***Preliminary Program***

*(February 24, 2020)*

# [DELIVERY OF NUCLEIC ACID THERAPEUTICS II: BIOLOGY, ENGINEERING AND DEVELOPMENT](http://engconf.us/conferences/biotechnology-2/delivery-of-nucleic-acid-therapeutics-ii-biology-engineering-and-development/)

**March 15-20, 2020**

[**Grand Hotel Minareto**](https://www.grandhotelminareto.it/en/index.html)

**Siracusa, Sicily**

**Conference Co-Chairs**

**Steven F. Dowdy**

**University of California, USA**

**Laura Sepp-Lorenzino**

**Intellia Therapeutics, USA**



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**Engineering Conferences International**

**32 Broadway, Suite 314 - New York, NY 10004, USA**

**www.engconfintl.org –** [**info@engconfintl.org**](mailto:info@engconfintl.org)

**Sunday, March 15, 2020**

15:00 – 17:00 Registration

17:00 – 17:15 Welcome and General Announcements

17:15 – 18:15 **Keynote Talk 1**

TBD

Mano Manoharan (Alnylam Pharmaceuticals, USA)

18:15 – 19:00 Welcome Reception

19:00 – 20:30 Dinner

20:30 – 23:00 Social Hour

**Monday, March 16, 2020**

07:30 – 09:00 Breakfast Buffet

09:00 – 09:25 1 Setting the Delivery Stage

Steve Dowdy (UCSD, USA)

Session 1: Nucleic Acid Chemistry and Design

Chairs: Jesper Wengel, University of Southern Denmark, Denmark

09:25 – 09:50 2 TBD

Punit Seth (Ionis, USA)

09:50 – 10:15 3 Oligonucleotide diversity and design

Nanna Albaek (Roche, Denmark)

10:15 – 10:45 Coffee Break

10:45 – 11:10 4 TBD

Jesper Wengel (University of Southern Denmark, Denmark)

11:10 – 11:35 5 The chemistry of something - TBD

Mano Manoharan (Alnylam Pharmaceuticals, USA)

11:35 – 11:50 6 Liposomes decorated with lipid anchored G4-Decoy DNA, miRNA and cell penetrating peptides against pancreas cancer (Short Talk)

Erik Pedersen (University of Southern Denmark, Denmark)

11:50 – 12:05 7 A silica-coating of messenger RNA-loaded polyion complexes with enhanced stability under gastrointestinal condition for oral delivery (Short Talk)

Rimpei Kamegawa (The University of Tokyo, Japan)

12:05 – 14:00 Lunch

Session 2: Endosomal Trafficking and Escape

Chairs: Steve Dowdy (UCSD, USA)

14:00 – 14:25 8 TBD

Anders Wittrup (Lund University, Sweden)

14:25 – 14:50 9 Dual target centyrin-siRNA conjugates for extra-hepatic KRAS dependent tumors

Karyn O'Neil (Aro Biotherapeutics Company, USA)

14:50 – 15:25 10 Nucleic acid chemical modifications and conjugates to enable and enhance

delivery

Takanori Yokota (Tokyo Medical & Dental School)

15:25 – 15:55 Coffee Break

15:55 – 16:20 11 Evidence for an intracellular depot that contributes to the extended duration of activity of GalNAc-siRNA conjugates

Chris Brown (Alnylam Pharmaceuticals, USA)

**Monday, March 16, 2020 (continued)**

16:20 – 16:55 12 Endoscape: Triterpene glycoside conjugates allow efficiently endosomal escape of diverse targeted payloads (Short Talk)

Guy Hermans (Sapreme Technologies B.V., the Netherlands)

16:55 – 17:10 13 Improving endosomal escape and knockdown efficiency of conjugated siRNAs using endosome-disrupting small molecules (Short Talk)

Hampus Du Rietz (Lund University, Sweden)

17:10 – 17:30 14 Round Table on Today's Lessons Learned

17:30 – 19:00 Local Wine Tasting and Poster Session 1

19:00 – 21:00 Dinner

21:00 – 23:00 Social Hour

Tuesday, March 17, 2020

07:30 – 09:00 Breakfast Buffet

Session 3: Delivery

Chairs: Anastasia Khvorova, University of Massachusetts, USA

09:00 – 09:25 15 TBD

Melissa Moore (Moderna, USA)

09:25 – 09:50 16 TBD

Tony De Fougerolles (Evox Therapeutics Limited, United Kingdom)

09:50 – 10:15 17 RNAntibody® - A potent mRNA technology for antibody therapies

Carolin Thiele (CureVac AG, Germany)

10:15 – 10:45 Coffee Break

10:45 – 11:10 18 TBD

Anastasia Khvorova (University of Massachusetts, USA)

11:10 – 11:25 19 Nucleic acid delivery using Gesicles (Short Talk)

Bruno Gaillet (Université Laval, Canada)

11:25 – 11:40 20 Topical delivery of oligonucleotides into the eye: An epic 12-layer cake (Short Talk)

Tamara Martinez (Sylentis, Spain)

11:40 – 11:55 21 Beyond GalNAc! Constructing complex oligosaccharides targeting ligands for the delivery of oligonucleotide therapeutics (Short Talk)

Joseph O’Sullivan (Northumbria University, United Kingdom)

11:55 – 14:00 Lunch

Session 4: Delivery, Delivery

Chairs: Matt Stanton, Generation Bio, USA

14:00 – 14:25 22 TBD

Pieter Cullis (University British Columbia, Canada)

14:25 – 14:50 23 Chemical evolution of artificial peptide carriers for nucleic acid delivery

Ernst Wagner (Ludwig-Maximilians-Universität Munich, Germany)

14:50 – 15:05 24 Topical gene therapy to prevent field cancerisation in skin pathologies (Short Talk)

Céline Reusch (University of Liège, Belgium)

15:05 – 15:20 25 Delivering bioengineered outer membrane vesicles for RNA therapeutics targeting lung disease (Short Talk)

Elena Eftychiou (Northumbria University, United Kingdom)

15:20 – 15:50 Coffee Break

15:50 – 16:15 26 Polypeptide mediated delivery of oligonucleotides  
 David Evans (Sirnaomics, USA)

Tuesday, March 17, 2020 (continued)

16:15 – 16:30 27 Selective Ablation of Solid Tumors using a p53-Targeted FAST-LNP Gene Therapy (Short Talk)

Douglas Brown (University of Alberta, Canada)

16:30 – 17:00 28 Just a Little Delivery Reality Check

Paul Burke (Burke Bioventures LLC, USA)

17:00 – 18:30 Poster Session 2 with wine and beer

18:30 – 21:00 Dinner

21:00 – 23:00 Social Hour

Wednesday, March 18, 2020

07:30 – 09:00 Breakfast Buffet

Session 5: Viral and Non-Viral Gene Therapy

Chairs: Mark Kay, Stanford University, USA

09:00 – 09:25 29 TBD

Dirk Grimm (Heidelberg University Hospital, Germany)

09:25 – 09:50 30 TBD

Matt Stanton (Generation Bio, USA)

09:50 – 10:15 31 Systemic messenger RNA therapy as a treatment for ornithine transcarbamylase (OTC) deficiency

Pad Chivukula (Arcturus Therapeutics, USA)

10:15 – 10:45 Coffee Break

10:45 – 11:10 32 TBD

Mark Kay (Stanford University, USA)

11:10 – 11:35 33 TBD

Kristy Wood (Intellia Therapeutics, USA)

11:35 – 12:00 34 Polysaccharide/DNA complexes to deliver therapeutic oligonucleotides to immunocytes

Kazui Sakurai (University of Kitakyushu, Japan)

12:15 Pick up boxed lunch

12:45 – 18:30 Buses leave for sightseeing excursion to Roman ruins and Old Town Siracusa

Stop at Cathedral Plaza for coffee/snacks

18:30 – 21:30 Free time for shopping and dinner on your own in Old Town Siracusa

21:30 Return to Grand Hotel Minaretto

Thursday, March 19, 2020

07:30 – 09:00 Breakfast Buffet

Session 6: Bioanalytical and Biophysical Methods

Chairs: Rubina Parmar, Intellia Therapeutics¸USA

09:00 – 09:25 35 Characterizing Drug Delivery Nanoparticles by use of Solution Scattering Techniques

Kazuo Sakurai (University of Kitakyushu, Japan)

09:25 – 09:50 36 Comparison of siRNA and LNA: Quantification of absolute numbers for target knock down

Annette Indlekofer (Roche Diagnostics GmbH, Germany)

09:50 – 10:15 37 SAXS and other biophysical techniques of LNPs

Ramsey Majzoub (Intellia Therapeutics, USA)

10:15 – 10:45 Coffee Break

10:45 – 11:10 38 Enhanced delivery with spherical nucleic acids and efficacy in the central nervous system

Samantha Sarett (Exicure, USA)

11:10 – 11:25 39 A G-quadruplex based sensing strategy for the rapid detection of VEGF165 using a portable suspending-droplet microfluidic device (Short Talk)

Dik-Lung Ma (Hong Kong Baptist University, Hong Kong)

11:25 – 11:40 40 Local and systemic nucleic acid therapies delivery case studies (Short Talk)

Annadoray Lavenniah (A\*STAR/Genome Institute of Singapore, NUS/Cardiovascular Research Institute)

11:40 – 14:00 Lunch

Session 7: Delivery, Delivery, Delivery

Chairs: Laura Sepp-Lorinzino, Intellia Therapeutics, USA

14:00 – 14:15 41 Tracking subcellular localization of siRNA after cytosolic entry (Short Talk)

Johanna Johansson (Lund University, Sweden)

14:15 – 14:30 42 Intracellular introduction of nucleotides and plasmid DNA by Pyro-drive jet injector (Short Talk)

Kunihiko Yamashita (Osaka University, USA)

14:30 – 14:45 43 I Progress and challenges of aptamer-mediated delivery (Short Talk)

Bethany Powell Gray (Duke University School of Medicine, USA)

14:45 – 15:00 44 Liposomes and polymeric nanoparticle delivery vehicles (Short Talk)

John Lewis (Entos Pharmaceuticals, University of Alberta)

15:00 – 15:10 45a A COST network to improve the Delivery of Antisense RNA therapeutics

Vittorio de Franciscis (IEOS-CNR, Italy)

Thursday, March 19, 2020 (continued)

15:10 – 15:25 45b A two aptamer-based selective targeting of GBM with therapeutic RNAs (Short Talk)

Vittorio de Franciscis (IEOS-CNR, Italy)

15:25 – 15:40 46 NATA: Vision and aspirations to unlock the potential of precision genetic medicines (Short Talk)

Loic Roux (NATA, United Kingdom)

15:40 – 16:00 Delivery Meeting Wrap-Up

Laura Sepp-Lorenzino (Intellia Therapeutics, USA)

16:00 – 16:15 Break

16:15 – 16:20 Poster Awards

16:20 – 17:20 Keynote Talk 2

How Oligonucleotides Enter Cells - Unlocking the Barriers

Tom Kirchhausen (Harvard Medical School, USA)

17:20 – 18:00 Reception

18:00 – 20:00 Banquet

20:00 – 23:00 Social time (belly to the bar)

**Friday March 20, 2020**

07:30 – 09:00 Breakfast Buffet

09:00– 12:00 Departures