

# FIGHTING BLINDNESS

## FUTURE CHALLENGES AND OPPORTUNITIES FOR VISUAL RESTORATION

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# FIGHTING BLINDNESS

## FUTURE CHALLENGES AND OPPORTUNITIES FOR VISUAL RESTORATION

September 6<sup>th</sup> and 7<sup>th</sup>, 2016

## WELCOME

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Dear Speakers and Participants,

It is our pleasure to welcome you for the meeting "Fighting Blindness. Future Challenges and Opportunities for Visual Restoration". This event is possible thanks to the great support of B-DEBATE, an initiative of Biocat and Obra Social "la Caixa", and the will of 3 research institutions, Barcelona Macula Foundation (BMF), Center of Regulation Genomic (CRG) and LEITAT Foundation.

Blindness is a major global health challenge because of the enormous impact it has on patients and their families, and their important socio-economic consequences. Besides the great emotional impact that this implies for patients and their families, also means the inability to pursue education, to find a quality job, an increased risk for falls and accidents, a limited autonomy and an impediment to have an active lifestyle and quality.

Over the coming years, 1 of every 4 people over 50 years-old will suffer some kind of degenerative eye disease. Currently in the EU there are 91 million people over 65 years and is expected to reach 127 million by 2020. Worldwide it is estimated that there are 285 million people with impaired vision and 39 million are blind.

These debates aim to propose ideas and explore the potential of new therapeutic approaches for retinal dystrophies, combining nanotechnology, regenerative medicine, stem cells, gene therapy, genomics, bioengineering, ophthogenetics and photonics. This meeting is the manifestation of this strategic commitment and the willingness to bring together international experts in the field. There will be basic and translational researchers, leaders and experts in all these fields, in order to share innovative therapeutic opportunities in each sector and especially in the exploration and discovery of possible synergies to generate new multidisciplinary collaborative research projects on this top. The invited talks and open debates will stimulate the discussion and will provide thoughts for future areas of research.

We thank you in advanced for your input and encourage you to actively participate in the discussions. We wish you a fruitful meeting over the next two days.

Yours sincerely,

Jordi Monés (BMF), CRG, Leitit Foundation and B-DEBATE (BIOCAT and "la Caixa Foundation")

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# PROGRAM

Tuesday, September 6<sup>th</sup>, 2016

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**9:00 Welcome**

**Jordi Portabella**, Director, Area of Research and Knowledge, la Caixa Foundation

**Jordi Fàbrega**, Director of Business Development, Biocat

**Jordi Monés**, Director, Barcelona Macula Foundation: Research for Vision and Institut de la Màcula

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**9:20 SESSION 1: MOLECULAR PATHOLOGY AND BIOMARKERS IN AGE-RELATED MACULAR DEGENERATION (AMD)**

Chairs: **Marco Zarbin**, New Jersey Medical School, New Jersey, USA

**Jayakrishna Ambati**, University of Virginia, Virginia, USA

**Macular degeneration. Where we are in clinical practice**

**Jordi Monés**, BMF, Barcelona, Spain

**Anti PDGF therapy for exudative AMD: addressing the fibrosis pathway**

**Samir Patel**, Ophthotech, New York, USA

**Markers and Mechanisms in Maculopathies: a systems view**

**Marius Ueffing**, Institute for Ophthalmic Research, Tübingen, Germany

**Towards a systems-oriented view of the molecular pathology of AMD**

**Christina Kiel**, CRG, Barcelona, Spain

**Cellular response associated to retinal diseases and therapeutics approaches**

**Nicolás Cuenca**, Alicante University, Alicante, Spain

**KEYNOTE SPEAKER: A molecular road to a therapy for Dry AMD**

**Jayakrishna Ambati**, University of Virginia, Virginia, USA

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**11:15 Open debate**

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**11:35 Coffee Break**

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**12:00 SESSION 2: NANOTECHNOLOGY**

Chairs: **Jose Luis Pedraz**, Universidad del País Vasco, Vitoria, Spain

**Joan Parra**, Fundació LEITAT, Barcelona, Spain

**Medical Nanochemistry, the use of reactive inorganic nanoparticles in medicine**

**Victor Puentes**, ICN2, Barcelona, Spain

**Non-viral gene delivery for the treatment of inherited retinal disorders**

**Gustavo Puras Ochoa**, University of the Basque Country, Vitoria, Spain

**Retinal degenerations: Animal models and new therapeutical approaches**

**Gema Martínez Navarrete**, Miguel Hernandez University, Elche, Spain

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**12:50 Open debate**

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**13:10 Poster session & discussion**

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**13:40 Lunch**

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**14:40 SESSION 3: GENETICS AND GENE THERAPY**

Chair: **Eduardo Fernández**, Miguel Hernández University, Alicante, Spain

**Genetics of AMD: Current status**

**Caroline Klaver**, Erasmus University, Rotterdam, Netherlands

**Role of Mitochondrial Genetics in AMD**

**Baruch Kupperman**, University of California, California, USA

**Gene therapy in retinal degenerative diseases, looking for therapeutic targets for dry AMD**

**Francisco Díaz Corrales**, CABIMER, Sevilla, Spain

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**15:40 Open debate**

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**16:00 SESSION 4: PHOTONICS AND IMAGING**

Chair: **Jordi Monés**, BMF, Barcelona, Spain

**Samir Patel**, Ophthotech, New York, USA

**The use of advanced microscopy techniques for ophthalmological applications**

**Pablo Loza**, ICFO, Barcelona, Spain

**High resolution retinal imaging: the case of LITE project**

**David Merino**, ICFO, Barcelona, Spain

**From taking a PEEK to wide-field imaging: how imaging best serves translational research?**

**Tunde Peto**, Moorfields Eye Hospital NHS Foundation Trust, UK

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**16:45 Open debate**

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**17:00 Coffe break**

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**17:30 SESSION 5: OPTOGENETICS AND ARTIFICIAL VISION**

Chair: **Nicolas Cuenca**, Alicante University, Alicante, Spain

**Marius Ueffing**, Institute for Ophthalmic Research, Tübingen , Germany

**Visual neuroprosthesis: Current status and future prospects**

**Eduardo Fernández**, Miguel Hernández University, Alicante, Spain

**KEYNOTE SPEAKER: OPTOGENETICS**

**Marco Zarbin**, New Jersey Medical School, New Jersey, USA

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**18:25 Open debate**

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# Wednesday, September 6<sup>th</sup>, 2016

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## 8:30 **SESSION 6: REGENERATIVE THERAPY. STEM CELLS**

Chairs: **Anna Velga**, CMRB, Barcelona, Spain

### **Advanced cell therapies for AMD**

**Berta de la Cerda Haynes**, CABIMER, Sevilla, Spain

### **Pluripotent stem cells for retinal disease, research and clinical application**

**Anna Veiga**, CMRB, Barcelona, Spain

### **Use of induced pluripotent stem cells and direct lineage reprogramming in cell therapy for eye regenerative medicine**

**Ana Belén Alvarez**, UB, Barcelona, Spain

### **Improving the quality and characterisation of stem cell reprogramming and analysing the genomic stability and immune response**

**Michael Edel**, UB, Barcelona, Spain

### **Adult progenitor stem cells: applications for cell-based therapy in degenerative retinal diseases**

**Ricardo Casaroli**, Hospital Clínic of Barcelona, Barcelona, Spain

### **Retinal regeneration via cell fusion mediated reprogramming**

**Pia Cosma**, CRG, Barcelona, Spain

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10:30 **Open debate**

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10:50 **Poster session and coffee break**

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## 11:30 **SESSION 7: ROUND TABLE. FROM BENCH TO BEDSIDE**

Chair: **Joan Parra**, Fundació LEITAT, Barcelona, Spain

### **Market Driven Technology Transfer**

**Xavier Rubies**, IBEC, Barcelona, Spain

### **Innovative schemes though demand-driven procurement for procuring early detection services: the case of the PRO4VIP**

**Ramon Maspons**, AQUAS, Generalitat de Catalunya, Barcelona, Spain

### **Technology transfer**

**Joan Parra**, Fundació LEITAT, Barcelona, Spain

### **KEYNOTE SPEAKER: HIV brought us to Microbiome study**

**Bonaventura Clotet**, IrsiCaixa, Badalona, Spain

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12:45 **Open discussion**

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## 13:10 **Concluding remarks**

Chairs: **Joan Parra**, Fundació LEITAT, Barcelona, Spain

**Jordi Monés**, BMF, Barcelona, Spain

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# SCIENTIFIC COMMITTEE



**Jordi Monés**, Director of **Barcelona Macula Foundation: Research for Vision** and **Institut de la Màcula**, Barcelona, Spain.

Dr. Jordi Monés, M.D., Ph.D., is an Ophthalmologist, Macula and Vitreoretinal Specialist and Researcher. He is the Director, and one of the founder governors of the Barcelona Macula Foundation: Research for Vision. He is also the Founder and Director of the Institut de la Màcula since 2007. Dr. Monés earned his Medicine and Surgery degree and his Ph.D. cum laude in Medicine and Surgery at the Universitat de Barcelona. He specialized in Ophthalmology at the Centro de Oftalmología Barraquer (UAB). He obtained his Retina Specialist degree at Massachusetts Eye and Ear Infirmary at Harvard Medical School, and at the Hospital San José at the Monterrey Institute of Technology and Higher Education. For the last 20 years, Dr. Monés has been a researcher and principal investigator in most international multicenter clinical trials for the treatment of AMD. He is currently participating in 15 clinical trials and in 4 major European Union projects performed by several agencies from different countries. Dr. Monés is a member of the steering committees of the Brighter and Crystal trials, as well as the Proxima trial and is on the advisory boards of many pharmaceutical companies. He is an active member of various scientific advisory societies, including the Macula Society, the Retina Society, the Club Jules Gonin, the American Society of Retina Specialists, AAO, ARVO and EURETINA. Dr. Monés' research interests include macular diseases, choroidal neovascularization, macular degeneration, anti-angiogenic therapy, anti-PDGF therapy, geographic atrophy, retinal degeneration, retinal transplant, stem cells, gene therapy, macular edema, and vitreoretinal and macular surgery. Member of the Board of Directors of Futbol Club Barcelona and, since 2010, has been responsible for the Medical and Sports Science Department. He is also the Commissioner of the FCB Universitas project.

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**Joan Parra Farre**, General Manager **Leitat Technological Center**, Barcelona, Spain.

Dr. Joan Parra, PhD in Chemistry from the University of Barcelona (1997) and a specialist in the field of Biochemistry. He also holds a Postgraduate in Business Management from the Faculty of Business at the University of Barcelona and has completed the Executive Development Program at IESE. In his professional career as a researcher in the field of Biochemistry, he had developed several projects in the Physiological Sciences and Human Nutrition Department (Faculty of Medicine, University of Barcelona) and the Clínic Foundation for Biomedical Research. International stays are also highlighted in the School of Biology at the University of Konstanz (Germany). He has extensive experience in both, scientific and technological fields management, and since 2000 is the General Manager of LEITAT Technology Center. LEITAT is dedicated to applied research with a great international recognition participating in more than 200 national and international projects per year with a deep social and industrial impact.

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# SCIENTIFIC COMMITTEE



**Luis Serrano**, Director of **Centre for Genomic Regulation (CRG)**, Barcelona, Spain.

Luis Serrano obtained a PhD in Biochemistry from the Universidad Autónoma de Madrid (1985). He received his PhD in Cell Biology at the Centro de Biología Molecular (CSIC-UAM) of the Universidad Autónoma de Madrid in 1987 and the University of Cambridge in 1991. He was Lecturer and Senior Lecturer at the Spanish National Research Council (CSIC), in Madrid, from 1988 to 1992. In 1993 he was appointed Head of Group at the European Laboratory of Molecular Biology (EMBL), in Heidelberg, Germany, and focused his work on protein folding and design. In the following years at the EMBL, he was named Senior Principal Investigator, in 2003, and Head of the Structural & Computational Biology programme, in 2006, the time he began to work on Systems Biology. In 2006 he was also named Head of programme at the Spanish National Cancer Research Centre (CNIO). In addition, in 1999, he was appointed research professor at the CSIC. Since the end of 2006, he directs the Systems Biology programme at the Centre for Genomic Regulation (CRG), where he also served as deputy director until his appointment as director in mid 2011. Since 2006 he has been an ICREA (Catalan Institution for Research and Advanced Studies) Research Professor. Currently, his research group focuses on synthetic biology, the engineering and design of biological systems.

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# INVITED SPEAKERS

Tuesday, September 6<sup>th</sup>, 2016

## Session 1: Molecular pathology and biomarkers in age-related macular degeneration (AMD)

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**Jayakrishna Ambati**, Professor and Vice Chair for Research, Ophthalmology; Director, Center for Advanced Vision Science, **University of Virginia**, Virginia, USA.

Dr. Ambati was trained as an electrical engineer at Johns Hopkins University, graduated with M.D. (magna cum laude) from SUNY Health Science Center at Brooklyn, completed ophthalmology residency at University of Rochester, and retina fellowships at Massachusetts Eye and Ear Infirmary (Harvard Medical School). His laboratory reported numerous seminal advances in Nature, Science, Cell, Nature Medicine, New England Journal of Medicine, Journal of Clinical Investigation, PNAS, and eLife. He pioneered the concept that AMD is an inflammatory disease that is driven by perturbations in innate immunity. His foundational contributions have led to fundamental conceptual advances that are on the cusp of improving the diagnosis and treatment of this disease.

He is a member of the National Academy of Inventors, and was the first ophthalmologist to win the NIH Director's Pioneer Award, Doris Duke Distinguished Clinical Scientist Award, Burroughs Wellcome Fund Clinical Scientist Award in Translational Research, Ellison Foundation Senior Scholar in Aging Award, and Harrington Discovery Institute Scholar-Innovator Award. He is a Fellow of the American Association for the Advancement of Science, American Society for Clinical Investigation and was the first ophthalmologist to be elected to Association of American Physicians. He won the ARVO Cogan Award and Carl Camras Translational Research Award, Roger Johnson Memorial Award for Macular Degeneration Research, Prix Soubrane, Junius Kuhnt Medal, and Chanchlani Global Vision Research Award. RPB awarded him its Senior Scientific Investigator Award, Wasserman Merit Award, and Physician-Scientist Award. He serves on the Editorial Boards of IOVS and Ophthalmic Research, is an Associate Editor of Ophthalmology and of TVST.

### A molecular road to a therapy for Dry AMD

Geographic atrophy (GA), a late stage of dry age-related macular degeneration (AMD), affects millions worldwide and has no treatment at present. Degeneration and death of the retinal pigmented epithelium is an important clinical and pathological hallmark of GA. Numerous molecular stressors accumulate in the locus of diseased RPE cells in GA. We identified the NLRP3 inflammasome as an integrator of the cellular response to these molecular stressors. We also observed that nucleoside analogs can be repurposed as inflammasome inhibitors that prevent RPE cell death in multiple mouse models of GA. We have also created new chemical derivatives of nucleoside analogs that shed their toxicity while preserving their efficacy.

Chair of the **SESSION 1**

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**Marco Attilio Eugenio Zarbin**, Professor and Chair at **Institute of Ophthalmology and Visual Science New Jersey Medical School**, **Rutgers University**, New Jersey, USA.

Dr. Zarbin graduated from the Johns Hopkins University School of Medicine (Medical Scientist Training Program,  $\alpha\theta\alpha$ ). Dr. Zarbin completed resident and fellowship training (vitreoretinal surgery and medical retina) at the Wilmer Ophthalmological Institute and was an Assistant Chief of Service at Wilmer. Dr. Zarbin is Chair of the Institute of Ophthalmology and Visual Science, Rutgers-New Jersey Medical School and Chief of Ophthalmology at University Hospital in Newark, N.J. He is a Professor of Ophthalmology and Neuroscience and is the Alfonse A. Cinotti, MD/Lions Eye Research Chair. Dr. Zarbin is co-Director of the Ocular Cell Transplantation Laboratory. Dr. Zarbin has published 207 peer-reviewed papers and editorials, 99 book chapters, 190 abstracts, one book on age-related macular degeneration, one book on stem cell therapy for degenerative retinal disease, and one book on the management of diabetic retinopathy. Dr. Zarbin is a NEI-NIH-funded investigator. Dr. Zarbin is a Vice Chair of the Scientific Advisory Board of the Foundation Fighting Blindness, Editor-in-Chief of Translational Vision Science and Technology, and an ex officio member of the National Advisory Eye Council. He is also a member of the American Ophthalmological Society, Academia Ophthalmologica Internationalis, the Retina Society, the Macula Society, the Gonin Society, and the ASRS.

Chair of the **SESSION 1**

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**Nicolás Cuenca**, Full professor at **University of Alicante**. Alicante, Spain.

Dr. Nicolás Cuenca is Full Professor of the Department of Physiology, Genetics and Microbiology at Alicante University, Spain. He obtained a degree in Biological Sciences from the University of Valencia. After predoctoral stays at the Universities of Harvard 1986 (USA) and Sheffield (UK) 1986, he earned a PhD from the University of Alicante in 1988. He developed his work as a postdoctoral researcher at the University of Utah (USA) during two years (1988-1990) in the laboratory of Dr. Helga Kolb at the University of Utah (USA).

His research is dedicated to the understanding of the functional organization of the mammalian retina. He is studying the disruption of the retinal architecture caused by degenerative diseases including blood vessels and glial cells. His interest is focused on the disease mechanisms and therapeutic treatments, for retinal degenerative diseases using animal models. Publications - 88 publications in PubMed registered journals, cumulative citations 1735, h-index 25. Principal Investigator of 25 projects of public agencies, 8 projects of private foundations, 2 collaborative projects with the industry. Associate editor of the *Frontiers in Neuroanatomy*. Editorial board of several Journals. Member of National and International evaluation committees. Reviewer of 20 international Journals. 25 National and international awards.

### **Cellular response associated to retinal diseases and therapeutics approaches**

Retinal tissue is the target of physical, chemical or biological insults that induce morphological and functional responses in the different retinal cells. An inflammatory response, oxidative stress and activation of apoptotic pathways are common features in all retinal diseases. As a consequence, apoptosis and morphological changes in retinal neurons and synaptic circuitries take place, leading to retinal remodeling and vision loss. Thus, the retinal changes underlying different diseases may modify the transmission of the information between cells and, as a consequence, the retina can undergo a marked remodeling. Retinal glial cells are responsible for maintaining the homeostasis of the retinal extracellular microenvironment, thus ensuring proper functioning of the healthy retina. Glial cells, including astrocytes, Müller cells and microglia, play an important role because their response to injury is decisive for maintaining the health of the retina or its degeneration. Several therapeutic approaches have been developed to preserve retinal function or restore eyesight in pathological conditions. Neuroprotective compounds, gene therapy, cell transplantation or artificial devices should be applied at the appropriate stage of retinal degeneration to obtain successful results.



**Christina Kiel**, Staff Scientist EMBL/CRG Systems Biology Research Unit at the **Centre for Genomic Regulation (CRG)**, Barcelona, Spain.

Christina Kiel studied Biochemistry at the Ruhr-University Bochum and obtained her PhD degree in the Structural Biology department of the Max-Planck-Institute for Molecular Physiology in 2003. She moved for her postdoctoral studies to the Department of Structural and Computational Biology at EMBL Heidelberg. Currently she is Staff Scientist in the Systems Biology Department at the Center for Genomic Regulation in Barcelona in the laboratory of Luis Serrano. Her major research area is the

quantitative, systems and structural analysis of signaling pathways and protein interaction networks relevant in human diseases.

### **Towards a systems-oriented view of the molecular pathology of AMD**

Retinopathies come in different flavours. As simple monogenic diseases they arise from rare genetic variations. As complex diseases, such as age-related macular degeneration (AMD), they develop as a result of a combination of multiple risks: genetic, environmental and lifestyle-related. Here, we contrast the disease mechanisms and underlying networks of monogenic forms of retinopathies with complex forms, using AMD as an example. We show that despite their mutual retinal disease manifestation, risk genes associated to different forms of the disease in general do not overlap and have disparate functions. Further, AMD risk genes are less retina-specific expressed, participate in fragmented networks with multiple interaction partners, and in addition to specific cellular functions the tissue integrity is perturbed. We propose that to understand AMD an integrated systems level analysis combining networks with processes and (patho) physiology is essential. In future, this knowledge may provide improved and additional treatment options that target multiple pathways and processes with the final aim of restoring tissue homeostasis.

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**Jordi Monés**, Director of **Barcelona Macula Foundation: Research for Vision and Institut de la Màcula**, Barcelona, Spain.

(See his CV at the Scientific Committee section)

### **Macular degeneration. Where we are in clinical practice**

Despite recent advances in treatment of the wet form of AMD, the clinical outcome of AMD often remains blindness. Nowadays, the great challenge to which the ophthalmological community is committed lies in finding a treatment that may slow the relentless progression of the atrophic form of the disease, together with other approaches that restore or regenerate the involved part of the diseased retinal tissue. Nowadays, the advanced form of dry AMD with geographic atrophy (GA) may be the first cause of legal blindness among elderly patients and it represents up to a third of cases of late AMD. The predicted increase in life expectancy may be accompanied by a greater prevalence of GA, especially in the older age group, resulting in a real epidemic burden in developed countries. Enormous progress has been made in the field of etiopathogenesis of AMD. However, it is still an extremely complex disease, with several poorly understood phenotypes with different rates and mechanisms of progression and with many unresolved questions. Although extensive research and evidence of the role of oxidative stress, inflammation and genetics on the pathogenesis of AMD has been provided, there is still no current treatment for GA forms.

Several approaches are under investigation to prevent progression of dry AMD or for the early stages of GA. Small topical or oral new molecules to modulate the visual cycle (Emixxustat HCL, Acucela), the choroidal blood flow (MC-1101, Macuclear), or exploring existing drugs (Doxycycline, metformin) are under investigation in phases 2/3 to prevent dry AMD and GA progression. Targeting the complement system is another approach and several attempts by different compounds have been made. Aptamer anti C5 (Zimura, Ophthotech), antibody and C5 (LFG 316, Novartis) or inhibition of complement C3 (APL-2, Apellis Pharmaceuticals) are under Phase II. Lampalizumab, a monoclonal antibody (Genentech) inhibiting Factor D has already shown benefit in patients with Factor I positive in ameliorating the progress of the GA, and currently is under Phase III clinical Trials, the Chroma and Spectry Studies.

Other approaches to remove drusen and subretinal deposits to explore if GA progression can be modified have been recently published by using high doses of statins. Revisiting old therapies such as laser to remove drusen or to stimulate RPE cellular function are also under investigation ( Lumithera, Ellex, Iridex ).

However, for advanced GA and severe visual loss, therapeutical approaches that attempt to restore vision are needed, such as the regenerative therapy by subretinal transplantation of stem cells.



**Samir Patel**, Co-founder, President and Vice Chairman at **Ophthotech Corporation**, New York, USA.

Previously, Dr. Patel was the co-founder of Eyetech Pharmaceuticals, Inc., which developed the first approved anti-VEGF agent for age-related macular degeneration (AMD). At Eyetech, he served as the chief medical officer and served on its Board of Directors.

Dr. Patel has in-licensed and developed multiple new molecular entities for the treatment of AMD. As a co-founder, he has led private biotech financings totaling over \$200 million, four public offerings with proceeds totaling of over \$300 million and two of the largest ex-US big pharma collaborations, with Pfizer and Novartis.

Previously, Dr. Patel spent over a decade in academic medicine, serving as the director of the Retina Service and the residency program at the University of Chicago, Department of Ophthalmology and Visual Science. His area of academic interest focused on cell-based therapies for age-related macular degeneration and was amongst the first to perform human retinal transplant. Dr. Patel has been a consultant to a number of ophthalmic biotechnology companies and healthcare venture firms. In addition to Ophthotech and Eyetech, he has served on the board of directors of AFER-ARVO, EyeGate Pharma and Mimetogen.

Dr. Patel received his medical degree from the University of Massachusetts Medical School and ophthalmology training from the University of Chicago. He received his training in retinal surgery from the Massachusetts Eye and Ear Infirmary at Harvard Medical School.

## Anti PDGF therapy for exudative AMD: addressing the fibrosis pathway

Visual outcome in neovascular AMD (NAMd) patients receiving mono therapy anti-VEGF has been remarkable compared to the era of laser therapy. However most patients do not achieve significant vision gain and on average, patients do not experience visual improvement over the longer term in the “real world” setting. Limitation related to visual benefit is often associated with an increase in neovascular lesion size, neurosensory or RPE atrophy and/or fibrosis.

Pathological neovascularization resulting from underlying macular disease has the characteristics of a wound healing response. PDGF is a known mediator of wound healing. The interplay of various cellular and inflammatory components orchestrating the wound healing response offer potential targets to enhance the short and/or long term visual outcome. Various associated molecular mechanisms will be discussed.



**Marius Ueffing**, Director at the Institute for Ophthalmic Research Co-Chair, Centre for Ophthalmology, Tuebingen, Germany.

Marius Ueffing is the Director of the Institute for Ophthalmic Research and Co-Chair of the Centre for Ophthalmology at the University Medical Center in Tuebingen, one of the largest centers for ophthalmology in Europe. He has a long-standing history in ophthalmic research as well as a background in medical and molecular genetics. His previous positions included that of a Research Associate at Columbia University, College of Physicians and Surgeons, New York, followed by group leader positions in pharmaceutical industry at Goedecke-Parke-Davis and at the University Medical Centre Munich, where he coordinated a clinical cooperation group for ophthalmology jointly with the LMU Eye-Hospital in Munich. Before and jointly with his appointment to Tübingen, he was Director of the Independent Research Unit/Division of Protein Science at the National Research Centre for Environment and Health in Munich. He has served on multiple boards including the German federal ministry's Steering Committee for Systems Medicine and the Board of Directors of the Human Proteome Organization, HUPO ([www.hupo.org](http://www.hupo.org)). Together with Ronald Roepman from Nijmegen, NL he has coordinated the 7thFW Project SYSCILIA on ciliopathies ([www.syscilia.org](http://www.syscilia.org)), and with Caroline Klaver from Erasmus University Medical Centre in Rotterdam, he currently coordinates the EU Horizon2020 Project EYE-RISK ([www.eyerisk.eu](http://www.eyerisk.eu)).

## Markers and Mechanisms in Maculopathies: a systems view

Maculopathies include complex common diseases like AMD as well as mendelian monogenic retinal degenerations. Although prevalence, onset, inheritance, and clinical manifestation may differ substantially among macular diseases, overlapping functional protein networks as well as shared clinical features suggest, that familial and sporadic maculopathies share pathological mechanisms. On the level of pathways, pathological markers and mechanisms, this likely also integrates non-genetic (environmental) risk factors contributing to their etiology. Based on the function and network connectivity of proteins and metabolites implicated in these maculopathies, three major areas can be outlined: lipid metabolism, the extracellular matrix and the complement system. All three areas, primarily defined by genetic association or linkage analysis likely compose a disease network with distinct but interconnected molecular areas. The concept of overlapping disease phenotypes based on overlapping physiological networks and etiologies bears potential to define directions of future research, advance our understanding of the pathomechanisms and help to pinpoint candidate targets for future rational therapy.

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## Session 2: Nanotechnology

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**Joan Parra Farre**, General Manager **Leitatz Technological Center**, Barcelona, Spain.

(See his CV at the Scientific Committee section)

Chair of the **SESSION 2**

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**Jose Luis Pedraz**, Professor at Faculty of Pharmacy and principal investigator of NanoBioCel group at **University of the Basque Country and Nanobiocel**, Vitoria, Spain.

José Luis Pedraz is the director of NanoBioCel group from the Pharmacy and Pharmaceutical Science's Department at the University of the Basque Country (UPV/EHU). It has been recognized as a University of the Basque Country's "Consolidated group" by the Basque Government. This group is part of the Biomedical Research Networking Center in Bioengineering, Biomaterial and Nanomedicine (CIBER BBN); this is a Spanish consortia to promote excellency in research. Also he is the Scientific Director of the Drug Formulation Unit of NANBIOSIS, one of the 29 ICTS (Singular Scientific Technological Infrastructures) in Spain. This infrastructure provides high technological resources and high qualified personnel and also offers services to the scientific community.

Professor Jose Luis Pedraz has an extensive research, academic and management background. He has directed 23 doctoral thesis, he has presented more than 298 papers in national and international congresses, he has developed 10 patents in the area of lipid nanoparticles, particles for vaccines, gene therapy and drug delivery and he has licensed one patent in formulation of semi-solid mucoadhesive. He has published 299 articles in high impact journals and 20 books and book articles. Eighty-six projects have been financed by national and international institutions: European Union Research and Innovation programme (H2020), Ministry of Economy and Competitiveness of Spain, Basque Government and The University of the Basque Country. He co-founded, in 1997, and nowadays runs the Unit of Pharmaceutical Development (UPD), which is a section of TECNALIA (the biggest research & innovation company in Spain). The main goal of the UPD Unit is the research and development of new pharmaceutical products according with the standards of the good laboratory, manufacturing and clinical practices (GLP, GMP and GCP respectively). He is a scientific consultant for different pharmaceutical labs: Praxis, ROVI and Normon, among others.

Chair of the **SESSION 2**

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**Gema Martínez Navarrete**, Postdoctoral Research at **CIBER BBN**, Elche, Spain.

Dr. Martínez-Navarrete received her Ph.D. in Biology from the University of Alicante, Spain. Her research has been focused on retina, investigating the cellular organization and neurogenesis of the vertebrate retina, especially in primates, by various bio-imaging technologies, including light microscopy, electron microscopy (SEM and TEM), laser scanning confocal imaging and molecular biology.

Her work includes cellular and molecular changes of hereditary eye disorders, particularly age-related macular degeneration, retinitis pigmentosa, glaucoma and Stargardt disease. She is currently working on both in vitro and in vivo retinal gene delivery and gene transfection for optogenetic applications, especially vision restoration, in Unit of Visual Rehabilitation and Neuroprosthesis (CIBER BBN-NanoBioCel Group, Institute of Bioengineering, University of Miguel Hernandez, Spain). Further, her research is supported through many communications to national and international conferences and different publications in scientific journals articles.

### **Retinal degenerations: Animal models and new therapeutical approaches**

Animal models of retinal degenerations are useful tools to study therapeutic approaches for patients affected by retinal dystrophies. We will introduce several animal models that develop retinal diseases with similar traits to humans. These models can be used to improve our understanding of the pathogenesis of retinal diseases and provide useful ways for testing new nanoparticle based technologies. While subretinal administration is able to transfect mainly photoreceptors and retinal pigment epithelial cells, intravitreal injection usually produces a more uniform distribution of the nanoparticles through the inner layers of the retina. We will highlight the flattering properties of non-viral vectors for efficient and safe delivery of genetic material to the retina.

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**Victor Franco Puentes**, ICREA Research Professor at **Vall d'Hebron Institut de Recerca (VHIR)** and **Catalan Institute of Nanoscience and Nanotechnology**, Barcelona, Spain.

Prof. Dr. Victor Puentes is ICREA Research Professor and Group Leader at the Catalan Institute of Nanoscience and Nanotechnology (ICN2) (2005), with joint appointments in Vall Hebron Institute de Recerca (VHIR) (2015). He received his Ph.D. degree in Physics from the University of Barcelona in 1998 and his M.S. degree in chemical engineering from University Louis Pasteur (Strasbourg) in 1994.

Between 2000 and 2004 he held a postdoc position as postdoctoral researcher at the University of California–Berkeley (UCB) and the Lawrence Berkeley National Laboratory (LBNL), in the groups of Prof. Paul Alivisatos and Prof. Kannan Krishnan, where he focused his research in the study of magnetic materials, especially in the synthesis of Co NCs. In 2003 obtained a Ramón y Cajal research position at the University of Barcelona. In 2005, he established the Inorganic Nanoparticles Group (ICN2) which focusses its research on the synthesis, functionalization and characterization of complex inorganic nanocrystals with applicability in the fields of energy harvesting, catalysis, biomedicine and nanotoxicology.

#### **Medical Nanochemistry, the use of reactive inorganic nanoparticles in medicine.**

Chemical processes performed or promoted by inorganic nanoparticles in physiological conditions allows to monitor and manipulate biological states, from its catalytic properties, as ROS quenchers CeO<sub>2</sub> NPs, to coordination chemistry, for the pH controlled release of selected drugs or the use the aggregate state of a compound in the form of a NP which progressively dissolves or corrodes, as a carrier of itself, as curcumin or iron oxide nanoparticles. This chemical approach will contribute to the development of tools for medicine and consequently nanomedicine.



**Gustavo Puras Ochoa**, Research Professor at **University of the Basque Country**, Vitoria-Gasteiz, Spain.

Gustavo Puras Ochoa obtained his PhD degree from the Pharmacology Department in the University of the Basque Country (Spain, June, 2002). He worked for the Lloyds Pharmacy company in the United Kingdom of Great Britain as European Pharmacist until June 2005.

In September of 2005, Dr Puras was hired as a Researcher-Assistant member in the University of Texas-Brownsville (UTB, EEUU), under the supervision of Dr Luis V Colom. In this period of time he was a faculty member of the Universidad Autónoma de Tamaulipas (MatamorOs, México). In September 2009, Dr Puras was hired as a researcher for the CIBER-BBN in the group directed by Dr José Luis Pedraz. Since March 2012, Dr Puras works as a faculty member in the Laboratory of Pharmacy and Pharmaceutical Technology of the University of the Basque Country. His research interest is focused mainly on ocular pharmacology, nanotechnology and drug delivery-systems.

#### **Non-viral gene delivery systems for the treatment of inherited retinal disorders**

Many devastating blinding disorders that affect the retina in the developed world have a well-known genetic background. Despite gene therapy strategies have made major advances in recent years, many of the patients affected by inherited retinal diseases must live under impaired vision, even with the best medical treatment. Therefore, the development of effective gene carriers represents a major challenge for the scientific community. At present, viral and non-viral vectors are the most employed approaches to deliver genetic material to the retina. Although first promising clinical trials results with viral vectors offer reasonable hope to patients affected by some inherited diseases that cause irreversible blindness such as Retinitis Pigmentosa, Stargardt's disease, Choroideremia and Age related Macular Degeneration, important concerns related to the risk of oncogenesis, immunogenicity, inflammatory responses, and the persistence of viral vectors in brain after intravitreal injection have garnered the interest to invest on non-viral gene transfer methods. Compared with their counterparts, non-viral vectors offer many important advantages, since are less limited by the size of the gene to transfect, do not raise major safety concerns, are easier and cheaper to produce, and are classified as drugs rather than as biologist by the regulatory authorities.

## Session 3: Genetics and gene therapy

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**Eduardo Fernández**, Full Professor at University Miguel Hernandez and CIBER BBN Elche, Spain.

Eduardo Fernandez is Professor of Cellular Biology and Director of the Visual Rehabilitation and Neuroprosthesis Unit at the Bioengineering Institute of the University Miguel Hernandez (Spain) and of the Neural Engineering group of CIBER-BBN. He received a M.D. degree from the University of Alicante (1986) and a Ph.D. in Neuroscience in 1990. He has been visiting professor at the University of Utah (USA), University of Oldenburg (Germany), Beth Israel Medical Deaconess Center (USA) and University of Vienna (Austria). His research interests is in developing solutions to the problems raised by interfacing the human nervous system and on this basis develop a two-way direct communication with neurons and ensembles of neurons. He is actively working on the development of neuroprostheses and brain-machine interfaces and coordinating the CORTIVIS Project which tries to demonstrate the feasibility of an intracortical neuroprostheses, interfaced with the occipital cortex, as a means through which a limited but useful sense of vision could be restored to profoundly blind. He is also working on brain plasticity and reorganization in severe vision loss and developing non-invasive methodologies for the selection of appropriate candidates for the implantation of a cortical visual neuroprosthesis.

Chair of the **SESSION 3**

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**Francisco J. Díaz Corrales**, Senior Researcher at Andalusian Molecular Biology and Regenerative Medicine Centre (CABIMER), Seville, Spain.

**EDUCATION:** Medical Doctor, Los Andes University, Venezuela, 1997. Neurologist Specialist, Los Andes University, 2002. PhD in Neurosciences, Okayama University, Okayama, Japan, 2006. Clinical Fellowship in Movement Disorders, Virgen del Rocio Hospital, Seville, Spain, 2008. **RESEARCH EXPERIENCE:** Research Assistant, Los Andes University, Pathology Department, 1997-1999. Clinical Research Assistant, Virgen del Rocio Hospital, Neurology Department, 2006-2008. Research Associate, Andalusian Molecular Biology and Regenerative Medicine Centre (CABIMER), Seville, Spain, 2008-2015. Senior Researcher, CABIMER, Cell Therapy and Regenerative Medicine Department, 2015-. **RELEVANT PUBLICATIONS:** 1) Span poly-L-arginine nanoparticles are efficient non-viral vectors for PRPF31 gene delivery. *Nanomedicine*, (in press). 2) pEPito-driven PEDF Expression Ameliorates Diabetic Retinopathy Hallmarks. *Hum Gene Ther Methods*. 2016. 3) Transplantation of melanocytes obtained from the skin ameliorates apomorphine-induced abnormal behavior in rodent hemi-parkinsonian models. *PLoS One*. 2013. 4) Hypoxia increases the yield of photoreceptors differentiating from mouse embryonic stem cells and improves the modeling of retinogenesis in vitro. *Stem Cells*. 2013. 5) Study of cerebello-thalamocortical pathway by transcranial magnetic stimulation in Parkinson's disease. *Brain Stimul*. 2013. 6) ATR localizes to the photoreceptor connecting cilium and deficiency leads to severe photoreceptor degeneration in mice. *Hum Mol Genet*. 2013. 7) Centrosomal aggregates and Golgi fragmentation disrupt vesicular trafficking of DAT. *Neurobiol Aging*. 2012. 8) Sensory perception changes induced by transcranial magnetic stimulation over the primary somatosensory cortex in Parkinson's disease. *Mov Disord*. 2011. 9) Neuroprotective effects of zonisamide target astrocyte. *Ann Neurol*. 2010.

### **Gene therapy in retinal degenerative diseases, looking for therapeutic targets for dry AMD.**

The results from the first clinical trials of gene therapy for Leber's congenital amaurosis, an early-onset childhood retinal dystrophy, have demonstrated the safety and efficacy of RPE65 gene augmentation using adeno-associated viral (AAV) vectors delivered into the subretinal space. These trials coupled with other results obtained from preclinical studies using animal models have provided a proof-of-principle that gene therapy works to treat monogenic retinal dystrophies, and they have established the framework to test this advanced therapy in other complex retinal diseases including age-related macular degeneration (AMD). Gene therapy clinical trials have been already conducted for wet AMD. One of this clinical trials uses lentivirus to over-express two anti-blood vessel proteins called angiostatin and endostatin. Another gene therapy approach uses subretinal and intravitreal injection of AAV vectors carrying the sFLT gene, a vascular endothelial growth factor blocker. For dry AMD few studies have been performed; however, there are several molecular pathways that could be modulated through gene therapy to produce retina protection based on anti-inflammatory, anti-oxidant, anti-protein or lipid aggregation and anti-apoptotic effects. Small molecule libraries could be useful to identify new therapeutic targets for dry AMD to design further gene therapy approaches.

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**Caroline Klaver, MD** Professor of epidemiology and genetics of eye diseases at **Erasmus Medical**, Rotterdam, The Netherlands.

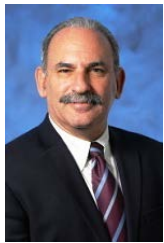
Caroline Klaver is professor of epidemiology and genetics of eye diseases at Erasmus MC Rotterdam. She is a retinal specialist who trained as a fellow at University of Iowa and at Columbia University and at Vitreous-Retina-Macula Consultants in New York and currently has her clinic at Radboud UMC, Nijmegen. Her research focuses on genetic-epidemiologic studies of various complex (myopia, age-related macular degeneration (AMD), glaucoma) and Mendelian eye disorders (retinal dystrophies). She is the principal investigator of ophthalmologic studies in 7 large epidemiologic cohorts from

Rotterdam. Recently, her interest has shifted towards functional studies as her ultimate ambition is to find targets for intervention and diminish patient load.

### Genetics of AMD: Current status

Genome wide association studies have identified various genes and loci associated with AMD. Currently, it is estimated that half of the genetic variants are explained. Stratifying patients by their AMD genetic profile using a state-of-the-art DNA chip will facilitate therapy decision making. Genetic studies have not only enabled researchers to understand the pathogenic mechanisms underlying AMD, but also provided therapeutic targets to prevent the disease in the future. This lecture will give an overview of the current status of AMD genetics.

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**Baruch Kupperman,** Professor and Chief Retina Service, Vice Chair for Clinical Research and of Academic Affairs, **Gavin Herbert Eye Institute-University of California**, Irvine, USA.

Baruch D. Kupperman, MD, PhD, is Professor and Chief, Retina Service, and Vice Chair for Clinical Research and of Academic Affairs at the Gavin Herbert Eye Institute in the Department of Ophthalmology at the University of California, Irvine, where he also holds a joint appointment with the Department of Biomedical Engineering. Dr Kupperman completed his PhD in neuroscience at the California Institute of Technology and earned his medical degree at the University of Miami in Florida. He completed his internship at Los Angeles County/University of Southern California Medical Center and residency in ophthalmology at the University of Southern California Doheny Eye Institute in Los Angeles. In addition, he completed fellowships in retina and vitreous at St Joseph Medical Center in Baltimore, Maryland and at the University of California, San Diego.

Dr Kupperman has extensive research collaborations with pharmaceutical and biotechnology companies, serving as a consultant and member of the scientific advisory board for a variety of companies. He also serves on the editorial boards of *Retina*, *Retina Today*, *Brazilian Archives of Ophthalmology*, and *Brazilian Journal of Ophthalmology*, and has been a peer reviewer for the *American Journal of Ophthalmology*, *Archives of Ophthalmology*, *Investigative Ophthalmology and Visual Science*, *Ophthalmology*, *Retina*, *Survey of Ophthalmology*, *Journal of Infectious Diseases*, and *Lancet*.

### Role of Mitochondrial Genetics in AMD

Mitochondria (mt) are unique organelles with circular, double-stranded DNA containing 16,569 nucleotide pairs that can be classified into haplogroups representing different geographic origins of populations. Maternally inherited mitochondria (mt) DNA encodes for 37 genes critical for oxidative phosphorylation. Most recently, it has been reported that biologically active mitochondrial-derived peptides (MDPs) are encoded from the 16s and 12s rRNA of the mtDNA.

Each mitochondrion contains 1 to 10 copies of mtDNA that are in close proximity to reactive oxygen species production and therefore, are often damaged. Studies show that advanced stages of AMD have been associated with (a) mtDNA haplogroups that confer either increased risk or protection; (b) higher levels of mtDNA lesions/fragments and (c) structural abnormalities of the mitochondria.

AMD has been associated with inflammation, oxidative stress, specific ApoE allele profiles and amyloid- $\beta$  deposits, along with risk factors including smoking, obesity, elevated cholesterol, hypertension and aging. In these diseases, mitochondrial dysfunction, retrograde signaling, and epigenetic abnormalities altering nuclear gene expression also contribute to their pathogenesis. Therefore, intense interest has developed to understand the underlying mechanisms of these intricate mitochondrial-nuclear interactions.

In the past, the variability of nuclear genes amongst individuals has made it difficult to clearly understand the effects of mtDNA upon cell behavior. However, we can now reliably characterize the retrograde signaling from the mitochondria to the nuclear genomes using transmitochondrial cybrids, which are cell lines with identical nuclei, but the mtDNA from different subjects. In this study, cybrids were prepared by fusing platelets from AMD and age-matched Normal subjects with Rho0 (lacking mtDNA) human ARPE-19 cells. Quantitative PCR and Western blotting were performed to examine gene and protein expression profiles, respectively, of complement and apoptosis markers in these cybrids. There were significant decreases in the gene and protein expression of complement inhibitors, along with significantly higher levels for complement activators and pro-apoptosis markers in AMD cybrids compared to Older-Normal cybrids. These events are consistent with levels of higher inflammation and cell death as seen in AMD retinas.



In summary, since all cybrids had identical nuclei and differed only in mtDNA content, the observed changes in components of complement and apoptosis pathways can be attributed to mtDNA variations in the AMD subjects, suggesting that mitochondrial genome and retrograde signaling play critical roles in this disease. Therefore, the mitochondria have become a target to a new field of drug development.

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## Session 4: Photonics and imaging

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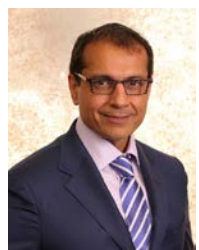


**Jordi Monés**, Director of **Barcelona Macula Foundation: Research for Vision and Institut de la Màcula**, Barcelona, Spain.

(See his CV at the Scientific Committee section)

Chair of the **SESSION 4**

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**Samir Patel**, Co-founder, President and Vice Chairman at **Ophthotech Corporation**, New York, USA.

(See his CV at Session 1)

Chair of the **SESSION 4**

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**Pablo Loza-Alvarez**, Head of the Super resolution light microscopy and nanoscopy facility at **ICFO- The institute of Photonic Sciences Catstelledels**, Barcelona, Spain.

Pablo Loza-Alvarez, received his PhD in Laser Physics from the University of St Andrews, Scotland in 2000. Currently he is a Group Leader at ICFO, where he is also responsible for the "Super Resolution Light Microscopy and Nanoscopy" facility. Dr. Loza-Alvarez has now a strong experience in microscopy and, by introducing novel photonics tools, has developed a number of novel imaging techniques. These have been used for a wide variety of applications ranging from the imaging of large

model organisms to the visualization of subcellular molecular components.

Dr Loza has directed 6 PhD students, co-authored over 150 publications in international journals and conferences (h=24) and has written 6 patents.

He has participated as Program committee and subcommittee member for CLEO/Europe-IQEC and SPIE (Photonics West, USA) and others. Dr. Loza has participated and directed several European and national projects and been involved in main EU networks of excellence and research infrastructures.

### **The use of advanced microscopy techniques for ophthalmological applications**

Advanced microscopy imaging techniques and related tools are now used for several applications in biomedicine. Some of these techniques include confocal, nonlinear (two-photon excited fluorescence (TPEF) and second-harmonic generation (SHG)), Raman and super resolution microscopy (Stimulated emission depletion (STED)). Using these techniques, I will show our efforts to obtain structural and functional information from excised corneas from both in vivo and in vitro retina samples. Finally, I will present our preliminary attempts to selectively modify and create highly localised structures in crystalline lenses using focused ultrashort pulsed lasers.

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**David Merino**, Research Fellow at ICFO-The Institute of Photonic Sciences, Castelldefels, Spain.

David Merino obtained his PhD in the National University of Ireland, Galway, where he was involved in the development of an Adaptive Optics (AO) retinal imaging system based on simultaneous Scanning Laser Ophthalmoscopy (SLO) and Optical Coherence Tomography (OCT). Using this system, he was able to report one of the first observations of photoreceptor cells using OCT.

He has also worked in the design and implementation of a new generation Adaptive Optics enhanced Scanning Laser Ophthalmoscope (AOSLO) system in a collaboration among the University of California, Berkeley and the University of California, San Francisco. As a result of this collaboration, he was able to report the observation and characterization of rod photoreceptors in healthy subjects and in patients, by means of AOSLO.

David is a research fellow in the Superresolution Light Nanoscopy facility at ICFO since 2011, where he is involved in the development of different microscopy techniques (such as second harmonic generation, light sheet microscopy or superresolution techniques) for different biomedical applications. In particular, he is using AOSLO to study patients with degenerative retinal diseases in collaboration with the Institut de la Macula.

### High resolution retinal imaging: the case of LITE project

Adaptive Optics (AO) retinal imaging is becoming a very useful tool to study the retina at a cellular level. The aberrations of the patient eye are corrected during the process of image acquisition, transforming the human eye into a perfect optical system with the best quality. Using this technique, different layers of the retina can be observed at an unprecedented resolution, and the photoreceptor mosaic is revealed using a safe non-invasive technique.

In the LITE project, we are using AO in combination with Scanning Laser Ophthalmoscopy (SLO) to observe the retina of patients affected by different retinal degenerative diseases, such as Stargardt's disease, Retinitis Pigmentosa and Age Related Macular Degeneration.

The technique allows us to study the same areas of the retina at different time points, and to identify the same photoreceptor cells. The images acquired using AOSLO will provide new information about the degenerative processes that affect the patients.



**Tunde Peto**, Head of the Super resolution light microscopy and nanoscopy facility at ICFO- The institute of Photonic Sciences Castelldefels, Barcelona, Spain.

Dr Tunde Peto, MD, PhD is the Head of the Image Reading Centre at NIHR Biomedical Research Centre at Moorfields Eye Hospital NHS Foundation Trust and UCL Institute of Ophthalmology. She is incoming Professor of Ophthalmology at Queen's University Belfast, Northern Ireland. Before joining Moorfields in 2001 Dr Peto completed her MD and Master of Health Education in Szeged, Hungary, lectured at the Albert Szent-Györgyi Medical University, Hungary and accomplished her PhD at the University of Newcastle, Australia. Dr Peto is currently serving as the Treasurer for EASDec, a study group of the

European Association for the Study of Diabetes (EASD) with special responsibility for the study of the eye complications of diabetes. Dr Peto is Head of the Reading Centre Committee for European Network of Clinical Research in Ophthalmology (EVICR) and Ophthalmology Section Board Member for the Royal Society of Medicine. Dr Peto's special interest is the epidemiology of common blinding disorders and how imaging might be able to help with prediction of sight loss and determining disease processes.

### From taking a PEEK to wide-field imaging: how imaging best serves translational research?

Ophthalmic imaging is an integral part of everyday clinical work, clinical trials as well as research. It has gone through rapid expansion since its inception and there is a wide array of imaging options available to use today. This presentation will cover some of the relevant historical events in ophthalmic imaging and then will focus on the contribution of new imaging modalities to our understanding of health and disease in the eye. There are several new imaging modalities that are becoming routine in the clinic. Adaptive optics allow detailed examination of individual photoreceptors and their surroundings with high resolution. Wide-field imaging includes the peripheral retina, expanding our view, albeit with lower resolution. Optical coherence tomography allows for detailed analysis of the retinal layers and vessel structures, without physical sectioning of the eye. All of these are transforming our thinking about health and diseases of the retina. However, it is imperative to understand the limitations of these modalities, the correct use of the equipment and how to interpret relevant changes as these will determine what clinical or research conclusions we draw. With the deepening understanding and the ever increasing range of imaging options eye imaging is now being used to monitor changes associated with brain degeneration, kidney and other systemic diseases, suggesting that eye imaging has the potential to become biomarker for many diseases. With the availability of imaging that can be utilised in under-served areas, this is becoming an even greater challenge and opportunity in places like rural Africa or space missions.

## Session 5: Optogenetics and artificial vision

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**Nicolás Cuenca**, Full professor at **University of Alicante**. Alicante, Spain.

(See his CV at Session 1)

Chair of the **SESSION 5**

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**Marius Ueffing**, Director at the **Institute for Ophthalmic Research** Co-Chair, Centre for **Ophthalmology**, Tuebingen, Germany.

(See his CV at Session 1)

Chair of the **SESSION 5**

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**Eduardo Fernández**, Full professor at **University Miguel Hernandez and CIBER BBN** Elche, Spain.

(See his CV at Session 3)

### **Visual neuroprosthesis: Current status and future prospects**

After more than 40 years of research, visual prostheses are moving from the laboratory into the clinic. We will review the effects of electrical stimulation of visual areas and provide an overview of different approaches for the developing of visual prosthesis for the blind, including optogenetics and optopharmacology. Furthermore we will introduce preliminary results of the feasibility of a cortical prosthesis, interfaced with the visual cortex, as a means through which a limited but useful visual sense may be restored to profoundly blind people. This approach could be the only treatment for blindness caused by glaucoma, optic atrophy or diseases of the central visual pathways, such as brain injuries or stroke.

We will emphasize the role of neural plasticity in order to achieve the desired behavioural outcome and introduce a new bioinspired retina able to work in real-time. Finally we will discuss some of the exciting opportunities and challenges that lie in this intersection of neuroscience research, biomedical engineering and neuro-ophthalmology.

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**Marco Attilio Eugenio Zarbin**, Professor and Chair at **Institute of Ophthalmology and Visual Science New Jersey Medical School**, New Jersey, USA.

(See his CV at Session 1)

## Optogenetics

Optogenetics involves genetically altering neurons to express light-sensitive ion channels (vs. electrodes) to make them light sensitive. This approach may serve as an alternative to the retinal prosthesis for ganglion, bipolar, & residual cone cell stimulation and can take advantage of signal amplification inherent in retinal cellular and synaptic architecture. Ontogenetic therapy involves minimally invasive surgery (intravitreal/subretinal injection) and has the potential for minimally invasive neuronal stimulation with high spatial resolution.

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# Wednesday, September 7<sup>th</sup>, 2016

## Session 6: Regenerative therapy. Stem cells

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**Anna Veiga Lluch**, Director of Barcelona Stem Cell Bank at **Centre de Medicina Regenerativa de Barcelona**, Barcelona, Spain

Graduated in Biology (UAB) (1979)

PhD in Biology. Universitat Autònoma de Barcelona (UAB) (1991)

Scientific Director. Servei de Medicina de la Reproducció, Departament d'Obstetrícia i Ginecologia l'Hospital Universitari Quirón-Dexeus. Barcelona (2005- )

Director. Banc de línies Cel·lular .Centre de Medicina Regenerativa de Barcelona (CMR[B]) (2005- )

Associate professor of Departament de Ciències Experimentals i de la Vida, Universitat Pompeu Fabra (UPF) (2002- ). President. European Society for Human Reproduction and Embryology (ESHRE) (2011- 2013).

### Pluripotent stem cells for retinal disease, research and clinical application

Pluripotent stem cells (PSC) such as Embryonic Stem Cells (ESC) and induced pluripotent stem cells (iPSC) have become an invaluable and unlimited source of cells for research and clinical application. The PSC ability to differentiate into all cell types, including retinal pigment epithelial (RPE) cells, make them suitable to treat retinal diseases (RD) by cell replacement.

We report the results of RPE differentiation from PSC and further transplantation in the Royal College of Surgeons (RCS) rat model, a good model for the treatment of RD.

Differentiation protocols towards RPE cells are well established and PSC derived RPE resemble mature RPE in terms of morphology and gene expression profiles. After transplantation, both hESC- and hiPSC-derived cells maintained the expression of specific RPE markers, lost their proliferative capacity, established tight junctions, and were able to perform phagocytosis of photoreceptor outer segments. Grafted areas showed increased numbers of photoreceptor nuclei and outer segment disk membranes. Transplanted cells protected retina from cell apoptosis, glial stress and accumulation of autofluorescence, and responded better to light stimuli. hESC- and hiPSC-derived cells survived, migrated, integrated, and functioned as RPE in the RCS rat retina, indicating the possible benefit of such therapy for RD.

Clinical trials performed with hESC have shown promising results. Japanese scientists are currently setting safety standards for iPS cell transplants in patients with degenerative eye diseases.

Chair of the **SESSION 6**

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**Belén Alvarez Palomo**, Biomedicine Department at **University of Barcelona**, Barcelona, Spain.

Dr. Belén Alvarez got her PhD on Biochemistry and Molecular Biology from the University of Barcelona working on the molecular mechanisms of muscle cachexia. Later she performed a postdoctoral stay in the The Netherland Cancer Institute, working on the cell and molecular biology of cell migration and metastasis. After that, she worked at IMIM on the transcriptional regulation of epithelial-to-mesenchymal transition of tumor cells.

For the last four years she has been working at the Control of Pluripotency lab with Dr. Michael Edel on cell reprogramming using messenger RNA transfection methods, both for reprogramming to pluripotency as for direct lineage reprogramming, and with an aim on clinical application. Last year, one her direct lineage reprogramming projects has obtained a CaixaImpulse grant for bringing it close to commercialization. At the moment she is developing, together with Dr. Ricardo Cassaroli, a protocol to make clinically useful limbal stem cell for corneal regeneration using clinical grade iPSC and investigating direct lineage reprogramming methods for making limbal stem cells.

### **Use of induced pluripotent stem cells and direct lineage reprogramming in cell therapy for eye regenerative medicine**

The loss of limbal stem cells (LSC), either due to disease or injury is a major cause of blindness worldwide. The current therapies include transplantation of cultivated limbal stem cells from the healthy eye of the patient. When LSC are lost in both eyes or not enough healthy tissue is available, transplantations have to be done from deceased donors, with the subsequent immune rejection. Long-term success shows a high degree of variation. Induced Pluripotent Stem Cells (iPSC) are a possible source of limbal stem cells that could be autologous or from HLA matched donors. In order to get iPSC-derived LSC closer to the clinic, we have created iPSC derived from BM-MSC using a clinical grade method and we have followed published protocols for corneal differentiation. We are testing the use of PAX6 mRNA transfection to improve the efficiency and shorten the times of LSC conversion of iPSC. We are also testing the effect of Pax6 mRNA on direct lineage reprogramming of BM-MSC into LSC.



**Ricardo P. Casaroli-Marano**, Professor of Ophthalmology & Researcher at, Hospital Clinic de Barcelona (IDIBAPS), University of Barcelona, Spain.

Held his medical degree in the Faculty of Medical Sciences of Santa Casa (São Paulo, Brazil), residency in ophthalmology at Barraquer Institute (Barcelona, Spain) and fellowship as Assistant Etranger (medical and surgical vitreoretinal pathology) at the Centre Hospitalier National d'Ophthalmologie des Quinze-Vingt (Paris, France). Had a professional master's degree in Data Analysis in Health Sciences, master's degree in Immunology, PhD in Biology and Pathology of the Cell, and post-PhD in Cell and Molecular Biology at the University of Barcelona (Barcelona, Spain). Currently he is Associate Professor of Ophthalmology at School of Medicine in the University of Barcelona and Chairman of Department of Surgery (<http://www.ub.edu/medicina/>). He develops his practice work as Consultant (Ophthalmology) at Hospital Clinic of Barcelona. He started his scientific career as post-PhD researcher in the Department of Cell Biology of University of Barcelona and after as Associate Researcher of Institute of Biomedical Research August Pi Sunyer (IDIBAPS) (<http://www.idibaps.org/>) and also at CellTec-UB, (<http://www.ub.edu/celltec-ub/>). At present he is also Affiliated Professor (Livre Docente Tenure) at the Department of Ophthalmology and Visual Sciences at Federal University of Sao Paulo (Escola Paulista de Medicina, Sao Paulo, Brazil) and Special Visiting Professor of CNPq (Centro Nacional de Pesquisa). Has experience in the area of Translational Medicine, acting on advanced therapies (cell therapy with human stem cells and adult mesenchymal progenitor cells) applied for the ocular surface and retinal degenerative diseases. He was co-founder of CellTec-UB (<http://www.ub.es/celltec-ub/>), spin-off of biotechnology at University of Barcelona and currently he is Research Coordinator of Barcelona Tissue Bank of Banc de Sang I Teixits (BST-GenCAT).

### **Adult progenitor stem cells: applications for cell-based therapy in degenerative retinal diseases**

Retinal degenerative diseases, such as age-related macular degeneration or hereditary degenerative diseases of the retina, constitute a major cause of irreversible blindness. Cell-based therapies offer hope for these patients at risk of blindness due to the deterioration of the neural retina and/or retinal pigment epithelial cells. Several sources of stem cells are currently being investigated for clinical use, ranging from human embryonic stem cells to adult-derived induced pluripotent stem cells. The restorative properties of stem cells hold the promise in the treatment of these retinal degenerative diseases. In this field, exciting progress has been made on cell-based therapy with adult progenitor stem cells in the last decade. Different stem cell types have been explored for their potential in treating the retinal degenerative diseases, including mesenchymal stem cells from adult tissue, retinal stem cells, and more recently induced pluripotent stem cells. Here, will summarize the recent progress in this interesting area and the challenges associated with their translation into clinical practice.





**Berta de la Cerda Haynes**, Postdoctoral researcher at **CABIMER - Fundación Progreso y Salud**, Seville, Spain

Berta de la Cerda, PhD. Biology grade in 1993 and PhD in Biology "cum laude" in 1999, Seville University. Researcher in Biochemistry Department, Seville University 2000-2005. Junior postdoctoral researcher, Spanish Research Council (CSIC) 2005-2008, working mainly in protein interactions and structure-function relationships in macromolecules. Senior postdoctoral researcher, Andalusian Molecular Biology and Regenerative Medicine Centre (CABIMER), Seville, 2009-. Focused in disease modelling and advanced therapies for retinal diseases. Currently leading a project on cellular modeling for monogenic retinal diseases, participant on EU Eyerisk project for the study of age-related macular degeneration, member of European Retinal Disease Consortium (ERDC) and European Retinal Therapeutics Consortium (ERTC) and member of the scientific advisory board of "Fundación Macula Retina".

### **Advanced cell therapies for AMD**

Currently there are no therapeutic options for dry AMD, what makes this condition a clear candidate for advanced therapies. No molecules or antibodies can stop or delay the progressive degeneration of the retinal pigmented epithelium (RPE) and subsequently of the photoreceptor layer. Regarding advanced cell therapy for AMD, the main hypothesis is that substitution of the aged and damaged RPE using a suspension or a patch of newly cultured cells could help the preservation of the photoreceptors. Recent advances in cellular biology have allowed the differentiation from human pluripotent stem cells towards different cell types. RPE is a relatively easy cell type to differentiate from both embryonic stem cells and induced pluripotent stem cells. Several clinical trials are being conducted in Asia, Europe and America to test the security of the RPE transplant in AMD and Stargardt's disease. From these initial studies it seems that eye-specific immune privilege and the homogeneity of laboratory-differentiated RPE leads to a safe procedure although therapeutic efficacy is still under debate.



**Maria Pia Cosma**, Senior Scientist and ICREA Research Professor at **Center for Genomic Regulation (CRG)**, Barcelona, Spain.

Maria Pia Cosma received the PhD in Cellular and Molecular Genetics, School of Medicine, Univ. of Federico II in the year 2000. From 1997-2000 she was Marie Curie Post-doc at IMP, Vienna and from 2000 to 2003 research associate at Telethon Institute of Genetics and Medicine (TIGEM), Naples. In 2003 she established her first group at TIGEM in Naples and become also EMBO Young Investigator (YIP). From 2004 to 2010 she has been Lecturer at SEMM (European School of Molecular Medicine) and Honorary Associate Investigator at IGB, CNR, Naples from 2009 to 2015. In April 2010 she moved to Barcelona, Spain, at the Center for Genomic Regulation (CRG), as Senior Scientist and ICREA Research Professor. She has given many invited talks at major international conferences and Research Institutes. She received several prizes including: Marie Curie Excellence Award in 2005, the honour of Order of Merit of the Italian Republic in 2007 and Barcelona City Prize in 2015. She is ERC stGrant awardee, 2009 and HFSP Grant awardee, 2010. She has been elected EMBO Member in 2010. Main interests of Cosma's group are to dissect mechanisms controlling somatic cell reprogramming and tissue regeneration. Publications can be found at: <http://www.ncbi.nlm.nih.gov/pubmed/?term=cosma+mp>

### **Retinal regeneration via cell fusion mediated reprogramming**

Main interests of my group are to dissect mechanisms and factors controlling somatic cell reprogramming and tissue regeneration in mammals. In particular we are interested to study the dynamics of the Wnt/ $\beta$ -catenin signaling pathway in the control of pluripotency, somatic cell reprogramming and tissue regeneration. We use experimental and modelling approaches to associate Wnt pathway dynamics to biological functions. We showed that Wnt-activated bone-marrow-derived cells can fuse with retinal neurons and Müller glia in degenerated retinas in mice. We observed transient reprogramming of the in-vivo formed hybrids and their differentiation in neurons, which can thereby functionally rescue ganglion/ amacrine cells and photoreceptors in drug-induced and genetic models of retinal degeneration. We also observed Parkinson's disease functional rescue by bone-marrow-derived hybrids. Overall neuroregeneration can be functional achieved via cell fusion-mediated reprogramming.

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**Michael Edel**, Group Leader at **University of Barcelona**, Barcelona, Spain.

M. Dr. Michael Edel has specialised his university studies in the basic and fundamental principles of Anatomy (achieving Honours), Embryology and Human Physiology, completing his PhD in Pathology. He currently holds a MINECO Spanish Government project grant (Type B, 2011 to 2015 and 2015 to 2017) as Principle Investigator (BFU2011\_26956 and BFU2014\_54467P). He is a senior researcher in the field of iPSCs, hESC, gene regulation, epigenetics, cell cycle, differentiation to neurons, lung or cardiac muscle cells for the use of such cells as a cell replacement therapy. Synthetic

RNA methods to directly change cell fate are at the fore-front of his technology for clinical applications to treat human disease.

### **Improving the quality and characterisation of stem cell reprogramming and analysing the genomic stability and immune response**

Main interests of my group are to dissect mechanisms and factors controlling somatic cell reprogramming and tissue regeneration in mammals. In particular we are interested to study the dynamics of the Wnt/ $\beta$ -catenin signaling pathway in the control of pluripotency, somatic cell reprogramming and tissue regeneration. We use experimental and modelling approaches to associate Wnt pathway dynamics to biological functions. We showed that Wnt-activated bone-marrow-derived cells can fuse with retinal neurons and Müller glia in degenerated retinas in mice. We observed transient reprogramming of the in-vivo formed hybrids and their differentiation in neurons, which can thereby functionally rescue ganglion/ amacrine cells and photoreceptors in drug-induced and genetic models of retinal degeneration. We also observed Parkinson's disease functional rescue by bone-marrow-derived hybrids. Overall neuroregeneration can be functional achieved via cell fusion-mediated reprogramming.

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## **Session 7: Round table. From bench to bedside**

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**Joan Parra**, General Manager **Leitat Technological Center**, Barcelona, Spain.

(See his CV at the Scientific Committee section)

### **Technology Transfer**

Scientific advances are an engine for the advancement of medicine, society and economics. The Technology Transfer process seeks the transformation of research into applied results. In this context, the multidisciplinary and holistic approach to research projects and the consideration of key points from the beginning to make these results transferable are important points to try to ensure success.

LEITAT is a non-profit private Research Centre carrying out applied Research. We support sustainable growth through the promotion of R&D&I and technology transfer, in the context of creating lasting value. LEITAT strongly believes and promotes the concept of Open Innovation. LEITAT has 8 R&D divisions set up by multidisciplinary teams with know-how and experience in different knowledge areas which comprises nanotechnology, electronics, advanced materials, biomedicine: from in vitro to in vivo experiments; from drugs to antibodies and stem cells, ... oriented to make the results transferable. For example, Leitat participate in TERET Project, which main objective is to develop and improve new ophthalmic therapies against retinal diseases: age-related macular degeneration (ARMD), diabetic retinopathy and pigment retinitis (PR). As the basis of the project, biotechnological solutions based on the application of gen silencing and interference RNA and humanized monoclonal antibodies will be used.

Chair of the **SESSION 7**

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**Bonaventura Clotet Sala**, Director of **IrsiCaixa**. Head of Infectious Diseases at **Hospital Universitari Germans Trias i Pujol**, Badalona, Spain.

Bonaventura Clotet, Specialized in Internal Medicine and Infectious Diseases. PhD in Medical Sciences in 1981. He is Head of the Infectious Diseases department and Director of the Retrovirology Laboratory “IrsiCaixa” Foundation at the Hospital Universitari Germans Trias i Pujol since 1993. He is also President of the Foundation “Lluita contra la SIDA”, founded in 1992. Since 2006, he is co-director of the HIVACAT project for the development of the AIDS vaccine in Catalonia, Spain. Director of the Master in AIDS Pathogenesis and Treatment (Universitat Autònoma de Barcelona) since 2011. Dr. Clotet is member of the steering committee of EuroSIDA Scientific Advisory Board Member. He is the director of the Chair of AIDS and Related Diseases at the University of Vic (UVic), created in October 2013. In October 2012, he was awarded by the Catalan Government (Generalitat de Catalunya) with the Josep Trueta medal to Sanitary Merit for his contributions to the research in the HIV field. In April 2016, Clotet has been awarded with the 'Creu de St. Jordi' for being one of the most internationally renowned HIV researchers. Dr. Clotet has created 2 spin-offs from IrsiCaixa: AELIX Therapeutics (December 2015) for the development of a therapeutic vaccine for HIV infected patients and AlbaJuna Therapeutics (January 2016) for the development of an Immunoglobulin for curing & preventing HIV. More than 700 papers in international journals (Impact factor: >5.000 // H-index: 81).

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**Ramon Maspons**, Chief Innovation Officer (CINO), **Agència de Qualitat i Avaluació Sanitàries de Catalunya (AQuAS)**, Barcelona, Spain.

MSc in Industrial Engineering from Polytechnical University of Catalonia (UPC), Executive Master in Public Administration. He holds a Diploma of Advances Studies in innovation and research management. Since 2010 Chief Innovation Officer at Agency for Healthcare Quality and Evaluation of Catalonia (AQuAS). Ramon has more than ten years' experience in hospital and medical innovation and more than twenty years of experience in international funded research projects, mainly EU projects. He has held positions as Deputy Secretary of Strategy at Catalan Ministry of health from 2004 to 2009, as Scientific Director of Fundació Parc de Salut for 6 years, as Strategics Project Unit Director of Biocat for 4 years, as Strategic Planning Director of Granollers City Council for 6 years, and as Director of IALE Tecnologia, a spin-off company of Polytechnical University of Catalonia (UPC) for 10 years. He was Assistant Lecturer of innovation and technology management at Business Administration Department, Polytechnical University of Catalonia (UPC) from 1993 to 1997 and since 1997 he is visiting researcher of technology monitoring and knowledge management at some Spanish universities. He is a guest lecturer at several educational centres, workshops and symposia.

#### **Innovative schemes through demand-driven procurement for procuring early detection services: the case of the PRO4VIP**

PRO4VIP. (<http://www.pro4vip.eu>) is a EU networking project related to Innovative Public Procurement. The mission of the project is to improve the quality of life of functional low-vision individuals through demand-driven procurement of either novel or fit-for-purpose, cost-effective ICT-based assistive technologies that could enhance their daily quality of life or novel and cost-effective clinical tools that could support physicians in the early detection of such conditions.

Innovative Public Procurement is an administrative action that encourages innovation geared to boosting the development of new markets from the demand side through the instrument of public procurement. This encompasses two different solutions: the Public Procurement of Innovation (PPI) and Pre-Commercial Public Procurement (PCP).

The objectives of PRO4VIP project are:

- (a) the creation and consolidation of a pan-European network of procurers;
  - (b) the definition of a common innovation procurement roadmap in the short term and in the long term;
  - (c) the definition of the cross-border and joint public procurement of innovation procedure(s) that best meet(s) procuring authorities' needs (that could be either PCP or PPI or both) and that in line with WHO Vision 2020 (<http://www.iapb.org/vision-2020>) would either support the early detection and treatment of functional low vision conditions or would support the provision for low vision services;
  - (d) the mutual learning, knowledge sharing and transferring within the consortium partners.
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**Xavier Rubies**, Head of Technology Transfer, **Institute for Bioengineering of Catalonia (IBEC)** Barcelona, Spain.

Dr. Xavier Rúbies has a wide experience in the healthcare sector over last 20 years; he is PhD in Veterinary Medicine and Executive MBA. Dr. Rúbies combines experience in the private and public sector. He has been Director of R&D of private companies like Innovative Health Technologies – IHT (European medical device company in interventional cardiology based in Barcelona) or Hipra Laboratories (pharmaceutical company with products in the vaccine, diagnostic and antibiotic areas). From the public perspective, Dr. Rúbies was Director of Technology Transfer at the CRG (Centre for Genomic Regulation) for more than 5 years, where he established the TTO and negotiated a 1.4M agreement with Sanofi. He was also the Strategy and Business Development Director during the first period of BIOCAT.

### **Market Driven Technology Transfer**

Efficient research and technology transfer of science and technologies to the healthcare sector should be market driven. Except for some specific medical practices, the research results and the new technologies developed in the research centres, hospitals and universities will reach the patients as products or services delivered by companies. The patient needs and the healthcare system should be the ultimate goal, but too often the research system misconceive or don't understand the rules and constrains of the companies. But, the companies are the real carriers of products and services to the patients and healthcare professionals. Time to market, finance intensity, competitors, regulatory affairs requirements, key opinion leaders, scalability, IP protection strategies, ... and many other points are key steeps in the way from the bench or the paper to the bedside.

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# POSTERS

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- 1 Silvia Albert, Riccardo Sangermano, Nathalie M. Bax, Miriam Bauwens, L. Ingeborgh van den Born, Elfride De Baere, Alejandro Garanto, Rob W.J. Collin, Carel B. Hoyng, Frans P.M. Cremers. **ABCA4 mRNA analysis of iPSC-derived photoreceptor cells of Stargardt patients reveals protein-truncating non-canonical and deep-intronic splice site variants**
- 2 Míriam Garcia, Marc Biarnés, Anabel Rodriguez, Jordi Monés. **Dark adaptation impairment in patients with drusen**
- 3 Marc Biarnés, Clara Ramon, Jordi Monés. **Identification of phenotypes in geographic atrophy using cluster analysis**
- 4 David Merino, Marc Biarnés, Miriam Garcia, Jordi Monés, Pablo Loza-Alvarez. **Images of the microscopic in-vivo human retina acquired using AOSLO**
- 5 Anna Borrell, Ramon Maspons, Jean Patrick Mathieu, Jordi Monés, Vincenzo Alberto Vella. **Innovative procurement for visually impaired people: Introducing the PRO4VIP project**
- 6 Laura Fontrodona, Laia Miquel, Anna Duarri, Yolanda Muñoz, Diana Mora Ramírez, Barbara Ferreira-de-Souza, Anna Salas Torras, Anna Veiga, Jose Garcia-Arumi. **Mixing RPE with photoreceptor precursor cells to cook an improved stem-cell based therapy for retinal degenerative diseases**
- 7 Jaume Català, Begonya Nafria Escalera, Ana Morales Becerra, Jesús Diaz Cascajosa, Jaume Pérez Payarols, Joan Prat Bartomeu. **Rare Commons: an innovative social media platform 2.0 for collaborative clinical research on inherited retinal dystrophies**
- 8 Lourdes Roque-Navarro, Jaume Adan, Laura Padilla, Toni Coll, Rosa Hervas, José L. Hernández, Ramon Messeguer, Carme Calvis, Sheila Dakhel, Marc Masa and Francesc Mitjans. **Recombinant antibodies for research, diagnosis and therapy with biological in vitro (cells) & in vivo (animal) characterization**

# PRACTICAL INFORMATION

## Venue: CaixaForum

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**Caixa Forum Barcelona**  
Avgda. Francesc Ferrer i Guàrdia, 6-8  
08038 Barcelona, Spain

**Conferences**  
Room 2

**Poster session**  
Room 2

## Contact persons during the event

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# SUGGESTED READING

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# OUTCOMES

## B·Debateca

On the website of B·Debate, you will find all the information related with the celebration of the meeting that includes reports, conclusions, scientific documents, interviews with the experts, the speaker's CVs, videos, images, press documentation and other related materials. We invite you to visit the section B·Debateca on [www.bdebate.org](http://www.bdebate.org)

Contents of the meeting “Fighting Blindness. Future Challenges and Opportunities for Visual Restoration”

The screenshot shows the B-DEBATE website interface. At the top, there is a navigation menu with the following items: DESTACATS, B-DEBATE, CONVOCATÓRIA, B-DEBATECA (highlighted with a red circle), NOTÍCIES, and CONTACTE. Below the navigation menu, there is a search bar and a dropdown menu for 'DEBATE' with 'Fighting Blindness' selected (also highlighted with a red circle). The main content area features a grid of speaker profiles, each with a photo, name, and affiliation. The speakers listed are: Baruch D. Kuppermann (University of California, Irvine), Jayakrishna Ambati (University of Kentucky), Caroline Klaver (Erasmus University Medical Center), Marco Zarbin (New Jersey Medical School), Jordi Monés (Barcelona Macula Foundation: Research for Vision), Christina Kiel (Centre de Regulació Genòmica (CRG)), and Belén Alvarez Palomo (University of Barcelona). The event title 'Fighting Blindness. Future Challenges and Opportunities for Visual Restoration' is prominently displayed at the top of the speaker list.

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# ORGANIZERS



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**B-Debate** International Center for Scientific Debate Barcelona is a **Biocat** initiative with support from “**la Caixa**” **Foundation**. It drives first-rate international scientific debates, to foster dialogue, collaboration and open exchange of knowledge with prestigious national and international experts, to approach complex challenges of high social interest in life sciences. B-Debate sees debate as a powerful, effective way to generate knowledge and strives to help position Barcelona as a benchmark in generating knowledge and Catalonia as a country of scientific excellence.

B-Debate sees debate as a powerful, effective way to generate new knowledge. The debates are top-notch international scientific meetings featuring a selection of experts of renowned international prestige and scientists that work in Barcelona and Catalonia, moderated by scientific leaders. Since 2009 B-Debate has invited about 1200 recognized speakers and over 7.000 attendees. B-Debate seeks out answers to the challenges and needs of society in the field of life sciences, taking into account the complex, ever-changing conditions of this global world. The debates foster the integration of different disciplines of science and deal with such diverse topics as ageing, new therapeutic approaches to various diseases, innovative technology to improve knowledge of the human genome, food resources, new tools to integrate knowledge management, clinical genomics, neurosciences, climate change, and new energy sources, among others. The knowledge and results obtained through these events is spread throughout both the scientific community and general society through the various **B-Debate** channels and instruments.

More info: [www.bdebate.org](http://www.bdebate.org)

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The main motivation behind the **Barcelona Macula Foundation: Research for Vision** is to conduct and support research into diseases of the retina and macula that cause blindness and have no effective treatment, in order to restore or prevent loss of vision. The principal focus of interest is macular and retinal degeneration, such as atrophic age-related macular degeneration (AMD), retinitis pigmentosa and Stargardt's disease. Through collaboration and partnerships with other centres, universities and companies, BMF promote research and innovation projects that enable us to build new therapeutic approaches to macular and retinal degeneration on cellular therapies, tissue engineering and transplantation, and biomaterials applied to these therapies.

The Foundation devotes special attention to pre-clinical research. The idea is to pass results on to the clinical phase in the shortest possible time. In fact, BMF researchers have already carried out phase-I, phase-II and phase-III clinical trials both for exudative and non-exudative age-related macular degeneration and for other degenerative diseases of the retina. The Foundation is also participating in four major European Union (EU) projects performed by several agencies from different countries.

For more information: [www.barcelonamaculafound.org](http://www.barcelonamaculafound.org)

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**LEITAT Foundation** is a public character and non profit organization created in 2007 and registered under number 1.017 in the Register of Foundations Protectorate of the Spanish Ministry of Education and Science. Its mission is to promote research, development and technological innovation within different economic industries to contribute to the development of society. Our activities are aimed at promoting the culture of innovation and improving competitiveness in order to contribute to the economic development, technological progress and social cohesion. Therefore, LEITAT Foundation beneficiaries are universities, research centers, the scientific community and the business sector that promote research, development and technological innovation.

More info: [www.fundacionleitat.org](http://www.fundacionleitat.org)

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The Centre for Genomic Regulation (CRG) is an international biomedical research institute of excellence, founded in December 2000 whose mission is to discover and advance knowledge for the benefit of society, public health and economic prosperity. The CRG believes that the medicine of the future depends on the groundbreaking science of today. This requires an interdisciplinary scientific team focused on understanding the complexity of life from the genome to the cell to a whole organism and its interaction with the environment, offering an integrated view of genetic diseases. Research at the CRG falls into four main areas: gene regulation, stem cells and cancer; cell and developmental biology; bioinformatics and genomics; and systems biology.

With 400 scientists from 43 countries, the CRG excellence is based on an interdisciplinary, motivated and creative scientific team that is supported by high-end and innovative technologies. Over 200 publications in high quality journals are published every year, and researchers are also active in facilitating the transfer of new basic findings into benefits for health and economic value for society.

The CRG is one of the Spanish institutes awarded with the Severo Ochoa grant of excellence, and has been ranked by the Scimago Institutions Rankings 2015 as the 6th best medical research institute worldwide, according to the Q1 indicator, within the health sector.

More info: [www.crg.eu](http://www.crg.eu)

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## COLLABORATORS

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**Fundación Quirónsalud** is the result of the integration of Fundación IDC, Fundación Quirón and Fundación Teknon with the commitment to supporting Quirónsalud group's endeavors to bring about continuous social welfare improvement. Clinical research and innovation, health education and social action are the three fields of activity with which Fundación Quirónsalud aims to be the benchmark institution in healthcare and healthy habits promotion. Fundación Quirónsalud collaborates with national and international prestigious institutions and universities to carry out its activities.

Fundación Quirónsalud actively promotes innovative medical and healthcare training programs and participates in several clinical and translational research projects, with an important contribution to the field of ophthalmology.

More info: [www.fudacionquironsalud.es](http://www.fudacionquironsalud.es)

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**CosmoCaixa** offers interactive, enjoyable science and an open door for anyone who is eager to learn and understand and who never stops wondering why things are the way they are. **CosmoCaixa Barcelona** boasts the Geological Wall and the Amazon Flooded Forest, which features more than 100 plant and animal species that convince visitors they have been transported from the Mediterranean to the very heart of the tropical jungle. In addition to its permanent facilities and its open areas, CosmoCaixa offers a scientific and educational programme that includes exhibitions, workshops, conferences, courses and debates involving experts from all over the world.

More info: [obrasocial.lacaixa.es](http://obrasocial.lacaixa.es)

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