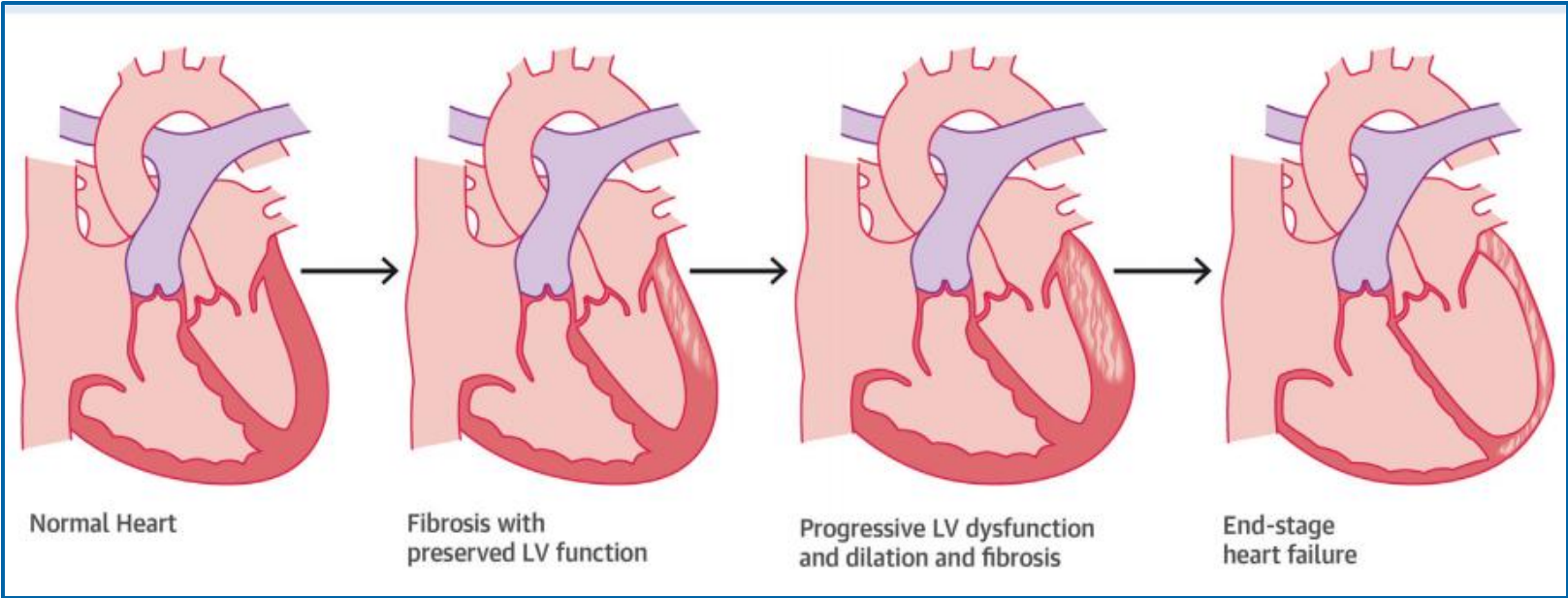
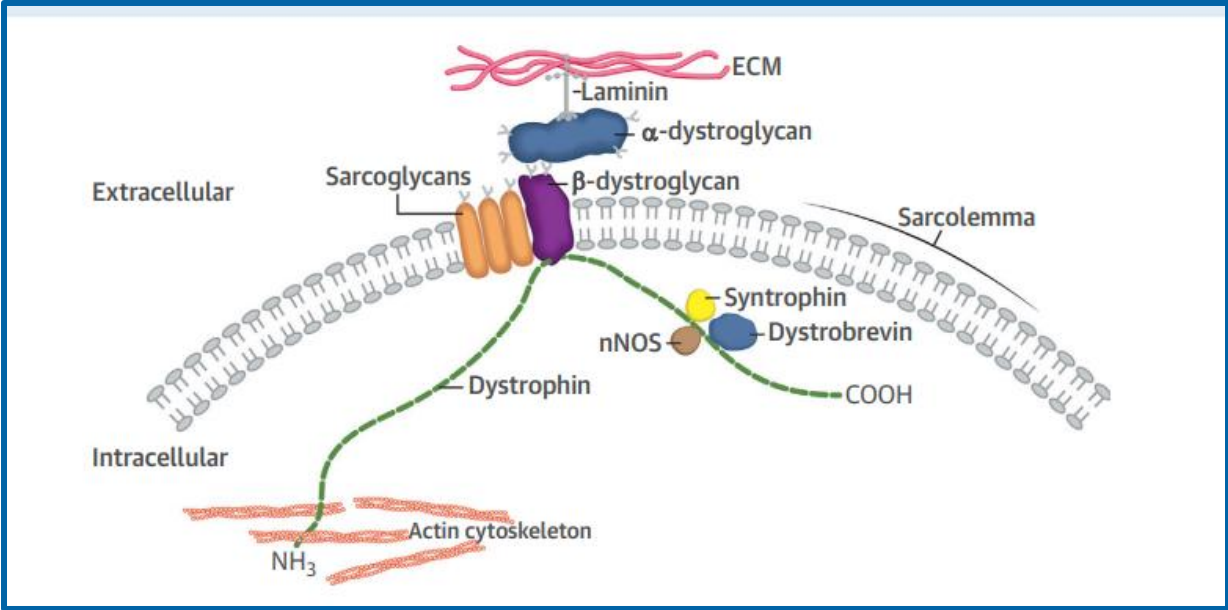


Cardiac Care in Children with Duchenne Muscular Dystrophy

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Background



¹ Kamdar F, Garry DJ. Dystrophin-Deficient Cardiomyopathy. JACC, Vol. 67, No. 21, p 2533-2546, 2016.

Cardiac Care in MD

- Boys with DMD will develop cardiac changes.
 - Dilated cardiomyopathy: Enlargement of heart chambers and decrease in cardiac function/squeeze
 - Variable progression for each child but majority by 18 years of age.
- Early detection is key
 - Echocardiography yearly until changes seen and then every 6 months.
 - Cardiac MRI offers better imaging and can see fibrosis but can be difficult to lay in the scanner.
 - Periodic lab tests, ECG and holter monitoring.



Therapy Options

- Start medications to hopefully delay or slow down changes in the heart.
 - Generally around 10 years of age, even if cardiac function normal.
 - Angiotensin-converting enzyme inhibitor or angiotensin receptor blocker, mineralocorticoid antagonist, beta-adrenergic receptor blockers, diuretics
 - At this time, we are unable to prevent changes from occurring but with ongoing clinical trials and research we can change the natural course.
- Indications for advanced therapies, such as an ICD or VAD, is reserved for those with advanced heart failure and risk/benefits should be review with your cardiologist.



Additional Considerations

- Establish healthy eating habits early in life.
- Your heart and lungs interact so be mindful of both.
- Screening of female gene carries.
 - Can find cardiac changes in women as well, generally less severe and later in life.
 - Should have cardiac screening, including echocardiograms.
 - Sometimes adult cardiologists are not aware of risk so referring them to your child's pediatric cardiologist to discuss can help.



PPMD and ACTION

- Collaboration between PPMD and ACTION to guide and standardize cardiac care and improve outcomes for patients.
- Database Expansion, increase institution participation, real-world data and clinical trials for treatment and more.

Thank you!

- Questions?

