Rare Diseases

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About the Future Vision Forum & Foundation

The **Future Vision Foundation** (FVF) is an independent 501(c)3 not-for-profit organization. Founded in 2017 by Dr. Suber Huang and Jennifer Deutsch, FVF's mission is to inspire and accelerate vision research by uniting visionary leaders worldwide in scientific collaboration and celebrate groundbreaking advancements in sight through impactful films filled with discovery and hope.

The **Future Vision Forum** is a strategic, think-tank-style meeting convened annually and chartered under the Future Vision Foundation. This first-in-kind scientific meeting unites visionary leaders in ophthalmology, visual science, and allied fields that share basic science, translational, and clinical viewpoints on the most critical topics of emerging research. It offers a unique opportunity for strategic leaders in research and development to interact as integrated faculty with distinguished, multidisciplinary experts to anticipate and forecast the future of ophthalmology and vision research.

For more information on the Future Vision Foundation and the Future Vision Forum, visit **www.futurevisionfound.org.**

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Sunday, September 10

P.M.	4:00 – 7:00	Washington Monuments Photo Tour (registered faculty meet at hotel lobby)
	7:00 – 9:00	Welcome dinner in Foxhall

Monday, September 11 *(Meeting held in DuPont A)*

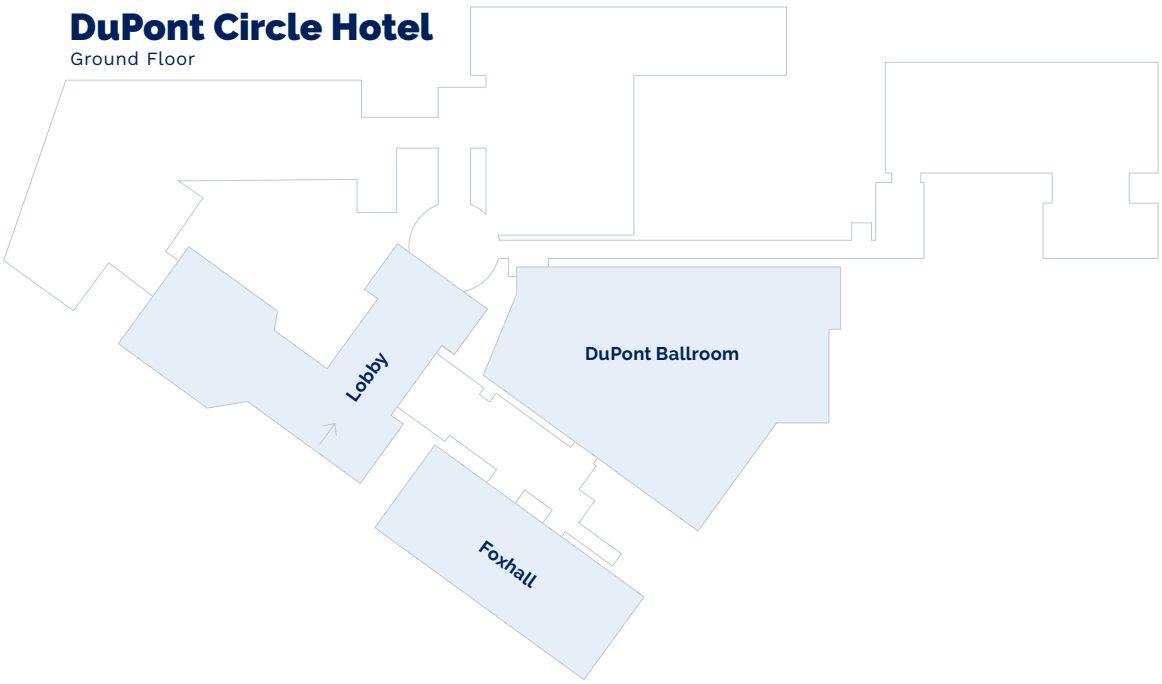
A.M.	7:30 – 8:30	Breakfast in DuPont B
	8:30 – 8:35	Welcome by Suber Huang
	8:35 – 10:10	**Session #1: Gene Therapy Approaches to Inherited Disease** Overview by Jean Bennett Panel Discussion moderated by Jean Bennett and Suber Huang • Conventional AAV (Bryan, S. Hopkins, Pennesi, Yiu, Wellman) • Strategies for large genes (Bell, Brooks, Song, Pennesi, Sorscher) • Strategies for dominant diseases with toxic gain of function mutations (S. Hopkins, LeRoy, Pierce, Singh, Stone) • CRISPR and DNA/RNA—Induced modification of physiologic status (Hardcastle, High, Pierce, Tsang, Yiu)
	10:10 – 10:25	Break
	10:25 – 12:00	**Session 2: Stem Cell Transplantation and Cell-Based Therapies for IRD** Overview by Budd Tucker Panel Discussion moderated by Budd Tucker and Suber Huang • Autologous or not (Bharti, Singh, Sorscher, Williams, Zack) • Type of cell (Clegg, Danos, Singh, Svendsen, Temple, Williams) • Role of polymer support (Baranov, Hardcastle, Lavik, Reh, Zarbin) • CGMP manufacturing considerations (Brooks, Bryan, Clegg, Su, Svendsen)
P.M.	12:00 – 1:00	Lunch in DuPont B
	1:00 – 2:20	**Session 3: Gene-Agnostic Approaches to Inherited Disease** Overview by Erin Lavik Panel Discussion moderated by Erin Lavik and Suber Huang • High-throughput drug discovery (J. Hopkins, Moroi, Shoichet, Trzupek, Young) • Small molecule therapy (Chan, Kiss, Sorscher, Yiu) • Gene therapy using “generic” approaches such as factors, which confer metabolic support, block apoptosis, reduction of oxidative stress, ad trophic factors (Hardcastle, Reh, Su, Temple, Young) • Optogenetics (Bell, Danos, Sahel, Su, Ting)
	2:20 – 2:35	Break
	2:35 – 4:00	**Session 4: Improving Clinical Trials – What’s Holding Us Back?** Overview by Mandeep Singh Panel Discussion moderated by Mandeep Singh and Suber Huang • Why “curing” disease is different than “managing” disease (Bharti, Klassen, Lavik, Sahel, Salzman) • Safeguarding patients and the public trust—The Regulatory Agencies (Chambers, Chiang, Petrou, Williams, Zarbin) • Understanding ethical and legal considerations of US and ex-US Regulatory Agencies. Is there a better way? (Bennett, Bryan, Chambers, Menzo, Stone, Truzpek) • Natural history of diseases and steps needed to approve new clinically relevant endpoints (Bell, Bryan, Csaky, S. Hopkins, Plowman) • What about glaucoma? (Baranov, Moroi, Saroj, Song, Yankovskaya)
	4:00 – 4:10	Day one review + preview of day two
	4:10	Adjourn
	5:45	Gather at hotel lobby to walk to Cosmos Club
	6:00 – 6:45	Cocktail hour
	6:45 – 7:15	**Future Vision Forum Distinguished Lecturer: Amber Salzman, Ph.D.**
	7:15 – 9:00	**Gala dinner**

Tuesday, September 12 *(Meeting held in DuPont A)*

A.M.	**7:30 – 8:30**	Breakfast in DuPont B
	8:30 – 8:35	Welcome by Suber Huang
	8:35 – 10:10	**Session 5: Improving Clinical Trials – How Can We Do Better?** Overview by Marco Zarbin Panel Discussion moderated by Marco Zarbin and Suber Huang • Development of novel biometrics and technology to permit early assessment of toxicity and efficacy of therapy (Brooks, Chambers, J. Hopkins, Plowman, Stone, Wellman) • Optimizing clinical trials and the role of clinical trial centers (Bennett, Csaky, LeRoy, Pennesi, Pierce, Sahel) • Role of artificial intelligence and federated machine learning in recruitment (Baranov, Chiang, Hardcastle, Moroi, Ting) • N1 of trials (Chambers, Bennett, Bryan, Kiss, Shoichet, Tsang) • Catalyze the cure—Role of affected individuals, families, advocacy, philanthropy (Chan, Klassen, Menzo, Salzman, Truzpek)
	10:10 – 10:25	Break
	10:25 – 12:00	**Session 6: Socioeconomics of Therapy for Rare Diseases** Overview by Jason Menzo Panel Discussion moderated by Jason Menzo and Suber Huang • Financing clinical trials, what can be done to lower the cost? (Chan, LeRoy, Schoichet, Stone, Tucker) • How is the price set for a drug? (Brooks, High, Salzman, Saroj, Yankovskaya) • How can the marketplace be incentivized to develop new therapies for rare diseases? (Danos, J. Hopkins, Saroj, Wellman, Yankovskaya) • How can patients, health systems, and stakeholders afford treatments for rare diseases? (Bell, Chiang, High, J. Hopkins, Petrou, Temple) • New paradigms in healthcare finance that accelerate biomedical innovation (Bennett, Klassen, Petrou, Salzman, Williams)
P.M.	**12:00 – 1:00**	Lunch in DuPont B
	1:00 – 4:00	**Session 7: Developing a Consensus Statement, Recommendations, and Action Steps** Overview of goals and objectives by Suber Huang Panelists include Jean Bennett, Edwin Stone, Budd Tucker, and Marco Zarbin
	4:00 – 4:05	Closing remarks, acknowledgments, adjourn, and departure

DuPont Circle Hotel

Ground Floor



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Faculty Directory

Petr Baranov, MD, Ph.D. Harvard Medical School/Massachusetts Eye and Ear	**Eric Pierce, MD, Ph.D.** Ocular Genomics Institute
Robert Bell, Ph.D. Ascidian Therapeutics	**Scooter Plowman, MD, Ph.D.** Verily Life Sciences
Jean Bennett, MD, Ph.D. University of Pennsylvania, Perelman School of Medicine	**Thomas Reh, Ph.D.** University of Washington
Kapli Bharti, Ph.D. National Eye Institute/NIH	**José-Alain Sahel, MD** University of Pittsburgh/Spring Vision
Brian Brooks, MD, Ph.D. National Eye Institute/NIH	**Amber Salzman, Ph.D.** Epic Bio
Wilson W. Bryan, Ph.D. Federal Drug Administration (Retired)	**Namrata Saroj, OD** All Eyes Consulting
Wiley Chambers, MD Federal Drug Administration	**Molly Shoichet, Ph.D.** University of Toronto
Hwei-Wuen Chan, MD National University Hospital, Singapore	**Mandeep Singh, MD, Ph.D., MBBS** Johns Hopkins University/Wilmer Eye Institute
Michael Chiang, MD, Ph.D. National Eye Institute/NIH	**Hongman Song, Ph.D.** National Institute of Health
Dennis Clegg, Ph.D. University of California, Santa Barbara	**Eric Sorscher, MD, Ph.D.** Emory University
Karl Csaky, MD, Ph.D. Retina Foundation	**Edwin Stone, MD, Ph.D.** University of Iowa/Institute of Vision Research
Olivier Danos, Ph.D. REGENXBIO	**Xinyi Su, Ph.D., MMed, FAMS, MBChir** National University Hospital, Singapore
Alison Hardcastle, Ph.D. UCL Institute of Ophthalmology	**Clive Svendsen, Ph.D.** Cedars Sinai Medical Center
Katherine High, MD SPARK Therapeutics/CRISPR Therapeutics	**Sally Temple, Ph.D.** Neural Stem Cell Institute
Jill Hopkins, MD Novartis	**Daniel Ting, Ph.D., MBBS** Singapore National Eye Center
Sam Hopkins, Ph.D. Therapetics	**Karmen Trzupek, MS, CGC** Global Genes
Suber S. Huang, MD, MBA Retina Center of Ohio/Bascom Palmer Eye Institute	**Stephen Tsang, MD, Ph.D.** Columbia University
Szilárd Kiss, MD Cornell University	**Budd Tucker, Ph.D.** University of Iowa/Institute of Vision Research
Henry Klassen, MD, Ph.D. University of California, Irvine	**Jennifer Wellman, COO** Akouos
Erin Lavik, MS, Sc.D. National Cancer Institute	**David Williams, MD** Boston Children's Hospital
Bart LeRoy, MD, Ph.D. Opus Genetics	**Yelena Yankovskaya, PharmD, MBA** Genentech
Jason Menzo Foundation Fighting Blindness	**Glenn Yiu, MD, Ph.D.** University of California, Davis
Sayoko Moroi, MD, Ph.D. Ohio State University	**Michael Young, Ph.D.** Massachusetts Eye and Ear
Mark Pennesi, MD, Ph.D. Oregon Health & Science University/Casey Eye Institute	**Don Zack, MD, Ph.D.** Johns Hopkins University/Wilmer Eye Institute
Karen Petrou Federal Financial Analytics	**Marco Zarbin, MD, Ph.D.** Rutgers University

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Dr. Ballios is a fellowship-trained clinician-scientist with a focus on medical retinal disease and a subspecialty in inherited retinal disease. He is an Assistant Professor in the Department of Ophthalmology and Vision Sciences at the University of Toronto. He holds an appointment as a clinician-scientist at the University Health Network in the Donald K. Johnson Eye Institute and is a staff physician at Sunnybrook Health Sciences Centre and the Kensington Vision and Research Centre, where he is also the Director of Clinical Electrophysiology. He is a Scientist at the Krembil Research Institute, where he has a laboratory investigating the mechanisms of acquired and inherited retinal disease and the development of new stem cell-based therapies.

Dr. Ballios obtained his MD and Ph.D. degrees at the University of Toronto in the combined MD/Ph.D. program. With a background in material science and Engineering Chemistry (Queen's University), his doctoral work focused on new approaches to the transplantation of stem cells and their progeny for the treatment of retinal degeneration. After completing his FRCSC in Ophthalmology at the University of Toronto, he undertook a subspecialty clinical fellowship in Inherited Retinal Disease at Massachusetts Eye and Ear and Harvard University.

Inherited retinal disorders (IRDs) affect an estimated 90,000 Canadians, with a total cost of disease—including healthcare costs, productivity, and well-being—at upwards of $6.7 billion. IRDs include conditions such as retinitis pigmentosa, Stargardt disease, Leber congenital amaurosis, choroideremia, cone (and cone-rod) dystrophy, and achromatopsia. More than 270 genes have been identified for inherited monogenic retinal disease. With rare exceptions, treatments do not exist for inherited conditions.

New therapies for retinal degeneration are focused on the next generation of regenerative medicines. These include gene and cell-based therapeutics, including stem cells. Several of these approaches are already being applied in clinical trials and therapies. While gene therapy has the potential to correct the underlying mechanism of disease in monogeneic disorders, it depends on the presence of viable light-sensitive cells. Stem cell therapy has the potential to replace the light-sensitive photoreceptors lost in later-stage disease, when patients have suffered significant vision loss.

Taking advantage of collaboration in fundamental neurobiology, stem cell research, and biomaterials, we developed the first injectable biomaterial-based delivery system for stem cell transplantation in the retina. We also demonstrated a method to differentiate stem cell-derived photoreceptors with unprecedented efficiency to replace damaged photoreceptors in retinal degeneration. We are developing clinical studies and trials to bring new gene and cell therapies to patients with retinal disease.

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Robert Bell, Ph.D., is the Head of Research at Ascidian Therapeutics, an RNA exon editing company with current programs in retinal, neurological, neuromuscular, and other rare disease. Prior to Ascidian, he held various roles of increasing responsibility in Pfizer's Neuroscience and Rare Disease Research Units, where he led lab-based research teams and participated in various business development activities.

Dr. Bell received a Ph.D. in Pathology, completed an AHA-funded postdoctoral fellowship at the Aab Cardiovascular Research Institute, and held a Research Assistant Professor position in the Department of Neurosurgery at the University of Rochester Medical Center.

He has authored over 30 scientific papers, served on several editorial boards, held a collaborating adjunct faculty appointment at the University of Rhode Island Institute for Neuroscience, was a steering committee member for the NIH's Accelerating Medicines Partnership for Parkinson's Disease, and is a Scientific Advisory Board member for the Hereditary Neuropathy Foundation.

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1. Bell RD (2023) RNA Exon Editing to Treat Stargardt Disease. Foundation Fighting Blindness and RD Fund Investing In Cures Summit. Invited Speaker and Panelist
2. Bell RD (2023) Delivering Novel RNA Exon Editing Therapies for Rare Diseases of the Eye and CNS. Drug Delivery to the Brain: Challenges and Progress Keystone Meeting. Invited Speaker
3. Bell RD. (2022) Considerations When Developing Blood-Brain Barrier Crossing Drug Delivery Technology. Handb. Exp. Pharmacol. 273, 83-95.
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Jean Bennett was recruited to the Scheie Eye Institute at the University of Pennsylvania's School of Medicine in 1992, where she has spent the past three decades developing gene-based strategies for treating inherited retinal degeneration. There she has run a true bench-to-bedside translational research program and, in the process, has trained hundreds of physician-scientists, many of whom are now leaders in translational research around the globe. She was the scientific leader of a team that translated the reversal of blindness in animal models to demonstrate the efficacy and safety of gene therapy in children and adults. She was the scientific director of clinical studies at the Children's Hospital of Philadelphia that led to the first FDA-approved gene therapy for genetic disease blindness due to RPE65 deficiency. Studies were sponsored by a company that she co-founded but in which she waived financial benefit (Spark Therapeutics). She helped develop the primary outcome measure for the 2017 trial that led to the first US FDA-approved gene therapy product. It also led to the approval of the reagent, "Luxturna," by the European Medicines Agency. Jean continues to develop gene-based therapies for retinal degenerative diseases and to tackle some of the limitations of current gene therapy technologies. She recently co-founded Opus Genetics to help those patients and families that suffer from conditions that are so rare that they have been neglected by big pharma.

Jean graduated from Yale College with a BS in Honors Biology, received her Ph.D. (Zoology, Cell, and Developmental Biology) from the University of California, Berkeley, and her MD from Harvard Medical School. She then received post-graduate training at Yale University and Johns Hopkins in Human Genetics and Developmental Genetics.

An internationally recognized expert in gene therapy, Dr. Bennett has authored more than 120 peer-reviewed papers. She received The Retina Research Foundation Pyron Award, the Retina International Special Recognition Award, The John Scott Award, the Albert C. Muse Prize, the Lluria Liggett Gund Award, the Charles L. Schepens Award, the Smithsonian Ingenuity Award, and was a Co-recipient of the Champalimaud Award, the Sanford Lorraine Cross Award, the Greenberg Prize to End Blindness and the Future Vision Award. She is a member of the National Academy of Medicine, the National Academy of Science, the Association of American Physicians, and the American Academy of Arts and Sciences.

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Dr. Kapil Bharti obtained his Ph.D. from J.W. Goethe University, Frankfurt, Germany, graduating summa cum laude. His Ph.D. work involved research in the areas of molecular chaperones and epigenetics. He did his postdoc at the National Institutes of Health (NIH), where he published numerous papers in the areas of transcription regulation, pigment cell biology, and developmental biology of the eye. His lab at the National Eye Institute (NEI) recently received started the first U.S. phase I/IIa trial to test an autologous iPSC-derived RPE patch in AMD patients. Currently, he is co-developing a dual RPE/photoreceptor cell therapy with Opsis Therapeutics.

He has given several keynote lectures and won several awards, including being the first Earl Stadtman Tenure Track Investigator at NEI, NIH Director's award, NEI Director's Dr. Karl Kupfer Visionary award, and 12th Sayer Vision Research Lecture at NEI for his revolutionary work on developing ocular cell-therapies.

Dr. Bharti serves on the advisory board (pro bono) of several companies and patient-advocacy groups. His current work as a Senior Investigator at NEI involves understanding the mechanism of retinal degenerative diseases using induced pluripotent stem cell-derived eye cells and tissues and developing cell-based and drug-based therapies for such diseases. He is also the Director of the NEI Intramural Research Program, where he oversees 21 research labs and six core facilities.

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Dr. Brooks received his MD and Ph.D. (pharmacology) degrees from the University of Pennsylvania and completed residency/pediatric ophthalmology fellowship training at the University of Michigan. He completed training in medical genetics and is one of only a few individuals in the US boarded both by the American Board of Medical Genetics & Genomics and the American Board of Ophthalmology. After fellowship training, he joined the National Eye Institute, where he has served as Chief of the Ophthalmic Genetics & Visual Function Branch (2014 – present) and Clinical Director (2016 – 2022).

Awards and honors include election to the American Society for Clinical Investigation (2013), the Camras Translational Research Award (Association for Research in Vision and Ophthalmology, 2011), and the Alan S. Rabson Award for Clinical Care (2017, the NIH's highest honor for excellence in clinical care). In 2010, President Obama awarded him the Presidential Early Career Award for Scientists and Engineers, the nation's highest award for early career scientists and engineers.

He currently serves as Editor-in-Chief of the journal *Ophthalmic Genetics*. He has published over 110 peer-reviewed publications, authored numerous book chapters and reviews, and has been an invited speaker throughout the US and internationally.

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Wilson Bryan is a neurologist who graduated from the University of Chicago Pritzker School of Medicine. Dr. Bryan served on the neurology faculty of the University of Texas Southwestern Medical School for 13 years. He has been an investigator on clinical trials in cerebrovascular disease and neuromuscular disorders, particularly amyotrophic lateral sclerosis.

Dr. Bryan joined the United States Food and Drug Administration (FDA) in 2000. From 2016 until his recent retirement, he served as Director of the Office of Tissues and Advanced Therapies (OTAT) in the FDA's Center for Biologics Evaluation and Research (CBER). OTAT was responsible for the regulation of gene therapies, cellular therapies, genetically-modified cells (e.g., chimeric antigen receptor T cells), tissue-engineered products, plasma protein therapeutics (e.g., immunoglobulins; coagulation factors), selected medical devices, and xenotransplantation, covering a full range of medical indications. Of the thousands of applications in the OTAT portfolio, approximately 50% were for the treatment of rare diseases. OTAT also developed processes and standards for the new Regenerative Medicine Advanced Therapy (RMAT) designation.

Dr. Bryan recently joined Greenleaf Health, Inc. (Greenleaf), an FDA regulatory consulting firm, as Executive Vice President, Drug and Biological Products.

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Wiley A. Chambers, MD, is the Director of the Division of Ophthalmology in the Office of New Drugs, Center for Drug Evaluation and Research at the Food and Drug Administration (FDA). After receiving an undergraduate degree from Colgate University, Dr. Chambers completed medical school and a residency in Ophthalmology at The George Washington University School of Medicine and Health Sciences in Washington, DC.

He is currently a Clinical Professor of Ophthalmology and Adjunct Assistant Professor of Computer Medicine at The George Washington University. He joined the FDA in 1987 as a primary reviewer for ophthalmic drug products and, in 1990, became a Supervisory Medical Officer for Ophthalmologic Drug Products. In this capacity, Dr. Chambers has supervisory responsibility for the clinical review of ophthalmologic drug products and ophthalmic therapeutic biologic products submitted to the Center for Drug Evaluation and Research.

Additionally, Dr. Chambers is the recipient of numerous Public Health Service, FDA, and Center for Drug Evaluation and Research awards for his work with the FDA, and he has served as the American Academy of Ophthalmology's Delegate to past United States Pharmacopeia Conventions.

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Dr. Chan is a Senior Consultant Ophthalmic Surgeon at the National University Hospital and Assistant Professor of Ophthalmology at Yong Loo Lin School of Medicine, National University Singapore. She serves as Program Director of the Ophthalmology Residency Program at National University Health Systems.

Dr. Chan is a specialist in medical retina, vitreoretinal, and complex cataract surgery. Her subspecialty interest is in Ophthalmic Genetics and Inherited Retinal Diseases (IRD). She leads Singapore's first dedicated IRD service caring for local and international patients. Her main research areas include phenotype-genotype correlation studies in IRD with a special focus on ABCA4-related retinopathy, for which she was awarded the NMRC grant (CS-IRG-NIG) and RNA-based therapeutics.

Dr. Chan is a founding member of the EURETINA IRD & Paediatric Retina Subspecialty Group. She is the founding leader of the Asia Pacific Inherited Eye Diseases (APIED) network with Prof Guy Chen. The network is supported by the Asia Pacific Academy of Ophthalmology (APAO) research standing committee and the Asia Pacific Society of Eye Genetics. Areas of clinical and research focus of the APIED network include standardization of phenotyping and genotyping, establishing a unified data registry, and harnessing therapeutic development in IRD within the Asia Pacific region.

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Michael F. Chiang is Director of the National Eye Institute, at the National Institutes of Health in Bethesda, Maryland. By background, he is a pediatric ophthalmologist and is also board-certified in clinical informatics. His research focuses on the interface of biomedical informatics and clinical ophthalmology in areas such as retinopathy of prematurity (ROP), telehealth, artificial intelligence, electronic health records, data science, and genotype-phenotype correlation. He is an Adjunct Investigator at the National Library of Medicine, and his group has published over 250 peer-reviewed papers and developed an assistive artificial intelligence system for ROP that received Breakthrough Status from the U.S. Food and Drug Administration.

Dr. Chiang began at NIH in November 2020. He serves as Co-Chair of a trans-NIH working group for high-value data asset sustainability, Chair of a trans-NIH clinical trials infrastructure working group, Co-Chair of a trans-NIH medical imaging working group, Co-Chair of the NIH AIM-AHEAD advisory committee, Co-Chair of the NIH Common Fund Bridge2AI program, and Co-Chair of the NIH Scientific Data Council. Before coming to NIH, he received a BS in Electrical Engineering and Biology from Stanford University, an MD from Harvard Medical School and the Harvard-MIT Division of Health Sciences and Technology, and an MA in Biomedical Informatics from Columbia University. He completed residency and pediatric ophthalmology fellowship training at the Johns Hopkins Wilmer Eye Institute. Between 2001-2010, he worked at Columbia University, where he was Anne S. Cohen Associate Professor of Ophthalmology & Biomedical Informatics, director of medical student education in ophthalmology, and director of the introductory graduate student course in biomedical informatics. From 2010-2020, he worked at Oregon Health & Science University (OHSU), where he was the Knowles Professor of Ophthalmology & Medical Informatics and Clinical Epidemiology and Associate Director of the Casey Eye Institute. He co-directed an NIH-funded T32 training program in visual science for graduate students and research fellows, as well as an NIH-funded K12 clinician-scientist program at OHSU.

He previously served as a member of the American Academy of Ophthalmology (AAO) Board of Trustees, Chair of the AAO IRIS Registry Data Analytics Committee, Chair of the AAO Artificial Intelligence Committee, Chair of the AAO Medical Information Technology Committee, and on numerous other national and local committees. He currently serves as an Associate Editor for the *Journal of the American Medical Informatics Association* and is an Associate Editor of the textbook *Biomedical Informatics: Computer Applications in Health Care and Biomedicine*. He has previously served as an Associate Editor for the *Journal of the American Association for Pediatric Ophthalmology and Strabismus*, and on the Editorial Boards for *Ophthalmology*, *Ophthalmology Retina*, and the *Asia-Pacific Journal of Ophthalmology*.

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Dr. Clegg earned his BS degree in biochemistry at UC Davis and his Ph.D. in biochemistry at UC Berkeley, where he used emerging methods in recombinant DNA to study the sensory transduction systems of bacteria. As a Jane Coffin Childs Postdoctoral Scholar at UCSF, he studied neural development and regeneration. He has continued this avenue of research since joining the UCSB faculty, with studies of extracellular matrix and integrin function in the developing eye. His current emphasis is in stem cell research, with a focus on developing therapies for ocular disease.

Dr. Clegg is the recipient of the UCSB Distinguished Teaching Award in the Physical Sciences, the Pacific Coast Business Times Champions in Health Care Award, the National Eye Institute Audacious Goals Award, the Braille Institute Award of Excellence, and served as Chair of the Department of Molecular, Cellular and Developmental Biology from 2004-2009.

He has been a Frontiers of Vision Research Lecturer at the National Eye Institute, a Keynote Lecturer at the Stem Cells World Congress, and a TEDx speaker. He is the founder and Co-Director of the UCSB Center for Stem Cell Biology and Engineering and has served on advisory boards for the California Institute for Regenerative Medicine and the National Institutes of Health Center for Regenerative Medicine. He is a Co-Principal Investigator of The California Project to Cure Blindness, a multi-disciplinary effort to develop a stem cell therapy for Age-Related Macular Degeneration.

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Dr. Csaky is the T. Boone Pickens Director of the Clinical Center of Innovation for Age-Related Macular Degeneration, Chief Executive, and Chief Medical Officer of the Retina Foundation of the Southwest. His main area of interest is studying both clinical research and drug delivery development as it pertains to retinal diseases. In particular, Dr. Csaky is involved in multiple clinical trials in retina treatment, is studying vision function assessments of patients, and developing drug delivery approaches for the treatment of various retinal diseases. In addition, Dr. Csaky has led several meetings with the FDA and NEI on evaluating novel endpoints for retinal diseases.

Dr. Csaky is a member of the Macula Society, Retina Society, and American Academy of Ophthalmology, ARVO, American Ophthalmologic Society and the American Society of Retinal Specialists. He finished a retina fellowship at the Wilmer Eye Institute, Johns Hopkins University and a post-doctoral fellowship at the National Cancer Institute. Dr. Csaky completed an internship in medicine at Duke University, his ophthalmology residency at Washington University, and was a Fulbright Scholar at the Essen Eye Clinic, Essen, Germany. He received his combined MD/Ph.D. degree from the University of Louisville. Dr. Csaky has over 140 peer-reviewed publications and book chapters.

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Olivier Danos is Executive Vice President and Chief Scientific Officer at REGENXBIO. He is a pioneer in the field of gene therapy and has dedicated his career to advancing the use of this technology to develop life-saving therapies for patients. Olivier joined REGENXBIO in 2017 from Biogen, where he was a Senior Vice President in charge of Cell and Gene Therapy. Over the past twenty years, he has played leadership roles in cell and gene therapy as Director of the Gene Therapy Consortium of the University College of London, at the Necker Hospital - Enfants Malades in Paris, as Chief Scientific Officer of Genethon and as senior director of research at Somatix Therapy Corporation.

He has held senior research positions in France at the Centre National de la Recherche Scientifique and at the Institut Pasteur. Olivier is the former President and a founding member of the European Society of Gene and Cell Therapy.

Olivier received a Master's in Genetics and Molecular Biology at the University of Paris Orsay and his Ph.D. at the Pasteur Institute and the University of Paris Diderot.

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Professor Alison Hardcastle, Deputy Director of UCL Institute of Ophthalmology, is a molecular geneticist specializing in inherited eye diseases. Her research harnesses next-generation sequencing to identify novel genomic causes of disease, including previously intractable variants such as structural variants, and non-coding mutations deep within introns or regulatory regions.

The Hardcastle Lab experimentally tests mechanisms of disease in patient-derived induced pluripotent stem cells differentiated to RPE, 3D retinal organoids, and corneal epithelial cells. These models enable the study of pathogenic variants in genomic and cellular contexts, and the development of potential therapies such as AAV gene augmentation, gene editing, and antisense oligonucleotides.

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Dr. Katherine High, a hematologist by training, began her career studying the molecular basis of blood coagulation and the development of novel therapeutics for the treatment of bleeding disorders. Her pioneering bench-to-bedside studies of gene therapy for hemophilia led to a series of studies that characterized the human immune response to gene delivery vectors. Her work evolved to encompass the clinical translation of genetic therapies for multiple inherited disorders, and as the director of the Center for Cellular and Molecular Therapeutics at The Children's Hospital of Philadelphia (CHOP), Dr. High assembled a multidisciplinary team of scientists and researchers working to discover new gene and cell therapies for genetic diseases and to facilitate rapid translation of preclinical discoveries into clinical application.

At CHOP and Penn, Dr. High was an investigator of the Howard Hughes Medical Institute and held an endowed chair at the medical school. In 2013, Spark Therapeutics, a biotechnology company based in Philadelphia, was formed based on programs that Dr. High had led at Children's Hospital, and in 2014, she joined Spark full-time as President and Head of R&D; in 2019, Spark was acquired by Roche. While at Spark, she led the team that achieved the first FDA approval of a gene therapy for a genetic disease in the US, for a rare form of congenital blindness, and led the development of a gene therapy for hemophilia B that has now completed late phase testing. Dr. High served a five-year term on the FDA Advisory Committee on Cell, Tissue, and Gene Therapies and is a past president of the American Society of Gene & Cell Therapy (ASGCT).

She received her A.B. in chemistry from Harvard University, an M.D. from the University of North Carolina School of Medicine, and business certification from the UNC Business School Management Institute for Hospital Administrators. She currently serves on the Board of Directors of CRISPR Therapeutics (NASDAQ: CRSP), and Incyte Corporation (NASDAQ: INCY), and on the Board of Trustees of the College of Physicians of Philadelphia. She is the recipient of many honors and awards, is the author of more than 200 scientific papers, and holds multiple issued patents on gene therapy. She is an elected member of the National Academy of Medicine (US), the American Academy of Arts and Sciences, the Faculty of Pharmaceutical Medicine of the Royal College of Physicians (London), and the National Academy of Sciences. She is currently a Visiting Professor at Rockefeller University in New York.

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Jill is an ophthalmologist and retinal specialist with a career spanning three decades in Ophthalmology. She has 20 years of experience in clinical medicine and academic research and over 12 years of industry experience in all stages of research development. Jill received her medical degree from McMaster University Medical School and completed her ophthalmology residency at the University of Toronto in Canada. Jill has fellowships in Medical Retina from Moorfields Hospital/University College London and Visual Electrophysiology from the University of Toronto and the University of Ottawa, Canada.

Jill spent over 15 years in clinical and academic practice, treating patients of all ages across the full spectrum of retinal diseases. She specialized in HIV-related eye disease in London and Toronto, holding cross-division appointments in Ophthalmology and Infectious Disease. She also sub-specialized in inherited retinal disorders, and her clinical and academic research included treatments for hereditary retinal degenerations, working on the Argus retinal prosthesis and the CNTF trials for retinitis pigmentosa and geographic atrophy while in practice in Los Angeles. She was an Assistant Professor of Ophthalmology at the Keck School of Medicine at the University of Southern California and held appointments at Children's Hospital Los Angeles and the University of Toronto Department of Ophthalmology and Visual Sciences.

Jill recently took on the role of SVP and Global Head of Ophthalmology Development at Novartis, leading a portfolio of drug, device, and digital development. Prior to that, she was Global Head of Personalized Healthcare in Ophthalmology at Roche-Genentech, leading a program focusing on the clinical application of data, artificial intelligence, and digital technologies to produce a meaningful impact on patient care and outcomes in eye disease. In over a decade at Roche-Genentech, she conducted research in age-related macular degeneration and diabetic eye disease, including long-acting delivery to the eye with the Port Delivery System. She spent time in early research at Unity Biotechnology as Vice President of Ophthalmology and has led large global cross-functional teams in a variety of stages of research. Originally from Canada, she currently lives in San Francisco, California, with her husband and three children.

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(Image Pending)

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Sam Hopkins is a 1980 alumnus of McDaniel College with a bachelor's degree in biology *cum laude*. In 2010, he was awarded the college's Trustee Alumni Award, and in 2016, he received the Alumni Professional Achievement Award. He currently serves as a member of the McDaniel College Board of Trustees.

After graduation from McDaniel College, he entered graduate school as the recipient of an A.D. Williams pre-Doctoral Fellowship and earned a Ph.D. in biochemistry and biophysics from the Medical College of Virginia in Richmond, Va. He then received a Wellcome Foundation Post-Doctoral Fellowship and completed post-doctoral training at the Wellcome Research Laboratories in Research Triangle Park, N.C.

With more than 39 years of experience in the pharmaceutical industry, Dr. Hopkins has held managerial, scientific, and executive leadership positions at the Wellcome Research Laboratories, Trimeris Inc., Parion Sciences, Scynexis Inc., and Autoimmune Technologies. He is currently the Senior Vice President of Therapeutics for Asklepios BioPharmaceutical Inc., a gene therapy platform company dedicated to the discovery, development, and marketing of Adeno-associated virus vectors for the treatment of patients with rare genetic disorders. He leads the company's clinical research and development efforts in the areas of metabolic/neuro-muscular diseases and hematologic disorders.

Throughout his career, he has participated in multiple aspects of anti-viral drug research and development. Most notably, while at Trimeris Inc., he was responsible for designing and implementing the non-clinical, clinical, and regulatory strategies that led to the global approval of Fuzeon® (enfuvirtide) for the treatment of HIV-1 infection, which was recognized by the U.S. Food and Drug Administration in 2003 as a major milestone in HIV-1 drug development.

A regular presenter at major national and international scientific conferences, he has co-authored over 125 abstracts, patents, and peer-reviewed journal articles, including his landmark 1998 publication in Nature Medicine, which has been referenced in the peer-reviewed scientific literature over 1,400 times. It established the HIV-1 gp41 transmembrane protein as a valid target for antiviral chemotherapeutic drug discovery and development. He has participated in private, as well as public financings and has raised more than $500 million for start-up companies from venture capital sources, National Institutes of Health grants and contracts, private equity, corporate partnerships, acquisitions, and public equity markets. In 1997, he completed the Initial Public Offering for Trimeris Inc. by registering the company on the NASDAQ. During his tenure, the market capitalization of Trimeris exceeded $4 billion dollars, after which the global sales and marketing rights for all company products were licensed to Hoffman LaRoche in 1999. In December 2021, he participated in the $4 billion dollar acquisition of his current company, Asklepios BioPharmaceutical Inc., by Bayer Pharma AG.

Sam lives in Raleigh, North Carolina, and has three children. His oldest, Ray, is a 2020 graduate of McDaniel College, his son, Will, is a 2022 graduate of Gettysburg College, and his youngest daughter, Claire, serves in the U.S. Army as a combat medic at Fort Stewart, Georgia.

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Suber S. Huang, MD, MBA, FASRS, is the CEO of the Retina Center of Ohio, Voluntary Assistant Clinical Professor of Ophthalmology at the Bascom Palmer Eye Institute, President and CEO of the Future Vision Foundation, and founder of the Future Vision Forum.

Dr. Huang is the founding Editor-in-Chief of the ASRS Retina Atlas and the Retina Image Bank—the world's largest and most comprehensive open-access resources on the retina—and a founding officer of Retina Global. In addition to being widely published, he actively lectures on and conducts clinical trials to develop gene and stem cell therapy, cortical vision prosthesis, therapy for onchocerciasis, and immunomodulatory research in diabetes and AMD.

Former leadership positions include President of the ASRS, Chair of the National Eye Health Education Program/NEI, AAO Vice-Chair of Federal Affairs and Chair of the Research, Regulatory, and External Scientific Affairs Committee, APVRS Leadership development program, Director of the Retina Diseases Image Analysis Reading Center, Director of the Visual Sciences Coordinating Center, and Professor and Vice-Chair of the Philip and Elizabeth Searle Department of Ophthalmology at Case Western Reserve University (CWRU).

Dr. Huang is an inductee to the Retina Hall of Fame, has received the "Top Doctors", "Best Doctors in America" awards each year since 2003, the AAO Secretariat (2), Senior Honor Awards, ASRS Presidential, Senior Achievement and Honor Awards. He received the APAO Jose Rizal International Medal, APVRS International Award, Chinese Vitreo-Retinal Society Senior Honor Award, Vitreo-Retinal Society of India President's Award, the Johns Hopkins University School of Medicine/Wilmer Eye Institute Distinguished Alumnus Award, CWRU Department of Ophthalmology Attending of the Year and cherishes the CWRU Humanism in Medicine award given to the faculty member who most demonstrates compassion and professionalism in the care of patients and their families. Other awards include the Rainbow Babies and Children's Hospital Pediatric Innovation, OPS J. Donald M. Gass MD award, International Congress of Ophthalmic Photographers, the National Diversity Council Leadership Excellence Award, and the Cleveland Sight Center Person of the Year. Dr. Huang has produced 12 documentary films including Seven Years of Darkness (best short documentary, National Short Film Festival) and Candle in the Distance (Rhett Buckler award, ASRS film fest).

He received his undergraduate degree at Johns Hopkins University and his medical degree from the Albert Einstein College of Medicine. Dr. Huang's ophthalmology residency was at the Wilmer Eye Institute/Johns Hopkins University, and his Vitreoretinal Diseases and Surgery fellowship was at the Bascom Palmer Eye Institute. He has graduate training from Harvard University and Wharton School of Business and an Executive MBA from the Weatherhead School of Management, CWRU.

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Szilárd Kiss, MD, FASRS, received his undergraduate degree with honors from Columbia College and medical school training at Columbia University Vagelos College of Physicians & Surgeons. He then moved to Boston for his ophthalmology residency and surgical vitreoretinal fellowship at Harvard Medical School and the Massachusetts Eye & Ear Infirmary. Szilárd was recruited to the Department of Ophthalmology at Weill Cornell in 2008, where he is now the Bob and Dolores Hope - Robert M. Ellsworth, MD Distinguished Professor in Ophthalmology, and the Chief of the Retina Service. Szilárd is the immediate past Chair of the General Faculty Council, where he now serves as Senior Councilor. Additionally, Szilárd is the Associate Dean of Clinical Compliance at Weill Cornell, overseeing compliance and regulatory matters related to patient care. Szilárd's research career started as an undergraduate at Columbia, where, in conjunction with NASA and the Department of Defense, he was part of a team that evaluated the implications of microgravity on early developmental patterning with experiments launched on the space shuttles Discovery and Columbia. Since coming to Weill Cornell, Szilárd has garnered an international reputation as a pioneer, leader, and prominent clinical expert who has had a significant impact on the practice of retina.

Szilárd has authored nearly 350 publications (20,734 citations on Google Scholar, with an h-index of 76), given over 300 invited lectureships worldwide, and serves on the Editorial Board and as a scientific reviewer to numerous journals. In addition to his scientific efforts, Szilárd is a renowned medical and surgical vitreoretinal specialist; his clinical practice draws patients from all regions of the world. When the international publication, The Ophthalmologist, selected Szilárd as one of the Top 40 Under 40 worldwide, they cited his work on '...retinal imaging, ocular gene therapy, novel therapeutic targets for ocular neovascularization, and genetic markers for retinal diseases.' In parallel with this selection, Szilárd was chosen to be part of the charter class '...of significant living and posthumous contributors to our field...' and inducted into The Retina Hall of Fame. Szilárd was the youngest inductee, recognized for 'a lifetime of...innovation and dedication to patient care, research, education, and leadership.' Additionally, Szilárd was named by Ocular Surgery News: Retina as one of the 150 Innovators in Medical and Surgical Retina worldwide, '...a compilation of specialists who either work to educate their colleagues or innovate by developing novel technologies and techniques to advance the specialty.'

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Henry Klassen is a Professor of Ophthalmology at the Gavin Herbert Eye Institute and Sue & Bill Gross Stem Cell Research Center at the University of California, Irvine. He completed a combined doctoral program at the University of Pittsburgh, followed by an internship at the Cambridge Hospital and a residency in ophthalmology at Yale Eye Center. He did a combined fellowship in medical retina and retinal transplantation research at Moorfields Eye Hospital and the Institute of Ophthalmology in London and held positions at the Children's Hospital of Orange County and the Singapore Eye Research Institute prior to joining UCI.

Dr. Klassen's research interests include the application of neural regeneration strategies to diseases of the retina and optic nerve. Current efforts are focused on the translational development of human retinal progenitor cells for use in retinitis pigmentosa and other degenerative conditions. He founded jCyte to bring this technology to market.

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Erin Lavik MS, Sc.D.

National Cancer Institute

Erin Lavik is the chief technology officer and deputy director of the division of cancer prevention at the National Cancer Institute.

Dr. Lavik's research focuses on engineering polymers to protect and repair the nervous system and treat trauma more broadly. The projects in the lab include developing intravenously administered nanoparticles to stop internal bleeding, drug delivery systems for diseases of the eye, and printing tissue models for high throughput screening applications. Dr. Lavik received her bachelor's, master's, and doctoral degrees from MIT in materials science and engineering. She has won numerous awards, including the TR100 Award in 2003 and the NIH Director's New Innovator Award in 2010. She became a Fellow of the American Institute of Medical and Biological Engineers in 2014 and a Fellow of the Biomedical Engineering Society in 2019. She is also an Associate Editor at *Bioconjugate Chemistry*.

Beyond her research, Dr. Lavik has developed classes, including Applied Tissue Engineering, where the students make and test artificial arteries and learn about the issues involved in translating the technologies from the bench to patients, as well as a new version of thermo that incorporates improvisational techniques to foster discussions about the concepts. She also collaborates with the theater department on a program to train UMBC students to work with middle schoolers to create theatrical interpretations of their science modules.

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Bart is Head of the Department of Ophthalmology, and Staff Member at the Center for Medical Genetics at the Ghent University Hospital and Ghent University. He is a Full Professor of Ophthalmology and Ophthalmic Genetics at Ghent University. Since August 2013, Bart also is a part-time (20%) attending physician at the Division of Ophthalmology & Center for Cellular & Molecular Therapeutics at Children's Hospital of Philadelphia, Philadelphia, PA, USA.

MD, Ghent University, 1992; residency in clinical genetics, Ghent University & Ghent University Hospital, 1992-1994; residency in ophthalmology, Ghent University & Ghent University Hospital, 1994-1997; certified as ophthalmologist 1997 & clinical geneticist in 2018; fellowship in medical retina and visual electrophysiology, Ghent University & Ghent University Hospital, 1997-1998; fellowship in ocular genetics, inherited retinal diseases, visual electrophysiology, medical retina & molecular genetics, Moorfields Eye Hospital & Institute of Ophthalmology, University College London, London, UK, 1998-2001 (Profs AC Bird, SS Bhattacharya & GE Holder); PhD in Medical Sciences, Ghent University, Ghent, Belgium, 2006.

Bart Leroy is an ophthalmologist and clinical geneticist specializing in inherited eye disorders. In collaboration with Profs J Bennett, AM Maguire, and T Aleman at the University of Pennsylvania and Children's Hospital of Philadelphia, Philadelphia, PA, USA, and the team in Ghent, Bart is involved in phenotyping and genotyping studies and gene therapy projects for inherited retinal blindness. He was part of the team that helped bring Luxturna to market. He is a Senior Clinical Investigator at the Research Foundation Flanders (Belgium) since 2010 until 2025. He is (co-)recipient of 28 scientific grants and prizes. Bart is a member and board member of several professional organizations, including ISGEDR, SGOF, ERN-EYE, and EURETINA.

Bart is the author and co-author of over 190 peer-reviewed publications, over 265 posters and over 300 scientific abstracts, and 14 book chapters on genetic eye disease. He frequently teaches on ophthalmic genetics and visual electrophysiology, with over 480 presentations at international scientific meetings. Bart regularly peer reviews scientific papers for 27 different international publications, as well as grant applications or career reviews for many international grant bodies and universities. He is a regular panel member for Ph.D. evaluations at leading national and international institutions and has trained over 20 international fellows.

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Jason Menzo

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Jason Menzo was appointed CEO of the Foundation Fighting Blindness on July 1, 2022. Prior to this appointment, he spent the previous four years serving as the Foundation's COO and President, leading the organization's fundraising, finance, operations, marketing, and community engagement efforts. The Foundation is the world's leading organization searching for treatments and cures of blinding retinal degenerative diseases. The Foundation has raised over $891 million since its inception and is currently funding more than 90 research projects globally.

Jason also serves on the management team for the Retinal Degeneration Fund (RD Fund), the venture arm of the Foundation. The RD Fund has nearly $100 million under management and has made investments in 10 portfolio companies to date, all aimed at fueling startup companies in the retinal degeneration space.

Prior to joining the Foundation, Jason was a co-founder and Business Unit Head of Sun Ophthalmics. In this role, he built and led the commercial team, launching the business into the well-known entity it is today.

Prior to his role with Sun Ophthalmics, he was a co-founder of the US business for Nicox SA, where he led the commercial launches of several ophthalmic brands and helped the business successfully exit by acquisition (Valeant Pharmaceuticals, 2014). He has previously held positions of increasing responsibility with Bausch + Lomb, Inspire Pharmaceuticals, and Bayer Healthcare.

Jason also serves on the Board of Directors for Retina International, the leading global umbrella organization for patient-led charities and foundations. He has also served as a member of the Global Council of Advisors and Advocates for Sightlife, a global non-profit seeking to end corneal blindness.

Sayoko Moroi MD, Ph.D.

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Sayoko Moroi is an MD, Ph.D. graduate from The Ohio State University College of Medicine and Dept. of Pathology. She completed her medicine internship at Duke University Medical Center, ophthalmology residency at Duke Eye Center, research fellowship at Duke University, and clinical glaucoma fellowship at the University of Michigan. Dr. Moroi developed as a clinician scientist with the first NEI K08 at the University of Michigan. She has served as principal investigator, multi-principal investigator, or co-investigator on numerous NIH/NEI and NIA grants, NSF grants, foundation grants, and industry-sponsored FDA glaucoma trials. After 25 years of service at the University of Michigan, she returned in January 2020 to Buckeye Nation to serve as the first female chair in the Department of Ophthalmology and Visual Sciences (DOVS).

She maintains a diverse research portfolio in genetics, glaucoma and cataract outcomes, precision medicine for eye health, technology and vision enhancement, vision health and relationships with mobility and cognitive function, and women's health. She has led the DOVS and OSU vision scientist community to their first NIH/NEI P30 core grant and first Research to Prevent Blindness New Chair Challenge Grant.

Dr. Moroi expanded her educational journey on implicit bias and is a certified facilitator through OSU Wexner Medical Center Diversity Council. Together with the outstanding faculty, staff, learners, and collaborators, Dr. Moroi and her department aspire to restore, preserve, and enhance vision to improve lives for all.

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Dr. Mark Pennesi is the Kenneth C. Swan Endowed Professorship in Ophthalmology and Molecular and Medical Genetics at Oregon Health & Science University. He is the chief of the Paul H. Casey Ophthalmic Genetics Division at the Casey Eye Institute. Dr. Pennesi attended the University of Pennsylvania, where he graduated summa cum laude with a bachelor's degree in biomedical engineering and was awarded the Herman P. Schwann award in bioengineering for exemplary scholarship. Dr. Pennesi pursued a combined MD/Ph.D. at Baylor College of Medicine in Houston, Texas. This was followed by a residency in Ophthalmology at the University of California, San Francisco. He is the recipient of an ARVO/Alcon Early Career Clinician-Scientist Research Award, An Alcon Young Investigator Award, an FFB career development award, an RPB career development award, and an FFB enhanced career development award.

Dr. Pennesi is a clinician scientist with a passion for developing novel therapeutic regimens for inherited retinal diseases. He has helped propel the Casey Eye Institute into a leader in novel therapies for inherited retinal dystrophies. He is the PI or Co-PI on numerous clinical trials, including gene augmentation therapy for RPE65-related retinopathy, ABCA4-related retinopathy, Type IB Usher syndrome, CNGA3 and CNGB3-related achromatopsia, X-linked retinoschisis, X-linked retinitis pigmentosa, and choroideremia. He is a principal investigator on the Editas Brilliance, where the first patient was treated with gene editing from CEP290-related retinopathy.

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Karen Petrou is the co-founder and Managing Partner of Federal Financial Analytics, Inc., a privately-held company that, since 1985, has provided analytical and advisory services on legislative, regulatory, and public-policy issues affecting financial services companies doing business in the U.S. and abroad. Petrou is a frequent speaker on topics affecting the financial services industry. In addition to testifying before the U.S. Congress, she has spoken before the Federal Reserve Banks of New York, St. Louis, San Francisco, and Chicago, the European Central Bank, the Office of the Comptroller of the Currency, the International Monetary Fund, the Clearing House, the Bank Policy Institute, the Institute of International Bankers, the Securities Industry and Financial Markets Association, the Japanese Diet, and many other governmental, industry and academic groups.

Petrou is the author of the book *Engine of Inequality, the Fed, and the Future of Wealth in America*. She has also authored numerous articles in publications such as the American Banker and the Financial Times and is frequently quoted as a bank policy expert in the *Wall Street Journal*, Bloomberg, Politico, the Hill, and other media outlets. Prior to founding her own firm in 1985, Petrou worked in Washington as an officer at Bank of America, where she began her career in 1977.

She is an honors graduate in Political Science from Wellesley College and also was a special student in an honors program at the Massachusetts Institute of Technology. She earned an M.A. in that subject from the University of California at Berkeley and was a doctoral candidate there. She has served on the boards of banking organizations and now sits as a director on the board of the Foundation Fighting Blindness and the Fidelco Guide Dog Foundation. In 2019, she and her late husband Basil were named “visionaries” by the Foundation Fighting Blindness.

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Dr. Eric Pierce is the Chatlos Professor of Ophthalmology at Harvard Medical School and the founding Director of the Ocular Genomics Institute (OGI) at Mass Eye and Ear. Dr. Pierce received his A.B. in Biochemistry from Dartmouth College, his Ph.D. in Biochemistry from the University of Wisconsin-Madison, and his M.D. from Harvard Medical School. He did his residency in Ophthalmology at Harvard and fellowship in Pediatric Ophthalmology at Children's Hospital, Boston, where he also took his first faculty position. He was then recruited to the Department of Ophthalmology at the University of Pennsylvania School of Medicine, where he was promoted to Associate Professor with tenure. He returned to Harvard in 2011 to establish the OGI.

The mission of the OGI is to translate the promise of precision medicine into clinical care for patients with inherited eye diseases. Dr. Pierce's research program is focused on improving our understanding of the genetic causality of inherited retinal degenerations (IRDs) and developing genetically informed therapies for these disorders. His work has led to the identification of several IRD disease genes, helped highlight the importance of non-coding mutations in IRDs, and supported the clinical translation of genetic therapies for several genetic forms of retinal degeneration. Dr. Pierce has been an investigator for multiple first-in-human clinical trials of genetic therapies for IRDs and is the senior PI for the Brilliance trial of in vivo genome editing for the treatment of CEP290-associated retinal degeneration. Dr. Pierce's research program has been funded by the NIH since 1994.

He has also received support from the Foundation Fighting Blindness, and Research to Prevent Blindness, among other foundations. Dr. Pierce received an Alcon Research Award and RPB Stein Innovation Award and is the 2023 recipient of the Proctor Medal from ARVO. He served for 10 years as the Chair of the Scientific Advisory Board for the Foundation Fighting Blindness, as a member of the National Advisory Eye Council, and is currently Chair of the Pathophysiology of Eye Disease 1 (PED1) study section of the NIH.

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Dr. Scooter Plowman is a physician leading clinical science strategy and development for Medical Devices at Verily Life Sciences. He has an M.D., M.B.A., and MHSA from the University of Kansas. He also graduated from Oxford University with an MSc in Diagnostic Imaging and has bachelor's degrees in Neuroscience, Geography, and Latin American Studies from BYU. His clinical residency training in radiology was at Mayo Clinic and Intermountain Healthcare, with fellowship training in Health System Design & Innovation at Stanford, where he still serves on the affiliate faculty.

Before Verily, Scooter was the Head of Digital Medicine at Amgen, responsible for digital endpoint development, and has held leadership roles in medical affairs and clinical development. He lives with his wife and six children in Southern California and enjoys hiking, college football, and racquet sports.

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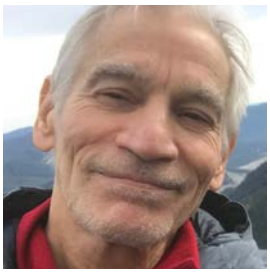

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Thomas A. Reh, Ph.D., is a Professor in the Department of Biological Structure at the University of Washington-Seattle. He received his Ph.D. in Neuroscience from the University of Wisconsin-Madison. Prior to joining the UW faculty in 1989, he held faculty positions at Princeton and the University of Calgary. He was Director of the UW Neuroscience Program from 2001 to 2007 and currently serves on the Scientific Advisory Board of the Foundation Fighting Blindness, Nayan Pharma, Vesigen, and the editorial boards of several journals.

He has received numerous awards for his research, including the Jerzy E Rose Prize, the FFB Directors' Award (twice), the Alfred Sloan Scholarship, the Paul G. Allen Distinguished Investigator Award, and the Gund-Harrington Scholar Award. He has over 150 publications in the field of retinal regeneration and development.

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José-Alain Sahel, MD, trained at Paris University Medical School, Louis Pasteur University, Strasbourg, and Harvard University. He is the Endowed Distinguished Professor and Chairman of the Department of Ophthalmology at the University of Pittsburgh School of Medicine/UPMC. He founded the Vision Institute (Sorbonne Université-Inserm-CNRS) in Paris, France, and the Vision Institute at UPMC, opening in May 2023, two large interdisciplinary research centers. His work has contributed to the understanding and prevention of vision loss from photoreceptor cell degeneration.

He conducted dozens of clinical trials on retinal conditions, including first-in-human trials of artificial retina, gene therapy, and optogenetics. He co-authored over 700 peer-reviewed articles and 40 patents. He has co-founded companies developing innovative therapies for vision restoration. He is a member of the Académie des Sciences-Institut de France, the Académie des Technologies, the Association of American Physicians, the American Ophthalmology Society, the German National Academy of Sciences Leopoldina. He holds an Honoris Causa Doctorate from the University of Geneva and held the Chaire d'Innovation Technologique Liliane Bettencourt at Collège de France (2015-2016).

He received, among numerous awards, the Foundation Fighting Blindness (FFB) Trustee Award, the Alcon Research Institute Award for Excellence in Vision Research, the Grand Prix NRJ-Neurosciences-Institut de France, the 2015 Foundation Fighting Blindness Llura Liggett Gund Lifetime Achievement Award, the Médaille Grand Vermeil, Ville de Paris (2019), the 2021 Breakthrough in the Life Sciences - Falling Walls Foundation, Berlin, Germany, the 2022 Chica and Heinz Schaller Foundation Award for Translational Neuroscience. And the 2023 International Prize for Translational Neuroscience from the Max Planck Society, Germany.

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Amber Salzman, Ph.D., is the CEO of Epic Bio, a company developing next-generation CRISPR therapies to control gene expression dynamically and treat complex diseases. Previously, she led Ohana Biosciences, leveraging sperm biology to improve assisted reproductive outcomes. Prior to that, she served as the President & CEO at Adverum Biotechnologies (NASDAQ: ADVM), a gene therapy company targeting unmet medical needs in ocular and rare diseases. Amber was a co-founder of Paris-based Annapurna Therapeutics and served as its President & CEO prior to its merger with Avalanche Biosciences to create Adverum. Before her role at Adverum, Amber served as President and CEO of Cardiokine until it was acquired by Cornerstone Therapeutics.

Earlier in her career, Amber was a member of GlaxoSmithKline's research and development executive team, where she was responsible for planning and managing global drug development projects and clinical trials with over 30,000 patients across the US, Europe, Asia, and South America. She also led initiatives to accelerate drug development and established a development support center in India. Amber serves on two UK Boards, including Osler Diagnostics and Aviado Bio.

In addition to her industry roles, Amber is President of the Stop ALD Foundation, a Medical Research Foundation dedicated to finding better therapies for people with adrenoleukodystrophy. In this capacity, she was instrumental in developing a gene therapy treatment for ALD and getting ALD added to the Recommended Uniform Screening Panel (for newborns). She also serves on the Boards of the Lankenau Institute of Medical Research and Drexel University's Dornsife School of Public Health. Amber holds a BS in computer science from Temple University and a Ph.D. in mathematics from Bryn Mawr College.

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Namrata Saroj, OD, is an independent consultant with over 20 years of experience focusing on developing new drugs and technologies for advancing ophthalmic care. Prior to this, she led Ophthalmology Medical Affairs as an Executive Director at Regeneron Pharmaceuticals, Inc supporting the commercialization of EYLEA and was involved in strategic planning and activities supporting new ophthalmology products in development. Prior to joining Regeneron, Namrata held positions at Genentech in a field-based Medical Affairs function supporting Lucentis. She started her career as a Research Associate at the Manhattan Eye, Ear & Throat Hospital, focusing on clinical trials in retinal diseases. Namrata has successfully supported multiple investigator-initiated studies and research collaborations, as well as co-authored multiple publications in the management of retinal diseases.

Namrata earned her Doctor of Optometry from the University of California at Berkeley. She holds a Bachelor of Science in Optometry from the University of California and a Bachelor of Arts in Biochemistry from Whittier College.

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Professor Molly Shoichet is a University Professor, a distinction held by less than 2% of the faculty at the University of Toronto, and Michael E. Charles Professor of Chemical Engineering. Dr. Shoichet served as Ontario's first (and only) Chief Scientist in 2018, where she worked to enhance the culture of science.

Dr. Shoichet has published over 650 papers, patents, and abstracts and has given over 420 lectures worldwide. She currently leads a laboratory of 30 and has graduated 275 researchers. Her research is multi-disciplinary and highly collaborative, focused on drug and cell delivery strategies in the central nervous system (brain, spinal cord, retina) and 3D hydrogel culture systems to model cancer.

Dr. Shoichet co-founded four spin-off companies, is actively engaged in translational research and science outreach, including co-Founding AmacaThera and Research2Reality.

Dr. Shoichet is the recipient of many prestigious distinctions and the only person to be inducted into all three of Canada's National Academies of Science of the Royal Society of Canada, Canadian Academy of Engineering, and Canadian Academy of Health Sciences. Professor Shoichet is an Officer of the Order of Canada and the Order of Ontario. Dr. Shoichet is the L'Oreal-UNESCO For Women in Science Laureate for North America, elected Foreign Member of the US National Academy of Engineering, elected Fellow of the National Academy of Inventors, won the Killam Prize in Engineering and elected to the Royal Society (UK). Dr. Shoichet was awarded the NSERC Herzberg Gold Medal—Canada's top prize in science and engineering—and the Margolese National Brain Disorders Prize.

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Mandeep S. Singh MD, Ph.D. holds the Andreas C. Dracopoulos Professorship of Ophthalmology and is Associate Professor of Ophthalmology and Genetic Medicine at the Johns Hopkins Wilmer Eye Institute. He is a vitreoretinal surgeon and a clinician-scientist in hereditary retinal diseases. He is the founding co-director of the Johns Hopkins Genetic Eye Diseases (GEDi) Center, where he coordinates multidisciplinary clinical care, research, and education pertaining to genetic diseases of the eye and visual system.

In his translational neuroscience laboratory, his team focuses on retinal stem cell therapy, specifically on photoreceptor transplantation as an approach to promote visual recovery in animal models of hereditary retinal diseases. His work – encompassing pluripotent stem cells, human retinal organoids, and animal models of genetic and acquired retinopathy – has contributed to the understanding of mechanistic models of photoreceptor repair based on synaptogenesis and cellular materials transfer. In the realm of clinical research, he leads several gene therapy clinical trials at Johns Hopkins and is the Study Chair of the Foundation Fighting Blindness (FFB) Clinical Consortium's Gyrate Atrophy Ocular and Systemic (GYROS) Study, a prospective international natural history study focusing on the retinal and metabolic consequences of ornithine aminotransferase gene mutations. In addition, he is a member of the FFB Regulatory Endpoints and Trial Design for Inherited Retinal Diseases (REDi) Working Group. His research in genetic ophthalmology also encompasses the application of artificial intelligence and the bioethics of clinical experimentation in people experiencing progressive and irreversible vision loss from genetic eye diseases.

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Dr. Song joined the Division of Extramural Science Programs (DESP) at National Eye Institute (NEI) in 2019 and currently serves as the Director of the Glaucoma Program. She manages research grants that focus on glaucoma genetics and genomics, the cell biology of outflow, the physiology of IOP regulation, and the development and testing of therapeutics to the anterior segment. In addition, she is the Inclusion Policy Officer, NEI contact for Alzheimer's Supplement, and NEI representative for the Office of Prevention Disease (ODP)/OD. Dr. Song is heavily involved in initiating programs to address the research priorities in the NEI Strategic Plan. Dr. Song also represents the NEI as Program Officer contact on the NIH/FNIH Bespoke Gene Therapy Consortium (BGTC) AAV Biology and Clinical subcommittees. She is currently a member of the NIH Coordinating Committee on Research on Women's Health and NEI's Data Access Committee.

Dr. Song studied Clinical Medicine at the Wuhan University School of Medicine, where she obtained her MD degree and had been an ophthalmologist for 10+ years. She completed a Ph.D. in Biochemistry and Molecular Biology at the West Virginia University School of Medicine. She then pursued a postdoctoral fellowship and transitioned to a research scientist in the Section for Translational Research in Retinal & Macular Degeneration (STRRMD) at the National Institute on Deafness and Other Communication Disorders (NIDCD) and NEI.

As a research scientist, Dr. Song led and managed multiple research projects, which focused on the molecular mechanisms underlying retinal degenerative diseases to develop novel therapeutics, AAV retinal gene therapy, and retinal cell biology with an emphasis on rod photoreceptor outer-segment morphogenesis and homeostasis.

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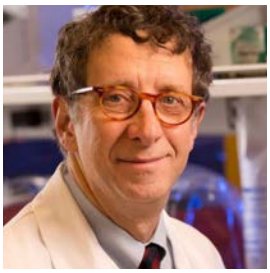

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Dr. Sorscher is the Hertz Endowed Professor at Emory University School of Medicine and a GRA Eminent Scholar. He graduated from Harvard Medical School and completed his internship and residency in internal medicine at the Massachusetts General Hospital. For many years, he directed the cystic fibrosis research program at the University of Alabama at Birmingham, where he pursued basic and translational studies of CFTR. He moved to his current position at Emory approximately eight years ago, where he has continued work on cystic fibrosis. For many years, Sorscher chaired the International CFTR Folding Consortium and currently chairs the Medical Advisory Council for CF Foundation and the “Path to a Cure” committee for the North American Cystic Fibrosis meetings.

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Edwin M. Stone is the Director of the University of Iowa Institute for Vision Research. He is best known for his work in defining the genetic basis of blinding eye diseases: ranging from two of the most common causes of blindness, macular degeneration and glaucoma, to much rarer conditions like retinitis pigmentosa and Leber congenital amaurosis.

Dr. Stone has been very active in removing the technical, legal, and financial barriers between genetic discoveries and the patients who could benefit from them. He founded the Carver Nonprofit Genetic Testing Laboratory at the University of Iowa, which provides low-cost genetic tests to patients in every state of the U.S. and more than 60 other countries. He also created an open-access web-based teaching tool with thousands of downloadable full-resolution images to help physicians around the world improve their ability to diagnose Mendelian retinal diseases (www.stonerounds.org).

His current research interest is in developing affordable gene- and stem-cell-based treatments for all molecular forms of inherited retinal disease. Dr. Stone received his M.D. and Ph.D. from the Baylor College of Medicine and his training in ophthalmology and vitreoretinal surgery at the University of Iowa, where he joined the faculty in 1990. He holds the Seamans-Hauser Chair of Molecular Ophthalmology at the University of Iowa Carver College of Medicine.

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Dr. Su, an A*STAR scholar, graduated with an MB BChir Ph.D. from the University of Cambridge (UK) and joined IMCB in 2016 as a clinician-scientist. She is currently the Acting Executive Director of the Institute of Molecular and Cell Biology (IMCB, A*STAR), where she runs a broad retinal research program as Senior Principal Investigator, and balances her time with providing clinical leadership as Senior Consultant Vitreo-Retinal Surgeon at the National University Hospital. Dr Su also holds joint appointments as Research Director at the Department of Ophthalmology, National University of Singapore, and Clinician-Scientist at the Singapore Eye Research Institute.

Her research interests focus on harnessing biomaterial, regenerative stem cells, and nucleic acid technologies for the treatment of age-related retinal degenerative disease. Her research has been published in, inter alia, Nature Biomedical Engineering, Nature Communications, Lancet Global Health, PNAS, and Advanced Materials. She has a career total of over SGD25 million in competitive research grants and is the recipient of multiple global and national awards, including the Asia-Pacific Academy of Ophthalmology's Young Ophthalmologist Award (2019), the Asia-Pacific Vitreo-Retinal Society Leadership Development Program Gold Award (2020), Ten Outstanding Young Persons of Singapore Award (for Medical Innovation, 2021), the Susan Lim Outstanding Stem Cell Young Investigator Award (2022) and National Medical Research Council Clinician Scientist Award (2022). In 2022, she was accepted into the prestigious international membership of The Macular Society.

Passionate about the clinical translation of research, Dr. Su holds several patents and co-founded an ISO 13485 (Medical Device Quality System) accredited spin-off company, Vitreogel Innovations, focussed on developing the next generation of vitreous substitutes. Beyond research, Dr. Su is committed to people development and has mentored numerous clinician-scientists as the Deputy Director of the Clinician-Scientist Academy, National University Health System

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Dr. Clive Svendsen's predoctoral training was at Harvard University, and he received his Ph.D. from the University of Cambridge in England, where he subsequently became a Welcome Fellow and established a laboratory focusing on stem cell research. He then moved to the University of Wisconsin in 2000 as Professor of Neurology and Anatomy and founded their Stem Cell and Regenerative Medicine Center.

In 2010, he moved to Los Angeles and founded the Cedars-Sinai Board of Governors Regenerative Medicine Institute, which currently has 28 faculty members and over 150 staff. Dr. Svendsen maintains a large lab that focuses on using patient-derived induced pluripotent stem cells to model diseases, including Spinal Muscular Atrophy, Parkinson's Disease, ALS, and recently to model BRCA1-induced cancer. The other focus of Dr. Svendsen's lab involves cutting-edge clinical trials using combinations of stem cells and growth factors. He spearheaded one of the first clinical trials to deliver a growth factor, GDNF, to patients with Parkinson's Disease, and is now developing novel ways to use stem cells modified to release GDNF for Parkinson's patients. He is also using stem cells for other degenerative diseases. He is the sponsor for an ongoing clinical trial using stem cells to treat the incurable eye disease Retinitis Pigmentosa.

Finally, Dr. Svendsen has a long-standing interest in ALS, and he was the Sponsor of the first-ever clinical trial delivering stem cells and GDNF to the spinal cord of ALS patients that was recently completed at Cedars-Sinai, and he is the Sponsor of an ongoing trial delivering stem cells and GDNF to the motor cortex of ALS patients.

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Dr. Sally Temple is the Scientific Director of the Neural Stem Cell Institute and oversees scientific programs with the goal of understanding the role of neural stem cells in Central Nervous System (CNS) development, maintenance, and repair. A native of York, England, Dr. Temple leads a team of 30 researchers focused on using neural stem cells to develop therapies for eye, brain, and spinal cord disorders. In 2008, she was awarded the MacArthur Fellowship Award for her contribution and future potential in the neural stem cell field.

Dr. Temple received her undergraduate degree from Cambridge University, Cambridge, UK, specializing in developmental biology and neuroscience. She performed her Ph.D. work in optic nerve development at University College London, UK. She received a Royal Society fellowship to support her postdoctoral work at Columbia University, NY, where she focused on spinal cord development.
In 1989, Dr. Temple discovered that the embryonic mammalian brain contained a rare stem cell that could be activated to proliferate in vitro and produce both neurons and glia. Since then, her lab has continued to make pioneering contributions to the field of stem cell research, by characterizing neural stem cells and the intrinsic and environmental factors that regulate their behavior. Her lab's research on the characterization of neural stems and progenitors brings us closer to developing effective clinical treatments for central nervous system damage in which tissue is lost, for example, due to neurodegenerative diseases or trauma.

As the Scientific Director of NSCI, Dr. Temple oversees the research mission from basic to translational projects. Dr. Temple is a member of the board of directors and is a past president of the International Society for Stem Cell Research.

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Dr. Daniel Ting is the Associate Professor at Duke-NUS Medical School, senior consultant vitreo-retinal surgeon and Chief Data and Digital Officer of the Singapore National Eye Center, Head of AI and Digital Innovation at the Singapore Eye Research Institute (SERI), Director of Singapore Health Service AI Office, and the innovation mentor at Byers Eye Institute, Stanford University. He was also the 2017/2018 US-ASEAN Fulbright Scholar visiting Johns Hopkins University to exchange expertise in AI and big data for medicine. Dr. Ting's research focus is related to AI and digital health-related applications for eye and retinal diseases that span across machine learning, deep learning, privacy-preserving technology such as blockchain technology, federated machine learning, and generative adversarial network, satellite technology (4G and 5G), conversational AI chatbot using natural language processing and cybersecurity (e.g., adversarial attack).

To date, Daniel has published >200 peer-reviewed publications in high-impact journals, such as JAMA, NEJM, Lancet, Nature Medicine, Nature Biomedical Engineering, Lancet Digital Health, Progress in Retinal and Eye Research, Diabetes Care, Nature Digital Medicine, Ophthalmology and etc. Of those, 45 were published in high-impact journals (IF >10), either as the 1st, last, or corresponding author's co-authors. Daniel has received > 50M research grants, of which 10 M as a principal investigator, and 40M as co-investigators on AI and digital innovation-related projects in health.

Dr. Ting serves in several AI leadership executive positions at the different international AI and eye societies, including the EXCO in American Academy of Ophthalmology, Equator Network for AI guidelines - STARD-AI, QUADAS-AI and DECIDE-AI, International Agency for Prevention of Blindness (IAPB) technology taskforce, and he also chairs the AI and Digital Innovation Standing Committee for the Asia-Pacific Academy of Ophthalmology and Asia-Pacific Vitreo-Retinal Society. He also serves on numerous editorial boards in top-tiered digital and medical journals, including Associate Editor for Nature Digital Medicine (Tier 1 for Digital Journal), Section Editor in British Journal of Ophthalmology (Tier 1 for Ophthalmology), and Editor in Ophthalmology (Tier 1 for Ophthalmology).

For the accomplishment, Dr. Ting was recognized by many top-tiered international AI and ophthalmology societies in winning many prestigious scientific awards, including US ARVO Bert Glaser Award for Innovative Research in Retina, US Macula Society Evangelos Gragoudas Award, MICCAI OMIA Prestigious Achievement Award, Tatler Asia Gen T Award, Asia-Pacific Academy Ophthalmology (APAO) Nakajima Award, Asia-Pacific Vitreo-Retinal Society Ian Constable Award, APAO Young Ophthalmologist's Award, and APTOS Young Innovator Award. In 2022 and 2023, Daniel was ranked on the Top 100 Ophthalmologists Power list by the Ophthalmologists. In 2022, he was also ranked top 0.001% (Top 100 in the clinical domain) in the World's Top 2% Scientists by the Stanford University world ranking. In 2021 and 2022, he is also ranked 1st for deep learning, 4th for AI, and 6th for machine learning in the world across all domains (>55K researchers) for the past 10 years (2010-2021) by the ExpertScape.

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Karmen Trzupek currently serves as the Sr. Director of Scientific Programs at Global Genes, a position that enables her to bring together her passion for supporting rare disease communities and her experiences with academia, telemedicine, advocacy, and industry.

Karmen has a long background and interest in inherited retinal diseases, and previously worked at the Casey Eye Institute in Portland, Oregon, providing counseling and education to patients and families with inherited eye diseases and coordinating clinical and molecular research. She developed the first nationwide telemedicine program for ocular genetic counseling and genetic testing at InformedDNA, and co-developed both pharma- and advocacy-sponsored genetic testing programs, as a consultant to Spark Therapeutics and the Foundation Fighting Blindness. She also spent more than 10 years serving on the Board of Directors for the Usher Syndrome Coalition and the Hear See Hope Foundation.

At Global Genes, Karmen drives collaborative programming and partnerships to maximize the RARE-X data platform for the advancement of patient-driven research and industry-supported therapeutic pipelines. In partnership with multiple global patient advocacy organizations, Karmen currently leads the Vision Consortium: a collaboration aimed at developing open access to robust research data across rare genetic disorders of vision loss to accelerate therapeutic development.

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Stephen H. Tsang MD, Ph.D. cares for individuals with retinal degenerations and is known worldwide for his pivotal contribution in metabolome engineering as a therapeutic avenue. He has been culturing embryonic stem (ES) cells since 1992 and created the first mouse model for a recessive form of retinitis pigmentosa (RP) by applying genome engineering to ES cell technology in 1995. He successfully treated preclinical models of Pde6a Pde6b Mfrp Rho Cngb1 and autosomal recessive bestrophin retinopathies. He has expertise in designing and testing genome engineering strategies in pre-clinical models developing patient-specific knock-in models generating of patient cell lines and providing care to patients with a precision medicine approach. He is also leading efforts in FDA trials for gene therapies including PDE6A RAB geranylgeranyl transferase RPGR CNGB3 CNGA3 and ABCA4 retinopathies. He wrote 4 books: “Precision Medicine CRISPR and Genome Engineering: Moving from Association to Biology and Therapeutics” and “Stem Cell Biology and Regenerative Medicine in Ophthalmology” Springer Press NY and “Stem Cell Biology and Regenerative Medicine in Ophthalmology” Springer Press NY. He is an elected member of several honorary societies including the American Society for Clinical Investigation Alcon Research Institute and American Ophthalmological Society. He is consistently named to various NIH study sections (DPVS standing member 2014-8; PED2 standing member 2022-6).

He graduated from Johns Hopkins University where he began his medical genetics training under the tutelage of Professor Victor A. McKusick. He received his MD/Ph.D. degrees from the NIH-National Institute of General Medical Sciences Medical Scientist Training Program (MSTP) at Columbia University. Dr. Tsang’s contributions has being recognized by the 2005 “Bernard Becker-Association of University Professor in Ophthalmology”-“Research to Prevent Blindness” Award Carl Camras Award the 2013 Bradley Straatsma Lectureship 2018 Young Investigator Award Macular Society. Dr. Tsang received 2008 resident teaching award and is the Columbia ophthalmology basic science course director (2006-2011). He is an elected member of several honorary societies including the American Society for Clinical Investigation Alcon Research Institute and American Ophthalmological Society (Thesis Committee chair, 2002-3).

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Budd Tucker was born and raised in a small fishing town on the coast of Newfoundland's Great Northern Peninsula, in northeast Canada. His formative years were spent on his father's commercial fishing boats, where during the summer, they fished the waters of the North Atlantic for Cod, Shrimp, Ocean Perch, and Greenland Halibut. In the fall of 1996, he attended Sir Wilfred Grenfell College, where he became the first person in his nuclear family to obtain a college degree. At the age of 23, Budd left commercial fishing behind and attended graduate school full time. In 2006, he obtained his Ph.D. degree in neuroscience under the mentorship of Dr. Karen M. Mearow at Memorial University of Newfoundland, School of Medicine. He subsequently moved to Boston to complete post-doctoral training under the mentorship of Dr. Michael J. Young at the Schepens Eye Research Institute, Harvard Medical School, where in 2009, he was promoted to the rank of faculty.

In 2010, Budd joined the Institute for Vision Research and the Department of Ophthalmology and Visual Science at the University of Iowa, where he is currently a full professor and holds the Ruby Endowed Chair of Regenerative Ophthalmology. He directs both the Ruby Retinal Engineering Laboratory, which is focused on the development of novel tissue engineering and robotic strategies for the production of autologous photoreceptor cell grafts, and the Dezii Translational Vision Research Facility, a cGMP manufacturing suite with ISO class 5 capabilities dedicated to the production of gene and cell-based therapeutics. The major focus of his laboratory is to combine state-of-the-art patient-specific induced pluripotent stem cells, CRISPR-based genome editing, and tissue engineering technologies to develop affordable gene and autologous photoreceptor cell replacement strategies for the treatment of patients with inherited retinal degenerative blindness.

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Jennifer Wellman has more than 20 years' experience in adeno-associated viral (AAV) vector gene therapy research and development. She is currently Chief Development Officer at Akouos, a wholly owned subsidiary of Eli Lilly and Company and a precision genetic medicine company focused on developing gene therapies with the potential to restore, improve, and preserve high-acuity physiologic hearing for individuals who live with disabling hearing loss. Previously, Ms. Wellman served as Akouos's Senior Vice President of Regulatory and Quality. Prior to that, Ms. Wellman was Co-founder, Head of Product Development Strategy, and Head of Regulatory Strategy at Spark Therapeutics, Inc., a member of the Roche Group and a fully integrated, commercial gene therapy company. While at Spark, and in her earlier role as Director of Regulatory for the Center for Cellular and Molecular Therapeutics at Children's Hospital of Philadelphia (CHOP), Ms. Wellman led the regulatory and clinical development for several AAV gene therapies, including Luxturna®, the first FDA-approved gene therapy for a genetic condition. Prior to her time at CHOP, she served as an Associate Scientist at Avigen, Inc.

In her current role leading Akouos's clinical, regulatory, and quality teams, Wellman is spearheading the clinical development of the first potential AAV gene therapies for inner ear conditions, including the first such product candidate to receive Orphan Drug and Rare Pediatric Disease designations from the U.S. FDA. She is widely regarded as a pioneer in the development of novel genetic medicines and has dedicated more than two decades to creating gene therapies, including for rare congenital eye and ear conditions.

Ms. Wellman also serves as a board member for the American Society of Gene & Cell Therapy, working to advance knowledge and awareness of, and education about, gene and cell therapies, with the hope of preventing and curing diseases. Ms. Wellman holds an M.S. from the University of New Haven and an Honours B.S. in microbiology and immunology from Queen's University (Canada).

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Dr. Williams is the Chief of the Division of Hematology/Oncology, and the Leland Fikes Professor of Pediatrics at Harvard Medical School. He previously served as Senior and Executive Vice President and Chief Scientific Officer of Boston Children's Hospital and President of the Dana Farber/Boston Children's Cancer and Blood Disorders Center. Williams originally trained in hematology/oncology at Boston Children's Hospital and Dana-Farber Cancer Institute. During his fellowship research at the MIT Cancer Center and the Whitehead Institute, he developed techniques that allowed for the introduction of genes into murine and human hematopoietic cells. Those techniques are still commonly utilized today. He was the inaugural Director of the Herman B Wells Center for Pediatric Research at Indiana University from 1991 – 2001.

Prior to rejoining Children's in December 2007, he was at Cincinnati Children's Hospital Medical Center (CCHMC), where he was the founding director of the Division of Experimental Hematology, attending physician in hematology/oncology, and associate chair for Translational Research in the Department of Pediatrics. He was a Howard Hughes Medical Institute Investigator for 16 years, and his laboratory has been continuously funded by the NIH since 1986. He has trained over 60 fellows and post-doctoral fellows and numerous residents and medical students in his laboratory, the majority of whom are still in academic medicine. He is a member of the National Academy of Medicine and has published over 400 peer-reviewed manuscripts and textbook chapters. He formerly served on the NIH Recombinant DNA Advisory Committee and Gene Therapy Safety Assessment Board. He is actively involved in gene therapy trials for hematologic, immunodeficiency, and neurological genetic diseases and has been the investigator, co-investigator, or sponsor (IND holder) of multiple previous gene therapy trials and is the sponsor or investigator of four current trials.

He served as the Editor-In-Chief of Molecular Therapy from 2004 – 2009 and is co-founder of the Transatlantic Gene Therapy Consortium and the North American Pediatric Aplastic Anemia Consortium (NAPAAC). His basic research has focused on hematopoietic stem cell biology, including genetic diseases of the blood and specifically molecular and biochemical analysis of the interaction between hematopoietic stem cells and the bone marrow supporting environment. His laboratory has significant experience in stem cell biology, hematopoiesis, and gene correction and transfer techniques. His basic research has focused on hematopoietic stem cell biology, including genetic diseases of the blood, and his laboratory has studied Rho GTPases for over 25 years resulting in his laboratory description of the molecular basis for three rare human diseases due to mutations of GTPases RAC2, RHOH and most recently SEPTIN6. He has multiple patents, of which two have been developed into FDA-approved drugs (Neumega™ and Retronectin™), and is co-founder of two biotech companies, Orchard Therapeutics and Alerion Biosciences. Dr. Williams has served as an expert consultant for insert site analysis for multiple biotech companies. He served as the coordinating investigator for the pivotal trial for eli-cel™ product, serving from protocol conception through expert testimony at the FDA FDA BLA 125755 Elivaldogene automcel (eli-cel) application Advisory Committee meeting. Dr. Williams is a past President of the International Society of Experimental and the American Society of Hematology.

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Born in Ukraine and growing up in the Philadelphia area, Dr. Yankovskaya received her Doctor of Pharmacy degree from the Philadelphia College of Pharmacy, where she was recognized with the Community Service Scholarship. During this time, she volunteered in the pharmacogenomics lab of Dr. Rajeev Sachdeva at the Philadelphia Veterans Affairs Medical Center. After graduation, she completed a fellowship in Managed Markets and received her Master of Business Administration degree in Pharmaceutical and Healthcare Business from the Mayes College of Healthcare Business and Policy. Over the past 10 years at Genentech, she has worked on commercial teams focused on product management and access and, more recently, on the medical side of Genentech, focusing on clinical trials and scientific exchange with ophthalmologists.

Dr. Yankovskaya volunteers as a scholarship reviewer for the eQuality Scholarship Collaborative, is involved with the board of a local reproductive health nonprofit organization and is a fellow of the Leadership Philadelphia Core Program.

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Dr. Glenn Yiu is a Professor of Ophthalmology at UC Davis. He earned his dual MD/Ph.D. degrees at Harvard Medical School, residency training at the Massachusetts Eye & Ear Infirmary, and vitreoretinal fellowship at Duke. He joined UC Davis as a clinician-scientist and vitreoretinal surgeon in 2014, where he now leads a translational research program studying age-related macular degeneration (AMD) and other retinal diseases. His focuses include ocular imaging technologies, gene editing and delivery, and animal models of retinal disease. He reported the first use of CRISPR-based genome editing as a treatment strategy for wet AMD, discovered the use of microneedles for suprachoroidal gene delivery, and pioneered important studies on drusen evolution in rhesus monkeys. He currently serves as Director of the UC Davis Reading Center and Director of Tele-ophthalmology, where he has pioneered a teleretinal screening program to expand eye screening among diabetic patients in Northern California.

Dr. Yiu has published numerous peer-reviewed scientific articles and book chapters, and is the editor of the textbook “Vitreoretinal Disorders.” He also serves on the editorial board of Scientific Reports and Frontiers in Genome Editing, as a member of the ARVO Annual Meeting Program Committee, and as a course lecturer at the American Academy of Ophthalmology. He has received numerous awards, including the Ronald G. Michels Fellowship, the Heed Fellowship, the Retina Society Fellowship Research Award, and the Macula Society Evangelos S. Gragoudas Award. He is also supported by the National Eye Institute, the US Department of Agriculture, the Foundation Fighting Blindness, the BrightFocus Foundation, the Lions Club International Foundation, the Macula Society, and the University of California Office of the President. In 2016, he was named one of 21 “Emerging Vision Scientists” by the National Alliance for Eye and Vision Research for his cutting-edge research.

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Michael Young, Ph.D., Associate Scientist at the Schepens Eye Research Institute, received his BS degree in behavioral neuroscience from the University of Pittsburgh in 1989. He then received his Ph.D. in anatomy/neuroscience from the University of Cambridge in 1995. A postdoctoral fellowship at the Institute of Ophthalmology, University College, London, in 1995 was followed by a two-year postdoctoral fellowship at the Massachusetts Institute of Technology, Department of Brain and Cognitive Sciences. In 1998, Young joined the Schepens Eye Research Institute as an Investigator. He was elected the Director of the Minda deGunzburg Center for Ocular Regeneration. In 2008, he was promoted to Associate Professor in the Department of Ophthalmology at Harvard Medical School. He is now also Co-director of the Ocular Regenerative Medicine Institute at MEE/HM.

His laboratory is focused on retinal transplantation of stem cells for the treatment of diseases such as RP (retinitis pigmentosa) and AMD. He and his team isolate stem cells from the neurosensory retina, expand them in culture, and then graft them into the diseased eye, where they differentiate as photoreceptors and restore light-induced activity.

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Donald J. Zack, MD, Ph.D., is the Guerrieri Professor of Genetic Engineering and Molecular Ophthalmology and Director of the Center for Stem Cells and Ocular Regenerative Medicine (STORM) at the Wilmer Eye Institute, Johns Hopkins University School of Medicine.

His lab studies the control of gene expression in retinal ganglion cells, photoreceptor cells, and retinal pigment epithelial cells. He and his colleagues are also studying the mechanisms by which these cells die in glaucoma, retinitis pigmentosa, and age-related macular degeneration (AMD), and are developing novel methods to slow down, and hopefully prevent, their death. Additionally, they are developing methods to promote the differentiation of stem cells into retinal ganglion cells, photoreceptors, and RPE cells, in the hope that someday such cells might offer the possibility of restoring vision to glaucoma and retinal degeneration patients who have already lost significant vision due to loss of retinal cells.

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Marco Zarbin, MD, Ph.D. received a B.A. (biochemistry) from Dartmouth College (Phi Beta Kappa, summa cum laude, with highest distinction) in 1978, matriculated to the Johns Hopkins University School of Medicine in 1978, and entered the Medical Scientist Training Program in 1980. Dr. Zarbin graduated from the Johns Hopkins Medical School in 1984 (Alpha Omega Alpha). He completed an ophthalmology residency at the Wilmer Ophthalmological Institute (Johns Hopkins Hospital) from 1985-1988. Dr. Zarbin completed fellowships in vitreoretinal surgery and in medical retinal disease at the Wilmer Ophthalmological Institute and was an Assistant Chief of Service at Wilmer in 1989. Dr. Zarbin was appointed Chair of the Institute of Ophthalmology and Visual Science, New Jersey Medical School, Rutgers University, and Chief of Ophthalmology at University Hospital in Newark, N.J., in January 1994. He is the Alfonse A. Cinotti, MD/Lions Eye Research Professor of Ophthalmology.

Dr. Zarbin's research is focused on developing treatments for age-related macular degeneration (AMD), including cell transplantation. Dr. Zarbin also collaborates with Prof. Ellen Townes-Anderson in the development of sight-preserving pharmacotherapy for patients with retinal detachment. This work has been supported by the National Eye Institute, the Department of Veterans Affairs, the Department of Defense, the Foundation Fighting Blindness, Aerie Pharmaceuticals, and other foundations.

Dr. Zarbin has co-authored 288 peer-reviewed scientific publications, and he has co-edited one book on AMD, two books on cell-based therapy for retinal degenerative disease, and two books on the management of diabetic retinopathy.

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