Saturday
April 28, 2018

Education Courses
Separate registration fee required
### Saturday, April 28 – Education courses (education courses require separate registration)

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Title</th>
<th>Location</th>
</tr>
</thead>
<tbody>
<tr>
<td>8am – 4:30pm</td>
<td>001</td>
<td>Big Data: Principles to practical application</td>
<td>313A</td>
</tr>
<tr>
<td></td>
<td>002</td>
<td>Gene Editing Using CRISPR/Cas Technology: From Discovery to Therapy</td>
<td>316B</td>
</tr>
<tr>
<td></td>
<td>003</td>
<td>Inherited retinal diseases: Divergent viewpoints of pathogenesis and treatment</td>
<td>316A</td>
</tr>
<tr>
<td>8am– 12:30pm</td>
<td>004</td>
<td>Introduction to AMD: Current research and therapeutics</td>
<td>316C</td>
</tr>
</tbody>
</table>
Room 313A
Saturday, April 28, 2018 8:00 AM–4:30 PM

001 Big Data: Principles to Practical Application

“Big Data” is a buzzword in healthcare today. The use of Big Data for improving healthcare outcomes and controlling costs promises significant promise. This course will help participants define what Big Data is, describe the Big Data sets available in vision research, explain the analytic methods behind Big Data, and summarize the potential applications of Big Data. This activity’s overall purpose is to ensure change in learner competence by uncovering associations, patterns and trends with the data, in order to improve professional practice.

— 8:00 Welcome and introduction. Anne L. Coleman, Jules Stein Eye Institute, University of California-Los Angeles, Santa Monica, CA

— 8:15 Tapping into Health Care Claims Databases to Learn About Ocular Diseases. Joshua D. Stein1, 2. 1Kellogg Eye Center/Ophthalmology, University of Michigan, Ann Arbor, MI; 2Health Management & Policy, University of Michigan School of Public Health, Ann Arbor, MI

— 8:35 Case Study: IRIS Registry. Anne L. Coleman, Jules Stein Eye Institute, University of California-Los Angeles, Santa Monica, CA

— 8:55 Big Data Studies: EHRs. Michael F. Chiang, Ophthalmology and Medical Informatics, Oregon Health & Science University, Portland, OR


— 9:35 Break

— 10:00 Visualizing Big Data. Aaron Y. Lee. Ophthalmology, UW Medicine, Seattle, WA


— 11:00 Panel Discussion

— 12:00 Lunch Break

— 1:00 Practical Considerations for Performing Analyses Using Health Care Claims Data. Joshua D. Stein1, 2. 1Kellogg Eye Center/Ophthalmology, University of Michigan, Ann Arbor, MI; 2Health Management & Policy, University of Michigan School of Public Health, Ann Arbor, MI

— 1:25 Practical Considerations of Working with Registries. Anne L. Coleman, Jules Stein Eye Institute, University of California-Los Angeles, Santa Monica, CA

— 1:50 Practical Considerations of Doing Big Data Studies with EHRs. Michael F. Chiang, Ophthalmology and Medical Informatics, Oregon Health & Science University, Portland, OR


— 2:40 Break


— 3:45 Panel Discussion, Next Steps, and Closing Remarks

Room 316B
Saturday, April 28, 2018 8:00 AM–4:30 PM

002 Gene Editing Using CRISPR/Cas Technology: From Discovery to Therapy

In the current era of personalized medicine, a large number of genetic variants in patients with various diseases using next generation sequencing have been identified. Recent advances in genetic engineering, genotyping, high-resolution imaging and biomarker testing have made it easier to deliver the right treatments to the right patients at the right time. This course presents an overview of CRISPR technology from the leading experts who have pioneered it in other disciplines, followed by examples in eye and vision science and practical applications. This course is designed to enhance learner competence in the area of CRISPR technology in order to appropriately apply in the learner’s professional practice.

— 8:00 What is Gene Editing? What Techniques are Available? What are the advantages and limitations of ZFN, TALEN, CRISPRn, CRISPRi, and CRISPRx? Stephen H. Tsang1, 2. 1Columbia Coll Phys Surg, Columbia Univ-Harkness Eye Inst, New York, NY; 2NIH, Rockville, MD

— 8:20 Designing CRISPR experiments: Tips and potential pitfalls to look out for/avoid in gRNA design process. Stephen H. Tsang1, 2. 1Columbia Coll Phys Surg, Columbia Univ-Harkness Eye Inst, New York, NY; 2NIH, Rockville, MD

— 8:40 GUIDE-Seq vs High-throughput genomic translocation sequencing vs Digenome-seq vs BLESS. Christine L. Xu. Columbia Medical Research Center, New York, NY


— 10:00 Break

— 10:15 Challenges of CRISPR gene editing in genetic eye disease. Tara C. Moore1, 2. 1Avellino Labs, San Francisco, CA; 2Biomedical Sciences Research Institute, University of Ulster, Coleraine, Northern Ireland, United Kingdom

— 10:45 CjCas9-mediated genome editing for macular degenerations. Taeyoung Koo1, 2. 1Center for Genome Engineering, Institute for Basic Science, Yuseong-gu, Korea (the Republic of); 2Basic Science , University of Science and Technology Daejeon, Korea (the Republic of)

— 11:15 High Throughput CRISPR Screening for Ophthalmology. Alex W. Hewitt1, 2. 1Department of Ophthalmology, Centre for Eye Research Australia, Sandy Bay, Tasmania, Australia; 2Menzies Institute for Medical Research, University of Tasmania, Hobart, Tasmania, Australia

— 11:45 Morning wrap-up

— 12:00 Lunch Break

— 1:00 Panel: Safety and efficacy of CRISPR in patients. Alex W. Hewitt1, 2. 1Department of Ophthalmology, Centre for Eye Research Australia, Sandy Bay, Tasmania, Australia; 2Menzies Institute for Medical Research, University of Tasmania, Hobart, Tasmania, Australia


— 1:40 Panel Discussion - Safety and efficacy of CRISPR moving into patients. Peter Bacia. Ocular Disease, Editas Medicine, Cambridge, MA

— 2:00 Designing optimal CRISPR gRNAs. Amy Guan. Benchling, San Francisco, CA

— 2:45 HDR ssODN and crRNA design for high efficiency and low OTEs. Mark Behlke. Integrated DNA Technologies, Inc., Coralville, IA

— 3:30 CRISPR design, experimental analysis and cell engineering services. Kevin Holden. Synthego, Redwood City, CA


*CR refers to the Program Number in the Commercial Relationships (CR) Index for Disclosures.
Inherited retinal diseases are a group of eye disorders caused by an inherited gene mutation and can cause vision loss or blindness. The primary goal of this course is to discuss opposing viewpoints related to the various treatment strategies for inherited retinal diseases. Further, learners will be able to identify and debate different clinical and research topics in the area of inherited retinal diseases. Emphasized will be perspectives on the use of stem cells and gene-directed therapy. In addition, the primary mechanism of retinal degeneration in patients with Stargardt disease will be discussed. These discussions highlight current obstacles clinicianscientists are facing in their fight against retinal degeneration. The overall goal of this course is to enhance learner competence in the area of retinal degeneration to utilize in professional practice.

— 8:00 Opening Remarks
— 8:15 Assessing the potential value of human pluripotent stem cell treatments for retinal degenerative diseases. David M. Gamm1, 2, 3, 4, 5. 1Ophthalmology and Visual Sciences, Univ of Wisconsin-Madison, Madison, WI; 2McPherson Eye Research Institute, University of Wisconsin-Madison, Madison, WI *CR
— 8:30 The uncertainties of stem cell treatment. Kapil Bharti. National Eye Institute, Bethesda, MD
— 8:45 Audience questions and discussion
— 9:05 The Potential Value of Gene Editing. Eric A. Pierce. Ocular Genomics Institute, Massachusetts Eye and Ear, Belmont, MA *CR
— 9:20 Optimizing safety and efficacy in gene editing therapies for Inherited retinal diseases. John G. Flannery1, 2, 3. 1Helen Wills Neuroscience Institute, University of California, Berkeley, Berkeley, CA; 2School of Optometry, University of California, Berkeley, Berkeley, CA
— 9:35 Audience questions and discussion
— 9:55 Break
— 10:10 CAIs should be the initial approach for treating CME in inherited retinal diseases. Mark E. Pennesi. Ophthalmology, Casey Eye Institute - OHSU, Portland, OR
— 10:25 Steroids, NSAIDS, or the use of anti-VEGF, are likely equally effective as CAIs for the treatment of CME in inherited retinal diseases. Alessandro Iannaccone. Duke Eye Center, Duke University Medical Center, Durham, NC
— 10:40 Audience questions and discussion
— 11:00 Genetic screening should be routinely implemented in the clinical evaluation of patients with inherited retinal diseases. Stephen P. Daiger. Human Genetics Center, School Pub Hlth, Univ Texas Hlth Sci Ctr Houston, Houston, TX
— 11:15 Genetic screening SHOULD NOT be routinely implemented in the clinical evaluation of patients with inherited retinal diseases. Byron L. Lam. Bascom Palmer Eye Institute, University of Miami Health System, Pinecrest, FL *CR
— 11:30 Audience questions and discussion
— 11:50 Lunch Break
— 1:00 Retinal autoimmunity can contribute to photoreceptor cell loss in retinitis pigmentosa and possibly other inherited retinal diseases. Grazyna Adamus. Ophthal-Casey Eye Inst, University of Oregon Health Sciences University, Portland, OR
— 1:15 Retinal autoimmunity is unlikely to contribute to photoreceptor cell loss in retinitis pigmentosa or other inherited retinal diseases. Robert K. Koenekoop. McGill Ocular Genetics Laboratory, McGill University Health Centre, Montreal, Quebec, Canada
— 1:30 Audience questions and discussion
— 1:50 Bisretinoids are the primary culprit in Stargardt disease. Janet R. Sparrow. Department of Ophthalmology, Columbia University, New York, NY
— 2:05 Bisretinoids restrict all-trans-retinal as a primary contributor to Stargardt disease. Krysztof Palczewski. Pharmacology School of Med, Case Western Reserve Univ, Cleveland, OH *CR
— 2:20 Audience questions and discussion
— 2:40 Break
— 2:55 Role of Abca4 in Photoreceptor Outer Segments. Gabriel H. Travis. Stein Eye Institute , UCLA School of Medicine, Los Angeles, CA
— 3:10 RPE is the primary site for the retinal degeneration in patients with Stargardt disease. Roxana A. Radu. Ophthalmology, Stein Eye Institute /UCLA, Los Angeles, CA
— 3:25 Audience questions and discussion
— 3:45 How Foundations can enhance clinical trial enrollment and advance Patients as Partners. Stephen M. Rose. Science Dept, Foundation Fighting Blindness, Columbia, MD
— 4:00 Doctors should ultimately be responsible for patient selection and participation in various treatment trials. Samuel G. Jacobson. Center for Hereditary Retinal Degenerations, Scheie Eye Institute, Philadelphia, PA
— 4:15 Summary and wrap-up
004 Introduction to AMD: Current research and therapeutics

Age-related macular degeneration (AMD) is one of the most common eye conditions leading to vision loss among people age 50 and older. In fact, the risk of acquiring advanced age-related macular degeneration increases from 2% for those ages 50-59, to nearly 30% for those over the age of 75. This course will examine key clinical and pathological findings in AMD. New insights into the genetics of AMD will be highlighted, including an in-depth discussion regarding the discovery of key biochemical pathways involved in the disease. Potential new therapies that could interrupt these pathways will also be explored. This course’s overall aim is to enhance learner competence in the area of AMD to utilize in professional practice.

— 8:00 Welcome and introduction. Debasish Sinha. Ophthalmology, Johns Hopkins Wilmer Eye Inst, Baltimore, MD


— 8:35 Questions and discussion

— 8:45 AMD – clinical imaging. Identifying disease, progression and therapeutic endpoints. Cynthia A. Toth1, 2. Ophthalmology, Duke Univ Eye Center, Durham, NC; 2Biomedical Engineering, Duke University, Durham, NC *CR, *

— 9:10 Questions and discussion

— 9:20 AMD – The translational value of preclinical animal models. Catherine Bowes Rickman. Ophthalmal & Cell Biology, Duke University Medical Center, Durham, NC

— 9:45 Questions and discussion

— 9:55 Break

— 10:10 AMD – from the immunologist perspective. Andrew D. Dick1, 2. 1Ophthalmology, Univ of Bristol-Bristol Eye Hosp, Bristol, United Kingdom; 2UCL-Institute of Ophthalmology, London, United Kingdom *CR

— 10:35 Questions and discussion

— 10:45 AMD—Identifying New and Relevant Pathways at Different Disease Stages using Systems Biology. Jiang Qian. Ophthalmology, Johns Hopkins School of Medicine, Baltimore, MD

— 11:10 Questions and discussion

*CR Refer to the Program Number in the Commercial Relationships (CR) Index for Disclosures.