



# Neugestaltung der Krebsbehandlung durch individualisierte Immuntherapie

Dr. Sierk Poetting, CFO  
Mainz, 23. März 2017

# Cancer is Personal

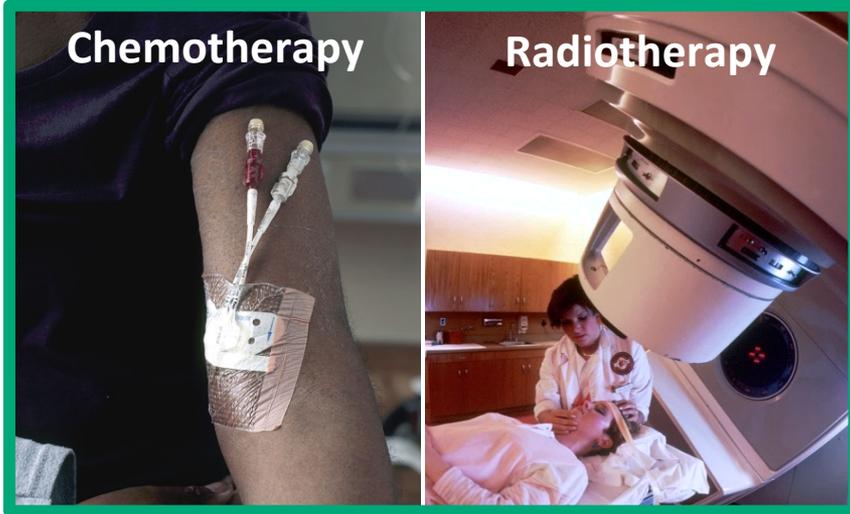
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*Every tumor is unique*



*This is why individualized medicine is so compelling*

# Limitations of Current Standard of Care



## Often Leading to the Doctor Consultation that Every Patient Dreads.....



### Today's Reality

*There are no more drugs available to treat your cancer....*

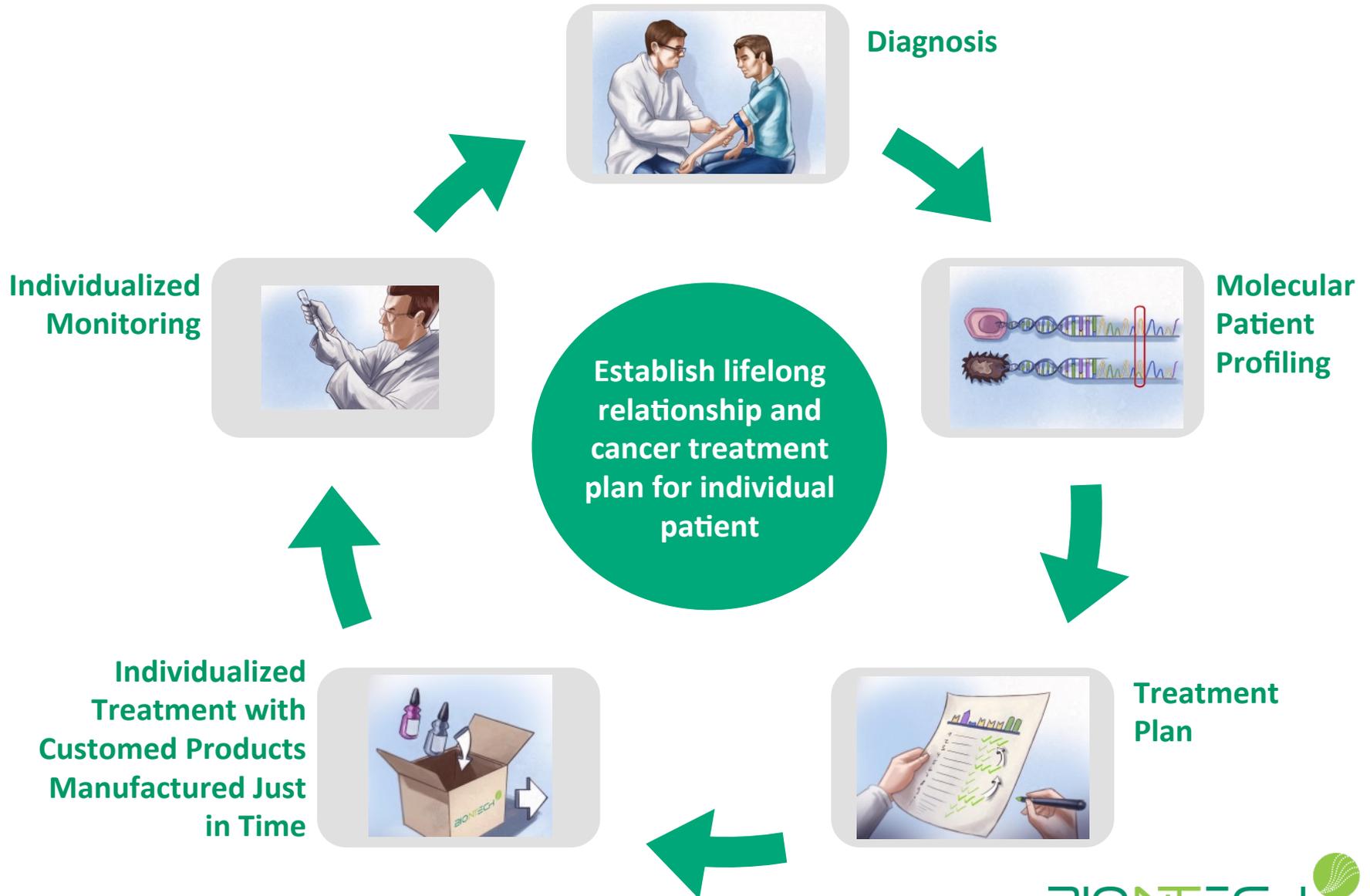
### Next Decade

*There are no more drugs available to treat your cancer....*

*but we are going to make a new drug for you*



# Transformation Over Next 10 Years: Individualized Treatment Model



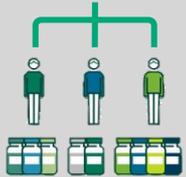
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# BioNTech's mission is to bring individualized, highly effective cancer therapies to market

BioNTech is the global leader in individualized immunotherapies



# At a Glance: Largest Privately Held Biotech in Europe



## Strategy

Bring individualized cancer immunotherapies to market



## Management

Prof. Dr. Ugur Sahin  
Sean Marett  
Dr. Sierk Poetting



**Founded in 2008**



## Differentiation

Tailor-made and individualized cancer immunotherapies



**4 Clinical Programs**



## Investors

Strüngmann Family Office  
MIG Fonds  
Salvia GmbH



**500 Employees**



**8 Affiliates, including in-house manufacturing**



## Partners



Parker Institute for Cancer Immunotherapy

# Disruptive Platforms in all major Immuno-Oncology Modalities

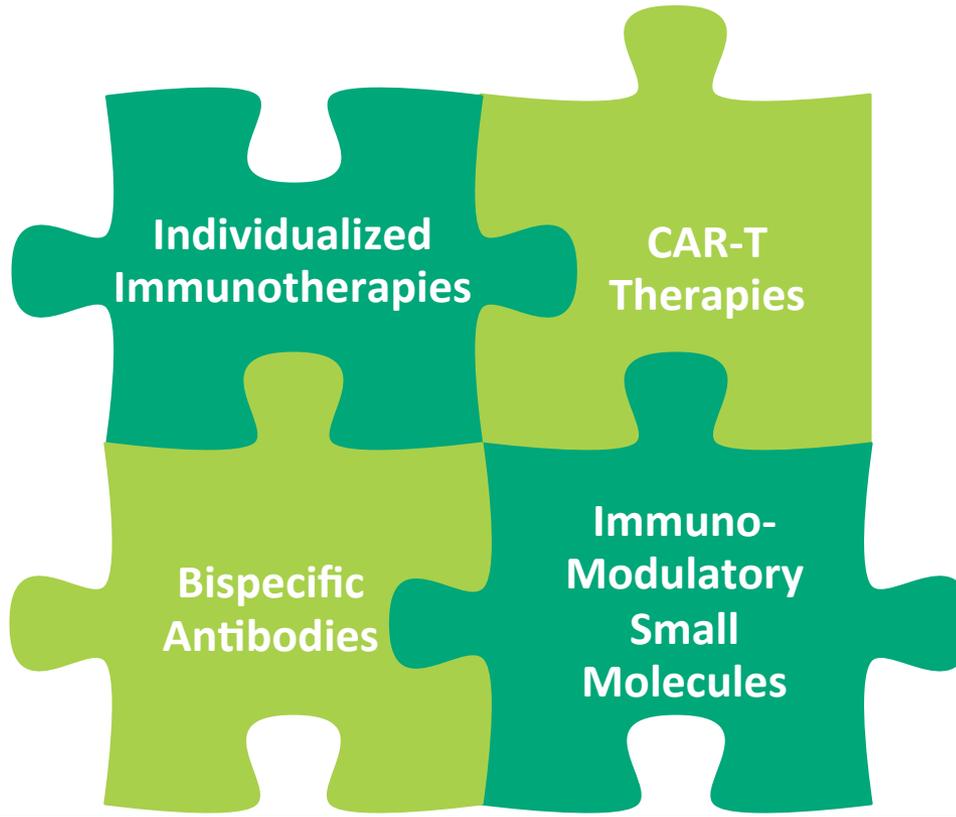
**BIONTECH**  **HOLDING** (with shared support functions)

mRNA Technology	Cell & Gene Therapy	Protein Therapeutics	Small Molecules
<p><b>Pharmacologically optimized protein coding RNA for targeted in vivo delivery</b></p> <ul style="list-style-type: none"> <li>• Cancer Immunotherapies</li> <li>• Prophylactic Vaccines</li> <li>• Protein Replacement</li> </ul> <p><b>BIONTECH</b>  RNA Pharmaceuticals</p>	<p><b>Immunotherapy with genetically engineered T cells, adoptive T cell transfer</b></p> <ul style="list-style-type: none"> <li>• T cell receptor therapies</li> <li>• CAR therapies</li> </ul> <p><b>BIONTECH</b>  Cell &amp; Gene Therapies</p>	<p><b>Engineered nano-particles for cancer immunotherapy</b></p> <ul style="list-style-type: none"> <li>• Bispecific antibodies</li> <li>• Microbodies</li> <li>• Virus-like-particles</li> </ul> <p><b>BIONTECH</b>  Protein Therapeutics</p>	<p><b>Small molecule drug discovery</b></p> <ul style="list-style-type: none"> <li>• TLR-agonists</li> <li>• Immuno-modulating small molecules</li> <li>• Drug discovery services</li> </ul> <p><b>BIONTECH</b>  Small Molecules</p>

 **BIONTECH**  **Diagnostics**
 **jpt** **Innovative Peptide Solutions**
**Diagnostics and Manufacturing Affiliates**
 **E-U-F-E-T-S**

## ... Allowing Combinations of Cancer Immunotherapies

Different treatment modalities for optimizing individualized therapies



# Multiple First-Mover Accomplishments in Immuno-Oncology

## First company worldwide to:

- Implement a **medicinal genomics-driven and GMP-approved manufacturing process** for individual patient-specific therapies
- Undertake clinical trials with:
  - an **mRNA-based individualized cancer vaccine targeting neo-antigens**
  - an **intravenous formulation of an mRNA vaccine**
  - an mRNA-based individualized vaccine drawn from a **warehouse of mRNAs** encoding cancer-selective antigens



# Immunotherapy Breakthrough Publications in Nature

Kreiter et al...Sahin, Nature 520, 692–696  
(30 April 2015)

LETTER

doi:10.1038/nature14426

## Mutant MHC class II epitopes drive therapeutic immune responses to cancer

Sebastian Kreiter<sup>1</sup>, Mathias Vormehr<sup>2\*</sup>, Niels van de Roemer<sup>2\*</sup>, Mustafa Diken<sup>1</sup>, Martin Löwer<sup>1</sup>, Jan Diekmann<sup>1,3</sup>, Sebastian Boegel<sup>1</sup>, Barbara Schrörs<sup>1</sup>, Fulvia Vascotto<sup>1</sup>, John C. Castle<sup>1</sup>, Arbel D. Tadmor<sup>1</sup>, Stephen P. Schoenberger<sup>4</sup>, Christoph Huber<sup>2</sup>, Özlem Türeci<sup>1,2,3</sup> & Ugur Sahin<sup>1,2,3</sup>§

The  INDEPENDENT

Tailor-made cancer vaccine raises hopes of personal drugs

'Personalised' cancer vaccine moves a step closer

Allgemeine Zeitung

RHEIN MAIN PRESSE

Mainz: Wissenschaftler setzen auf neuen Therapieansatz bei der Bekämpfung von Tumoren - erfolgreiche Studie mit Mäusen

Daily  Mail  
Personalised drugs could revolutionise the war on cancer

BIOWORLD™

The Daily Biopharmaceutical News Source  
Biontech gives scientific rationale for personalized RNA cancer vaccines

THE HUFFINGTON POST

Cancer Research Moves Towards Revolutionary Personalised Vaccines



THE TIMES

Vaccine helps body break down cancer

Kranz et al...Sahin Nature 534, 396–401  
(16 June 2016)

LETTER

doi:10.1038/nature18300

## Systemic RNA delivery to dendritic cells exploits antiviral defence for cancer immunotherapy

Lena M. Kranz<sup>1,2\*</sup>, Mustafa Diken<sup>1,3\*</sup>, Heinrich Haas<sup>3</sup>, Sebastian Kreiter<sup>1,3</sup>, Carmen Loquai<sup>4</sup>, Kerstin C. Reuter<sup>3</sup>, Martin Meng<sup>3</sup>, Daniel Fritz<sup>3</sup>, Fulvia Vascotto<sup>1</sup>, Hossam Hefesha<sup>3</sup>, Christian Grunwitz<sup>2,3</sup>, Mathias Vormehr<sup>2,3</sup>, Yves Hüseemann<sup>3</sup>, Abderraouf Selmi<sup>1,2</sup>, Andreas N. Kuhn<sup>3</sup>, Janina Buck<sup>3</sup>, Evelyn Derhovanessian<sup>3</sup>, Richard Rae<sup>1</sup>, Sebastian Attig<sup>1,2</sup>, Jan Diekmann<sup>3</sup>, Robert A. Jabulowsky<sup>3</sup>, Sandra Heesch<sup>3</sup>, Jessica Hasset<sup>5</sup>, Peter Langguth<sup>6</sup>, Stephan Grabbe<sup>4</sup>, Christoph Huber<sup>1,3</sup>, Özlem Türeci<sup>1,2,3</sup> & Ugur Sahin<sup>1,2,3</sup>§

theguardian

Immunotherapy: the big new hope for cancer treatment

Daily  Mail

One step closer to the Holy Grail of a universal cancer vaccine: Therapy masks the disease as an invading virus - prompting the body to mount an attack

THE  TIMES

Immune system tricked into combating cancer

FiercePharma

Experimental 'universal' cancer vaccine tested in early trial

THE HUFFINGTON POST

A Universal Cancer Vaccine Closer To Becoming A Reality After Genetic Breakthrough

The Telegraph

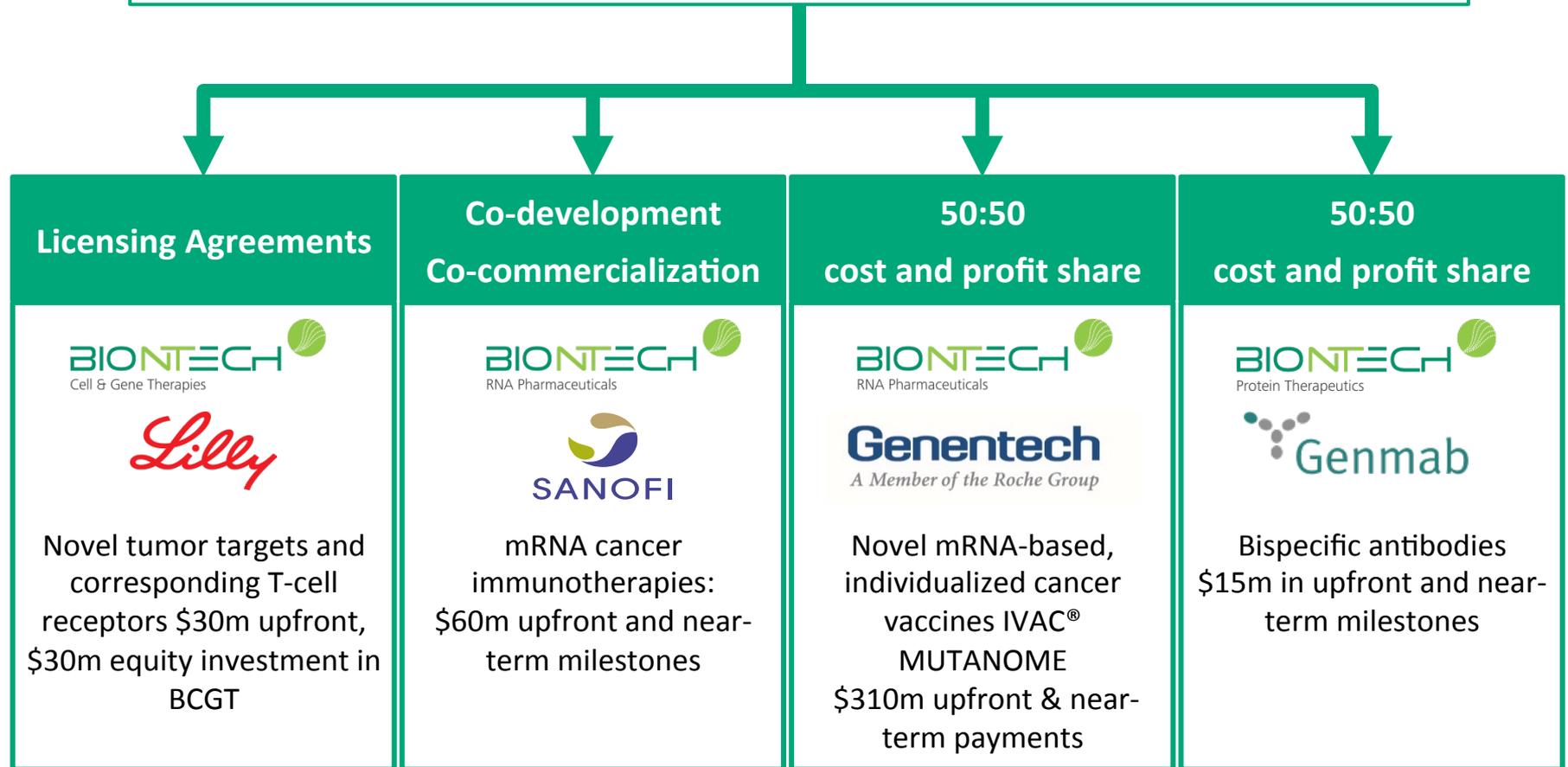
Universal cancer vaccine on horizon after genetic breakthrough

Wirtschafts  Woche

Erste universelle Krebsimpfung in Sicht

# Validation Deals Across Key Immuno-Oncology Platforms

Step-wise, first-mover, commercialization strategy through selected partnering deals and propriety products to market strategy

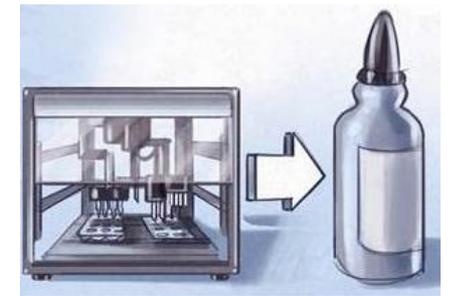
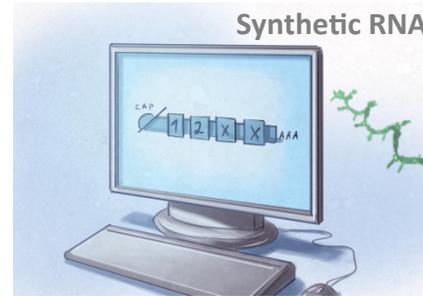


# IVAC<sup>®</sup> Cancer Vaccines Custom-Made For The Individual Patient

← Disruptive technologies →

## Cancer mutation discovery & target prioritization

## Synthetic vaccine design & production

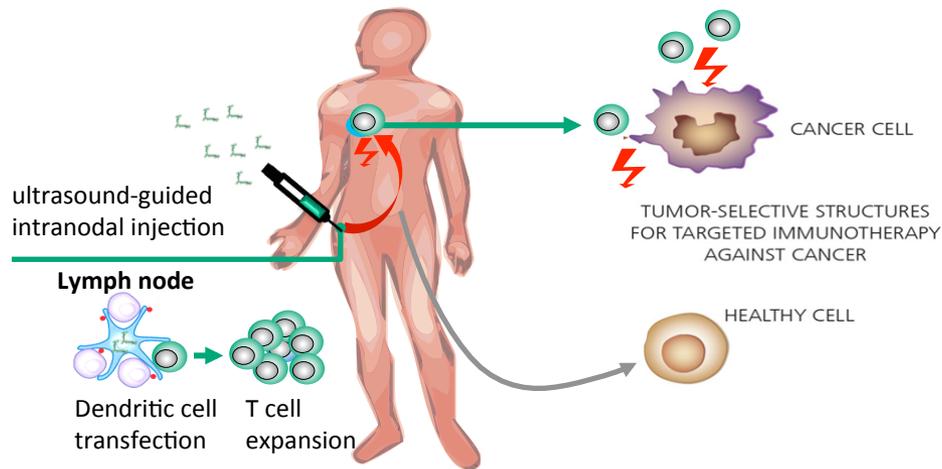


↑ Individualized Cancer Immunotherapy

# IVAC<sup>®</sup> Mutanome: Stimulates Patients' Immune System to Attack Cancer

Phase I/IIa **clinical evidence** demonstrates:

- IVAC<sup>®</sup> Mutanome **stimulates the human immune system** successfully
- BioNTech-optimized, synthetic mRNA with a 5000 times increase in potency induces **mutation-specific T cells**

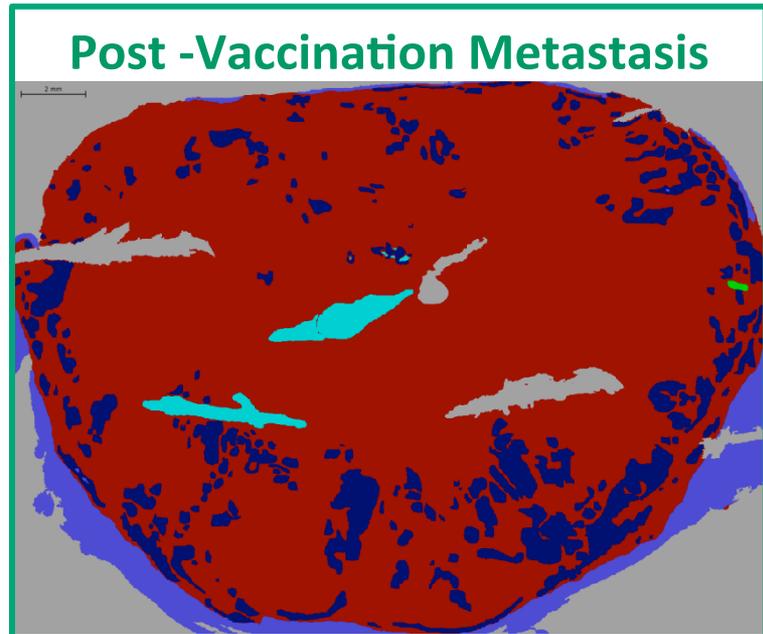
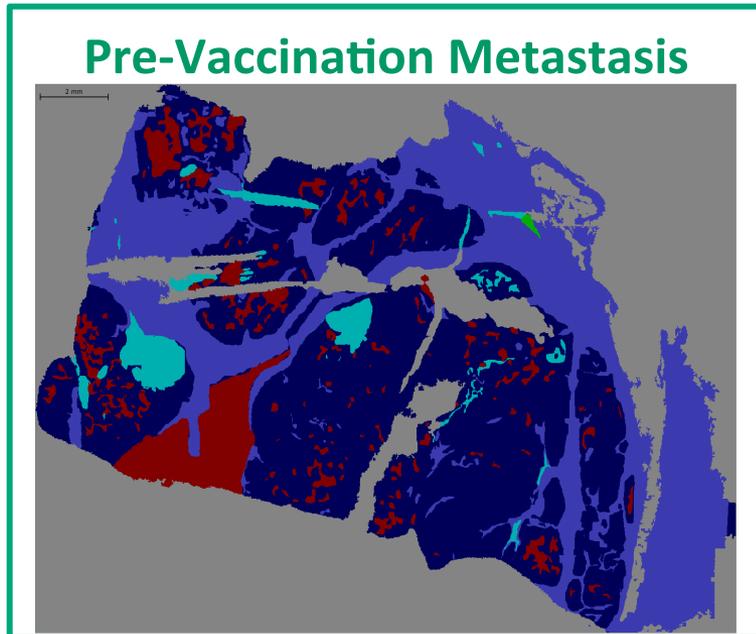


## Clinical validation of the induction of mutation specific T cells



# IVAC<sup>®</sup> Mutanome: Evidence of Patient Antitumor Activity

## Computerized analyses of necrotic tumor area from melanoma metastasis\*



\* Definiens Imaging Software

- Normal tissue
- Tumor tissue
- Necrosis
- Whitespace
- Background
- Artefact

- Targeting neoepitopes with RNA Mutanome  
Vaccine is **safe**
- Vaccine induces **neoepitope specific CD4+ and CD8+ T cell responses** and **neoepitopic specific T cells that infiltrate tumor lesions**
- Evidence of medically relevant **antitumoral activity**

# IVAC<sup>®</sup> Mutanome Individualized Therapy Poised to Address Majority of Cancers in All Patients

Drug Class	Chemotherapy Drugs e.g. Abraxane <sup>®</sup>	Growth Inhibitors e.g. Herceptin <sup>®</sup>	Mutation Specific Drugs e.g. Xalkori <sup>®</sup>	BNT Mutanome Drugs IVAC <sup>®</sup> Mutanome
<i>Cancer Cell Specificity</i>	No	No	Yes	Yes
<i>Molecular Targets</i>	Proliferating Cells	Growth Receptors	1 Mutation	10-20 Mutations
<i>Adaptable to Disease Evolution</i>	No	No	No	Yes
<i>Toxicity Tolerability</i>	Severe Toxicity	Moderate Toxicity	Excellent Tolerability	Excellent Tolerability (n=55*)
<i>Applicability to cancer types</i>	All patients, a few cancer types 	Subgroups of patients, a few cancer types 	Subgroups of patients, limited cancer types 	All Patients, many cancer types 

\* Cumulative patient number treated to date with any BioNTech mRNA vaccine

# BioNTech: A truly unique biotechnology company for the 21st Century

## We are

- Largest privately held immunotherapy cancer company in Europe
- Disruptive
- Clinical stage
- 500 people
- Tailor-making treatments for each cancer patient

## We have

- Assembled all key immunotherapy platform technologies under one roof
- A revolutionary clinical pipeline with most of the pipeline unpartnered
- Pharma validation deals in key platforms netting \$450m in upfront/near-term payments and supported by two publications in Nature
- Built in-house clinical manufacturing platforms and use latest advances in artificial intelligence, robotics and automation for commercial manufacture

## We plan

- To continue to aggressively expand in 2017 and beyond
- To maintain and increase our leadership position in individualized cancer immunotherapy

# Thank you for your attention

## BioNTech: Bringing individualized, highly effective cancer therapies to



**BioNTech Headquarters**  
Mainz, Germany  
6000 m<sup>2</sup> Labs and Offices  
1000 m<sup>2</sup> additional Offices in Mainz  
ISO13485, ISO9001, GMP, GCLP, GCP



**BioNTech / JPT**  
Berlin  
2300 m<sup>2</sup> Labs and Offices  
Distributors in 13 countries  
ISO9001, BSL2, Clean Rooms



**BioNTech / EUFETS**  
Idar-Oberstein, Germany  
1100 m<sup>2</sup> Labs and Offices  
incl. 500 m<sup>2</sup> GMP facilities  
GMP, GLP, BSL 2, BSL 3



**BioNTech Small Molecules**  
Planegg-Martinsried, Germany  
1600 m<sup>2</sup> Labs and Offices



**BioNTech RNA Synthesis**  
Dedicated Single Patient  
Batch Manufacturing Unit  
in Mainz  
(under construction)

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55131 Mainz  
Germany

[www.biontech.de](http://www.biontech.de)