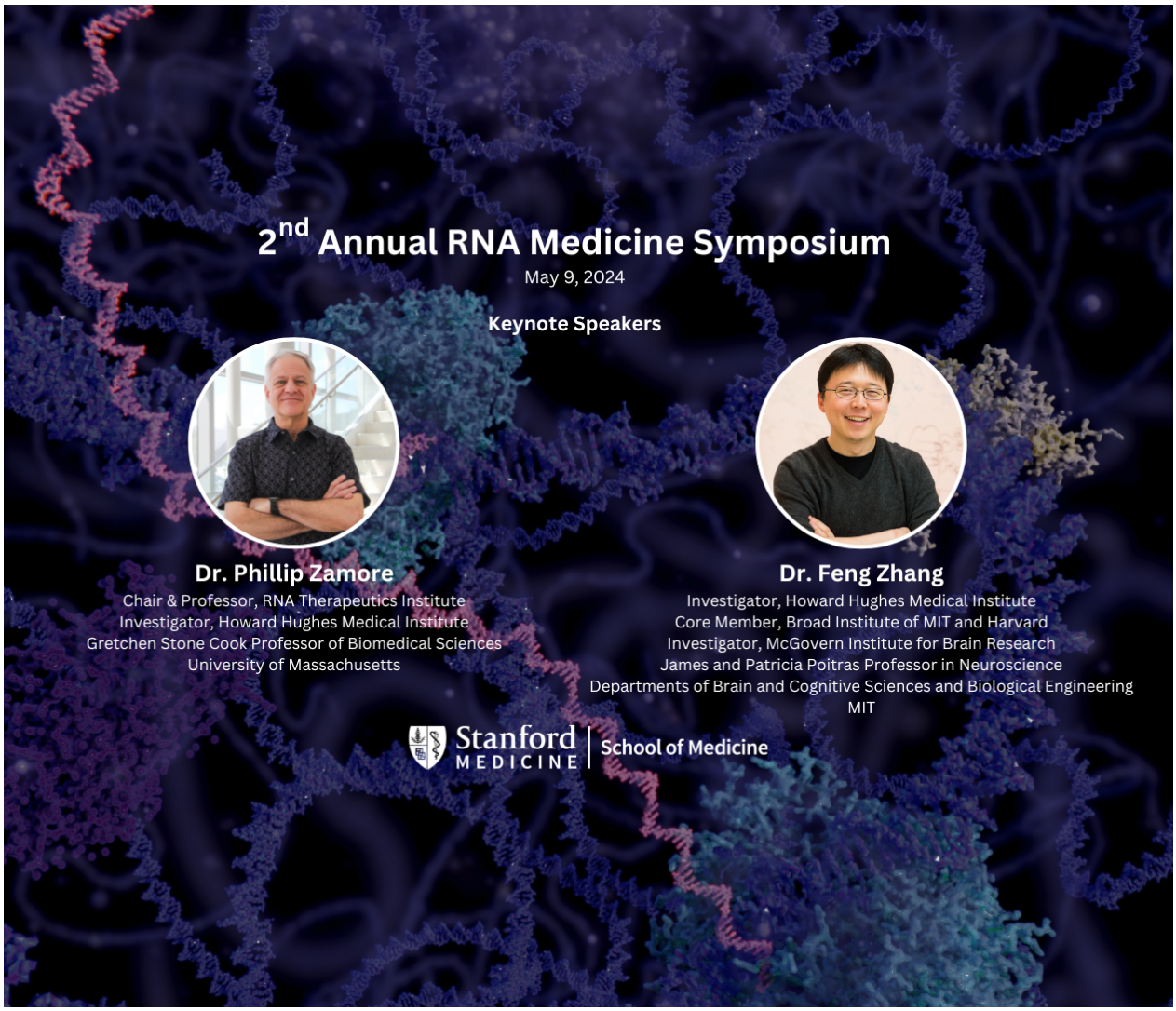

Stanford MEDICINE

School of Medicine

RNA Medicine Program

Thursday, May 9, 2024

8:30-9:00 am — Registration and Breakfast

9:00-9:10 am — **WELCOME AND OPENING REMARKS**

Howard Y. Chang, M.D., Ph.D.
Director, RNA Medicine Program
Virginia and D.K. Ludwig Professor of Cancer Research,
Stanford University School of Medicine

9:10 – 9:50 am — **OPENING KEYNOTE**
Moderated by Howard Y. Chang, M.D., Ph.D.

Phillip D. Zamore, Ph.D.
Chair, RNA Therapeutics Institute, UMass
Investigator- Howard Hughes Medical Institute
Gretchen Stone Cook Professor of Biomedical Sciences
Adventures with the Argonautes

9:50 – 10:50 am — **SESSION 1: RNA DELIVERY**

Niren Murthy, Ph.D.
Professor of Bioengineering, UC Berkeley
Innovative Genomics Institute
Acid degradable lipid nanoparticles deliver mRNA efficiently in vivo to. multiple organs

Netra Unni Rajesh
Ph.D Candidate- DeSimone Lab, Bioengineering, Stanford University
3D-printed Lattice Micro-Array Patches for Transdermal RNA Delivery

Gilles Besin, Ph.D.
CSO, Orbital Therapeutics
The challenges and opportunities in the RNA medicine space

10:50 - 11:10 am — Coffee Break

11:10 – 12:40 pm — **SESSION 2: AI AND RNA BIOINFORMATICS**

Julia Salzman, Ph.D.
Associate Professor of Biomedical Data Science of Biochemistry and by courtesy, of Statistics and Biology, Stanford University.
Ultra-fast reference-free unified genomic discovery with SPLASH

Brian Hie, Ph.D.
Assistant Professor of Chemical Engineering and Data Science, Stanford University
Sequence modeling and design from molecular to genome scale with Evo

Yue Wan, **Ph.D.**
Deputy Executive Director, Genomics Institute of Singapore
Studying RNA structures to understand RNA function

Ron Dror, Ph.D.
Cheriton Family Professor of Computer Science and by courtesy, Molecular and Cellular Physiology and Structural Biology, Stanford University
Machine learning for RNA structure prediction and drug design

Poster-Teaser

12:40 – 1:50 pm Lunch and Round-Table Networking

Phil Zamore (UMass), Feng Zhang (M.I.T.), Gilles Besin (CSO, Orbital Therapeutics), John Pham (Editor, *Cell*), Andrew Lee (Director, Northpond Venture), Ron Shigeta (Advisor for startups), John Dimos (Alexandria Venture), and sponsors.

1:50 -3:10 pm **SESSION 3: RNA TECHNOLOGY**

Silvana Konermann, Ph.D.
Executive Director and Core Investigator, ARC Institute
Assistant Professor of Biochemistry, Stanford University

Michael R. Angelo, Ph.D.
Associate Professor of Pathology, Stanford University
Spatial encodings of tumor function and clinical outcome in breast cancer

Billy Li, Ph.D
Professor of Genetics, Stanford University
RNA Editing: From Biology to Therapy

Stanley Qi, Ph.D.
Associate Professor of Bioengineering and Sarafan ChEM-H, Stanford University
Translational Science and RNA Delivery

3:10 - 3:30 pm Coffee Break

3:30 – 4:30 pm **SESSION 4: RNA THERAPEUTICS**

Howard Y. Chang, M.D., Ph.D.
Director, RNA Medicine Program
Virginia and D.K. Ludwig Professor of Cancer Research,
Stanford University School of Medicine
RNA Origin of sex biased immunity

Judith Frydman, Ph.D.
Professor of Genetics Biology, Stanford University
The making and unmaking of proteome: The Voyage from RNA to folded protein

Peter Sarnow, M.D., Ph.D.
Professor of Microbiology and Immunology, Stanford University
Roles for virus-derived circular RNAs during RNA virus infections

4:30 – 5:20 pm **CLOSING KEYNOTE**
Moderated by Howard Y. Chang, M.D., Ph.D.

Feng Zhang, Ph.D.
Professor of Neuroscience and Biological Engineering, M.I.T.
Exploration of Biological Diversity

5:20 – 6:30 pm **POSTER SESSION AND DRINK RECEPTION**

KEYNOTE SPEAKERS

Phillip D. Zamore, Ph.D.

Chair- RNA Therapeutics Institute, UMass
Investigator- Howard Hughes Medical Institute
Gretchen Stone Cook Professor of Biomedical Sciences

Dr. Zamore received his A.B. (1986) and Ph.D. (1992) degrees in Biochemistry and Molecular Biology from Harvard University. He then pursued postdoctoral studies on the role of the RNA binding proteins in Drosophila development at The Whitehead Institute for Biomedical Research, in Cambridge, Massachusetts. In 2023, he was elected to the American Academy of Arts & Sciences, the National Academy of Sciences and the National Academy of Medicine. His laboratory studies small RNA silencing pathways in eukaryotes and prokaryotes, including RNA interference (RNAi), microRNA, and PIWI-interacting RNA pathways. Dr. Zamore and his collaborators seek to use these insights to design therapies for human diseases.

Feng Zhang, Ph.D.

Professor of Neuroscience and Biological Engineering, M.I.T.

Dr. Zhang is a molecular biologist focused on improving human health. He played an integral role in the development of two revolutionary technologies, optogenetics and CRISPR-Cas systems, including pioneering the use of Cas9 for genome editing and discovering CRISPR-Cas12 and Cas13 systems and developing them for therapeutic and diagnostic applications. Current research in the Zhang laboratory is centered on the discovery of novel biological systems and processes, uncovering their mechanisms, and developing them into molecular tools and therapies to study and treat human disease. Zhang is a core member of the Broad Institute, an Investigator at the McGovern Institute for Brain Research, the James and Patricia Poitras Professor of Neuroscience at MIT, and a Howard Hughes Medical Investigator. He is also a member of the National Academy of Sciences, the National Academy of Medicine, and the American Academy of Arts and Sciences as well as a fellow in the National Academy of Inventors.

Michael Angelo, M.D., Ph.D.

Associate Professor of Pathology
Stanford University

Michael Angelo, MD PhD is a board-certified pathologist in the department of Pathology at Stanford University School of Medicine. Dr. Angelo is a leader in high-dimensional imaging with expertise in tissue homeostasis, tumor immunology, and infectious disease. His lab has pioneered the construction and development of Multiplexed Ion Beam Imaging by time of flight (MIBI-TOF). MIBI-TOF uses secondary ion mass spectrometry and metal-tagged antibodies to achieve rapid, simultaneous imaging of dozens of proteins at subcellular resolution. His lab used this technology to discover previously unknown rule sets governing the spatial organization and cellular composition of immune and stromal cells within the tumor microenvironment in triple-negative breast cancer and ductal carcinoma in situ. This effort has led to ongoing work aimed to define broader structural mechanisms that promote tolerogenic niches in cancer, tuberculosis, and the maternal-fetal interface. His lab is expanding this spatial biology framework to leverage new technologies that can map the spatial distribution of transcripts, lipids, and glycans. Dr. Angelo is the recipient of 2014 NIH Director's Early Independence, 2020 DOD Era of Hope Award and is a principal investigator on multiple extramural awards from the National Cancer Institute, Breast Cancer Research Foundation, Parker Institute for Cancer Immunotherapy, the Bill and Melinda Gates Foundation, and steering committee co-director of the Human Biomolecular Atlas (HuBMAP) initiative.

Gilles Besin, Ph.D.

CSO, Orbital Therapeutics

With more than 15 years of research experience in immunology and vaccines for infectious diseases, oncology, and metabolic disorders, including rare diseases, Dr. Gilles Besin is now the CSO of Orbital Therapeutics. Prior to joining Orbital Therapeutics, Dr. Besin led all discovery research efforts at Affinivax as Head of Discovery. In this role, he led a team to discover and develop vaccine candidates (targeting Staphylococcus, Klebsiella, Pseudomonas, bacteria Clostridium difficile and more recently SARS CoV-2 vaccine), and support the development of the

Streptococcus pneumoniae vaccine pipeline. Previously to Affinivax, Dr. Besin led the Moderna immunology platform and efforts to optimize the mRNA delivery platform and modulate T cell responses in cancer and autoimmune diseases using lipid nanoparticles. of mRNA. In addition, Dr. Besin has collaborated on numerous mRNA vaccines against cancer and infectious diseases, some in the clinical phase. Prior to Moderna, Dr. Besin was involved in the delivery of therapeutic nucleic acids and has been involved in the development of mRNA vaccines in collaboration with Sanofi Pasteur and CureVac under the Defense Advanced Research Projects Agency (DARPA).

Howard Chang, M.D., Ph.D.

Virginia and D.K. Ludwig Professor of Cancer Research
Director of the Center for Personal Dynamic Regulomes
Professor of Dermatology and Genetics, Stanford University
Investigator, Howard Hughes Medical Institute

Howard Y. Chang M.D., Ph.D. is the Virginia and D.K. Ludwig Professor of Cancer Research and Director of the Center for Personal Dynamic Regulomes at Stanford University. He is a Howard Hughes Medical Institute Investigator; he is also Professor of Dermatology and of Genetics at Stanford University School of Medicine. Chang earned a Ph.D. in Biology from MIT, M.D. from Harvard Medical School, and completed Dermatology residency and postdoctoral training at Stanford University. His research addresses how large sets of genes are turned on or off together, which is important in normal development, cancer, and aging. Chang discovered a new class of genes, termed long noncoding RNAs, can control gene activity throughout the genome, illuminating a new layer of biological regulation. He invented ATAC-seq and other new methods for defining DNA regulatory elements genome-wide and in single cells. The long term goal of his research is to decipher the regulatory information in the genome to benefit human health. Dr. Chang is a Member of the US National Academy of Sciences, National Academy of Medicine, and American Academy for the Arts and Sciences. Dr. Chang's honors include the NAS Award for Molecular Biology, Outstanding Investigator Award of the National Cancer Institute, Paul Marks Prize for Cancer Research, Judson Daland Prize of the American Philosophical Society, and the Vilcek Prize for Creative Promise. His work was honored by the journal Cell as a Landmark paper over the last 40 years and by Science as "Insight of the decade".

John Dimos, Ph.D.

Alexandria Venture Investments

John Dimos is a Principal of Science & Technology at Alexandria Real Estate Equities, Inc. and Alexandria Venture Investments and the Head of Alexandria LaunchLabs in the San Francisco Bay Area. He oversees the proprietary platform's general management, the recruitment and support of its member companies, and the development of its entrepreneurial community, as well as supports Alexandria's ecosystem-building, business development, and venture activities in the Bay Area. Prior to joining Alexandria in 2020, Dr. Dimos was CEO at Fountain Therapeutics, a company that uses a deep learning-based platform for the discovery and development of novel therapeutics for diseases associated with aging. Previously, he helped launch Mavericks Capital (an investment bank for early-stage life science companies developing new products in biotech, biopharma, medical devices, and digital health), led U.S. operations for a revenue-generating company that uses DNA sequencing and machine learning to predict disease risk and develop mitigation strategies in agriculture, and was a founding scientist at iPierian. Dr. Dimos has a BS from Tufts University and a PhD from Princeton University and completed a fellowship at Harvard University.

Ron Dror, Ph.D.

Cheriton Family Professor of Computer Science and by courtesy, Molecular and Cellular Physiology and Structural Biology, Stanford University

Ron Dror is the Cheriton Family Professor of Computer Science in the Stanford Artificial Intelligence Lab. He is also affiliated with the Department of Structural Biology and of Molecular and Cellular Physiology, the Institute for Computational and Mathematical Engineering, Bio-X, ChEM-H, and the Biophysics and Biomedical Informatics Programs. Dr. Dror leads a research group that uses molecular simulation and machine learning to elucidate biomolecular structure, dynamics, and function, and to guide the development of more effective medicines. He collaborates extensively with experimentalists in both academia and industry. Before moving to Stanford, he served as second-in-command of D. E. Shaw Research, a hundred-person company, having joined as its first hire. Dr. Dror earned a PhD in Electrical Engineering and Computer Science at MIT,

where he developed machine learning methods for computer vision and genomics. He earned an MPhil in Biological Sciences as a Churchill Scholar at the University of Cambridge, as well as undergraduate degrees in Mathematics and in Electrical and Computer Engineering at Rice University, summa cum laude. He has published over 30 papers in Nature, Science, and Cell, and has won several Gordon Bell Prizes and Best Paper Awards.

Judith Frydman, Ph.D.

Professor of Genetics Biology, Stanford University

Judith Frydman is a native of Argentina, where she studied Chemistry and carried out her PhD studies in Biochemistry. She discovered the eukaryotic ring-shaped chaperonin TRiC/CCT and showed protein folding in eukaryotic cells occurs cotranslationally with the aid of molecular chaperones as polypeptides emerge from ribosomes. The Frydman lab is focused on understanding how the network of molecular chaperones and the ubiquitin-proteasome pathway maintain proteostasis in eukaryotic cells and how its dysfunction leads to disease and aging. Her lab also harnesses these insights to develop therapeutic approaches to ameliorate human diseases including neurodegenerative diseases and to identify specific interventions that disfavor the production of toxic protein species.

Brian Hie, Ph.D.

Assistant Professor of Chemical Engineering and Data Science, Stanford University

Brian Hie is an Assistant Professor of Chemical Engineering and Data Science at Stanford University, a David T. Morgenthaler II Faculty Fellow in the Stanford School of Engineering, and an Innovation Investigator at Arc Institute. He supervises the Laboratory of Evolutionary Design, where research is conducted at the intersection of biology and machine learning.

Silvana Konermann, Ph.D.

Executive Director and Core Investigator, ARC Institute
Assistant Professor of Biochemistry, Stanford University

Silvana is the Executive Director and Core Investigator of the Arc Institute and an Assistant Professor of Biochemistry at Stanford. Her research laboratory aims to understand the molecular pathways that drive the development of Alzheimer's disease using next-generation functional genomics, with the long-term goal of developing rationally targeted therapeutics for neurodegenerative disorders. She received her Ph.D. in Neuroscience from MIT. Silvana's pioneering work on tools to directly perturb the transcriptomic landscape of the cell using CRISPR has previously been recognized by her faculty appointment as a Chan Zuckerberg Biohub Investigator and Hanna Gray Fellow of the Howard Hughes Medical Institute.

Andrew Lee, Ph.D.

Director, Northpond Ventures

Andrew Lee is a Director at Northpond Ventures, specializing in biomanufacturing, life science R&D solutions, and environmental sciences. He is a board director at Culture Biosciences, Slingshot Biosciences, and EnPlusOne Biosciences as well as a board observer at 64x Bio, Ansa Biotechnologies, Refeyn, Sherlock Biosciences, and Sound Agriculture. Previously, Andrew was an Associate at Booz Allen Hamilton working in the Biological Technologies Office at the Defense Advanced Research Projects Agency (DARPA), where he worked on the development and management of a high-risk, high-reward research funding portfolio in gene editing and synthetic biology. Earlier, he worked at Sangamo Biosciences (now Sangamo Therapeutics) and the Joint BioEnergy Institute. Andrew earned a Ph.D. in Microbiology and Immunology from Columbia University. He received a B.A. with honors in Molecular and Cell Biology, with an emphasis in biochemistry and molecular biology, from The University of California, Berkeley.

Billy Li, Ph.D.

Professor of Genetics, Stanford University

Jin Billy Li is a Professor of Genetics at Stanford University. He earned his bachelor's and master's degrees at Tsinghua University in Beijing, China, and completed his Ph.D. at Washington University in St. Louis. Following postdoctoral training at Harvard Medical School, he established his laboratory at Stanford in 2010. Dr. Li's research focuses on the study of RNA editing mediated by ADAR enzymes.

Maddie Maloney, Ph.D.

Senior Associate, Northpond Ventures

Maddie Maloney is a Senior Associate at Northpond Ventures. Maddie supports Northpond's origination, sourcing, diligence, and monitoring efforts. Previously, Maddie was a management consultant at TRINITY Life Sciences where she worked with emerging and established biotech and pharmaceutical companies from pre-clinical development through peri-launch lifecycle management. Maddie also held research roles at the Gates Center for Regenerative Medicine and Boston Children's Hospital. She graduated summa cum laude with Phi Beta Kappa honors from Middlebury College with a B.A. in Molecular Biology and Biochemistry.

Niren Murthy, Ph.D.

Professor of Bioengineering, UC Berkeley
Innovative Genomics Institute

Niren Murthy is a professor at UC Berkeley in Bioengineering. His lab focuses on drug delivery and molecular imaging. He also is a member of the Innovative Genomics Institute.

John Pham, Ph.D.

Editor in Chief of Cell, Cell Press

John is the Editor in Chief of *Cell*. Before joining *Cell*, he was the Editor in Chief of *Molecular Cell*. He received his PhD from Northwestern University, where he studied the mechanisms of RNA splicing and RNA interference with Erik Sontheimer and conducted postdoctoral work at Harvard Medical School and Brigham and Women's Hospital.

Stanley Qi, Ph.D.

Associate Professor of Bioengineering and Sarafan ChEM-H, Stanford University

Dr. Lei Stanley Qi is an Associate Professor of Bioengineering and Sarafan ChEM-H at Stanford University. He developed the nuclease-dead dCas and a range of CRISPR tools for epigenetic engineering, such as CRISPR interference (CRISPRi), CRISPR activation (CRISPRa), and live cell DNA/RNA imaging. His research

has led to discoveries in the function of the 3D genome and noncoding genetic elements. His lab has been engineering the human transcriptome to enhance cell therapy. Dr. Qi received a B.S. in Physics and Math from Tsinghua University, China, in 2005 and a Ph.D. in Bioengineering from the University of California, Berkeley, where he worked with Prof. Adam Arkin and Dr. Jennifer Doudna. He was a Faculty Fellow at UCSF before joining Stanford University.

Netra Unni Rajesh

Ph.D Candidate- DeSimone Lab, Bioengineering, Stanford University

Netra Unni Rajesh, from Toronto, Canada is pursuing a PhD in bioengineering at the Stanford School of Engineering and School of Medicine. She graduated from the University of Toronto with a bachelor's degree in Engineering Science, specializing in Biomedical Engineering. In Joseph DeSimone's lab, Netra works at the intersection of materials science and medicine to design novel, 3D-printed devices for transdermal delivery and diagnostics . Through research internships at the Koch Institute at MIT and the MEMS Lab at Caltech, she developed technologies for cancer drug delivery and vaccine production. An advocate for STEM outreach, she has done a TEDx talk titled, "Why students need science and technology." Netra is a Knight-Hennessy scholar, a recipient of the U of T Student Leadership Award and was recognized as a 3M National Student Fellow.

Julia Salzman, Ph.D.

Associate Professor of Biomedical Data Science of Biochemistry and by courtesy, of Statistics and Biology, Stanford University.

Julia Salzman is an Associate Professor in the Department of Biomedical Data Science, Biochemistry and Statistics (by Courtesy). She received her A.B. in Mathematics from Princeton University Magna Cum Laude and Ph.D. from Stanford University in the Department of Statistics supervised by Dr. Persi Diaconis. As a postdoctoral scholar in Dr. Patrick Brown's lab, Dr. Salzman developed statistical algorithms that led to the discovery of a

ubiquitous expression of circular RNA missed by other computational and experimental approaches for decades. Her research spans the interface of statistical methodology and genomics aiming to use data driven experiments to uncover organizing principles of biological regulation, historically focused on RNA processing. Recently her group has introduced a new approach to sequencing analysis called SPLASH that performs inference on raw sequencing data, bypassing genome alignment. This approach is providing new insights into genome regulation in several biological domains.

Peter Sarnow, Ph.D.

Professor of Microbiology and Immunology, Stanford University

Peter Sarnow is the Burt and Marion Avery Professor of Immunology in the Department of Microbiology and Immunology at Stanford University School of Medicine. His laboratory has been studying the mechanism by which certain microRNA molecules regulate expression of cellular and viral mRNAs. Very recent research in the lab concentrates on the function of host- and virus-derived circular RNAs in viral pathogenesis. Sarnow's scientific service record spans education, research and industry. He has received numerous awards for his research, including from the Studienstiftung des Deutschen Volkes, the Deutsche Forschungsgemeinschaft, a Faculty Research Award from the American Cancer Society, the Sidney Frank Research Prize, and Merit and Transformative Awards from the NIH. He has been elected a Fellow of the American Association for the Advancement of Science, the American Society of Microbiology and the National Academy of Sciences.

Ron Shigeta, Ph.D.

Ron Shigeta is a serial Biotech entrepreneur at iAccelerate.tech, a virtual advisory accelerator he is also a co-founder and former Science Officer of the leading biotech accelerator, IndieBio. As an investor and advisor Ron has helped to fund over 120 startups that have redefined the role of biotech startups, especially food and consumer products. He now does biotech product development and marketing and advising for multiple VC funds and startups. Ron trained at Stanford, Harvard Medical School and Princeton University in protein structure and biophysics.

Yue Wan, Ph.D.

Deputy Executive Director, Genomics Institute of Singapore

Yue Wan studies how RNA structure impacts RNA function.

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