao Ma

Presenter affiliation: Fudan University, Shanghai, China.

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Defining the ecosystem and molecular mechanisms of refractory and relapsed acute myeloid leukemia using scRNA-seq

Jianbiao Zhou, Henry Yang, Nicole Xin-Ning Tang, Sabrina Hui-Min Toh, Yi Chai, Keir Audrey Mendoza Bono, Jun Rong Cuithbert Lim, Melissa Ooi, Wee Joo Chng

Presenter affiliation: National University of Singapore, Singapore; Yong Loo Lin School of Medicine, NUS, Singapore.

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Modification of RNAs in natural rG4 (MoRNiNG), a database of RNA modification sites associated with the dynamic of RNA secondary structures

Yicen Zhou, Shanxin Lyu, Xi Mou, Shiau Wei Liew, Ian Hoffecker, Jian Yan, Chun Kit Kwok, Jilin Zhang

Presenter affiliation: City University of Hong Kong, Hong Kong, China.

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TUESDAY, June 10—3:00 PM

Chinese Tea and Beer Tasting

TUESDAY, June 10—7:00 PM

SESSION 3 RNAs AS DISEASE TARGETS OR DRUGS

Chairperson: **Martine Simonelig**, Institute of Human Genetics – CNRS, Montpellier, France

mRNA regulation by RNA condensates

Anne Ramat, Ali Haidar, Martine Simonelig [20'+10']

Presenter affiliation: Institute of Human Genetics, Montpellier, France. 23

Mechanism underlying progressive disruption of neuromuscular junctions in amyotrophic later sclerosis

Shuyuan Qi, Weidong Xiong, Chengyong Shen, Xiaorong Zhang,

Xiang-Dong Fu [20'+10']

Presenter affiliation: Westlake University, Hangzhou, China. 24

Recognition of RNA secondary structures with a programmable peptide nucleic acid based platform

Gang Chen [10'+5']

Presenter affiliation: The Chinese University of Hong Kong, Shenzhen (CUHK-Shenzhen), Shenzhen, China. 25

RNA binding proteins screening and regulatory mechanisms of mRNA translation on mitochondrial surface in yeast

Jing Zhu, Tatsuhisa Tsuboi [10'+5']

Presenter affiliation: Tsinghua University, Shenzhen, China. 26

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

Chairperson: **Yihan Wan**, Westlake University, Hangzhou, China

Systematic nascent RNA kinetics imaging unveils principles for genetic information flow in real-time

Danlin Xie, Xiangyu Wu, Lingling Li, Rui Sun, Hongjian Qi, Yangye Zhang, Jiahang Xiong, Longxi Li, Tianxin Zhang, Manshu Ye, Xibo Ma, Yanxiao Zhang, Yihan Wan [20'+10']

Presenter affiliation: Westlake University, Hangzhou, China. 27

Treatments against miR-320 for NAFLD

Yufei Zhou, Chen Chen [10'+5']

Presenter affiliation: Tongji Hospital, Wuhan, China. 28

SNORD80-guided 2'-O-methylation stabilizes the lncRNA GAS5 to regulate cellular stress responses

Liang Xu, Xiao Hong Zhao, Xu Dong Zhang, Lei Jin [10'+5']

Presenter affiliation: The University of Newcastle, Newcastle, Australia; Zhengzhou University, Zhengzhou, China. 29

Break

Chairperson: **Michelle Hastings**, University of Michigan, Ann Arbor, Michigan, USA

Splice-switching antisense oligonucleotides as medicines for neurological disease

Michelle L. Hastings [20'+10']

Presenter affiliation: University of Michigan, Ann Arbor, Michigan. 30

Synthetic macrolides overcoming MLS_BK-resistant pathogens

Congxuan Ma, Ye Li, Wentian Liu, Jian Li, Xiaotong Pei, Jinzhong Lin, Jianhua Liang [10'+5']

Presenter affiliation: Fudan University, Shanghai, China. 31

Leveraging type III CRISPR for programmable and flexible RNA excision editing
Rui Zhang [10'+5']
 Presenter affiliation: Sun Yat-Sen University, Guangzhou, China. 32

Meet the editors of the RNA Journal
Michelle L. Hastings and Mikiko Siomi [15']

WEDNESDAY, June 11—1:30 PM

Visit to Old Suzhou

WEDNESDAY, June 11—7:00 PM

SESSION 5 RNA DELIVERY

Chairpersons: **Keiji Itaka**, University of Osaka, Osaka, Japan
Andrea Kasinski, Purdue University, West Lafayette, Indiana, USA

mRNA medicine for treating osteoarthritis
Keiji Itaka [20'+10']
 Presenter affiliation: The University of Osaka, Osaka, Japan. 33

From MicroRNA biology to clinical breakthroughs—Advancing microRNAs as anti-cancer agents
Andrea L. Kasinski, Shreyas Iyer, Ozcan Kenan, Anjum Sharjeel, Sohal Ikjot [20'+10']
 Presenter affiliation: Purdue University, West Lafayette, Indiana. 34

Break

Raw signal segmentation for estimating RNA modification from Nanopore direct RNA sequencing data
 Guangzhao Cheng, Aki Vehtari, Lu Cheng [10'+5']
 Presenter affiliation: Aalto University, Espoo, Finland; University of Eastern Finland, Kuopio, Finland. 35

RNA modifications in RNA metabolism, pathogenesis, and therapeutic responses
Yu-Ying He [10'+5']
 Presenter affiliation: University of Chicago, Chicago, Illinois. 36

Harnessing RSV RNA synthesis machinery for advancing RNA therapeutics

Dongdong Cao, Yunrong Gao, Zhenhang Chen, Yuqing Wang, Claire Roesler, Inesh Gooneratne, Cristopher Mera, Bo Liang [10'+5']
Presenter affiliation: Emory University School of Medicine, Atlanta, Georgia.

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THURSDAY, June 12—9:00 AM

SESSION 6 GENOME EDITING

Chairperson: **Polly Chen**, National University of Singapore, Singapore

Reactivating immunity—Targeting tumors via RNA editing

Polly Leilei Chen [20'+10']
Presenter affiliation: National University of Singapore, Singapore. 38

Nonsense-mediated mRNA decay in human health and disease

Lynne E. Maquat [20'+10']
Presenter affiliation: University of Rochester School of Medicine and Den, New York. 39

One round RNA evolution leveraging artificial intelligence informed by biofidelity screening

Yu Wang [10'+5']
Presenter affiliation: Chinese Academy of Sciences, Shenzhen, China. 40

Break

Chairperson: **Makoto Saito**, RIKEN, Wako, Japan

TIGR-Tas—Modular RNA-guided systems with Nop domain-containing proteins

Makoto Saito [20'+10']
Presenter affiliation: RIKEN Pioneer Research Institute, Wako, Saitama, Japan. 41

Mitigating reactive gliosis and neuroinflammation in the brain via gene editing

Platon Megagiannis, Guy Rouleau, Yang Zhou [10'+5']
Presenter affiliation: Montreal Neurological Institute and Hospital, McGill University, Montreal, Canada. 42

The ribonuclease DISL-2 mediates piRNA degradation

Benjamin Pastore, Hannah L. Hertz, Wen Tang [10'+5']

Presenter affiliation: The Ohio State University, Columbus, Ohio. 43

SENCr mediates VSMC-restricted protein synthesis and alleviates atherosclerosis

Yuyan Lyu, Songmao Wang, Ahmed M. Awad, Ahmed R. El-Sheakh, Xudong Duan, Qing R. Lyu [10'+5']

Presenter affiliation: Chongqing University, Chongqing, China. 44

THURSDAY, June 12—2:00 PM

SESSION 7 RNA CHEMISTRY

Chairperson: **Hirohide Saito**, University of Tokyo and Kyoto University, Japan

Synthetic RNA technologies to control gene expression and cell fate

Hirohide Saito [20'+10']

Presenter affiliation: The University of Tokyo, Tokyo, Japan; Kyoto University, Kyoto, Japan. 45

Application of a novel antisense oligonucleotide technology to treat human disease

Yimin Hua [20'+10']

Presenter affiliation: Guangzhou ASOCURA Pharmaceuticals, Guangzhou, China. 46

Epi-transcriptional regulation of stress granule pathology by non-coding RNA methylation

Ge Bai [10'+5']

Presenter affiliation: Zhejiang University, Hangzhou, China. 47

Break

Chairperson: **Chengqi Yi**, Peking University, Beijing, China

Precise RNA targeting and manipulation

Nan Luo, Qiang Huang, Cheng qi Yi [20'+10']

Presenter affiliation: Peking University, Beijing, China. 48

First-in-class modular ribozyme-based biosensor

Chermaine Tan, Mandy Lim, Charannya Subhramanyam, Shi Jie Teo, Sherry Aw [10'+5']

Presenter affiliation: Agency for Science, Technology and Research, Singapore. 49

Co-administration with transportan peptide improves the delivery of oligonucleotides and genes into mammalian cells

Hongbo Pang [10'+5']

Presenter affiliation: University of Minnesota, Minneapolis, Minnesota. 50

Mitochondrial dsRNA—A hidden source of immunogenic RNA in ADAR1 deficiency

Haoqing Shen, Vincent Tano, Jian Han, Vanessa Hui En Ng, Leilei Chen [10'+5']

Presenter affiliation: Cancer Science Institute of Singapore, Singapore. 51

THURSDAY, June 12—5:00 PM

COCKTAILS and BANQUET

FRIDAY, June 13—9:00 AM

SESSION 8 RNA CHEMISTRY II

Chairpersons: **Bai Rui**, Westlake University, Hangzhou, China
Shi-Mei Zhuang, Sun Yat-sen University, Guangzhou, China

Visualizing the minor spliceosome—Unveiling the mystery of U12-type pre-mRNA splicing

Rui Bai [20'+10']

Presenter affiliation: Westlake University, Hangzhou, China. 52

RNA regulation in male germ cells and human male infertility

Mo-Fang Liu [20'+10']

Presenter affiliation: Shanghai Institute of Biochemistry and Cell Biology, Chinese Academy of Sciences, Shanghai, China. 53

Functional analysis of essential RNA-binding proteins in mouse spermatogenesis

Soichiro Yamanaka, Mikiko C. Siomi [20'+10']

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

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Break

Chairpersons: **Fan Lai**, Yunnan University, Kunming, China

Ru-Jian Liu, ShanghaiTech University, Shanghai, China

hMTR4 enhances p53 protein degradation and promotes tumor growth by accelerating rRNA processing and regulating RPL5-MDM2 axis

Chen Xie, Xin-Ling Liang, Bin Chen, Sui Chen, Fang-Xin Xiong, Zhan-Li Chen, Qi-Cheng Wang, Feng-Jun He, Shi-Mei Zhuang [20'+10']

Presenter affiliation: Sun Yat-sen University, Guangzhou, China.

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The molecular mechanism of RNase H1-dependent ASO targeting

Fan Lai, Shunkai Chen, Yunkun Dang [20'+10']

Presenter affiliation: Yunnan University, Kunming, China.

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Ultra-sensitive sequencing uncovers extensive tRNA dynamics during cell differentiation and guides therapeutic tRNA design

Hao Li, You-Jia Zhou, Zi-Ce Zhou, Jian-Yang Ge, Ru-Juan Liu [20'+10']

Presenter affiliation: ShanghaiTech University, Shanghai, China.

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CLOSING REMARKS
The Organizing Committee