The Effect of Two Different Corticosteroids Regimens on Clinical Outcome in Patients With Duchenne Muscular Dystrophy in Fayoum University Hospitals

Thesis

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ABSTRACT

Background: Duchenne Muscular Dystrophy (DMD) is X-linked recessive disorder that affects 1 in 3500-5000 male births. The disease manifests as progressive muscle weakness, affecting both skeletal and cardiac muscles. Despite advancements in research, corticosteroids remain the only proven therapy to delay disease progression.

<u>Objectives:</u> this study aims to detect the effect of two different corticosteroids regimens on clinical outcome in patients with Duchenne Muscular Dystrophy in Fayoum University Hospitals.

<u>Methods:</u> Analytic, cohort study of boys with Duchenne Muscular dystrophy presented to Neurology Clinic in Fayoum University Hospitals receiving prednisone of different regimens (weekend & every other day regimens). During a period of eight months between May 2024 and December 2024. Patient drug selection was based on Closed envelope method.

Results:

✓ The Every -Other- Day (EOD) regimen showed superior outcomes in maintaining motor function, particularly in standing, transfers, and proximal motor capabilities

(D1 and D2 dimensions of the Motor Functional Measures; MFM scale) with $\, p \,$, values $\, 0.002 \,$ and $\, 0.02 \,$ respectively .

✓ The EOD regimen demonstrated better preservation of cardiac function, as evidenced by lower left ventricular end-systolic diameter (LVESDD) and higher fractional shortening (FS%) compared to the weekend regimen with **P-values 0.01** and **0.003** respectively.

<u>Conclusions:</u> Both regimens improved motor function, but the EOD regimen demonstrated better outcomes in preserving standing and proximal motor abilities, Cardiac and safety profiles were comparable, EOD regimen is more safe.

Keywords: DMD, Corticosteroids, MLPA, Echo, MFM scale.