

INAUGURAL MEETING

DELIVERY OF ANTISENSE RNA THERAPIES (DARTER) COST ACTION CA17103

25th-28th February 2019

Salón de Actos, Hospital Universitario Cruces,
Biocruces Bizkaia Health Research Institute, Barakaldo, Spain

MONDAY 25th February 2019

Morning

10:15 Collection of accreditation

Signing of COST attendance sheets

10:30 Welcome to participants

Luis Castaño, (Scientific director, Biocruces Bizkaia Health Research Institute)

11:00 Welcome to participants and outline of the Action.

Virginia Arechavala-Gomez (Action Chair, Biocruces Bizkaia Health Research Institute, Spain)

11:15 Short networking activity/Introductions

DELIVERY SYSTEMS (Working group 1)

All participants invited to attend this session, facilitated by working group 1 leaders: Taavi Lehto (Karolinska Institute, Sweden) & Camila Foged (University of Copenhagen, Denmark)

11:35 Short lecture 1: Overview and the present status of the various naked ASO chemistries in the pre-clinic and clinical settings.

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

12:00 Short lecture 2: Next generation ASO chemistries and various functionalized chemical ASO conjugates

Suzan Hammond (University of Oxford, United Kingdom)

12:20 Short lecture 3: Nanoparticle-based delivery systems and other novel materials for delivery of ASOs.

Taavi Lehto (Karolinska Institute, Sweden)

----- Lunch break -----

***Any participant interested in visiting the institute during this break, please sign up here:
<https://goo.gl/forms/ON3pTkgY3nENgZmY2>

Afternoon

14:00 Short lecture 4: Local administration of ASOs

Camilla Foged (University of Copenhagen, Denmark)

14:20 Overview lecture: Current status and outstanding challenges with the delivery and translation of antisense oligonucleotide therapeutics

Steve Hood (GlaxoSmithKline, United Kingdom)

15:00-19:00 POSTER SESSION & COFFEE ***BRING YOUR OWN BISCUIT "COMPETITION"***

All participants are invited to bring a poster showcasing the interests and expertise of their research groups. Preferably, participants should prepare a poster for this meeting, but posters presented at other congresses would be acceptable. One poster per research group/principal investigator will also be preferable.

If you are bringing a poster, please let us know by the 18th of February using this link:

<https://goo.gl/forms/Hsj9eKgOOjUOOxGB3>

Participants are encouraged to **bring a box of their favorite local biscuits** for the coffee break. (We do not want fancy biscuits, just those you may buy in bulk in the supermarket. As we have participants from many different countries, we expect to have quite a selection.)

19:30-22:30 EVENING NETWORKING ACTIVITY: PINTXO CRAWL (€)

To participate, please contact the company organising this activity at this address:

grupos2@stipaisvasco.com

TUESDAY 26th February 2019

Morning

9:20 Signing of COST attendance sheets

9:30 Working group one- Brainstorming session- Delivery hurdles for antisense therapies and proposal of activities.

Facilitated by working group 1 leaders: Taavi Lehto (Sweden) & Camila Foged (Denmark)

All participants, but particularly participants of working group one (delivery systems) are invited to participate in this session.

DISSEMINATION (Working group 4)

All participants invited to attend this session, facilitated by working group 4 leaders: Marisol Montolio (Duchenne Parent Project, Spain) & Michela Denti (University of Trento, Italy)

10:30 Overview lecture: Open Access to Publications and Data within Horizon 2020

Alicia Fátima Gómez Sánchez (Spanish Foundation for Science and Technology – FECYT, Spain)

----- Coffee break -----

11:45 Overview lecture: Science dissemination for all

Daniel Closa Autet (Institute of Biomedical Research of Barcelona, Spain)

12:30 DARTER Webpage presentation

Magdalena Guzowska (Warsaw University of Life Sciences, Poland)

----- Lunch break -----

MODEL SYSTEMS (Working group 2)

All participants invited to attend this session

14:00 Introduction to working group 2:

Facilitated by working group 2 leaders: Alex Garanto (Radboud University, Netherlands,) & Gisela Gaina (University of Bucharest, Romania)

14:20 Short lecture 1: Use of several cellular models (incl. iPSC technology) to assess and correct splicing defects in inherited retinal diseases

Rob W.J. Collin (Radboud University, Netherlands)

14:40 Short lecture 2: Use of different small models for Duchenne and parameters to assess efficiency upon ASO treatment.

Aurelie Goyenville (University of Versailles, France)

15:00 Short lecture 3: Use of large animal models for ASO assessment

Sabine Krause (Friedrich Baur Institute, Germany)

15:20 Short lecture 4: Organ-on-a-chip: concept to application, structural features, working principles, potential and feasibility for biomedical applications.
Ronald Buijsen (Leiden University Medical Centre, Netherlands)

----- Coffee break -----

16:00 Elevator pitch/short results 1.

Participants are invited to present a 5-minute summary of a research topic or research result. Negative/inconclusive results are particularly welcome. The 5-minute limit will be STRICTLY reinforced.

- 1 Unexpected splicing outcomes/AON effects in humanized mouse models.** Lourdes R. Desviat, Universidad Autonoma de Madrid, Spain
- 2 Nanoencapsulation and advanced characterisation for enhanced delivery of RNA therapeutics - helping translation towards the clinic.** Sven Even Borgos, SINTEF, Norway
- 3 U1 small nuclear RNA-mediated exon skipping to correct Retinitis Pigmentosa GTPase-Regulator (RPGR) splice defect.** Guiseppeina Covello, D-Cibio University of Trento, Italy
- 4 Alternative Splicing modulation a novel therapy for lung cancer treatment** Jordi Hernández Ribera, CRG, Spain
- 5 DM1 patients with variant repeats – outliers in therapeutic approaches?** Jovan Pesovic, University of Belgrade-Faculty of Biology, Serbia

17:00 Working group two- Brainstorming session- Model systems for antisense therapies and proposal of activities

Facilitated by working group 2 leaders: Alex Garanto (Netherlands) & Gisela Gaina (Romania)
All participants, but particularly participants of working group two (model systems) are invited to participate in this session.

WEDNESDAY 27th February 2019

Morning

9:20 Signing of COST attendance sheets

SAFETY AND TOXICOLOGY

All participants invited to attend this session, facilitated by working group 3 leaders: Aurelie Goyenville (University of Versailles, France) & Elena Ionica (University of Bucharest, Romania)

9:30 Overview lecture: Preclinical safety assessment of Antisense oligonucleotides

Patrik Andersson (AstraZeneca, Sweden)

10:20 Short lecture 1: Evaluation of tox/immune effects of ASO in the brain

Willeke Van Roon-Mom (Leiden University Medical Center, Netherlands)

10:40 Short lecture 2: Evaluation of tox in other organs

Cathaline de Besten (Toxicology Department, ProQR, Netherlands)

----- Coffee break -----

11:30 Overview Lecture: an EU regulator's perspective on the drug development of antisense oligonucleotides

David Jones (Medicines and Healthcare Products Regulatory Agency, United Kingdom)

12:20 Elevator pitch/short results 2.

Participants are invited to present a 5-minute summary of a research topic or research result. Negative/inconclusive results are particularly welcome. The 5-minute limit will be STRICTLY reinforced.

- 1 Biocompatible and biodegradable nanosystems for gene therapy applications.** María de la Fuente Freire, Health Research Institute of Santiago de Compostela, Spain
- 2 The nucleotide-modified RNA-aptamer as the potential carrier molecule to target the malignant B cells.** Irina Holodnuka Kholodnyuk, Riga Stradins University, Latvia
- 3 High frequency of mutations affecting the splicing process among Basque patients with Inherited Retinal Diseases.** Javier Ruiz Ederria, Biodonostia Health Research Institute, Spain
- 4 Bacteria penetrating peptides (BPPs) for in vivo delivery of antisense antibiotics.** Peter Nielsen, University of Copenhagen, Denmark
- 5 Nintedanib decreases muscle fibrosis and improves muscle function in a murine model of dystrophinopathy.** Esther Fernández, Institut de Recerca Sant Pau, Spain

----- Lunch break -----

14:15 Working group three- Brainstorming session- Toxicity hurdles for antisense therapies and proposal of activities

Facilitated by working group 3 leaders: Aurelie Goyenvalle (University of Versailles, France) & Elena Ionica (University of Bucharest, Romania).

All participants, but particularly participants of working group three (safety and toxicity) are invited to participate in this session.

15:15 How to fill in your COST reimbursement claim

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

15:30 General meeting wrap up and introduction of negative results and ECI task groups.

Virginia Arechavala Gomeza (Action Chair, Biocruces Bizkaia Health Research Institute, Spain)

16:00 Negative results task group meeting

Participants interested in this task group are invited to participate in this session to plan the future activities of this task group.

16:45 Early Career Investigators' group meeting

Early Career Investigators are invited to participate in this session to plan future activities of this task group.

THURSDAY 28th February 2019

Morning

Management Committee Meeting (Only management committee members will be invited to this meeting)

Summary of Bilbao meeting and planning of grant period 2