Session SESSION 27 - New Tools for the Kids: Diagnosis of Heart Transplant Rejection and Registries for Heart Function in Pediatrics

51. Taking ACTION. Creation of a Prospective Registry of Boys with Dystrophinopathy and Ventricular Dysfunction to Define Cardiac Medication Use and Optimize Guideline Directed Medical Therapy

April 19, 2023, 4:15 PM - 4:25 PM | Rooms 405-407

Topic:
HEART-Pediatrics-Heart Failure

Presenter

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Disclosures

C.A.Wittlieb-weber: None. **A.Lorts:** n/a. **H.Martinez:** n/a. **D.Mokshagundam:** Other; ; CareDx. **D.Nandi:** Consulting/Advisory Fee; ; CareDx. **J.Parent:** None. **F.Raucci:** n/a. **N.Soares:** n/a. **M.Shezad:** n/a. **R.Shih:** None. **S.Shugh:** n/a. **B.Birnbaum:** n/a. **C.Villa:** n/a. **S.Wilkens:** n/a. **B.L.Wisotzkey:** n/a. **J.Conway:** Other; ; Abott, Medical monitor for the Pumpkin Trial. **C.Castleberry:** n/a. **P.Esteso:** None. **K.Gambetta:** n/a. **E.Hayes:** n/a. **D.Hsu:** n/a. **B.Kaufman:** None. **A.Lal:** n/a.

Abstract or Presentation Description

Purpose This project seeks to define cardiac medication use for boys with Duchenne and Becker Muscular Dystrophy (DBMD) and ventricular dysfunction to optimize the use of guideline directed medical therapy (GDMT).
Methods DBMD subjects with a history of left ventricular ejection fraction (EF) $\le$ 45% were enrolled in an Advanced Cardiac Therapies Improving Outcomes Network (ACTION) database. Cardiac medication use in the presence of dysfunction was analyzed at enrollment. GDMT was defined as current use of angiotensin-converting-enzyme inhibitor/angiotensin II receptor blocker /angiotensin receptor-neprilysin inhibitor plus beta-blocker plus mineralocorticoid receptor antagonist.
Results 128 boys with DBMD from 20 centers in North America were enrolled;

median age of 18.9 [IQR 16.8-21.9] years with 29 (22.7%) ambulatory, 61 (47.7%) using respiratory support, and 59 (46.1%) on steroids. The median EF recorded (N=107, 84%) at enrollment was 41.8% [IQR 34%-49.5%]. Cardiac medication use is outlined in Table 1. Frequency of use of GDMT by EF is shown in Figure 1. GDMT was being used for 79 (43.4%) subjects at the time of enrollment; 8/15 (53.3%) of those with severe ventricular dysfunction (EF <30%) were on GDMT.

Conclusion Initial analysis of a prospective registry of boys with DBMD and ventricular dysfunction shows that the majority are not receiving GDMT. More work is needed to better understand barriers to optimization of GDMT particularly given the extension of life for DMD patients and increasing frequency of cardiac causes of death.

Table 1. Cardiac medication use among males with Duchenne and Becker Muscular Dystrophy and ventricular dysfunction.

Medication use at enrollment	N = (%)
Currently on ACE-I	83 (64.8%)
Most commonly used ACE-I	Lisinopril 68 (81.9%)*
Median dose of most commonly used ACE-I	33 mg [IQR 27.5 mg-36 mg]
Currently on ARB	13 (10.2%)
Most commonly used ARB	Losartan 13 (100%)*
Median dose of most commonly used ARB	25 mg [IQR 25 mg-62.5 mg]
Currently on ARNI	22 (17.2%)
Currently on BB	96 (75%)
Most commonly used BB	Carvedilol 49 (51.0%)*
Median dose of most commonly used BB	25 mg [IQR 12.5 mg-42.5 mg
Currently on MRA	96 (75%)
Most commonly used MRA	Spironolactone 66 (68.8%)*
Median dose of most commonly used MRA	25 mg [Range 12.5 mg-50 m
Currently on non-potassium sparing diuretic	9 (7%)
Most commonly used non-potassium sparing diuretic	Furosemide 8 (88.9%)
Median dose of most commonly used non-potassium sparing diuretic	40 mg [IQR 40 mg- 50 mg]
Currently on Digoxin	5 (3.9%)
Currently on SGLT2-inhibitor	2 (1.6%)
Currently on Ivabradine	1 (0.8%)

ACE-I= angiotensin-converting-enzyme inhibitor; ARB= angiotensin II receptor blocker; ARNI= angiotensin receptor-neprilysin inhibitor, BB = Beta-blocker; MRA= mineralocorticoid antagonist; *= % of subjects taking that class of medication

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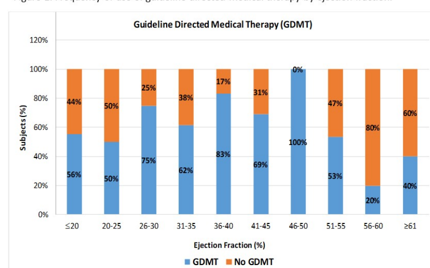
Figure 1. Frequency of use of guideline directed medical therapy by ejection fraction.



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