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New approaches to the treatment of lysosomal storage diseases

Qinwen Mao

Northwestern University Feinberg School of Medicine, USA

Abstract

Many lysosomal storage diseases (LSDs) have devastating consequences. Although enzyme replacement therapy is a potential treatment option, this approach needs repeated injection for the lifetime of the patients. The gene-addition strategy using viral vectors has the potential for long-term expression of the therapeutic protein, however, serious adverse effects, e.g., immune response to in vivo administered viral vectors, and insertional activation of proto-oncogenes, can occur. An alternative to gene addition that can minimize the oncogenic risk of gene therapy is targeted gene correction via homologous recombination. Zinc-finger nucleases (ZFNs) show promise as reagents that can mediate high-frequency homologous recombination in the presence of a donor. This technique has shown success in *ex vivo* correction of a disease-causing mutation by using induced pluripotent stem cells, which can be useful for the treatment of diseases affecting cells that can be removed and returned to the patient. However, many LSDs might need in vivo gene correction in affected organs, which requires the efficient introduction of gene-targeting components (i.e., ZFNs and donor fragment) *in vivo*. We utilized adenovirus, a high-efficiency and safe vector with sufficient size to fit both ZFN and donor to mediate gene correction in vivo. We have overcome the challenges of packing both ZFN and donor in a single adenovirus and producing the ZFN expressing adenovirus with high titer. We also showed that this novel system efficiently mediates targeted genome editing. In summary, adenovirus can be a promising vector for *in vivo* gene correction in disease-affected organs in lysosomal storage diseases.

Biography

Mao received her Ph.D. in Physiology and M.D. from the Fourth Military Medical University in China in 1997. She obtained her postgraduate training at the University of Iowa, followed by combined Anatomic Pathology and Neuropathology training at UT Southwestern Medical Center. She is currently an Assistant Professor at the Department of Pathology, Northwestern University Feinberg School of Medicine. Her research focuses on gene therapy for genetic disorders, and viral therapy for brain tumors. She has published over 40 papers in reputed journals.