CARDIAC TRANSPLANT IN DUCHENNE AND BECKER

Parent Project Muscular Dystrophy
JOIN THE FIGHT. END DUCHENNE.

WHY THE HEART MATTERS IN DUCHENNE AND BECKER

Thanks to improvements in breathing care, people with Duchenne and Becker muscular dystrophy are living longer. As a result, heart muscle disease (cardiomyopathy) has become the leading cause of serious illness and death. When the heart becomes very weak, it can lead to advanced heart failure. For some patients, advanced heart treatments may help improve both length of life and quality of life. Although heart transplant in Becker is performed regularly, heart transplantation in people with Duchenne is relatively new.

WHAT ARE ADVANCED HEART TREATMENTS?

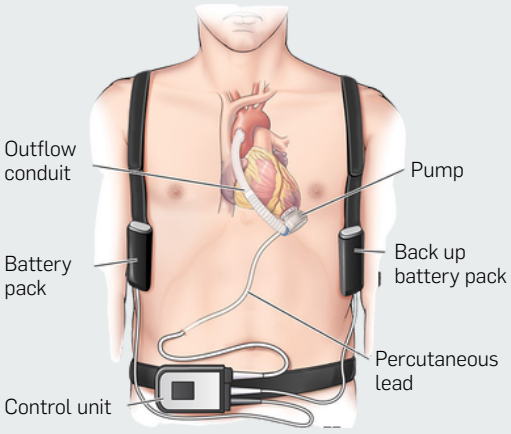
Two treatments are sometimes used when heart failure becomes severe:

VENTRICULAR ASSIST DEVICE (VAD)

A mechanical pump that helps the heart circulate blood throughout the body.

HEART TRANSPLANT

Surgery to replace a failing heart with a healthy donor heart.



While heart transplant in Becker muscular dystrophy is performed regularly, historically, people with Duchenne were often not offered these treatments. However, experts now believe that some individuals with Duchenne may benefit from them. This decision is usually made by a cardiologist outside their neuromuscular care team who specializes in advanced treatments for heart failure. The table below lists the situations in which referral to a heart failure specialist is recommended.

Indications for Referral for Advanced Cardiac Therapy Evaluation

Note: Referral is appropriate if one or more criteria are met; not all indications are required.

- Ejection Fraction <40% (the heart is not pumping as strongly as it should)
- End-organ dysfunction (kidney or liver dysfunction) due to heart failure, usually determined by blood tests
- Persistently elevated NT-proBNP (blood test show ongoing signs that the heart is under stress)
- The body is retaining fluid or needing more medication to get rid of fluid
- Multiple shocks from a cardiac defibrillator to correct the heart rhythm
- Need for hospitalization due to heart failure
- Need to reduce/discontinue heart failure medications due to side effects

Symptoms Suggesting Worsening Heart Failure

- Shortness of breath
- Fatigue
- Rapid weight gain (i.e. 2-3 lbs. in one day)
- Unexplained abdominal pain or weight loss
- New or increased leg swelling
- Persistent cough

THE PPMD TEAM IS HERE TO HELP

Schedule a one-to-one meeting with the PPMD team for personalized support and resources. Visit www.parentprojectmd.org/ppmd-for-you.

Partnering with Your Care Team

If you have worsening heart failure symptoms, it's important to know what questions to ask your medical team and how to advocate for yourself/your child.

- What are the exact signs that mean I should call you immediately?
- What stage or severity is my heart failure now?
- At what point should I go to the ER instead of calling you?
- How much weight gain is too much, and over what time period?
- Do any of my medications need to be adjusted?
- Do I need any additional tests (blood work, echocardiogram, imaging)?
- Should I be monitoring anything at home (weight, blood pressure, oxygen levels)?
- How often should I check in or follow up?
- Should I change my sodium or fluid intake?
- Are there advanced treatments I should start thinking about?
- Do I need to be referred to a different cardiologist with more experience in advanced heart failure?

What Research Is Showing

Studies suggest encouraging outcomes:

- About 84% of Duchenne patients receiving VAD support had positive outcomes after one year.
- Several patients with Duchenne have successfully received a heart transplant
- Results in Duchenne appear similar to other pediatric patients who have received a VAD or transplant.

How Doctors Decide If Someone Is a Candidate

Traditional heart tests alone may not give the full picture of heart failure for people with Duchenne. Doctors now look at several factors when considering advanced heart therapies, including:

- Heart imaging (such as cardiac MRI)
- Blood biomarkers
- Adapted exercise testing
- Patient-reported symptoms
- Breathing function
- Nutrition and overall health

Every patient is considered individually, with input from a multidisciplinary team.

KEY TAKEAWAYS

Advanced heart therapies like VADs and heart transplantation may be possible for some people with Duchenne.

Decisions should be:

- Individualized
- Made by experienced teams
- Discussed early with families

As research and collaboration grow, more patients with Duchenne may gain access to life-extending heart treatments.



References

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