DEFLAZACORT OR PREDNISONE TREATMENT FOR DUCHENNE MUSCULAR DYSTROPHY (DMD): REAL-WORLD OUTCOMES AT CINCINNATI CHILDREN'S HOSPITAL MEDICAL CENTER (CCHMC)

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Corticosteroids are the standard of care for DMD patients. This study assessed real-world data between deflazacort (DFZ) or prednisone (PRED) steroid treatment, and ambulatory, pulmonary, cardiac, growth and bone-health outcomes. Between 225 and 414 boys at CCHMC were included in the analysis for each outcome. About 75% received DFZ (~95% daily regimen), 13% PRED (~75% daily), and 12% switched from PRED to DFZ. For the subset of switchers with duration data available (28%), average duration on PRED was 3.1 years and average subsequent duration on DFZ was 2.5 years. On average, after accounting for age and steroid duration in a linear regression model, patients on DFZ compared with those on PRED had: 0.59 stairs/second greater 4-stair climb velocity, 4.6 points higher North Star Ambulatory Assessment total score and 9.9% higher forced vital capacity %-predicted ($P<0.05$ for each). Total body mass was lower by 6.9kg and height was lower by 6.2cm; % lean body mass was higher by 4.4%. No significant difference between treatment groups was observed in whole body bone mineral density or in left ventricular ejection fraction. In a Kaplan-Meier analysis, by age 15 (age 20), 56.4% (85.1%) of PRED-initiated patients used a wheelchair as their primary means of mobility, compared to 43.7% (78.7%) of DFZ-initiated patients ($P<0.01$). By age 15 (20), 13.7% (64.8%) of PRED-initiated patients had scoliosis compared to 8.9% (33.7%) of DFZ-initiated patients ($P=0.05$). These differences persisted in Cox proportional hazards models adjusting for age at steroid initiation. In sensitivity analyses, risk of scoliosis and total wheelchair use among PRED-to-DFZ switchers was numerically lower compared to patients consistently on PRED but higher compared to those consistently on DFZ. This study adds to the evidence associating DFZ use with greater functional preservation relative to PRED, and further indicates concurrent preservation of lean body mass and delay of scoliosis.

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