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
369 Lexington Avenue, 3rd Floor #389
New York, NY 10017, USA
www.engconfintl.org – info@engconfintl.org
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  **Keynote Talk 1**  
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-Gomeza, 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 (Neson 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)**

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<tr>
<td>19:00 – 21:00</td>
<td>Dinner (Sicilian Buffet on Titanic Terrace with Ortigia Sea View)</td>
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<td>21:00 – 22:00</td>
<td>Social Hour</td>
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Saturday, April 6, 2024

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

Session 3: Delivery Chemistry
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)

Session 4: New Delivery Strategies
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)

**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 – 10:15 **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 Fougerolles, 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 **Keynote Talk 2**
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