Genes and Cells 2014 vol.9 N4, pages 6-33

Gene- and cell-based therapy of muscle system hereditary disorders: State-of-art

Deev R., Mavlikeev M., Bozo I., Pulin A., Eremin I.
Kazan Federal University, 420008, Kremlevskaya 18, Kazan, Russia

Abstract

Genetic disorders primarily affecting skeletal muscles can be caused by dysfunction of more than 30 genes. To date there is no effective etiotropic and pathogenetic treatment of such disorders. Investigators focus on search for new therapeutic agents based on gene and cell technologies, small molecules as well. There are numerous preclinical and several dozens of clinical studies in the world. Unfortunately tested technologies did not lead to significant advance in treatment of patients with such disorders. At the same time resulting data allow to determine the most feasible directions of future development combining of genome correction methods with cell delivery of corrected genome to skeletal muscles. This review is intended to give general information about etiology of skeletal muscles genetic disorders, the main directions of biotechnological development and results of the clinical studies.

Keywords

Cell therapy, Clinical trials, Duchenne muscular dystrophy, Gene therapy, Muscular dystrophy