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

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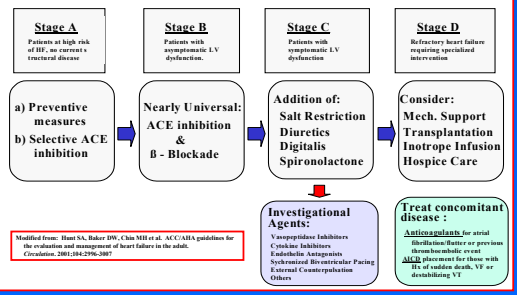
Background:

- The NHBLI funded Pediatric Cardiomyopathy Registry has enrolled > 2700 patients 18 years of age or younger with selected cardiomyopathies since 1996.
- Prospective PCMR data has determined the annual incidence of pediatric cardiomyopathy in the United Stages to be 1.13 per 100,000 children (SEL et al., NEJM 2003; 348:1647-55).
- Current medical therapy for pediatric dilated cardiomyopathy (DCM) is inadequate. Progressive LV 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.

ACC/AHA Practice Guidelines for Chronic Heart Failure Treatment in the Adult



Modified from: Hunt SA, Baker DW, Chin MH et al. ACC/AHA guidelines for the evaluation and management of heart failure in the adult. *Circulation*. 2001;104:2996-3007

Demographics of 493 Children with Dilated Cardiomyopathy Diagnosed 1990-1995

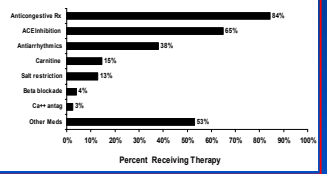
Male	270 (54.8 %)
Age at Dx (yr)	5.5 ± 6.0 Median: 1.9
< 1 year	182 (36.9%)
1 to < 6 year	127 (25.8%)
6 to < 12 years	70 (14.2%)
12 to 18 years	114 (23.1%)
Race	
White	300 (60.9%)
Black	105 (21.3%)
Hispanic	56 (11.4%)
Other	10 (2.0%)

Clinical Status at Diagnosis of 493 Children with Dilated Cardiomyopathy

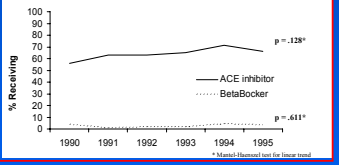
Congestive Heart Failure:	360 (73.0%)	
Functional Class (Combined NYHA and Canadian Consensus Classification for Infants) :		
Class I (includes asymptomatic pts.)	141 (28.6%)	
Class II	63 (12.8%)	
Class III	122 (24.7%)	
Class IV	159 (32.3%)	
Etiology:		
Myocarditis	89 (17.6%)	
Neuromuscular Disease	44 (8.9%)	
Familial Isolated CM	25 (5.1%)	
Inborn Error of Metabolism	10 (2.0%)	
Malformation Syndrome	2 (0.4%)	
Idiopathic	325 (65.9%)	
Echocardiographic Function:	n	BSA-adjusted Z-score*
ESD	313	6.03 ± 2.81
EDD	363	4.20 ± 2.63
Fractional Shortening	400	-8.60 ± 3.57
Posterior Wall Thickness	286	-0.38 ± 2.44
Septal Wall Thickness	256	-0.76 ± 1.95

* represents the number of standard deviations from the mean of healthy children of similar BSA

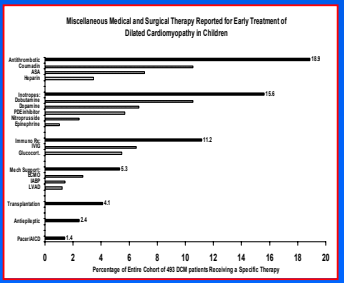
Early Medical Therapy Offered to 493 Children with Dilated Cardiomyopathy Diagnosed 1990 - 1995



Use of Beta Blockade and ACE Inhibition (During Month of Diagnosis) for Children with Dilated Cardiomyopathy 1990-1995



Miscellaneous Medical and Surgical Therapy Reported for Early Treatment of Dilated Cardiomyopathy in Children



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 cardiopulmonary 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.