Learning more about your disease and treatment options can help you make informed decisions about your health care. Be The Match® can help you understand how transplant may be used to treat severe aplastic anemia (SAA).

READ ON TO LEARN ABOUT:
• How transplant can treat SAA
• If transplant is right for you or your child
• Questions to ask your doctor
• Transplant outcomes for SAA
• Initial treatment of SAA
• Making treatment decisions

ABOUT SEVERE APLASTIC ANEMIA (SAA)
Aplastic anemia is a disease in which the bone marrow does not make enough blood cells for the body. The blood cells your body needs are: red blood cells (to carry oxygen), white blood cells (to fight infection) and platelets (to control bleeding).

Aplastic anemia is rare. In the United States, about 600-900 people are diagnosed each year, according to the Aplastic Anemia and MDS International Foundation. Aplastic anemia occurs more frequently in eastern Asian countries. It can affect people of any age, but it is most common in young adults. For most cases, the cause is unknown. There are some rare inherited diseases, such as Fanconi anemia, where SAA can occur over time in some patients.

HOW TRANSPLANT CAN TREAT SAA
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 SAA 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. The entire transplant process, from chemotherapy until hospital discharge, can last weeks to months with many months of recovery.

A transplant can be the first treatment chosen or it can be used after drug therapy. For many patients with SAA, getting a referral to a transplant doctor early in their disease may offer the best route to a cure.

Key points:
• Early referral to a transplant doctor can help ensure the best route to a cure for patients with SAA
• All transplants for SAA are allogeneic
UNDERSTANDING IF TRANSPLANT WOULD HELP YOUR SAA

Aplastic anemia can be moderate or severe. Only severe aplastic anemia is treated with a transplant. Whether a transplant is right for you depends on several things, such as your age, overall health and severity of your disease. A transplant doctor can help you decide if a transplant is right for you.

Depending on a variety of factors, getting a transplant early in the course of the disease may offer the best route to a cure. Patients with SAA typically get blood transfusions to ease the symptoms of SAA, but that may also lower the chance that a later transplant will be successful. For this reason, doctors recommend that patients be referred to a transplant doctor as soon as they are diagnosed.1 When you talk to a transplant doctor, you will learn about the risks and benefits of an allogeneic transplant.

**Key points:**
- Ask for a referral to a transplant doctor soon after diagnosis to find out if transplant is right for you or your child
- Early referral to a transplant doctor may be the best route to a cure for patients with SAA

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

To find out if transplant is right for you or your child, a transplant doctor will do a physical check-up. During the check-up, the lungs, heart, liver, kidneys and nervous system will be checked. The transplant doctor will also review your or your child’s health history. Understanding how likely a well-matched donor will be found may also influence initial treatment. This is why early referral to a transplant doctor is important.

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 during your transplant journey. Most transplant centers (hospitals that do transplants) require you to have a dedicated caregiver to help you through the recovery process.

**Key points:**
- A transplant doctor will look at many things including your health history, disease status and 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 doctor recommends and so you can make decisions about your treatment or treatment for your child. Questions you may want to ask your doctor include:

- What are the chances of living disease-free with a transplant? Without a transplant?
- What are the risks of waiting or trying other treatments before a transplant?
- Do I or my child have any risk factors that might affect transplant outcomes?
- What are the possible side effects of transplant? How can they be reduced?
- What can you tell me about quality of life, with or without a transplant?
- How might quality of life change over time, with or without transplant?
Key point:
• Don’t be afraid to ask questions so you understand which treatments are right for you or your child

TRANSPLANT OUTCOMES FOR SAA
Outcomes data (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 you how you or 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 2 people are exactly the same, and you or your child may respond differently to a transplant than someone else. Talk to your transplant doctor about how outcomes data may apply to your 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 chances are of doing well

INITIAL TREATMENT OF SAA
For many patients with SAA, supportive care with or without immunosuppressive therapy are the most common treatments used first.

Supportive care
Supportive care can include blood (platelet or red blood cell) transfusions and growth factors. Platelet transfusions reduce the risk of life-threatening bleeding caused by very low numbers of platelets. Red blood cell transfusions reduce problems with being very tired and short of breath caused by low numbers of red blood cells (anemia).

Doctors try to give patients as few transfusions as possible. This is because over time getting many blood transfusions can cause other problems. Too many red blood cell transfusions can lead to a buildup of iron in the body, which could require treatment to remove iron to prevent organ damage.

Immunosuppressive therapy
Immunosuppressive therapy uses medicines to hold back the immune system. This can sometimes help the marrow make more blood cells. For many patients with SAA, immunosuppressive therapy helps improve blood counts to safe levels.

MAKING TREATMENT DECISIONS
It is important to know your treatment options so you can make the best decision for yourself or your child. Soon after diagnosis, ask your doctor if talking to a transplant doctor is right for you or your child. When you meet with a transplant doctor, there are 2 main decisions to make.

The first decision is whether to have a transplant. A transplant doctor can help you understand the risks and benefits of transplant for you or your child.

The second decision is when to have a transplant. Getting a transplant at the right time in the course of the disease may offer the best chance of a cure. The transplant doctor will work with you to decide what timing for the transplant is best for you or your child.
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: Transplant Basics
- Brochure: Understanding Transplant Outcomes
- Video: Insights - Experiencing Transplant as a Young Adult

REFERENCE

1 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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AT EVERY STEP, WE’RE HERE TO HELP

As you journey through transplant, you’re not alone. Be The Match® is ready to help.

We offer many free programs and resources to support you, your caregivers and family members before, during and after transplant. Connect with us in the way that works best for you.

LEARN: BeTheMatch.org/patient
EMAIL: patientinfo@nmdp.org
ORDER: BeTheMatch.org/request
CALL: 1 (888) 999-6743

Our programs and resources offer support in 11 languages, including Spanish bilingual staff, and translation is available in more than 100 languages.

VISIT: BeTheMatch.org/translations

Every individual’s medical situation, transplant experience and recovery is unique. You should always consult with your own transplant team or family doctor regarding your situation. This information is not intended to replace, and should not replace, a doctor’s medical judgment or advice.