



# Neugestaltung der Krebsbehandlung durch individualisierte Immuntherapie

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

# Cancer is Personal

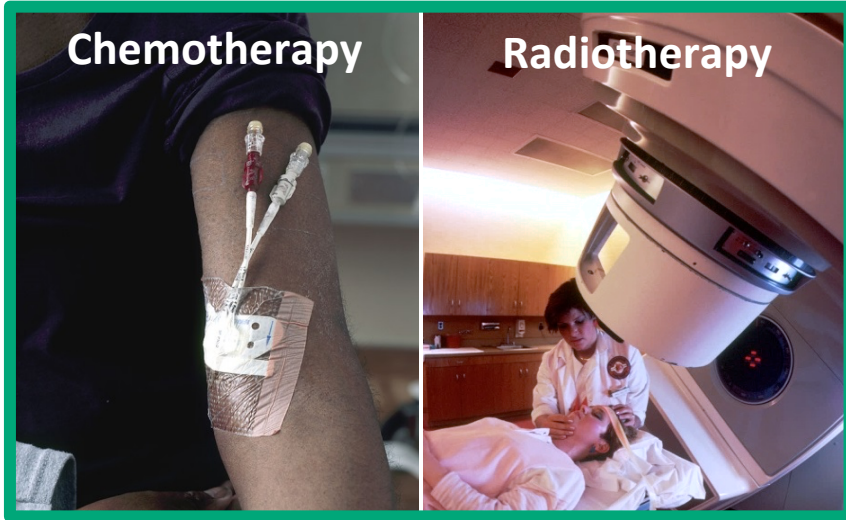
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*Every tumor is unique*



*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

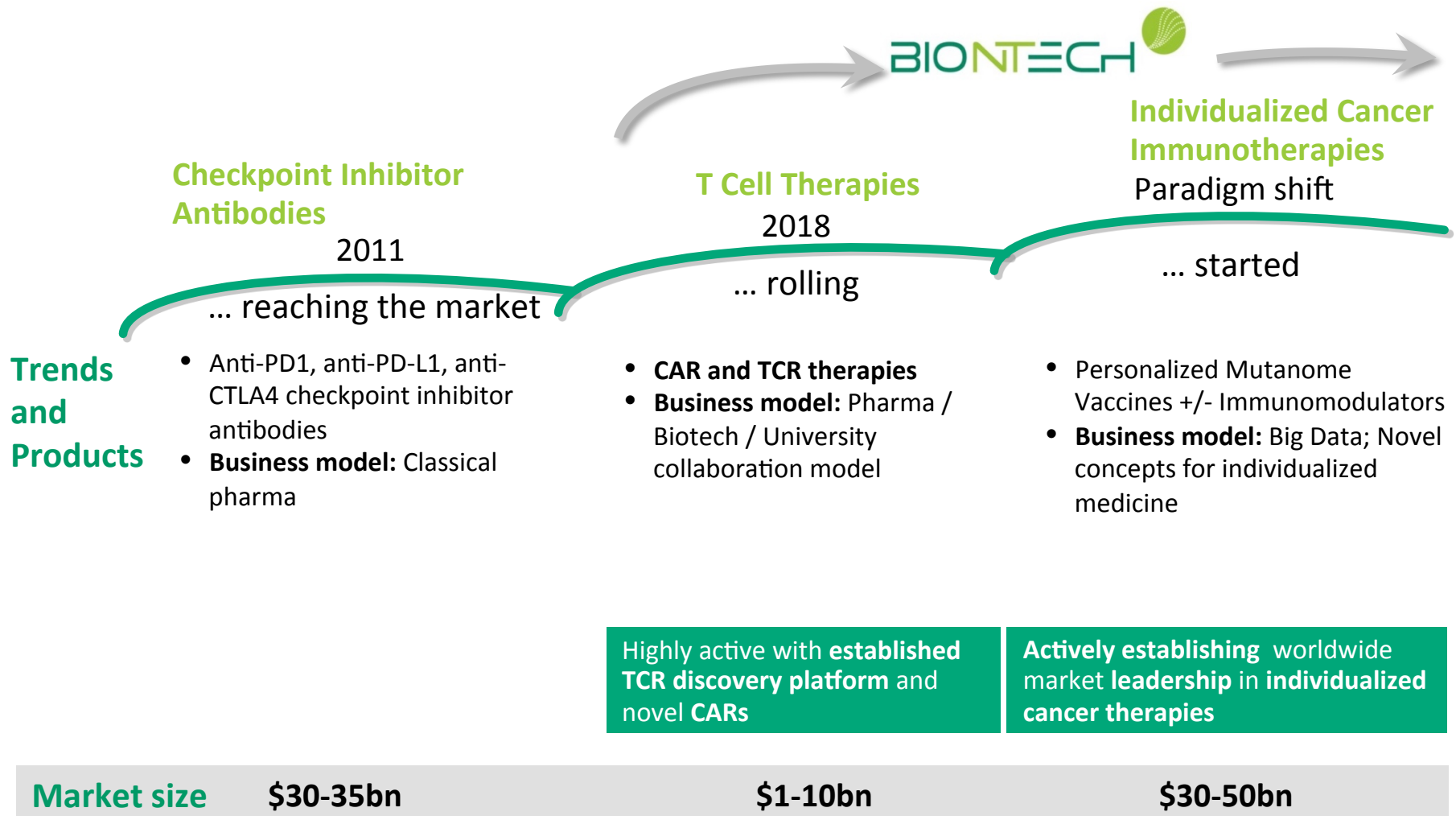
*There are no more drugs available to treat your cancer....*

### Next Decade

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

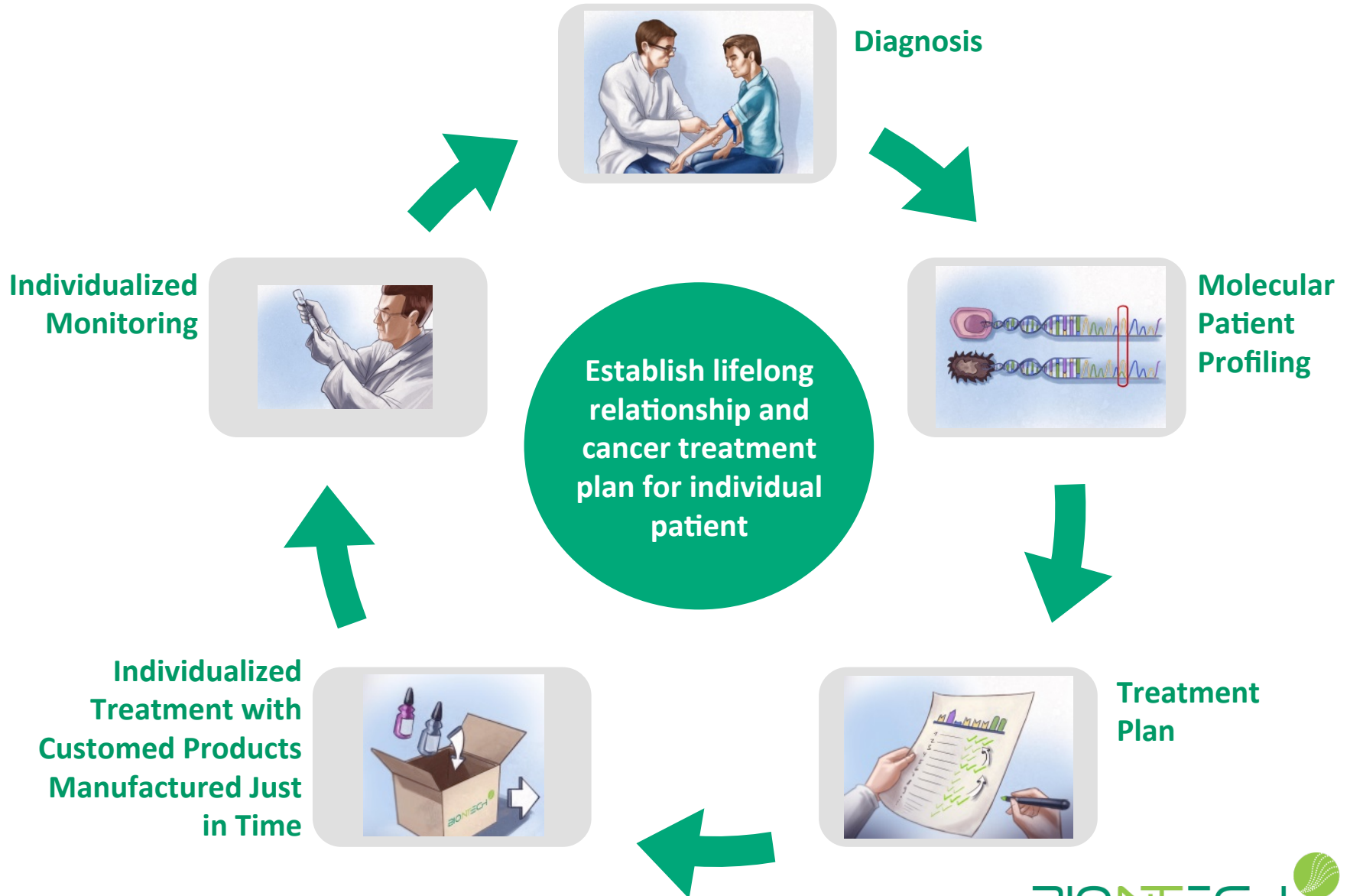
*but we are going to make a new drug for you*

# Pioneering Immunotherapy





# Transformation Over Next 10 Years: Individualized Treatment Model

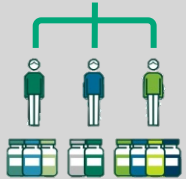


# BioNTech's mission is to bring individualized, highly effective cancer therapies to market

BioNTech is the global leader in individualized immunotherapies



# At a Glance: Largest Privately Held Biotech in Europe



## Strategy

Bring individualized cancer immuno-therapies 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



**Genentech**  
A Member of the Roche Group



**SANOFI**

**Ci3**





**TRON** Genmab

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

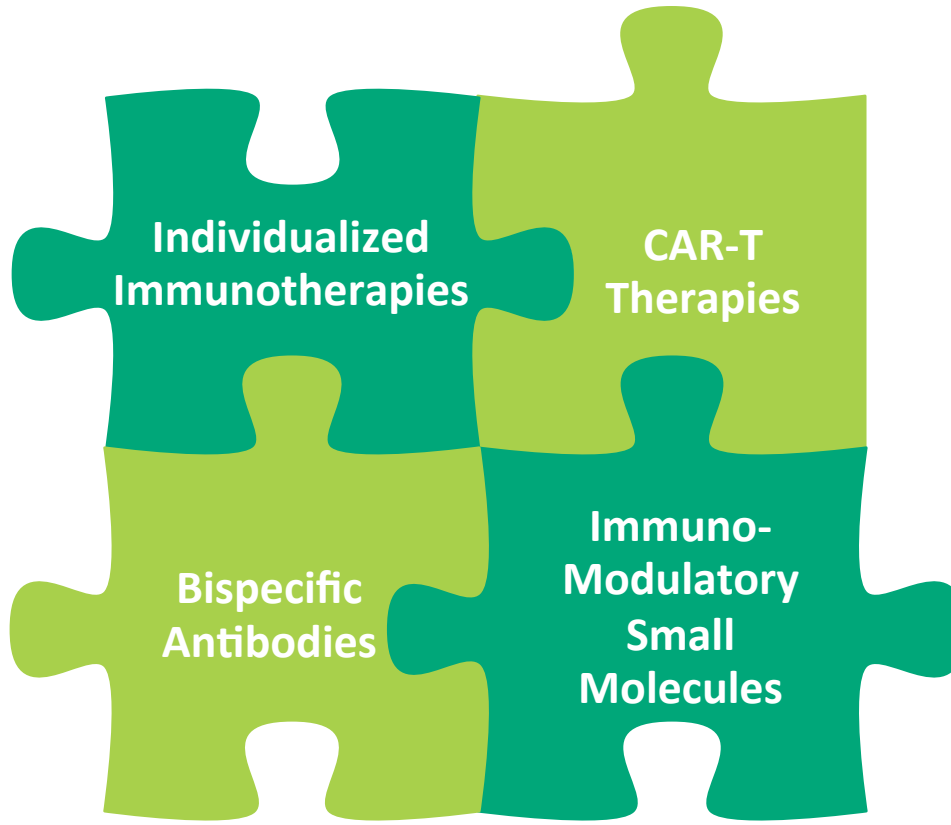
**jpt**  
Innovative Peptide Solutions

**Diagnostics and Manufacturing Affiliates**



## ... 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>1,2,3,5</sup> & Ugur Sahin<sup>1,2,3,5</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

## BIO WORLD™

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



## THE TIMES

Vaccine helps  
body break  
down cancer

## THE HUFFINGTON POST

Cancer Research Moves Towards Revolutionary Personalised Vaccines

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 Hüsemann<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 Hassel<sup>5</sup>, Peter Langguth<sup>6</sup>, Stephan Grabbe<sup>4</sup>, Christoph Huber<sup>1,3</sup>, Özlem Türeci<sup>7,8</sup> & Ugur Sahin<sup>1,2,3,9</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

## THE HUFFINGTON POST

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

## FiercePharma

Experimental 'universal' cancer vaccine tested in early trial

## The Telegraph

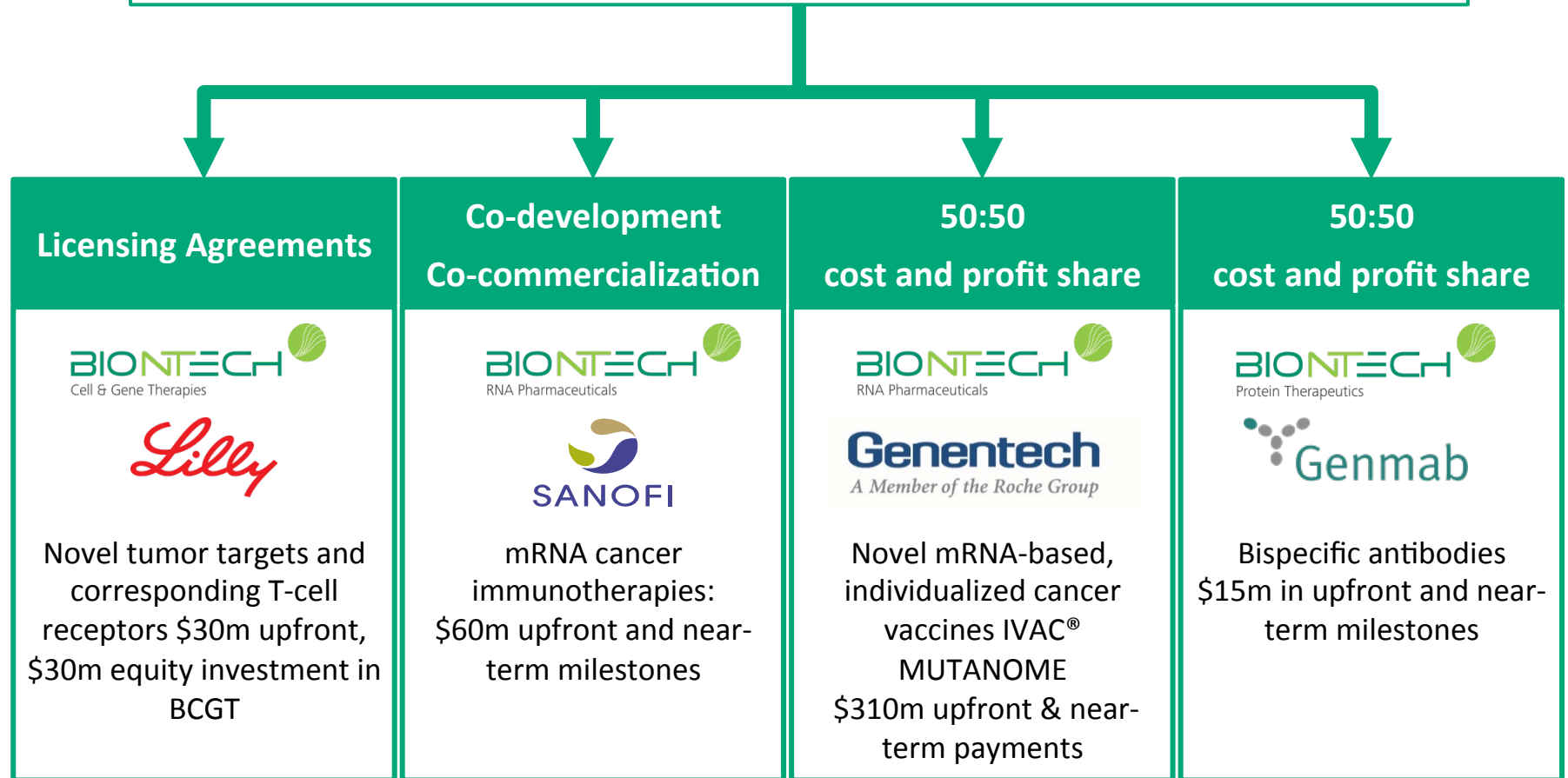
Universal cancer vaccine on horizon after genetic breakthrough

## 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® Cancer Vaccines Custom-Made For The Individual Patient

← Disruptive technologies →

Cancer mutation discovery & target prioritization

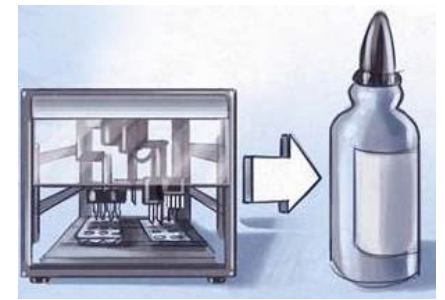
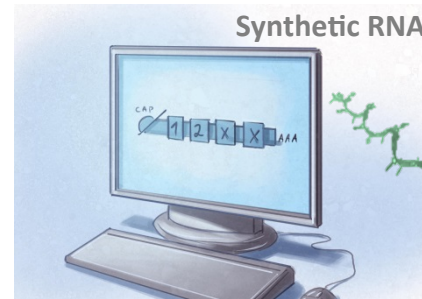
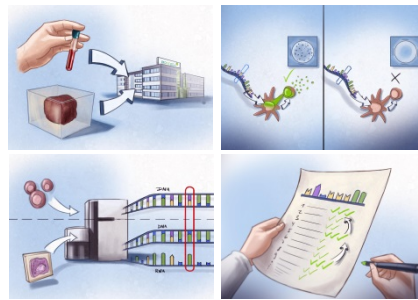
Synthetic vaccine design & production

Diagnosis

Mutanome Analyses

IVAC® Vaccine  
Tailoring

Just-in-time tailored  
synthetic RNA drugs

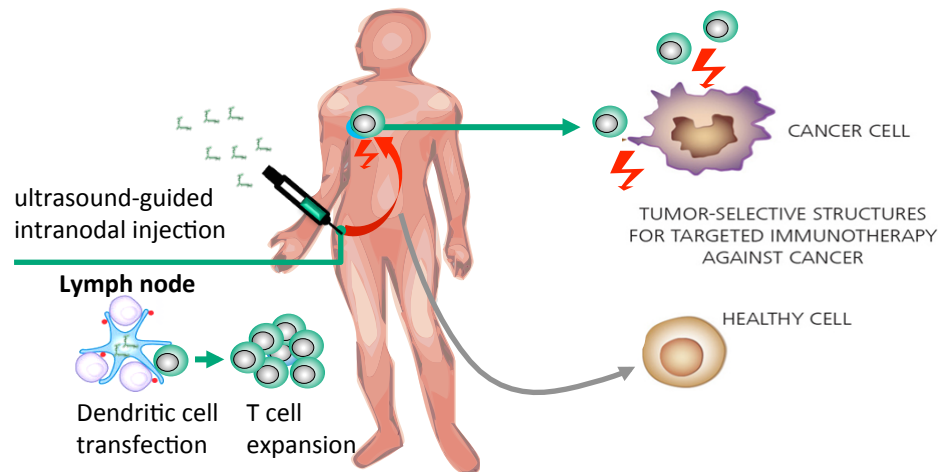


Individualized Cancer Immunotherapy

# IVAC® Mutanome: Stimulates Patients' Immune System to Attack Cancer

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

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

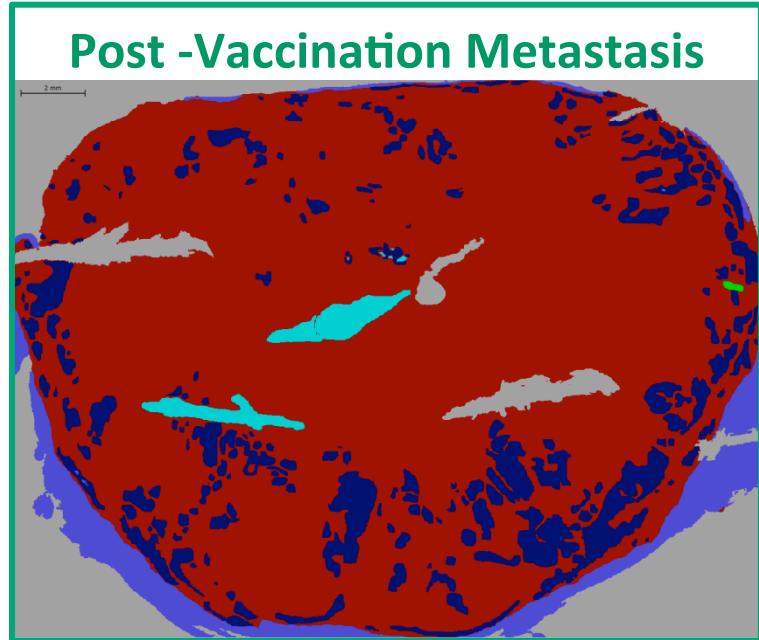
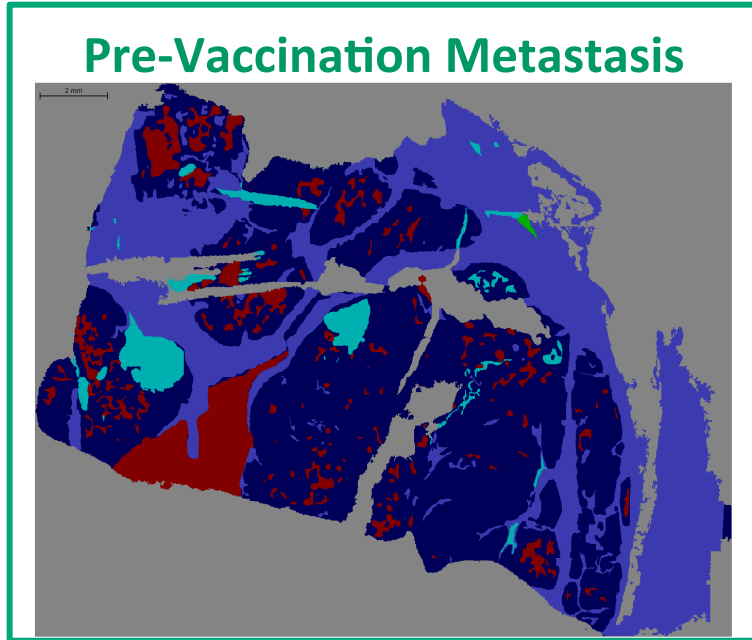


## Clinical validation of the induction of mutation specific T cells



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

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

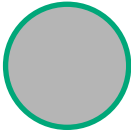





\* Definiens Imaging Software

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

- Targeting neopeptides with RNA Mutanome Vaccine is **safe**
- Vaccine induces **neopeptide specific CD4+ and CD8+ T** cell responses and **neopeptide 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)

**BioNTech AG**  
An der Goldgrube 12  
55131 Mainz  
Germany

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