

THE 6-MINUTE WALK TEST AND OTHER CLINICAL ENDPOINTS IN DUCHENNE MUSCULAR DYSTROPHY: RELIABILITY, CONCURRENT VALIDITY, AND MINIMAL CLINICALLY IMPORTANT DIFFERENCES FROM A MULTICENTER STUDY

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ABSTRACT: Introduction: An international clinical trial enrolled 174 ambulatory males ≥ 5 years old with nonsense mutation Duchenne muscular dystrophy (nmDMD). Pretreatment data provide insight into reliability, concurrent validity, and minimal clinically important differences (MCIDs) of the 6-minute walk test (6MWT) and other endpoints. Methods: Screening and baseline

evaluations included the 6-minute walk distance (6MWD), timed function tests (TFTs), quantitative strength by myometry, the PedsQL heart rate–determined energy expenditure index, and other exploratory endpoints. Results: The 6MWT proved feasible and reliable in a multicenter context. Concurrent validity with other endpoints was excellent. The MCID for 6MWD was 28.5 and 31.7 meters based on 2 statistical distribution methods. Conclusions: The ratio of MCID to baseline mean is lower for 6MWD than for other endpoints. The 6MWD is an optimal primary endpoint for Duchenne muscular dystrophy (DMD) clinical trials that are focused therapeutically on preservation of ambulation and slowing of disease progression.

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Abbreviations: 6MWD, 6-minute walk distance; 6MWT, 6-minute walk test; ACP, American Thoracic Society; CNGR, Cooperative International Neuromuscular Research Group; CK, creatine kinase; DMD, Duchenne muscular dystrophy; EEI, energy expenditure index; HRQL, health-related quality of life; MCID, minimal clinically important difference; MPS, mucopolysaccharidosis; nmDMD, nonsense mutation DMD; NSAA, North Star Ambulatory Assessment; PedsQL, Pediatric Quality of Life Inventory; TFT, timed function test

Key words: 6-minute walk test; ambulation; Duchenne muscular dystrophy; energy expenditure index; muscular dystrophy; myometry; natural history; PedsQL; timed function test

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Additional Supporting Information may be found in the online version of this article.

*Collaborating authors are listed in Appendix 1. Other members of the PTC124-GD-007-DMD Study Group are listed in Appendix 2 in the Supporting Information.

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Reliability, Validity and MCID of 6MWT and other Endpoints in DMD

Duchenne muscular dystrophy (DMD) is a disabling and life-threatening X-linked genetic disorder caused by defects in the gene for dystrophin, a protein that stabilizes muscle cell membranes.¹ DMD is characterized by complete loss of dystrophin and is the most common neuromuscular disease of childhood. It affects 1 in 3800–6300 males, and there are an estimated 15,000 patients with the disease in the USA.^{2,3} There is no approved therapy that addresses the underlying cause of DMD.^{4,5} In $\sim 13\%$ of boys with nonsense mutation DMD (nmDMD), which represents ~ 1700 boys in the USA and ~ 2400 boys in Europe), the causative defect in the dystrophin gene is a nonsense mutation that truncates dystrophin protein production by introducing a premature stop codon into dystrophin mRNA.^{6–8}

THE NEED FOR CLINICALLY MEANINGFUL ENDPOINTS IN DMD

Given that several novel approaches to treatment of DMD have shown promise in preclinical and/or proof-of-concept clinical studies,^{9–12} the research community has faced the need to identify and develop clinically meaningful outcome measures for use in pivotal therapeutic trials. In boys with DMD, walking abnormalities are a major disease manifestation that has great importance to patients and

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