CRS/IPEC Americas Biologics Summit 2025

Unlocking the Code: Cutting-Edge Advances in Gene & RNA Delivery

Simon Matoori

Faculté de Pharmacie, Université de Montréal

Montreal (QC), Canada

Beate Bittner

Hoffmann La Roche

Basel, Switzerland

biologics summit

advancing science as one industry

*The Biologics Summit ignites collaboration between **academia and industry** to tackle pressing scientific challenges in biologics. Powered by the **Controlled Release Society (DDS) and IPEC-Americas (excipients)**, this dynamic forum unites delivery science innovators and pharmaceutical leaders to shape the future of drug delivery and enable clinical translation*

Simon Matoori (Université de Montréal)

Beate Bittner (Roche)

Ronald Smith (Pharmawyze)

Nigel Langley (Gaylord Chemical)

CRS Annual Meeting, 2023, Las Vegas (NV)
Subcutaneous administration of biologics to enable decentralized care

CRS Annual Meeting, 2024, Bologna (Italy)
Medical devices enabling home administration of biologics

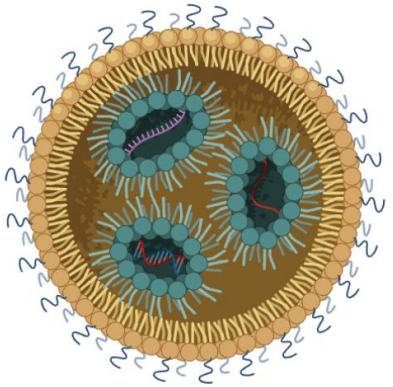
CRS Annual Meeting, 2025, Philadelphia (PA)
Unlocking the Code: Cutting-Edge Advances in Gene & RNA Delivery

Nucleic Acids: The Blueprint of Life and Therapy

- DNA and RNA are **information-carrying molecules**
- DNA stores genetic instructions; RNA translates them into proteins
- In therapeutics, synthetic nucleic acids can:
  - Replace missing genes (gene therapy)
  - Silence disease-causing genes (siRNA, ASOs)
  - Encode therapeutic proteins (mRNA)

Nucleic Acid Delivery Systems: Overcoming the Delivery Barrier

- Nucleic acids are large, charged, and unstable → need delivery systems
- Main **delivery platforms**: non-viral vectors vs. viral vectors
  - Lipid nanoparticles (LNPs): current gold standard
  - Polymers, exosomes
  - Viral vectors (AAV, lentivirus, HSV)
- Key goals:
  - Protect from degradation
  - Enable efficient cellular uptake and endosomal escape
  - Tissue-specific delivery

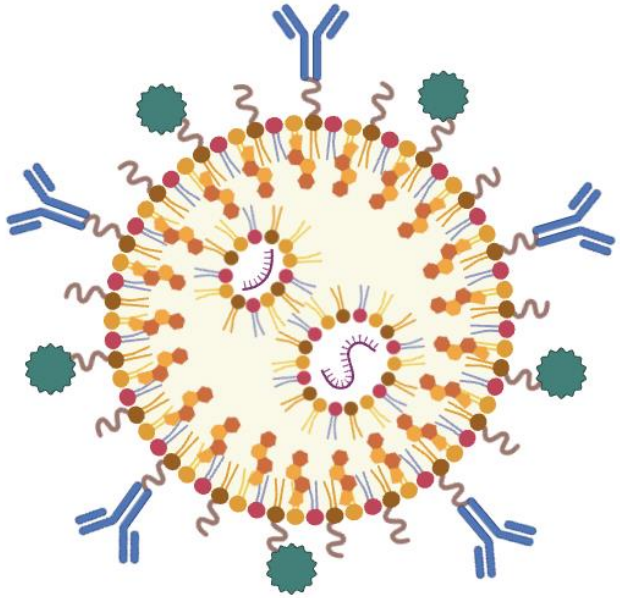


FDA-Approved Nucleic Acid Drugs

- 20 nucleic acid therapeutics approved to date, including:
  - **mRNA vaccines** (Comirnaty®, Spikevax®)
  - **siRNA** (Onpattro®, Givlaari®, Leqvio®)
  - **ASOs** (Spinraza®, Tegsedi®)
  - **Gene therapy** (Zolgensma®, Luxturna®)
- First approvals: ASOs in 1998, siRNA in 2018, mRNA vaccines in 2020
- **Emerging areas**: gene editing (CRISPR), circular RNA, self-amplifying RNA

Current Challenges & Future Directions

- **Biological barriers**: endosomal escape, tissue specificity, immunogenicity
- **Manufacturing hurdles**: scalability, stability/cold chain
- **Regulatory landscape**: facilitating the exploration of novel modalities
- Next frontiers:
  - AI-driven design of delivery systems
  - Organ-targeted delivery (lung, brain, tumors)
  - Shelf-life
  - Stimuli responsiveness and combination therapy



Part 1: May 12, 2025 at Excipient World Conference & Expo, National Harbor, MD

Talks on excipients for LNPs (vaccines, therapeutics), USP monographs for lipids used in LNPs, and excipients for cryoprotection

Speakers: Nigel Langley, Basak Clements (biomatria), Sarah Poynter (Wilfrid Laurier University, Waterloo, ON), Magali Hickey (Stealth Biotech PBC), Catherine Sheehan (USP)

Part 2: Unlocking the Code: Cutting-Edge Advances in Gene & RNA Delivery

8:30 AM - 8:35 AM EDT	Words of Welcome & Opening Chair: Simon Matoori, PhD – Université de Montréal
8:35 AM - 8:45 AM EDT	Introduction to Nucleic Acid Delivery Chair and Speaker: Simon Matoori, PhD – Université de Montréal
8:45 AM - 9:15 AM EDT	Optimizing Non-Viral Delivery to Enable Gene Editing Therapies Speaker: Luis Brito, PhD (he/him/his) – Beam Therapeutics
9:15 AM - 9:45 AM EDT	Understanding Hypoxia's Impact on mRNA Lipid Nanoparticle Therapies Speaker: Owen S. Fenton, PhD – University of North Carolina at Chapel Hill
9:45 AM - 10:15 AM EDT	Redefining Lung Therapy Through Inhalable RNA Dry Powder Formulations Speaker: Benjamin Winkeljann, PhD (he/him/his) – Ludwig-Maximilians-University Munich
10:15 AM - 10:30 AM EDT	Break

Part 2: Unlocking the Code: Cutting-Edge Advances in Gene & RNA Delivery

10:30 AM - 11:00 AM EDT	Gene Delivery for Glioblastoma: At the Interface of Immunotherapy and Precision Medicine Speaker: Flavia Sousa, PhD (she/her/hers) – University of Groningen
11:00 AM - 12:00 PM EDT	Biologics Summit Part II: Keynote Speaker Presentation Speaker: Daniel G. Anderson, PhD – Massachusetts Institute of Technology
12:00 PM - 12:30 PM EDT	AI-Driven Design of Lipid Nanoparticles for mRNA Delivery Speaker: Bowen Li, PhD (he/him/his) – University of Toronto
12:30 PM - 1:00 PM EDT	Conclusion & Closing Remarks Chair and Speaker: Simon Matoori, PhD – Université de Montréal