# GENOME EDITING: ALL THINGS CONSIDERED

November 18-November 22, 2024

Arranged by

Jens Boch, Leibniz University Hannover Dana Carroll, University of Utah Jin-Soo Kim, National University of Singapore Haoyi Wang, Institute of Zoology, CAS Wensheng Wei, Peking University



#### **GENOME EDITING: ALL THINGS CONSIDERED**

Monday, November 18- Friday, November 22, 2024

Monday	7:00 pm	1 Mechanistic Studies Related to Genome Editing
Tuesday	9:00 am	2 New Developments in Genome Editing: Methods and Tools
Tuesday	2:00 pm	Poster Session
Tuesday	3:00 pm	Chinese Tea and Beer Tasting
Tuesday	7:00 pm	<b>3</b> High-throughput Screening and Target Discovery
Wednesday	9:00 am	4 Repurposing of Genome Editing
Wednesday	1:30 pm	Visit to Old Suzhou*
Wednesday	7:00 pm	5 Genome Editing in Animals and Plants
Thursday	9:00 am	6 Therapeutic Application
Thursday	2:00 pm	7 Genome Writing and Synthetic Biology
Thursday	5:00 pm	Cocktails and Banquet
Friday	9:00 am	8 All Things Considered

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, November 18—7:00 PM

SESSION 1	MECHANISTIC STUDIES RELATED TO GENOME EDITING	
Chairperson:	Wensheng Wei, Peking University, Beijing, China	
Cas genome ed Martin Jinek [2	udies and structure-based engineering of CRISPR- ditor nucleases and transposons '5'+5'] tion: University of Zurich, Zurich, Switzerland.	1
RNA-guided nu Jun-Jie Gogo Li Presenter affilia		2
approaches an Shin-ichi Arimur	g in plant mitochondria and chloroplasts—Current d applications a, Issei Nakazato [25'+5'] tion: University of Tokyo, Tokyo, Japan.	3
	TUESDAY, November 19—9:00 AM	
SESSION 2	NEW DEVELOPMENTS IN GENOME EDITING: METHODS AND TOOLS	
Chairperson:	Jin-Soo Kim, National University of Singapore	
Samuel H. Sterr	synthesis by an antiviral reverse transcriptase nberg [25'+5'] tion: Columbia University, New York, New York.	4

Sequence modeling and design from molecular to genome scale with Evo	
Eric Nguyen, Michael Poli, Matthew G. Durrant, Brian Kang, Dhruva Katrekar, David B. Li, Liam J. Bartie, Armin W. Thomas, Samuel H. King, Garyk Brixi, Jeremy Sullivan, Madelena Y. Ng, Ashley Lewis, Aaron Lou, Stefano Ermon, Stephen A. Baccus, Tina Hernandez-Boussard, Christopher Ré, Patrick D. Hsu, Brian L. Hie [25'+5'] Presenter affiliation: Arc Institute, Palo Alto, California; University of California, Berkeley, California.	5
Break	
A new RNA base editor repairs nonsense mutations  Chengqi Yi [25'+5']  Presenter affiliation: Peking University, Beijing, China.	6
Amplification editing enables efficient and precise duplication of DNA to chromosomal scale	
Hao Yin [25'+5'] Presenter affiliation: Wuhan University, Wuhan, China.	7
TUESDAY, November 19—2:00 PM	
POSTER SESSION	
An engineered mitoCBE facilitates mitochondrial DNA editing compatibility and edited mitochondrial transfer Jie Liu, <u>Jun Chen</u> , Yunbo Qiao Presenter affiliation: Qilu Hospital, Shandong University, Jinan, China.	8
All-RNA-mediated targeted gene integration in mammalian cells with rationally engineered R2 retrotransposons Yangcan Chen, Shengqiu Luo, Yanping Hu, Bangwei Mao, Xinge Wang, Zongbao Lu, Qi Zhou, Wei Li	
Presenter affiliation: Key Laboratory of Organ Regeneration and Reconstruction, State Key Laboratory of Stem Cell and Reproductive Biology, Beijing, China.	9
Flexible TAM requirement of TnpB enables efficient single- nucleotide editing with expanded targeting scope Xu Feng, Ruyi Xu, Jianglan Liao, Xiaoxue Wang, Wenyuan Han, Qunxin She	
Presenter affiliation: Shandong University, Qingdao, China.	10

CDK11 is a novel therapeutic target in hepatocellular carcinoma Zhijian Kuang, Jack Wong Presenter affiliation: The University of Hong Kong, Hong Kong, China.  A highly efficient direct gene transformation system unlocks genome editing opportunities in oil palm Fong Chin Lee, Norkhairunnisa Che Mohd Khan, Maimon Abdullah, Muhammad Rashdan Muad, Wan Chin Yeap Presenter affiliation: SD Guthrie Technology Centre, Serdang, Malaysia.  Functional profiling of serine, threonine and tyrosine sites Yizhou Li, Tao Xu, Huazheng Ma, Di Yue, Qiezhong Lamao, Ying Liu, Zhuo Zhou, Wensheng Wei Presenter affiliation: Peking University, Beijing, China; Changping Laboratory, Beijing, China.  Enhanced eMAGE applied to identify genetic factors of nuclear hormone receptor dysfunction via combinatorial gene editing Zhuobin Liang, Peter N. Ciaccia, Anabel Y. Schweitzer, Eli Metzner, Farren J. Isaacs Presenter affiliation: Yale University, New Haven, Connecticut; Shenzhen Bay Laboratory, Shenzhen, China.  A nucleic acid detection platform based on DNA sulfur modification Hang Gao, Yuting Shuai, Dini Ma, Anan Xu, Xinyi He, Zixin Deng, Guang Liu	glycosylase variant enables programmable T-to-G and T-to-C base editing	
Zhijian Kuang, Jack Wong Presenter affiliation: The University of Hong Kong, Hong Kong, China.  A highly efficient direct gene transformation system unlocks genome editing opportunities in oil palm Fong Chin Lee, Norkhairunnisa Che Mohd Khan, Maimon Abdullah, Muhammad Rashdan Muad, Wan Chin Yeap Presenter affiliation: SD Guthrie Technology Centre, Serdang, Malaysia.  13  Functional profiling of serine, threonine and tyrosine sites Yizhou Li, Tao Xu, Huazheng Ma, Di Yue, Qiezhong Lamao, Ying Liu, Zhuo Zhou, Wensheng Wei Presenter affiliation: Peking University, Beijing, China; Changping Laboratory, Beijing, China.  14  Enhanced eMAGE applied to identify genetic factors of nuclear hormone receptor dysfunction via combinatorial gene editing Zhuobin Liang, Peter N. Ciaccia, Anabel Y. Schweitzer, Eli Metzner, Farren J. Isaacs Presenter affiliation: Yale University, New Haven, Connecticut; Shenzhen Bay Laboratory, Shenzhen, China.  A nucleic acid detection platform based on DNA sulfur modification Hang Gao, Yuting Shuai, Dini Ma, Anan Xu, Xinyi He, Zixin Deng, Guang Liu Presenter affiliation: Shanghai Jiao Tong University, Shanghai, China.  High fidelity PAMless base editing of hematopoietic stem cells to treat chronic granulomatous disease Linyuan Ma, Vera Bzhilyanskaya, Siyuan Liu, Lauren R. Fox, Madelynn N. Whittaker, Ronald J. Meis, Michelle Ma, Cicera R. Lazzarotto, Shengdar Q. Tsai, Justin Lack, Xiaolin Wu, Gary Dahl, Harry L. Malech, Suk See De Ravin, Benjamin P. Kleinstiver Presenter affiliation: Massachusetts General Hospital, Boston,		11
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treat chronic granulomatous disease Linyuan Ma, Vera Bzhilyanskaya, Siyuan Liu, Lauren R. Fox, Madelynn N. Whittaker, Ronald J. Meis, Michelle Ma, Cicera R. Lazzarotto, Shengdar Q. Tsai, Justin Lack, Xiaolin Wu, Gary Dahl, Harry L. Malech, Suk See De Ravin, Benjamin P. Kleinstiver Presenter affiliation: Massachusetts General Hospital, Boston,	hormone receptor dysfunction via combinatorial gene editing Zhuobin Liang, Peter N. Ciaccia, Anabel Y. Schweitzer, Eli Metzner, Farren J. Isaacs Presenter affiliation: Yale University, New Haven, Connecticut;	15
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Improved strategies for precise gene knock-in with large genomic fragment removal using CRISPR-Cas3 <u>Daiki Nagatomo</u> , Kazuto Yoshimi, Tomoji Mashimo, Tetsushi Sakuma Presenter affiliation: Kyoto University, Kyoto, Japan.	18
Massively parallel interrogation of human functional variants modulating cancer immunosurveillance Ying Liu, Yongshuo Liu, Xuran Niu, Ang Chen, Yizhou Li, Ying Yu, Zhiheng Liu, Tao Xu, Jie Cheng, Zeguang Wu, Wensheng Wei	10
Presenter affiliation: Peking University, Beijing, China.  Evaluation of <i>in vivo</i> genome editing efficiency using a reporter	19
mouse model  Masato Ohtsuka, Jurai Imafuku, Aki Kurosaki, Kenya Kamimura, Yumu Yaku, Keiko Yokoyama, Akihide Kamiya, Haruno Onuma, Yusuke Sato, Channabasavaiah B. Gurumurthy, Hiromi Miura	20
Circular ADAR-recruiting RNA alleviates long-term disease phenotype in Duchenne muscular dystrophy by RNA editing Huixian Tang, Zongyi Yi, Wenting Guo, Raoxian Bai, Shuaiwei Ren, Xiwen Chang, Jiwu Ren, Wei Tang, Ying Yu, Pengfei Yuan, Weizhi Ji, Yongchang Chen, Wensheng Wei Presenter affiliation: Peking University, Beijing, China.	21
Prime editor-based high-throughput screening reveals functional synonymous mutations in the human genome Xuran Niu, Wei Tang, Yongshuo Liu, Ying Liu, Binrui Mo, Ying Yu, Wensheng Wei Presenter affiliation: Peking University, Beijing, China.	22
Identification and validation of DNA barrier elements using a novel high-throughput multifluorescent assay  Aqeel F. Taqi, Illeana Guerrini, Rio Hermantara, Valentine Jeantet, Lura Richmond, Sabari Chilaka, Katherine West, Adam West Presenter affiliation: University of Glasgow, School of Cancer Sciences, Glasgow, United Kingdom; Dasman Diabetes Institute, Kuwait City, Kuwait.	23
Mining and implementation of CRISPR-associated transposases for multiplexed genome editing Siqi Yang, Xiaojie Zhou, Shijie Cai Presenter affiliation: Chinese Academy of Sciences, Shanghai, China.	24

modifications for Lumeng Ye, Ha	single-stranded DNA payloads with multiple for gene and cell therapy studies iiye Sun, Qian Gao, Hong Li ition: GenScript Biotech, Nanjing, China.	25
of mitochondri Xiaoxue Zhang, Zongyi Yi, Wens	mization of mitoBEs empowers the establishment ial disease models, Xue Zhang, Jiwu Ren, Jiayi Li, Xiaoxu Wei, Ying Yu, sheng Wei tion: Changping Laboratory, Beijing, China.	26
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	Chinese Tea and Beer Tasting	
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SESSION 3	HIGH-THROUGHPUT SCREENING AND TARGET DISCOVERY	
Chairperson:	<b>Caixia Gao,</b> Institute of Genetics and Developmental Biology, Chinese Academy of Sciences, Beijing, China	
Screening the Sidi Chen [25'	different dimensions of immunological genome	
	ition: Yale University, West Haven, Connecticut.	27
functional gen Wensheng Wei		28
Genome-wide regulatory eler Yufeng Li, Mink Kip, Shengnan Susan L. Kloet, Michael P. Snyo	Cas9-mediated screening of essential non-coding ments via libraries of paired single-guide RNAs tang Tan, Almira Akkari-Henic, Limin Zhang, Maarten Sun, Jorian J. Sepers, Ningning Xu, Yavuz Ariyurek, Richard P. Davis, Harald Mikkers, Joshua J. Gruber, der, Xiao Li, Baoxu Pang [25'+5'] tition: Leiden University Medical Center, Leiden, the	29

modulating ca Ying Liu, Yongs Zhiheng Liu, Ta [15'+5']	allel Interrogation of human functional variants incer immunosurveillance shuo Liu, <u>Xuran Niu</u> , Ang Chen, Yizhou Li, Ying Yu, ao Xu, Jie Cheng, Zeguang Wu, Wensheng Wei ation: Peking University, Beijing, China.	30
synonymous r Xuran Niu, <u>Wei</u> Wensheng Wei	pased high-throughput screening reveals functional mutations in the human genome [Tang, Yongshuo Liu, Ying Liu, Binrui Mo, Ying Yu, i [15'+5'] ation: Peking University, Beijing, China.	31
	WEDNESDAY, November 20—9:00 AM	
SESSION 4	REPURPOSING OF GENOME EDITING	
Chairperson:	<b>Haoyi Wang</b> , Institute of Zoology, Chinese Academy of Sciences, Beijing, China	
editing Omar O. Abuda Presenter affilia Brigham and W	e technologies for genome and transcriptome  ayyeh, Jonathan S. Gootenberg [25'+5'] ation: Harvard University, Cambridge, Massachusetts;  /omen's Hospital, Harvard Medical School, Boston, ; Beth Israel Deaconess Medical Center, Boston, .	32
Albert W. Chen Presenter affilia Sciences, Beijir Regenerative M	iting cancer therapy  ig [25'+5'] ation: Institute of Zoology, Chinese Academy of ing, China; Beijing Institute for Stem Cell and Medicine, Beijing, China; University of Chinese iences, Beijing, China.	33
Break		
editing Omar O. Abuda Presenter affilia Brigham and W	e technologies for genome and transcriptome  ayyeh, Jonathan S. Gootenberg [25'+5'] ation: Harvard University, Cambridge, Massachusetts; /omen's Hospital, Harvard Medical School, Boston, ; Beth Israel Deaconess Medical Center, Boston, .	34

Yongrong Lai, F Gaohui Yang, L Lan, Yan Zhan Presenter affilia	o treat β-thalassemia Rongrong Liu, Lijie Wang, Xu-Kai Ma, Yaliang Li, Lingling Shi, Jia Wei, Zhenbin Wei, Xuemei Zhou, Kewei g, Xiaodun Mou, Li Yang, Jia Chen [25'+5'] ation: ShanghaiTech University, Shanghai, China; cal Research and Trial Center, China.	35
novel high-through Ageel F. Taqi, I Lura Richmond Presenter affilia	and validation of DNA barrier elements using a oughput multifluorescent assay Illeana Guerrini, Rio Hermantara, Valentine Jeantet, I, Sabari Chilaka, Katherine West, Adam West [15'+5'] ation: University of Glasgow, School of Cancer gow, United Kingdom; Dasman Diabetes Institute, wait.	36
	WEDNESDAY, November 20—1:30 PM	
	Visit to Old Suzhou	
	WEDNESDAY, November 20—7:00 PM	
SESSION 5	GENOME EDITING IN ANIMALS AND PLANTS	
Chairperson:	Dali Li, East China Normal University, Shanghai, China	
applications in Caixia Gao [28] Presenter affilia	on precise genome editing technologies and their n crop improvement 5'+5'] ation: Institute of Genetics and Developmental Biology, amy of Sciences, Beijing, China.	37
Jin-Soo Kim [2	A editing in vitro and in vivo 25'+5'] ation: National University of Singapore, Singapore.	38
of mitochondr Xiaoxue Zhang Zongyi Yi, Wen	mization of mitoBEs empowers the establishment ial disease models , Xue Zhang, Jiwu Ren, Jiayi Li, Xiaoxu Wei, Ying Yu, sheng Wei [15'+5']	39

mouse primar Yangyang Li, Y Zhang [25'+5'	•	
Presenter affiliation: Hefei Comprehensive National Science Center, Hefei, China.		40
	THURSDAY, November 21—9:00 AM	
SESSION 6	THERAPEUTIC APPLICATION	
Chairperson:	<b>Wei Li</b> , Institute of Zoology, Chinese Academy of Sciences, Beijing, China	
innovative cel Matthew H. Po	ation: Stanford University, Stanford Medical School,	41
Dali Li [25'+5'	ng and its clinical applications ] ation: East China Normal University, Shanghai, China.	42
phenotype in Huixian Tang, Ziwen Chang, Yongchang Ch	R-recruiting RNA alleviates long-term disease Duchenne muscular dystrophy by RNA editing Zongyi Yi, Wenting Guo, Raoxian Bai, Shuaiwei Ren, Jiwu Ren, Wei Tang, Ying Yu, Pengfei Yuan, Weizhi Ji, en, Wensheng Wei [15'+5'] ation: Peking University, Beijing, China.	43
Break		
humanized mi therapy	ution of adenine base editors and generation ce: tackling ABE applications for human gene	
Feng Gu [25'- Presenter affilia	եծ լ ation: Hunan Normal University, Changsha, China.	44

hemostasis in r Zhenjie Zhang, X Presenter affiliati Kong SAR, Chin	indiated liver-specific knock-in restored ineonatal hemophilia B mice Kiangjun He, Siqi Zhang, <u>Bo Feng</u> [25'+5'] ion: The Chinese University of Hong Kong, Hong a; Hong Kong Institute of Science & Innovation, by of Sciences, Hong Kong SAR, China.	45
	THURSDAY, November 21—2:00 PM	
SESSION 7	GENOME WRITING AND SYNTHETIC BIOLOGY	
Chairperson:	Hao Yin, Wuhan University, Wuhan, China	
engineered R2 i Wei Li [25'+5'] Presenter affiliati	gene integration via primed micro-homologues and retrotransposons ion: Key Laboratory of Organ Regeneration and Institute of Zoology, Chinese Academy of Sciences,	46
polymerases ar Joana Ferreira d Christopher R. C Benjamin P. Klei Presenter affiliati	—Programmable genome writing using DNA nd HUH endonucleases la Silva, Connor J. Tou, David Rufino-Ramos, cromwell, Madeline Eller, Emily M. King, Linyuan Ma, instiver [25'+5'] ion: Massachusetts General Hospital, Boston, Harvard Medical School, Boston, Massachusetts.	47
with rationally e Yangcan Chen, S Wang, Zongbao Presenter affiliati	ed targeted gene integration in mammalian cells engineered R2 retrotransposons Shengqiu Luo, Yanping Hu, Bangwei Mao, Xinge Lu, Qi Zhou, Wei Li [15'+5'] ion: Key Laboratory of Organ Regeneration and State Key Laboratory of Stem Cell and Reproductive China.	48
Break		
Haoyi Wang [25	f transposon based genome engineering tools 5'+5'] ion: Chinese Academy of Sciences, Beijing, China.	49

editor toolkit Zhichao Qiu, Ke Li, Xiang Lei, R Alessandra Gio	eke Sun, Qingwei Zeng, Ziwei Luo, Xinran Liu, Yaping uilin Zhao, Xuan Wang, Joe Z. Zhang, Xiaolong Wang, rgetti, Zhuobin Liang [25'+5'] tion: Shenzhen Bay Laboratory, Shenzhen, China.	50
Improved strat fragment remo Daiki Nagatomo [15'+5']	regies for precise gene knock-in with large genomic oval using CRISPR-Cas3	51
	THURSDAY, November 21—5:00 PM  COCKTAILS and BANQUET	
	FRIDAY, November 22—9:00 AM	
SESSION 8	ALL THINGS CONSIDERED	
Chairperson:	<b>Zhuobin Liang,</b> Shenzhen Bay Laboratory, Shenzhen, China	
identifies nove Guangming Zha Yan [25'+5']	type I-F cascade-based CRISPRi library screening antibiotic resistance determinants in superbugs ang, Yingtong Su, Xiaoyang Han, Mengyao Guo, Aixin ation: The University of Hong Kong, Hong Kong SAR,	52
systems Peng-Fei Xia, Y	cterial genome editing with non-killing CRISPR-Cas  (ing Wei, Pei-Ru Chen [25'+5']  tion: Shandong University, Qingdao, China.	53

#### Break

Linginical carried with a yatem with improved base calling
efficiency for disease correction via single AAV delivery in mice
Ruochen Guo, Xiaozhi Sun, Feizuo Wang, Dingyi Han, Qiaoxia Yang,
Hua Gao, Zhifang Li, Zhuang Shao, Jinqi Shi, Rongrong Yang, Xiaona
Huo, Junda Yan, Guoling Li, Qingquan Xiao, Yuanhua Liu, Leyun
Wang, Chunyi Hu, Chunlong Xu [25'+5']
Presenter affiliation: Lingang Laboratory, Shanghai, China;
ShanghaiTech University, Shanghai, China; Shanghai Center for Brain
Science and Brain-Inspired Technology, Shanghai, China.

Engineered IscR-WPNA system with improved base editing

## Total solution for efficient cell therapy engineering with non-viral gene knock-in solutions

Lumeng Ye, Fan Zhou, Fei Huang, Hong Li [15'+5']
Presenter affiliation: GenScript Biotech, Nanjing, China.

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