

Disease

Dystrophinopathies / Duchenne muscular dystrophy^{1, 2}

Gene

DMD

Inheritance	XL
Current Treatments	Eteplirsen, Casimersen and Golodirsen for exon skipping 51, 45 and 53, respectively. Vitolarsen has also been approved for exon 53 skipping. Translarna approved for nonsense variants.
Evidence for Treatment	guideline ³
Non-Molecular Testing Availability	yes
Non-Molecular Test	serum creatine kinase (CK) level ⁴
Experimental / Investigational Therapies	https://clinicaltrials.gov/ct2/results?cond=dmd&term=&cntry=&state=&city=&dist=

References

- ¹ <https://www.ncbi.nlm.nih.gov/books/NBK1119/>
- ² <https://www.ncbi.nlm.nih.gov/pubmed/30856119>
- ³ <https://pubmed.ncbi.nlm.nih.gov/33075081/>
- ⁴ <https://pubmed.ncbi.nlm.nih.gov/32591061/>
- ⁵ <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4671860/>