Innovative lipid nanoparticles for cell and gene therapy

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The overall aim of the project is to develop a novel process for the delivery of nucleic acid-based drugs into human T cells to make the gene-editing strategies of innovative cell and gene therapies (CGT) more effective, safer and less expensive. The clinical need for such molecularly modified CGT products with improved therapeutic efficacy is high, as they have already provided cures for some chronic immunological and tumor-related diseases. Current transfection methods, which are mainly based on retro/lentiviral vectors, have a number of limitations and are characterized by inefficiency, high costs and safety risks.

In the “NanoGen” project, in which a team of scientists from Charité led by Prof. Petra Reinke and a team of experts from Fraunhofer IPK led by Dr. Christoph Hein are working together on an interdisciplinary basis, non-viral multiplex gene editing methods in T cells for potential CGT are being developed and extensively tested in vitro and in vivo to subsequently test these results in clinical trials. All preclinical development work is focused as proof-of-concept (PoC) on an innovative multiplex T-cell therapy for treating B-cell-mediated autoimmunity.

This means that Fraunhofer IPK's novel proprietary FDmix mixing technology for drug delivery using lipid nanoparticles (LNP) will be exploited for the application of gene editing of immune cells. This technological solution leads to a greatly improved particle morphology with significantly increased transfection efficiency.

The adaptation is crucial to create a stable, efficient and scalable platform for the next generation of CGTs. A central aspect of the project is the parallel development of an application-specific lipid formulation for different Cargos, with a focus on transfection and cellular uptake of the LNPs into human T cells.