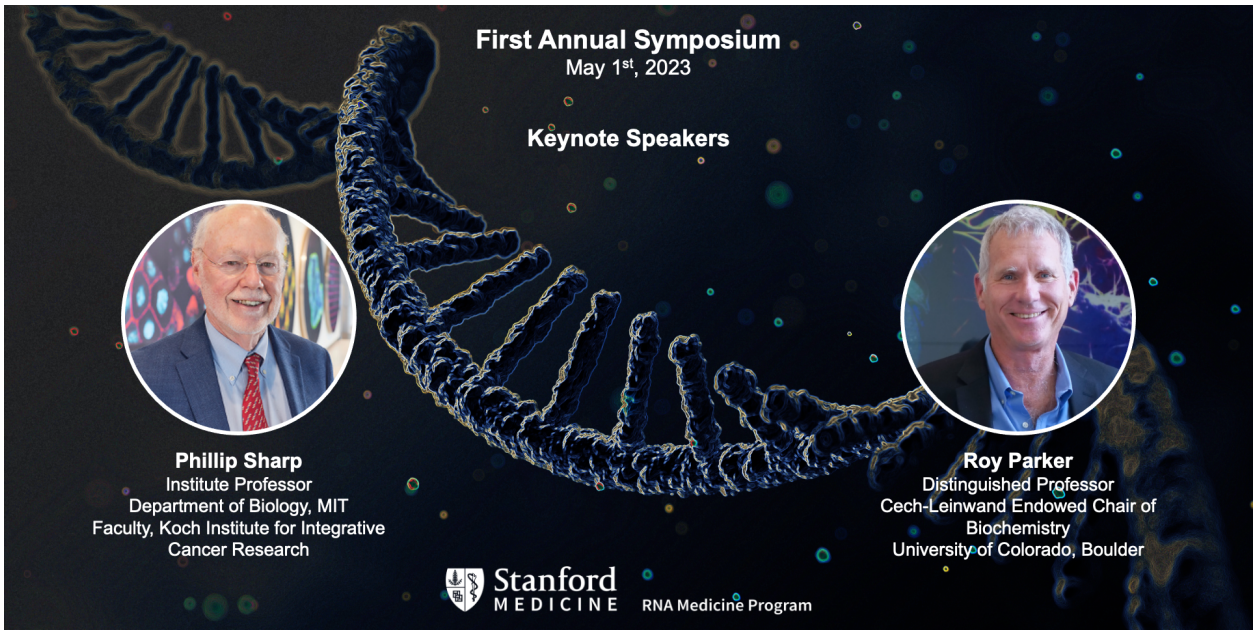


Stanford MEDICINE School of Medicine

RNA Medicine Program

Welcome from the Director

Dear Friends, Colleagues, and Distinguished guests,

On behalf of the Stanford RNA Medicine Program, it is my great pleasure to welcome you to our Inaugural Annual Symposium. We are thrilled to have you join us for this exciting event, which marks a foundational moment as we launch the program.

RNA medicine is a rapidly evolving field that has the potential to transform the way we approach diseases and develop new therapies. Our program is at the forefront of this exciting area of research, and this symposium provides a unique opportunity for us to come together and share our latest findings, insights, and discoveries.

Over the course of today, we will hear from luminaries and rising stars in the field, including our own researchers and esteemed colleagues from around the world. We will explore a wide range of topics, from the fundamental mechanisms of RNA biology, the latest advances in RNA-based therapeutics, and the challenges and rewards of entrepreneurship.

As we embark on this journey together, I am excited by the possibilities that lie ahead. Our program is made up of some of the most innovative and dedicated scientists and researchers in the field, and I am confident that our collective efforts will drive progress and make a real difference in the lives of patients around the world.

I hope that you find this symposium to be informative, engaging, and inspiring. I look forward to the conversations and collaborations that will emerge from this gathering, and I am excited to see where our collective efforts will take us.

Thank you for joining us, and welcome to the Stanford RNA Medicine Program-Inaugural Annual Symposium!

Sincerely,

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

Monday, May 1, 2023

8:30–9:00 AM Registration and Breakfast

9:00–9:10 AM **WELCOME AND OPENING REMARKS**

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

Lloyd Minor, M.D.
Carl and Elizabeth Naumann Professorship for the Dean of the School of Medicine
Professor of Otolaryngology - Head and Neck Surgery

9:10–10:00 AM **SESSION 1: OPENING KEYNOTE**
Moderated by Howard Y. Chang, M.D., Ph.D.

Phillip A. Sharp, Ph.D.
Institute Professor Emeritus Massachusetts Institute of Technology, 1993 Nobel Laureate in Physiology or Medicine
RNA Biology to therapeutics: An Emerging Story

10:00–10:40 AM **SESSION 2: RNA BIOLOGY AND GENOME REGULATION**
Moderated by James Zou, Ph.D.

Nicole Martinez, Ph.D.
Assistant Professor, Chemical and Systems Biology and of Developmental Biology, Stanford University
Uncovering new functions of RNA modifications in mRNA processing

Patrick Hsu, Ph.D.
Co-founder of the Arc Institute and Assistant Professor of Bioengineering and Deb Faculty Fellow in the College of Engineering at the University of California, Berkeley.
Frontiers in transcriptome engineering

10:40–11:00 AM Coffee Break

11:00–12:20 PM **SESSION 3: RNA TECHNOLOGY DEVELOPMENT**
Moderated by Rhiju Das, Ph.D.

Andrew Fire, Ph.D.

Professor, Pathology and Genetics, Stanford University, 2006 Nobel Laureate in Physiology or Medicine.
'Never gonna give you up': Exploring multigeneration inheritance at the RNA level

James Zou, Ph.D.
Assistant Professor, Biomedical Data Science and by courtesy, Computer Science and Electrical Engineering, Stanford University
AI for learning RNA grammar and regulation

Polly Fordyce, Ph.D.
Associate Professor, Bioengineering and Genetics, Stanford University
Using microfluidic affinity assays to understand how sequence encodes specificity in nucleic acid binding proteins

William Greenleaf, Ph.D.
Professor, Genetics Department, Stanford University School of Medicine
High throughput quantitative nucleic acid measurements on chip

12:20–1:00 PM Lunch (served until 1:40 pm)

1:00–1:40 PM **SESSION 4: ENTREPRENEURS PANEL DISCUSSION**
Moderated by Miao-Chih Tsai, Ph.D.

Janice Chen, Ph.D.
Co-Founder & CTO, Mammoth Biosciences

Rachel Meyers , Ph.D.
Entrepreneurial Scientist

Jeffrey Bird, M.D., Ph.D.
Founder & Managing Director, Bluebird Ventures

Murali Venkatesan, Ph.D.
Head of Danaher Ventures and VP, Science and Technology, Danaher

1:40–3:00 PM **SESSION 5: RNA BIOLOGY AND DELIVERY**
Moderated by Nicole Martinez, Ph.D.

Maria Barna, Ph.D.
Associate Professor, Genetics, Stanford University
Ribosomes in gene regulation: controlling the diversity of proteins made in specific cells, tissues & organisms

Rhiju Das, Ph.D.
Associate Professor, Biochemistry, Stanford University School of Medicine
Progress in RNA modeling and design

Paul Wender, Ph.D.
Bergstrom Professor of Chemistry, Courtesy Professor of Chemical and Systems Biology, Stanford University
Translational science and RNA delivery

Alice Ting, Ph.D.
Professor, Genetics, Biology, and by courtesy, Chemistry, Stanford University
Technologies for mapping RNA localization and interactions in living cells

3:00–3:30 PM Coffee Break

3:30–4:30 PM **SESSION 6: RNA THERAPEUTICS**
Moderated by Maria Barna, Ph.D.

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
Harnessing circular RNA for immunotherapy

Mira Moufarrej, Ph.D.
Swanson Fellow, The Column Group
Improving prenatal care through cell-free RNA liquid biopsies

Mark Kay, M.D., Ph.D.
Dennis Farrey Family Professor
Pediatrics and Genetics, Stanford University School of Medicine
Linking the Lnc122 RNA (the miR122 precursor) to MYC and cancer

4:30–5:20 PM **SESSION 7: CLOSING KEYNOTE**
Moderated by Howard Y. Chang, M.D., Ph.D.

Roy Parker, Ph.D.
Distinguished Professor, Cech-Leinwand Endowed Chair, Biochemistry, University of Colorado Boulder
RNP granules in health and disease

5:20–6:30 PM Drink Reception and Poster Session

POSTERS:

#1 Defining the landscape and function of m5U in mRNA of breast cancer cells

Nicolas Robalin, Department of Chemistry, Stanford University

Extensive chemical nucleoside modifications within eukaryotic messenger RNAs broadly regulate gene expression by impacting diverse aspects of mRNA metabolism. Many of the enzymes that chemically modify RNAs have been associated with human diseases ranging from cancer to neurodevelopmental disorders. High expression of TRMT2A, one such RNA modifying enzyme, has been predictive of recurrence risk in HER2+ breast cancer patients. Whether and how TRMT2A is required for breast cancer pathogenesis is not understood. TRMT2A is known to catalyze methylation of uridine into m5U at position 54 within tRNAs. Intriguingly, TRMT2A was shown to crosslink with mRNAs suggesting that TRMT2A is also an mRNA modifying enzyme and m5U could regulate gene expression through effects on mRNA metabolism. Aberrant modification of mRNA targets could therefore contribute to the function of TRMT2A in breast cancer, underscoring the need to determine the location of m5U transcriptome-wide. My preliminary data demonstrates that introducing a mutation in TRMT2A covalently traps RNA targets which will be used to enrich for m5U modified mRNAs and to map the locations of m5U with single nucleotide resolution by high-throughput sequencing in breast cancer cells. I will develop tools to chemically label m5U sites, identify m5U modification sites in mRNAs, and investigate the function of TRMT2A in breast cancer cells. This will reveal new mechanisms by which RNA modifications regulate gene expression and how their dysregulation contributes to cancer, paving the way for the development of new cancer therapies.

#2 Covalent Conjugation of RNA Using Stable Sulfonyl Electrophiles

Sayantan Chatterjee, Department of Chemistry, Stanford University

Approved and experimental RNA therapies often benefit from conjugation to improve their pharmacokinetics, permeability, selectivity and safety. Acylation of RNA 2'-OH is a rapidly growing method of covalent conjugation of unmodified RNA of any length. Acylation chemistry employing the nucleophilic character of RNA 2'-OH groups can be performed both in vitro and in mammalian cells. Until recently, all chemical agents that have performed such covalent modifications have been short-lived carbonyl electrophiles that are unstable and difficult to purify. We report here that certain activated sulfonyl species can exhibit specific, high-yield and tunable reactivity with RNA 2'-OH and possess extended stability in water. A sulfonyl conjugate on RNA is shown to be stable in water for at least 8 weeks at 37oC compared to the tendency for hydrolysis of many acyl adducts on RNA 2'-OH within hours. After sulfonylation with an

azide-containing reagent, this strategy enables the linkage of a fluorophore onto RNA using click chemistry. Investigations with a sulfonyl reagent applied to folded RNAs in vitro and in intact human cells demonstrate selectivity for reaction at unpaired nucleotides over those in duplex regions, allowing a readout of RNA structure and indicating possible cell permeability. This new class of reagents offers a promising approach for RNA conjugation and structure mapping with improved stability and versatility.

#3 Cracking the Nut: Peptoid based nanoparticle delivery of mRNA therapeutics

Bette Webster, Nutcracker Therapeutics

Messenger RNA-based therapeutics have emerged as powerful tools in vaccine development, immune-oncology, and enzyme replacement therapy. A critical component of mRNA therapeutics are the delivery vehicles which encapsulate the mRNA molecule, protect it during transit, and facilitate internalization of the mRNA into the cell allowing translation. Nutshells®, are a new class of delivery vehicle which utilize N-lipidated peptoids in concert with additional helper lipids to encapsulate mRNA cargos. Tuning the peptoid structure by modulating the hydrophobic tails and the amino-fuctionalized head group impacts Nutshell physical properties and mRNA expression. To date, over 350 unique peptoid structures have been generated and evaluated as Nutshells for mRNA delivery, including investigating particle pKa, encapsulation, endosomal escape, immunogenicity, and in vivo expression to generate structure-function relationships. With these trends, Nutshells® can be tailored towards specific routes of administration and therapeutic areas. NTX-250 is Nutcracker's first IND candidate targeting HPV-driven cervical dysplasia. The Nutshell® for this therapy was designed for local administration with tailored particle pKa and distribution as compared to therapies requiring systemic IV delivery. Investigation of how peptoid structure impacts particle function has established Nutshells® as a high-performing, tunable platform for mRNA therapeutics.

#4 High-throughput screening identifies favorable antisense oligonucleotide features for programmable RNA editing

Inga Jarmoskaite, Department of Genetics, Stanford University

Site-directed RNA editing by endogenous human ADAR proteins holds great therapeutic promise in precision medicine, with important safety and delivery advantages over other gene and mRNA editing platforms. ADAR proteins catalyze adenosine-to-inosine (A-to-I) conversion in double-stranded RNA (dsRNA) and can be efficiently directed to specific adenosines of therapeutic interest by short oligonucleotide guides that are complementary to the target mRNA. To achieve maximum efficiency and specificity of RNA base editing, it is essential to define the design rules for ADAR oligonucleotide guides. Importantly, the varied structures of ADAR's natural dsRNA substrates suggest that deviations from Watson-Crick base-pairing in the

target-guide RNA duplex may enhance editing efficiency and specificity. To systematically probe the effects of mismatches in the vicinity of the editing site, we have quantified editing of 100,000s of dsRNA variants that mimic the target–guide RNA complex, focusing on therapeutically relevant target RNAs. Indeed, for every target sequence tested, we identified mismatch-containing guide sequences that enhance adenosine editing relative to the editing level measured with complementary guides. Our results reveal previously unrecognized modularity in ADAR editing and will provide an important resource for the design of therapeutic ADAR oligonucleotide guides.

#5 Modular, programmable RNA sensing using ADAR editing in living cells

Natalie Kolber, Department of Bioengineering, Stanford University

A major challenge in the field of gene therapy is the selective delivery of genetic cargo to cell types of interest. While the abundance of available RNA transcriptomics data means that cell types can be identified by their RNA signature, there are few tools available to act on this data in living human cells, many of which either are limited to microRNAs or require engineering around guide RNAs, ribozymes, or internal ribosome entry sites. We therefore set out to develop a modular, programmable tool for live RNA sensing using adenosine deaminases acting on RNA ("RADAR"). We demonstrate strategies for increasing output levels and dynamic range as well as the combinatorial sensing of multiple RNAs via AND logic. We show that RADAR enables the sensing of disease-relevant sequence alterations such point mutations and gene fusions. As RADAR can be delivered as mRNA, this tool has the promise to be combined with advances in RNA delivery towards highly targetable gene therapies.

#6 The (mis)behavior of E. coli RNA polymerase on RNA replicons: RNA replication by a cellular RNA polymerase

Drew Galls, Department of Genetics, Stanford University

Some canonical 'DNA-dependent' RNA polymerases ('RNAPs'), including human RNAP II, can accept RNA as a template and replicate RNA. A subset of these polymerases, when incubated in vitro without explicitly added DNA or RNA template, produce novel RNA replicons. RNAP-catalyzed RNA replication is exploited by hepatitis delta virus, could provide a mechanism for RNA-based heredity, and, given the ubiquity of RNAPs, may have other, unappreciated biological functions. While some in vitro data exists on RNA replication catalyzed by T7 RNAP, knowledge on cellular RNAPs, which are evolutionarily unrelated, is limited. We assembled E. coli RNAP reactions without any explicitly added template. We observed RNA products ~100-~1000+ nucleotides in length. This RNA could catalyze further RNA generation, consistent with this RNA being a replicon. Analysis of this RNA via Illumina and Nanopore sequencing has been challenging. Preliminary results suggest that E. coli RNAP-derived RNAs are composed of

stretches of A and U interspersed with other nucleotides, consistent with biochemical results and Wettich Biochemistry 2001. Sequencing difficulties suggest that if E. coli RNAP generates these RNAs in vivo, they may have gone undetected so far. Future work will characterize the repertoire of RNAs generated by E. coli RNAP, reconstitute RNA replication with defined RNA templates, and characterize the origin of these RNAs. Broadly, the ability of E. coli RNAP to generate and propagate RNA replicons has implications for the possibility of RNA replication in cells, including in RNA-based heredity.

#7 Xist ribonucleoproteins promote female sex-biased autoimmunity

Diana Dou, Department of Genetics, Stanford University

Autoimmune diseases disproportionately affect females more than males. The XX sex chromosome complement is strongly associated with susceptibility to autoimmunity. The Xist long noncoding RNA (lncRNA) is expressed only in females to randomly inactivate one of the two X chromosomes to achieve gene dosage compensation. Here, we show that the Xist ribonucleoprotein (RNP) complex, comprised of numerous autoantigenic components, is an important driver of sex-biased autoimmunity. Inducible transgenic expression of a non-silencing form of Xist in male mice introduced Xist RNP complexes and sufficed to produce autoantibodies. Male SJL/J mice expressing transgenic Xist developed more severe multiorgan pathology in pristane-induced model of lupus than wild-type males. Xist expression in males reprogrammed T and B cell population and chromatin states to more resemble wild type females. Human patients with autoimmune diseases displayed significant autoantibodies to multiple components of XIST RNP. Thus, a sex-specific lncRNA scaffolds ubiquitous RNP components to drive sex-biased immunity.

#8 Design, synthesis, and characterization of drug delivery systems (DDS) to address global biomedical challenges

Zhijian Li, Department of Chemistry, Stanford University

RNA delivery holds enormous potential for various biomedical applications such as prophylactic and therapeutic vaccines, immunotherapies for cancer and strategies for genome editing. Despite the enormous progress, naked RNA is large and polyanionic, unable to efficiently cross non-polar biological barriers such as plasma membrane and reach cytosol to elicit its function in vivo. To address this challenge, Wender lab and others have developed a new type of mRNA transporters called Charge-Altering Releasable Transporters (CARTs) enabling efficient delivery of a variety of RNA cargoes (pDNA, mRNA, siRNA, saRNA, circRNA). Significantly, by systematic tuning chemical structure of CARTs, we develop different CART variants able to transfect hard-to-transfect lymphocytes with high efficiency and selective organs in vivo, leading to successful implementation of various biomedical applications.

#9 Circular RNA uptake programs systemic immunity

Laura Amaya, Department of Genetics, Stanford University

Circular RNAs (circRNAs) are a class of RNAs commonly found across eukaryotes and viruses, characterized by their resistance to exonuclease-mediated degradation. Their superior stability compared to linear RNAs, combined with previous work showing that engineered circRNAs serve as efficient protein translation templates, make circRNA a promising candidate for RNA medicine. Here we systematically examine the adjuvant activity, route of administration, and antigen-specific immunity of circRNA vaccination in mice. Potent circRNA adjuvant activity is associated with RNA uptake and activation of myeloid cells in the draining lymph nodes and transient cytokine release. Immunization of mice with engineered circRNA encoding a protein antigen delivered by a charge altering releasable transporter (CART) induced innate activation of dendritic cells, robust antigen-specific CD8 T cell responses in lymph nodes and tissues, and strong antitumor efficacy as a therapeutic cancer vaccine. These results highlight the potential utility of circRNA vaccines for stimulating potent innate and T cell responses in tissues.

KEYNOTE SPEAKER

Phillip A. Sharp, Ph.D.

Institute Professor Emeritus
Massachusetts Institute of Technology

Phillip A. Sharp is an Institute Professor Emeritus at the Massachusetts Institute of Technology and member of the Department of Biology and the Koch Institute for Integrative Cancer Research. He joined the Center for Cancer Research (now the Koch Institute for Integrative Cancer Research at MIT) in 1974 and served as its director for six years, from 1985 to 1991, before taking over as head of the Department of Biology, a position he held for the next eight years. He was founding director of the McGovern Institute, a position he held from 2000 to 2004. His research interests have centered on the molecular biology of gene expression relevant to cancer and the mechanisms of RNA splicing. Dr. Sharp has authored over 430 papers. His landmark work in 1977 provided the first indications of discontinuous genes in mammalian cells. The discovery fundamentally changed scientists' understanding of gene structure and earned Dr. Sharp the 1993 Nobel Prize in Physiology or Medicine.

He is an elected member of the National Academy of Sciences, the National Academy of Medicine, the American Academy of Arts and Sciences, the American Philosophical Society, and the Royal Society, UK. Among his many awards are the Gairdner Foundation International Award, the Lasker Basic Medical Research Award, and the National Medal of Science. His long list of service includes the presidency of the AAAS (2013) and Chair of the Scientific Advisory Committee of the SU2C Project, AACR. Dr. Sharp co-founded Biogen in 1978 and served as a member of the Board of Directors (to 2009) and Chair of the Scientific Advisory Board (to 2002). He is also a co-founder of Alnylam Pharmaceuticals Inc. (2002) and continues to serve as a member of the Board of Directors and Chair of the Scientific Advisory Board. Dr. Sharp consults and serves on boards of other biotechnology companies. A native of Kentucky, Dr. Sharp earned a BA degree from Union College, Barbourville, KY, and a Ph.D. in chemistry from the University of Illinois, Urbana-Champaign.

KEYNOTE SPEAKER

Roy Parker, Ph.D.

Distinguished Professor, Cech-Leinwand Endowed Chair
Biochemistry, University of Colorado Boulder

Roy Parker is an Investigator with the Howard Hughes Medical Institute; Executive Director, BioFrontiers Institute; Cech-Leinwand Endowed Chair of Biochemistry and Distinguished Professor at the University of Colorado Boulder. He has a joint appointment with the Department of Molecular, Cellular and Developmental Biology. He received his Ph.D. from the University of California, San Francisco and completed his Postdoctoral work at the University of Massachusetts, Worcester. His research focuses on the translation, localization and degradation of eukaryotic RNA, how cells regulate different steps in this process to modulate gene expression, and how alterations in RNA regulation lead to human disease. He has served on, and chaired, the NIH CDF-1 study section, and co-organized the Nucleic Acids Gordon Conference (1997), the RNA Processing Meeting at CSHL (2001), and the 2004 FASEB Conference on Post-Transcriptional Control (2004). He is, or has been, on the editorial boards of MCB, Science, Cell, RNA, Nucleic Acids Research, and was an editor of the Journal of Cell Biology and eLife. He was the President of the RNA Society (2010). He is an elected Fellow of the American Academy of Arts & Sciences (2010) and Member of the National Academy of Sciences (2012).

Jeffrey Bird, M.D., Ph.D.

Founder & Managing Director
Bluebird Ventures

Jeffrey Bird, MD, PhD is the founder and Managing Director of Bluebird Ventures. Jeff was previously a Managing Director for nearly 20 years at Sutter Hill Ventures, where he has invested in early-stage life sciences companies.

Jeff is currently a board member at Epirium Bio (mitochondrial and muscle diseases), Helix (genomic sequencing), Procyrion (intravascular pump for heart failure), and Teneobio (multivalent antibodies).

Jeff was a founding investor in the immuno-oncology company Forty Seven and lead director when they went public in 2018. The company was acquired by Gilead in 2020 for $4.9 billion. Jeff was also a Series A investor and board member at Portola Pharmaceuticals which was acquired in 2020 by Alexion for $1.4 billion.

Jeff served as CEO as well as investor of Verinata Health, a pioneer in non-invasive prenatal testing which was sold to Illumina in 2013. He served as a General Manager for Illumina for one year after the acquisition in order to help lead the commercialization efforts.

Scientific work at Verinata and Illumina was key in the founding of GRAIL, an early cancer detection testing company, where Jeff was a Series A investor. Grail announced its acquisition by Illumina in September 2020 for $8 billion plus royalties.

Jeff worked at Gilead Sciences for 12 years, starting as one of the first ten employees and most recently serving as SVP Business Operations with responsibilities including Commercial and Business Development activities. Highlights of his time at Gilead include the discovery and rapid development of Tamiflu, the oral treatment for influenza, as well as the development Gilead's first HIV therapies.

Jeff received his bachelor degree, MD and PhD in Cancer Biology at Stanford.

His favorite pastime is playing music, and he is the lead singer and a guitar player in a local band.

Maria Barna, Ph.D.

Associate Professor
Genetics, Stanford University

Dr. Barna obtained her B.A. in Anthropology from New York University and her Ph.D. from Cornell University, Weill Graduate School of Medicine. Dr. Barna was subsequently appointed as a UCSF Fellow through the Sandler Fellows program, which enables exceptionally promising young scientists to establish independent research programs immediately following graduate school. She is presently an Associate Professor in the Genetics Department at Stanford University. Dr. Barna has received a number of distinctions including being named a Pew Scholar, Alfred P. Sloan Research Fellow, and top '40 under 40' by the Cell Journal. She has received the Basil O' Connor Scholar Research Award and the NIH Directors New Innovator Award. She is the recipient of the Elizabeth Hay Award, H.W. Mossman Award, Tsuneko and Reiji 'Okazaki Award', American Society for Cell Biology Emerging Leader Prize, the Rosalind Franklin Young Investigator Award, and the RNA Society Early Career Award. She is presently a NYSCF Robertson Stem Cell Investigator.

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

Professor
Virginia and D.K. Ludwig Professor of Cancer Research and Director of the Center for Personal Dynamic Regulomes, Stanford University

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".

Janice Chen, Ph.D.

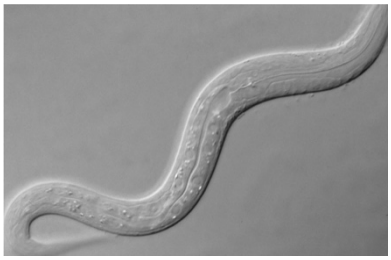
Co-Founder & CTO
Mammoth Biosciences

Janice Chen is the co-founder and CTO of Mammoth Biosciences, a biotechnology company based in the San Francisco Bay Area that is harnessing the diversity of nature to power the next generation of CRISPR products across diagnostics and therapeutics. Through its discovery of novel CRISPR systems, the company is enabling the full potential of its platform to read and write the code of life. Janice received her PhD from the lab of Nobel Laureate Jennifer Doudna at University of California, Berkeley. She investigated mechanisms of CRISPR proteins and developed technologies leading to multiple papers and patents, and co-invented the programmable CRISPR-based detection technology called DETECTR®. Janice was selected as a Forbes 30 Under 30 in Healthcare, Business Insider's 30 Under 40 in Healthcare, Endpoints Top 20 Women in Biopharma, MIT Technology Review 35 Innovators Under 35, EY Entrepreneur Of The Year, SF Business Times Most Influential Women, and delivered a TEDx talk on the potential for CRISPR to democratize diagnostics.

Rhiju Das, Ph.D.

Associate Professor
Biochemistry, Stanford University School of Medicine

Dr. Das is an Associate Professor of Biochemistry at Stanford University School of Medicine and Investigator of the Howard Hughes Medical Institute. After training in particle physics and cosmology at Harvard, Cambridge, University College London, and Stanford, Dr. Das did postdoctoral research in computational protein folding at the University of Washington with David Baker. On returning to Stanford, Dr. Das set up his lab to focus on computer modeling and design of RNA molecules, which underlie important molecular machines in biology and medicine. As a core part of this research, Dr. Das leads Eterna, an open science platform that crowdsources intractable RNA design problems to 250,000 players of an online videogame and provides scoring feedback based on actual wet-lab experiments.



Andrew Fire, Ph.D.

Professor
Pathology and Genetics, Stanford University

A native of Santa Clara County, California, Dr. Fire received training at UC Berkeley (Mathematics BA: 1975-1978), MIT (Biology Ph.D.: 1978-1983), and the Medical Research Council Laboratory in Cambridge UK (Postdoctoral: 1983-1986). From 1986 to 2003, Dr. Fire was on the staff of the Carnegie Institution of Washington's Department of Embryology in Baltimore Maryland. During his time in Baltimore, Dr. Fire assumed the position of Adjunct Professor of Biology at Johns Hopkins University. In 2003, Dr. Fire joined the faculty of the Departments of Pathology and Genetics at Stanford University School of Medicine.

Polly Fordyce, Ph.D.

Associate Professor
Bioengineering and Genetics, Stanford University

Polly Fordyce is an Associate Professor of Bioengineering and Genetics and Institute Scholar of ChEM-H at Stanford, where her lab develops and applies new microfluidic platforms for quantitative and high-throughput biophysics, biochemistry, and single-cell biology. She graduated from the University of Colorado at Boulder with undergraduate degrees in physics and biology before moving to Stanford University, where she earned a Ph.D. in physics for work with Professor Steve Block developing instrumentation and assays for single-molecule studies of kinesin motor proteins. For her postdoctoral research, she worked with Professor Joe DeRisi to develop a new microfluidic platform for understanding how transcription factors recognize and bind their DNA targets as well as a new technology for bead-based multiplexing. She is the recipient of an NSF CAREER Award, an NIH New Innovator Award, and the Eli Lilly Award in Biological Chemistry, and she is a Chan Zuckerberg Biohub Investigator.

William Greenleaf, Ph.D.

Professor
Genetics Department, Stanford University School of Medicine

William Greenleaf is a Professor in the Genetics Department at Stanford University School of Medicine, with a courtesy appointment in the Applied Physics Department. He is a member of Bio-X, the Biophysics Program, the Biomedical Informatics Program, and the Cancer Center. He received an A.B. in physics from Harvard University in 2002, and received

a Gates Fellowship to study computer science for one year in Trinity College, Cambridge, UK. After this experience abroad, he returned to Stanford to carry out his Ph.D. in Applied Physics in the laboratory of Steven Block, where he investigated, at the single molecule level, the chemo-mechanics of RNA polymerase and the folding of RNA transcripts. He conducted postdoctoral work in the laboratory of X. Sunney Xie in the Chemistry and Chemical Biology Department at Harvard University, where he was awarded a Damon Runyon Cancer Research Foundation Fellowship, and developed new fluorescence-based high-throughput sequencing methodologies. He moved to Stanford as an Assistant Professor in November 2011. Since beginning his lab, he has been named a Rita Allen Foundation Young Scholar, an Ellison Foundation Young Scholar in Aging (declined), a Baxter Foundation Scholar, and a Chan-Zuckerberg Investigator. His highly interdisciplinary research links molecular biology, computer science, bioengineering, and genomics a to understand how the physical state of the human genome controls gene regulation and biological state. Efforts in his lab are split between building new tools to leverage the power of high-throughput sequencing and cutting-edge microscopies, and bringing these new technologies to bear against basic biological questions of genomic and epigenomic regulation. His long-term goal is to unlock an understanding of the physical “regulome” — i.e. the factors that control how the genetic information is read into biological instructions — profoundly impacting our understanding of how cells maintain, or fail to maintain, their state in health and disease.

Patrick Hsu, Ph.D.

Co-Founder of the Arc Institute and Assistant Professor of Bioengineering and Deb Faculty Fellow in the College of Engineering at the University of California, Berkeley.

Patrick Hsu is Co-Founder of the Arc Institute and Assistant Professor of Bioengineering and Deb Faculty Fellow at the University of California, Berkeley. The Hsu lab develops biotechnologies to improve human health through next-generation diagnostics and therapeutics, recently reporting platforms for gene writing with DNA integrases, programmable RNA targeting, and point-of-care CRISPR diagnostics. Patrick received A.M. and Ph.D. degrees from Harvard University and the Broad Institute, where he was an early pioneer of CRISPR-Cas9 technologies for genome editing. His research has been recognized by MIT Technology Review's Innovators Under 35, the Amgen Young Investigator Award, Forbes' 30 Under 30, the NIH Early Independence Award, and the Rainwater Prize for Innovative Early Career Scientist.

Mark Kay, M.D., Ph.D.

Dennis Farrey Family Professor
Pediatrics and Genetics, Stanford University School of Medicine

Mark A. Kay, MD, PhD, is the Dennis Farrey Family Professor in the Departments of Pediatrics and Genetics, and Head of the Division of Human Gene Therapy in Pediatrics at the Stanford University School of Medicine. Professor Kay received his MD-PhD at Case Western Reserve University and completed a residency in pediatrics, fellowship in medical genetics and inborn errors of metabolism, and post-doctoral research at Baylor College of Medicine. Dr. Kay was an assistant/associate professor at the University of Washington in the Department of Medicine from 1993-1998 before moving to Stanford. Dr. Kay's group has published over 275 papers in leading journals. Dr. Kay is most well-known for his contributions in the field of gene-based therapeutics and non-coding RNA biology specifically related to microRNA biogenesis and tRNA derived small RNAs.

Dr. Kay is one of the founders of the American Society of Gene and Cell Therapy and served as the President in 2005-2006 and received the society's outstanding investigator award in 2013. In 2021, he was elected to the National Academy of Inventors. He spends much of his spare time doing landscape and nature photography.

Nicole Martinez, Ph.D.

Assistant Professor
Chemical and Systems Biology and of Developmental Biology, Stanford University

Dr. Nicole M. Martinez is an Assistant Professor in the Departments of Chemical and Systems Biology and of Developmental Biology at Stanford University. Her lab studies RNA modifications, mRNA processing and their roles in development and disease. Dr. Martinez is a K99/R00 NIH Pathway to Independence Awardee, Gabilan Fellow, Chan Zuckerberg Biohub Investigator and a member of the RNA Society. She was a postdoctoral fellow at Yale University where she worked on RNA modifications and obtained her PhD from the University of Pennsylvania studying alternative splicing.

Rachel Meyers, Ph.D.

Entrepreneurial Scientist

Rachel Meyers is an entrepreneurial scientist currently consulting within the biotech community. Most recently she served as the Founder and Chief Scientific Officer at Faze Medicines, a Third Rock Ventures spawned biotech company focused on treating diseases of high unmet need through the perturbation biomolecular condensates. Rachel has more than twenty-five years of drug discovery and development expertise as well as extensive experience in building R&D organizations. She has deep expertise in the development of RNA-based therapies having spent over 13 yrs at Alnylam Pharmaceuticals, ultimately as the SVP of Research and RNAi Lead Development. She remains an active member of the Alnylam SAB. Prior to Alnylam, Rachel was a senior scientist at Millennium Pharmaceuticals. She also serves on several scientific advisory boards, including the National Advisory Board on Innovation and Entrepreneurship through the Department of Commerce, is listed as an inventor on many patents and patent applications, and has numerous peer-reviewed publications. She completed her postdoctoral training with Lew Cantley at Harvard Medical School in the field of signal transduction and received her PhD from Nobel laureate Phil Sharp at MIT, in the field of in vitro transcription.

Rachel also enjoys participating in women-in-science panels and events, guest lecturing at Harvard, MIT and Brandeis, mentoring young scientists, contributing to ED&I initiatives and playing a variety of sports, particularly racket sports. When Rachel isn't engaged in one of the many things above, she is traipsing around the world with her husband and 2 kids.

Lloyd B. Minor, M.D.

Carl and Elizabeth Naumann Professorship for the Dean of the School of Medicine
Stanford University School of Medicine

Lloyd B. Minor, MD, is a scientist, surgeon, and academic leader. He is the Carl and Elizabeth Naumann Dean of the Stanford University School of Medicine, a position he has held since December 2012. He also is a professor of Otolaryngology–Head and Neck Surgery and a professor of Bioengineering and of Neurobiology, by courtesy, at Stanford University.

As dean, Dr. Minor plays an integral role in setting strategy for the clinical enterprise of Stanford Medicine, an academic medical center that includes the Stanford University School of Medicine, Stanford Health Care, and Stanford Medicine Children's Health. With his leadership, Stanford Medicine leads the biomedical revolution in Precision Health. His book, "Discovering Precision Health," describes this shift to more preventive, personalized health care and highlights how biomedical advances are dramatically

improving our ability to treat and cure complex diseases. In 2021, Dr. Minor articulated and began realizing a bold vision to transform the future of life sciences at Stanford University and beyond – a multi-decade journey enabled by Precision Health.

Before Stanford, Dr. Minor was provost and senior vice president for academic affairs of Johns Hopkins University. Prior to this appointment in 2009, Dr. Minor served as the Andelot Professor and director (chair) of the Department of Otolaryngology–Head and Neck Surgery in the Johns Hopkins University School of Medicine and otolaryngologist-in-chief of The Johns Hopkins Hospital.

With more than 160 published articles and chapters, Dr. Minor is an expert in balance and inner ear disorders perhaps best known for discovering superior canal dehiscence syndrome, a debilitating disorder characterized by sound- or pressure-induced dizziness. He subsequently developed a surgical procedure that corrects the problem and alleviates symptoms.

In 2012, Dr. Minor was elected to the National Academy of Medicine.

Mira Moufarrej, Ph.D.

Swanson Fellow, The Column Group

Mira Moufarrej is presently a Swanson Fellow at The Column Group, a science-driven venture capital firm. She previously developed 3 liquid biopsy tests that measure cell-free RNA (cfRNA) to predict how far along a pregnancy is and whether a pregnant individual is at risk of preeclampsia and preterm delivery long before traditional diagnostics. This work paves the way for affordable, simple, and reliable tests for preeclampsia and preterm delivery – risks that no other test can presently diagnose early enough to allow for meaningful clinical intervention. To enable such discoveries, she has also built a semi-automated pipeline to process samples, a notoriously tedious task, which to date, has extracted cfRNA from ~1100 samples in 9 days. For this work, Mira received the 2021 “Cure it!” Lemelson-MIT Student Prize. Her work has been published in Science and Nature. It has also been highlighted by the New York Times, MIT Technology Review, CNN and Insider, among others. Mira holds a PhD in bioengineering and MS in computer science from Stanford University.

Alice Ting, Ph.D.

Professor
Genetics, Biology, and by courtesy, Chemistry, Stanford University

Alice Ting is a Professor of Genetics, Biology, and by courtesy, Chemistry at Stanford University. Before joining Stanford in 2016, Alice was a Professor of Chemistry at MIT. Alice received her training at Harvard University (working with EJ Corey), UC Berkeley (PhD with Peter Schultz) and USCD (postdoctoral training with Roger Tsien). Alice's work lies at the interface of chemistry and biology and the molecular technologies she has developed, including enzyme-catalyzed proximity labeling, have been widely adopted in cell biology and neuroscience to probe organelle proteomes and protein interaction networks. She has received the NIH Pioneer Award, the Arthur Cope Scholar Award, and the McKnight Technological Innovations in Neuroscience Award. Alice has been a Chan Zuckerberg Biohub investigator since 2017.

Miao-Chih Tsai, Ph.D.

Scientific Director
RNA Medicine Program, Stanford University

As the Scientific Director of RNA Medicine Program at Stanford University, Dr. Miao-Chih Tsai leads and manages research portfolio of RNA Medicine Program. Before this role, she was a senior editor of *Cell*. Dr. Tsai was trained at University of Cambridge and Stanford University, and had over a decade of experience in evaluating the top developments in biomedical research. Having experienced its power to inspire, she is an ardent proponent of science and strives to directly promote further advancements and shape the direction of biomedical research, with a goal of therapeutic application and patient impact.

Murali Venkatesan, Ph.D.

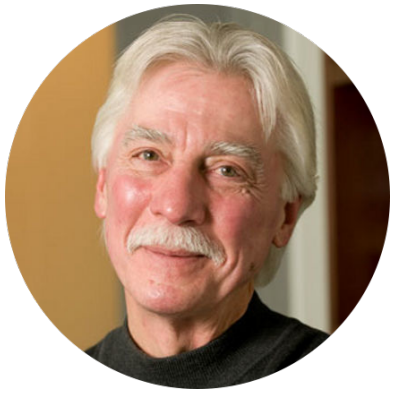
VP, Science, Technology & Innovation
Danaher Corporation

Murali has a deep passion for transforming Science, Technology and Medicine at scale to impact patient lives.

Murali joined Danaher (NYSE: DHR) in 2019, a global Science and Technology leader with $32.5 Billion in revenue and >$180 Billion market cap, operating companies including Beckman Coulter, Leica Micro- and Biosystems, IDT, Sciex, Aldevron, Cytiva and more. As Head of Danaher Ventures, Murali's team supports Danaher's strategy and investments across healthcare and are responsible for an active portfolio of over 40 companies valued at >$11 Billion. Murali serves as a Board Director for more than five companies and has joint responsibilities across Mergers & Acquisitions, including supporting previously the acquisition of Aldevron.

Prior to joining Danaher, Murali was Director of Business Development at Lam Research (NASDAQ: LRCX), a leader in Semiconductor manufacturing tools and responsible for Lam's strategy and investments in Life Sciences. Murali had previously spent ~7 years at Illumina (NASDAQ: ILMN) as a Scientist, Inventor and Senior Manager, across Advanced Research, Technology Development and Product Development, leading technology teams that enabled the $1000 and $100 genomes. These products have generated over $4B in revenue for Illumina.

Murali holds a Ph.D. in Electrical & Computer Engineering from the University of Illinois at Urbana-Champaign and is a graduate of the Stanford Executive Program. He is an inventor on 20 patents and has published in various scientific journals including Nature Nanotechnology, Nature Scientific Reports, Advanced Materials and Lab on Chip with >3000 citations. Murali also holds Bachelor of Science and Bachelor of Engineering degrees in Pure Mathematics, Computer Science and Electrical Engineering from University of Western Australia.



Paul Wender, Ph.D.

Bergstrom Professor of Chemistry
Courtesy Professor of Chemical and Systems Biology, Stanford University

Professor Wender (PhD Yale University; NIH postdoctoral Fellow Columbia University) served on the faculty at Harvard University before joining the faculty at Stanford University where he is the Bergstrom Professor of Chemistry and a Courtesy Professor in Chemical and Systems Biology. He is a member of the National Academy of Sciences, an advisor to the Stanford Molecular Imaging Program, a Sarafan ChEM-H Fellow, and affiliated with the Bio-X Program, the Center for Molecular Analysis and Design, the Stanford Cancer Institute, the Cancer Nanotechnology Program, and the Molecular Pharmacology Training Program. He is a cofounder of several biotech companies and serves/served on several science advisory boards, visiting committees and as a consultant to process and medicinal chemistry groups in the pharmaceutical and biotech industries. His research is directed at unsolved problems in chemistry, synthesis, biology, materials science, drug delivery, and medicine and includes approaches to HIV eradication, enhanced tumor targeted therapy, Alzheimer's disease, resistant cancer, resistant infections, and prophylactic and therapeutic vaccinations.

James Zou

Assistant Professor
Biomedical Data Science and by courtesy, Computer Science and Electrical Engineering, Stanford University

James Zou is an assistant professor of Biomedical Data Science at Stanford University. He develops machine learning methods for biology and medicine, and is especially interested in translational research. Several of his machine learning innovations are also widely used by tech companies. He has received a Sloan Fellowship, an NSF CAREER Award, two Chan-Zuckerberg Investigator Awards, a Top Ten Clinical Achievement Award, several best paper awards, and faculty awards from Google, Amazon, Tencent and Adobe.

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