



# Synergistic Meeting on Oligonucleotide Therapeutics

**Thursday 27th February**

**08:45-09:00. Welcoming:** Organizing committee (Beatriz Llamusi, Santiago Vernia, Virginia Arechavala, Álex Garanto, Aurelie Goyenvalle) Susana Rodriguez Navarro, IBV-CSIC director

**09:00-9:45. Key note session:** Ramón Eritja (IQAC)  
*"The development of therapeutic oligonucleotides, an historical perspective".*

**09:45- 10:45. Screening platforms and readouts.**

Chair: Virginia Arechavala (Biobizkaia)

Speakers and titles:

- *Álex Garanto, Associate professor at Radboud University. "Advanced human in vitro models to accelerate therapeutic development".*
- *Juan Martínez, CSO at CERTEST. "Choosing Between Targeted LNPs or Direct Conjugation for Oligonucleotide Delivery"*
- *Vicent Nebot, CTO at Curapath. "Leveraging the high-throughput and vertically integrated platforms for accelerated nanoparticle formulation development and GMP manufacturing".*

**10:45- 11:15. . Coffee break with poster session.** Posters will be showcased in the Hall virtual screens

**11:15-12:30. Flash presentations selected among abstracts received**

Chair: Santiago Vernia (IBV-CSIC)

Speakers and titles:

- *Andrea López-Martínez. "Characterization of myotonic dystrophy type I cell cultures for drug screening using In-Cell Western".*
- *Mathilde Blitek. "Improving angiogenesis ameliorates the efficacy of ASO-based exon-skipping for the treatment of Duchenne muscular dystrophy".*
- *Noemi Rotllán. "Tailored rHDL with Bioactive Antiatherosclerotic miRNAs".*
- *Giuseppina Covello. "Isoform-specific siRNAs as a potential therapeutic approach for tau-dependent neurodegenerative diseases".*
- *Dulce Peris-Moreno. "Targeting Musashi-2 with Antisense Oligonucleotides: A Therapeutic Approach to Mitigate Muscle Pathology in Myotonic Dystrophy Type 1".*
- *Fernando Pastor. "Oligonucleotide aptamers as a potential toolbox for RNAi therapeutics".*
- *Milly Coxhead. "T-REX: Engineered exosomes for combined loading and cell-specific delivery of RNA".*
- *Ana Delgado "Delivery of Polypurine Reverse Hoogsteen therapeutic oligonucleotides by using a novel tricationic compound TRIFAPY".*

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**12:30-13:00. Speed networking event.**

Interactive session for fast-dating other attendees.  
Get ready to introduce yourself in 3 min.

**13:00-14:00 Lunch**

**14:00-16:00. Therapeutic targets and preclinical development.**

Chair: Álex Garanto (Radboud university medical center)

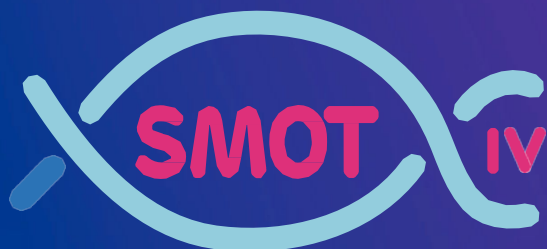
Speakers and titles:

- Patrik Andersson, Senior Director at AstraZeneca. *"Nonclinical safety assessment of oligos"*.
- Steven Van Cruchten, Professor at University of Antwerp. *"Minipigs as an alternative to nonhuman primates in safety testing of antisense oligonucleotides"*.
- Lourdes R. Desviat, Professor at University Autonoma of Madrid. *"Antisense oligonucleotides as a therapeutic strategy for correcting PAH splicing defects in weakly defined exons"*.
- Sandor Batkai, Co-founder of Cardior Pharmaceuticals. *"Therapeutic targeting miRNAs in the chronic disease space"*.
- Anna Altieri, Translational Research associate at Resalis Therapeutics. *"Promoting superior weight loss with lean mass preservation: RES-010 enters clinical trials to revolutionize obesity treatment"*

**16:00-17:00 Coffee break with Poster session**

Posters will be showcased in the Hall virtual screens





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## **09:00-11:00. Optimizing design and chemistry to improve delivery**

Chair: Ramón Eritja.

Speakers and titles:

- Aurélie Goyenvalle, Lab Director at Inserm. "*Tricyclo-DNA: promising antisense oligonucleotides for the treatment of neuromuscular diseases*".
- Álvaro Somoza, Research Professor at IMDEA nanociencia. "*Oligonucleotide-functionalized Gold Nanoparticles as Therapeutics*".
- Pablo Lores, CMC Director at Sylentis. "*Innovation in therapeutics-oligonucleotide manufacturing to sustainably meet future demand*".
- Daniel Bachmann, Business Dev Manager at Bachem. "*A CMC perspective on Oligonucleotide drug delivery*".
- Steffi Kruba Stephen, Scientist at Axolabs. "*Evaluation of GalNAc assemblies for siRNA delivery*".

**11:00-11:30. . Coffee break with poster session.** Posters will be showcased in the Hall virtual screens

## **11:30-12:30. Discussion group: Driving R&D through cross-collaboration.**

Moderator: Beatriz Llamusi (Arthex Biotech)

Participants: Laura Rodríguez, Partner at Invivo Partners

Haiyan Zhou, Professor at University College London

José Terencio, VP at Grifols-GIANT

Begoña García, knowledge transference office, UMH

## **12:30-13:00. Speed partner search.**

- Alex Garanto: "EFFecT: A European Training Program to Foster the Full Therapeutic Potential of Antisense Technology across Tissues"
- Virginia Arechavala: "Beyond the Grant: The Next Chapter for the DARTER Network"
- Haiyan Zhou: "UPNAT:UK Platform of Nucleic Acid Therapy for rare disease treatment"
- Ruben Artero (UV): "ENTRY-DM: Interdisciplinary doctoral training on oligonucleotide-based therapies for myotonic dystrophy"

**13:00-14:00. Lunch**





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**13:00-14:00. Lunch**

**14:00-16:00. Targeted delivery of oligonucleotides.**

Chair: Aurelie Goyenvallé (Inserm)

Speakers and titles:

- Haiyan Zhou, Professor at University College London. *"Saving Sense by Antisense – a Novel Allele-Specific ASO Therapy for Hereditary Sensory Neuropathy"*
- Ekkehard Leberer, Elbiocon, Professor at Technical University of Munich. *"Delivery of therapeutic RNAs into the brain"*
- Bruno Godinho, Senior Scientist at Atalanta Therapeutics. *"New Generation of RNAi Drugs for Therapeutic Gene Silencing in the Central Nervous System"*
- Atze Bergsma, Assistant Professor at Erasmus MC. *"Leashed antisense oligonucleotide-carrying lipid nanoparticles (LASO-LNPs) for efficient delivery of PMO-based ASOs to cultured iPSC-derived skeletal muscle cells"*
- Beatriz Llamusi, CSO at Arthex Biotech. *"Fatty acid conjugation as a BBB shuttle to enable efficient oligonucleotide delivery into the CNS"*

16:00-16:10. Wrap up session including Best Flash presenter award: Organizing committee

**16:10-17:00. Coffee break with poster session**

Posters will be showcased in the Hall virtual screens