

THE IMPACT OF BISPHOSPHONATES ON SURVIVAL IN DUCHENNE MUSCULAR DYSTROPHY

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Objective: To describe the effect of bisphosphonate therapy on survival within a regional cohort of patients with Duchenne muscular dystrophy (DMD) receiving steroid therapy

Patients and Methods: The records of all patients with DMD who were born from 1963 to 2006 and who had received at least one year of steroid therapy were reviewed from birth until they reached the study endpoints: death; loss to follow-up; or the last follow-up in 2009. A survival analysis was used to account for the variable follow-up duration within this cohort.

Findings: Forty-four boys with DMD from this cohort were exposed to continuous steroid use. Bisphosphonate therapy had been initiated for 16 patients (36%) between 1997 and 2007, at a median age of 12.5 years (range: 7-23 years). By the last follow-up in 2009, 13 patients had died (30%), at a median age of 16 years and range 14 to 27 years. Survival curves demonstrate that prescription of bisphosphonates was associated with a significant improvement in survival ($p=0.005$ log-rank test). Furthermore, a possible therapy duration effect could be shown for bisphosphonate use ($p=0.007$, log-rank test)

Conclusion: The combination treatment with steroids and bisphosphonates in patients with Duchenne muscular dystrophy appears to be associated with significantly improved survival compared to steroids alone.