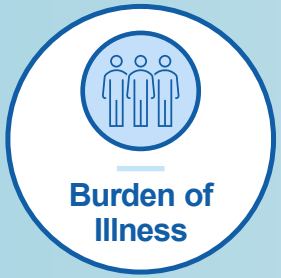
DMD

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 DMD[12]

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 Cin 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.