Trabalhos Científicos

Título: Disease Burden And Treatment Landscape In Duchenne Muscular Dystrophy (Dmd) In The United States

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Resumo: OBJECTIVES: To portray disease burden and treatment landscape in the United States (US) associated with DMD, a rare fatal genetic disorder which causes progressive functional decline. METHODS: A targeted review of data from published studies (2010-2017), recent scientific conferences, and DMD product package inserts was undertaken, along with a new analysis of the placebo-arm of the Ataluren Confirmatory Trial in DMD (ACT DMD) which evaluated changes in 6-minute walk distance (6MWD), timed function tests (TFTs), North Star Ambulatory Assessment, and parent-reported quality-of-life from baseline to week 48. Mixed-model repeated measures analysis comparing deflazacort (n=53) and prednisone/prednisolone (n=62) in ACT DMD was undertaken; delay in loss of ambulation (LOA) was modelled. RESULTS: DMD disease burden includes loss of muscle strength, diminished function and quality of life, LOA, progressive cardiomyopathy and respiratory dysfunction affecting survival (mean~19yrs without treatment). Treatment with deflazacort, indicated for >5yrs old, improved muscle strength (vs. placebo and/or prednisone) and delayed LOA; safety profile of deflazacort was comparable to prednisone. New analysis of ACT DMD placebo arm data demonstrated mean differences from baseline to Wk-48 comparing deflazacort vs. prednisone/prednisolone in 6MWD (31.6 meters; p=0.0484) and key TFTs (4-stair climb (-2.9sec; p=0.0189), 4-stair descend (-1.8sec; p=0.2040), supine-to-stand (-2.6sec; p=0.0506)). A mathematical model used in ACT DMD analyses, which projects the impact of prolongation of ambulation (as measured by 6MWD) to age at LOA, showed an incremental delay in LOA of 3.8yrs among patients on deflazacort (vs. prednisone/prednisolone). CONCLUSIONS: A few treatment options are now available to manage DMD and alleviate disease burden.