USC Ginsburg Institute for Biomedical Therapeutics

USC Dr. Allen and Charlotte Ginsburg IBT
Presents The Inaugural USC Ginsburg Symposium

Cell Therapy for Retinal Disease

June 26, 2019, 8:30-1:15 pm
USC Health Sciences Campus
HCC4, 3rd floor Conference Room
1537 Norfolk Street, Los Angeles, CA 90033

Patients are our True Heroes

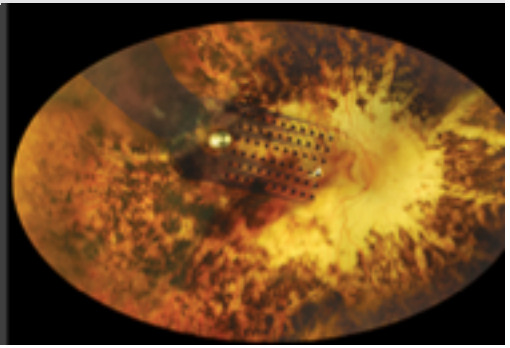
"I got my independence back, and I'm just so happy about it." -Anna Kuehl, recipient of the phase I/IIa clinical trial CPCB-RPE1 stem cell-based implant for advanced dry AMD.

MISSION

Through a multidisciplinary approach, our **mission** at **USC Ginsburg IBT** is to further our basic understanding of debilitating neurosensory disorders, ultimately leading to the development of novel diagnostic and treatment options.

VISION

At the USC Dr. Allen and Charlotte Ginsburg IBT our **vision** is to transform bioengineered neural interfaces into treatments for patients for whom there is no foreseeable cure.



Program

Session 1

8:30-9:00 am	Breakfast and Registration
9:00–9:10 am	Introduction and Welcome Mark Humayun, MD, PhD
9:10–9:30 am	Autologous Cell Therapy for Eye Disease Kapil Bharti, PhD
9:30–9:50 am	The London Project: Clinical outcomes following RPE sheet transplantation in AMD Lyndon da Cruz, MD, PhD
9:50–10:10 am	Optimizing Stem Cell-Derived RPE Cell Suspensions Steven Schwartz, MD, PhD
10:10–10:30 am	Engineering Material Scaffolds for Transplantation / hESC-RPE Patch for Atrophic Age-related Macular Degeneration Mark Humayun, MD, PhD / Amir Kashani, MD, PhD
10:30–10:50 am	Panel Discussion with Speakers from Session I Moderators: David Hinton, MD & Dennis Clegg, PhD

Session 2

11:20–11:40 am	Clinical Endpoints for Ophthalmological Cell Therapy Trials Jane Lebkowski, PhD
11:40–12:00 pm	Vision for the Future of Stem Cell Research in California Robert Klein
12:00–12:20 pm	Break

Lunch and Keynote Address

12:20–12:35 pm	Presentation of the Ginsburg Award for Translational Medicine by Dr. Humayun & Dr. Allen and Charlotte Ginsburg
12:35–12:50 pm	Derivation of Human Embyronic and Induced Pluripotent Stem cells James A. Thomson, VMD, PhD
12:50–1:05 pm	Interview with James A. Thomson, VMD, PhD Dr. Dennis Clegg
1:05pm	Meeting Adjourns

USC Ginsburg IBT

Mark S. Humayun, MD, PhD
Director, USC Ginsburg Institute for Biomedical Therapeutics

Dr. Humayun is an internationally recognized pioneer in vision restoration. He assembled a team of multidisciplinary experts to develop the first FDA approved artificial retina, Argus II, for sight restoration. He is a member of the U.S. National Academies of Medicine, Engineering, and Inventors. He has more than 100 patents/patent applications and more than 200 peer reviewed publications. For his extraordinary contributions he was awarded the United States' highest technological achievement, the 2015 National Medal of Technology and Innovation by President Obama. He is principal investigator for the California Project to Cure Blindness for the development of a bioengineered human embryonic stem cell (hESC)-derived retinal pigment epithelial (RPE) monolayer for advanced, dry age-related macular degeneration (AMD), which was used in phase I/IIa clinical trial.

Jamie Thomson, VMD, PhD
Ginsburg Award for Translational Medicine Recipient

The Ginsburg Vision Research Prize was established through a generous gift from Dr. Allen and Charlotte Ginsburg and is intended to reward one individual who has made, and continues to make, significant and transformative breakthroughs towards vision research.

Dr. Thomson's current research areas of focus are differentiation of vascular progenitors for transplantation therapies and mammalian developmental clocks. He is the director of Regenerative Biology at the Morgridge Institute for Research in Madison, Wisconsin, and professor of Cell & Regenerative Biology at the University of Wisconsin. He is also a professor in the Molecular, Cellular, and Developmental Biology Department at UC Santa Barbara. Dr. Thomson's research group reported the first derivation of embryonic stem cells from a non-human primate in 1995, work that led them to the first derivation of human embryonic stem cells in 1998 and derivation of the first human induced pluripotent stem (iPS) cell (simultaneously with Dr. Shinya Yamanaka's research group at Kyoto University in Kyoto, Japan) in 2007.

Speakers & Moderators

Kapil Bharti, PhD

Dr. Kapil Bharti obtained his PhD from Rao University, Baroda, India, graduating *summa cum laude*. His lab was recently awarded two prestigious grants: 1) the only NIH Intramural Common Fund grant to develop a phase I Investigational New Drug (IND) for Autologous induced pluripotent stem cell-derived retinal pigment epithelium tissue; and 2) a Department of Defense grant to develop a 3D-retina tissue to model retinal diseases *in vitro*. As head of the National Eye Institute Unit on Ocular and Stem Cell Translational Research Bharti focuses on understanding mechanism of retinal degenerative diseases using induced pluripotent stem cell technology, and developing cell and drug-based therapies.

Dennis Clegg, PhD

Dr. Clegg is the Wilcox Family Chair in Biomedicine Professor at UC Santa Barbara. His stem cell research focuses on developing therapies for ocular disease. Dr. Clegg is the recipient of the UCSB Distinguished Teaching Award, the National Eye Institute's Audacious Goals award, and served as UCSB Chair of the Department of Molecular, Cellular and Developmental Biology from 2004-2009. He is founder and Co-Director of the UCSB Center for Stem Cell Biology and Engineering. He was co-principal investigator for California Project to Cure Blindness (CPCB), a multi-disciplinary effort to develop a stem cell therapy for AMD.

Lyndon da Cruz, MD, PhD

Lyndon da Cruz began his research interest in retinal disease at the Nuffield Laboratory of Ophthalmology, Oxford. He completed a PhD at the Lions Eye Institute and a vitreo-retinal surgical fellowship in Perth, Australia. He was named the Menzies Scholar in Science and Medicine for Australia in 2000, the highest ranked postdoctoral fellow in Australia in medicine and science. He won the Howard Florey Post-doctoral Fellowship from the Royal Society to study with Prof. Alan Bird in London. As a Consultant Retinal Surgeon and Medical Retina Specialist at Moorfields Eye Hospital, his research interests focus on AMD and the artificial retina. He is also Clinical Lead of the The London Project and Head of the Artificial Retina Program at Moorfields Eye Hospital.

David R. Hinton, MD

Dr. Hinton, USC Professor of Pathology, Gavin S. Herbert Professorship in Vision Research; and Associate Dean for Vision Science, investigates the pathogenesis of blinding retinal diseases including AMD and proliferative vitreoretinopathy. As a world expert, his work has established the central role of the RPE cells in these disorders, and has demonstrated novel mechanisms for growth factor activation of the RPE with resulting alterations in migration, proliferation and gene expression. Dr. Hinton's lab also evaluates endogenous neuroprotectants and chaperones for their therapeutic potential. As co-principal investigator for the CPCB, he has also developed methods for differentiating RPE cells from hESC for their potential for cellular therapy in patients with AMD.

Amir H. Kashani, MD, PhD

Dr. Amir H. Kashani attended Johns Hopkins School of Medicine where he obtained his MD and PhD. He completed his ophthalmology residency at USC and fellowship in Vitreoretinal surgery at the top-rated Associated Retinal Consultants (Michigan). He was awarded both the national Heed and Michel's Fellowships during his training. Dr. Kashani is a USC clinician-scientist and vitreoretinal surgeon. His research focuses on advanced imaging spectroscopy and optical coherence tomography methods to improve the diagnosis and treatment of retinal diseases. Dr. Kashani is the principal investigator for a clinical trial to test a novel stem cell therapy for severe vision loss from advanced dry AMD.

Robert Klein

As a patient advocate, Robert Klein authored and chaired the campaign for California's Proposition 71, the $6 billion "California Stem Cell Research and Cures" general obligation bond and constitutional initiative. Mr. Klein served as the chairman of the Governing Board of the California Institute of Regenerative Medicine, the state funding entity established by Proposition 71. Mr. His recognitions include: 2005 *Time Magazine's* "one of the World's 100 Most Influential People of the Year"; one of 2005 Scientific American 50, as a leader shaping the future of science; and 2011 inaugural International Society for Stem Cell Research Public Service Award. He is working with a coalition of California leaders to explore continuing this state funding, returning to the ballot in 2020.

Jane Lebkowski, PhD

Dr. Lebkowski is an internationally recognized leader in the development of cell and gene-based therapies with direct management experience in the multidisciplinary functions required to translate research discoveries to therapeutic products. She is President of R&D at Regenerative Patch Technologies, a biotechnology firm developing composite stem cell-based implants such as CPCB-RPE1, targeting restoration of retinal architecture and function in patients with macular degeneration. She also served as Chief Scientific Officer and President of R&D at Asterias Biotherapeutics Inc, where she headed all preclinical, product, regulatory, and clinical development of regenerative medicine and dendritic cell based-cancer immunotherapy products.

Steven D. Schwartz, MD

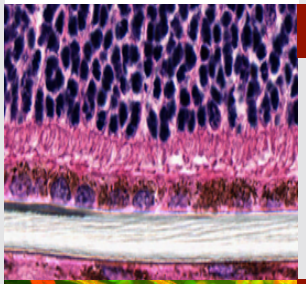
Dr. Schwartz is an Ahmanson Professor in Ophthalmology and Retina Division Chief at UCLA's Stein Eye Institute. An expert in translational research, he co-developed anti-vascular endothelial growth factor therapeutics, drug delivery systems, vitreoretinal diagnostic imaging technology, novel therapeutic lasers and surgical devices including a microsurgical robotic system. He has received numerous awards for scientific, clinical and humanitarian contributions including the American Academy of Ophthalmology's Secretariat Award for special and distinct contributions to the field of Ophthalmology, and the Morton Rubinstein UCLA Venice Family Free Clinic Outstanding Volunteer Physician of the Year Award.

About USC Ginsburg IBT

Our multidisciplinary approach involves collaboration across schools, institutes and industries. USC Ginsburg IBT continues to be at the forefront of scientific discovery in advancing the field of biomedical engineering in the treatment of neurosensory diseases. Our physicians and scientists work closely together, bringing clinical challenges to the bench, which spans the continuum of basic, translational and clinical research across all disciplines.

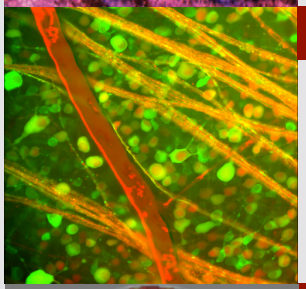
Neural Electronics

Converging both medicine and engineering, USC IBT experts create the world's first FDA approved bioelectronics retinal prosthesis device, known as the Argus II which brings functional sight back to those who have been blind for decades.



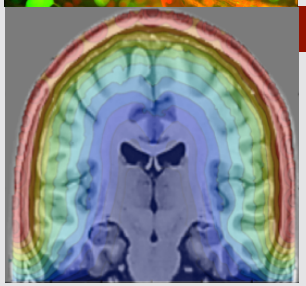
Neural Scaffolds

Through collaboration with world experts in the field, a subretinal implant containing polarized hESC-RPE on ultrathin parylene was developmd for the treatment of advanced dry AMD, which was recently used in a phase I/IIa clinical trial.



NeuroRx

The creations of neurophotonic systems, nano-photoswitches, capable of inducing light response in normally non-photosensitive neurons are being developed. The novel photoswitches have the potential to significantly expand the role of neuroprosthetics.



Neurophotonics

Novel drug delivery systems and bioelectronic implants that could deliver drugs across the blood-brain barrier in a highly selective manner using micro and nanoeletromechanical systems, known as MEMS or NEMS are being developed.

Dr. Allen & Charlotte Ginsburg

We are **grateful for Dr. Allen and Charlotte Ginsburg's philanthropy**, as it will enable our team to fulfill our mission: translating research that addresses the unmet medical needs of our patients. Along with this extraordinary gift of $10 million, the Ginsburgs have always given back to their community through numerous philanthropic initiatives.

"Now there is great hope with revolutionary breakthroughs made possible by Dr. Humayun and his colleagues. It's a distinct and sincere pleasure to be involved in this exciting research that holds promise to restore the precious gift of eyesight."-Dr. Ginsburg

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