# STEM CELLS AND ORGANOID AS MODELS OF TISSUE DIFFERENTIATION AND EYE DISEASES

Organizer: Lisa A. Neuhold, PhD

## AGENDA

<table>
<thead>
<tr>
<th>Time</th>
<th>Topic</th>
<th>Faculty</th>
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<tr>
<td>8:30-8:45am</td>
<td>Stem Cell Therapy and Organoids as Potential Therapeutic Intervention</td>
<td>Paul A Sieving, MD, PhD, FARVO National Eye Institute National Institutes of Health</td>
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<tr>
<td>8:45-9:15am</td>
<td>Introduction</td>
<td>Thomas A. Reh, PhD University of Washington School of Medicine</td>
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<td>9:15-9:25am</td>
<td>Questions</td>
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<td>9:25-9:50am</td>
<td>Current Advances in Stem Cell Therapies and 3D Culture Systems: Anterior Segment</td>
<td>Scheffer C.G. Tseng, MD, PhD, FARVO Ocular Surface Center Ocular Surface Research &amp; Education Foundation</td>
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<tr>
<td>9:50-10:15am</td>
<td>Lens: regeneration of lens from endogenous stem cells</td>
<td>Kang Zhang, MD, PhD Shiley Eye Institute University of California San Diego</td>
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<td>10:15-10:25am</td>
<td>Questions</td>
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<td>10:25-10:45am</td>
<td>Break</td>
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<tr>
<td>10:45-11:10am</td>
<td>Retinal Ganglion Cells: Differentiation and integration of RGCs into adult retinas</td>
<td>Jeffrey L. Goldberg, MD, PhD Byers Eye Institute Stanford University</td>
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<td>11:10-11:35am</td>
<td>Photoreceptor synapse formation in vivo and in retinal organoid cultures</td>
<td>Anand Swaroop, PhD, FARVO National Eye Institute National Institutes of Health</td>
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| 11:35-12:00pm    | RPE Stem Cell Transplantation and Endogenous Activation               | **Jeff Stern, MD, PhD**  
Neural Stem Cell Institute  
Regenerative Research Foundation |
| 12:00-12:15pm    | Questions                                                            |                                                                         |
| 12:15-12:30pm    | Lunch                                                                |                                                                         |
| **Niches and Scaffolds: Stem Cell Microenvironment Controlling Cell Fate and Function** |                                                                         |
| 1:25-1:50pm      | Bioengineering Cornea Tissues for in vitro and in vivo Utility       | **David Kaplan, PhD**  
Bioengineering and Biotechnology Center  
Tufts University School of Engineering |
| 1:50-2:15pm      | Development of biomaterials to control the microenvironment for cell-based retinal regeneration strategies | **Rebecca L. Carrier, PhD**  
Northeastern University School of Engineering |
| 2:15-2:25pm      | Questions                                                            |                                                                         |
| **Modeling Eye Development and Diseases in 3D culture**      |                                                                         |                                                                         |
| 2:25-2:50pm      | A retinal organoid view into the mechanisms of human eye development and regeneration | **Valeria Canto Soler, PhD**  
Retinal Degeneration Research Center  
Wilmer Eye Institute  
Johns Hopkins University School of Medicine |
| 2:50-3:15pm      | Patient-specific 3D Engineered Ocular Tissue to Identify Mechanism of AMD Onset and Progression | **Kapil Bharti, PhD**  
Intramural Research Program  
National Eye Institute  
National Institutes of Health |
| 3:15-3:25pm      | Questions                                                            |                                                                         |
| 3:25-3:40pm      | Break                                                                |                                                                         |
| **Therapeutics and Translation**                          |                                                                         |                                                                         |
| 3:40-4:05pm      | Microengineered Physiological Biomimcry: Human Organ-on-Chips        | **Dongeun (Dan) Huh, PhD**  
University of Pennsylvania |
Time | Topic | Faculty
---|---|---
4:05-4:15pm | Questions | 
4:15-4:30pm | Summary and Closing Remarks | Valeria Canto Soler, PhD  
Retinal Degeneration Research Center  
Wilmer Eye Institute  
Johns Hopkins University School of Medicine

**PROGRAM INFORMATION**

**Statement of need**
Clinician-scientists involved in these areas of research should know the multiple aspects of organogenesis including morphological and molecular events underlying organ formation in vitro and in vivo, experimental systems, parallel pathways for organ formation in various models organisms, adult organ structure and pathology, organ regeneration and repair, 3D stem cell culture systems, cell and tissue engineering.

The use of stem cell populations and organoids to generate replacement tissues/organs to correct acquired and genetic human eye diseases as well as drug screening is proving to be crucial to the development of new therapies for retinal degenerations.

**Course description**
The use of stem cell populations and organoids to generate replacement cells/tissues/organs/ to correct acquired and genetic human eye diseases as well as drug screening is proving to be crucial to the development of new therapies for eye diseases. This course will discuss the multiple aspects of cell replacement strategies including stem cell differentiation and integration into existing tissues. Regenerative therapies including morphological and molecular events underlying organ formation in vitro and in vivo, 3D stem cell culture systems, cell and tissue engineering, and transplantation methods will also be discussed.

**Target audience**
The course will address the educational needs of clinicians and basic scientist seeking to learn more about differentiation of stem cells into ocular tissues including retinal neurons and how 3D organoids might be a better therapeutic strategy for stem cell therapies.

**Educational objectives**
After completing this activity, participants should be to:

- List the basic processes by which retinal, corneal, and other ocular tissues are regenerated from stem cell sources
- Describe various technologies including niches and scaffolds needed to regenerate or create replacement tissues and organs for the treatment of eye diseases
- Discuss transplantation techniques for retinal and corneal tissue
- Recall strategies to promote replacement cell survival and migration upon injection
- Explain the latest advances in the development of human 3D microphysiological systems as in vitro preclinical models for predicting the efficacy of new therapies in clinical trials
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ARVO Education Courses provide a forum for the open exchange and discussion of research results and scientific advancements in the field of ophthalmology; however, ARVO makes no representation or warranty as to the truth, originality, or accuracy of the information presented at the courses or in materials distributed in connection with them. Nor are the views expressed by the individual speakers necessarily the views of ARVO. ARVO supports the ACCME’s policy on evidence-based content and encourages faculty to adhere to these standards when planning a presentation.

Participants have an implied responsibility to use the newly acquired information to enhance patient outcomes and their own professional development. The information presented in this activity is not meant to serve as a guideline for patient management. Any procedures, medications, or other courses of diagnosis or treatment discussed in this activity should not be used by clinicians without evaluation of patient conditions and possible contraindications on dangers in use, review of any applicable manufacturer’s product information, and comparison with recommendations of other authorities.

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Certificate of attendance
Certificates of Attendance can be obtained in the course room, ARVO Central in the Exhibit Hall or by contacting ARVO Education at education@arvo.org.

Registration fees
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<th>By March 3</th>
<th>By April 28</th>
<th>After April 28</th>
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<tr>
<td>Member</td>
<td>$189</td>
<td>$214</td>
<td>$239</td>
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<tr>
<td>Member-in-training</td>
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<td>$149</td>
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<tr>
<td>Non-member</td>
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<td>Student Non-member</td>
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Cancellation and refund policy
The deadline to receive refund requests is Friday, April 28, 2017. Registration fees will be refunded for the amount paid, less a $40 administrative fee ($20 for students). No refunds will be given for cancellations
CONTINUING MEDICAL EDUCATION INFORMATION

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The AMA has determined that physicians not licensed in the USA who participate in this CME activity are eligible for AMA PRA Category 1 Credits™.

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Attendees interested in claiming AMA PRA Category 1 Credits™ credit for attendance at this education course, should contact ARVO education at education@arvo.org. A link to the required online course evaluation form will be sent within 2-business days of receipt of your request. Attendees will need to complete the form and indicate the extent of their participation in the course before downloading a CME Certificate or Certificate of Participation. Certificates are official. You will not receive additional documentation.

All requests for credit must be submitted no later than 11:59pm U.S. Eastern Time on Thursday, Aug. 31, 2017. Late request for credit may not be honored.

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*Some accrediting agencies may award equivalent Continuing Education (CE) credit when presented with a Certificate of Participation from an activity that offered AMA PRA Category 1 Credit™. Check with your accrediting agency for information.

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Faculty disclosures
The following faculty members have reported they have no financial relationships to disclose.
Kapil Bharti, PhD; Rebecca L. Carrier, PhD; Jeffrey L. Goldberg, MD, PhD; David Kaplan, PhD;; Jeff Stern, MD, PhD; Anand Swaroop, PhD, FARVO; Kang Zhang, MD, PhD

The faculty reported the following financial relationships or relationships to products or devices they or their spouse/life partner have with commercial interests related to the content of this activity.

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<tr>
<td>Valeria Canto Soler, PhD</td>
<td>Johns Hopkins University (P)</td>
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<tr>
<td>Dongeun (Dan) Huh, PhD</td>
<td>Emulate Inc. (P)</td>
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Planner/Manager disclosures
The following planners and managers have reported they have no financial relationships to disclose.

Course Organizer:
Lisa A. Neuhold, PhD

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Lindsay Scott, PT, DPT, ATC

Other managers and staff:
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CME Committee:

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<tr>
<td>Jacque L. Duncan, MD</td>
<td>AGTC (C); Avalanche (C); California Institute for Regenerative Medicine (C), (F); Foundation Fighting Blindness (C), (F); Ionis Pharmaceuticals, Inc. (C); Neurotech USA, Inc. (F); Ocugen, Inc. (C) Okuvision (C); QLT, Inc. (C); Shire Human Genetic Therapies, Inc. (C); Spark Therapeutics (C); US Food and Drug Administration Office of Orphan Product Development (F)</td>
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<td>Anton B. Hommer, MD</td>
<td>Alcon (R); Aerie (R); Allergan (R); Haag Streit (R); Heidelberg (R); Pfizer (R); Santen (R), (F)</td>
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PDEC Committee:

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<td>Rajashekhar Gangaraju, PhD</td>
<td>Cell care Therapeutics, Inc. (F), (I), (P), (C)</td>
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Faculty disclosures for non-CME program

The following faculty members have reported they have no financial relationships to disclose.
Paul A Sieving, MD, PhD, FARVO

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<td>Thomas A. Reh, PhD</td>
<td>Inception Biosciences (F); Inception Biosciences (C)</td>
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<td>Scheffer Tseng</td>
<td>NIH (F); TissueTech (E); TissueTech: (F); TissueTech: (I)</td>
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Financial relationships disclosure codes key

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