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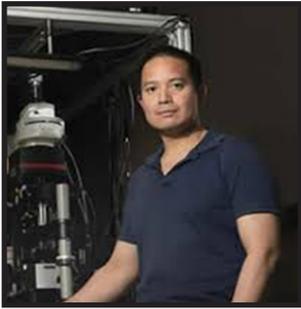
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# WEBINARS

## OCULAR DISEASE

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### OCULAR WEBINAR

## ORIGINS OF EXTRAORDINARY VISUAL ACUITY IN PRIMATES

**Michael Tri Do | Associate Professor, Harvard Medical School  
and Boston Children's Hospital**

#### Top Keywords

- Macular degeneration
- Fovea function
- Fovea anatomy

#### Short Bio

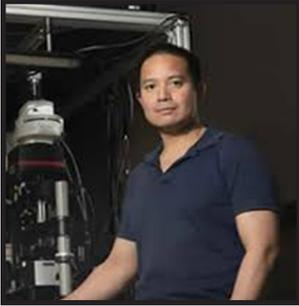
Dr. Michael Do is an Associate Professor at Harvard Medical School and Boston Children's Hospital. Mike obtained his PhD in Neurobiology at Harvard Medical School and completed his post-doctoral training at Johns Hopkins University in 2011. The research focus of Mike's lab is to understand how light drives visual perception and the setting of the internal body clock.

#### Webinar title

Origins of Extraordinary Visual Acuity in Primates

#### Webinar abstract

In this webinar, Dr. Gao presents a comprehensive overview on the key principles, history, current challenges, and future directions of human gene therapy with a focus on AAV gene therapy for rare diseases. The webinar also showcases AAV capsid engineering to modulate target tissue tropism and biodistribution and includes examples of using AAV for gene replacement, gene addition, gene silencing and *in vivo* gene editing in preclinical studies. Additionally, strategies to overcome immunological barriers to improve the efficacy of AAV-mediated gene therapy are reviewed.



## OCULAR WEBINAR

### SENSING LIGHT FOR PHYSIOLOGICAL CONTROL

**Michael Tri Do | Associate Professor, Harvard Medical School  
and Boston Children's Hospital**

#### **Top Keywords**

- Retinal ganglion cells
- Depolarization
- Iprgc discovery
- Iprgc melanopsin

#### **Short Bio**

Dr. Michael Do is an Associate Professor at Harvard Medical School and Boston Children's Hospital. Mike obtained his PhD in Neurobiology at Harvard Medical School and completed his post-doctoral training at Johns Hopkins University in 2011. The research focus of Mike's lab is to understand how light drives visual perception and the setting of the internal body clock.

#### **Webinar title**

Sensing Light for Physiological Control

#### **Webinar abstract**

In this webinar, Dr. Do describes the structure and function of intrinsically photosensitive retinal ganglion cells (ipRGCs) in mouse and primate eyes focusing on tuning of the cells to specific light intensities. This work demonstrates an important depolarization block as light intensity increases highlighting the precise firing of the ipRGCs for rapid decoding of irradiance.

## WEBINAR SERIES

# NOVEL APPROACHES IN CELL AND GENE THERAPIES

### Introduction

Rapid advancements in the development of novel cell and gene therapies have been powered by technological innovations in gene delivery and viral engineering. One of the big challenges in the gene therapy space is the immune response to vectors that has short- and long-term consequences, so it is critical to engineer gene therapy vectors to overcome the neutralizing antibody response. Another area of rapid growth is stem cell therapy space and promising therapies are being developed in several disease areas including eye diseases and neurodegenerative diseases.

In this webinar series, we highlight the key principles of virus mediated gene therapy and the development of novel cell therapies. The webinars showcase multiple approaches to viral vector development including strategies to overcome the neutralizing immune response, and highlights the development of novel cell therapies for the central nervous system and Parkinson's disease.



## WEBINAR SERIES

### GENE THERAPY FOR RARE DISEASES – A NEW PARADIGM IN MEDICINE

**Guangping Gao | Professor, University of Massachusetts  
Medical School**

### Top Keywords

- Gene therapy
- AAV
- AAV capsid
- Rare diseases

### Short Bio

Dr. Gao is the Co-Director of the Li Weibo Institute for Rare Diseases Research, Director, Horae Gene Therapy Center and Viral Vector Core, Professor of Microbiology and Physiological Systems, Penelope Booth Rockwell Professor in Biomedical Research at the University of Massachusetts Medical School.

He was elected as a fellow of the US National Academy of Inventors and American Academy of Microbiology and is the outgoing President of the American Society of Gene and Cell Therapy. He was ranked #4 in the World Top 20 Translational Researchers in 2018 by Nature Biotechnology.

Dr. Gao has focused on molecular genetics and viral vector gene therapy of rare genetic diseases over his 30-year career, and has co-founded several companies including Voyager Therapeutics, that focus on developing AAV gene therapeutics for rare diseases.

### Webinar title

Gene Therapy for Rare Diseases – A New Paradigm in Medicine

### Webinar abstract

In this webinar, Dr. Gao presents a comprehensive overview on the key principles, history, current challenges, and future directions of human gene therapy with a focus on AAV gene therapy for rare diseases. The webinar also showcases AAV capsid engineering to modulate target tissue tropism and biodistribution and includes examples of using AAV for gene replacement, gene addition, gene silencing and *in vivo* gene editing in preclinical studies. Additionally, strategies to overcome immunological barriers to improve the efficacy of AAV-mediated gene therapy are reviewed.



## WEBINAR SERIES

# STRATEGIES FOR IN VIVO BARRIERS TO GENE THERAPY VECTORS

**Casey Maguire | Associate Professor of Neurology,  
Harvard Medical School**

### Top Keywords

- Gene therapy
- AAV
- AAV vector
- AAV capsid
- Gene delivery
- Microvesicles
- Extracellular vesicles

### Short Bio

Dr. Casey Maguire is currently an Associate Professor of Neurology at Harvard Medical School and an Investigator at the Massachusetts General Hospital (MGH). Casey received his PhD from the University of Rochester Medical School. He has been at MGH since 2006 starting with a post-doctoral fellowship followed by Instructor and Assistant Professor positions. His research focuses on improving virus vectors to allow effective human gene therapy in the face of delivery and immune-related barriers.

### Webinar title

Strategies for in vivo barriers to gene therapy vectors

### Webinar abstract

In this webinar, Dr. Maguire discusses technological innovation of AAV (adeno-associated virus) vectors to overcome challenges associated with AAV vector delivery to target organs and immune evasion. His talk focuses on two innovative approaches to improve AAV gene delivery to the central nervous system (CNS). The first approach is screening AAV capsid libraries using *in vitro* and *in vivo* models to identify and validate clones that have enhanced delivery to the CNS. The second approach uses extracellular vesicles or microvesicles to deliver AAVs more efficiently to multiple organs including the brain, eye, liver and inner ear.



## WEBINAR SERIES

# RETARGETING AND SHIELDING MEASLES VIRUS VECTORS FOR ONCOLYTIC VIROTHERAPY

**Miguel Muñoz-Alía | Research Associate, Mayo Clinic**

### Top Keywords

- Gene therapy
- Measles
- Oncolytic virus
- Measles vaccination

### Short Bio

Dr. Muñoz-Alía is a Research Associate in the Department of Molecular Medicine at the Mayo Clinic. He received his PhD in Cell Biology and Genetics at Autonomous University of Madrid, Spain, after completing his bachelor's degree in Biology. He began his career at Spanish National Reference Laboratory for Measles and Spanish National Center for Biotechnology, where he acquired a keen interest in molecular epidemiology and evolution of measles virus genotypes. In 2015, he joined the Mayo Clinic where he is working on the clinical development of oncolytic measles viruses.

### Webinar title

Retargeting and Shielding Measles Virus Vectors for Oncolytic Virotherapy

### Webinar abstract

In this webinar, Dr. Muñoz-Alía discusses how the measles virus can be engineered to specifically attack tumors as an oncolytic virus (OV). One of the primary challenges with using the measles virus as an OV is the presence of neutralizing antibodies due to the measles vaccination in childhood.

In order to efficiently target the measles virus to specific tumors, viral envelope proteins from the canine distemper virus are inserted to replace the measles virus membrane fusion proteins. This gene therapy approach showed efficient transduction of tumor cells while avoiding the neutralizing antibody response in myeloma and ovarian cancer animal models.



## WEBINAR SERIES

# REGENERATIVE STRATEGIES IN THE CENTRAL NERVOUS SYSTEM

**Molly Shoichet | University of Toronto**

### Top Keywords

- Hydrogel
- Age related macular degeneration
- Amd eye
- Retinitis pigmentosa treatment
- Stroke damage
- Cell therapy for blindness

### Short Bio

Professor Molly Shoichet holds the Tier 1 Canada Research Chair in Tissue Engineering at the University of Toronto. Her research is focused on drug and cell delivery strategies in the central nervous system and 3D hydrogel culture systems to model cancer, and she has also co-founded four spin-off companies. 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, Engineering and Health Sciences. Additionally, she is a Fellow of the Royal Society and Foreign Member of the US National Academy of Engineering. Dr. Shoichet received her Bachelor's degree from MIT and her PhD from the University of Massachusetts, Amherst in Polymer Science and Engineering.

### Webinar title

Regenerative Strategies in the Central Nervous System

### Webinar abstract

In this 2-part webinar, Dr. Shoichet discusses two methods of cell regeneration in the CNS – one method is a cell transplantation in the retina and the other method is the stimulation of endogenous stem cells to replace neurons after a stroke.

The cell transplantation in the eye is a method where PR (photoreceptor) and RPE (retinal pigment epithelium) cells in a hyaluronan-methylcellulose hydrogel are injected subretinally into two mouse models of blindness. Injection of both PR and RPE cells in the hydrogel resulted in improved vision, and the hydrogel prevented cell clumping and also had an anti-inflammatory effect. This cell therapy for blindness could be potentially used as an age-related macular degeneration therapy or retinitis pigmentosa treatment.

In the second segment, Dr. Shoichet describes a less invasive strategy to stimulate endogenous stem cells to differentiate into neurons after stroke damage. An osmotic minipump is used to deliver therapeutics like cyclosporine A and erythropoietin in the hydrogel to repair cell damage and induce tissue regeneration. The delivery of known stem cell differentiators in the hydrogel was shown to improve tissue regeneration and function in mouse and rat models of stroke.



## WEBINAR SERIES

# CHAMELEON'S EVADER TECHNOLOGY: A MORE POTENT AAV VECTOR WITH LESS IMMUNE RESPONSE

**Jeffrey Vick | Chief Business Officer, Chameleon Biosciences**

### Top Keywords

- Gene therapy
- AAV
- AAV vector
- Gene delivery
- Immune response

### Short Bio

Jeffrey Vick is a co-founder and chief business officer at Chameleon Biosciences, an early stage gene therapy company that is developing a proprietary vector platform EVADER. Jeff has more than 25 years of senior management experience in the global biopharmaceutical industry and has particular expertise in Gene Therapy. He has led the Business development and IP groups at Genethon and Gencell and has also led multiple collaborations with companies like Audentes and GenSight. Jeff has also worked as a venture capitalist where he founded, grew and sold multiple companies, and also served as the CEO of Silence Therapeutics. He earned an MBA from Stanford University following a Master's degree in Chemistry from UC San Diego and a Bachelor's degree in Chemistry from the University of Virginia.

### Webinar title

Chameleon's EVADER technology: a more potent AAV vector with less immune response

### Webinar abstract

In this webinar, Jeff discusses Chameleon's EVADER™ gene therapy platform that is designed to evade the immune system, thus overcoming the neutralizing immune response, expanding the treatable patient population and supporting multiple dosing. The platform envelopes AAVs in a lipid bilayer with specific immune cell inhibitors and is shown to have increased infectivity, delivery and transgene expression in preclinical models. The EVADER™ platform is currently being used to develop gene therapies for multiple diseases in partnership with biotech and pharma companies.

## WEBINAR SERIES

# BIOANALYSIS OF LARGE MOLECULE THERAPIES

### Introduction

Bioanalytical studies are an integral part of pharmacokinetics (PK) and pharmacodynamics (PD) studies, as well as the evaluation of drug toxicity. Typically, an array of methodologies is used to obtain a comprehensive profile of the behavior of the therapy in a systemic biology model. Bioanalysis of large molecule therapies is an evolving area with unique requirements and challenges.

In this 2-part webinar series, experienced bioanalysis scientists highlight the needs and challenges of regulated and non-regulated bioanalysis of therapies with a focus on large molecule drugs. The webinars include a review of bioanalytical methods including ligand binding assays and immunogenicity assays.



## WEBINAR SERIES

### LARGE MOLECULE BIOANALYSIS: CONCEPTS, TOOLS AND CHALLENGES

**Franklin Spriggs | Primary Consultant, Spriggs Bioanalytical Consulting LLC**

### Top Keywords

- Bioanalysis in drug discovery and development
- Large molecule bioanalysis
- PK bioanalysis
- Ligand binding assays
- Types of ligand binding assays

### Short Bio

Franklin Spriggs is a bioanalytical scientist and primary consultant at Spriggs Bioanalytical Consulting LLC. Frank joined Pfizer at the Groton, Connecticut site in 2007 where he became active in the AAPS organization, holding positions in the BIOTEC and Regulatory Sciences sections. In 2015, he received his master's degree in Regulatory Affairs and Quality Assurance from Temple University and then spent 5 years at CROs, working at AIT Bioscience and KCAS Bioanalytical and Biomarker Services.

### Webinar title

Large Molecule Bioanalysis: Concepts, tools and Challenges

### Webinar abstract

In this webinar, Franklin introduces the basic concepts of bioanalysis including the important role of bioanalysis in successful drug development and reviews currently used plate based and non-plate based assay methods to analyze large and small molecule therapies. He highlights some of the requirements and challenges associated with the bioanalysis of large molecule therapies including antibody-drug conjugates (ADCs), bispecific monoclonal antibodies (mAbs) and protein replacement therapies.



## WEBINAR SERIES

### ASPECTS OF REGULATED BIOANALYSIS

**Andrea Wakefield | Bioanalysis Consultant**

#### **Top Keywords**

- Bioanalysis in drug discovery and development
- Large molecule bioanalysis
- PK bioanalysis
- Ligand binding assays
- Types of ligand binding assays
- Anti-drug antibodies

#### **Short Bio**

Andrea Wakefield is an experienced bioanalytical scientist and independent consultant. Andrea has over 15 years' experience at biotechs and CROs including Alexion Pharmaceuticals, IBEX Pharmaceuticals and Charles River Laboratories. She has supported multiple INDs, clinical trial applications and market authorizations and has experience in gap assessment and mitigation, as well as providing bioanalytical solutions for rare disease therapy development. She obtained her Bachelor's degree with Honors from the University of Ottawa and completed her Master's degree at the University of New Brunswick.

#### **Webinar title**

Aspects of Regulated Bioanalysis

#### **Webinar abstract**

In this webinar, Andrea focuses on the importance of ligand binding assays in the bioanalysis of small and large molecule therapies and highlights the role of orthogonal assays such as LC-MS to complement the data from ligand binding assays. She discusses preclinical, nonclinical (GLP) and clinical assay development and design including the development of immunogenicity assays and the detection of anti-drug antibodies (ADAs). The importance of bioanalysis data is highlighted with a case study on the development of a therapy for a pediatric rare disease.

# KEYNOTE WEBINARS

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## KEYNOTE WEBINAR LEAVING NO DRUG BEHIND

**Patrick Dixon | Author, Physician and Futurist,  
Chairman of Global Change Ltd.**

### Top Keywords

- Drug discovery and development
- Drug development
- Artificial intelligence
- Anti-aging
- Pharmacogenomics

### Short Bio

Dr. Patrick Dixon is a renowned author, physician and futurist. He has consulted extensively with large pharmaceutical companies, and had also founded Virtu Biologics, an oncolytic virus biotech. Patrick is the chairman of Global Change Ltd, a leading growth strategy and forecasting company and has been ranked as one of the 20 most influential business thinkers alive today.

### Webinar title

Leaving No Drug Behind

### Webinar abstract

In this 5-part webinar, Patrick discusses several topics that are highly relevant in the drug discovery and development space.

- In the first segment, Patrick highlights the need for innovation and collaboration between pharma companies and CROs that have specialty expertise in medtech and infotech, to improve clinical success rates and increase the speed to market, especially for therapies relevant to COVID-19.
- In the second segment, he explores seven mechanisms of aging that are sources for druggable targets for anti-aging therapies, and highlights the healthcare and economic impact of an aging population.
- In the third segment, Patrick discusses the impact of recent technologies such as CRISPR gene editing and whole genome sequencing and the critical role of the microbiome on disease management and drug development.
- In the fourth segment, he shares examples on how AI (artificial intelligence) is being used to more accurately diagnose and monitor therapeutic response.
- In the final segment, he tackles a challenging topic – the high cost of drug development that can preclude promising drugs from being commercialized and limits access to therapies. He predicts that drug development costs will fall partly due to the rapid growth of biosimilars and low cost of generic drug manufacturing.



## KEYNOTE WEBINAR

# THE FUTURE OF HEALTH, BIOMEDICINE AND LIFE SCIENCES: WHERE CAN TECHNOLOGY TAKE US?

**Daniel Kraft | Physician-scientist, Inventor, Entrepreneur, and Innovator**

### Top Keywords

- Biomedicine
- Artificial Intelligence
- Medical apps
- Digital Health

### Short Bio

Dr. Daniel Kraft is a Stanford and Harvard trained physician-scientist, inventor, entrepreneur, and innovator. With over 25 years of experience in clinical practice, biomedical research and healthcare innovation, Daniel has chaired the Medicine for Singularity University since its inception in 2008, and is founder and chair of Exponential. Daniel received undergraduate degrees from Brown University and completed medical school at Stanford. After completing his residency at Massachusetts General Hospital & Boston Children's Hospital, and fellowships in hematology, oncology and bone marrow transplantation at Stanford, Daniel was Board Certified in both Internal Medicine & Pediatrics.

### Webinar title

The Future of Health Biomedicine and Life Sciences: Where Can Technology Take Us?

### Webinar abstract

In this 2-part webinar, Daniel Kraft discusses the crucial impact of technological innovations on improving healthcare and drug development. In the first segment, he explores the power of exponential thinking to reimagine healthcare and medical research, in combination with the continuous development of medical apps and devices to support end to end patient care, digital health and accelerate drug discovery and development. In the second segment, Daniel discusses novel approaches in drug development including:

- Leveraging pharmacogenomics to design smarter clinical trials and develop personalized therapies
- Using artificial intelligence or AI to improve diagnosis and prognostic monitoring of therapeutic response
- Collaboratively using AI in conjunction with traditional scientific methods to accelerate drug development
- Novel methods of targeted drug delivery



**MODERATOR: Anjali Venkateswaran, PhD**

**Short Bio**

Dr. Anjali Venkateswaran is a scientific marketing consultant with 15+ years' experience in the life sciences industry, including 10 years as a product manager and strategic marketer. Most recently, she was leading strategic marketing at a venture funded cancer diagnostic company. Prior to that, she managed marketing for the neuroscience and rare disease Discovery portfolio at Charles River Laboratories. Anjali started her career at Cell Signaling Technology as a scientist followed by commercial roles. She received her PhD in Biochemistry from the Ohio State University