

Preliminary Program

(February 26, 2024)

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

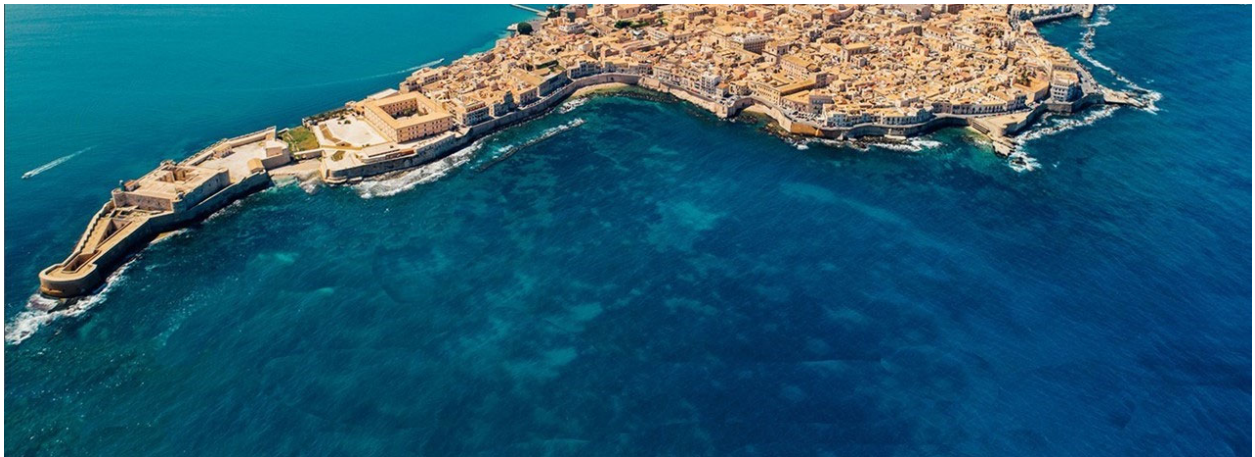
**April 4 – 8, 2024
Grand Hotel Minareto
Siracusa, Sicily, Italy**

Conference Chairs:

Steven F. Dowdy
UCSD School of Medicine
USA

Laura Sepp-Lorenzino
Intellia Therapeutics
USA

Matt Stanton
Generation Bio
USA



Engineering Conferences International

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Thursday, April 4, 2024

15:00 – 16:30	Conference check-in (Hotel Lobby)
16:30 – 16:40	Welcome and General Announcements
16:40 – 17:30	<u>Keynote Talk 1</u> The Chemistry of Something Mano Manoharan, Alnylam Pharmaceuticals, USA
17:30 – 19:00	Welcome Reception on Titanic Terrace with Ortigia Sea View followed by Local Sicilian Wine Tasting
19:00 – 20:30	Dinner (Neos Restaurant)
20:30 – 21:30	Social Hour

Friday, April 5, 2024

07:30 – 09:00 Breakfast Buffet (Neos Restaurant)

09:00 – 09:25 **Setting the Delivery Stage**
Steve Dowdy, UCSD, USA

Session 1: Clinical Stage Delivery

Chair: Virginia Arechavala-Gomez, Biocruces Bizkaia Health Research University, Spain

09:25 – 09:50 **Selective Organ Targeted (SORT) LNPs: focus on lung delivery and PCD program**
Dan Siegwart, UT Southwestern/ReCode, USA

09:50 – 10:15 **TfR Ab conjugated oligos for muscle delivery**
Art Levin, Avidity Biosciences, USA

10:15 – 10:45 Coffee Break

10:45 – 11:10 **TfR-Fab conjugated oligos for muscle delivery**
Oxana Beskrovnaya, Dyne Therapeutics, USA

11:10 – 11:35 **CRISPR LNPs (include particle/cargo analytics)**
Kristy Wood, Intellia Therapeutics, USA

11:35 – 11:50 **TBD** Short talk

11:50 – 12:05 **TBD** Short talk

12:05 – 14:00 Lunch (Nesos Restaurant or Latomie Terrace, depending on weather)

Session 2: Endosomal Trafficking and Escape

Chair: Steve Dowdy, UCSD, USA

14:00 – 14:25 **LNP Endosomal Escape**
Anders Wittrup, Lund University, Sweden

14:25 – 14:50 **TBD**
Marino Zerial, Max Plank Institute, Germany

14:50 – 15:15 **ENDOSCAPE: Triterpene glycoside conjugates allow efficiently endosomal escape of diverse targeted payloads (Short Talk)**
Guy Hermans, Supreme Technologies B.V., the Netherlands

15:15 – 15:40 **Nucleic acid immunity: the key to successful nucleic acid therapeutics**
Gunther Hartmann, University of Bonn Hospital, Germany

15:40 – 16:00 Coffee Break

16:00 – 16:25 **Predictive Ex Vivo Models of LNP Behavior**
Dwight Morrow, Moderna

16:25 – 16:40 **TBD** Short talk

16:40 – 16:55 **TBD** Short talk

16:55 – 17:20 **Round Table on Today's Lessons Learned**

17:20 – 19:00 Poster Session 1 (Conference Center - with beverages)

Friday, April 5, 2024 (continued)

19:00 – 21:00	Dinner (Sicilian Buffet on Titanic Terrace with Ortigia Sea View)
21:00 – 22:00	Social Hour

Saturday, April 6, 2024

07:30 – 09:00	Breakfast Buffet (Nesos Restaurant)
	<u>Session 3: Delivery Chemistry</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
09:50 – 10:15	TBD
10:15 – 10:45	Coffee Break
10:45 – 11:10	Muscle targeted centyrin-siRNA conjugates as substrate reduction therapy for Pompe Disease Sukumar Sakamuri, Aro Biotherapeutics, USA
11:10 – 11:25	TBD Short talk
11:25 – 11:40	TBD Short talk
11:40 – 11:55	TBD Short talk
11:55 – 14:00	Lunch (Nesos Restaurant or Latomie Terrace, 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	Design and Evaluation of Novel ionizable Lipids for RNA Delivery Pad Chivukula, Arcturus Therapeutics, USA
14:50 – 15:05	TBD Cosmin Mihai, Nvelop, USA
15:05 – 15:20	TBD Akin Akinc, Aera Tx, USA
15:20 – 15:50	Coffee Break
15:50 – 16:15	TBD
16:15 – 16:30	LNP delivery Guarav Sahay, Oregon State University, USA
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 – 22:00	Social Hour

Sunday, April 7, 2024

07:30 – 09:00	Breakfast Buffet (Nesos Restaurant)
	<u>Session 5: Non-Viral and Viral Gene Therapy</u> 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 <i>In Vivo</i> Gene Delivery Leah Sabin, Regeneron, New York, USA
09:50 – 10:15	DNA LNP delivery Matt Stanton, Generation Bio, USA
10:15 – 10:45	Coffee Break
10:45 – 11:10	Engineering Exosomes to Enable and Create Improved Genetic Medicines Tony De Fougères, Evox Therapeutics Limited, United Kingdom
11:10 – 11:35	Targeting mRNA LNPs to New Cell types Stefaan De Koker, Etherna, Belgium
11:35 – 12:00	LNP Structure Jay Kulkarni, Nanovation Therapeutics, Canada
12:15	Pick up boxed lunch (Hotel Lobby)
12:30 – 17:30	Buses leave for sightseeing excursion to Roman ruins and Old Town Siracusa
18:00 – 18:10	Poster Awards
18:10 – 19:00	<u>Keynote Talk 2</u> The World of LNPs Pieter Cullis, University of British Columbia, Canada
19:30 – 21:30	Gala Dinner (Nesos Restaurant)
21:30 – 22:30	Social Hour

Monday, April 8, 2022

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