# **CONTINUING MEDICAL EDUCATION**



Johns Hopkins University School of Medicine Presents

# Johns Hopkins **Retinal Degeneration** and Visual Electrophysiology Conference 2023

# September 15-16, 2023

Johns Hopkins School of Medicine Thomas B. Turner Building Baltimore, Maryland





This activity has been approved for AMA PRA Category 1 Credits™.

#DEGEN2023 #diverseDeGen23 #ophthalmology #optometry #IHopthth #WilmerRDC #WilmerIHDEGEN2023 #WomeninScience #WomeninSTEM #DiversityinSTEM #ScienceInclusion #BlackinScience #BlackinSTEM #LatinxinScience #LatinxinSTEM #MedTwitter #OphthoTwitter #Ophthalmology #Retina

#### DESCRIPTION

This two-day conference is designed to provide an update on the latest exciting and cutting-edge developments in the field of retinal degeneration, repair, and regeneration, including recent advances in stem cells and gene therapy, cellular and molecular mechanisms of disease, outcome measures, natural history, nanomedicine, systemic manifestations, surgery and delivery. ethics and global perspectives. The program includes a refresher course on visual electrophysiology for clinicians and researchers.

#### WHO SHOULD ATTEND

This activity is intended for ophthalmologists, optometrists, researchers, trainees, fellows, technicians, industry, and professionals of all levels.

# OBIECTIVES

After attending this activity, the learner will demonstrate the ability to:

- · Recall cellular and molecular basis of selected IRDs, with an emphasis on mechanistic insights that inform treatment strategies and outcome measures.
- · Review recommendations from the International Society of Clinical Electrophysiology of Vision (ISCEV) regarding visual neurophysiology testing.
- Review structural and functional outcome measures of IRDs, including those relevant to subjects with ultra-low vision.
- · Review data and results from Phase I, II, and III clinical trials, and translational studies, with an emphasis on stem cells, genetherapy, and other treatment approaches.
- Review aspects relating to equitable access to clinical care and research for IRD patients, and ethical considerations unique to people with vision loss due to IRDs.

#### **ACCREDITATION STATEMENT**

The Johns Hopkins University School of Medicine is accredited by the Accreditation Council for Continuing ACCME Medical Education (ACCME) to provide continuing medical CCREDITED WITH education for physicians.



The Johns Hopkins University School of Medicine designates this live activity for a maximum of 12.50 AMA PRA Category 1 Credits TM. Physicians should claim only the credit commensurate with the extent of their participation in the activity.

# **OTHER CREDIT**

American Academy of Nurse Practitioners National Certification Program accepts AMA PRA Category 1 Credit<sup>™</sup> from organizations accredited by the ACCME.

American Nurses Credentialing Center (ANCC) accepts AMA PRA Category 1 Credit<sup>™</sup> from organizations accredited by the ACCME.

PAs may claim a maximum of 12.50 Category I credits for completing this activity. NCCPA accepts AMA PRA Category 1 Credit<sup>™</sup> from organizations accredited by ACCME or a recognized state medical society.

The Johns Hopkins University has approved this activity for 12.50 contact hours for non-physicians.

### POLICY ON PRESENTER AND PROVIDER DISCLOSURE

It is the policy of the Johns Hopkins School of Medicine that the presenter and provider globally disclose conflicts of interest. The Johns Hopkins School of Medicine OCME has established policies in place to identify and mitigate relevant conflicts of interest prior to this educational activity. Detailed disclosure will be made prior to presentation of the education.

# TRAVEL GRANTS

A limited number of travel awards are available for early bird registrants! Travel awards are available for young investigators (within 10 years of terminal degree), trainees (students, residents, post-doctoral fellows, research or clinical fellows), women and underrepresented minorities (Blacks or African Americans, Hispanics or Latinos, American Indians or Alaska Natives, Native Hawaiians, and other Pacific Islanders).

To apply for a travel award, please send a 2-page CV and brief (250 words max.), personal statement regarding their interest in inherited retinal diseases research, and/or clinical care to Minda McNally (irdtravelgrants@lists.jh.edu) by August 15,2023.

# **General Information**

REGISTRATION September 15 - 16, 2023 | 7:00 - 8:00 a.m.

#### LOCATION

Thomas B. Turner Building, Johns Hopkins School of Medicine 720 Rutland Avenue, Ground Floor Baltimore, Maryland 21205

The Turner Building is located on Rutland Avenue at Monument Street. Directions and campus parking information are available on our website under the Contact Us tab at https://HopkinsCME.cloud-cme.com. Handicapped parking is available in the nearby Rutland Garage. Johns Hopkins is smoke-free.

#### HOTEL INFORMATION

Residence Inn by Marriott Baltimore at the Johns Hopkins Medical Campus 800 North Wolfe Street Baltimore, Maryland 21205 (443) 524-8400 | Website: www.marriott.com

# HOTEL RESERVATION CUT-OFF DATE: AUGUST 15, 2023

The Residence Inn Baltimore is located on the Johns Hopkins Medical Campus. Make your reservation at www.marriott.com or call the hotel directly and specify that you are attending the Johns Hopkins Retinal Degeneration conference to receive the special group rate of \$139 for one bedroom king suite, plus tax. On-site parking is available at the hotel at an additional charge. Check-in time is 4:00 p.m. Check-out time is 12:00 p.m.

#### FEES

Register Online: hopkinscme.cloud-cme.com/default.aspx?P=5&EID=45880

Methods of Payment: Learners must register and submit full payment prior to the start of the activity. On-site payments can only be accepted if made by learners via credit card through the online portal by 5:00 p.m. ET on the first day of the activity. The registration fee includes instructional materials, continental breakfast, refreshment breaks and lunches.

Registration Fees:	Early Bird Rates Until 6/30/2023	Regular Rates After 6/30/2023
Physician/Scientist/Optometrist/ Industry	\$585	\$685
Allied Health Professional (RN/NP/PA)	\$485	\$585
Trainees (Student/Resident/Fellow/Post-Doc)	\$75	\$100

possible.

A \$50 late fee applies to registrations received after 5:00 p.m. ET on September 9, 2023. A handling fee of \$50 will be deducted for cancellation. An additional fee may apply for cancellation of other events, including workshops and social activities. Refund requests must be received by fax or mail by September 9, 2023. No refunds will be made thereafter. Transfer of registration to another Johns Hopkins activity in lieu of cancellation is not possible.

# **SYLLABUS**

# HOW TO OBTAIN CREDIT

Post activity, an online evaluation will be available to attendees to evaluate the activity and individual presentations and to identify future educational needs. Upon completion of the evaluation, the learner must attest to the number of hours in attendance. Credits earned will be added to the learner's transcript and immediately available for print. The last day to access the evaluation and attest to your credits is October 31, 2023.

# **AMERICANS WITH DISABILITIES ACT**

The Johns Hopkins School of Medicine fully complies with the legal requirements of the ADA and the rules and regulations there of. Please notify us if you have any special needs.

You will receive a confirmation by e-mail. If you have not received it by September 9, 2023, call (410) 502-9636 to confirm that you are registered. A transcript of attendance will be available upon attestation of your credit hours and submission of the post activity online evaluation.

The Johns Hopkins University reserves the right to cancel or postpone any activity due to unforeseen circumstances. In this event, the University will refund the registration fee but is not responsible for travel expenses. Additionally, we reserve the right to change the venue to a comparable venue. Under such circumstances registrants will be notified as soon as

# LATE FEE AND REFUND POLICY

The syllabus will be accessible online and via your mobile device in the CloudCME App prior to the activity.

An outcome survey will be sent to all physician attendees within two months post activity to assist us in determining what impact this activity had on the learner's practice.



# Program

#### DAY I • September 15, 2023 Gen Registration, Breakfast and Exhibits 8:00 8:30 1:30 8:30 8:35 Welcome, Introduction and Conference Goals . Fernando Arevalo, MD, PhD; Mandeep Singh, MD, PhD 1:45 **Opening Remarks** 8:35 8:40 Peter McDonnell, MD Visual Neurophysiology 2:00 Multifocal Electroretinogram and Visual Evoked 8:40 8:55 Potential 2:15 Mary Johnson, PhD Visual Fields and Microperimetry 8:55 9:10 2:30 Gislin Dagnelie, PhD, MS 9:25 Retinal Light and Dark Adaptation 2:45 9:10 Omar Mahroo, PhD, MBBCh 9:25 9:40 Pediatric Electroretinogram Assessment lefferson Doyle, MD, MBBCh, PhD, MHS 3:00 9:40 9:55 **ISCEV Standard for Full-field Clinical** 3:15 Electroretinography Omar Mahroo, MBBCh, PhD Equi Genetic Testing in IRDs 9:55 10:10 Christy Smith, ScM, COC 1:30 10:10 10:40 Refreshment Break and Exhibits 1:45 Cellular and Molecular Mechanisms General Principles and Practice of Clinical 2:00 10:40 10:55 Genetic Testing Robert Hufnagel, MD, PhD 2:15 Role of ABCA4 in Photoreceptors/ RPE 10:55 11:10 Mitra Farnoodian, PhD Gyrate Atrophy of the Choroid and Retina 2:30 ||:10 |1:25 David Valle, MD 11:25 11:40 Understanding Autophagy and Cell Stress 2:45 lames Handa, MD 11:40 11:55 **Understanding Mitochondrial Diseases in the Eye** Hilary Vernon, MD, PhD Super Enhancer-promoter Interactions 11:55 12:10 Anand Swaroop, PhD

<ul> <li>Novel Approaches in Viral Gene Therapy Seth Blackshaw, PhD</li> <li>Subretinal Gene Therapy Targeting Cone Photoreceptors in Achromatopsia Dominik Fischer, MD, PhD</li> <li>2:15 Base Editing Gregory Newby, PhD</li> <li>2:30 Mutation-independent Treatment Using RdCVF Daniel Chung, DO, MA</li> <li>2:45 Gene Therapy for LCAS Ash Jayagopal, PhD</li> <li>3:00 Gene Therapy for X-linked Retinitis Pigmentosa Jasmina Kapetanovic, MA, MBBCh, Oxon, MRCOphth,MSc, PhD, FRCOphth</li> <li>3:15 IRD Clinical Trials Successes and Failures David Birch, PhD</li> <li>3:30 Refreshment Break and Exhibits</li> <li>http:// Ethics, Global Perspectives</li> <li>1:45 Insights from Genetic Studies in Africa Ambroise Wonkam, MD, PhD</li> <li>2:00 Overview of IRDs Characterization in Brazil Juliana Sallum, MD, PhD</li> <li>2:15 The Fight Blindness Registry Matthew Simunovic, MBBCh, PhD, FRANZCO</li> <li>2:30 Genetic Landscape of Retinitis Pigmentosa in a Larg Korean Cohort Joo Yong Lee, MD</li> <li>2:45 Racial Disparities in Eye Care Adrienne Scott, MD</li> <li>3:00 Mental Health Care in IRDs Annabelle Pan, BS</li> </ul>			
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		3:00	

# DAY 2 • September 16, 2023

8:00	8:30	Registration, Breakfast and Exhibits	Nanon
Syster	nic Mani	ifestations	12:45
8:30	8:45	Allelic Hierarchy in USH2A Robert Hufnagel, MD, PhD	1:00
8:45	9:00	<b>Usher Syndrome</b> Wadih Zein, MD	1:15
9:00	9:15	Retinal Manifestations of Mitochondrial Oxidative Phosphorylation Disorders	1:30
9:15	9:30	Stephen Tsang, MD, PhD <b>Natural History Study of SCA7</b> Laryssa Huryn, MD	1:45
9:30	10:00	Refreshment Break and Exhibits	Cell T 2:00
Outco	me Mea	sures and Natural History	2:15
10:00	10:15	Update on FFB Natural History Studies	2:15
10:15	10:30	Rachel Huckfeldt, MD, PhD Outcomes Measures in Patients with Low Vision Lauren Ayton, PhD	2:30
10:30	10:45	Functional Outcome Measures for Clinical Trials of Stargardt Disease: Insights from a Prospective Natural History Study of Visual Dysfunction in ABCA4-retinopathy Brett Jeffrey, PhD	2:45 <b>Surger</b> 3:00
10:45	11:00	Genetic Neuro-ophthalmic Conditions Andrew Carey, MD	3:15
11:00	11:15	Challenges and Some Remedies in Evaluating New Treatments for IRDs – A Biostatistician's Perspective	3:30
11:15	11:30	Maureen Maguire, PhD <b>Natural History of Bardet-Beidl Syndrome</b> Ajoy Vincent, MBBS, MS	3:45
11:30	11:45	Adaptive Optics Imaging in Choroideremia Johnny Tam, PhD	4:00
11:45	12:45	Lunch and Exhibits	4:15

You will receive an email notification to complete the evaluation form and to attest to the number of hours in attendance. The schedule is subject to change. The registration desk will remain open during conference hours. The Johns Hopkins School of Medicine takes responsibility for the content, quality and scientific integrity of this CME activity.

# ACTIVITY DIRECTORS

Lunch and Exhibits

12:10

1:30

J. Fernando Arevalo, MD, PhD Professor of Ophthalmology Wilmer Eye Institute Johns Hopkins School of Medicine

Mandeep Singh, MD, PhD Associate Professor of Ophthalmology Wilmer Eye Institute Johns Hopkins School of Medicine

# ACKNOWLEDGEMENT

Applications for commercial support from health care related industries are pending. A complete list of contributors will be provided in the syllabus. Please note that commercial support received is solely for the educational component of the activity and will not be used to provide food and beverage.

Please note: The Physician Payments Sunshine Act was enacted by Congress to increase public awareness of financial relationships between drug and medical device manufacturers and physicians. In compliance with the requirements of this Act, the commercial supporter/s of this activity may require the Johns Hopkins School of Medicine to report certain professional information (such as name, address, National Provider Identifier (NPI), and State License number) of physician attendees who receive complimentary food and beverage in conjunction with a CME activity. The commercial supporter is required to submit the collected data to the Centers for Medicare and Medicaid Services which will then publish the data on its website.

# medicine and Pharmacotherapy

1:00	Nanomedicine Approaches to Bypassing the Mucus Barrier for Improved Local Drug Delivery
	Laura Ensign, PhD
1:15	Therapeutic Applications of Dendrimers
	Kannan Ramaranganujam, PhD, MS
I:30	<b>Metformin for Štargardt Disease</b> Brian Brooks, MD, PhD
l:45	Updates on NAC Treatment for Retinitis Pigmentosa Peter Campochiaro, MD
2:00	Updates on the ALK-001 for Stargardt Disease Hendrik Scholl, MD, MA

# Therapy and Retinal Organoids

2:15	Understanding Cellular Material Transfer
	Mandeep Singh, MD, PhD
2:30	Autologous iPSC-RPE Transplantation for AMD
	Kapil Bharti, PhD
2:45	Cell Therapy Approaches for Glaucoma

- Thomas Johnson, MD, PhD
- 3:00 Refreshment Break and Exhibits

# ery and Delivery

3:15	OCT Imaging for Retinal Surgery Applications
	Jin Kang, PhD

- Surgical Insights from Retinal Gene Therapy Trials 3:30 Ninel Gregori, MD
- Standard-care Surgical Procedures in IRDs 3:45 Bani Antonio, MD, MPH
- 4:00 Immune Responses in AAV Gene Therapy lan Han, MD
- Gene and Cell Therapy Testing in Nonhuman Primates 4:15 Ala Moshiri, MD, PhD Adjourn

# TO REGISTER OR FOR FURTHER INFORMATION

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# **Speakers**

Bani Antonio, MD, MPH Postdoctoral Research Felllow Wilmer Eye Institute Johns Hopkins School of Medicine

Lauren Ayton, PhD Associate Professor Department of Optometry and Vision Sciences at the University of Melbourne Centre for Eye Research Australia Royal Victorian Eye and Ear Hospital

Kapil Bharti, PhD Senior Investigator Ocular and Stem Cell Translational Research Section at the National Eye Institute National Institutes of Health

David Birch, PhD Scientific Director Retina Foundation of the Southwest

Seth Blackshaw, PhD Professor of Neuroscience Johns Hopkins School of Medicine

Brian Brooks, MD, PhD Director The Pediatric, Developmental and Genetic Ophthalmology Section at the National Eye Institute National Institutes of Health

Peter Campochiaro, MD Director, Retinal Cell and Molecular Laboratory Professor of Ophthalmology Wilmer Eve Institute Johns Hopkins School of Medicine

Andrew Carey, MD Assistant Professor of Ophthalmology Wilmer Eye Institute Johns Hopkins School of Medicine

Daniel Chung, DO, MA Chief Medical Officer SparingVision

Gislin Dagnelie, PhD, MS Professor of Ophthalmology Wilmer Eye Institute Johns Hopkins School of Medicine

Jefferson Doyle, MD, MBBCh, PhD, MHS Assistant Professor of Ophthalmology Wilmer Eye Institute Johns Hopkins School of Medicine

Laura Ensign, PhD Professor of Ophthalmology Center for Nanomedicine Wilmer Eve Institute Johns Hopkins School of Medicine Mitra Farnoodian, PhD Scientist National Eve Institute National Institutes of Health

Dominik Fischer, MD, PhD Consultant Ophthalmologist Oxford Eye Hospital Professor of Ophthalmology University of Oxford

Ninel Gregori, MD Professor of Clinical Ophthalmology Bascom Palmer Eve Institute University of Miami Leonard M. Miller School of Medicine

Ian Han, MD Chief, Vitreoretinal Service Associate Professor of Ophthalmology and Visual Sciences Carver College of Medicine University of Iowa

James Handa, MD Professor of Ophthalmology Wilmer Eve Institute Johns Hopkins School of Medicine

Rachel Huckfeldt, MD, PhD Assistant Professor of Ophthalmology Massachusetts Eye and Ear Harvard Medical School

Robert Hufnagel, MD, PhD Director of Ophthalmic Genomics Laboratory National Eve Institute National Institutes of Health

Laryssa Huryn, MD **Ophthalmic Genetics Specialist** National Eye Institute National Institutes of Health

Ash Javagopal, PhD Assistant Professor of Ophthalmology Vanderbilt University

Brett Jeffrey, PhD Head Human Visual Function Core at the National Eve Institute National Institutes of Health

Mary Johnson, PhD Associate Professor Ophthalmology and Visual Sciences University of Maryland School of Medicine

Thomas Johnson, MD, PhD Shelley and Allan Holt Rising Professor of Ophthalmology Wilmer Eve Institute Johns Hopkins School of Medicine Jin Kang, PhD Jacob Suter Jammer Professor of Electrical and Computer Engineering Whiting School of Engineering Johns Hopkins School of Medicine

Jasmina Kapetanovic, MA, MBBCh, Oxon, MRCOphth, MSc. PhD. FRCOphth MRC Clinician Scientist and Consultant Vitreo-Retinal Surgeon University of Oxford

Joo Yong Lee, MD Professor of Ophthalmology Asan Medical Center University of Ulsan College of Medicine

Maureen Maguire, PhD Professor CE of Ophthalmology Vice Chair for Clinical Research Department of Ophthalmology University of Pennsylvania, School of Medicine

Omar Mahroo, PhD, MBBCh Professor of Retinal Neuroscience University College London Institute of Ophthalmology

Ala Moshiri, MD, PhD Associate Professor of Ophthalmology UC Davis Eye Center Ernest E. Tschannen Eye Institute

Gregory Newby, PhD Assistant Professor of Genetic Medicine Johns Hopkins School of Medicine

Annabelle Pan, BS Johns Hopkins School of Medicine

Kannan Ramaranganujam, PhD, MS Co-Director, Center for Nanomedicine Professor of Ophthalmology Wilmer Eye Institute Johns Hopkins School of Medicine

Juliana Sallum, MD, PhD Professor of Ophthalmology Universidade Federal de São Paulo

Hendrik Scholl, MD, MA Founding and Scientific Director Institute of Molecular and Clinical Ophthalmology Basel (IOB) Professor and Chairman Department of Ophthalmology University of Basel

Adrienne Scott, MD Chief, Wilmer Eye Institute - Bel Air Associate Professor of Ophthalmology Wilmer Eye Institute Johns Hopkins School of Medicine

Matthew Simunovic, MBBCh, PhD, FRANZCO Professor of Ophthalmology and Visual Science Save Sight Institute University of Sydney

Christy Smith, ScM, CGC Scientist Department of Genetic Medicine Johns Hopkins Medicine

Anand Swaroop, PhD Senior investigator National Eye Institute National Institutes of Health

Johnny Tam, PhD Stadtman Investigator National Eye Institute National Institutes of Health

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