Abstract

Early DNA-based therapies were tested for therapeutic applications, but they sooner or later revealed multiple hurdles and risks preventing their use in further clinical trials. Recently, they have been replaced by rapidly evolving gene editing using programmed nucleases capable of precise genome modifications by cleaving specific DNA sequences. Zinc finger nucleases (ZFNs), transcription activator-like effector nucleases (TALENs) and CRISPR/Cas9 system are currently under investigation as potential therapeutics. However, their off-target effects must be controlled. Targeted delivery of nucleases in a form of mRNA seems as the most promising method. Various types of nanoparticles enable mRNA transfer and could be used to facilitate the nuclease application. Some of these nanoparticles together with characterization of the programmed nucleases are described in this thesis.