



II Spanish Meeting on Oligonucleotide Therapeutics

Thursday 28th November

Registration (8:00-8:50 am)

Welcome (8:50-9:00 am)

Session I: Chemical diversity and synthetic issues (9:00-11:00 am)

1. Mano Manoharan (Alnylam): "Realizing the promise of RNAi Therapeutics through Biomimetic Chemistry of siRNAs"
2. Juergen Mueller (LGC Axolabs): "LGC's Contribution to Accelerate Nucleic Acid Medicines"
3. Marc Caminals (Nitto Avecia): "Steps of an Oligo project from CMO perspective"

Coffee break (10:30-11:00) Posters visualization

4. Tamara Martínez (Sylentis): Drugs 2.0. "siRNA-based therapies generated by artificial intelligence (AI)"
5. Jesper Wengel (University of Southern Denmark): "Functionalized LNA designs for antisense applications"

Poster Flash presentations (12:00-13:45)

1. Juan Manuel Fernández (IBEQ): "Magnetic bead-based immunosensing platform for *in-situ* detection of secreted cytokines in response to oligonucleotide treatments in engineered muscles".
2. Pieter Mestdagh (Biogazelle): "Identification of potent and specific antisense oligonucleotides for target gene knockdown using a high-throughput RT-qPCR based cellular screening platform".
3. Javier Checa (Idibell): "Identification of oxidative stress-related genes in CF airway epithelial cells through functional genomics".
4. Sarah Overby (UV): "Proof of concept of the therapeutic use of blockmirs in Myotonic dystrophy models".
5. Santiago Vernia (LMS): "Antisense oligonucleotides in obesity-related metabolic pathologies"
6. Javier Poyatos (Hospital La Fe): "Dystrophinopathic subjects with a specific mega-deletion of exons 45-55 in the DMD gene, as a template for CRISPR/Cas9 therapy in Duchenne Muscular Dystrophy".
7. Paula Milán (IMDEA): "Cocktail of non-coding RNAs delivered by Magnetic and Gold Nanoparticles for the treatment of Uveal Melanoma".

Lunch break (13:45-15:00) Posters visualization

Session II. Delivery of therapeutic oligonucleotides (15:00-17:00)

1. Virginia Arechavala (Biocruces Bizkaia Institute, Bilbao): "Delivery of antisense therapeutics: the last hurdle for successful therapies"
2. Ramón Eritja (IQAC, Barcelona): "DNA nanostructures for delivery of therapeutic oligonucleotides"
3. Carles J. Ciudad (University of Barcelona): "PolyPurine Reverse Hoogsteen (PPRH) hairpins oligonucleotides: design, usage and delivery"
4. Dr. Jordi Hernández (CRG, Barcelona): "Alternative splicing modulation as a novel therapy for the treatment of lung cancer".



Friday 29th November

Session III. Discovery and preclinical studies (9:00-11:00 am)

1. Beatriz Llamusi (University of Valencia): "ARTHEX-DM, antisense RNA therapeutics in Myotonic Dystrophy".
2. Peter E. Nielsen (University of Copenhagen): "Precision PNA-antisense antibiotics against multidrug resistant Gram-negative bacteria".
3. Lucia Echevarria (SQY therapeutics): "Preclinical safety evaluation of tcDNAs".
4. Pieter Mestdagh (Biogazelle): "HTPathwaySeq, a novel application for high-throughput RNAi off-target gene identification".

Coffee break (11:00-11:30) Posters visualization

Session IV Clinical studies (11:30-13:00)

1. Marga Galan and Arancha Maciá (Experior): "Research and clinical trials, the Role of the CRO"
2. Alex McCampbell (Biogen): "Identification and development of ASOs for treatment of familiar ALS"
3. Juan Jesús Vílchez (Sarepta-Hospital La Fe): "Precision medicine in DMD treatment: the role of RNA technology"

Lunch break (13:00-14:00) Posters visualization

Session V. Regulatory and IP issues. (14:00-16:00)

1. Lidia Cánovas (Asphalion): "Regulatory considerations for therapeutic oligonucleotides"
2. Gustavo Fuster (Hoffmann Eitle): "Patents and trade marks, at national, Community and international level"
3. Joaquín Sáez (Ecrin España): "Translational research from a multistakeholder perspective: from the idea to its practical implementation"
4. Gonzalo Calvo Rojas (Medicament): "Translational research from a multistakeholder perspective: the regulatory viewpoint"

Round table. (16:00-17:30). Effective translation of research. Chairman: Dr. Manuel Pérez Alonso

Participants: Esther Rimbau (Upf Ventures), Sonia Vidal (La Caixa Foundation), Gema Tur (Agencia Valenciana de la innovación), Lluís Pareras (IN VIVO ventures), Ana Isabel Jiménez (Sylentis and ASEBIO), Lucas Martínez Clar (DCN), and Jose Luis Garcia (Epidisease).



Provisional poster list

P1. Magnetic bead-based immunosensing platform for *in-situ* detection of secreted cytokines in response to oligonucleotide treatments in engineered muscles. Jordina Balaguer-Trias, Juan M. Fernández-Costa, Xiomara Fernández-Garibay, Javier Ramón-Azcón. Biosensors for Bioengineering Group, Institute for Bioengineering of Catalonia (IBEC), 08028 Barcelona Spain.

P2. Identification of potent and specific antisense oligonucleotides for target gene knockdown using a high-throughput RT-qPCR based cellular screening platform. Mestdagh P, Van Peer G, Vanderstraeten H, Vereecke H, Luypaert M, Steyaert S, Fierro C, Revets H, Hellemans J, Vandesompele J. Biogazelle, Technologiepark 82, B-9052 Zwijnaarde, Belgium.

P3. Identification of oxidative stress-related genes in CF airway epithelial cells through functional genomics. Javier Checa and Josep M. Aran. Immuno-Inflammatory Processes and Gene Therapeutics Group. Human Molecular Genetics Laboratory. Instituto de Investigación Biomédica de Bellvitge (IDIBELL).

P4. BlockmiR oligonucleotides rescue DM1 phenotypes in cells and mice. Sarah Overby, Estefania Cerro-Herrero, Beatriz Llamusi and Rubén Artero. Translational Genomics Group, INCLIVA Health Research Institute, and Interdisciplinary Research Structure for Biotechnology and Biomedicine (ERI BIOTECMED), University of Valencia. Valencia, Spain.

P5. Dystrophinopathic subjects with a specific mega-deletion of exons 45-55 in the DMD gene, as a template for CRISPR/Cas9 therapy in Duchenne Muscular Dystrophy. Poyatos-García, J1 ; Soblechero-Martín, P2 ; Albiasu-Arteta, E2 ; Vázquez-Manrique, R.P3,4; Muelas, N1,4,5.; Arechavala-Gomez, V*2,6; Vilchez, J.J* 1 1. Neuromuscular Pathology and Ataxias Research Group, Health Research Institute La Fe (Hospital Universitario y Politécnico La Fe), València, Spain 2. Neuromuscular Disorders Group, IIS Biocruces Bizkaia, Barakaldo, Spain, 3. Cellular Genomic Lab Valencia Spain, Health Research Institute La Fe (Hospital Universitario y Politécnico La Fe), València, Spain; 4. CIBER de Enfermedades Raras (CIBERER), Madrid, Spain; 5. Neuromuscular Diseases Unit, Neurology Department, Hospital Universitari i Politécnic La Fe, Valencia; 6. Ikerbasque- Basque Foundation for Science, Bilbao, Spain.

P6. Cocktail of non-coding RNAs delivered by Magnetic and Gold Nanoparticles for the treatment of Uveal Melanoma. Paula Milán Rois, David García Soriano, Nuria Lafuente, Ciro Rodríguez Diaz, Sonia Romero Pérez, Yurena Luengo, Alfonso Latorre, Gorka Salas, Alvaro Somoza*. IMDEA Nanociencia, Faraday 9, Madrid.

P7. Contract Research Organization (CRO) and their support during the development of a clinical trial. Laura Rodríguez, Marga Galán, Arancha Maciá, Jaime Ballester. EXPERIOR S.L

P8. A COST network to improve the Delivery of Antisense RNA therapeutics. Virginia Arechavala-Gomez^{1,2} on behalf of DARTER COST Action. ¹Neuromuscular Disorders Group, Biocruces Bizkaia Health Research Institute, Barakaldo, Bizkaia, Spain.

P9. Development of RNA based approaches to exploit alternative therapies for Lysosomal Storage Diseases. Liliana Matos¹, Juliana Inês Santos^{1,2}, Melissa Rocha¹, Maria Francisca Coutinho¹, Paulo Gaspar³, Renata Voltolini Velho⁴, Thomas Bräulke⁴, Maria João Prata^{2,5}, Sandra Alves¹. ¹ Research and Development Unit, Department of Human Genetics, INSA, Porto, Portugal; ² Biology department, Faculty of Sciences, University of Porto, Portugal; ³ Newborn Screening, Metabolism and Genetics Unit, Department of Human Genetics, INSA, Porto, Portugal; ⁴ University Medical Center Hamburg-Eppendorf, Hamburg, Germany.⁵ i3S – Health research and innovation institute, University of Porto, Portugal.