Dilated Cardiomyopathy: An Analysis of Therapy Offered to Children Enrolled in the Pediatric Cardiomyopathy Registry (PCMR)

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Supported by the National Heart, Lung, & Blood Institute grant 5RO1HL53392

Background:
- The NIHBI funded Pediatric Cardiomyopathy Registry has enrolled > 2700 patients 10 years of age or younger with selected cardiomyopathies since 1996.
- Prospective PCMR data has determined the annual incidence of pediatric cardiomyopathy in the United States to be 1.13 per 100,000 children (SEL et al., NEJM 2003; 348:167-55).
- Current medical therapy for pediatric dilated cardiomyopathy (DCM) is inadequate. Progressive L.V. failure often occurs, with approximately 40% of symptomatic children either dying or undergoing cardiac transplantation within 2 years following their diagnosis. There has been little or no improvement in transplant-free survival over the past 3 decades.
- Evidence-Based guidelines are available to guide heart failure management in the adult. No such guidelines exist for the pediatric practitioner.
- This study examines the early (30 days surrounding diagnosis) medical and surgical therapy applied to the retrospective component of the registry which enrolled patients diagnosed with DCM between 1990-1995. Detailed medication data was not collected on patients diagnosed 1996 to present.
- Findings are compared to adult treatment standards.

Results:
- Anticongestive therapy (defined as the use of digoxin and/or diuretics) was the mainstay of treatment in this large, recent historical cohort of children with DCM.
- ACE inhibition was widely, but not universally, prescribed. ACE inhibitor use was reported in patients with relatively advanced HF (SF Z-score = 9.2 ± 3.09).
- Minimal (4%) ß-blocker use was reported, with no increasing trend identified over the study era.
- Antithrombotic therapy was prescribed relatively frequently (18.9%).
- Intravenous inotropes were administered to 1 out of 6 children (15.6 %) surrounding their diagnosis of DCM.
- 5.3% of children received mechanical cardiorespiratory support. Cardiac transplants were performed early in 4.1%.

Conclusions:
- Adult derived evidence-based guidelines for HF treatment were not widely applied to this cohort of pediatric heart failure patients.
- These data support the need for pediatric-specific heart failure investigations.