Wednesday, November 1, 2017

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8:00 AM	Opening Registration and Reception	12:05 PM	Steve F. Dowdy UCSD School of Medicine, USA Delivery of RNA Therapeutics:	Molecular Therapy, Antibodies & Protein Replacement		Panel Discussion »Challenges and future of mRNA products«	
9:00 AM Keynote	Welcome remarks e Session	12:25 PM	Overcoming a Billion Years of Evolutionary Defenses Christine Esau Arcturus Therapeutics, USA LUNAR-mRNA for protein replacement therapy Tom Madden Acuitas Therapeutics, Canada Optimizing the Therapeutic Index of messenger RNA-Lipid Nanoparticles for Clinical Application Lunch Break	3:15 PM	Chair Introduction by Nigel Horscroft CureVac, Germany	5:40 PM	Moderated by Sascha Karberg Tagesspiegel, Germany Participants: Ingmar Hoerr,
9:10 AM 9:50 AM	Reinhold Messner Mountaineer, adventurer & explorer, Italy Moving Mountains Schraga Schwartz Weizmann Institute of Science, Israel Cracking the epitranscriptome	12:45 PM 1:05 PM		3:40 PM 4:00 PM 4:20 PM 4:40 PM	Regina Fritsche-Danielson AstraZeneca, Sweden VEGF-A Modified mRNA Therapy for Treatment of Chronic Skin Ulcers and Ischemic Heart Disease. From Preclinical Studies to Phase2A Keiji Itaka Tokyo Medical and Dental University, Japan mRNA-based therapy for intractable diseases and regenerative medicine Coffee Break Michael Kormann University of Tübingen, Germany Chemically modified CFTR mRNA normalizes critical lung function parameters in vivo Lior Zangi Icahn School of Medicine at Mount Sinai, USA Cardiomyocytes Specific Modified mRNA of Cell Cycle Inducer Promote Mammalian Cardiac Regeneration Post Injury Lianne Smith Translate Bio, USA mRNA Therapeutics for the Treat- ment of Rare Diseases	Stephen Hoge, Christian Mandl, Norbert Pardi, Ugur Sahin Closing of Conference day 1 6:20 PM Closing remarks day 1 Networking Evening	
	Chair Introduction by	2:05 PM (Chantal Pichon Université d'Orléans, France Insights from intracellular trafficking studies for mRNA delivery and bioproduction Alexandra Stolzing Loughborough University, UK mRNA for cell reprogramming Poster talk by Renata Grzela University of Warsaw, Poland Stability, translational efficiency of capped mRNA and induction of anti- viral protein IFIT1 in HEK293 cells Poster talk by Katrien Remaut Ghent University, Belgium Advanced microscopy methods to elucidate bottlenecks in lipid- and polymer assisted mRNA delivery:			evening with community in	Enjoy an exclusive networking evening with the mRNA community in Berlin's old
10:20 AM 10:25 AM 10:50 AM 11:15 AM 11:35 AM	Chair Introduction by Katalin Kariko BioNTech, Germany Stephen Hoge Moderna Therapeutics, USA The Clinical Development of mRNA Drugs and Vaccines Ingmar Hoerr CureVac, Germany mRNA Therapeutics: Getting to Products Coffee Break Ugur Sahin BioNTech, Germany Delivering Messages for Cancer Immunotherapy	2:25 PM 2:45 PM 2:55 PM				Address: Wasserwerk, Hohenzollerndamm 208 a, 10717 Berlin Please see the map in your conference bag. Busses will bring you to the evening location and back to the Sofitel. 6:30 PM First bus departure from the hotel to the evening location 9:00 PM First bus departure from the evening location back to the hotel You can also reach the location for our networking evening on foot in just 15 minutes (1 km / 0.62 mi)	
mRNA Delivery & Design		3:05 PM	Finding the balance Poster talk by Asha K. Patel Massachusetts Institute of Technology, USA	5:20 PM	Poster talk by Ellese Carmona Harvard Medical School, USA Circular mRNA as a Novel Template for Therapeutic Gene Transfer		
12:00 PM	Chair Introduction by Melissa Moore Moderna Therapeutics, USA		Chemically versatile, degradable nano-carriers for systemic and localized delivery of mRNA to the lung	5:30 PM	Poster talk by Robert Overell PhaseRx, USA Targeted mRNA delivery to the liver as a treatment for ornithine trans- carbamylase deficiency (OTCD)		

Thursday, November 2, 2017

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9:00 AM Weld	ome remarks	day 2
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Cancer Therapy

9:05 AM **Chair Introduction by Kris Thielemans** Vrije Universiteit Brussel, Belgium 9:10 AM Hayat Bähr-Mahmud BioNTech, Germany Elimination of solid tumors in mice by mRNA-encoded bispecific antibodies **Karine Breckpot** 9:30 AM Vrije Universiteit Brussel, Belgium TriMix mRNA-based cancer vaccines Stefaan De Koker 9:50 AM eTheRNA Immunotherapies,

10:10 AM **Smita Nair**

Belgium

Duke University School of Medicine, USA

or just expandable?

RNA-based Cancer Vaccines

Type I IFN and T-cell immunity

to mRNA vaccines: good, bad

10:30 AM Poster talk by Irina Tcherepanova **Argos Therapeutics, USA**

> Interim analysis of Rocapuldencel-T testing in the ongoing Phase 3 study

in mRCC patients

Coffee Break 10:40 AM

Vaccines against Infectious Diseases

Chair Introduction by 11:10 AM Luc Aujame **Independent Consultant, France**

11:15 AM Michela Brazzoli **GSK Vaccines, Italy**

Self-amplifying mRNA-based vaccine promotes different types of immune responses correlating with protection in influenza animal models

Justin Richner 11:35 AM

> **IIT Research Institute, USA** A novel mRNA vaccine protects against Zika virus in a mouse model

11:55 AM Jörg Vogel

University of Würzburg, Germany RNA-based Infection Research

12:15 PM Lunch Break with Poster Session

1:15 PM **Drew Weissman**

> University of Pennsylvania, USA Nucleoside-modified mRNA-lipid nanoparticle vaccine platform induces potent T follicular helper and anti-

body responses

1:35 PM Frank Baehner CureVac. Germany

> mRNA based rabies vaccine: From first clinical proof of principle towards competitive product candidates

1:55 PM Poster talk by Hanne Huysmans

Ghent University, Belgium Comparison of the expression kinetics and immunostimulatory activity of replicating mRNA, non-replicating mRNA and pDNA after intradermal electroporation in pigs

Closing of the Conference

3:50 PM **Awarding of the Poster Prizes** & Closing remarks by the Organizing Committee 4:00 PM **End of Conference**

Product Development

2:05 PM **Chair Introduction by Christian Mandl** Vir Bio, USA 2:10 PM Arlen Avakian Alexion, USA mRNA, Developing a New Therapeutic Modality for the Treatment of Rare Diseases

2:30 PM Coffee Break

2:50 PM **Thomas Hinz** Paul-Ehrlich-Institut, Germany

Regulatory Aspects of RNA-Based Medicines

3:10 PM Klaus T. Preissner

Justus-Liebig-Universität Gießen,

Germany

RNase1 administration as an effective and safe regimen against damaging extracellular RNA in cardiovascular diseases

3:30 PM Janos Szebeni

> Semmelweis University, Hungary Immune reactivity of lipid nanoparticle formulations of nucleic acid-based therapeutics: animal

models