







## Draft Agenda – ERN-Workshop: New Therapeutical Approaches for Inherited Retinal Dystrophies

Venue: CIPF – Príncipe Felipe Research Center, Eduardo Primo Yúfera, 3, 46012, Valencia, Spain

Date: 27-28. March 2023

## Day 1.

Time	Speaker and Affiliation	Session name/Topic		
9:30 - 12:15	Genetics and Retinal Phy	Genetics and Retinal Physiology		
9:30 - 10:15	Nicolás Cuenca, UMH, Alicante, Spain	Neuronal and glial cells response associated with inherited retinal dystrophies		
10:15 - 11:00	Rosa Coco, IOBA, Valladolid, Spain	Approach to clinical diagnosis		
11:30 - 12:15	Carmen Ayuso, FJD, Madrid, Spain. CIBERER	Diagnosis and genetic counselling		
12:15 - 13:30	New therapeutic approaches and Drug Delivery systems for			
	Inherited Retinal Dystrophies			
12:15 - 12:45	José María Millán, IIS La Fe, Valencia, Spain. CIBERER	Introduction of new th <mark>erapie</mark> s		
12:45 - 13:30	Gustavo Puras, UPV/EHU, Vitoria-Gasteiz, Spain. CIBERBBN	Drug Delivery Systems: Targeting the posterior segment		
15:00 - 16:30	Pharmacological Therapies			
15:00 - 15:30	François Paquet-Durand, IOR, University of Tübingen, Germany	The novel PKG inhibitor CN238 provides multi-level functional neuroprotection of photoreceptors and retinal ganglion cells		
15:30 - 16:00	Regina Rodrigo, CIPF, Valencia, Spain. CIBERER	Nutraceuticals and inherited retinal dystrophies		
16:00 - 16:30	Blanca García-Sandoval, FJD, Madrid, Spain. CIBERER	Oral administration of Emixustat in patients with Stargardt disease.		
16:30 - 17:30	Panel Discussion I			











## Day 2.

Time	Speaker and Affiliation	Session name/Topic
9:00 - 11:00	Gene Therapies	
9:00 - 9:30	Alex Garanto, Radboudumc, Nijmegen, The Netherlands	Antisense oligonucleotide-based therapies for inherited retinal dystrophies
9:30 - 10:00	Eduardo Silva, Hospital Dona Estefânia, Lisbon, Portugal	State-of-the-Art Clinical Trials based on gene therapy
10:00 - 10:30	Katarina Stingl, University Hospital Tübingen, Germany	Voretigene neparvovec: what do we know about safety and efficacy today?
10:30 - 11:00	Noa Solá, Barcelona, Spain	My exp <mark>erience with L</mark> uxturna
11:30 - 13:00	Cellular Therapies	
11:30 - 12:00	Maijlinda Lako, Biosciences Institute, Newcastle University, UK	Pluripotent Stem Cell-Derived Retinal Organoids to Repair Vision
12:00 - 12 :30	Mike Cheetham, UCL Institute of Ophthalmology, London, UK	Retinal organoids as a tool for understanding disease mechanisms and developing therapies
12:30 - 13 :00	Christelle Monville, I-Stem, Corbeil-Essonnes, France	Retinal Pigment Epithelial cells derived from human embryonic stem cells disposed on human amniotic membrane: An update on the French RP clinical trial
13:00 - 16:00	Optogenetics and Electro	onic Devices
13:00 - 13:30	Eduardo Fernández Jover, UMH, Alicante, Spain. CIBERBBN	Non viral vectors for gene therapy
15:00 - 15:30	Jasmina Cehajic Kapetanovic, University of Oxford, Oxford, UK	Genetic Therapies for Retinal Degenerations
15:30 - 16:00	Guadalupe Iglesias, Madrid, Spain	My experience with Argus II
16:00-17:00	Panel discussion II	

