

Delivery of Nucleic Acid Therapeutics II: Biology, Engineering and Development

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Friday, April 5, 2024

07:30 – 09:00	Breakfast Buffet (Nesos Restaurant)	
09:00 – 09:25	Setting the Delivery Stage Steve Dowdy, UCSD, USA	
	<u>Session 1: Clinical Stage Delivery</u> Chair: Art Krieg, USA	
09:25 – 09:50	TfR Ab conjugated oligos for muscle delivery Art Levin, Avidity Biosciences, USA	
09:50 – 10:15	TfR-Fab conjugated oligos for muscle delivery Oxana Beskrovnaya, Dyne Therapeutics, USA	
10:15 – 10:45	Coffee Break	
10:45 – 11:10	CRISPR LNPs Kristy Wood, Intellia Therapeutics, USA	
11:10 – 11:35	Design and Evaluation of Novel ionizable Lipids for RNA Delivery Pad Chivukula, Arcturus Therapeutics, USA	
11:35 – 11:50	Delivery of a First-in-Class miR-10b Antagomir to Treat Metastatic Cancer: The Long Winding Road from Pre-Clinical Development to Initial Clinical Experience Zdravka Medarova, Transcode Therapeutics, USA (Short Talk)	1
11:50 – 12:05	Splice-switching oligo conjugates for the treatment of Erythropoietic Protoporphyrria Phil Becker, ETH, Switzerland (Short Talk)	2
12:05 – 14:00	Lunch (Terrace or Nesos Restaurant, depending on weather)	
	<u>Session 2: Endosomal Trafficking and Escape</u> Chair: Steve Dowdy, UCSD, USA	
14:00 – 14:25	LNP Endosomal Escape Anders Wittrup, Lund University, Sweden	
14:25 – 14:50	Harnessing endocytosis to improve the delivery of oligonucleotide-based drugs Marino Zerial, Max Plank Institute, Germany	
14:50 – 15:15	ENDOSCAPE: Triterpene glycoside conjugates allow efficient endosomal escape of diverse targeted payloads Guy Hermans, Supreme Technologies B.V., the Netherlands	3
15:15 – 15:40	Predictive Ex Vivo Models of LNP Behavior Dwight Morrow, Moderna, USA	4
15:40 – 16:00	Coffee Break	
16:00 – 16:25	GaIAhead and PNP, double targeting the way forward! Jim Weterings, Sirnaomics, China and USA	
16:25 – 16:50	Exploring cellular uptake and trafficking of lipid conjugated antisense oligonucleotides Emma Kay, AstraZeneca, Sweden (Short Talk)	5

Friday, April 5, 2024 (continued)

- 16:50 – 17:05 **Deep learning-enhanced single particle tracking reveals intracellular delivery and escape of oligonucleotides**
Nikos Hatzakis, University of Copenhagen, Denmark (**Short Talk**)
- 17:05 – 17:30 **Round Table on Today's Lessons Learned**
- 17:30 – 19:00 Poster Session 1 (Conference Center - with beverages)
- 19:00 – 21:00 Dinner (Sicilian Buffet on Ortigia Terrace)
- 21:00 Social Hour

Saturday, April 6, 2024

07:30 – 09:00	Breakfast Buffet (Nesos Restaurant)	
	<u>Session 3: Delivery Chemistry, Immunity and Escape</u> Chair: Masad Damha, McGill University, Canada	
09:00 – 09:25	Lipidated conjugates for CNS delivery Anastasia Khvorova, University of Massachusetts, USA	
09:25 – 09:50	Chemical evolution of artificial peptide carriers for nucleic acid delivery Ernst Wagner, Ludwig-Maximilian-University of Munich, Germany	7
09:50 – 10:15	Selective Organ Targeted (SORT) LNPs: focus on lung delivery and PCD program Dan Siegwart, UT Southwestern/ReCode, USA	
10:15 – 10:45	Coffee Break	
10:45 – 11:10	Nucleic acid immunity: the key to successful nucleic acid therapeutics Gunther Hartmann, University of Bonn Hospital, Germany	
11:10 – 11:25	Designing Thermostable Hermes lipopolyplex nanoparticles for mRNA and synthetic hpDNA delivery Amy Walker, 4BaseBio, UK (Short Talk)	8
11:25 – 11:40	Visualization of lipid nanoparticle disintegration and localized endosomal membrane damage Johanna M. Johansson, Lund University, Sweden (Short Talk)	9
11:40 – 11:55	Molecular Nano-Motors (MNMs): A novel delivery modality for oligonucleotides Hamutal Ben Dov, Aposense, Israel (Short Talk)	10
11:55 – 14:00	Lunch (Terrace or Nesos Restaurant, depending on weather)	
	<u>Session 4: New Delivery Strategies</u> Chairs: Ernst Wagner, Ludwig-Maximilians-Universität, Germany	
14:00 – 14:25	Recent Discoveries in ExtraHepatic Delivery of RNA Cargoes Pete Smith, ReNegade, USA	
14:25 – 14:50	Centyrin-targeted siRNA Conjugates for Extra-hepatic Delivery: ABX1100, a CD71 Centyrin-Gys1 siRNA Conjugate for Treatment of Pompe Disease Sukumar Sakamuri, Aro Biotherapeutics, USA	11
14:50 – 15:15	Engineering Exosomes to Enable and Create Improved Genetic Medicines Tony De Fogerolles, Evox Therapeutics Limited, United Kingdom	
15:15 – 15:40	LNP delivery Guarav Sahay, Oregon State University, USA	
15:40 – 16:00	Coffee Break	
16:00 – 16:14	In Vivo Delivery of RNA Gene Writers to the Liver and Beyond William Salomon, Tessera, USA (Short Talk)	12
16:15 – 16:30	“I translate, therefore I deliver”: understanding LNP delivery capabilities on a granular scale to reveal therapeutic opportunities Charlotte Dunne, Pantherna, Germany (Short Talk)	13

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| 16:30 – 17:00 | Just a Little Delivery Reality Check
Paul Burke, Burke Bioventures LLC, USA |
| 17:00 – 18:30 | Poster Session 2 (Conference Center with beverages) |
| 18:30 – 21:00 | Dinner (Nesos Restaurant) |
| 21:00 | Social Hour |

Sunday, April 7, 2024

- 07:30 – 09:00 Breakfast Buffet (Nesos Restaurant)
- Session 5: Non-Viral and Viral Gene Therapy**
Chairs: Matt Stanton, Generation Bio, USA
- 09:00 – 09:25 **AAV delivery**
Dirk Grimm, Heidelberg University Hospital, Germany
- 09:25 – 09:50 **A Modular, Antibody-Based AAV Retargeting Platform For Efficient And Specific *In Vivo* Gene Delivery**
Leah Sabin, Regeneron, New York, USA
- 09:50 – 10:05 **Development of a human hematopoietic stem cell targeted platform through multiplexed targeting and de-targeting modifications of a common viral gene transfer vector for safe and effective editing of stem cell compartment in vivo. (Short Talk)** 14
Dmitry Shayakhmetov, Emory University, USA
- 10:05 – 10:30 Coffee Break
- 10:30 – 10:55 **DNA LNP delivery**
Matt Stanton, Generation Bio, USA
- 10:55 – 11:20 **Targeting mRNA LNPs to New Cell types**
Stefaan De Koker, Ethernal, Belgium
- 11:20 – 11:45 **LNP Structure**
Jay Kulkarni, Nanovation Therapeutics, Canada
- 12:00 Boxed Lunch distribution (Hotel Lobby)
- 12:45 Meet in lobby for excursion (departing 13:00)
- 13:00 – 17:00 Sightseeing excursion to Roman ruins and Old Town Siracusa
- 18:00 – 18:10 Poster Awards
- 18:10 – 19:00 **Keynote Talk 2 The World of LNPs**
Pieter Cullis, University of British Columbia, Canada
- 19:15 – 21:30 Gala Dinner (Terrace of Nesos Restaurant)
- 21:30 Social Hour

Monday, April 8, 2022

- 07:30 – 09:00 Breakfast Buffet (Nesos Restaurant)
- 09:00– 12:00 Departures

Poster Presentations

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Covadonga Paneda, Altamira Therapeutics, Switzerland
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Daniel Quevedo, METiS Therapeutics, USA
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Florian Mann, Bayer AG, Germany
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John Lewis, Entos Pharmaceuticals, USA
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Vineeta Tripathi, Vitarka Therapeutics Ltd, United Kingdom
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Gavin Garland, University of Cambridge, United Kingdom
7. **Dechapering endosomal escape of oligonucleotides from Lipid Nanoparticles by the single particle** 21
Frank Schulz, University of Copenhagen, Denmark
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Anna Rydzik, AstraZeneca, Sweden
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Sean Bedingfield, Eli Lilly, USA
10. **Innovative development of COVID-19 mRNA vaccine using the gold nanoparticle delivery platform** 24
Jeehyeon Bae, Chung-Ang University, South Korea
11. **Characterization of triterpene-mediated endosomal escape of cholesterol siRNA** 25
Myriam Cerezo-Magaña, Lund University, Sweden
12. **Exploring the Traut's reagent as a versatile bifunctional linker for oligonucleotide conjugates** 26
Daniele Addis, Axolabs GmbH, Germany