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Press release

More than just a messenger - The secret potential of RNA therapeutics in fighting cancer and respiratory diseases

Alcimed, an innovation and new business company, presents an outline of the future potential of messenger RNA (mRNA) therapeutics in addressing unmet needs

28 July 2017, Alcimed Cologne, Germany - **Current therapy approaches hold drawbacks and challenges.** Medical therapies have advanced greatly over the past decades of research and many have been proven effective in combating diseases and enhancing the patient´s quality of life. The use of antibodies, growth-factors and plenty of other complex biomedical drugs are just a few examples of modern medicine. Despite the fact that these conventional therapies are often life-saving, they do often harbor strong side-effects.

A new class of drugs with a strong future prospect

The idea of mRNA-based therapy approaches seems quite straight forward and simple: If a patient is unable to produce a specific protein by themselves, or a defect version of it, specialists can inject the patient´s cells with the mRNA that encodes the desired protein as a replacement.
The main advantage of messenger RNA (mRNA)-based therapy approaches is the high specificity of its target, cost-effective production alongside a minimal amount of side effects and toxicity. Compared to the strategy of directly editing at the patient´s gene level or using recombinant proteins produced by bacteria, injecting single molecules of mRNA before it produces the protein of choice is considered less dangerous and better tolerated by the patient. Most importantly, mRNA can be specifically modified in order for this molecule to enter literally any cell of the human body and replace almost any given protein. With the advantages certain challenges still need to be overcome such as to identify (i) the suitable route of administration into the correct cell types, (ii) specific modifications to avoid harmful immune reactions and (iii) specific formulations to protect the mRNA from degradation.

The use of mRNA as a therapeutic has been under investigation since the early 1990s and has recently come back into focus as a potential new class of drugs to deliver genetic information. mRNA can be synthetically custom engineered by in-vitro transcription and represents a new platform for developing effective and precise therapy approaches for both, therapeutic as well as prophylactic vaccines.

“If clinical development is successful demand for messenger RNA based therapeutics could quickly increase in the future and companies active in the field aim for an annual production yield of hundreds of grams and more ”, Dr. Volker Bischoff.

Many mRNA therapeutics currently focus on oncology

mRNA therapeutics often target patients in the field of oncology, where the treatment against cancer is very much specific to each patient and requires individual gene therapy. Therapeutic cancer vaccines with a variety of targets have been developed with many more to follow and the first clinical trials are potentially heading towards Phase III, with a clear oncological focus in prostate cancer and advanced melanoma (currently ~13 clinical trials ongoing in oncological indications).
Besides the vast field of oncology, mRNA-based therapy approaches hold a great potential in combating respiratory diseases. For patients suffering from Cystic Fibrosis (CF), for example, existing therapies are

not able to satisfy the current unmet medical needs. mRNA which transcribes for the “healthy” version of the CFTR (Cystic Fibrosis Transmembrane Conductance Regulator), a protein which in CF patients is defect, can be applied via nebulization into the patient´s lung for direct uptake into lung-resident cells. Especially for CF, some patients are not properly treatable with any existing therapy which makes the mRNA-based therapy approach an indispensable mean for treatment.

“The field of oncology currently represents the most advanced field for messenger RNA therapeutics with therapeutic vaccines in clinical development in various oncological diseases”, Dr. Volker Bischoff.

The big players in mRNA

mRNA-based therapeutics have gone through an exceptional development and it has therefore generated a lot of excitement recently. When looking at the global players in clinical mRNA research, basically three major players come into mind: the two Germany-based companies CureVac (Tübingen) and BioNTech (Mainz) as well as Moderna Therapeutics (Cambridge, MA) in the United States.
The commendable funding through various, well-known investors like the Bill & Melinda Gates Foundation and several Big Pharma companies from around the world have accumulated several hundreds of millions of Dollars for the research in mRNA; let alone the $1 billion which was granted solely to Moderna Therapeutics.

“With Moderna (USA), CureVac and BioNTech (both DE) three key players lead the development of mRNA therapeutics, drive innovation and have obtained large funding to further develop their research activities” *Dr. Volker Bischoff.*