

## FINAL MEETING

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

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

#### Local organizing committee

M<sup>a</sup> Angeles Solinís Aspiazu, University of the Basque Country UPV/EHU  
Ainara Vallejo Illarramendi, University of the Basque Country UPV/EHU  
Olatz Villate Bejarano, Biocruces Bizkaia Health Research Institute  
Virginia Arechavala Gomeza, Biocruces Bizkaia Health Research Institute

#### Scientific committee

Puri Fortes, Spain  
Alex Garanto, the Netherlands  
Aur lie Goyenvalle, France  
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  
Virginia Arechavala Gomeza, Spain

WEDNESDAY 22<sup>nd</sup> February 2023

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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)

09:30-10:45 WORKING GROUP 1 – DELIVERY SYSTEMS

*Chair: Camila Foged*

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

*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)

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

Chillida Room, first floor

11:15-12:45 SELECTED ORAL COMMUNICATIONS (Session I)

*Chairs: to be confirmed*

*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 Padua, 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.

**13:15-14:30 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- **Alex Garanto**. Molecular Therapies for Inherited Retinal and Neurometabolic diseases. Radboudumc. Netherlands.
- 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**.  $\beta$ -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)**

TBA

16:15 Group Picture

16:15-18:30 POSTER SESSION & NETWORKING with COFFEE  
Odd numbered posters

18:30-21:30 EVENING NETWORKING ACTIVITY (to be booked and paid for individually).

**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)

*Invited talk (15+5 Q&A)*

**Ronald Buijsen** - *Optimizing ASO delivery in 2D and 3D hiPSC-derived neuronal in vivo models* (Leiden University Medical Center, the Netherlands)

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

- 1- **Andrea López Martínez** - *Characterisation of cell culture models of myotonic dystrophy type 1 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: to be confirmed*

*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 Mucopolipidosis 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** - *Title TBC* (Katholieke Universiteit Leuven, Belgium)
- **Johan Rooryck** - *Plan S and CoalitionS: Policies and tools* (Coalition S, the Netherlands)
- **Alicia Estancio Gomez** - *Title TBC* (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 Ederra**. Inherited Retinal Dystrophies: ASO and small molecule therapies and diagnostic tools. Biodonostia HRI. Spain.

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

Even numbered posters.

**19:00-21:30 EVENING NETWORKING ACTIVITY** (to be booked and paid for individually).

**FRIDAY 24th February 2023**

**Morning**

**8:45** Signing of COST attendance sheets

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

**Annemieke Aartsma-Rus** (Grant holder, Leiden University Medical Center, Netherlands)

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

*Chair: Aurelie Goyenvalle*

Overview lecture “*Considerations in the Preclinical Assessment of the Safety of Antisense Oligonucleotides*” by **Aur lie Goyenvalle** (University of Versailles, France)

Overview and achievements by working group leaders: **Aur lie 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 L pez-Marquez*



Overview and introduction by the chairs

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

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

#### 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- **Aurelie Goyenvalle**. 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 - IIS Biodonostia. Spain.
- 5- **Álvaro Somoza**. Oligonucleotide conjugation to gold, iron oxide, and albumin nanoparticles. IMDEA Nanociencia. Spain.
- 6- **Dusanka Savic-Pavicevic**. Genetic and epigenetic basis of phenotype variability in rare neuromuscular disorders. University of Belgrade - Faculty of Biology. Serbia.
- 7- **Mónica López 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 and achievements by working group 4 leaders:

- + ITC grants summary
- + Website overview
- + Training schools
- + Seminars
- + Mentoring

15:15-16:00 FINAL REMARKS & CLOSING

Virginia Arechavala-Gomez, DARTER Chair

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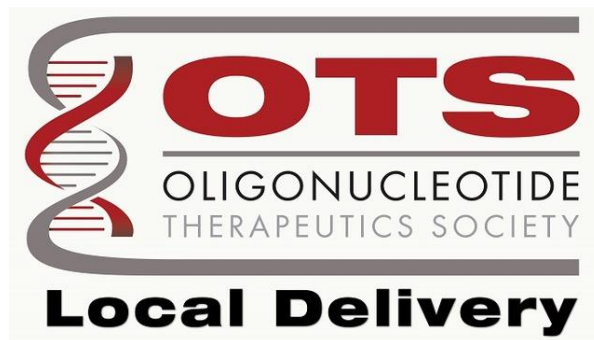


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