

FINAL MEETING

DELIVERY OF ANTISENSE RNA THERAPIES (DARTER) COST ACTION CA17103 <https://antisenserna.eu/>

Mitxelena Auditorium, Bizkaia Aretoa, Bilbao
22nd-24th February 2023

Program committee

Puri Fortes, CIMA. University of Navarra, Spain
Alex Garanto, Radboud University Medical Center, the Netherlands
Aur lie Goyenvalle, University of Versailles, UVSQ, France
Virginia Arechavala-Gomez, Biocruces Bizkaia HRI, Spain

Local organizing committee

M^a Angeles Solin s Aspiazu, University of the Basque Country UPV/EHU, Spain
Ainara Vallejo Illarramendi, University of the Basque Country UPV/EHU, Spain
Olatz Villate Bejarano, Biocruces Bizkaia HRI, Spain
Virginia Arechavala-Gomez, Biocruces Bizkaia HRI, Spain

Scientific committee

Lourdes Ruiz Desviat, Spain; Haiyan Zhou, United Kingdom; Camilla Foged, Denmark; Cecilia Jimenez Mallebrera, Spain; Michela Denti, Italy; Veronika Karcagi, Hungary; Sandra Alves, Portugal; Liliana Matos, Portugal; Francisca Coutinho, Portugal; Annemieke Aartsma-Rus, the Netherlands

WEDNESDAY 22nd February 2023

Morning

08:30 Collection of accreditations

Signing of COST attendance sheets

9:00 Welcome to participants and outline of the Action.

Virginia Arechavala-Gomez (DARTER Chair, Ikerbasque/Biocruces Bizkaia HRI, Spain)

09:25-10:50 WORKING GROUP 1 – DELIVERY SYSTEMS

Chair: Camila Foged

-Overview and achievements WG1: **Camila Foged** (University of Copenhagen, Denmark) (10 min)

-Invited talk **Tom Baladi** - *The challenges of delivery* (AstraZeneca, Sweden) (20min +5 Q&A)

Selected short talks (10 min + 5 min Q&A)

- 1- **Julen Rodríguez Castejón** - *Gene supplementation with non-viral vectors for the treatment of Fabry disease* (University of the Basque Country (UPV/EHU), Spain)
- 2- **Marina Beraza-Millor** - *Novel substrate reduction therapy with siRNA formulated in golden lipid nanoparticles for the treatment of Fabry Disease* (University of the Basque Country (UPV/EHU), Spain)
- 3- **Maria Maloverjan** - *Enhancing the efficiency of messenger RNA delivery with cell-penetrating peptides in cell culture and in vivo* (University of Tartu, Institute of Technology, Estonia)

10:50-11:20 Coffee break & Poster / Sponsor boot viewing

Chillida Room, first floor

11:20-12:50 SELECTED ORAL COMMUNICATIONS (Session I)

Chairs: Ainara Vallejo-Illarramendi & Liliana Matos

Selected short talks (10 min + 5 min Q&A)

- 1- **Laura Prats Mari** - *DNA double-strand break synapsis and ligation is promoted by a small hairpin of long noncoding RNA NIHCOLE* (CIMA, University of Navarra, Spain)
- 2- **Giuseppina Covello** - *Isoform-specific siRNAs: A potential therapeutic approach for 4R tauopathies* (University of Padua, Italy)
- 3- **Ilaria Brentari** - *Validation of therapeutic siRNAs in hiPSCs-derived neurons, a model of FTDP-17* (University of Trento, Italy)
- 4- **Jinhong Meng** - *Mutant allele-specific silencing of SPTLC1 by antisense oligonucleotides to treat Hereditary Sensory Neuropathy Type 1A* (UCL Great Ormond Street Institute of Child Health, United Kingdom)
- 5- **Mathilde Blitek** - *Development of a combined antisense therapy for the treatment of Duchenne Muscular Dystrophy* (UVSQ U1179 Biotherapies for neuromuscular diseases, France)
- 6- **Nuria Suárez Herrera** - *Implementing oligo-walk to understand and rescue exon skipping caused by the frequent intronic ABCA4 variant c.4253+43G>A* (Radboud University Medical Center, the Netherlands)

12:45-13:15 NETWORKING SESSION: ELEVATOR PITCHES FROM PIs

5-minute presentations on research topics and interests (time strictly enforced)

Chair: Alex Garanto

- 1- **Virginia Arechavala-Gomez**. Neuromuscular disorders. Biocruces Bizkaia HRI. Spain.
- 2- **Ling Peng**. Nanovectors for nucleic acid delivery. Centre Interdisciplinaire de Nanoscience de Marseille, CNRS, Aix-Marseille University. France.
- 3- **Puri Fortes**. Non coding RNAs in therapy. CIMA. University of Navarra. Spain.
- 4- **Sandra Alves**. ASOs for lysosomal storage diseases. National Institute of Health Doutor Ricardo Jorge. Portugal.
- 5- **Saulius Serva**. Use of natural nanoscaffolds for delivery of bioactive compounds. Vilnius University. Lithuania.

13:20-14:30 Lunch break & Poster / Sponsor boot viewing

Chillida Room, first floor

14:30-15:45 COLLABORATIVE SESSION: ELEVATOR PITCHES FROM PI

5-minute presentations on research topics and interests

Chair: Puri Fortes

- 1- **Sebastien Ulrich**. Design of smart gene delivery vectors. Discovery of self-produced targeting siRNA vectors. CNRS - IBMM, Montpellier. France.
- 2- **Álvaro Somoza**. Oligonucleotide conjugation to gold, iron oxide, and albumin nanoparticles. IMDEA Nanociencia. Spain.
- 3- **Alberto Malerba**. Gene therapy applications and antisense therapeutics for the treatment of neuromuscular diseases. Royal Holloway University of London. UK.
- 4- **Lorea Blázquez**. RNA misprocessing in neurological disorders to develop RNA-targeting therapeutic strategies. Biodonostia. Spain.
- 5- **Haiyan Zhou**. New approach design, modeling system, delivery and toxicology. University College London. UK.
- 6- **Anna Scomparin**. β -Cyclodextrin-based polymers. University of Turin. Italy.
- 7- **Lourdes Desviat**. RNA Therapies to correct splicing defects in neurological disorders. Universidad Autónoma de Madrid. Spain.
- 8- **Michela Denti**. RNA as a tool and as a target for FrontoTemporal Dementia (FTD). Department CIBIO, University of Trento. Italy.
- 9- **Javier Ramón**. Tissue engineering, cell lines, new disease models. Biosensors for Bioengineering. IBEC. Spain.
- 10- **Marta Guerra Rebollo**. Development of proprietary polymers as biomaterials to formulate polymeric nanoparticles. Institut Químic de Sarrià (IQS). Spain.
- 11- **Malgorzata Honcharenko**. Bioconjugation and targeted delivery of oligonucleotides. Karolinska Institutet. Sweden.

15:45-16:15 Poster teasers (2 min each)

Chair: Puri Fortes

1. **Christina Galanakou** - *Nucleic acid therapeutics hold great promise for precision cancer management* (Centre Interdisciplinaire de Nanoscience de Marseille, CNRS, Aix-Marseille University, France)
2. **Ane Rubio** - *Splicing misregulation as a source of new therapeutic targets in glioblastoma* (Biodonostia, Spain)
3. **Alessandro Michienzi** - *RNA-based therapeutic approaches for Aicardi-Goutières Syndrome* (University of Rome Tor Vergata, Italy)
4. **Gisela Nogales-Gadea** - *Three new immortalized myotube cell lines for the study of Steinert disease* (Germans Trias i Pujol Research Institute, Spain)
5. **Laurie Kerkhof** - *Exploring the role of 5' UTR-mediated regulation of ataxin-1 protein expression using splice-modulating antisense oligonucleotides* (Leiden University Medical Center, the Netherlands)
6. **Alexandre Khuu** - *A new quantitative cell-imaging technique to study ASOs productive cellular uptake* (Servier research institute, France)
7. **Irene Pardo** - *Long non-coding RNA FOXD3-AS1 as a therapeutic target for breast and colon cancer* (IMDEA nanociencia, Spain)
8. **Gizem Kursunluoglu** - *Improvement of the siRNA Delivery with Different Shape Gold Nanocones and Evaluated its Efficiency on the Breast Cancer* (Erciyes University, Turkey)
9. **Andrés Ramos Valle** - *Maximizing sequential gene expression and protection with a novel and accessible silica vector technology* (Universidad de Cantabria, Spain)
10. **Paula Milán-Rois** - *Reprogramming cancer cells with non-coding RNAs* (IMDEA nanociencia, Spain)
11. **Ainhoa Martínez** - *Developing an antisense therapy for the frequent PKU splicing variant c.1066-11G>A* (Centro de Biología Molecular Severo Ochoa, Spain)
12. **Patrick Theunissen** - *Development of a tumor-specific cancer vaccine based on lncRNA-derived microproteins* (CIMA, University of Navarra, Spain)
13. **Jing Wu** - *Amphiphilic dendrimer vector for the delivery of siRNA and saRNA in cancer therapy* (Centre Interdisciplinaire de Nanoscience de Marseille, CNRS, Aix-Marseille University, France)
14. **Beatriz Llamusi** - *Lead antimiR-23b drug rescues cell phenotypes in a range of Myotonic Dystrophy genetic backgrounds* (Arthex Biotech, Spain)

16:15 Group Picture

16:25-18:30 POSTER SESSION & NETWORKING with COFFEE

Even numbered posters

18:30-21:30 EVENING NETWORKING ACTIVITY: [Pintxo crawl tour](#) (to be booked in advance and paid for individually). Meeting point: Bizkaia Aretoa.

THURSDAY 23rd February 2023

Morning

8:45 Signing of COST attendance sheets.

09:00-10:30 WORKING GROUP 2 – MODEL SYSTEMS

Chairs: Haiyan Zhou & Alex Garanto

-Overview and achievements by working group leaders: **Alex Garanto** (Radboud University Medical Center, the Netherlands) & **Haiyan Zhou** (UCL, United Kingdom) (20 min)

-Invited talk **Ronald Buijsen** - *Optimizing ASO delivery in 2D and 3D hiPSC-derived neuronal in vivo models* (Leiden University Medical Center, the Netherlands) (15 min + 5 min Q&A)

Selected short talks (10 min + 5 min Q&A)

- 1- **Andrea López Martínez** - *Characterisation of cell culture models of myotonic dystrophy type I by In-Cell Western technology* (Biocruces Bizkaia HRI, Spain)
- 2- **Ainoa Tejedera Villafranca** - *Functional bioengineered 3D skeletal muscle model of Duchenne muscular dystrophy that reproduces disease phenotypes and evaluates potential therapeutic compounds* (Institute for Bioengineering of Catalonia, Spain)
- 3- **Tomasz Tomkiewicz** - *Correction of the splicing defect caused by a recurrent variant in ABCA4 (c.769-784C>T) that underlies Stargardt disease* (Radboud University Medical Center, the Netherlands)

10:30-11:00 Coffee break & Poster / Sponsor boot viewing
Chillida Room, first floor

11:00-12:30 SELECTED ORAL COMMUNICATIONS (Session II)

Chairs: Olatz Villate

Selected short talks (10 min + 5 min Q&A)

- 1- **Madalen Arribas** - *Solid lipid nanoparticles as delivery systems of a highly conserved sequence of SARS-COV2 for vaccine development* (University of the Basque Country (UPV/EHU), Spain)
- 2- **María Rodríguez Hidalgo** - *ABCA4 c.6480-35A>G, a novel branchpoint variant susceptible to be corrected by antisense oligonucleotides* (IIS Biodonostia, Spain)
- 3- **Ngoc Lu-Nguyen** - *Long-term systemic antisense treatment of a mouse model displaying chronic FSHD-like pathology* (Royal Holloway University of London, United Kingdom)
- 4- **Liliana Matos** - *Exploring an antisense oligonucleotide exon skipping therapeutic strategy to correct a frequent causing mutation in Mucopolisaccharidoses II* (National Health Institute Dr. Ricardo Jorge, Portugal)
- 5- **Omer Aydin** - *Combination Therapy with Doxorubicin and Autophagy-related LC3 siRNA effectively treats Triple Negative Breast Cancer* (Erciyes University, Turkey)
- 6- **Maria Francisca Coutinho** - *Modelling Lysosomal Storage Disorders in an innovative way: Establishment and Characterization of Stem Cell Cultures from the Dental Pulp of Mucopolysaccharidoses type II patients* (National Health Institute Dr. Ricardo Jorge, Portugal)

12:30-14:00 Lunch break & Poster / Sponsor boot viewing
Chillida Room, first floor

Afternoon

14:00-16:00 ROUND TABLE / TIPS FOR PUBLISHING NEGATIVE RESULTS AND OPEN SCIENCE
Chairs: Michela Denti, Annemieke Aartsma-Rus and Alberto Malerba.

Overview short lectures:

- **Virginia Arechavala-Gomez** - *On "negative" results and why they should be published* (Biocruces Bizkaia HRI, Spain)
- **Devang Mehta** - *How to publish negative results and reform the positivity bias in research culture* (Katholieke Universiteit Leuven, Belgium)
- **Johan Rooryck** - *Plan S and CoalitionS: Policies and tools* (Coalition S, the Netherlands)
- **Alicia Estacio Gomez** - *"How to publish your negative results, and other research outputs on Open Research Europe"* (Open Research Europe, United Kingdom)

Selected short talks (10 min + 5 min Q&A)

- 1- **Ane Velasco** - *Functional characterization of c.709-1G>A mutation in GRN gene and investigation of splicing modulation strategy* (Bilbao, Biodonostia HRI, Spain)
- 2- **Ona Navikaite** - *Development of a novel antibody-mediated antisense oligonucleotide delivery system* (Royal Holloway University of London, United Kingdom)

Round table

16:00-16:30 NETWORKING SESSION: ELEVATOR PITCHES FROM PI

5-minute presentations on research topics and interests

Chair: Aurélie Goyenvalle

- 1- **Amelia Aranega**. miRNAs in satellite cells involved in muscle regeneration. University of Jaén. Spain.
- 2- **Nikolaos Balatsos**. Deadenylases and exosomes to modulate the degradation of specific RNAs. Department of Biochemistry and Biotechnology, University of Thessaly. Greece.
- 3- **Carlos Ciudad**. PolyPurine Reverse Hoogsteen oligonucleotides for gene silencing, editing, repair or biosensors. University of Barcelona. Spain.
- 4- **Juanma Fernández Costa**. Organ-on-chip devices integrated with biosensor as platforms to drug testing for neuromuscular diseases. Institute for Bioengineering of Catalonia. Spain.
- 5- **Javier Ruiz Eder**. Inherited Retinal Dystrophies: ASO and small molecule therapies and diagnostic tools. UPV/EHU Biodonostia HRI Spain.
- 6- **Clara I. Rodríguez**. Stem Cell and Cell Therapy Laboratory. Biocruces Bizkaia HRI. Spain.

16:30-18:30 POSTER SESSION & NETWORKING with COFFEE

Odd numbered posters.

19:30 NETWORKING ACTIVITIES: Grab a bite/drink at [Mercado de la Ribera](#) (anytime from 20:00 onwards, no need to book), PLUS: 19:00-21:30 Outdoors escape room in Bilbao's old town (to be [booked](#) and paid for individually). Plazuela de Santiago (Casco Viejo/Old Town).

FRIDAY 24th February 2023

Morning

8:45 Signing of COST attendance sheets.

9:00-9:15 How to fill in your COST reimbursement claims.

Anita Remmelzwaal (Grant holder manager, Leiden University Medical Center, the Netherlands)

09:15-10:30 WORKING GROUP 3 – SAFETY AND TOXICOLOGY

Chair: Aurélie Goyenvalle

- Overview lecture “*Considerations in the Preclinical Assessment of the Safety of Antisense Oligonucleotides*” by **Aurélie Goyenvalle** (University of Versailles, France)
- Overview and achievements by working group leaders: Aurélie Goyenvalle (University of Versailles, France) & Cecilia Jimenez Mallebrera (Hospital Sant Joan de Deu, Spain)

Selected short talks (10 min + 5 min Q&A)

- 1- **Flavien Bizot** - *Histone deacetylase inhibitors improve antisense-mediated exon-skipping efficacy in mdx mice* (University of Versailles, France)
- 2- **Irene Vazquez-Dominguez** - *Assessing differences in splicing modulation and safety amongst different modified antisense oligonucleotides in the retina* (Radboud university medical center, the Netherlands)

10:30-11:00 Coffee break & Poster / Sponsor boot viewing

Chillida Room, first floor

11:00-12:30 SHORT TERM SCIENTIFIC MISSIONS PRESENTATIONS

Chairs: Sandra Alves & Aristides López-Marquez

- Overview and introduction by the chairs

Selected short talks (10 min + 5 min Q&A)

- 1- **Jose García Coll** - *Dynamic Covalent Polymers for mRNA complexation* (Université de Montpellier, France)
- 2- **Luciana Moreira** - *CRISPR-Cas9 in zebrafish for generation of a knock-out model for MPS III (Sanfilippo Syndrome)* (National Health Institute Dr. Ricardo Jorge, Portugal)
- 3- **Laura Taina-González** - *Studying the impact of formulation method in Lipid Nanoparticles (LNPs)* (University Santiago de Compostela / DIVERSA Technologies, Spain)
- 4- **Katarzyna Owczarek** - *Target identification for antiviral therapy drugs* (Jagiellonian University, Poland)
- 5- **Mirella Barboni** - *Retinal dystrophins and the retinopathy of Duchenne muscular dystrophy* (Semmelweis University, Hungary)

12:30-13:15 COLLABORATIVE SESSION: ELEVATOR PITCHES FROM PI

5-minute presentations on research topics and interests

Chair: Virginia Arechavala-Gomez

- 1- **Gisela Nogales Gadea**. Biomarkers and treatments for neuromuscular and neuropediatric diseases. Germans Trias i Pujol Research Institute. Spain.
- 2- **Omer Aydin**. Smart polymer-based and gold nanoparticles for delivery. Erciyes University. Turkey.
- 3- **Aur lie Goyenville**. Splice switching approaches for neuromuscular disorders, specific delivery of NATs to muscles and CNS, evaluation of ASO-mediated toxicities. University of Versailles. France.
- 4- **Ainara Vallejo**. Calcium dysregulation in muscle, heart and neurons. UPV/EHU-Biodonostia HRI. Spain.
- 5- **Alex Garanto**. Molecular Therapies for Inherited Retinal and Neurometabolic diseases. Radboudumc. The Netherlands.
- 6- **Dusanka Savic-Pavicevic**. Genetic and epigenetic basis of phenotype variability in rare neuromuscular disorders. University of Belgrade - Faculty of Biology. Serbia.
- 7- **M nica L pez Fanarraga**. Self-propelled DNA delivery nanoparticles. University of Cantabria. Spain

13:15-14:30 Lunch break & Poster / Sponsor boot viewing
Chillida Room, first floor

Afternoon

14:30-15:15 WORKING GROUP 4 – DISSEMINATION

Chairs: Michela Denti & Francisca Coutinho

- Overview lecture: *Why did we choose to have a WG on Science Communication?* by **Michela Denti** (University of Trento, Italy)

Achievements of WG4:

- 1- Training Schools by **Michela Denti** (University of Trento, Italy)
- 2- Social media and DARTER website by **Francisca Coutinho** (National Health Institute Dr. Ricardo Jorge, Portugal)
- 3- Online seminars by **Liliana Matos** (National Health Institute Dr. Ricardo Jorge, Portugal)
- 4- Mentoring Programme by **Irene Vazquez-Dominguez** (Radboudumc, the Netherlands)
- 5- ITC grants by **Veronika Karcagi** (Istenhegyi Genetic Diagnostic Centre, Hungary)

15:15-16:00 FINAL REMARKS & CLOSING
Virginia Arechavala-Gomez, DARTER Chair

16:00-19:00 AFTERNOON NETWORKING ACTIVITY: Visit the Guggenheim Museum (5-min walk from the meeting venue). Buy your tickets at the door or: <https://tickets.guggenheim-bilbao.eus/en>



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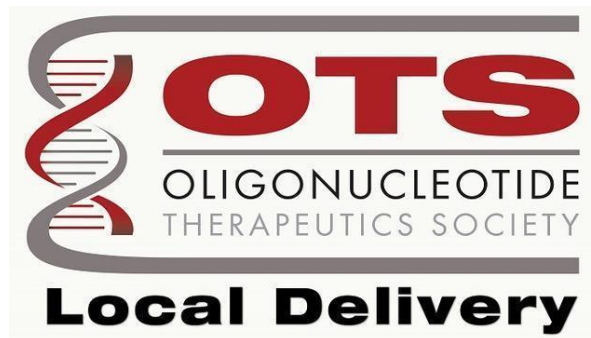
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