**Treating heart failure in Duchenne muscular dystrophy: is proactive better than reactive?**

**Authors:**

Cassandra B. Moylan, BS, Colton G. Brown, BS, Jennifer R. Maldonado, BS, RTR, R. Erik Edens, MD, PhD, Katherine D. Mathews, MD, Ravi C. Ashwath, MD

**Abstract:**

**Background:** Duchenne muscular dystrophy (DMD) is an x-linked childhood muscular dystrophy. Cardiomyopathy is a major cause of morbidity and mortality. Mean age of heart failure (HF) onset in DMD is 14.3 years(1). HF medications (HFM) are often initiated at HF onset. However, evidence for a cardioprotective effect of HFM has led to the recent recommendation for initiation before onset of HF.

**Objective:** To determine if prophylactic initiation of HFM delays onset of HF in DMD.

**Methods:** Retrospective review of DMD patients allowed classification into prophylaxis group (PG) or treatment group (TG) based on whether HFM was initiated before or after evidence of HF, defined by ejection fraction (EF) <55% or fractional shortening (FS) <28%. Age at start of HFM, specific HFM, EF and/or FS at or before start of HFM, and age at HF onset were collected. Mean values were compared using two-sample Mann-Whitney U or Z-test.

**Results:** 104 subjects (77 PG and 27 TG subjects) were included. Mean age in years at start of HFM in the TG was 17.8 (95% CI, 15.9-19.8) and 10.8 (95% CI, 10.0-11.7) in the PG (p < .0001). Forty-seven (61%) PG subjects are alive without HF; mean age at last visit was 13.5 (95% CI, 11.9-15.1). PG subjects (n=23) developed HF at 18.1 (95% CI, 16.0-20.2) years, TG (n=27) at 17.8 (95% CI, 15.9-19.7) years. HF onset was later than reported by Barber et al. (P < .001), but there was no difference in HF onset between TG vs. PG (Table 1).

**Conclusions:** In our cohort, prophylactic initiation of HFM in DMD did not alter age at HF onset, however most of our PG subjects are currently alive with normal EF and are younger than the mean age at HF onset. Continued follow up of this population will clarify if cardiac prophylaxis delays HF.

**References:**

1. Barber, B.J., et al., *Oral Corticosteroids and Onset of Cardiomyopathy in Duchenne Muscular Dystrophy.* The Journal of Pediatrics, 2013. **163**(4): p. 1080-1084.e1.

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| --- | --- | --- | --- | --- | --- | --- | --- |
| **Group** | **Number of subjects** | **Mean age at start of HFM** | **95% CI (+/-)** | **Mean age at HF onset** | **Mean age at last visit** | **Mean age at death** | **95% CI (+/-)** |
| Barber et al. | 202 | --- |  | 14.3 | --- | --- | 0.579 |
| TG | 27 | 17.8 | 1.934 | 17.8 | --- | --- | 1.934 |
| PG | 77 | 10.8 | 0.856 | --- | --- | --- | --- |
| **Subgroup of PG** |  |  |  |  |  |  |  |
| PG with HF | 23 | 13.1 | 1.26 | 18.1 | --- | --- | 2.078 |
| PG without HF | 47 | 9.2 | 0.947 | --- | 13.5 | --- | 1.628 |
| PG deceased prior to HF | 7 | 14.1 | 2.808 | --- | --- | 19.9 | 2.206 |