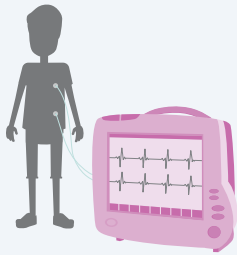


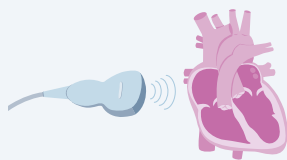
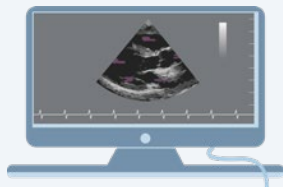
# Gene Therapy for the Heart

## Dystrophinopathies (Duchenne, Becker, & Carriers)



### What is gene therapy and how does it work?

Gene therapy gives the body new instructions to make a smaller version of the dystrophin protein, called microdystrophin. It is given as a one-time treatment through an IV. The therapy uses a special virus, called a vector, to deliver the new gene into the body's cells. This protein helps strengthen muscles. However, there is still limited information about how this treatment affects the heart muscle—whether it helps, causes harm, or has no effect.



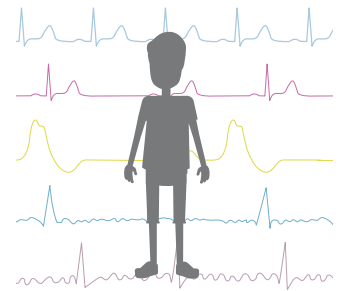
### How is the heart evaluated?

Gene therapy can sometimes cause a small risk of damage to the heart muscle, which is called myocarditis. That's why doctors check how your heart is working before you begin treatment. After gene therapy, doctors use different tests to look for signs of heart injury. The most common ones are blood tests, EKGs, and echocardiograms. A blood test might show a higher level of a protein called troponin, which can mean the heart has been hurt. An echocardiogram is a special kind of ultrasound that shows how well the heart is pumping. If any of these early tests look unusual, other tests like a cardiac MRI or a heart catheterization might be done to learn more about how much damage there is.

## SIGNS & SYMPTOMS OF HEART FAILURE

The symptoms of myocarditis may include:

- stomach/abdominal pain
- low appetite
- nausea/vomiting
- shortness of breath
- coughing
- fever
- feeling tired
- low energy
- chest pain
- palpitations (unusual heartbeat)



Some of these symptoms can happen after gene therapy but may not be related to the heart. That's why your doctors carefully monitor your lab results and other tests after gene therapy—to check for any signs of heart problems or other side effects.

## HEART MONITORING CATEGORIES

Right now, patients are placed into two categories before receiving gene therapy: **standard risk** and **high risk**. These categories help doctors decide how closely each patient's heart should be monitored. Patients who have **heart scarring** (seen on a cardiac MRI), a history of **abnormal heart rhythms**, or **dysfunction** (signs that the heart isn't working well) may need extra monitoring to help keep their heart safe.

See *reverse side* for the heart monitoring schedule based on risk category.

Heart Monitoring Schedule

S = Standard Cardiac Risk (EF≥55%) H = High Cardiac Risk (EF<55%)

	Prior Baseline*	2-3 DAYS	1 WK	2 WK	3 WK	4 WK	2 MOS	3 MOS	6 MOS	9 MOS	1 YR
Cardiology Office Visit	S H						H	H	SH	H	SH
BLOOD LABS											
NT-proBNP	H								H		
Troponin I	S H	SH	SH	SH	SH	SH	H	H	SH	H	SH
IMAGING											
Echocardiogram (Echo)	S H		SH			SH	H	H	SH	H	SH
CMR (Cardiac MRI) <i>un-sedated</i>	H										SH
RHYTHM MONITORING											
Electrocardiogram (ECG)	S H		SH			SH	H	H	SH	H	SH
Ambulatory Rhythm Monitor	H										SH

*\*Standard Cardiac Risk patients need to have baseline testing within 6 months prior to gene therapy starting.  
High Cardiac Risk patients need to have baseline testing within 3 months prior to gene therapy starting.*

My testing before gene therapy was completed on: \_\_\_\_\_

My risk category is: \_\_\_\_\_

I have talked to my team about mechanical support options: ☐ YES ☐ NO

If your heart tests show any changes after gene therapy, your doctors may decide to keep you in the hospital for closer monitoring. If they find that your heart has been injured, they will talk with you about possible treatments. These may include extra steroids, IV medications, or—in rare cases—machines that help your heart work while it heals. Before you receive gene therapy, your medical team should explain these options so you know what to expect if they’re needed.

It’s important to talk openly with your care team about your heart health. If you have any questions or concerns, don’t hesitate to speak up. Your care team is here to support you and make sure you receive the best care possible.