TRANSPLANT AND HURLER SYNDROME

Learning more about a disease and treatment options can help you make informed medical decisions. Be The Match® can help you understand how transplant may be used to treat a child with Hurler syndrome.

READ ON TO LEARN ABOUT:
• How transplant can treat Hurler syndrome
• If transplant is right for your child with Hurler syndrome
• How a transplant doctor helps you decide if transplant is right for your child
• Questions to ask your doctor
• Transplant outcomes for Hurler syndrome
• Making treatment decisions

ABOUT HURLER SYNDROME

Hurler syndrome is one of many inherited disorders. An inherited disorder means that the disease is caused by faulty genes passed on from parents to children. Genes carry a set of instructions that tells the body how to work properly.

In Hurler syndrome, the body has a defective gene and cannot make an important enzyme. Enzymes are proteins inside cells that break down larger building block chemicals into smaller ones. When the body is missing a certain type of enzyme, the cells can't work properly. In Hurler syndrome, the body is missing an enzyme that breaks down large molecules called glycosaminoglycans (GAG).

These molecules help the body build bones and tissue. In patients with Hurler syndrome, the body cannot break down these large molecules. As a result, the GAG molecules build up and damage organs and tissues.

Hurler syndrome occurs on average in about one out of every 100,000 babies born, although it is more common in some parts of the world than in others. Children with Hurler syndrome have many physical and mental problems. If the damage is not stopped, children born with Hurler syndrome usually die by 5 to 10 years of age.

HOW TRANSPLANT CAN TREAT HURLER SYNDROME

A bone marrow or cord blood transplant is the only treatment that can stop the effects of Hurler syndrome at this time. A bone marrow or cord blood transplant begins with chemotherapy, with or without radiation, to destroy the diseased cells and marrow. The transplant replaces diseased blood-forming cells with healthy ones.

The type of transplant used for Hurler syndrome is an allogeneic transplant. This type of transplant uses healthy blood-forming cells from a family member, unrelated donor, or umbilical cord blood unit.

At the start of the transplant process, a patient gets chemotherapy to prepare his or her body for the treatment. Then the replacement cells are infused into the patient’s bloodstream. From there, the cells find their way into the bone marrow, where they start making healthy red blood cells, white blood cells and platelets. These new cells have the enzyme needed to break down GAG and stop further damage to the body.

The entire process, from start of chemotherapy or radiation until hospital discharge, can last weeks to months followed by many months of recovery at home.

Key points:
• Transplant is the only treatment that stops the progression of Hurler syndrome at this time
• All transplants for Hurler syndrome are allogeneic
UNDERSTANDING IF TRANSPLANT WOULD HELP YOUR CHILD WITH HURLER SYNDROME

Because children with untreated Hurler syndrome have physical and mental problems that get worse over time, it is best to have a transplant as soon as possible. Children who get a transplant early enough can have normal or near-normal mental development, and damage to the organs is stopped. Some physical problems, except for those affecting the skeleton and eyes, may also be improved.

However, transplants for children who have already developed severe damage have had poor results. If the disorder has caused a lot of organ damage, a child has a higher risk of developing life-threatening complications from transplant. In addition, a transplant may not undo damage the disease has already done to the body.

For these reasons, doctors recommend that children with Hurler syndrome be referred to a transplant doctor as soon as they are diagnosed. A transplant doctor who is an expert in Hurler syndrome can explain the risks and benefits of transplant.

Key points:
• Transplant is best when done early
• Children who get a transplant early enough can have normal or near-normal mental development

HOW A TRANSPLANT DOCTOR HELPS YOU DECIDE IF TRANSPLANT IS THE RIGHT TREATMENT

To find out if transplant is right for your child, he or she will need a physical check-up by a transplant doctor. During the check-up, the lungs, heart, liver, kidneys, and nervous system will be checked. The transplant doctor will also review your child’s health history and current status of the disease.

You will also meet with other members of the health care team. A social worker or other professional will meet with you to talk about your concerns related to transplant (for example: emotional, financial, travel, lodging, work and/or school). The social worker can help you find resources to support you and your family during your transplant journey.

Key point:
• Don’t be afraid to ask questions so you understand which treatments are right for your child

Key points:
• A transplant doctor will look at many things and weigh the risks and benefits of transplant before recommending a transplant
• A transplant social worker is available to help you and your family with emotional and practical support

QUESTIONS TO ASK YOUR DOCTOR

It is important to ask questions so you are comfortable with the treatments that your doctors recommend and so you can make decisions about your child’s treatment. Questions you may want to ask your doctor include:
• What are the chances of living disease-free with a transplant?
• What are the risks of waiting or trying other treatments before a transplant?
• Does my child have any risk factors that might affect the outcome of a transplant?
• How will my child’s age influence any risk factors?
• What are the possible side effects of transplant? How can they be reduced?
• What can you tell me about my child’s quality of life with a transplant? Without a transplant?
• How might my child’s quality of life change over time after a transplant?

Key point:
• Don’t be afraid to ask questions so you understand which treatments are right for your child

TRANSPLANT OUTCOMES FOR HURLER SYNDROME

Outcomes data (statistical information on how patients have done after their transplant) are used to estimate transplant outcomes. Outcomes data only show how other patients have done as a group. This information can’t tell how your child will do for sure. It can only give you an idea of how other patients have done with a similar disease and treatment. No two people are exactly the same, and your child may respond differently to your transplant than someone else. Talk to your transplant doctor about how outcomes data may apply to your child’s specific situation. Fortunately, transplant outcomes have continued to improve over time.
Key point:
• Transplant outcomes data overall are useful, but only your transplant team can tell you what your child’s chances are of doing well

MAKING TREATMENT DECISIONS
It is important to know all available treatment options so you can make the best decision for your child. A transplant doctor can help you understand the risks and benefits of transplant for your child’s specific situation.

A transplant doctor can also help you decide between transplant and enzyme replacement therapy. Enzyme replacement therapy is one way to treat children with Hurler syndrome. Children take a drug with the enzyme that their bodies lack. While this can improve many physical symptoms, there is no evidence that it has any affect on the mental decline caused by Hurler syndrome.

Enzyme replacement therapy may also help your child while doctors plan for an allogeneic transplant. In addition, enzyme replacement therapy may be a good option for children who have milder forms of Hurler syndrome (Scheie syndrome or Hurler/Scheie syndrome).

OTHER RESOURCES TO HELP YOU LEARN MORE
Be The Match has a variety of free resources to help you learn about transplant. Visit BeTheMatch.org/patient-learn and choose the resources that best meet your needs. Here are just a few that you might find helpful:
• Webcast: An Introduction to Marrow and Cord Blood Transplant
• Booklet: An Introduction to Marrow and Cord Blood Transplant
• Brochure: Understanding Transplant Outcomes Data

REFERENCES

2 Recommended Timing for Transplant Consultation. Guidelines developed jointly by National Marrow Donor Program/Be The Match and the American Society for Blood and Marrow Transplantation (ASBMT). Available at: BeTheMatchClinical.org/guidelines

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