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Synthetic mRNAs as optimised tools for stem cell generation and for manipulating cellular phenotypes

Availability of synthetic mRNAs enabled progress in their applications. Tremendous interest of private investors and PHARMA has created a billion € business. AmpTec supports new players by providing customized, high quality mRNA products. Important features, technical options for high-amount, high-quality mRNA synthesis and GMP-compliant manufacturing will be presented. Specific mRNA features will be presented for diverse applications like (i) mRNA-directed expression of antigens in dendritic cells for vaccination projects in oncogenesis, infectious disease and allergy prevention; (ii) reprogramming of adult cells to induced pluripotent stem cells with their subsequent differentiation to the desired cell type; (iii) applications in gene therapy. A recent overview has summarised applications and syn-mRNA quality requirements. Syn-mRNAs can be generated by *in vitro* transcription (IVT) from defined templates containing the synthetic gene of interest. In principle, linearised plasmids (with a restriction enzyme) can be used directly as templates in IVT reactions, However, this procedure is hampered by several disadvantages: incomplete plasmid cleavage results in variable amounts of very long and

undefined background transcripts; high amounts of plasmid DNA introduce undesired bacterial components. Furthermore, optimal mRNA activity depends on a very long, unmasked poly(A) tail, like 120 A. However homopolymeric repeats are prone to random deletions/elongations during plasmid propagation in bacteria. Instead of plasmids, we use well defined PCR-products as IVT-templates. This approach with examples will be shown. Technical problems in IVT-based mRNA synthesis and problem-solutions will be presented, plus a detailed list of quality requirements for GMP-compliant synthetic mRNAs.

Speaker Biography

Guido Krupp, PhD, is the CEO and President of AmpTec GmbH. In 1981, he received PhD degree from Würzburg University & Max-Planck-Institute Martinsried. From 1983 to 1987, he was a Post-doc at Yale University. From 1987 to 2002, he worked as Research Group Leader at Kiel University. He is also the Founder of Artus GmbH (1998) & AmpTec GmbH (2005) & KSK Diagnostics GmbH (2015). His primary area of research includes nucleic acid technology with focus on RNA, plant pathogens (viroids), ribozymes and telomerase. He has more than 60 publications, Editor of *Ribozyme Biochemistry & Biotechnology*, and of *Telomeres, Telomerases & Cancer*, Editorial Board Member of *Biotechnology Annual Review*.

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