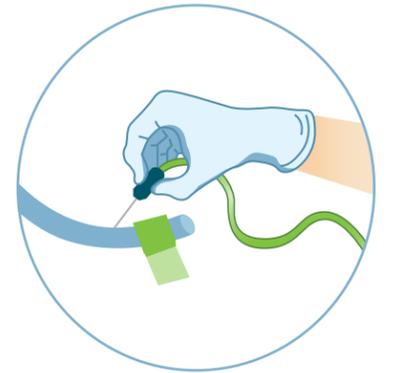




# EXPLORING TREATMENT OPTIONS FOR RARE DISEASE: CORD BLOOD TRANSPLANTS

**RARE TOOLKITS**  
Global Genes,  
Mason Shaffer Foundation,  
Parent's Guide to Cord  
Blood Foundation

# EXPLORING TREATMENT OPTIONS FOR RARE DISEASE: CORD BLOOD TRANSPLANTS

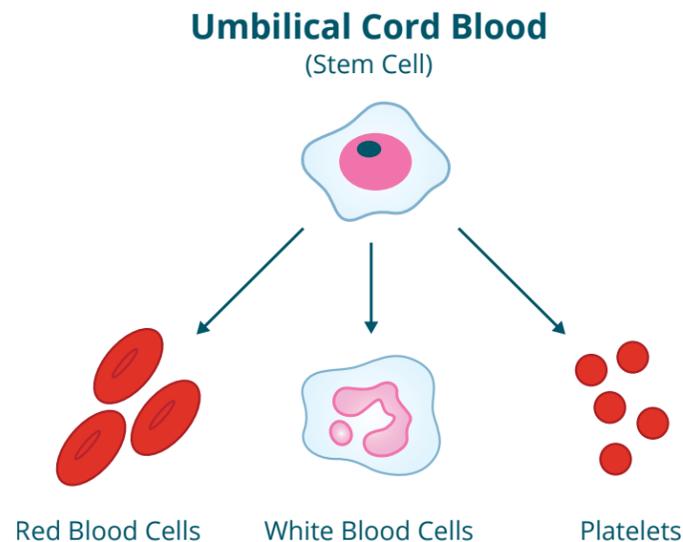


## Introduction

There are no drug treatments for approximately 95 percent of the nearly 7,000 identified rare diseases. There is, however, an FDA-approved cellular therapy option available for more than 80 rare diseases: cord blood transplants.

This toolkit provides an introduction on this treatment option, how the process works, and the resources available to learn more.

# SECTION 1: WHAT IS A CORD BLOOD TRANSPLANT?



Many rare diseases impair the function of blood cells. Some of these are cancers, some are inherited conditions, and some are triggered by infections. But many of these can be treated with blood **stem cell transplants**, or the injection of blood-forming stem cells from a healthy donor into the body to replace damaged or diseased cells. Blood-forming **stem cells** are undifferentiated cells in the body that can give rise to specialized cells.

To learn more about stem cells and their unique properties, visit the **National Institutes of Health** (<http://stemcells.nih.gov/info/basics/pages/basics1.aspx>).

Although the procedure of replenishing the body's supply of healthy blood-forming cells is generally referred to as a stem cell transplant, it can also be known as a bone marrow transplant, peripheral blood stem cell transplant, or **umbilical cord blood transplant**, depending on the source of the stem cells.

A typical umbilical cord holds about two ounces of blood that is teeming with millions of stem cells. This **cord blood** is drawn out after the baby is born and the umbilical cord has been clamped. Neither the mother nor the baby will notice the process of collecting the cord blood.

Sarah Shaffer, co-president of the Mason Shaffer Foundation, has a son who was diagnosed with an extremely rare and life-threatening disease called Malignant Infantile Osteopetrosis. After her son received a cord blood transplant, the Mason Shaffer Foundation made it a goal to expand the network of qualified cord blood collection hospitals and provide education on the cord blood donation process.

*"Once people hear stem cells, they immediately think embryonic," says Sarah Shaffer. "There is a misconception that donating cord blood will bring harm to either the child or the mother. This becomes less and less of a problem when they know more about cord blood. It is an educational challenge."*

Once harvested, the cord blood is rushed to a laboratory where the stem cells are extracted and cryogenically preserved.

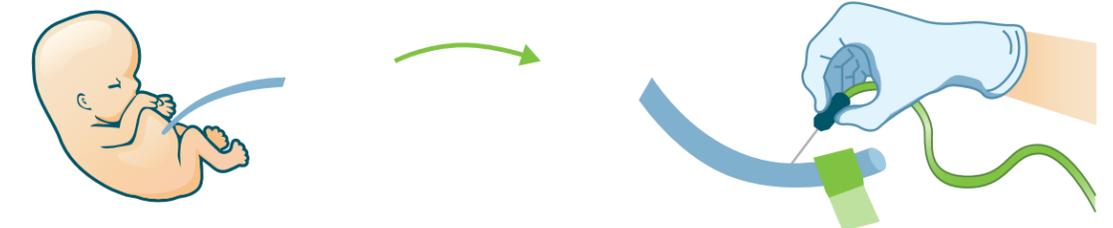
Stem cells from cord blood can provide a successful transplant when the donor and patient only match at four out of six of their tissue types. That compares to the need for a six out of six tissue type match between donor and patient for bone marrow and peripheral

## SECTION 1: WHAT IS A CORD BLOOD TRANSPLANT?

blood transplants. Because people inherit their tissue types from their ancestors, patients who belong to minority or mixed racial groups may have a hard time finding a donor. Cord blood is expanding access to transplants for minority groups and is increasingly used for these patients.

Cord blood has the advantage that the stem cells are kept frozen in a bank where they can be quickly available to patients in need. And cord blood transplants can treat certain rare diseases, such as inherited metabolic disorders, where bone marrow transplants are not as effective.

To learn which rare diseases can be treated by a cord blood transplant, visit the **Parent's Guide to Cord Blood Foundation's List of Diseases** (<http://parentsguidecordblood.org/diseases.php>). The top of the page lists more than 70 diseases for which stem cell transplants are a standard, FDA-approved therapy. All of these diseases are rare in the United States.



### Umbilical Cord

Baby is born with the umbilical cord attached. The cord is clamped and cut so that the baby can be cleaned and taken care of.

### Blood Collected & Analyzed

Cord blood will be drawn from the clamped cord into a special collection bag by the doctor. The blood is sent to a laboratory for analysis and the red blood cells are separated.



### Storage

The cord blood stem cells will be kept in liquid nitrogen storage tanks at -190 degrees Celsius inside a secured facility.



## SECTION 2: MAKING TREATMENT DECISIONS

Learning more about treatment options, like cord blood transplants, is the first step in making an informed decision. If you or your child's disease was listed on the Parent's Guide to Cord Blood Foundation's list of diseases (<http://parentsguidecordblood.org/diseases.php>), speak to your physician about cord blood transplants, get second opinions, and discuss the possible risks and benefits.

Often patients considering a transplant seek **second opinions** from a number of specialists. A second opinion from physicians and specialists provides additional information that can confirm or rule out a treatment recommendation.

Devon Vickers, who was diagnosed with Chronic Myeloid Leukemia (a rare blood cancer in children) at the age of 11, learned more about cord blood transplant by talking to her doctors, and reaching a decision together.

*"Because the leukemia I had was so rare, I had two sets of oncology and treatment teams. They presented the transplant option to me as a team," says Devon Vickers. "My doctors were very sensitive and made sure, although I was still considered a minor, that they informed me of both the advantages and potential disadvantages of undergoing a cord blood transplant. They answered any and all questions I had, and gave me time to make my decision. Because of my history with these doctors, the trust I developed with them and the mutual respect we shared, my parents and I did not seek second opinions from other specialists."*

Before attending a doctor's appointment, make sure that the doctor has your complete medical history. And during the first appointment, come prepared with a list of questions. For a list of sample questions, go to **Be The Match's Talking with your Doctor** (<http://bethematch.org/For-Patients-and-Families/Considering-transplant-and-other-treatment-options/Making-treatment-decisions/Talking-with-your-doctor/>). Find additional information on how to speak to doctors in the **Becoming an Empowered Patient: A Toolkit for the Undiagnosed** (<http://globalgenes.org/toolkits/becoming-an-empowered-patient-a-toolkit-for-the-undiagnosed/introduction-2/>).

### Understanding Transplant Outcomes

If a cord blood transplant is an option, it is normal to have a lot of questions. And one of these may be, *"What are the possible risks of choosing a transplant?"*

The risks and benefits of receiving a transplant vary from person to person, and they depend on patients' disease type, health history, and current health. **Outcomes data** are gathered from hospitals that report on how well patients have recovered after receiving a specific treatment. Although statistics cannot predict outcomes for any particular individual, they may be useful for making treatment decisions.

## SECTION 2: MAKING TREATMENT DECISIONS

Learn more about outcomes data and how to use them through **Be The Match's Understanding Transplant Options** (<http://bethematch.org/WorkArea/DownloadAsset.aspx?id=1214>). Also turn to the **Appendix** for additional information on survival rates and transplant procedure that might help you make decisions easier.

Remember, when making decisions, you are not alone. Share any information you gather with your healthcare professionals—they can help interpret the data.

### Emotional Considerations

Undergoing a stem cell transplant may be an anxiety-provoking experience. It is common to feel anxiety, fear, hope, worry, or excitement. Pediatric patients often have particular needs and concerns. By providing information on their disease and treatment in an age-appropriate manner, pediatric patients may be better able to understand and cope. Shielding them from information, on the other hand, may worsen the situation because they may imagine things that are worse. Allow them to express their emotions and help them stay positive.

Krystal Brinson, a mother of a child who was diagnosed with mucopolysaccharidosis, offers advice to parents of young children getting a cord blood transplant.

*"Since Maalik was only two years old at the time, I didn't have to explain what a transplant was to him. It was more important to get myself ready to help him in whatever way I could," says Krystal Brinson. "If he was getting ready to get a shot or take medicines, I would try to soothe him as much as possible and let him play, or I would entertain him, sing a song to make it less traumatic, etc."*

Most transplant centers provide handouts or resource materials to walk potential patients through the transplant process. People who feel too overwhelmed by the process should reach out to others. The **National Bone Marrow Transplant Link** (<http://www.nbmtlink.org/>) can link patients and their families with someone who has already gone through this journey and can provide support and answers.

*"The most important advice I give to anyone in a similar situation is to get as much support as possible from friends, family, and sometimes even strangers," says Linda Kosinski, a cord blood transplant recipient. "The doctor in California, who did the transplant, got me in touch with a patient who recently had a cord blood transplant. Speaking with her helped me a great deal to know what to expect. It was comforting to know that someone else had been through a similar experience. I have since been that person for other people going through the same thing."*

Helpful resources for teens include:

- **Insights E-Community** ([https://community.bethematch.org/?\\_ga=1.57663082.1332602614.1400170964](https://community.bethematch.org/?_ga=1.57663082.1332602614.1400170964)): This community was created to help teens and young adults connect, learn, and share. Users can watch videos, participate in discussion groups, and connect with others before, during, and after receiving a transplant.
- **Starbright World** ([www.starbrightworld.org](http://www.starbrightworld.org)): A social network for teens with chronic and life-threatening medical conditions and their siblings, it allows users to connect globally with others experiencing similar medical journeys to provide information and support.

## SECTION 3: WHAT ARE THE COSTS?



A transplant procedure includes several components, including:

- **Donor Search Fees:** Preliminary search of donors and cord blood registries is done free of charge by the doctor. Charges start to accrue, however, when donors or cord blood units are tested.
- **Compatibility Testing Costs:** Once a potential donor is identified, each test will cost money. And those costs vary.
- **Stem Cell Harvest and Donor Expenses:** These are the costs associated with the collection from stem cell donors and their associated medical tests.
- **Transplant Costs:** These have declined over time for patients. And it is possible that much, if not all, of the transplant will be covered by the insurance plan.
- **Post-Transplant Expenses:** These will depend on several factors, like recovery time. Medications, too, can be more expensive without prescription coverage.

Fortunately for the cord blood transplant recipient, Linda Kosinski, her health insurance covered most of these costs above, but this is not always the case for some patients.

“We have very good health insurance that covered just about everything,” says Linda Kosinski. “I’m sure you can imagine the cost of such a procedure. We only had to take care of co-pays. Those co-pays added up for sure, but nothing in comparison to what it could have cost us.”

If you are unsure what your insurance provider will cover, call them to ask what your transplant center options are, and request a general estimate to help plan ahead of time. Determining out-of-pocket expenses is especially important for limited provider networks. Be The Match has a **transplant cost worksheet** ([http://bethematch.org/uploadedFiles/BeTheMatchContent/For\\_Patients\\_and\\_Families/Getting\\_a\\_transplant/Planning\\_for\\_transplant\\_costs/Financial\\_assistance\\_for\\_transplant\\_patients/DOCs/Transplant\\_Costs\\_Worksheet.pdf](http://bethematch.org/uploadedFiles/BeTheMatchContent/For_Patients_and_Families/Getting_a_transplant/Planning_for_transplant_costs/Financial_assistance_for_transplant_patients/DOCs/Transplant_Costs_Worksheet.pdf)) to help calculate the transplant costs not covered by insurance.

### Financial Aid Resources

Social workers at the transplant center can help direct you to financial aid resources and organizations. Read about how a social worker helped one cord blood transplant recipient’s mom find resources and financial support in the **Appendix**.

Checking with nonprofits affiliated with your disease may help offset some of the costs as well. Some organizations to look into include:

- **Blood and Marrow Transplant Information Network** (<http://www.bmtinfor.net>): The organization has information available on its website on choosing transplant centers, finding appropriate doctors, and obtaining financial aid. It gives patients and survivors a place to turn to for accurate information.

## SECTION 3: WHAT ARE THE COSTS?

- **Bone Marrow Foundation** (<http://bonemarrow.org/financial-assistance/>): The Bone Marrow Foundation is dedicated to improving the quality of life for bone marrow, stem cell, and cord blood transplant patients and their families by providing vital financial assistance, educational information, and emotional support programs.
- **Children’s Organ Transplant Association** (<http://cota.org>): COTA assists patients who require life-saving organ, bone marrow, cord blood or stem cell transplants. It works with patients who need transplants due to genetic diseases, like cystic fibrosis and Sickle Cell Anemia, and all patients 21 and under for any transplant disease diagnosis. It also guides families and their volunteers through every fundraising step.
- **National Foundation for Transplants** ([www.transplants.org](http://www.transplants.org)): This organization benefits thousands of organ and tissue transplant candidates and recipients. Their fundraising campaigns have generated \$60 million to assist many patients during their transplant journeys.

If a transplant is for a child under 18, contact your **State Department of Health** ([www.naic.org/state\\_web\\_map](http://www.naic.org/state_web_map)). Many states have Children’s Healthcare Services that provide financial aid or insurance coverage.

If your insurance company rejects coverage of a transplant, ask for a written explanation and find out how to appeal the denial. Also ask your doctor to formally request again on your behalf. Doctors can supply medical information that will support the treatment within the appeal.

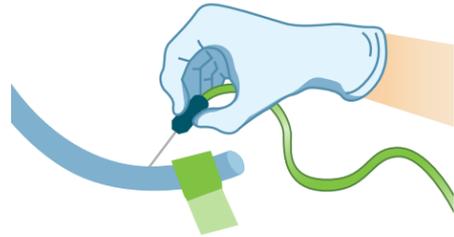
For additional information on how to appeal insurance denials, check out the following resources:

- **Global Genes’ Navigating Health Insurance Issues RARE Webinar series** ([https://globalgenes.org/navigating\\_insurance\\_issues\\_webinar\\_2/](https://globalgenes.org/navigating_insurance_issues_webinar_2/)): There are many health insurance issues that are unique for patients with rare diseases. This webinar series details points to consider when attempting to overcome obstacles to accessing your insurance provider and appealing their denials.
- **Patient Advocate Foundation** (<http://www.patientadvocate.org/resources.php?p=36>): This national 501(c)(3) nonprofit organization provides professional case management services to Americans with chronic, life-threatening, and debilitating illnesses. Use the direct link above to access its step-by-step guide on how to appeal a denial and reference sample appeal letters.

### Insider Tip:

Did you know that every claim document sent from your insurance company must outline your appeal rights and the appeals process? Look for it in the ones sent to you.

## SECTION 4: CHOOSING TO DONATE CORD BLOOD



“In the United States, about one in 200 people will have a stem cell transplant during their lifetime. And every year, thousands of Americans receive a diagnosis that requires this type of transplant,” says Frances Verter, Founder and Director, Parent’s Guide to Cord Blood Foundation.

However, according to **Be The Match** (<http://bethematch.org/>), the organization charged with operating the national registry of volunteer bone marrow donors and donated cord blood units, 70 percent of all patients who need a stem cell transplant do not have a matching donor in their family. Instead, these patients must rely on Be The Match to find an unrelated donor.

It is important to our public health to collect as many cord blood donations as possible from families with a wide diversity of backgrounds because patients are more likely to find a matching donor from people within their own racial group. A patient’s likelihood of having at least one matched umbilical cord blood unit on the Be The Match Registry ranges from 81 to 99 percent, depending on race or ethnicity.

Be The Match continually strives to increase the size and diversity of its registry to improve all patients’ chance for a cure. To help enrich the diversity of the Be The Match Registry, people may want to consider donating their cord blood stem cells. This may save the life of a patient who is in desperate need of a matching cord blood transplant.

### How to Donate

Cord blood donation and storage programs are available to expectant parents throughout the 48 connected United States and Hawaii. The Parent’s Guide to Cord Blood Foundation provides a **searchable map** (<http://parentsguidecordblood.org/donationspot/>) that shows which hospitals collect cord blood donations.

It is important to note here the differences between public and private cord blood banks. For a fee, private or “family” cord blood banks will collect, process, and **cryopreserve** (preserve through controlled freezing) umbilical cord blood for an individual family’s future medical use.

Family cord blood banking is a way for families to save their baby’s cord blood exclusively for their family. In contrast, **public cord blood banks** don’t store donations for a particular person. Instead, donations that are added to the national registry are available to anyone, anywhere in the world, who needs a cord blood transplant. Read more complete descriptions of both options through **Save the Cord Foundation’s** website (<http://www.savethecordfoundation.org/banking-choices/public-cord-blood-banking-and-donation/>).

Want to learn more about how to donate cord blood? **Be The Match** provides additional information on the process (<http://bethematch.org/Support-the-Cause/Donate-cord-blood/>

## SECTION 4: CHOOSING TO DONATE CORD BLOOD

[How-to-donate-cord-blood/](#)). The tool available through this website not only assesses an expectant mother’s eligibility to donate, but connects expectant mothers with the appropriate public cord blood bank or mail-in kit program.

In all public cord blood donation programs, there is no cost to the family for their public service. Additionally, the labor and delivery is not affected in any way. During delivery, all the focus is on the mother and baby. It is only after the baby is born that blood is collected from the cord and placenta. No blood is ever taken from the baby.



## SECTION 5: CHOOSING TO PRIVATELY BANK CORD BLOOD

Families who have a child with a rare disease that can be treated by a cord blood transplant, and are expecting another child, should investigate whether the new baby can be a cord blood donor to the older sibling. Remember, a bone marrow transplant requires a donor who is a perfect match, but a cord blood transplant is just as successful with a four out of six tissue type match. A baby who is a full sibling to the patient has a 25 percent chance of being a perfect match and about 40 percent chance of being a four out of six tissue type match. Studies have shown that a matched sibling is the ideal stem cell donor.

Before pursuing this option, parents should consult their doctor to find out if their child's rare disease is genetic. If it is, any additional child may also have the disease or be a carrier of the disease. A good resource for families considering another pregnancy after having a child with a rare disease is to consult an expert in genetic counseling. Genetic counselors can review the genetics of any conditions of concern, in each family, so that this information can be factored into the decision to save or use cord blood for therapy.

To locate a genetic counselor, use the search engine on the **American Board of Genetic Counseling** website ([https://abgcmember.goamp.com/Net/ABGCWcm/Find\\_Counselor/ABGCWcm/PublicDir.aspx?hkey=0ad511c0-d9e9-4714-bd4b-0d73a59ee175](https://abgcmember.goamp.com/Net/ABGCWcm/Find_Counselor/ABGCWcm/PublicDir.aspx?hkey=0ad511c0-d9e9-4714-bd4b-0d73a59ee175)) or the "Find a Genetic Counselor" tool on the **National Society of Genetic Counselors** website (<http://nsgc.org/p/cm/ld/fid=164>).

When parents of children who have rare diseases that can be treated by a cord blood transplant are expecting a new baby, they are eligible for free or deeply discounted cord blood banking at public and private cord blood banks. The federal government used to provide funding so that public cord blood banks could reserve "directed donations" for families in need. That program has been discontinued, so this is one area where families are more likely to get help in the private sector than through the public network. The **Parent's Guide to Cord Blood** has a page that lists programs that provide help for families in need: <http://parentsguidecordblood.org/help/>.

Private or "family" cord blood banks can be contracted to cryopreserve your baby's cord blood for the exclusive use of your family. This is a service and the family still owns the cord blood, so there is no concern that it might be used for someone else. Normally, the family has to pay to have cord blood processed and stored in a private bank, plus there are annual fees to continue storage.

## SECTION 5: CHOOSING TO PRIVATELY BANK CORD BLOOD

Private cord blood banks are registered with and inspected by the FDA. However, this is a for-profit business and parents should not just pick the first bank whose advertising they see. You can research a list of family banks in the USA (or any other country) on the Parent's Guide to Cord Blood website. A short list of top criteria to look for in a family cord blood bank is below:

1. Pick a bank that has a cord blood accreditation from **AABB** (<http://www.aabb.org/sa/facilities>) or **FACT** (<http://www.factwebsite.org/>).
2. Make sure the cord blood will be transported to the bank by a medical courier service.
3. Check to make sure that the bank's laboratory has experience providing cord blood for patient therapy.
4. Ask the bank how long they have been in business under the current owners.

The decision to publicly donate or privately store cord blood is the parents' choice— do not let any sales people convince you to purchase family banking if it does not fit your budget or your philosophy.

# RESOURCE GUIDE



## Diseases Treated by Stem Cell Transplants (Including Cord Blood Transplants)

**Parent's Guide to Cord Blood Foundation's List of Diseases** (<http://parentsguidecordblood.org/diseases.php>): This list includes dozens of genetic diseases, many of which are so rare that only a handful of children have ever been treated. Consult this list, which is updated regularly to stay current, to learn which genetic diseases can be treated by cord blood transplants.

## Donating Cord Blood

**Be The Match** (<http://bethematch.org/>): This nonprofit organization manages the national registry of available donated cord blood units and adult stem cell donors. It provides one-on-one support to patients and their families every step of the way along the transplant journey.

**Mason Shaffer Foundation** (<http://masonshafferfoundation.org>): As parents of a child whose only option of survival was a stem cell transplant, Sarah Shaffer and her husband wanted to do something that would allow their family a way to share their positive outcome. The primary purpose of the Mason Shaffer Foundation is to take their son's success story and pay it forward. Its Public Cord Blood Program provides families with the educational guidance necessary to make an informed decision about cord blood donation.

**Parent's Guide to Cord Blood Foundation's Searchable Map** (<http://parentsguidecordblood.org/donationspot/>): This searchable map details the hospitals available that accept cord blood donations.

**Parent's Guide to Cord Blood Foundation's Mail-In Donation Program** (<http://parentsguidecordblood.org/donationspot.php?state=&mailonly=10>): If your hospital does not participate in a cord blood donation program, there is an option to mail in your donation. This site details the locations that accept mail in donations.

## Financial Support

**Be The Match's Transplant Cost Worksheet** ([http://bethematch.org/uploadedFiles/BeTheMatchContent/For\\_Patients\\_and\\_Families/Getting\\_a\\_transplant/Planning\\_for\\_transplant\\_costs/Financial\\_assistance\\_for\\_transplant\\_patients/DOCs/Transplant\\_Costs\\_Worksheet.pdf](http://bethematch.org/uploadedFiles/BeTheMatchContent/For_Patients_and_Families/Getting_a_transplant/Planning_for_transplant_costs/Financial_assistance_for_transplant_patients/DOCs/Transplant_Costs_Worksheet.pdf)): Use this worksheet to help plan ahead of time and determine out-of-pocket expenses for a transplant not covered by insurance.

**Blood and Marrow Transplant Information Network** (<http://www.bmtinfonet.org/>): The organization has information available on its website on choosing transplant centers, finding appropriate doctors, and obtaining financial aid. It gives patients and survivors a place to turn to for accurate information.

# RESOURCE GUIDE



**Bone Marrow Foundation's Lifeline Project** (<http://bonemarrow.org/financial-assistance/lifeline-project/>): The Project lets donors make a direct and instant difference. Patients can send in their stories and photos to be posted where people can donate to help cover costs associated with transplant.

**Bone Marrow Foundation's Patient Aid Program** (<http://bonemarrow.org/financial-assistance/patient-aid-program/>): This program offers direct financial assistance for donor searches, compatibility testing, stem cell collection, cord blood banking, and other services. The program is not limited to specific diagnoses or age requirements, and they will work with many transplant centers across the United States

**Children's Organ Transplant Association** (<http://cota.org>): COTA assists patients who require life-saving organ, bone marrow, cord blood or stem cell transplants. It works with patients who need transplants due to genetic diseases, like cystic fibrosis and Sickle Cell Anemia, and all patients 21 and under for any transplant disease diagnosis. It also guides families and their volunteers through every fundraising step.

**Global Genes' RARE Webinar on Navigating Health Insurance Issues** ([https://globalgenes.org/navigating\\_insurance\\_issues\\_webinar\\_2/](https://globalgenes.org/navigating_insurance_issues_webinar_2/)): If your insurance company rejects coverage of a transplant, ask for a written explanation and find out how to appeal the denial. If you want additional information on how to appeal insurance denials, watch this webinar and hear directly from patient advocates who have successfully completed these steps.

**Medicaid** (<http://medicaid.gov/>): This healthcare program assists families and individuals with low income and limited resources. This government website answers questions and provides information.

**National Foundation for Transplants** ([www.transplants.org](http://www.transplants.org)): This organization benefits thousands of organ and tissue transplant candidates and recipients. Their fundraising campaigns have generated \$60 million to assist many patients during their transplant journeys.

**Patient Advocate Foundation** (<http://www.patientadvocate.org/resources.php?p=36>): This national 501(c)(3) nonprofit organization provides professional case management services to Americans with chronic, life-threatening, and debilitating illnesses. Use the direct link above to access its step-by-step guide on how to appeal a denial and reference sample appeal letters.

**Patient Services, Inc.** (<http://www.patientservicesinc.org/>): Patient Services, Inc. is a nonprofit organization that provides financial assistance to those that are chronically ill.

# RESOURCE GUIDE



## Learning More about Cord Blood

**Health Resources and Services Administration's Blood Cell Transplant** (<http://bloodcell.transplant.hrsa.gov/>): This resource provides information about bone marrow and cord blood transplantation available for patients, families, healthcare professionals, and the public. It also aims to collect data and expand research to improve patient outcomes.

**National Institutes of Health** (<http://stemcells.nih.gov/info/basics/pages/basics1.aspx>): The National Institutes of Health provides comprehensive information on stem cells, their unique properties, and how they can be used.

**Save the Cord Blood Foundation** (<http://www.savethecordfoundation.org/>): This resource provides unbiased educational information on cord blood and its life-saving qualities, banking choices, and other resources.

## Locating Genetic Counselors

**American Board of Genetic Counseling** ([https://abgcmember.goamp.com/Net/ABGCWcm/Find\\_Counselor/ABGCWcm/PublicDir.aspx?hkey=0ad511c0-d9e9-4714-bd4b-0d73a59ee175](https://abgcmember.goamp.com/Net/ABGCWcm/Find_Counselor/ABGCWcm/PublicDir.aspx?hkey=0ad511c0-d9e9-4714-bd4b-0d73a59ee175)): This professional organization has a search engine to help locate genetic counselors.

**Canadian Association of Genetic Counselors** ([https://cagc-accg.ca/component/option,com\\_sobi2/Itemid,30/](https://cagc-accg.ca/component/option,com_sobi2/Itemid,30/)): This association lists the genetic clinics within Canada.

**National Society of Genetic Counselors** (<http://nsgc.org/p/cm/ld/fid=164>): This society provides a network for professional communications for genetic counselors. Through its "Find a Genetic Counselor" tool, physicians, patients and other genetic counselors can locate genetic counseling services.

## Talking with Doctors

**Be The Match's Talking with your Doctor** (<http://bethematch.org/For-Patients-and-Families/Considering-transplant-and-other-treatment-options/Making-treatment-decisions/Talking-with-your-doctor/>): During your doctor's appointment, come prepared with a list of questions. Be The Match provides a list of sample questions.

**Becoming an Empowered Patient: A Toolkit for the Undiagnosed** (<http://globalgenes.org/toolkits/becoming-an-empowered-patient-a-toolkit-for-the-undiagnosed/introduction-2/>): Additional information on how to speak to doctors can be found within this resource.

# RESOURCE GUIDE



**Be The Match's Understanding Transplant Options** (<http://bethematch.org/WorkArea/DownloadAsset.aspx?id=1214>): This resource provides more information on outcomes data and how to use them. Review this information and share with healthcare professionals to interpret the data together.

## Talking with Other Transplant Recipients

**Be The Match Peer Connect Program** (<http://www.BeTheMatch.org>): Be The Match's Peer Connect Program can put you in touch with trained volunteers — who are transplant recipients and caregivers — to answer questions and share their own transplant experiences. The program is available to both transplant patients and caregivers. The program works to connect patients with someone that most closely matches the situation (based on age, disease, etc.).

**Child Cancer Foundation** (<http://www.childcancer.org.nz/>): This organization aims to reduce the impact of cancer by offering services to ensure children and their families are supported, informed, and well cared for on their journey with cancer.

**Childhood Cancer Support** (<http://ccs.org.au/>): Childhood Cancer Support brings together those newly diagnosed, survivors, the families of sufferers, and families who have lost loved ones, creating a network of support and understanding. Since 1975, this organization has provided a range of support services to families of children undergoing oncology (cancer) treatment at the Royal Children's Hospital Brisbane in Australia.

**Cure Search for Children's Cancer** ([www.curesearch.org](http://www.curesearch.org)): This organization offers guidance for patients, parents, siblings, friends, teacher, and anyone else who cares. It also offers educational webinars, palliative care information, and other important information.

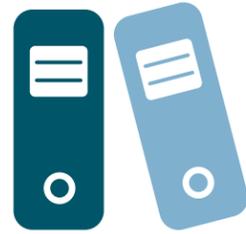
**Insights E-Community** ([https://community.bethematch.org/?\\_ga=1.57663082.1332602614.1400170964](https://community.bethematch.org/?_ga=1.57663082.1332602614.1400170964)): This community was created to help teens and young adults connect, learn, and share. Users can watch videos, participate in discussion groups, and connect with others before, during, and after receiving a transplant.

**National Bone Marrow Transplant Link** (<http://www.nbmtlink.org>): The mission of the National Bone Marrow Transplant Link is to help patients, caregivers, and families cope with the social and emotional challenges of bone marrow/stem cell transplant from diagnosis through survivorship by providing vital information and personalized support services.

**Starbright World** ([www.starbrightworld.org](http://www.starbrightworld.org)): A social network for teens with chronic and life-threatening medical conditions and their siblings, it allows users to connect globally with others experiencing similar medical journeys to provide information and support.

# APPENDIX: CORD BLOOD TRANSPLANT DETAILS

Del Steckler and Mary Thompson, Be The Match



## Clarification of the Transplant Procedure:

In general, patients will receive chemotherapy, sometimes in combination with radiation therapy. This treatment is called the preparative, or conditioning, regimen. The conditioning regimen hampers the patient's immune system so it cannot attack the donated cells; such an attack would lead to graft rejection. Depending on the disease being treated, this treatment also aims to destroy diseased cells in the body. In the most intensive forms of conditioning, the doses of chemotherapy and radiation therapy are much higher than would be used to treat the same disease in a patient who was not getting a transplant. While these higher doses may cause more severe side effects, they may also destroy more disease cells. Other patients will receive a scaled-back level of treatment, called a reduced intensity transplant when an intensive **conditioning regimen** is not desired or will not be well-tolerated by the patient.

The preparative regimen may last four to ten days. The length and strength of this treatment depends on the patient's disease, age, any previous treatments and whether the patient's disease is active.

The transplant usually occurs one or two days after completion of the conditioning regimen. The donated cells are **infused** (put into the patient's body) through an intravenous (IV) line, much like a blood transfusion. The infusion process takes about one hour, sometimes longer, depending on the volume of cells.

The donated cells "know" where they belong in the body. They move through the bloodstream to settle in the bone marrow. This is where the donated cells will begin to grow and produce new white blood cells, then platelets, and finally, red blood cells. This is called engraftment. Engraftment is an important milestone in a patient's transplant recovery. The first 100 days post-transplant are also critical to a patient, because that is when he/she will be at the highest risk for infections. Recovery after transplant can take a year or more.

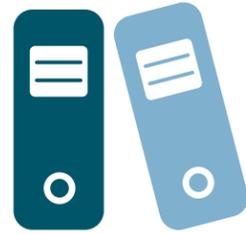
## APPENDIX: CORD BLOOD TRANSPLANT DETAILS

## Information about Survival:

A stem cell transplant (from marrow, peripheral blood stem cells or cord blood) can be a high-risk procedure, but can also be the best chance for a cure for many patients with blood cancers like leukemia and lymphoma, and more than 80 other life-threatening diseases.

One-year survival of unrelated transplant recipients at U.S. transplant centers has improved over recent years. In 2011, one-year survival of unrelated transplant recipients at U.S. transplant centers was 60.3%, compared to 42.2% in 2003.

Recently published studies have found unrelated transplant survival outcomes comparable to related transplant results in several patient populations. Over the past several years, changes in clinical practice—including advances in human leukocyte antigen (HLA) typing, better supportive care, and expanded sources of stem cells (marrow, peripheral blood stem cell and umbilical cord blood)—have contributed to improved outcomes in both pediatric and adult patients.



# APPENDIX: SHIFTING THROUGH FINANCIAL OBSTACLES

Tisha Newkirk, Mother of Lauryn

When your child goes through any extensive medical treatment, such as a cord blood transplant, it takes a financial toll on you. I had insurance that covered my daughter Lauryn at the time of her illness, but because I wasn't willing to travel out of state to a hospital that specialized in hemophagocytic lymphohistiocytosis, a rare, life-threatening genetic disease, my insurance carrier would only cover a portion of the cord blood transplant and none of the temporary housing required to keep her isolated from others.

### Secondary Insurance:

To alleviate some of the stress of my financial responsibility, I applied for **Medicaid** as a secondary insurance and was approved. For me, this was a big deal because I no longer had to worry about how I would be able to afford the co-pays for appointments, which were sometimes four to five times a week, as well as the several medications that she had to take before and after the cord blood transplant.

### Other Sources for Financial Assistance:

Prior to the cord blood transplant, the hospital assigned a social worker who played a major role in helping ease the financial stresses associated with the transplant. She made phone calls to various foundations, which donated funds to help me pay my bills since I was out of work. Those bills included several months of rent, utilities, health insurance, and car insurance.

There were times when I didn't have money to make it to appointments and the family support for the cord blood transplant unit provided me with gas cards to help me get to and from the appointments. Once my daughter was released from the hospital, we had to remain within a five minute drive, which meant we had to live at the Ronald McDonald House of Durham because my insurance didn't pay for temporary housing.

Families also provided gift cards for any personal expenses and groceries that I needed during my stay. My advice to parents would be to get their community and family involved. Fundraising events are helpful in bridging the gap of unexpected medical or just day-to-day living expenses. Rally.org and GoFundMe are two fundraising websites that allow communities to get involved.

# LET OTHERS BENEFIT FROM YOUR KNOWLEDGE BY SHARING YOUR TIPS AND TRICKS!



If you would like to contribute your experience or have a comment/suggestion, please enter it online at <http://globalgenes.org/toolkits>.

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