## **AGENDA**

AGENDA Day 1: Monday, November 12			
	7:00 - 8:30	Breakfast	
	8:30	Welcome and meeting organization (Melissa & Stephane)	
	8:45 - 10:15	Ben Zander, Boston Philharmonic Orchestra Director <a href="http://benjaminzander.com/">http://benjaminzander.com/</a> <a href="https://en.wikipedia.org/wiki/Benjamin_Zander">https://en.wikipedia.org/wiki/Benjamin_Zander</a>	
	10:15 - 10:30	Coffee Break	
	10:30 - 11:30	Session I: Platform Overviews	[2 x (20 min talk + 5 min questions) = 50 min]
		<u>Ugur Sahin</u> - BioNTech <u>Dan Menichella</u> - CureVac	
	11:30 - 12:30	Session II: Delivery I	[3 x (15 min talk + 5 min questions) = 60 min]
		<u>Daniel Siegwart</u> - UT Southwestern  "Chemical design of carriers for delivery long RNAs: From mRNA-mediated protein replacement therapy to CRISPR/Cas gene editing"	
		Tom Madden - Acuitas "Characterizing the therapeutic application of messenger RNA-lipid nanoparticles	
		Andreas Wagner - Polymun  "Guiding RNA formulations from laboratory into clinical trials. Lessons learned from development and optimization of liposomal formulations"	
	1:00 - 2:00	Lunch	
	2:00 - 4:00	Session III: Vaccines and Cancer  Norbert Pardi - Univ. Pennsylvania "Vaccination with nucleoside-mod  Dong Yu - GSK "Vaccine development using self	[5 x (15 min talk + 5 min questions) = 100 min]
		Andrew Bett - Merck "An mRNA-based vaccine expre	essing RSV F is immunogenic and protective in

"An mRNA-based vaccine expressing RSV F is immunogenic and protective in non-human primate models of Respiratory Syncytial Virus infection"

Hangwen Li - Stemirna

"Development of mRNA based cancer vaccines in China"

Tim Wagenaar - Sanofi

"From cold to hot: Using mRNA to alter the tumor microenvironment"

## **AGENDA**

## Day 1: Monday, November 12 (cont.)

## Session III (cont):

Short Talks: [2 x (8 min talk + 2 min questions) = 20 min]

## Lien Van Hoecke - Ghent Univ

"Therapeutic anti-tumor immunity directed against neo-epitopes by intratumor delivery of mRNA encoding MLKL, a mediator of necroptosis"

# Colin Pouton - Monash University

"Self-crosslinking lipopeptide/nucleic acid/polymer particles: a new platform for nucleic acid vaccination enabling assembly in aqueous solution"

#### 4:00 - 4:15 Coffee Break

## 4:15 - 5:45 Session IV: Controlling mRNA and Protein Synthesis

 $[3 \times (15 \text{ min talk} + 5 \text{ min questions}) = 60 \text{ min}]$ 

## Georg Seelig - University of Washington

"Human 5'UTRDesign and Variant Effect Prediction from a Massively Parallel Translation Array"

## Sun Hur - Harvard Medical School

"An Origin of the Immunogenicity of In Vitro Transcribed RNA"

## Anton McCaffrey - Trilink

"Exploring the Messenger RNA Capping Code: CleanCap Co-transcriptional Capping Allows One Pot Synthesis of Previously Inaccessible Types of Caps"

Short Talks: [3 x (8 min talk + 2 min questions) = 30 min]

#### Craig Martin - UMass Amherst

"3' end additions by T7 RNA polymerase are RNA self-templated, distributive, and diverse in character – RNA-Seq analyses"

## Stefano Gustincich – Istituto Italiano di Tecnologia

"SINEUPs: a new antisense, long non-coding RNA-based platform to increase endogenous protein levels for therapy"

## Scott Tenenbaum - SUNY Polytech

"sxRNA: Controlling the expression of a therapeutic mRNA based on the unique microRNA profile of a cell"

## **AGENDA**

# Day 1: Monday, November 12 (cont.)

5:45 - 6:00 Session V: Flash Poster Talks

 $[4 \times (2 \text{ min talk}) = 8-10 \text{ min}]$ 

Bijoyita Roy - New England Biolabs

"High-temperature transcription generates synthetic RNA with reduced immunogenicity"

Chinping Chng- Codexis

" Enzyme engineering for effective mRNA therapeutics: T7 RNA Polymerase and Actives"

Dal-Hee Min - Lemonex

"DegradaBALL® : New RNA delivery platform technology for sustained release inside target cells"

Xavier de Mollerat du Jeu - Thermo Fisher Scientific

" Invivofectamine™ Reagents: Novel Lipid Nanoparticles (LNPs) for Therapeutic In Vivo mRNA delivery to the Liver, Lung, Spleen and Topical applications"

6:00 - 7:45 **Poster Session** 

Cocktails, Appetizers and Buskers from Berkeley College of Music & New England Conservatory

7:45 - **Dinner** with Music and Dancing

## Day 2: Tuesday, November 13

7:00 - 8:30	Breakfast	
8:30	Welcome and Keynote Introduction: Stephane Bancel	
8:35 - 9:15	Keynote: John Maraganore, CEO, Alnylam	
	(30 min talk + 10 min questions) = 40 min	
9:15 - 10:30	Session VI: Regulatory Questions and Public Perception of mRNA Therapies [75 min]	
	Discussion of key questions relevant to mRNA therapeutics by industry regulatory affairs officers and representatives from European and US regulatory agencies	
	Panelists: <u>Thomas Hinz</u> (PEI), <u>Andreas Kuhn</u> (BioNTech), <u>Otmar Pfaff</u> (CureVac), <u>Christine Swenson</u> (Moderna), <u>Wellington Sun</u> (Moderna, Former FDA), <u>Mark Tracy</u> (Tracy Bioconsulting)	
10:30 - 10:45	Coffee Break	

## **AGENDA**

# Day 2: Tuesday, November 13 (cont.)

10:45 - 12:35 **Session VII: Delivery II** 

 $[5 \times (15 \text{ min talk} + 5 \text{ min questions}) = 100 \text{ min}]$ 

Paula Hammond - MIT

"Designer Polypeptides and Electrostatic Assembly for RNA-protein Enhanced Delivery"

Yhizou Dong - Ohio State

"Development of nanomaterials for mRNA therapeutics and genome editing"

Pad Chivikula - Arcturus

"LUNAR®: Enabling Delivery of mRNA Therapeutics and Vaccines"

James Heyes - Genevant

"Developing mRNA Therapeutics with LNPs"

John Androsavich - Translate Bio

"Advancing the development of mRNA therapeutics to restore protein function"

Short Talk:

 $[1 \times (8 \text{ min talk} + 2 \text{ min questions}) = 10 \text{ min}]$ 

Piotr Kowalski - MIT

"lonizable amino-polyesters synthesized via ring opening polymerization with tertiary amino-alcohols for tissue selective mRNA delivery"

12:35 - 1:45 **Lunch** 

1:45 - 3:45 **Session VIII: Therapeutic Proteins** 

 $[5 \times (15 \text{ min talk} + 5 \text{ min questions}) = 100 \text{ min}]$ 

Adam Resnick - Children's Hospital of Philadelphia

"Translating large-scale data to novel therapeutic platforms on behalf of childhood diseases"

Christian Plank - Ethris

"Messenger RNA therapeutics for bone regeneration"

Keiji Itaka - Tokyo Medical and Dental University

"mRNA as a New Paradigm of Gene Therapy for Intractable Diseases and Regenerative Medicine"

Romesh Subramanian - Atlas Venture

"Promise of mRNA therapies in rare disease and dimensions of optimization"

Anna Collén - AstraZeneca

"VEGF-A mRNA: A novel therapeutic for treatment of ischemic heart disease"

# **AGENDA**

# Day 2: Tuesday, November 13 (cont.)

# Session VIII (cont.):

Short Talks: [2 x (8 min talk + 2 min questions) = 20 min]

Ajit Magadum - Mount Sinai

"Ablation of a single N-glycosylation site in human FSTL1 induces cardiomyocyte proliferation and cardiac regeneration"

Sonia Golombek - University Hospital Tübingen

"Intradermal delivery of synthetic tropoelastin mRNA by microinjection results in production of elastin in the skin"

3:45 - 4:00 Awards and Poster Prizes
Closing Remarks by Organizing Committee