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Synthetic messenger RNA (MRNA) as a therapeutic tool

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Disorders caused by missing or defective protein synthesis can lead to early-onset complications and the patients need a life-long treatment. Today, treatment options are often limited or ineffective and associated with high costs. Here, the *in vitro* transcribed (IVT) mRNA can be used as a potential drug. Synthetic mRNA has several advantages over conventional gene therapy and protein substitution strategies. The mRNA-based therapy is founded on the induction of the transient translation with the cell own ribosomes of fully functional proteins without integration into host genome. This approach minimizes the risk of mutagen and carcinogen effects. The control of translation duration as well as immunogenicity of mRNA can be achieved though different modifications and provide for different therapeutically applications, like replace or supplement proteins. Moreover, mRNA cocktails contacting mRNAs encodes for different proteins can be applied to induce the expression of different proteins simultaneously in one cell. The use of mRNA to develop therapeutic drugs opens up new perspectives and challenges in disease treatment.

Recent Publications:

1. T Michel, D Luft, M-K Abraham, S Reinhardt, M L. Salinas Medina, J Kurz, M Schaller, M Avci-Adali, C Schlensak, K Peter, H P Wendel, X Wang, S Krajewski (2017) Nanoliposomes meet mRNA: Efficient delivery of modified mRNA using hemocompatible and stable vectors for therapeutic applications, Molecular Therapy – Nucleic Acids
2. Abraham MK, Peter K, Michel T, Wendel HP, Krajewski S, Wang X (2017) Nanoliposomes for Safe and Efficient Therapeutic mRNA Delivery: A Step Toward Nanotheranostics in Inflammatory and Cardiovascular Diseases as well as Cancer, Nanotheranostics
3. Tatjana Michel, Hans-Peter Wendel and Stefanie Krajewski (2016) Next-Generation Therapeutics: mRNA as a Novel Therapeutic Option for Single-Gene Disorders, Book: Modern Tools for Genetic Engineering, InTech
4. Michel T, Kankura A, Salinas Medina ML, Kurz J, Behring A, Avci-Adali M, Nolte A, Schlensak C, Wendel HP, Krajewski S (2015) *In Vitro* Evaluation of a Novel mRNA-Based Therapeutic Strategy for the Treatment of Patients Suffering from Alpha-1-Antitrypsin Deficiency, Nucleic Acid Therapeutics
5. Avci-Adali M, Hann L, Michel T, Steinle H, Stoppelkamp S, Stang K, Narita M, Schlensak C, Wendel HP (2015) *In vitro* test system for evaluation of immune activation potential of new single-stranded DNA-based therapeutics, Drug Testing and Analysis
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Biography

Tatjana Michel works with synthetic modified mRNA treatment strategy since 2013. Her work is focused on the development and evaluation of mRNA-based drugs for rare monogenetic diseases like alpha-1-antitrypsin deficiency or familial hypercholesterolemia. Prior to that, she worked on strategies for cell trans differentiation for heart regeneration and investigated immunogenic effects caused through synthetic nucleic acids.

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