[MUSIC PLAYING]

TIMOTHY J. NELSON: The ability to bioengineer and make new heart muscle, and apply that to patients with weak hearts is not just a hope, but a reality today.

JOSEPH A. DEARANI: Regenerative medicine is the next great realistic hope for patients with heart disease of all ages, particularly children.

TIMOTHY J. NELSON: We're doing that today. We're putting stem cells into the hearts of infants with congenital heart disease with the goal of making the hearts bigger, better, and stronger. We use stem cells from bone marrow or umbilical cord blood or from your own body. In fact, we're even able to bioengineer stem cells from ordinary pieces of skin that can make cells that can grow in the heart muscle. It's also important to realize that we don't use embryonic stem cells in this research.

JOSEPH A. DEARANI: There is a short list of lesions, congenital heart anomalies that a child is born with that you fix. And pretty much, they can go on and live a normal life and it's life as usual, business as usual, no concerns down the road. The other end of the extreme is a short list of lesions that are notorious for requiring repeated procedures over the course of a lifetime-- the eventual expectation of the development of heart failure.

Hypoplastic left heart syndrome could be used as a sort of poster example of a lesion where that is the case. Where the child is born with a complicated heart lesion-- essentially, half the heart is not developed at all.

TIMOTHY J. NELSON: Hypoplastic left heart is a rare congenital heart disease, with 1,000 children being born every year in the United States.

MUHAMMAD YASIR QURESHI: Patients will have three different surgeries in a staged manner. The first is done in the first week of life. The second surgery is done between three to six months of age. And then the third surgery is done around two to four years of age. After that, their [INAUDIBLE] is completed. But again, it's still a palliative procedure and then you'll still keep on looking for long-term complications.

JOSEPH A. They are left with a circulation that is far from normal. So the quality of life is good, but it's not

DEARANI: normal. Children can go to school. They can participate in some sporting activities, but their exercise tolerance and capacity would be below their peers. And then over time, they will develop progression of heart failure.

TIMOTHY J. NELSON: Those that live long enough likely will require a heart transplant. And the question of organ availability, or ability to transplant, becomes a major limitation for how we take care of these kids.

JOSEPH A. DEARANI: We do up to 2,500 heart transplants a year in North America. And there are probably greater than 100,000 patients that could benefit from heart transplant.

TIMOTHY J. NELSON: There might not be an organ available. They might have antibodies in their body that prevent them from having a good matched organ.

JOSEPH A. DEARANI: Transplant is a wonderful solution for many patients that meet the appropriate criteria, but we still fall very, very short of being able to make this a realistic solution for the vast majority of patients.

MUHAMMAD YASIR QURESHI: Heart transplant is not an easy option, so it's very important to have more options, more treatment options, more tools to take care of these patients without a transplant so that you never have to get to the point where you are needing a heart transplant. Regenerative medicine and cell therapy offers this kind of an option that can prevent or delay heart failure.

So we have two clinical trials that are ongoing right now. The first one is for the babies, in which we can derive cord blood cells and take the stem cells from the umbilical cord blood and inject them back into the cells at the time of the surgery. The other one is for older children and adults with hypoplastic left heart syndrome or any other single right ventricle patient who is not doing well.

We can collect the stem cells from their bone marrow, process them and inject them back into their heart. Patients that have already done have shown a lot of improvement in their ventricular function. And it gives us hope that we're on the right path, doing the right thing, and we can change the outcome of these patients.

TIMOTHY J. NELSON: Because we can create cardiac tissue in the lab-- we can start with a piece of skin and make your heart muscle in the lab-- we can begin testing the drugs on your heart muscle without ever giving you the drug. This allows us to test for things like long QT syndrome or other arrhythmias that your heart muscle can have if we give you the wrong drugs. And we can see

how your heart responds to these drugs without ever exposing our patients to those drugs directly.

Patients that had a normal heart and may develop a heart attack or lost part of their heart muscle may also require rebuilding of the heart muscle with new stem cells.

JOSEPH A. DEARANI: This is it. This is the cutting edge right now. We are just touching what the possibilities could be.

TIMOTHY J. NELSON: It's a whole new spectrum of possibilities of what we're able to do surgically and medically to take care of these patients. So most people on the team of regenerative medicine really dream about this becoming the standard of care and really dreaming about how this transforms and transcends current therapies for heart disease.

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