

2016

November 1-2, 2016 **Westin Boston Waterfront Boston, MA, USA** 

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## **Table of Contents**

Welcome5
Conference Program
• Day One6
Keynote
Therapeutics Program
Poster Sessions
• Dinner
• Day Two7
Vaccines Program
Best Abstract Oral Presentations
Best Poster Awards
Bios
• Speakers8
Conference Planning Committee14
Sponsor Profiles16



#### Welcome

#### Welcome to Boston and the 4th International mRNA Health Conference!

It is hard to believe that four years have passed since the 1st International mRNA Health Conference was held in Germany. Over the course of these four years, we have witnessed tremendous progress in the field of mRNA science that has brought us notably closer to making the promise of mRNA-based medicines a reality for patients.

Advancements across the research, discovery and development continuum have yielded an increasingly broader mRNA field that now encompasses investigational infectious disease vaccines, cancer vaccines and therapeutic applications. As more programs advance into the clinic, mRNA drug development is among the most closely watched areas in biotech today. Like most truly innovative endeavors, as a new class of investigational medicines, mRNA has sparked enthusiastic interest, measured skepticism and heightened anticipation.

Those of us immersed in this space understand that we are united by our shared desire to harness mRNA as a powerful new mechanism to address unmet medical needs and help patients. We are peer pioneers – learning together and pushing one another to succeed. We recognize the vital role collaboration among a cadre of stakeholders will play in continuing to propel this field forward.

We can only imagine where the field of mRNA drug development will be four years from now – what hurdles we will have overcome and what achievements we will have realized in that timeframe.

We hope you enjoy this year's conference and make the most of this valuable opportunity to share data, insights and perspectives as, together, we navigate this exciting world of mRNA science.

Sincerely,

International mRNA Health Conference Planning Committee

Stéphane Bancel CEO, Moderna

**Ingmar Hoerr** CEO, CureVac **Ugur Sahin** CEO, BioNtech

# **Conference Program**

# **TUESDAY, NOV 1**

7:30 – 9:00am	Registration and Breakfast		
9:00 – 9:10am	Welcome	Conference Planning Committee	
9:10 – 9:20am	Keynote Intro	Jack Szostak, Ph.D.  Harvard Medical School/Massachusetts General Hospital; Nobel Laureate	
9:20 – 10:00am	Keynote The Dynamic Epitranscriptome: Control of mRNA Fate and Function by RNA Modifications	Samie Jaffrey, M.D., Ph.D.  Greenberg-Starr Professor Department of Pharmacology Weill Cornell Medical College	
10:00am – 12:00pm	THERAPEUTICS		
	VEGF-A Modified mRNA in Diabetic Wound Healing and Future Treatment Opportunities	Kenny Hansson, Ph.D. Associate Principal Scientist, Cardiovascular and Metabolic Diseases, Innovative Medicines and Early Development Biotech Unit, AstraZeneca	
	Improved Outcomes after Intratumoral Administration of Immunostimulatory mRNA in a Novel Cationic Lipid	Stephen Kelsey, M.D. President, Onkaido, a Moderna venture	
	Optimal mRNA in the Making	Katalin Karikó, Ph.D. VP, BioNTech RNA Pharmaceuticals	
	mRNA Tailored for Molecular Therapy	Nigel Horscroft, DPhil Vice President Development RNArt, CureVac AG	
	Therapeutic mRNA Delivery: Beyond Hepatocytes	Adrien Weingärtner, Ph.D. Silence Therapeutics	
	Rebuilding the Heart via Human Heart Progenitor- Paracrine ModRNA Technology	Nevin Witman, Ph.D.  Department of Cell and Molecular Biology & Department of Medicine (AZ/KI ICMC), Ken Chien Lab, Karolinska Institutet	
12:00 – 2:30pm	Lunch & Posters		
2:30 – 3:30pm	Therapeutics Panel Discussion and Q&A	Ugur Sahin, M.D. CEO, BioNTech	
3:30 – 4:30pm	Posters		
4:30pm	Break for Day		
5:15pm	Buses Depart from Westin for Museum of Science		
6:00pm	Cocktails (Museum of Science)		
6:30pm	Dinner (Museum of Science)		
Post-Dinner	Museum of Science Lightning Show		

The Museum's Theater of Electricity houses the world's largest air-insulated Van de Graaff generator (37 feet high), offering a risk-free opportunity to witness live lightning shows and find out about lightning safety from the educator inside the birdcage. The generator, originally designed to split atoms, can put out up to 2.5 million volts of electricity, creating sparks long as 15 feet!

# **Conference Program**

# **WEDNESDAY, NOV 2**

8:00 – 9:00am	Breakfast		
9:00 – 10:00am	INFECTIOUS DISEASE VACCINES		
	RNActive Vaccine Technology – Potent Platform for Prophylactic Vaccines	Mariola Fotin-Mleczek, Ph.D. Chief Scientific Officer, CureVac AG	
	Moderna Vaccines: Pathway to the Clinic	Giuseppe Ciaramella, Ph.D. Chief Scientific Officer, Valera, a Moderna venture	
10:00 – 10:30am	Infectious Disease Vaccines Panel Discussion and Q&A	Stéphane Bancel CEO, Moderna Therapeutics	
10:30 – 11:00am	Break		
11:00am – 12:00pm	CANCER VACCINES		
	The RNActive® Cancer Immunotherapy BI 1361849 (CV9202) - Intended Development in Non-Small Cell Lung Cancer	Eric Block, Ph.D.  Associate Director, US Field Medical Lead Boehringer Ingelheim Pharmaceuticals, Inc.	
	Liposomal RNA Immunotherapy	Mustafa Diken, Ph.D. VP, BioNTech RNA Pharmaceuticals	
	mRNA-Based Personal Cancer Vaccines: From Opportunity to Obligation	Ted Ashburn, M.D., Ph.D. Head of Operations, Caperna, a Moderna venture	
12:00 – 12:30pm	Cancer Vaccines Panel Discussion and Q&A	Ingmar Hoerr, Ph.D., MBA Chairman and CEO, CureVac	
12:30 – 1:30pm	Lunch		
1:30 – 2:30pm	TECHNICAL LANDSCAPE: INSIGHTS AND ADVANCES		
	Intro & Moderator	Mustafa Diken, Ph.D. VP, BioNTech RNA Pharmaceuticals	
	Best Abstract Oral Presentations*		
	Best Poster Awards**		
2:30pm	Closing Remarks	Conference Planning Committee	

<sup>\*</sup>Best Abstract Oral Presentations will be selected and presenters notified in advance by the Conference Planning Committee.

<sup>\*\*</sup>Best Poster Awardees will be selected by jury during the conference and announced during this session.

#### **Speaker Bios**



Ted. T. Ashburn, M.D., Ph.D.

Head of Operations Caperna, a Moderna Venture

Dr. Ashburn is Head of Operations for Caperna, a wholly owned venture of Moderna Therapeutics. Dr. Ashburn is responsible for overall design and execution of the venture's personalized cancer vaccine programs. Previously he was Senior Vice President of Product Strategy and Operations at Dicerna Pharmaceuticals, and the Global Product Head for Leukine® (sargramostim) and Elitek®/Fasturtec® (rasburicase) for Sanofi Oncology. In this latter role, Dr. Ashburn provided global leadership for R&D, global marketing, and regional commercial teams to identify, develop, prioritize, and execute on new commercialization and life-cycle management strategies to drive growth.

Dr. Ashburn joined Sanofi Oncology from Genzyme's Corporate Development group, where he led business development efforts for many of Genzyme's franchises including rare diseases. Prior to joining Genzyme, Dr. Ashburn worked for privately held Dynogen Pharmaceuticals, Inc. and the venture capital firm Oxford Bioscience Partners. While at Oxford, he co-founded both Dynogen and BrainCells, Inc. and led investments in Alantos Pharmaceuticals (acquired in June 2007 by Amgen for \$300 million in cash) and superDimension, Ltd. (acquired by Covidien in March 2012 for more than \$300 million). Before joining Oxford, Dr. Ashburn worked at Pfizer Inc., where he was involved in the analysis of several acquisition and new business opportunities, including Pfizer's acquisition of Warner-Lambert in 2001.

Dr. Ashburn graduated magna cum laude from Ball State University, where he studied chemistry and computer science. He earned his M.D. from Harvard Medical School and Ph.D. from the Massachusetts Institute of Technology, where he focused on elucidating the structure and properties of amyloid plaques associated with Alzheimer's disease and type II diabetes mellitus.



Eric Block, Ph.D.

Associate Director, U.S. Field Medical Lead Boehringer Ingelheim

Dr. Block is Associate Director and U.S. Field Medical Lead at Boehringer Ingelheim (BI). He provides support for clinical development and research and development, and informs corporate strategy based on insights gained from regular exchanges with oncology experts.

Prior to joining BI in 2015, Dr. Block worked at Saladax Biomedical as East Coast Oncology Medical Science Liaison, where he provided clinical and scientific support to external stakeholders and internal management for Saladax's suite of oncology products. Before Saladax, Dr. Block worked at Grey Healthcare Group, a healthcare communications agency, in a variety of positions - Biomedical Writer, Associate Medical Director and Medical Director

- where he advised oncology companies regarding their medical affairs activities. Dr. Block received his undergraduate degree in biochemistry from Brown University and his Ph.D. in philosophy of biomedical sciences from New York University School of Medicine.



Giuseppe Ciaramella, Ph.D.

Chief Scientific Officer Valera, a Moderna Venture

Dr. Ciaramella is the Chief Scientific Officer of Valera, a wholly owned venture of Moderna that focuses on the discovery of vaccines and therapeutics for infectious diseases using Moderna's mRNA technology. He joined Moderna in January 2014 as Vice President of Immunology and Biotherapeutics and was appointed Chief Scientific Officer of the newly formed Valera in October 2014.

Dr. Ciaramella has more than 20 years of drug discovery experience at Moderna. AstraZeneca (AZ), Boehringer Ingelheim (BI), Pfizer, and Merck, and has held several leadership roles, with a particular focus in the fields of antivirals, immunology, and biotherapeutics. Prior to joining

Moderna, he led the small molecule antiviral strategy at AZ. At BI, he was Vice President and Head of Collaborative Research where he had responsibility for external R&D and was a member of the WW Research Leadership Team. Prior to BI, Dr. Ciaramella spent 14 years at Pfizer in the UK where he held several Discovery leadership positions, including Head of Biotherapeutics, Head of Antiviral, and Head of Lead Discovery. During his career, he has contributed to several clinical candidates, both small molecule and biologics, and to the anti-HIV drug Maraviroc (Selzentry™), which won the USA Prix Galien for Best Pharmaceutical in 2008.

Dr. Ciaramella holds a Ph.D. in Biochemistry and Molecular Biology from University College London. He is a Fellow of the Royal Society of Chemistry (UK) and he is a member of the Infectious Diseases Society of America (IDSA).



Mustafa Diken, Ph.D.

Deputy Vice President, Immunotherapies Department

Dr. Diken is the Deputy Vice President of the Immunotherapies Department at BioNtech RNA Pharmaceuticals. His research focuses on the development of novel cancer vaccines based on antigen-encoding messenger RNA (mRNA) and the elucidation of immunomodulatory mechanisms for cancer immunotherapy. His other scientific interests include assay development for preclinical testing of cancer vaccines. Dr. Diken also serves as the scientific secretary of the Association for Cancer Immunotherapy (CIMT), a non-profit organization aimed at advancing cancer immunotherapy. He received his Ph.D. in tumor immunology from Johannes Gutenberg University, Mainz.



#### **Speaker Bios**



Mariola Fotin-Mleczek, Ph.D.

Chief Scientific Officer CureVac

Dr. Fotin-Mleczek is Chief Scientific Officer, CureVac AG. She studied biology at the University of Stuttgart with focus on genetics, cellular biology, and immunology. In her Ph.D. at the Institute of Cell Biology and Immunology at the University of Stuttgart, she dedicated herself to study the crosstalk between TNF receptors 1 and 2 by the induction of the programmed cell death apoptosis. The results of her work contributed significantly to the better understanding of the interaction between TNF receptors.

During her postdoc time at the Intrafacultary Institute of the Eberhard Karl University of Tübingen, she worked on the cell-penetrating peptides, a potent tool to introduce desired cargo

into the cells. Together with colleagues from neighboring disciplines, she explained in detail the mechanism by which specific short peptides are able to cross cellular membranes and bring pro-apoptotic peptide into the tumor cells, forcing them into the programmed cell death.

She joined CureVac in 2006 and was responsible for the preclinical development of mRNA-based RNActive technology with the goal to enter first in men clinical studies in tumor patients. In the course of the preclinical development, she was responsible for the elucidation of mechanism of action of mRNA-based vaccines. She was critically involved in a number of scientific publications and patents in the field of mRNA-based therapeutics. In 2013, she took over the responsibility for the research at CureVac as Chief Scientific Officer. At the moment she coordinates the research of around 50 employees, and together with her colleagues in the Board of Directors, refines the strategy of CureVac.



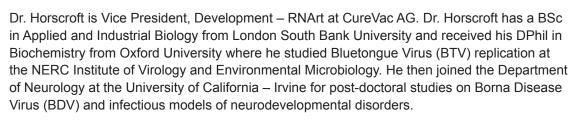
Kenny Hansson, Ph.D.

Associate Principal Scientist, Innovative Medicines and Early Development Biotech Unit AstraZeneca

Dr. Hansson is Associate Principal Scientist in AstraZeneca's Innovative Medicines and Early Development Biotech Unit. Based in Gothenburg, Sweden, he is currently leading the wound healing initiative with modified mRNA within Cardiovascular and Metabolic Diseases. He has 14 years of experience in pharmaceutical development and has held responsibility for advancing projects from early preclinical development into the clinic, including also responsibility for development of translatable in vivo models and biomarkers. His scientific background is within the cardiovascular disease, anemia/kidney & thrombosis/hemostasis areas.







Following his post-doctoral studies, Dr. Horscroft remained in California and transitioned to industry when he joined ICN Pharmaceuticals, which later became Valeant Pharmaceuticals.

Here he developed a Bovine Viral Diarrhoea Virus (BVDV) replicon system designed to be used in parallel with a Hepatitis C Virus (HCV) replicon system to identify highly selective nucleoside and non-nucleoside HCV replication inhibitors. He returned to the UK to join the virology therapeutic area at Pfizer Global Research and Development in Sandwich and headed discovery projects on nucleoside inhibitors, innate immune stimulators and novel interferons as anti-HCV agents. While at Pfizer, he joined the New Opportunities Unit, a group charged with developing new ideas for therapies that fell outside the existing therapeutic area structure. He then took on the role of Head of Research at Pike Pharma in Zurich, Switzerland, where he was in charge of research projects aimed at blocking protein-protein interactions in the fields of virology and oncology.

In 2012 Dr. Horscroft joined CureVac in Tübingen, Germany as Director of Alliance Management. He became Vice President, Development in 2016 and is responsible for research and development of the company's RNArt molecular therapeutics portfolio.



Samie Jaffrey, Ph.D.

Greenberg-Starr Professor, Department of Pharmacology Weill Cornell Medical College

Dr. Jaffrey is the Greenberg-Starr Professor in the Department of Pharmacology at the Weill Cornell Medical College. He received an M.D. and Ph.D. in 1999 from Johns Hopkins School of Medicine where he studied mechanisms of nitric oxide signaling with Dr. Solomon H. Snyder. After brief postdoctoral training with Dr. Snyder, Dr. Jaffrey started his own laboratory at Weill Cornell Medical College in 2001.

Dr. Jaffrey's work has fundamentally advanced our understanding of RNA biology and gene regulation. Most recently, he launched the field of "epitranscriptomics," which has revealed that mRNA and long noncoding RNAs are regulated by nucleotide modifications that impact

their fate and function in cells. Dr. Jaffrey's transcriptome-wide mapping of N6-methyladenosine (m6A) in 2012 revealed that m6A is a pervasive modification in the transcriptome, thereby identifying this modification as a fundamentally novel form post-transcriptional mRNA regulation. Since this seminal study, Dr. Jaffrey mapped dimethyladenosine (m6Am) and established functions of m6A and m6Am as well as m6A and m6Am reader, writer, and eraser proteins. As a result of his early studies, epitranscriptomics is a rapidly developing area of molecular biology that is transforming our understanding of gene regulation in normal and disease states.

Dr. Jaffrey is the recipient of the Klingenstein Neuroscience Award, the Irma T. Hirschl Scholar

Award, the McKnight Foundation Technology Development Award, NIH EUREKA Award, the NIH Director's Transformative R01 Award, the 2013 Blavatnik Award for Young Scientists, and the 2014 American Society for Biochemistry and Molecular Biology Young Investigator Award.

10 | 4th International mRNA Health Conference Nov. 1-2, 2016 • Westin Boston Waterfront • Boston, MA, USA | 11

#### **Speaker Bios**



Katalin Karikó, Ph.D.

Vice President BioNTech

Dr. Karikó is Vice President, BioNTech RNA Pharmaceuticals and leads the mRNA-based protein therapy program for BioNTech RNA from 2013. Prior to that, she investigated RNA-mediated immune activations at the University of Pennsylvania, where she remained Adjunct Professor. She is co-inventor of the first patents awarded for describing the lack of immunogenicity of mRNA containing modified nucleotides, including pseudouridine. Dr. Karikó received her Ph.D. in Biochemistry from the University of Szeged, Hungary. She studied the antiviral activity of 2'-5'-linked oligonucleotides, containing cordycepin, a modified nucleoside. As a postdoc, she studied dsRNA-dependent systems, such as activation of OAS, RNaseL, and PKR at Temple University in Philadelphia, and interferon-induced antitumor mechanisms at USUHS in Bethesda.



Stephen Kelsey, M.D.

President
Onkaido, a Moderna Venture

Dr. Kelsey is President of Onkaido, a Moderna venture focused on the development of mRNA Therapeutics™ for oncology. Dr. Kelsey has extensive pharmaceutical industry experience in oncology. After 16 years as an academic clinician, he started his industry career at Sugen, and later was Vice President of Hematology/Oncology at Genentech. While at Genentech, Dr. Kelsey played a significant role in the development of key products Perjeta®, Kadcyla®, and Erivedge®, as well as other molecules in the company's oncology portfolio. He left Genentech in 2009 to run Geron's oncology division, where he served for four years as Executive Vice President, Research and Development, and Chief Medical Officer, helping to develop therapeutics and vaccines to fight cancer.

Most recently Dr. Kelsey was Senior Vice President, New Projects at the cancer therapies company Medivation. Dr. Kelsey graduated with a Doctorate of Medicine (M.D.) from the University of Birmingham, UK and is a fellow of both the Royal Colleges of Physicians and Pathologists, UK.



Jack Szostak, Ph.D.

Howard Hughes Medical Institute Investigator
Professor of Genetics at Harvard Medical School
Alex Rich Distinguished Investigator, Dept. of Molecular Biology and the Center for
Computational and Integrative Biology at Massachusetts General Hospital
Nobel Laureate

Dr. Szostak is an Investigator of the Howard Hughes Medical Institute, Professor of Genetics at Harvard Medical School, and the Alex Rich Distinguished Investigator in the Department of Molecular Biology and the Center for Computational and Integrative Biology at Massachusetts General Hospital. Dr. Szostak's early research on telomere structure and function, and the role of telomere maintenance in preventing cellular senescence, was recognized by the 2006 Albert Lasker Basic Medical Research Award and the 2009 Nobel Prize in Physiology or Medicine,

shared with Drs. Elizabeth Blackburn and Carol Greider. In the 1990s, Dr. Szostak and his colleagues developed in vitro selection as a tool for the isolation of functional RNA, DNA, and protein molecules from large pools of random sequences. Dr. Szostak's current research interests are in the laboratory synthesis of self-replicating systems and the origin of life. Dr. Szostak is a member of the National Academy of Sciences and a fellow of the American Academy of Arts and Sciences and the American Association for the Advancement of Science.



Adrien Weingärtner, Ph.D.

Silence Therapeutics

Dr. Weingärtner holds a Ph.D. in biophysics from Humboldt University of Berlin. His academic research was focused on host-pathogen interactions of various intracellular pathogens. In 2013 he joined Silence Therapeutics as a scientist. Currently he leads a group focused on in vivo applications of therapeutic mRNA. For this identifying suitable delivery systems as well as optimizing mRNA constructs in order to reduce immune stimulation and to increase the half-life of mRNA is of particular interest.



Nevin Witman, Ph.D.

Post-Doctoral Candidate Ken Chien Lab, Karolinska Institutet

Dr. Witman is a post-doctoral candidate at the Ken Chien Lab at the Karolinksa Institutet, Department of Cell and Molecular Biology & Department of Medicine, located in Stockholm, Sweden. His research theme is predominantly focused around developmental biology, stem cell biology, and modified mRNA.

Dr. Witman earned a B.S. from the Pennsylvania State University in 2006 in the Life Sciences. He then worked within Q.C. and R&D at Pfizer Pharmaceuticals. Later, he continued his studies and completed an M.S. in Medicine at St George's University of London, and in 2013 he received a Ph.D. in Cardiac Regeneration from The Wenner-Gren Institute in Stockholm, Sweden



12 | 4th International mRNA Health Conference Nov. 1-2, 2016 • Westin Boston Waterfront • Boston, MA, USA | 13

#### **Conference Planning Committee Bios**



Stéphane Bancel

Chief Executive Officer Moderna Therapeutics

Mr. Bancel is Chief Executive Officer of Moderna Therapeutics. He joined Moderna in the summer of 2011 when it was a one-employee company. He has assembled a world-class team and raised more than \$1 billion between equity financing and upfront from licensing collaborations.

He was previously CEO of bioMérieux, a world leader in the diagnostics industry. bioMerieux has more than 6,000 employees, a market capitalization of €2.5 billion, and sales of more than €1.3 billion.

Prior to his time at bioMérieux, Mr. Bancel was the managing director of Eli Lilly Belgium and Executive Director of Global Manufacturing Strategy and Supply Chain at Eli Lilly in Indianapolis, Indiana. He started at Lilly in its UK manufacturing plant outside London.

He holds a Master of Engineering from École Central Paris, a Master of Science in Chemical Engineering from the University of Minnesota, and an MBA from Harvard Business School.

Mr. Bancel was elected a 2009 Young Global Leader by the World Economic Forum. He was elected best CEO for investor relations in France in 2009 and was ranked the number one CEO in the biotech sector according to the 2011 Thomson Reuters EXTEL Study. Mr. Bancel serves as a supervisory director of Qiagen N.V., and on the Board of Directors of Syros Pharmaceuticals.



Ingmar Hoerr, Ph.D., MBA

Chairman and Chief Executive Officer CureVac

Dr. Hoerr is Chairman and CEO of CureVac AG, Germany and founded the biopharmaceutical company in 2000 together with Florian von der Mülbe and other colleagues. His entrepreneurship was motivated by a surprising discovery during his doctoral research. Experiments conducted for this research showed that mRNA is capable of generating a strong specific immune response, contrary to what had previously been believed. From this key discovery, Dr. Hoerr and his colleagues built up a company that is now a global leader in the research and development of mRNA-based drugs.

Since its inception, CureVac has received approximately \$330 million (€300 million) in equity investments. In 2015 the company has been valuated at €1,5 billion.

Dr. Hoerr received his Ph.D. from the University of Tübingen and his MBA from Danube University, Krems, Austria.



Ugur Sahin, M.D.
Chief Executive Officer
BioNTech

Dr. Sahin is Chief Executive Officer of BioNTech. He is a doctor of medicine and translational researcher with longstanding expertise in managing projects in the public-private interface. A pioneer in cancer target discovery using high throughput immunological methods and bioinformatics approaches, Dr. Sahin holds more than 70 independent patent applications covering novel cancer biomarkers and targeted therapeutics platforms. His key focus is solving deeply rooted challenges in the multifaceted process of translating innovation from bench to bedside, an interest that was originally prompted by his experiences as a physician. Dr. Sahin's publications have more than 6000 citations, and he is the recipient of prestigious awards from the German Hemato-Oncology Association, German Association for

Immunology, German Federal Ministry of Education and Research (BMBF) and American Society of Clinical Oncology.



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BioNTech AG is an immunotherapy leader with bench-to-market capabilities, developing truly personalized, well-tolerated and potent treatments for cancer and other diseases. Established by clinicians and scientists the Group is pioneering disruptive technologies ranging from individualized mRNA based medicines through innovative Chimeric Antigen Receptors /T-cell Receptor-based products and novel antibody checkpoint immunomodulators. BioNTech's clinical programs are supported by an in-house molecular diagnostics unit whose products include MammaTyper® a molecular in-vitro diagnostic kit, marketed under CE and IVD marking in Europe and certain other countries. Founded in 2008, BioNTech is privately held and shareholders include the MIG Fonds, Salvia, and the Strüngmann Family Office, with the Strüngmann Family Office as the majority shareholder. Information about BioNTech is available at www.biontech.de.



Founded in 2000, CureVac is a technology leader in the development of mRNA-based drugs with the most advanced product pipeline and IP portfolio in the industry. Until today, CureVac tested its products in eight clinical studies with about 450 humans and received clinical trial approvals in Europe, Asia and the US.

CureVac entered into various collaborations with multinational corporations and organizations, including Boehringer Ingelheim, Sanofi Pasteur, the Bill & Melinda Gates Foundation and IAVI.

In 2006, CureVac successfully established the first GMP facility worldwide for manufacturing of mRNA. In 2016, CureVac begins the construction of an industrial scale GMP production facility. www.curevac.com

# moderna

Moderna is a clinical stage pioneer of messenger RNA Therapeutics™, an entirely new in vivo drug technology that produces human proteins, antibodies and entirely novel protein constructs inside patient cells, which are in turn secreted or active intracellularly. This breakthrough platform addresses currently undruggable targets and offers a superior alternative to existing drug modalities for a wide range of diseases and conditions. Moderna is developing and plans to commercialize its innovative mRNA drugs through its own ventures and its strategic relationships with established pharmaceutical and biotech companies. Its current ventures are: Onkaido, focused on oncology, Valera, focused on infectious diseases, Elpidera, focused on rare diseases, and Caperna, focused on personalized cancer vaccines. Founded by Flagship VentureLabs™, Cambridge-based Moderna is privately held and currently has strategic agreements with AstraZeneca, Alexion Pharmaceuticals, Merck and Vertex Pharmaceuticals. To learn more, visit www.modernatx.com.

#### SILVER SPONSORS



Aldevron is a recognized leader in contract manufacturing and development services for nucleic acids, proteins and antibodies. Founded in 1998, we provide companies with essential components for research, clinical and commercial applications. Our products have supported numerous programs in gene therapy, cell therapy and regenerative medicine from the bench to the bedside. Aldevron's services include GMP-Source™ and GMP plasmid manufacturing, linear DNA and mRNA production, gene synthesis, RNA synthesis enzymes and fully human and recombinant antibody generation. Our collaborative approach and commitment to providing quality materials allow us to meet precise client requirements and provide innovative solutions to advance science. Aldevron's headquarters is in Fargo, North Dakota and has facilities in Madison, Wisconsin and Freiburg, Germany. www.aldevron.com



eTheRNA's mission is to help patients to overcome certain cancers and infectious diseases by developing novel immunotherapies that target the fundamental role of dendritic cells in the human immune system. eTheRNA's proprietary mRNA-based TriMix technology boosts dendritic cells leading to a more comprehensive, sustainable and safer enhancement of the patient's immune system than any other similar approach investigated until now. TriMix comprises three mRNA molecules that jointly have a triple boost effect: (1) they enhance the activation and maturation of dendritic cells, (2) they stimulate the processes that lead to activated helper T-cells, and (3) they also promote the processes that result in activated cytotoxic T-cells.

eTheRNA was founded in January 2013 out of the VUB Laboratory for Molecular and CellularTherapy (LMCT) which has driven both the discovery and development of TriMix. This mRNA-based technology focuses on enhancing and modulating dendritic cells which fulfill a fundamental role in eliciting the human immune response to cancer and infectious diseases.

Building upon a convincing set of preclinical and clinical data in melanoma, eTheRNA has succeeded in raising EUR 24 million of proceeds. The round was led by LSP Life Sciences Partners (The Netherlands) and PMV (Belgium), who formed a strong international investment syndicate that also comprises Omega Funds (US). www.etherna.be



Based in the biotech hub of Martinsried just outside Munich, Germany, Ethris was founded by Dr. Carsten Rudolph and Prof. Christian Plank in 2009. We are a technical innovator in the emerging field of messenger RNA therapeutics. Ethris' proprietary Stabilized Non-Immunogenic mRNA (SNIM® RNA) platform includes a toolbox of technologies to generate mRNA molecules that are stable, result in production in the patient's own body of therapeutic proteins, and which escape recognition by the body's immune system. These mRNA technologies paired with Ethris proprie-tary delivery systems for organ- and cell- targeted delivery of mRNA has generated a pipeline of Transcript Therapies, which Ethris is developing to treat serious diseases. Ethris is focused inter-nally on advancing its products to address severe respiratory disorders, and works with corporate and academic partners across the globe to realize the potential for its patented SNIM® RNA Transcript Therapies in other areas of human disease. www.ethris.com

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Since 1996 TriLink has offered cutting edge services to researchers in the fields of gene therapy, nucleoside chemotherapy, oligonucleotide therapy and diagnostics. We specialize in the manufacture of modified nucleic acid products including custom oligonucleotides, mRNA, modified nucleoside triphosphates, CleanAmp™ PCR Products, phosphoramidites and other small molecules. TriLink's founders have been at the forefront of the advancements in oligonucleotide and mRNA synthesis. Our scientists and technicians have decades of collective experience in synthesizing nucleic acid products for research, diagnostics and therapeutic applications. www.trilinkbiotech.com

#### **BRONZE SPONSORS**



Axolabs is a leading preclinical research organization in the field of oligonucleotide therapeutics and nucleic acid medicines. Based on the foundations of the former Roche Center of Excellence for RNA Therapeutics, we leverage our world-leading know-how and 16+ years of experience to provide high-end preclinical solutions and consultancy tailored to our clients' specific needs. Our products and services include oligonucleotide manufacturing, analytics of nucleic acids, detection of oligonucleotides and mRNAs from biological matrices under the standards of GLP/GCP, oligonucleotide lead identification, functional cell type-specific delivery of oligonucleotides and mRNAs as well as a portfolio of biological and pharmacological services. www.axolabs.com

Contact: info@axolabs.com



Established in the mid 1970's, **New England Biolabs, Inc.** (NEB) is the industry leader in the discovery and production of enzymes for molecular biology applications and now offers the largest selection of recombinant and native enzymes for genomic research. NEB continues to expand its product offerings into areas related to PCR, gene expression, sample preparation for next generation sequencing, synthetic biology, glycobiology, epigenetics and RNA analysis. Additionally, NEB is focused on strengthening alliances that enable new technologies to reach key market sectors, including molecular diagnostics development. New England Biolabs is a privately held company, headquartered in Ipswich, MA, and has extensive worldwide distribution through a network of exclusive distributors, agents and seven subsidiaries located in Canada, China, France, Germany, Japan, Singapore and the UK. For more information about New England Biolabs visit www.neb.com.

Thank you for attending this year's International mRNA Conference.

See you next year in Germany!



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