

**HEB**

Journal of Hospital Pharmacy
An Official Publication of Bureau for Health & Education Status Upliftment
(Constitutionally Entitled As Health-Education, Bureau)

JOHP

An Overview on Inherited Incurable Disease: Duchenne Muscular Dystrophy

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ABSTRACT:

Duchenne muscular dystrophy (DMD) is not only one of the most severe forms of inherited muscular dystrophies but also the most common hereditary neuromuscular disease. Sadly, there is no known treatment modality that halts the progression of the disease. The available treatment options are palliative. Affected patients usually die in their twenties due to respiratory muscle weakness or cardiomyopathy. Current therapy is centered on treatment with glucocorticoids and physiotherapy to prevent orthopedic complications. The advent of molecular genetic therapies and advanced medicine has opened up new avenues and raised hopes that one day a cure for this debilitating orphan disease. The main purpose of this short review is to enable pediatricians to have informed discussions with the care takers with DMD about recent scientific advances affecting their child's clinical care.

Keywords: Duchenne muscular dystrophy (DMD), dystrophin, gene therapy, muscles dysfunction.