Common Medicines Dystrophinopathies (Duchenne, Becker, & Carriers)

Duchenne Muscular Dystrophy (DMD) and Becker Muscular Dystrophy (BMD) patients have an increased risk of heart dysfunction and scarring of heart muscle. When heart function decreases, **neurohormone** levels can increase. Neurohormones are proteins that are released from cells into the blood and can be harmful to the heart, blood vessels, and circulatory system. Increased levels of neurohormones may lead to worsening heart function and development of heart failure symptoms (such as trouble breathing and upset stomach).



The following medicines, **often used in combination,** help by blocking or lowering the effect of the neurohormones on your heart and may prevent more scar from developing and the heart function from continuing to decrease. For DMD patients, starting some of these medicines before the heart muscle develops scaring and/or heart function decreases, we may be able to slow down this process.

Please let your care team know of any changes in how you are feeling while taking these medicines.

ANGIOTENSIN RELATED

- Angiogiotensin Converting Enzyme Inhibitors (ACEi) Medicines that end in "-pril" (ex: captopril, enalapril, lisinopril, perindopril)
- Angiotensin Receptor Blockers (ARBs) Medicines than end in "-artan" (ex: losartan, valsartan)
- Angiotensin Receptor Blocker Neprolysin
 Inhibitor (ARNIs)

This is a combination of sacubitril + valsartan (ex: Entresto®)



What they do: Starting these medicines early has been shown to slow the development of heart failure in DMD and may slow progression of scarring in the heart. These medicines help the blood vessels relax (easier for the heart to pump),

help preserve or potentially improve heart function, and help decrease salt and water in the body.

Possible side effects: Lower blood pressure (light-headedness or dizziness), elevated levels of potassium, a dry cough, swelling of the lips, and change in kidney function.



BETA BLOCKERS

• Medicines that end in "-lol" (ex: metoprolol, carvedilol)

What they do: These medicines help slow the heart rate, which usually runs fast in DMD patients, so the heart doesn't work as hard. They also help the blood vessels relax (easier for the heart to pump), and help preserve or potentially improve heart function. They can also be used to treat arrhythmias, or abnormal heart rhythms.



Possible side effects: Lower blood pressure (light-headedness or dizziness), lower heart rate (light-headedness or dizziness), and feeling tired or drowsy.



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Common Medicines continued...

MINERALOCORTICOID RECEPTOR ANTAGONISTS (MRA's)

• *(ex. spironolactone and eplerenone)*

What they do: These medicines may help slow the scarring in the heart. They are also known as "potassium sparing diuretics", but they do much more than help remove excess water and salt from the body. They also help preserve or potentially improve heart function.

Possible side effects: Elevated levels of potassium.



DIURETICS

• (ex. furosemide – Lasix®, chlorothiazide – Diuril®)

What they do: These medicines help the body make more urine, which gets rid of excess water and salt. It is started when there is decreased heart function

and concern for swelling or fluid overload. This medicine helps improve swelling, and it can help reduce the amount of fluid in the lungs.



Possible side effects: Decreased levels of potassium and sodium, dehydration, as well as other electrolyte changes, and decreased blood pressure (light-headedness or dizziness).

SODIUM-GLUCOSE COTRANSPORTER-2 INHIBITOR (SGLT2I)

• (ex: dapagliflozin, empagliflozin)

What they do: These medicines help the body make more urine in order to get rid of extra sodium and sugar. This medicine is started when there is a moderate or severe decrease in heart function. It can also help patients with high blood sugar (often seen in patients taking steroids). This medicine also helps to protect the kidneys and heart in patients with decreased heart function.



Possible side effects: Low blood sugar (sweating, nausea, confusion), increased acid in the blood, dehydration, urinary tract and kidney infection, and skin infections around the genitals.

Tips For Starting a New Medicine

- Please let your care team know of any changes in how you (or your child) are feeling while taking these medicines, especially after a new medicine is started or a dose of medication is increased.
- For some of these medicines, it is important for blood work to be done after the medicine is started or adjusted. Be sure to follow the instructions given by your care team.



