

Foundation Fighting Blindness & RD Fund



INVESTING IN CURES

Summit 2021

April 12–17, 2021

FOUNDATION
**FIGHTING
BLINDNESS**

RD FUND

The 2021 Investing in Cures Summit (ICS) convenes key stakeholders of the Foundation Fighting Blindness and RD Fund to learn from the world's leading industry, clinical and retinal science innovators poised to take emerging therapies across the finish line. This week-long, exclusive virtual event will feature discussions about preclinical and clinical assets focused on retinal disease conditions. ICS will also showcase the remarkable advances being made in the development of therapies for retinal degenerative diseases.

FOUNDATION
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RD FUND

Most treatments in the clinical and commercial pipeline you'll hear about were made possible by funding from the Foundation Fighting Blindness, including the RD Fund, our venture philanthropy fund which has more than \$70 million in investments for 10 projects. Overall, we now have more than 40 clinical trials for emerging treatments underway – most of which were made possible by the many researchers and partners you will be learning about through this summit.

We have about 20 sessions over six days which include many leaders and experts in retinal disease. Some highlights include: The Executive Roundtable on April 17 which features conversations with CEOs from Iveric Bio, SparingVision and Atsena Therapeutics. Also on April 17, we have a session titled The Ecosystem, which features a panel of representatives from all sectors of the ocular research and development pipeline.

Throughout the week, on-demand videos will be posted every morning for you to view prior to the live sessions. Live sessions will also be recorded for instant playback.



David Brint
Chairman
Foundation Fighting Blindness



Warren Thaler
Chairman
RD Fund

LIVE EVENT SCHEDULE

Monday April 12 2:15 p.m. ET	<p>Welcome to Investing in Cures Summit David Brint (Chairman, Foundation Fighting Blindness), Warren Thaler (Chairman, RD Fund)</p>
	<p>Patient Journeys: Stories of Hope and Courage</p>
	<p>Evan Mittman Means Business <i>Moderated by: Ben Shaberman (Sr. Director, Scientific Outreach, Foundation Fighting Blindness)</i> Evan Mittman (Board Director, Foundation Fighting Blindness) and Jason Menzo (COO, Foundation Fighting Blindness)</p>
	<p>RP Warriors <i>Moderated by: Ben Shaberman (Sr. Director, Scientific Outreach, Foundation Fighting Blindness)</i> John Corneille, JD (Director, Legacy Giving, Foundation Fighting Blindness) and Michelle Glaze (Associate Director, Professional Outreach, Foundation Fighting Blindness)</p>
	<p>Low Vision, High Ambition <i>Moderated by: Ben Shaberman (Sr. Director, Scientific Outreach, Foundation Fighting Blindness)</i> Denny Moyer, BS, COTA/L (Founding Member and CEO, Ensign Skills for Low Vision Rehabilitation)</p>
Tuesday April 13 2:15 p.m. ET	<p>Using Adeno-Associated Virus as a Tool to Study Retinal Barriers in Disease <i>Moderated by: Chad Jackson, PhD (Director, Preclinical Translational Research Program, Foundation Fighting Blindness)</i> Deniz Dalkara, PhD (INSERM Researcher, Paris Vision Institute)</p>
Wednesday April 14 2:15 p.m. ET	<p>A Virtual-Reality System to Measure Visual Acuity and Contrast Sensitivity in Laboratory Rodents <i>Moderated by: Chad Jackson, PhD (Director, Preclinical Translational Research Program, Foundation Fighting Blindness)</i> Glen Prusky, PhD (Professor, Department of Physiology and Biophysics, Weill Cornell Medicine)</p>
Thursday April 15 2:15 p.m. ET	<p>Developing Cell Therapies for Blinding Eye Diseases: From Bench-to-Bedside <i>Moderated by: Chad Jackson, PhD (Director, Preclinical Translational Research Program, Foundation Fighting Blindness)</i> Khaphil Bharti, PhD (Senior Investigator, NEI, NIH)</p>
Friday April 16 2:30 p.m. ET	<p>Improving IRD Diagnostics: Lab Tour at Blueprint Genetics <i>Moderated by: Todd Durham, MS, PhD (VP, Clinical & Outcomes Research, Foundation Fighting Blindness)</i> Tero-Pekka Alastalo, MD, PhD (Executive Medical Director and Co-Founder, Blueprint Genetics)</p>
Saturday April 17 11:00 a.m. ET	<p>The Art of Translation: Moving Beyond the Bench <i>Moderated by: Amy Laster, PhD (VP, Science & Awards Program, Foundation Fighting Blindness)</i> and <i>Chad Jackson, PhD (Director, Preclinical Translational Research Program, Foundation Fighting Blindness)</i> Ilyas Washington, PhD (Co-Founder, Alkeus Pharmaceuticals), Christelle Monville, PhD (Professor, Evry's University and Team Leader, Istem lab – France), Rob Collin, PhD (Professor, Radboud University Medical Center), and Mark Vézina, BSc (Director of the Ocular and Neuroscience Department, Charles River)</p>
Saturday April 17 12:10 p.m. ET	<p>The Executive Roundtable <i>Moderated by: Rusty Kelley, PhD, MBA (SVP, Investments & Alliances, Foundation Fighting Blindness),</i> and <i>Jason Menzo (COO, Foundation Fighting Blindness)</i> Patrick Ritschel, MBA (CEO, Atsena Therapeutics), Stéphane Boissel (CEO, SparingVision), Walter Strapps, PhD (Chief Scientific Officer, Gemini Therapeutics), and Glenn Sblendorio, MBA (CEO & President, Iveric Bio)</p>
Saturday April 17 1:20 p.m. ET	<p>The Ecosystem <i>Moderated by: Ben Yerxa, PhD (CEO, Foundation Fighting Blindness),</i> and <i>Eugene de Juan, Jr., PhD (Retinal surgeon, inventor, and entrepreneur, RD Fund Board Director)</i> Wiley Chambers, MD (Director, Division of Ophthalmology, Office of Specialty Medicine, Office of New Drugs, Food and Drug Administration), Stéphane Boissel (CEO, SparingVision), Richard Awdeh, MD (CEO and Founder, CheckedUp), Laura Manfre (Co-Founder, Sofia Sees Hope), Kevin Bitterman, PhD (Partner, Atlas Venture), Tarran Pierfelice, PhD, MBA (Business Head of National Gene Therapy Franchise, Resilience), and Steve Pakola, MD (Chief Medical Officer, REGENXBIO)</p>
Saturday April 17 2:25 p.m. ET	<p>Investing in Cures Summit Closing Remarks Adrienne Graves, PhD (Board Director, Foundation Fighting Blindness and RD Fund)</p>

ON-DEMAND SCHEDULE

Tuesday April 13	<p>Ultrahigh Resolution Mouse Optical Coherence Tomography to Aid Intraocular Injection in Retinal Gene Therapy Research Mark C. Butler and Jack M. Sullivan</p>
	<p>Company Showcase: Alkeus Gabrielle DeBartolomeo (Clinical Trial Scientist, Alkeus Pharmaceuticals)</p>
	<p>Company Showcase: Gensight Biologics Magali Tael, MD (Chief Medical Officer, Gensight Biologics)</p>
Wednesday April 14	<p>Optokinetic Response As Structural and Functional Visual System Readouts</p>
	<p>Using Optical Coherence Tomography and Optokinetic Response As Structural and Functional Visual System Readouts in Mice and Rats Dietrich et al., 2019</p>
	<p>Company Showcase: Clearside Biomedical Thomas A. Ciulla, MD, MBA (Chief Medical Officer and Chief Development Officer, Clearside Biomedical)</p>
	<p>Company Showcase: Nacuity Halden Conner (CEO and President, Nacuity Pharmaceuticals)</p>
Thursday April 15	<p>Developing Cell Therapies for Blinding Eye Diseases: From Bench-to-Bedside Khakil Bharti, PhD (Senior Investigator, NEI, NIH)</p>
	<p>What is RPE and how can replacing it help patients with AMD?</p>
	<p>We are NIH</p>
	<p>Company Showcase: Iveric Bio Pravin Dugel, PhD (Executive Vice President, Chief Business and Strategy Officer, Iveric Bio)</p>
	<p>Company Showcase: 4DMT Robert S. Fishman, MD (Chief Medical Officer, 4DMT)</p>

Welcome to Investing in Cures Summit



David Brint *Chairman, Foundation Fighting Blindness*

David Brint is CEO of Brinshore Development Company, where he is responsible for Brinshore's real estate development, acquisitions, development financing, equity syndication, and property management activities. David has arranged over \$1 billion in complex financial transactions utilizing a variety of tools including: tax exempt bonds, Federal HOPE VI, CDBG, Empowerment Zone and HOME funds, mortgage-based and cash-flow loans, credit enhancements, Historic and Low Income Tax Credits, TIF financing, and assorted grant programs. These creative financing tools have been used to leverage public and private resources and have enabled the development of over 4,000 units of successful affordable housing.

David, on behalf of Brinshore Development, is also the Midwest Regional representative and Vice President for the Richman Group Capital Corporation, L.L.C., a national syndicator of low income housing tax credits. He oversees all of the due diligence, market rate analysis, and acquisition closing for the company's Midwest region, which has amounted to over \$300 million since 1994.

David previously served as Board President of the Foundation for Retinal Research, which he co-founded and which has now merged with Foundation Fighting Blindness. He is also a board member of a number of non-profit institutions including Director of the Illinois Housing Council, Director of The Chicago Lighthouse for People Who are Blind and Visually Impaired and Director of Kids Can Do Foundation. He is a former member of the Chicago Federal Home Loan Bank Board Community Investment Advisory Council, and Founding Member and past Director of Highland Park Community Land Trust. He formerly served as Board President of the Victor C. Neumann Foundation, a nonprofit organization dedicated to serving developmentally disabled individuals.

David received a bachelor's degree in economics from the Wharton School at the University of Pennsylvania.

David has been involved with Foundation Fighting Blindness since 1998; his son Alan is affected with LCA. David is actively involved with Foundation Fighting Blindness events in the Chicago area.



Warren Thaler *Chairman, RD Fund*

Warren is the former President of Gund Investment Corporation, where he led investment activities for Gordon Gund. Typical investments included: direct private equity investments, fund investments, and public equities.

Warren is an early investor and serves on the board of several companies including Align Technology, the maker of "Invisalign" (an alternative to traditional wire and bracket orthodontics). Warren served on the board of the Cleveland Cavaliers and Gund Arena Company for 15 years and was the franchise's Alternative Governor on the National Basketball Association's Board of Governors from 2000 until 2005. Other privately-held Gund corporations Warren has had operational and board of director's responsibilities with include: Nationwide Advertising Services (a recruitment advertising agency), JobOptions (an internet job board) and Gus Hotel Management Corporation (an operator of Sheraton hotels in Silicon Valley).

Warren is an early investor and board member in ARdVRk Technologies, Inc. ARdVRk Technologies focuses on developing augmented and virtual reality apps for healthcare, life sciences and medical device companies. In addition, Warren serves on the board of One Focus Vision and Foresight Vision 6, and serves as a trustee of The Baldwin School, a K-12 private girls school in the Philadelphia area.

Prior to joining Gordon Gund in 1990, Warren worked in the Corporate Finance Department of Drexel Burnham Lambert.

Warren has been involved with Foundation Fighting Blindness since 1998. Neither he nor anyone in his family is affected by a retinal disease. Warren is actively involved with Foundation Fighting Blindness events in Philadelphia, San Francisco, Orange County and Boston.

Warren is a graduate of the Harvard Business School, Princeton University and Groton School.



**DEVELOPING TRANSFORMATIVE
THERAPIES FOR RETINAL DISEASES**

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Evan Mittman Means Business

Moderated by: Ben Shaberman (*Senior Director, Scientific Outreach, Foundation Fighting Blindness*)



Evan Mittman

Board Director, Foundation Fighting Blindness

At 19 years old, I was diagnosed with an incurable retinal disease and told I had only one year left to see. I was advised to learn Braille and invest in a seeing-eye dog. Driven to overcome this incredible obstacle, I graduated university using reel-to-reel tapes – as I could no longer read a book – and launched my first company soon after in the fashion industry. I grew the business from zero to over \$150 million in volume, and forty years later sold it to a \$16 billion multinational company.

After selling the business, I wanted to make a positive impact on the lives of young people, so I became a professor of fashion branding and licensing at Marist College and began my involvement with the Foundation Fighting Blindness, where I currently serve on the Board of Directors. Nothing brings me greater joy than to coach the next generation of business leaders, share the things I've learned across my 30-year career, and watch others thrive with my guidance.



Jason Menzo

COO, Foundation Fighting Blindness

Jason Menzo serves as the Chief Operating Officer for the Foundation Fighting Blindness, the world's leading organization searching for treatments and cures of blinding retinal degenerative diseases. The Foundation has raised over \$815 million since its inception and is currently funding more than 90 research projects globally. Jason also serves on the management team for the Retinal Degeneration Fund (RD Fund), the venture arm of the Foundation. The RD Fund has nearly \$100 million under management and has made investments in 10 portfolio companies to date, all aimed at fueling startup companies in the retinal degeneration space.

Prior to joining the Foundation, Jason was a co-founder and Business Unit Head of Sun Ophthalmics. As one of four founding members, he built and led the commercial team, launching the business into the well-known entity it is today.

Prior to his role with Sun Ophthalmics, he was a founding member of the US business for Nicox SA, where he led the commercial launches of several ophthalmic brands and helped the business successfully exit by acquisition (Valeant Pharmaceuticals, 2014). He has previously held positions of increasing responsibility with Bausch + Lomb, Inspire Pharmaceuticals, and Bayer Healthcare.

Jason also serves on the Board of Directors for Retina International, the leading global umbrella organization for patient-led charities and foundations. He has also served as a member for the Global Council of Advisors and Advocates for Sightlife, a global non-profit seeking to end corneal blindness.

Jason is a devoted husband to wife Leanne and loving father to son Gabe, and two daughters Clara and Addie. Jason and Leanne are very active in the autism advocacy and research community.

RP Warriors

Moderated by: Ben Shaberman (*Senior Director, Scientific Outreach, Foundation Fighting Blindness*)



John Corneille, JD

Director, Legacy Giving, Foundation Fighting Blindness

John is the Director of Legacy Giving at the Foundation Fighting Blindness. He was diagnosed at age five with retinitis pigmentosa and his vision has diminished to slightly more than light perception only. He relies 100% on screen reading

software, last drove 22 years ago at age 40 and was an early participant in the ReNeuron stem cell clinical trial at Mass Eye and Ear Infirmary. He is the father of two adult daughters and two granddaughters.



Michelle Glaze

Associate Director, Professional Outreach, Foundation Fighting Blindness

Michelle Glaze is the Associate Director of Professional Outreach for the Foundation Fighting Blindness. Michelle joined the Foundation in February of 2019 after 15 years in medical sales. Her passion for this role with the Foundation stems from personal challenges with vision loss due to retinitis pigmentosa. Michelle also serves on the board for the Alabama Department of Rehabilitation Services, as a representative of the Mobile district.

Michelle received a master's degree in Education of the Deaf, with a minor in mathematics, from California State University,

Fresno in 2000. She went on to teach Algebra at Central West High School for 3 years. After moving to Alabama, she began her career as a medical sales professional in 2004.

Michelle has one son, Parks, who keeps her very busy. Parks enjoys many extracurricular activities including basketball, baseball, and band. When she has spare time, Michelle enjoys exercise and staying active, as well as music and cooking.

Low Vision, High Ambition

Moderated by: **Ben Shaberman** (*Senior Director, Scientific Outreach, Foundation Fighting Blindness*)



Denny Moyer, BS, COTA/L *Founding Member and CEO,* Enight Skills for Low Vision Rehabilitation

Denny Moyer is a founding member and the current CEO of the Enight Skills Center for Low Vision Rehabilitation and the Curtis Strong Center for Visually Impaired in Colorado. After the onset of vision loss at age 19 due to Stargardt's macular degeneration, Denny earned degrees in occupational therapy and healthcare administration and has practiced in low vision rehabilitation since 1999. Currently Denny serves on the committee for the development of the outcomes measurement tool (RSO) and is a committee member for the Aging & Vision Loss National Commission, sub-committee for Data and Research. Denny served as a member of the American

Occupational Therapy Association specialty board for the development of low vision competencies for occupational therapists and was awarded her specialty certification in low vision. Denny served as a board member of the National Accreditation Counsel serving Blindness and Low Vision (NAC), now AER, from 2006-2018 and served on the Older Individuals who are Blind-Technical Assistance Committee (OIB-TAC) from 2019-2020. As an occupational therapy practitioner with extensive education and experience in low vision, Denny has established herself as a nationally recognized professional in the field of low vision.

Lab Experiences and Q&A

Get a quick, in-depth overview of commonly used and talked about research experiments through engaging and easy-to-understand visual demonstrations. Then spend time with a research scientist who will answer questions on any technical aspect of the lab experience or further your understanding on how it applies to retinal diseases.

USING ADENO-ASSOCIATED VIRUS AS A TOOL TO STUDY RETINAL BARRIERS IN DISEASE



Deniz Dalkara, PhD
INSERM Researcher, Paris Vision Institute

Deniz Dalkara is a tenured INSERM researcher and team leader on gene therapies and animal models of neurodegenerative disease at the Paris Vision Institute. She trained as a postdoc at the Max Planck Institute and UC Berkeley. She received

Euretina Science and Medicine Innovation award and was recognized as an Innovator under 35 –by MIT Technology Review. Her current research strongly focuses on translational aspects of retinal gene therapy and optogenetics.

A VIRTUAL-REALITY SYSTEM TO MEASURE VISUAL ACUITY AND CONTRAST SENSITIVITY IN LABORATORY RODENTS



Glen Prusky, PhD
*Professor, Department of Physiology and Biophysics,
Weill Cornell Medicine*

Glen Prusky has been a Professor in the Department of Physiology and Biophysics at Weill Cornell Medicine in New York, New York since 2007. Glen also serves as the Director of the Center for Vision Repair at the Burke Neurological Institute in White Plains, New York, and the Director of Vision Research at Blythedale Children's Hospital in Valhalla, New York. Glen leads a basic research program that focuses on understanding how the visual system develops, how it is shaped by experience, and how abnormal visual experience or injury leads to visual disease and dysfunction, such as amblyopia, retinal degenerative disease, age-related visual decline, and visual

system trauma. Glen also leads a clinical research program that uses a new generation of computerized visual assessments procedures that were developed in the lab to measure useful visual metrics faster and more quantitatively than current procedures. This work has enabled for the first time, the ability to measure vision in brain injured children with limited verbal and cognitive abilities who have Cerebral Visual Impairment. The information gleaned from the basic and clinical research studies has provided insight into how visual disease and dysfunction can be treated using therapeutic visual experience.

Lab Experiences and Q&A *(Continued)*

DEVELOPING CELL THERAPIES FOR BLINDING EYE DISEASES: FROM BENCH-TO-BEDSIDE



Khapil Bharti, PhD
Senior Investigator, NEI, NIH

Dr. Kapil Bharti obtained his PhD from J.W. Goethe University, Frankfurt, Germany, graduating summa cum laude. His PhD work involved research in the areas of heat stress, chaperones, and epigenetics. He did his postdoc at the National Institutes of Health, where he published numerous papers in the areas of transcription regulation, pigment cell biology, and developmental biology of the eye. His lab at the National Eye Institute recently received approval for the first U.S. phase I/IIa trial to test autologous iPSC-derived RPE patch in AMD patients. Currently, he is co-developing a dual RPE/photoreceptor cell therapy with Opsi Therapeutics.

He has given several keynote lectures, won several awards including being the first Earl Stadtman Tenure Track Investigator at NEI, NIH Director's award and NEI Director's Dr. Karl Kupfer Visionary award for his revolutionary work on developing ocular cell-therapies, and serves on the advisory board of several companies and patient-advocacy groups. His current work as a Senior Investigator at NEI involves understanding the mechanism of retinal degenerative diseases using induced pluripotent stem cell derived eye cells and tissues, and developing cell-based and drug-based therapies for such diseases.

IMPROVING IRD DIAGNOSTICS: LAB TOUR AT BLUEPRINT GENETICS



Tero-Pekka Alastalo, PhD
Executive Medical Director and Co-Founder,
Blueprint Genetics

Tero-Pekka Alastalo is the Executive Medical Director, and co-founder of Blueprint Genetics, a Quest Diagnostics company. He is based in San Francisco and holds various responsibilities in the North American operations of

the company. Tero-Pekka has an MD-PhD degree and is specialized in pediatrics. He has a PhD in molecular and cellular biology as well.



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The Art of Translation: Moving Beyond the Bench

This panel will showcase the experiences and lessons learned from researchers who have succeeded in moving their preclinical translational projects toward clinical studies.

Moderated by: Amy Laster, PhD (*VP, Science & Awards Program, Foundation Fighting Blindness*) and **Chad Jackson, PhD** (*Director, Preclinical Translational Research Program, Foundation Fighting Blindness*)



Ilyas Washington, PhD
Co-Founder, Alkeus Pharmaceuticals

Dr. Washington's scientific research has led to the formation of Alkeus Pharmaceuticals and what has been called the most promising treatment for Stargardt disease and possibly age-related macular degeneration. His efforts to better understand vision have resulted in the invention of a new

form of vitamin A to stop retinal degeneration, referred to as C20D3-vitamin A (ALK-001), currently in late-stage clinical trials. Dr. Washington works in a private research laboratory called bi0Org3.14, where he continues to develop novel interventions for serious unmet medical needs.



Christelle Monville, PhD
*Professor, Evry's University
Team Leader, Istem lab (France)*

Christelle Monville obtained a PhD in Neurobiology at the French University of Créteil in 2000. She is currently professor at Evry's University and team leader at the Istem lab (France). For the past ten years, the objectives of her group (Istem, www.istem.eu) are (1) to develop pre-clinical studies required for the development of human pluripotent stem cells cellular therapy for the treatment of a number of monogenic retinal diseases and (2) disease modeling and drug discovery using patient-specific human induced Pluripotent Stem (hiPS). Replacement of defective retinal pigment epithelium (RPE) by new RPE cells derived from human embryonic stem cells

(hESC) provides a novel rational approach for treating forms of blindness that affect the RPE. First attempts in clinical trials demonstrated safety for the delivery of such cells as a suspension. Transplanting a functional monolayer of RPE cells is the next challenge to effectively cure patients. We successfully developed, under clinically compatible conditions, a tissue-engineered product (TEP) consisting of RPE cells derived from hESCs disposed on a biocompatible substrate: the human amniotic membrane. The future will be to provide more complex tissue and upscale the production of retinal cells.

The Art of Translation: Moving Beyond the Bench *(Continued)*



Rob Collin, PhD

Professor, Molecular Therapies for IRDs
Radboud University Medical Center

Rob Collin has recently been appointed Full Professor in Molecular Therapies for Inherited Retinal Diseases at the Department of Human Genetics and the Donders Institute for Brain, Cognition and Behavior, in the Radboud University Medical Center, Nijmegen, The Netherlands. He heads a research team dedicated to find novel molecular therapies for inherited retinal diseases, and is a module leader of the Nijmegen Foundation Fighting Blindness USA Program Project Award entitled 'Splice Modulation to Treat Inherited Retinal Diseases'.

Prof. Collin obtained his PhD degree in 2006 with the thesis entitled 'Exploring the role of the amyloid- β precursor protein APP and its relative APLP2 in *Xenopus* intermediate pituitary'. After his graduation, he continued his academic career at the Radboud University Medical Center in the genetics of inherited sensory diseases, first of hereditary hearing impairment and later of inherited (vitreo)retinal diseases. Between 2006 and 2014, he was primarily involved in the identification of novel genes associated to these diseases, including LHFPL5, ESRRB and LRTOMT (hearing impairment), and EYS, IMPG2, C2orf71, TSPAN12, ZNF408 and MVK (visual impairment).

In 2009, he decided to switch gears and dedicate his career to develop novel molecular therapies to help visually impaired individuals. Supported by an Innovation grant (VENI) from the Netherlands Society for Scientific Research, he visited the lab of Prof. Jean Bennett at the Scheie Eye Institute in Philadelphia, a pioneer in gene therapy for retinal diseases. Back in the Netherlands, he continued to develop the first splicing therapy for a genetic subtype of congenital blindness, i.e. caused by a recurrent mutation in the CEP290 gene. Based on his work,

and a collaboration with ProQR Therapeutics, a clinical trial to assess safety and efficacy of antisense oligonucleotides for the treatment of CEP290-associated LCA is ongoing, with positive interim results reported.

Prof. Collin has published over 120 peer-reviewed manuscripts, including several in high-impact journals such as *The American Journal of Human Genetics*, *Nature Genetics*, *Genome Research*, *PNAS*, *Genetics in Medicine* and *Progress in Retinal Eye Research*. He received funding from several Dutch Blindness foundations, the Dutch Organisation for Scientific Research, the EU and the Foundation Fighting Blindness USA. In addition, he is a board member of the Dutch Society for Gene and Cell Therapy, and recently joined the Scientific Advisory Board of the Foundation Fighting Blindness USA and that of the Bayer Ophthalmic Research Awards. Last year, together with colleagues from Leiden University Medical Center, he founded the Dutch Center for RNA Therapies, a center that is dedicated to ensure 'n-of-1' treatments for patients with ultrarare mutations for which an AON-based therapy cannot undergo 'classical' clinical trial testing.

Since January 1, 2020, Rob became Theme Leader of the Research Theme 'Sensory Disorders' within the Radboud University Medical Center, and as such involved in shaping the future directions of research within the institute. The current work of his research team is dedicated to understand the molecular mechanisms underlying different subtypes of inherited retinal dystrophy, and to directly apply that knowledge for the development of new treatment strategies for the many visually impaired individuals worldwide.

The Art of Translation: Moving Beyond the Bench *(Continued)*



Mark Vézina, BSc

Director, Ocular and Neuroscience Department
Charles River

Mark is the Director of the Ocular and Neuroscience Department at the Charles River facility in Montreal. He has 32 years industry experience including 26 years of ocular pharmacology/toxicology experience. Mark was a co-founder of the Ocular Toxicology Specialty Section (OTSS) of the Society of Toxicology (SOT) and served as president from 2010

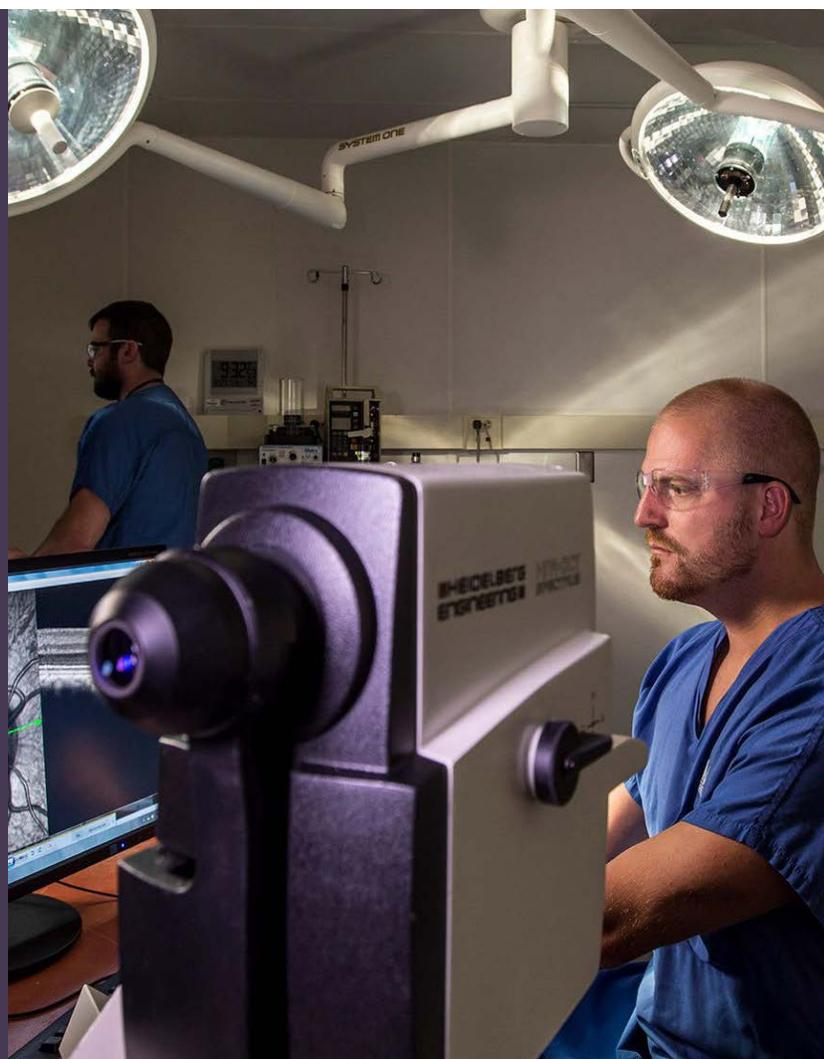
to 2011. He has authored/co-authored numerous peer reviewed articles, scientific posters abstracts and a book chapter, and has lectured at universities, conferences and to industry and regulatory authorities on various topics pertaining to ocular toxicology and pharmacology in North America and Europe.


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The Executive Roundtable

Leading pharma executive panel will delve into the strategic process for advancing promising treatments through the development pipeline.

Moderated by: Rusty Kelley, PhD, MBA (*SVP, Investments & Alliances, Foundation Fighting Blindness*) and **Jason Menzo** (*COO, Foundation Fighting Blindness*)



Patrick Ritschel, BS, MBA
CEO, Atsena Therapeutics

Patrick Ritschel is an experienced executive and entrepreneur with over 25 years of experience in business management, business development and technology licensing. Mr. Ritschel was co-founder and President of StrideBio, a gene therapy company based in Durham, North Carolina, and continues to serve on the StrideBio Board of Directors. Prior to StrideBio, he was an angel investor and played an instrumental role in the formation of gene therapy company Bamboo Therapeutics (acquired by Pfizer in 2016). Mr. Ritschel spent his early career with Monsanto Enviro-Chem Systems, Inc. in St. Louis and Hong Kong. He participated in the management buyout of the

Enviro-Chem subsidiary in 2005, and upon the subsequent sale of the company to DuPont in 2010, he joined DuPont as Director of Business Development.

He is active in the rare disease community and currently serves on the Board of Directors of the Friedreich's Ataxia Research Alliance (FARA).

Mr. Ritschel holds a B.S. in Mechanical Engineering from the University of Illinois Urbana-Champaign, cum laude, and an MBA from Washington University in St. Louis.



Stéphane Boissel
CEO, SparingVision

Stéphane was appointed Chief Executive Officer of SparingVision concurrently to the first closing of the series A financing in August 2020.

He was previously Chairman of the Board of the Company. Prior to joining SparingVision as CEO, Stéphane served as Executive Vice President of Corporate Strategy of Sangamo Therapeutics, based in San Francisco (USA). He joined Sangamo Therapeutics in 2018 on the back of the acquisition by the latter of TxCell SA, a CAR-Treg company he was the CEO of. Stéphane is an experienced biotech professional who brings over 25 years of leadership experience across corporate finance, strategy and business development. Prior to his

appointment as CEO of TxCell in 2015, he served as CEO of Genclis, a molecular diagnostics company. From 2002 to 2010, he served as CFO then Deputy-CEO of Innate Pharma SA, and from 2010 to 2014 he served as Deputy-CEO of Trangene SA. Earlier in his career, Stéphane worked in investment banking for Lazard, where he focused on principal investment in France, Singapore and Hong Kong. He also served as board member or Chairman of the board in different companies in the tech and biotech spaces across different countries.

Mr. Boissel completed his undergraduate work in management and finance at the University of Lyon and Paris-Dauphine in France and received his MBA from the University of Chicago.

The Executive Roundtable *(Continued)*



Walter Strapps, PhD
Chief Scientific Officer, Gemini Therapeutics

Walter Strapps, PhD, is Gemini's Chief Scientific Officer. Prior to that Dr. Strapps was most recently Vice President, Discovery Biology at Intellia Therapeutics where he led internal and external CRISPR/Cas9 therapeutic programs through discovery into IND-enabling studies across a range of tissues and indications.

Prior to that he served as Director, RNAi Therapeutics at Merck & Co., Inc. where he led siRNA identification, lead optimization,

assay development and management of screening automation and helped Merck to integrate and build up its RNAi Therapeutics group after the acquisition of Sirna Therapeutics in 2006. Prior to that acquisition, he was a scientist at Sirna Therapeutics.

Dr. Strapps earned his B.Sc in biology from McGill University and his Ph.D. in genetics and biology from Columbia University.



Glenn Sblendorio, MBA
CEO and President, Iveric Bio

Glenn Sblendorio, MBA, is Chief Executive Officer and President of Iveric Bio. He joined the company in 2016 as Executive Vice President, Chief Operating Officer, and Chief Financial Officer and was appointed President in January 2017 while continuing to serve as Chief Financial Officer. He ceased being CFO in April 2017. Mr. Sblendorio served as a member of the board of directors of Iveric Bio from 2013 to 2016 and rejoined the board in May 2017. Prior to joining Iveric Bio, he served as President and Chief Financial Officer and member of the board of directors of The Medicines Company from 2006 to 2016. Mr. Sblendorio served as Executive Vice President and Chief

Financial Officer of Eyetech Pharmaceuticals, Inc. from 2002 through 2005 when it was sold to OSI Pharmaceuticals. From 2000 through 2002, he held the title of Senior Vice President of Business Development for The Medicines Company. His pharmaceutical experience also includes 12 years at Hoffmann-LaRoche, Inc. in a variety of senior financial positions. Mr. Sblendorio currently serves on the board of directors of Amicus Therapeutics and Intercept Pharmaceuticals. Mr. Sblendorio received his BA from Pace University and an MBA from Fairleigh Dickinson University and is a graduate of the Harvard Business School Advanced Management Program.

The Ecosystem

A panel of representatives from all sectors of the ocular research and development pipeline will discuss leveraging current successes and momentum in biotechnology to advance treatments for retinal degenerations.

Moderated by: Ben Yerxa, PhD (CEO, Foundation Fighting Blindness) and Eugene de Juan, Jr., PhD (Board Director, RD Fund)



Wiley Chambers, MD

Director, Division of Ophthalmology, Office of Specialty Medicine, Office of New Drugs, Food and Drug Administration

Wiley A. Chambers, MD, is the Director of the Division of Ophthalmology, Office of Specialty Medicine, Office of New Drugs, Center for Drug Evaluation and Research at the Food and Drug Administration (FDA). After receiving an undergraduate degree from Colgate University, Dr. Chambers completed medical school and a residency in Ophthalmology at The George Washington University School of Medicine and Health Sciences in Washington, DC. He is currently a Clinical Professor of Ophthalmology and Adjunct Assistant Professor of Computer Medicine at The George Washington University. He joined the FDA in 1987, as a primary reviewer for ophthalmic

drug products and in 1990 became a Supervisory Medical Officer for Ophthalmologic Drug Products. In this capacity, Dr. Chambers has supervisory responsibility for the clinical review of ophthalmologic drug products and ophthalmic therapeutic biologic products submitted to the Center for Drug Evaluation and Research. Additionally, Dr. Chambers is the recipient of numerous Public Health Service, FDA and Center for Drug Evaluation and Research awards for his work with the FDA and he has served as the American Academy of Ophthalmology's Delegate to past United States Pharmacopeia Conventions.



Stéphane Boissel

CEO, SparingVision

Stéphane was appointed Chief Executive Officer of SparingVision concurrently to the first closing of the series A financing in August 2020.

He was previously Chairman of the Board of the Company. Prior to joining SparingVision as CEO, Stéphane served as Executive Vice President of Corporate Strategy of Sangamo Therapeutics, based in San Francisco (USA). He joined Sangamo Therapeutics in 2018 on the back of the acquisition by the latter of TxCell SA, a CAR-Treg company he was the CEO of. Stéphane is an experienced biotech professional who brings over 25 years of leadership experience across corporate finance, strategy and business development. Prior to his

appointment as CEO of TxCell in 2015, he served as CEO of Genclis, a molecular diagnostics company. From 2002 to 2010, he served as CFO then Deputy-CEO of Innate Pharma SA, and from 2010 to 2014 he served as Deputy-CEO of Trangene SA. Earlier in his career, Stéphane worked in investment banking for Lazard, where he focused on principal investment in France, Singapore and Hong Kong. He also served as board member or Chairman of the board in different companies in the tech and biotech spaces across different countries.

Mr. Boissel completed his undergraduate work in management and finance at the University of Lyon and Paris-Dauphine in France and received his MBA from the University of Chicago.

The Ecosystem *(Continued)*



Richard Awdeh, MD
CEO and Founder, CheckedUp

Richard Awdeh, MD, Chief Executive Officer and Founder of CheckedUp, is a dedicated medical technology entrepreneur & highly regarded practicing ophthalmologist who founded CheckedUp in 2012 to revolutionize the patient experience and empower treatment decisions through point of care education.

Dr. Awdeh continues to practice at the Bascom Palmer Eye Institute, as a Cornea-Refractive Surgeon and Professor. He also serves as Chairman of the FDA Dermatologic and Ophthalmic Drugs Advisory Committee. In 2010, he founded a medical technology incubator, Cirle, that develops state of the art medical devices for use in ophthalmic surgeries. The company maintains a robust research and development pipeline, and developed a 3-dimensional surgical navigation system for eye surgery, which has recently been registered with the U.S. FDA.

Dr. Awdeh holds a BBA in Finance and Computer Science from Emory University and an MD from Yale University School of Medicine. He practiced as an Ophthalmologist at Duke University before moving to Miami to begin his practice at Bascom Palmer Eye Institute, the country's #1 Eye Hospital by US News & World Report for 15 years.

Dr. Awdeh serves on the Yale University CBIT Board of Directors and lectures at the Yale University School of Management on Healthcare Innovation and New Venture Creation. He is widely published and lectures around the world on ophthalmic disease and interventions.

In his personal time, Dr. Awdeh enjoys traveling, visiting his twin nephews, and supporting non-profit benefits such as St. Jude's Children's Research Hospital, Seed Global Health, and Amigos For Kids.



Laura Manfre
Co-Founder, Sofia Sees Hope

Laura is the Director of Hybrid Cloud Learning and Development for Hewlett Packard Enterprise (HPE). In addition to her work at Sofia Sees Hope, she also serves as a Director on the board of the Foundation Fighting Blindness. In her local community, she is a member of the Mystic (CT) Lions Club and volunteers with Always Home, an organization dedicated to the prevention of family homelessness. Laura has been recognized in southeastern Connecticut as a Volunteer of the Year, and as a

40 Under 40 award recipient. As a founder of Sofia Sees Hope and with the help of friends and family, Laura has seen Sofia Sees Hope grow from a small backyard fundraiser in 2014, into a thriving global patient advocacy organization. Laura holds a BA from Goucher College in Baltimore, Maryland and an MBA from the University of Connecticut. She lives in Ledyard, CT with her husband, Charles, their three children, Dante, Sofia and Luca, ages 20, 17 and 10.

The Ecosystem *(Continued)*



Kevin Bitterman, PhD

Partner, Atlas Venture

Kevin serves on the boards of Akero Therapeutics (NASDAQ: AKRO), Chroma Medicine, Kinaset Therapeutics and Remix Therapeutics. He is a co-founder and board member of Disc Medicine and is chair of the board of Vedere Bio II. He was the founding CEO of Editas Medicine (NASDAQ: EDIT), Morphic Therapeutic (NASDAQ: MORF) and Visterra (acquired by Otsuka), and co-founded Genocea Biosciences (NASDAQ: GNCA). He previously served as a director of Kala Pharmaceuticals (NASDAQ: KALA), Neuronetics (NASDAQ: STIM), Taris Biomedical (acquired by Johnson & Johnson) and Vedere Bio (acquired by Novartis) among other ventures. Prior to joining Atlas Venture in 2017, Kevin was a

partner at Polaris Partners, where he had been a member of the healthcare team since 2004.

Kevin is active in the life science and healthcare start-up community serving as Chair Emeritus of the New England Venture Capital Association (NEVCA) and as a member of the Harvard Medical School Blavatnik Biomedical Accelerator, Blavatnik Therapeutics Challenge Awards and Q-FASTR Steering Committees. He received a BA in biology, summa cum laude, from Rutgers University before completing his PhD in genetics at Harvard Medical School.



Tarran Pierfelice, PhD, MBA

Business Head of National Gene Therapy Franchise, Resilience

Tarran Pierfelice, PhD, MBA is a stem cell biologist by training who is currently the Business Head of National Resilience's Gene Therapy Franchise where her team is developing transformative technologies and platforms to improve how gene therapies are manufactured. Dr. Pierfelice has >10 years of gene therapy manufacturing experience spanning business development and commercial operation roles both at CDMOs and cell and gene therapy innovator companies. She was the Director of Business Development at Paragon Bioservices, an industry-leading gene therapy CDMO, where she was heavily

involved in developing the company's commercial strategy to drive organic growth to position the company for its sale to Catalent. After completing her MBA, Dr. Pierfelice worked at Editas Medicine leading its cell therapy pipeline and CMC strategy business development efforts where she worked on several collaborative partnerships to advance the company's pipeline to clinic. Dr. Pierfelice received a BS in Molecular Biology from Florida Institute of Technology, a PhD in Cellular and Molecular Medicine from Johns Hopkins University School of Medicine, and MBA from Harvard Business School.

The Ecosystem *(Continued)*



Steve Pakola, MD
Chief Medical Officer, REGENXBIO

Steve Pakola, MD is the Chief Medical Officer for REGENXBIO. Dr. Pakola brings more than 20 years of biopharmaceutical experience, including holding key leadership roles in preclinical and clinical development, regulatory affairs and medical affairs. Dr. Pakola's therapeutic area experience encompasses retinal disorders, including diabetic retinopathy, diabetic macular edema and age-related macular degeneration, as well as other non-ophthalmic disease areas. He was the lead inventor and program lead for the Jetrea® (ocriplasmin) program, from inception in 2002 through the therapy's United States and European regulatory submissions in 2012.

Prior to Aerpio, Dr. Pakola served as Chief Medical Officer at Amakem NV and ThromboGenics Inc. Earlier in his career, Dr. Pakola served as Associate Director of Cardiovascular Clinical Research at Boehringer Ingelheim, where he was Global Medical Lead on the Lipid-Lowering Development Program and the USA Medical Lead for the Direct Thrombin Inhibitor Dabigatran Program. Prior to that, Dr. Pakola served in senior-level clinical development positions at Quintiles Cardiovascular Therapeutics and at Organon.

Dr. Pakola earned a BA, summa cum laude, and an MD from the University of Pennsylvania, where he was elected to the Alpha Omega Alpha Honor Medical Society.

Investing in Cures Summit Closing Remarks



Adrienne L. Graves, PhD
Board Director, Foundation Fighting Blindness and RD Fund

Dr. Graves is a visual scientist by training and a global industry leader in ophthalmology. She served as President and CEO of Santen Inc. from 1995 to 2010, where she successfully established a strong global presence, brought multiple ophthalmic products through development to approval and commercialization and led global teams through successful acquisitions and partnerships. Prior to her fifteen years at Santen, she spent nine years at Alcon, where she established their first Retinal Electrophysiology Laboratory; she later directed clinical development in multiple therapeutic areas, and served as Director of International Ophthalmology.

Dr. Graves currently serves as an independent director on the boards of Iveric Bio, Oxurion, Greenbrook TMS, Nicox, Qlaris Bio, TherOptix, and Surface Ophthalmics. Dr. Graves also serves on the boards of directors of American Society of Cataract and Refractive Surgery (ASCRS) Foundation, Glaucoma Research Foundation, Retina Global, Himalayan Cataract Project, Foundation Fighting Blindness, and the RD (Retinal Degeneration) Fund. Dr. Graves received an AB with Honors in psychology from Brown University, a PhD in psychobiology/neuroscience from the University of Michigan, and completed a postdoctoral fellowship in visual neuroscience at the University of Paris.

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Iveric Bio is an innovative biopharmaceutical company focused on the discovery and development of treatments for retinal diseases with significant unmet medical needs.

ivericbio.com

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REGENXBIO Inc. is a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. Our gene therapy product candidates are designed to deliver functional genes to address genetic defects in cells, enabling the production of therapeutic proteins or antibodies that are intended to impact disease.

regenxbio.com



At Biogen, we are pioneering new science that takes us deep into the body's nervous system, and stretches wide across digital networks and patient communities, to better understand, and preserve, the underlying qualities of our essential human nature.

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Charles River has the extensive historical control data, advanced surgical techniques and equipment, and skilled scientific staff you need to complete successful ophthalmology programs.

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