Abstract
Extracellular matrix (ECM) myopathies and muscular dystrophies are a group of genetic diseases caused by mutations in genes encoding proteins that provide critical links between muscle cells and the extracellular matrix. These include structural proteins of the ECM, muscle cell receptors, enzymes, and intracellular proteins. Loss of adhesion within the myomatrix results in progressive muscle weakness. For many ECM muscular dystrophies, symptoms can occur any time after birth and often result in reduced life expectancy. There are no cures for the ECM-related muscular dystrophies and treatment options are limited to palliative care. Several therapeutic approaches have been explored to treat muscular dystrophies including gene therapy, gene editing, exon skipping, embryonic, and adult stem cell therapy, targeting genetic modifiers, modulating inflammatory responses, or preventing muscle degeneration. Recently, protein therapies that replace components of the defective myomatrix or enhance muscle and/or extracellular matrix integrity and function have been explored. Preclinical studies for many of these biologics have been promising in animal models of these muscle diseases. This review aims to summarize the ECM muscular dystrophies for which protein therapies are being developed and discuss the exciting potential and possible limitations of this approach for treating this family of devastating genetic muscle diseases. © 2017 American Physiological Society. Compr Physiol 7:1519-1536, 2017.

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