

DMD

DMD is a devastating disease that affects both those with DMD and family members/ caregivers



Presentation

DMD is a rare, X-linked, recessive, progressive disorder that affects 1 in 3,500 to 1 in 5,000 newborn boys^{1–3}

It is a multisystem disease with progressive muscle weakness followed by LoA and death due to cardiorespiratory failure^{4,5}



Burden of Illness

DMD leads to loss of ambulation and wheelchair reliance, which can negatively impact quality of life for individuals with DMD and their caregivers, and pose a substantial economic burden^{6–11}



Diagnosis

For the past 3 decades, a 2.2-year time interval persists between first signs and symptoms of DMD and diagnostic confirmation, with an average age of 4.9 years at diagnosis among males without a family history of DMD¹²



Treatment

Current treatments for DMD aim to maintain ambulation, cardiac, and respiratory function, anticipate and manage associated disease complications, and preserve quality of life^{13–18}

Some approved and investigational therapies aim to restore dystrophin production – even low dystrophin expression has been associated with delayed clinical milestones, compared to no dystrophin expression^{19,20}

DMD, Duchenne muscular dystrophy; LoA, loss of ambulation.

1. Crisafulli S, et al. *Orphanet J Rare Dis*. 2020;15:141; 2. Emery AEH. *Neuromuscul Dis*. 1991;1:19–29; 3. Birnkrant DJ, et al. *Lancet Neurol*. 2018;17:251–267; 4. Sayed MM, et al. *Middle East Curr Psychiatry*. 2022;29:76; 5. Lionarons JM, et al. *Life*. 2021;11:772; 6. Humbertclaude V, et al. *Eur J Paediatr Neurol*. 2012;16:149–160; 7. Davis SE, et al. *J Clin Neuromuscul Dis*. 2010;11:97–109; 8. Schwartz CE, et al. *J Patient Rep Outcomes*. 2021;5:124; 9. Powell PA, Carolton J. *Qual Life Res*. 2023;32:225–236; 10. Landfeldt E, et al. *Neurology*. 2014;83:529–536; 11. Magliano L, et al. *Acta Myol*. 2014;33:136–143; 12. Thomas S, et al. *Muscle Nerve*. 2022;66:193–197; 13. Biggar WD, et al. *Neuromuscul Dis*. 2006;16:249–255; 14. Birnkrant DJ, et al. *Lancet Neurol*. 2018;17:347–361; 15. Schram G, et al. *J Am Coll Cardiol*. 2013;61:948–954; 16. Servais L, et al. *Nucleic Acids Ther*. 2022;32:29–39; 17. Clemens PR, et al. *JAMA Neurol*. 2020;77:982–991; 18. Mitelman O, et al. *J Neuromuscul Dis*. 2022;9:39–52; 19. Yao S, et al. *Front Cell Dev Biol*. 2021;9:689533; 20. de Feraudy Y, et al. *Ann Neurol*. 2021;89:280–292.