



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

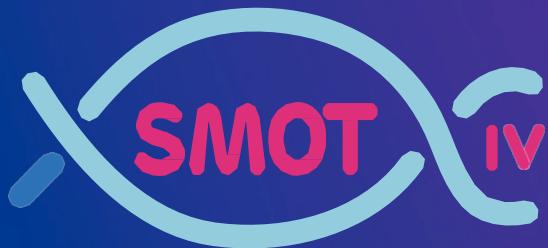
Chair: Santiago Vernia (IBV-CSIC)

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".
- Noemí 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

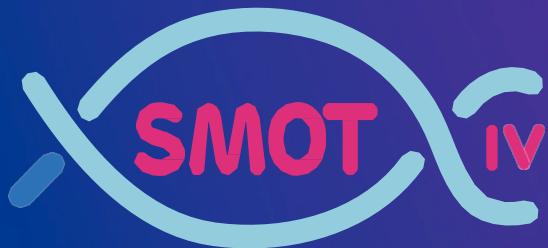
José Terencio, VP at Grifols-GIANT

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 Goyenvalle (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