

## Nature publication describes first example of a clinically applicable and systemic mRNA cancer immunotherapy vaccine

### *Major step towards truly personalized treatment of any cancer*

- Elegantly simple, charge-adjusted RNA lipid complexes are easy to manufacture and can be systemically and easily administered to effectively target dendritic cells in key organs
- Avoids need to engineer complex targeting ligands
- Rapidly stimulates highly potent innate and adaptive immune response akin to anti-viral defense in both pre-clinical tumor models and early human studies
- Any peptide-based antigen or epitope can be encoded as RNA making this type of immunotherapy applicable to all cancer types

**Mainz, Germany, June 01, 2016** – BioNTech AG, a fully integrated biotechnology company developing individualized cancer immunotherapies, together with its translational research partner institute TRON, announces a publication in the internationally renowned scientific journal Nature, describing the first example worldwide of a clinically relevant and systemic mRNA cancer immunotherapy. The study entitled “Systemic RNA delivery to dendritic cells exploits antiviral defense for cancer immunotherapy” can be found here: <http://dx.doi.org/10.1038/nature18300>

The paper outlines a novel and elegantly simple approach to target a nanoparticle mRNA vaccine (RNA-LPX) body-wide to dendritic cells in the spleen, lymph nodes and bone marrow, where a highly potent, dual-mechanism immune response mimicking a natural antiviral immune response is rapidly elicited. The dual mechanism involves both adaptive (T-cell-mediated) and innate (type-I interferon (IFN)-mediated) immune responses, with the IFN response being essential for full anti-tumor effects of the vaccines.

**Professor Dr. Ugur Sahin, CEO of BioNTech AG commented:** “Our study introduces a novel class of extraordinarily potent cancer vaccines that enables efficient redirection of the immune system against a wide range of tumor antigens. This is a major step towards our aim to make truly personalized cancer immunotherapies available and applicable to all cancer types.”

The publication presents new findings from the interdisciplinary collaboration between scientific and clinical teams at BioNTech AG, TRON, Research Center for Immunotherapy (FZI) and partners at the University Medical Center at the Johannes Gutenberg University Mainz and the Heidelberg University Hospital. The Group has pioneered individually tailored mRNA cancer vaccines and aims to progress clinical development to provide cancer patients and their treating physicians with new therapy options.

The paper further provides mode of action and efficacy data for this novel vaccine class in several preclinical tumor models and reports early data from a phase I dose-escalation, safety and tolerability trial (NCT02410733) of an intravenous RNA-LPX vaccine in melanoma patients. Crucially, in these patients, very low initial doses, lower than those used in preclinical studies, very rapidly elicited such a strong CD4+ and CD8+ T cell response that *ex vivo* culture was not required for detection. To date this vaccine has been very well tolerated and no severe toxicities have been observed.

The phase I melanoma study continues to recruit patients and BioNTech is planning and executing additional RNA-LPX vaccine studies for different cancer types. BioNTech owns all commercial rights for exploitation of the entire concept.

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**About BioNTech AG**

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

**About TRON - Translationale Onkologie an der Universitätsmedizin der Johannes Gutenberg-Universität Mainz gGmbH**

TRON, or Translational Oncology at the University Medical Center of the Johannes Gutenberg University, is located in Mainz, Germany and is a not-for-profit biopharmaceutical research organization. TRON integrates cutting-edge technologies and interdisciplinary expertise in genomics and next-generation sequencing, bioinformatics and biostatistics, immunology, immunotherapy, and molecular biology to accelerate transfer of novel concepts and innovation from basic research into clinical applications. With its core competencies of highly specialized technologies and methods, TRON supports academic institutions, biotech companies and the pharmaceutical industry in the development of innovative products.

More information about TRON can be found at [www.tron-mainz.de](http://www.tron-mainz.de).