3rd Annual

Gene Therapy for Ophthalmic Disorders

REGISTER NOW!

September 13-16, 2022
Boston, MA

Revolutionizing Genetic Therapy Development & Delivery to the Eye

Your 40+ Expert Speakers Include:

Daniel Chung
Chief Medical Officer
SparingVision

Shannon Boye
Founder & Director
Atsena Therapeutics

Steve Pakola
Chief Medical Officer
REGENXBIO

Theresa Heah
President & Chief Medical Officer, Ophthalmology
Kriya Therapeutics

Richard Beckman
Chief Medical Officer
Adverum

Catherine O'Riordan
Head Translation Cluster, Genomics Unit
Sanofi

Stylianos Michalakis
Co-Founder & Chief Scientific Advisor
ViGeneron

Abraham Scaria
Chief Scientific Officer
AGTC

Hemant Khanna
Vice President of Preclinical Ocular Research
IVERIC Bio

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Macular Society
Beating Macular Disease

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hansonwade

WELCOME TO THE 3RD ANNUAL GENE THERAPY FOR OPHTHALMIC DISORDERS

2nd Gene Therapy for Ophthalmic Disorders Attendees Said:

"Very informative and interesting"
GenSight

"Outstanding meeting. The science was great, but it was also very well run"
Foundation Fighting Blindness

"High quality, relevant, and up-to-date presentations from leaders in ophthalmic gene therapy provided an informative and insightful virtual meeting experience"
AbbVie

Discovering Next Generation Administration Routes for the Eye; Overcoming the Translational Challenges of Ophthalmic Preclinical Models; & Discussing Toxicity & Targeting Challenges of Vector-Based Approaches

The ophthalmology field is bursting with novel genetic approaches for targeting many disorders, with gene therapy taking centre stage as a one-time treatment option. With more invasive subretinal approaches being used in the clinic, as well as more innovative outpatient methods such as suprachoroidal delivery, there are a lot of pros and cons to discuss as the field progresses towards developing efficacious and durable treatments for many ophthalmic disorders.

Returning for its 3rd year, the **Gene Therapy for Ophthalmic Disorders** is the definitive forum for those working on ophthalmic gene therapy. This is the only conference that unites pharma and biotech to address the most pressing challenges, with focus on discussing and sharing solutions with the key leaders in the industry.

This year we have expanded into two streams of learning, putting more emphasis on the individual challenges faced in both the preclinical and clincal phases of ophthalmic gene therapy drug development.

Gene therapy for ophthalmic disorders has only seen one FDA approved therapy, which drug will be the next? **Join 110+ of your peers** to discuss and overcome the barriers preventing drugs getting to market.

Leave the definitive **3rd Annual Gene Therapy for Ophthalmic Disorders** forum equipped with knowledge of **novel methods and developments, actionable lessons** learned and **new connections**. Don't miss your opportunity to be a part the conversation which could lead to the **next drug approval**.

Your Top 5 Reasons to Attend this Year's Gene Therapy for Ophthalmic Disorders Summit:

1. **Uncover novel methods of capsid engineering with Atsena Therapeutics, ViGeneron and IVERIC Bio, showing the next generation of vector development**
2. **Engage in three, expert-lead workshops that will explore non-viral delivery modalities, retinal biomarkers for ophthalmic gene therapy and novel gene therapies for the protection of retinal ganglion cells, with workshop leaders from Novartis, Intergalactic Therapeutics, Nanoscope Therapeutics & AXONIS Therapeutics**
3. **Hear from Daniel Chung at SparingVision, on how to develop an effective patient outreach program that ensures patient comprehension and input in clinical development**
4. **Explore the use of immunosuppressants and anti-inflammtory agents in aiding preclinical safety data with Janssen, Northern Biomedical Research & Novartis**
5. **Discover the next generation of technology and devices being used in the delivery of ophthalmic gene therapy from Clearside Biomedical, REGENXBIO, University of Oxford and Everads Therapy during our highly anticipated post-conference delivery day**

YOUR EXPERT SPEAKERS

Abraham Scaria
Chief Scientific Officer
AGTC

Aniz Girach
Chief Medical Officer
ProQR

Arun Upadhyay
Chief Scientific Officer
Ocugen

Ashwath Jayagopal
Chief Scientific Officer
Opus Genetics

Catherine O'Riordan
Head Translational Cluster, Genomics Medicine Unit
Sanofi

Cathleen Gonzales
Director of Pharmacology
Intergalactic Therapeutics

Claire Gelfman
Chief Scientific Officer
Foundation Fighting Blindness

Daniel Chung
Chief Medical Officer
SparingVision

Erin O'Neil
Vice President of Clinical Development
Opus Genetics

Gayathri Ramaswamy
Vice President, Drug Discovery & Disease Biology
Intergalactic Therapeutics

Gerard Caelles
Chief Business Officer
SpliceBio

Hemant Khanna
Vice President, Pre-Clinicla Ocular Research
IVERIC Bio

Jake Ternent
Patient
Luxturna Patient (UK)

Jasmina Kapetanovic
Clinician & Consultant Vitreo-Retinal Surgeon
University of Oxford

Lauren Ayton
Assocaite Professor, & Head - Vision Optimisation
University of Melbourne

Lee Morris
Patient
Luxturna Patient (UK)

Shannon Boye
Founder & Director
Atsena Therapeutics

Maen Obeidat
Executive Director - Therapeutics Area Head, Biomarker Development, Ophthalmology
Novartis (NIBR)

Magali Taiel
Chief Medical Officer
Gensight Biologics

Markus Peters
Chief Executive Officer
Aevitas Therapeutics (Formerly)

Matthew Wood
Patient
Luxturna Patient (UK)

Rachel Eclov
Gene Therapy Development Project Leader
Kriya Therapeutics

Richard Beckman
Chief Medical Officer
Adverum

Samarendra Mohanty
Chief Scientific Officer & President
Nanoscope Therapeutics

Sanghoon Kim
Project Engineer
Nanoscope Therapeutics

YOUR EXPERT SPEAKERS

Stephen Poor
Director of External Innovation and Translational Biomarkers
Novartis (NIBR)

Steve Pakola
Chief Medical Officer
REGENXBIO

Stylianos Michalakis
Co-Founder & Chief Scientific Advisor
ViGeneron

Subrata Batabyal
Senior Technical Officer
Nanoscope Therapeutics

Theresa Heah
President & Chief Medical Officer, Ophthalmology
Kriya Therapeutics

Viral kansara
Vice President of Preclinical Development
Clearside Biomedical

William Beltran
Director, Division of Experimental Retinal Therapies
University of Pennsylvania

Yoreh Barak
Director of Retina, Department of Ophthalmology
Everads Therapy

Nida Sen
Senior Director, Retina Clinical Lead
Janssen

Jake Ternent
Patient
Luxturna Patient (UK)

Diane Doughty
Director of Device Development
REGENXBIO

Ramkumar Ramamirtham
Associate Director & Optometrist
Novartis (NIBR)

Shane Hegarty
Chief Scientific Officer & Co-Founder,
AXONIS Therapeutics

Catherine Cukras
Director, Medical Retinal Fellowship Program
National Eye Institute (NEI)

Catarina Santos
Medical Director of Ophthalmology, Europe
Novartis

Amy Mawdesley
Product Manager
Newcells Biotech

Brianna Barrett
Associate Director of Technical Sales
Forge Biologics

Eric Buckland
Founder & Chief Executive Officer
Translational Imaging Innovations (TII)

Simon Kaja
Chief Scientific Officer & Vice President
Experimentica

Miquel Vila-Perello
Chief Executive Officer & Chief Scientific Officer
SpliceBio

PRE-CONFERENCE WORKSHOP DAY
TUESDAY, SEPTEMBER 13, 2022

These workshops will offer a one-day vital learning experience to establish the most critical areas of ophthalmic gene therapy research and development. Designed to help established and new companies understand a range of important topics in a greater depth than the main conference allows, giving you more time for discussion and to ask your burning questions.

These workshop will provide:

- Insight and discussion from companies working non-viral vector therapies
- Exploration into biomarkers utilized in ophthalmic gene therapy clinical trials
- Discovering the use of gene therapy to protect and repair retinal ganglion cells and a novel optic nerve crush model

WORKSHOP **A** 8:30 - 11:00

Discussing the Use of Non-Viral Gene Delivery as a Therapeutic Modality to the Eye

- Reviewing the conventional approaches of non-viral delivery (microinjection, electroporation, lipofection)-advantages and limitations
- Laser based approach for non-viral targeted delivery-safety and efficacy
- Clinical translation roadmap of image-guided laser-based gene delivery-safety, regulatory path

Subrata Batabyal, Senior Technical Officer, **Nanoscope Therapeutics**

Sanghoon Kim, Project Engineer, **Nanoscope Therapeutics**

Gayathri Ramaswamy, VP, Drug Discovery & Disease Biology, **Intergalactic Therapeutics**

WORKSHOP **B** 11:30 - 2:00

Preclinical Advances, Challenges & Tools for Developing Novel Gene Therapies for Retinal Ganglion Cell Neuroprotection, Regeneration and Repair In Optic Neuropathies

- State of the art on retinal ganglion cell neuroprotection and regeneration
- Challenges remaining for retinal ganglion cell repair in optic neuropathies
- AAV-CRISPR screening in optic nerve crush model for identification of novel gene therapy candidates

Shane Hegarty, Chief Scientific Officer & Co-Founder, **AXONIS Therapeutics**

WORKSHOP **C** 2:30-5:00

Opportunities & Challenges for Ocular, Systemic, Functional & Structural Biomarkers for Retina Indications

- What are the challenges in IRD trials?
- Functional and structural end points for IRD, past, present and future
- Physical demonstration of select low vision tests
- Biomarkers in clinical development

Stephen Poor, Director of External Innovation and Translational Biomarkers, **Novartis Institutes for BioMedical Research**

Maen Obeidat, Executive Director – Therapeutics Area Head, Biomarker Development, Ophthalmology, **Novartis Institutes for BioMedical Research**

Ramkumar Ramamirtham, Associate Director & Optometrist, **Novartis Institutes for BioMedical Research**

CONFERENCE DAY ONE - WEDNESDAY, SEPTEMBER 14, 2022

8.15 **Chairs Opening Remarks**

Daniel Chung
Chief Medical Officer
SparingVision

Overviewing Recent Developments In The Ophthalmic Gene Therapy Space

8.25 **Panel Discussion: Gene Therapy for Ophthalmic Disorders – A Year in Review**

- Developments and setback in the last 12 months
- Overview of instrumental work that has worked towards solving major issues
- Discussing the sustainability of ophthalmic gene therapy
- What does the coming year look like?

Moderator:

Daniel Chung
Chief Medical Officer
SparingVision

Theresa Heah
President & Chief Medical Officer, Ophthalmology
Kriya Therapeutics

Markus Peters
Chief Executive Officer
Aevitas Therapeutics (Formerly)

Samarendra Mohanty
Chief Scientific Officer & President
Nanoscope Therapeutics

Catherine O'Riordan
Head Translational Cluster, Genomics Medicine Unit
Sanofi

Claire Gelfman
Chief Scientific Officer
Foundation Fighting Blindness

9.15 **Highlighting the Modifier Gene Therapy Approach for the Treatment of Retinitis Pigmentosa**

- Discussing the preclinical data of our Retinitis Pigmentosa treatment
- Reviewing the early clinical data and outlining their indications
- Explaining the science behind the modifier gene therapy approach

Arun Upadhyay
Chief Scientific Officer
Ocugen

9.45 **Evaluating Gene Therapy Assets in Preclinical Ocular Models**

- Species considerations for drug development of ocular gene therapy assets
- Ocular routes of delivery for gene therapy in preclinical model
- Efficacy and safety assessment of gene therapy assets in preclinical ocular models

Simon Kaja
Chief Scientific Officer & Vice-President
Experimentica

9.55 **Utilizing Gene Therapy to Induce Long-Term Treatment of Wet AMD & Diabetic Retinopathy**

- Outline the RGX-314 gene therapy program
- Discussing why suprachoroidal/subretinal delivery are optimal for this treatment
- Reviewing the current clinical data from Phase III clinical trials

Stephen Pakola
Chief Medical Officer
REGENXBIO

10.25 **Speed Networking**
This session is the ideal opportunity to get face-to-face time with many of the brightest minds working in the gene therapy ophthalmic field and establish meaningful business relationships to pursue for the rest of the conference.

11.25 **Coffee Break**

PRECLINICAL STREAM

Chair: **Shannon Boye,** Founder & Director, **Atsena Therapeutics**

Examining Current & Next Generation Disease Models for the Eye to Provide Better Translational Data

11.55 **Highlighting the Most Efficacious Animal Models for Testing the Inflammatory Response of Gene Therapy Delivery to the Eye**

- Considerations for model development
- Testing of anti-inflammatory therapy in a mouse mode
- Observed challenges and next steps

Rachel Eclov, Gene Therapy Development Project Leader, **Kriya Therapeutics**

12.25 **Discovering Canine Models for Inherited Retinal Diseases to Evaluate Retinal Gene Therapies**

- Natural history of disease in dogs versus humans
- Route of delivery of AAV-mediated gene therapy
- Outcome measures of efficacy/safety studies in dogs

William Beltran, Director, Division of Experimental Retinal Therapies, **University of Pennsylvania**

CLINICAL STREAM

Chair: **Daniel Chung,** Chief Medical Officer, **SparingVision**

Mastering Patient Outreach & Enrolment for Better Communication with the Drug Recipients

11.55 **Creating an Effective Patient Outreach Programme to Ensure Patient Comprehension & Input into Clinical Development**

- Exploring appropriate methods of patient outreach
- Building relationships with Patient Advocacy groups
- Discovering the best methodology of ensuring patient education and expectations
- Benchmarking and recognizing the importance of the patient's voice in clinical development

Daniel Chung, Chief Medical Officer, **SparingVision**

12.25 **Looking Into Patient Perspectives on Ocular Gene Therapy**

- Development and validation of the Attitudes to Gene Therapy – Eye (AGT-EYE) tool
- Results of an Australian national survey of almost 700 people with IRDs and/or their carers on the knowledge and perspectives on gene therapy
- How can academics and industry work more closely with people with IRDs to codesign research and clinical trials?

Lauren Ayton, Associate Professor, & Head – Vision Optimisation**, University of Melbourne**

CONFERENCE DAY ONE - WEDNESDAY, SEPTEMBER 14, 2022

12.55 **Lunch Break & Networking**

Exploring Novel Developments in Vector Engineering & Selection to Benchmark New Therapeutic Platforms

1.55 Sharing Approaches for Splice Regulation & Modular Protein Function in Retinal Gene Therapy

- Limitations of AAV payload capacity
- Concept of miniaturizing large genes for AAV packageability
- Modular protein function as a modality to design minigenes

Hemant Khanna, Vice President, Preclinical Ocular Research, **IVERIC Bio**

2.25 Addressing the Unmet Need in Retinal Gene Therapy: Focus on IVT Delivery & Cargo capacity of AAV Vectors

- Overview of the benefits and limitations of AAV vectors and unmet needs in retinal gene therapy
- Introduction and update on next generation vgAAV-AAV capsids
- Introduction to REVeRT dual AAV technology

Stylianos Michalakis, Co-founder & Chief Scientific Advisor, **ViGeneron**

2.55 Tropism of AAV Vectors in Photoreceptor-Like Cells of Human iPSC-Derived Retinal Organoids

- The advantages of iPSC-derived human retinal organoids as an in vitro preclinical model
- The utility of iPSC-derived retinal organoids in gene therapy applications
- Expansion of AAV capsid and transgene options for preclinical testing of gene therapy

Amy Mawdesley Product Manager, **Newcells Biotech**

3.05 Exploring the Laterally Spreading AAV.SPR Capsid for Treatment of Inherited Retinal Diseases

- Outlining the efficacy features of this novel capsid
- Detailing the safety features of AAV.SPR capsid

Shannon Boye, Founder & Director, **Atsena Therapeutics**

Approaching Commercial Challenges of Ophthalmic Gene Therapies to Help your Product Reach Market

1.55 Attracting Financial Investment for Biotech Startups: the Case of SpliceBio

- Walking through a case study of an exciting and promising new genetic platform in ophthalmology
- Sharing the processes involved in attaining investment for a novel idea in a competitive field
- Showcasing innovating science to overcome the payload capacity of the AAV vectors to demonstrate a competitive edge in ophthalmology

Gerard Caelles, Chief Business Officer, **SpliceBio**

2.25 Understanding Gene Therapy After the Data: How Does it Reach the People in Need?

- Main issues in the implementation of innovative gene therapies from the country perspective (Payers, Doctors and Patients)
- Main opportunities of improvement - what have we learned so far?

Catarina Santos, Medical Director of Ophthalmology, **Europe, Novartis**

Remote

2.55 Establishing a Sustainable Gene Therapy Pipeline for Inherited Retinal Diseases

- Challenges and solutions for creating a sustainable gene therapy pipeline for rare inherited retinal diseases
- Opus Genetics' gene therapy portfolio for LCA and other classes of IRDs
- Outlining phase I clinical trial data for LCA5-IRD

Ash Jayagopal, Chief Scientific Officer, **Opus Genetics**

3.35 **Afternoon Refreshments**

4.05 **Panel Discussion: Ophthalmic Gene Therapy Patient Perspective**

- Do patients feel they know what treatments are available to them?
- Is there trial data available to patients so they can decide if they want to participate?
- How can an open line of communication be developed between patients and the industry?

Moderator:

Jasmina Kepatanovic
Clinician, Scientist & Consultant Vitreo-Retinal Surgeon
University of Oxford

Jake Ternent
Patient
Luxturna Patient (UK)

Matthew Wood
Patient
Luxturna Patient (UK)

Lee Morris
Patient
Luxturna Patient (UK)

CONFERENCE DAY ONE - WEDNESDAY, SEPTEMBER 14, 2022

4.35 **Efficient Gene Therapy Manufacturing for Ocular Diseases**

- Targeting sensitive biological spaces, such as the eye, require highly purified and well characterized products
- AAV therapeutics for ophthalmic dosing require low endotoxin, residuals, and empty particle contaminant levels
- Scalable processes that maintain high productivity and purity of the vector

Brianna Barrett
Associate Director of Technical Sales
Forge Biologics

4.45 **Exploring the Use of Gene Therapy to Treat Dry AMD**

- Comparing treatment for Wet AMD vs. Dry AMD
- Outlining the role of complement system in Dry AMD
- Displaying gene delivery of complement factor H

Abraham Scaria
Chief Scientific Officer
AGTC

5.15 **End of Conference Day One: Chair's Closing Remarks**

Daniel Chung
Chief Medical Officer
SparingVision

5:30 **Scientific Poster Session & Drinks Reception**

After the formal presentations have finished for the afternoon, the learning and networking carries on. The Poster Session allows you to connect with your peers in a relaxed atmosphere and continue to forge new and existing relationships. During this session scientific posters will be presented on the latest advancements in the gene therapy for opthalmic disorders field.

"The update by outstanding speakers demonstrated the tremendous momentum in the field of ophthalmic gene therapy"

Horama

CONFERENCE DAY TWO - THURSDAY, SEPTEMBER 15, 2022

8.50 **Chairs Opening Remarks**

Claire Gelfman
Chief Scientific Officer
Foundation Fighting Blindness

Utilizing Gene Therapy to Restore Protein Function in Common Ophthalmic Disorders to Generate Therapies with a Large Patient Population

9.00 **Exploring Ambient Light Activatable Optogenetics for Vision Restoration in Retinal Degenerative Diseases**

- Mutation-agnostic therapy with potential to be disease-agnostic platform therapy preclinical data
- Clinical data on Retinitis Pigmentosa
- Novel end points for low vision patients deployed in a multi-site randomized, sham-controlled optogenetic study

Samarendra Mohanty
Chief Scientific Officer & President
Nanoscope Therapeutics

9.30 **Showcasing ADVM-022 as a Treatment for Neovascular AMD**

- Wet AMD: the rationale for using gene therapy for treatment
- Utilizing aAV.7m8 vector for intravitreal administration and preclinical and clinical support for dosing
- Clinical experience with ADVM-022 in Wet AMD

Richard Beckman
Chief Medical Officer
Adverum

10.00 **Providing an Update on Antisense Oligonucleotides for Inherited Retinal Disease**

- Antisense oligonucleotides have so much promise in Ph1/2 clinical trials in LCA10 and Usher's Syndrome
- Recent interim analyses in key trails have shed much light on mechanism of action of antisense oligonucleotides
- Overview of the data available from clinical trials
- Clinical trial endpoints for inherited retinal diseases

Aniz Girach
Chief Medical Officer
ProQR

10.30 **Morning Break & Networking**

PRECLINICAL STREAM

Chair: **Gayathri Ramaswamy,** Vice President, Drug Discovery & Disease Biology, **Intergalactic Therapeutics**

Controlling Inflammation & Toxicity to Provide a Safer Treatments for the Eye

11.30 **Building In Vivo Pharmacology For Targeting ABCA4 to the Retina Using a Novel Non-viral DNA/Delivery Approach**

- Discuss Intergalactic's non-viral gene therapy platform for ocular indications
- Discuss strategy for retina in vivo pharmacology studies using large animal species
- Demonstrate delivery of the target gene to the relevant cell type in the retina
- Assess the impact of the delivery method on retinal structure

Cathleen Gonzales, Director of Pharmacology, **Intergalactic Therapeutics**

12.00 **Round-Table Discussion: Discussing the Immunogenicity of the Eye**

- Discussing the implications of treatments delivery to the immuno-privileged eye
- What are the most common causes of inflammation?
- How do we overcome ocular inflammation - is it possible?

Richard Beckman, Chief Medical Officer, **Adverum**

CLINICAL STREAM

Chair: **Claire Gelfman,** Chief Scientific Officer, **Foundation Fighting Blindness**

Benchmarking Ophthalmic Clinical Trial Design to Get your Therapy to Market

11.30 **Identifying the Right Endpoints for your Therapy to Optimize your Clinical Trials**

- Proposing a framework to select disease-specific and stage-specific endpoints including comparison measures
- Reviewing strengths and limitations of visual function tests currently used as outcome measures
- Addressing practical aspects of measuring outcomes to minimize patient burden

Erin O'Neil, Vice President of Clinical Development, **Opus Genetics**

12.00 **Data Solutions for Imaging Biomarker Discovery and Clinical Endpoint Validation**

- Dearth of meaningful endpoints remains hurdle in ophthalmic gene therapy trials
- Biomarkers and endpoints are not born, they are discovered and validated
- Clinical standards of care are insufficient, and must be supported with emerging imaging modalities and analyses

Eric Buckland, Founder & Chief Executive Officer, **Translational Imaging Innovations (TII)**

CONFERENCE DAY TWO - THURSDAY, SEPTEMBER 15, 2022

12.10 **Clinical Trial Development for Interventional Study in Outer Retinal Degenerative Diseases**

- Overview of the unmet need in clinical trial outcomes
- Dark adaptation as a potential outcome for intermediate AMD
- Structural and Functional parameters in X-linked retinoschisis

Catherine Cukras, Diretor, Medical Retina Fellowship Program, **National Eye Institute (NEI)**

1.00 **Lunch Break & Networking**

Discussing the Successes & Learning Opportunities of Recent Therapies to Further Understanding of the Correct Protocols of Ophthalmic Gene Therapy Translation

2.00 **Discussing the Involvement of Immunosuppressants in Therapies to Understand the Benefits & Drawbacks**

- Anti-inflammatory and immunosuppressives as prophylaxis for gene therapy associated inflammation
- Management of treatment emergent gene therapy associated inflammation
- Translational aspects of inflammation to guide immunosuppressive therapy decisions

Nida Sen
Senior Director, Retina Clinical Lead
Janssen

2.30 **Delving Into Lumevoq Gene Therapy in Leber Hereditary Optic Neuropathy**

- LHON, a rare disease with unmet medical need
- Final steps of Lumevoq clinical development
- Importance of early access programs

Remote

Magali Taiel
Chief Medical Officer
GenSight Biologics

3.00 **Understand the Novel Protein Splicing Platform that Overcomes the Payload Capacity of AAV-Vectors**

- Outlining the methodology of protein splicing
- Discovering split inteins: auto processing domains
- Exploring protein splicing as a novel gene therapy modality to the eye

Miquel Vila-Perello
Chief Executive Officer & Chief Scientific Officer
SpliceBio

3.30 **End of Conference Day Two: Chair's Closing Remarks**

Claire Gelfman
Chief Scientific Officer
Foundation Fighting Blindness

"It was a very informative and intellectual discussion on the future of medicine"
Regeneron

POST-CONFERENCE DELIVERY DAY
FRIDAY, SEPTEMBER 16

This day will offer a one-day technology and device centred insight experience to establish the most critical areas of ophthalmic gene therapy research and development. Designed to help established and new companies understand a range of important topics.

This day will provide:

- Insight into the technology used to develop the methods of delivery
- An deeper understanding of subretinal administration for gene therapies to the eye
- Surgical perspective on the key criteria for suprachoroidal delivery
- Exploration of surgical and robot assisted delivery of gene therapy

9.50 **Chairs Opening Remarks**

Viral Kansara
Vice President of Preclinical Development
Clearside Biomedical

Exploring the Technologies & Devices Involved with Suprachoroidal Delivery to Prepare for the Next Generational Delivery Route

10.00 **Recent Updates on Microinjector Based Suprachoroidal Gene Therapy: Potentials & Challenges**

- SCS microinjector based suprachoroidal gene delivery enables minimally invasive office-based treatment for choroiretinal diseases
- Nanoparticle based non-viral gene delivery via SC injection has potential to be a repeatable office-based gene therapy

Viral Kansara
Vice President of Preclinical Development
Clearside Biomedical

10.30 **Novel Suprachoroidal Delivery System Enables Rapid Distribution to the Posterior Segment & Macula**

- Considerations for rapid and broad distribution to posterior segment when delivering gene therapy
- Innovative approach for suprachoroidal delivery using Everads' suprachoroidal delivery system
- Perspectives of a retinal surgeon on key criteria for a suprachoroidal delivery technology

Yoreh Barak
Director of Retina, Department of Ophthalmology
Everads Therapy

11.00 **Morning Break**

Exploring Novel Technology & Devices Involved with Ophthalmic AAV-Mediated Delivery

11.30 **Looking Into Surgical & Robot-assisted Delivery of Gene Therapy Vectors**

- Clinical methods of AAV-mediated delivery
- Gene therapy and optogenetic approaches
- Robot-assisted delivery method

Jasmina Kapetanovic
Clinician, Scientist & Consultant Vitreo-Retinal Surgeon
University of Oxford

12.00 **Exploring the Use of Subretinal Administration for Ophthalmic Gene Therapy**

- Summarizing the use of subretinal and suprachoroidal administration for gene therapy
- Reviewing the current and emerging technologies for subretinal administration
- Device development considerations for subretinal delivery

Diane Doughty
Director of Device Development
REGENXBIO

12.30 **Lunch Break & Networking**

Grab your lunch, join a table and introduce yourself to share your views with your fellow gene therapy experts

1.30 **Panel Discussion: Discussing the Future of AAV-Mediated Delivery**

- Addressing the future of delivery – suprachoroidal, subretinal and robot assisted methods
- Discussing the suitability of subretinal delivery
- How can we make delivery more effective in the future?

Jasmina Kapetanovic
Clinician, Scientist & Consultant Vitreo-Retinal Surgeon
University of Oxford

Viral Kansara
Vice President of Preclinical Development
Clearside Biomedical

Yoreh Barak
Director of Retina, Department of Ophthalmology
Everads Therapy

2.00 **End of Post-Conference Delivery Day**

Viral Kansara
Vice President of Preclinical Development
Clearside Biomedical

WHY PARTNER

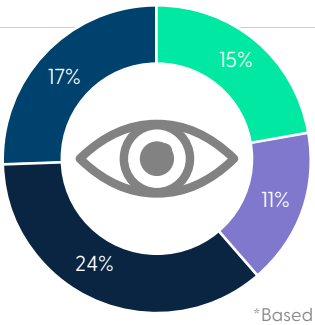
Elevate Your Brand & Have Your Services Showcased in Front of the Leading Experts in the Field of Ophthalmic Gene Therapy

Partnering with the **3rd Annual Gene Therapy for Ophthalmic Disorders** conference is a prime opportunity to demonstrate the availability and reliability the industry is looking for in service providers.

Elevate your brand and show how you can enable drug developers to overcome the barriers preventing their drugs reaching the patients with ophthalmic disorders. This could involve speaking on the agenda, hosting networking sessions or creating exhibitions. **We'll work with you to develop a bespoke partnership** to ensure you meet your business objectives.

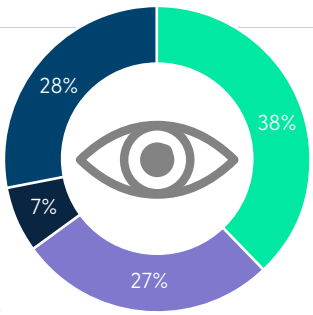
Get in touch today to learn more about how we can help you to create the most valuable connections within the ophthalmic gene therapy industry.

WHO WILL YOU MEET?



AUDIENCE SENIORITY*

- C-Level – 15%
- VP – 11%
- Senior Director/Director – 24%
- Scientist – 17%



ATTENDING COMPANIES*

- Drug Developer 38%
- Healthcare Providers – 27%
- Service Providers - 7%
- Other – 28%

Based on 2021 Gene Therapy for Ophthalmic Disorders attendees

LAST YEAR'S ATTENDING COMPANIES

abbvie, ADVERUM BIOTECHNOLOGIES, agtc, Apellis, astellas, ATSENA THERAPEUTICS, Boehringer Ingelheim, Daiichi-Sankyo, eyevensys, FIGHTING BLINDNESS, GenSight BIOLOGICS, graybug vibrant.vision, GYROSCOPE A Novartis Company, HORAMA, INTERGALACTIC, IVERIC bio, janssen, Johnson & Johnson, KODIAK, KRIYA THERAPEUTICS, MEIRAGTx, NOVARTIS, ProQR, REGENXBIO, Santen, SPARING VISION, Spark THERAPEUTICS, SPLICEBIO, vedere, ViGeneron

GET INVLOVED

Adam Haras-Gummer
Senior Partnership Director
sponsor@hansonwade.com / (+1) 617 455 4188

2022 PARTNERS

FORGE BIOLOGICS
INNOVATION PARTNER

Forge Biologics is a hybrid gene therapy contract manufacturing and therapeutics development company. Forge's mission is to enable access to life changing gene therapies and help bring them from idea into reality. Forge has a 175,000 ft2 facility in Columbus, Ohio, "The Hearth," to serve as its headquarters. The Hearth is the home of a custom-designed cGMP facility dedicated to AAV viral vector manufacturing and will host end-to-end manufacturing services to accelerate gene therapy programs from preclinical through clinical and commercial stage manufacturing. By taking a patients-first approach, Forge aims to accelerate the timelines of these transformative medicines for those who need them the most.

www.forgebiologics.com

NEWCELLS BIOTECH
INNOVATION PARTNER

Newcells Biotech are a fast-growing team of global scientific and industry experts based in the Helix Science Campus of Newcastle-upon-Tyne in the UK. We provide in vitro models to most accurately predict in vivo outcomes, building confidence in the decision making process. Using our expertise in iPSCs, cellular physiology and organoid technology, we have built functional pre-clinical in vitro models of the retina in the form of fully developed retinal organoids and RPE cells.

www.newcellsbiotech.co.uk

EXPERIMENTICA
INNOVATION PARTNER

Experimentica is a global CRO developing and offering novel preclinical ocular models and services to sponsors from Pharma, Biotech, and Academia. We offer an industry-leading portfolio of in vivo, in vitro, and ex vivo PoC models in rodents and larger animals, as well as custom models, PK/PD, and Toxicity ocular studies. Our laboratory sites are located in Finland, Lithuania, and the United States. This enables our team of interdisciplinary scientists to understand and support local and international companies fully.

www.experimentica.com

TRANSLATIONAL IMAGING INNOVATIONS (TII)
INNOVATION PARTNER

Translational Imaging Innovations, Inc. (TII) was formed in 2019 as a spin-out of the Advanced Ocular Imaging Program of the Medical College of Wisconsin to accelerate image-driven innovations in ophthalmology. The TII Integrated Translational Imaging™ platform combines a scalable and extensible database with data automation workflows for biomarker discovery and clinical endpoint validation. Our objective is to dramatically increase access to ophthalmic images and data for empowering breakthroughs in next-generation ocular therapies.

www.tiinnovations.com

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www.ocugen.com

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Our U.S. bi-coastal locations offer an extensive portfolio of preclinical research services from in vitro analyses, early discovery and proof-of-concept to discovery toxicology. Nonclinical capabilities include: pharmacokinetics, lead compound screening, proof of concept, efficacy assessment, pharmacology, mechanism of action, and early lead optimization. Biomere also provides supportive colony maintenance, backup colony support and on site room use agreements.

www.biomere.com

CYAGEN
EXHIBITION PARTNER

Founded in 2006, Cyagen is an innovative CRO enterprise that accelerates the research and development of new drugs with data, algorithms and animal models. Based on animal models and combined with the in-depth exploration of artificial intelligence, Cyagen is at the forefront of the industry in the field of gene editing animal models. Our comprehensive services cover the entire process of model generation: from transgenic/gene targeting strategy design, through model development, breeding, cryopreservation, and phenotype analysis. At the same time, Cyagen has continuously strengthened its advantages in data and models, and proactively deployed in the field of cell and gene therapy. Combining many high-tech platforms, such as target prediction and validation, viral vector development, evaluation model construction and effectiveness evaluation, we have also made significant achievements in this brand new field.

www.cyagen.com

"Very well-organized conference covering gene therapy vectors, preclinical and clinical research and patient experience"

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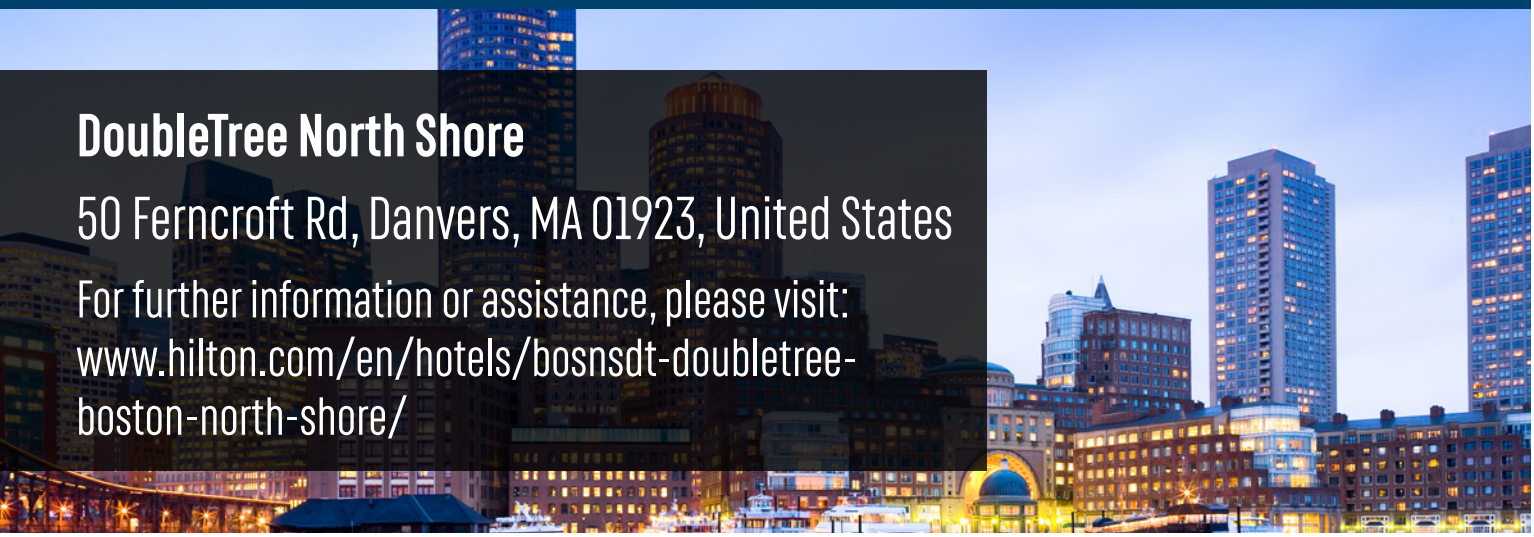

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