

Research in the field of gene therapy has potential to become a revolutionary way to the existing treatment for a wide spectrum of neurological diseases. To treat these disorders causally, by specific substituting, deleting, silencing or editing faulty genes could be a privilege of gene therapy. The concept of translational medicine is to facilitate the transfer of working principles in preclinical research into treatment in humans. Its key issue is to overcome limitations associated with the gap between the tremendous variety molecular biology tools of preclinical research and the lack of simple corresponding options in humans. Clinical implementation of most of the preclinical approaches is still considered to be limited. The main focus of this thesis is to summarize latest advancements of molecular and genetic engineering tools that themselves or in combination have the potential to promote most preclinical gene therapy of neurological diseases to clinical use. Based on that, this study aims to suggest perspective methods of treatment for selected neurological diseases.