



Organizers:
Paulo J Oliveira
(Coimbra, Portugal)
Catarina R Oliveira
(Coimbra, Portugal)



Coimbra, Portugal, 22-24 May 2019



Symposium 6 | Gene and Cell Therapy: from Fundamental Science to the Clinic

Organizer: Lino Ferreira (Coimbra, Portugal)
Albert Rizvanov (Kazan, Russia)
Costanza Emanuelli (London, UK)

Day: Wednesday afternoon and Thursday morning
Meeting room: Meeting Room Coimbra, Hotel Dona Inés (Congress venue 2)

Wednesday, 22 May

14.00-16.00 Session 1: Cell and gene-based therapies I

Chairs: Lino Ferreira (Coimbra, Portugal)
Albert Rizvanov (Kazan, Russia)
Costanza Emanuelli (London, UK)

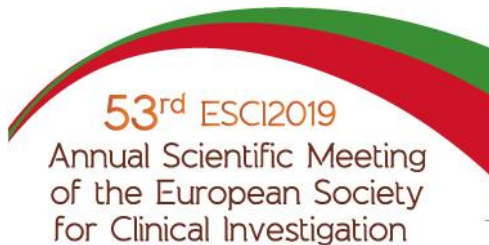
14.00-14.30 **S6-L1 | Exosomes a double-edged sword in cardiovascular medicine**
Costanza Emanuelli (London, UK)

14:30-15:00 **S6-L4 | Role of peribronchiolar mesenchymal cell subpopulations in instructing epithelial behavior during repair processes**
Saverio Bellusci (Giessen, Germany)

15.00-15.15 **S6-O1 | PET imaging and in vivo bio-distribution of Small Extracellular Vesicles**
Arnab Banerjee¹, Tiago Rondão¹, José Sereno², Vítor Alves², Miguel Lino¹, Anderia Reberio¹, Antero Abrunhosa², Lino Ferreira
¹CNC-Center for Neurosciences and Cell Biology, University of Coimbra, Coimbra, Portugal, ²ICNAS – Institute for Nuclear Sciences Applied to Health; University of Coimbra, Coimbra, Portugal

15.15-15.30 **S6-O2 | CRISPR interference-mediated silencing of mutant ATXN3 decreases motor impairments of an in vivo Machado-Joseph disease model**
Carlos Matos^{1,2}, Frederico Pena¹, André Conceição¹, Sara Lopes^{1,3}, Sónia Duarte¹, Catarina Miranda, Luís Pereira de Almeida^{1,4}
¹Center for Neuroscience and Cell Biology - CNC, Coimbra, Portugal, ²Centre for Biomedical Research - CBMR, Faro, Portugal, ³Institute for Interdisciplinary Research, University of Coimbra, Coimbra, Portugal, ⁴Faculty of Pharmacy, University of Coimbra, Coimbra, Portugal

15.30-15.45 **S6-O3 | Orchestrating the release of small extracellular vesicles with a light-triggerable hydrogel**
Helena Henriques-Antunes¹, Renato Cardoso¹, Alessandra Zonari¹, Joana Correia¹, Ermelindo Leal¹, Adrian Jimenez-Balsa¹, Miguel Lino¹, Ana Barradas¹, Ivana Kostic¹, Célia Gomes², Eugénia Carvalho¹, Lino Ferreira¹
¹Cnc, Coimbra, Portugal, ²Faculdade de Medicina da Universidade de Coimbra, Coimbra, Portugal



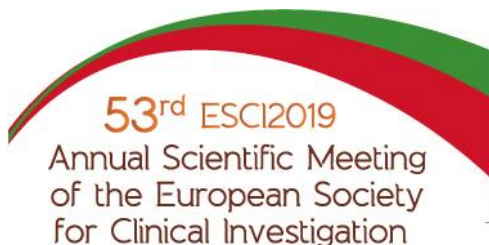
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- 15.45-16.00 **S6-O4 | Intersection of autophagy and exosome secretion on alpha-synuclein spreading pattern in vitro models of Parkinson's disease**
Rita Perfeito^{1,2}, Vanessa Anjos¹, Rui Jorge Nobre^{1,2}, Manuel Garrido^{1,3}, Jens Schwamborn⁴, Luís Pereira de Almeida^{1,5}
¹CNC- Center For Neuroscience And Cell Biology, University of Coimbra; 3004-504 Coimbra, Coimbra, Portugal, ²Instituto de Investigação Interdisciplinar, University of Coimbra; 3030-789 Coimbra, Coimbra, Portugal, ³Genibet Biopharmaceuticals; 2780-157 Oeiras, Oeiras, Portugal, ⁴LCSB- Luxembourg Centre for Systems Biomedicine, University of Luxembourg, Luxembourg, Luxembourg, ⁵Faculty of Pharmacy, University of Coimbra; 3000-548 Coimbra, Coimbra, Portugal
- 16:00-16:30 Coffee-break
- 16.30-18.00 Session 2: Cell and gene-based therapies II**
Chairs: Lino Ferreira (Coimbra, Portugal)
Albert Rizvanov (Kazan, Russia)
Costanza Emanuelli (London, UK)
- 16.30-17.00 **S6-L3 | Cytochalasin B-induced membrane vesicles – a new cell-free strategy for regenerative medicine**
Albert Rizvanov (Kazan, Russia)
- 17:00- 17:30 **S6-L2 | Adult myocardial slices: a viable and highly functional platform to study cardiac tissue**
Filippo Perbellini (London, UK)
- 17.30-17.45 **S6-O5 | HISTONE ACETYLATION AND APOPTOSIS IN PENUMBRA AFTER PHOTOTHROMBOTIC STROKE IN THE RAT CEREBRAL CORTEX**
Prof. Anatoly Uzdensky¹, Dr. Svetlana Demyanenko¹, Ms. Valentina Dzreyan¹
¹Southern Federal University, Rostov-on-don, Russian Federation
- 17.45-18.00 **S6-O6 | A miRNA-based gene therapy approach to knock-down mutant ATXN3: alleviation of Machado-Joseph disease phenotype in two mouse models upon two routes of administration**
Rui Jorge Nobre^{1,2,3}, Joana Saraiva¹, Clelia Fusco¹, Susana Paixao¹, Magda Santana^{1,2}, Catarina Miranda^{1,2}, Lorena Petrella⁴, Jose Sereno⁴, Joao Castelhana⁴, Miguel Castelo-Branco⁴, Miguel Sena-Esteves⁵, Luis Almeida^{1,3,6}
¹Center for Neuroscience and Cell Biology, Coimbra, Portugal, ²Institute for Interdisciplinary Research (IIIUC), Coimbra, Portugal, ³ViraVector - Viral Vectors for Gene Transfer Core Facility, Coimbra, Portugal, ⁴ICNAS - Institute of Nuclear Sciences Applied to Health, Coimbra, Portugal, ⁵University of Massachusetts Medical School, Worcester, USA, ⁶Faculty of Pharmacy, University of Coimbra, Coimbra, Portugal



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Thursday, 23 May

08.30-10.00 **Session 3: RNA therapeutics, on behalf of COST Action CardioRNA CA17129**

Chairs: Lino Ferreira (Coimbra, Portugal)
Albert Rizvanov (Kazan, Russia)
Costanza Emanuelli (London, UK)

08.30-08:45 **S6-L5 | The CardioRNA COST Action**
Costanza Emanuelli (London, UK)

08:45-09:15 **S6-L6 | Repeated transplantation of mesenchymal stromal cells in Machado-Joseph disease/Spinocerebellar ataxia type-3 mice can sustainably alleviate phenotypic symptoms and ameliorations can be monitored through magnetic resonance spectroscopy**
Catarina Oliveira Miranda¹, Adriana Marcelo¹, Teresa Pereira Silva¹, João Barata¹, Ana Vasconcelos-Ferreira¹, Dina Pereira¹, Sónia Duarte¹, Inês Barros¹, Clévio Nóbrega¹, José Sereno², Lorena Petrella², Vitor Hugo Paiva³, João Castelhanos², Rui Jorge Nobre¹, Célia Gomes⁴, Miguel Castelo-Branco², Luís Pereira de Almeida¹
¹CNC-University of Coimbra (UC), Coimbra, Portugal, ²ICNAS-UC, Coimbra, Portugal, ³MARE-UC, Coimbra, Portugal, ⁴ICBR-UC, Coimbra, Portugal, ⁵IBILI-UC, Coimbra, Portugal, ⁶FFUC, Coimbra, Portugal

09:15-09:45 **S6-L7 | Regulatory RNAs to treat the diseased heart**
Antonio Salgado Somoza (Luxembourg)

09:45-10:00 **S6-O7 | A cross-sectional and longitudinal study on plasma derived miRNAs revealed novel dysregulated signatures in Duchenne muscular dystrophy patients**
Francesco Catapano¹, Dominic Scaglioni¹, Kate Maresh¹, Joana Domingos¹, Pierpaolo Ala¹, Valeria Ricotti¹, Lauren Phillips², Laurent Servais³, Imelda de Groot⁴, E.H. Niks⁵, J.J.G.M. Verschuuren⁵, Volker Straub^{2,6}, Thomas Voit⁷, Jennifer Morgan¹, Francesco Muntoni¹
¹The Dubowitz Neuromuscular Centre, UCL Great Ormond Street Institute of Child Health, London, United Kingdom, ²John Walton Muscular Dystrophy Research Centre, Institute of Genetic Medicine, Newcastle University, Newcastle upon Tyne, United Kingdom, ³Service of Clinical Research and Databases, Institute of Myology, Paris, France, ⁴UMC St. Radboud, Nijmegen, The Netherlands, ⁵Department of Neurology, Leiden University Medical Center, Leiden, The Netherlands, ⁶Northern Genetics Service, Newcastle upon Tyne Hospitals NHS Foundation Trust, Institute of Human Genetics, International Centre for Life, Newcastle upon Tyne, United Kingdom, ⁷NIHR Great Ormond Street Hospital Biomedical Research Centre, UCL Great Ormond Street Institute of Child Health, London, United Kingdom

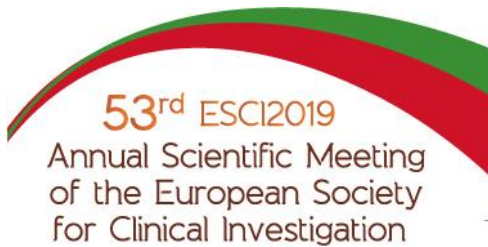
10:00-10:30 Coffee-break

10.30-12.30 **Session 4: Cell and gene-based therapies III**

Chairs: Lino Ferreira (Coimbra, Portugal)
Albert Rizvanov (Kazan, Russia)
Costanza Emanuelli (London, UK)

10.30-11.00 **S6-L8 | Therapies and stem cell-based models for cardiovascular applications**
Lino Ferreira (Coimbra, Portugal)

11.00-11.30 **S6-L9 | Advances in Pluripotent Stem Cells cell processing: New technologies for new therapies**
Paula Alves (Oeiras, Portugal)



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- 11.30-11.45 **S6-O8 | Quiet! They May Be at Rest: Metabolic Regulation of Paused-Pluripotency by Manipulating Culture Conditions**
Bibiana Silva¹, Ana Sofia Rodrigues¹, Maria Inês Sousa^{1,2}, João Ramalho-Santos^{1,2}
¹Biology of Reproduction & Stem Cells Group, Center for Neuroscience and Cell Biology, University of Coimbra, Coimbra, Portugal, ²Department of Life Sciences, University of Coimbra, Coimbra, Portugal
- 11.45-12.00 **S6-O9 | Efficient non-coding RNA delivery in wound healing by photo-triggerable nanoparticles**
Vitor Francisco^{1,2}, Josephine Blersch¹, Catarina Rebelo^{1,2}, Adrian Jimenez¹, Helena Antunes^{1,2}, Carlo Gonzato³, Sandra Pinto¹, Susana Simões¹, Klaus Liedl⁴, Karsten Haupt³, Lino Ferreira^{1,2}
¹Center For Neuroscience and Cell Biology - University of Coimbra, Coimbra, Portugal, ²Faculty of Medicine - University of Coimbra, Coimbra, Portugal, ³Campiegne University of Technology, CNRS Institute Enzyme and Cell Engineering, Compiègne Cedex, France, ⁴Faculty of Chemistry and Pharmacy - University Innsbruck, Leopold-Franzens, Austria
- 12.00-12.15 **S6-O10 | Natural Rubber Latex as Stem Cell Scaffold for Cell Therapy**
Dr. JULIANA Floriano^{1,2}, Dr. Carlos Graeff², Dr. Angélica Barbosa³, Ms. Patricia Lima¹, Ms. Thaís Pinto⁴, Dr. Willian Zambuzzi⁴, Dr. Marilza Rudge¹
¹São Paulo State University (UNESP), Medical School, Botucatu, Brazil, ²São Paulo State University (UNESP), School of Sciences, Bauru, Brazil, ³São Paulo State University (UNESP), School of Philosophy and Sciences, Marília, Brazil, ⁴São Paulo State University (UNESP), Bioscience Institute, Botucatu, Brazil
- 12.15-12.30 **S6-O11 | Tackling the Controversia: Decoding Human Cardiac Progenitor Cells Regenerative Potential In Acute Myocardial Infarction**
Maria João Sebastião^{1,2}, Ivo Reis^{1,2}, Itziar Palacios³, Margarida Serra^{1,2}, Patrícia Gomes-Alves^{1,2}, Paula M Alves^{1,2}
¹iBET- Instituto de Biologia Experimental e Tecnológica, Oeiras, Portugal, ²ITQB-NOVA- Instituto de Tecnologia Química e Biológica António Xavier, Oeiras, Portugal, ³Coretherapix, S.L.U. (Tigenix Group, Takeda), Tres Cantos, Spain