Disease statistics

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The Life Saving Cord Blood Bank

APLASTIC ANEMIA

INTRODUCTION

- Aplastic anemia is a type of anemia. The term "anemia" usually refers to a condition in which your blood has a lower than normal number of red blood cells.

- Paul Ehrlich introduced the concept of Aplastic anemia in 1888 when he studied the case of a pregnant woman who died of bone marrow failure. However, it was not until 1904 that Anatole Chauffard named this disorder Aplastic anemia. [1]

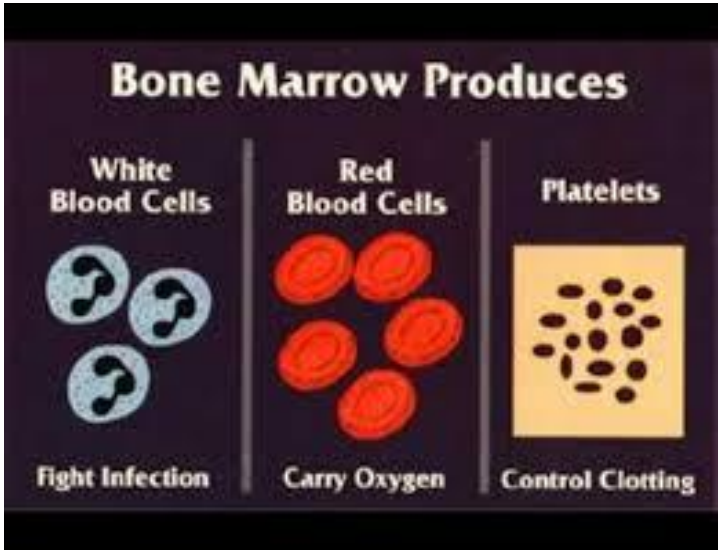
- In people who have aplastic anemia, the body doesn't make enough red blood cells, white blood cells, and platelets. This is because the bone marrow's stem cells are damaged. [2]

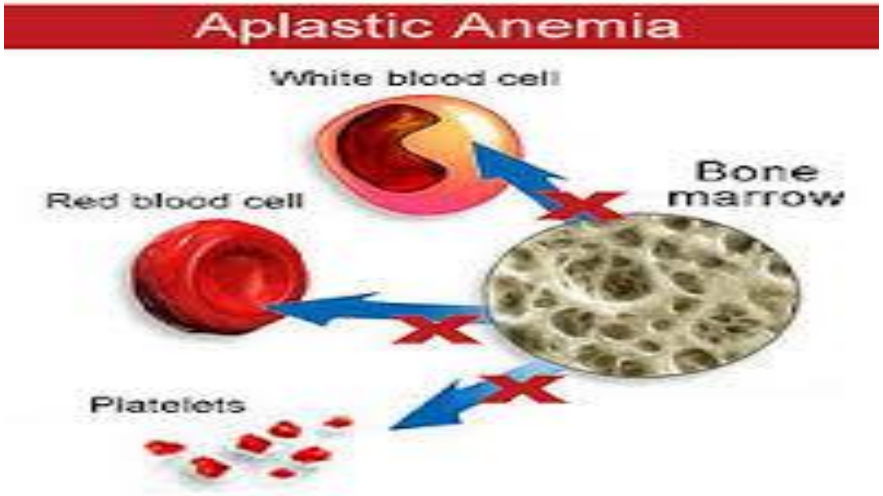
1. http://emedicine.medscape.com/article/198759-overview#showall
2. http://www.nhlbi.nih.gov/health/health-topics/topics/aplastic

WHAT CAUSES APLASTIC ANEMIA ? [1]

- Aplastic anemia is caused by destruction of the blood-forming stem cells in your bone marrow.

- These stem cells normally develop into three types of blood cells: red blood cells, white blood cells, and platelets.





1. http://www.aamds.org/about/aplastic-anemia/causes

- Aplastic anemia is generally thought to be an autoimmune disease.

- Aplastic anemia can be acquired or hereditary:

1.Acquired Aplastic anemia can begin any time in life. About 75 out of 100 cases of Acquired Aplastic anemia are idiopathic. This means they have no known cause.

2. Hereditary Aplastic anemia is passed down through the genes from parent to child. It is usually diagnosed in childhood and is much less common than Acquired Aplastic anemia.

- People who develop hereditary Aplastic anemia may have other genetic or developmental abnormalities.

- For instance, certain inherited conditions can damage the stem cells and lead to aplastic anemia. Examples include Fanconi anemia, Shwachman-Diamond syndrome, Dyskeratosis congenital and Diamond-Blackfan anemia.

- About 25 out of 100 cases of acquired Aplastic anemia can be linked to one of several causes. These include:

1. Toxins, such as pesticides, arsenic, and benzene
2. Radiation and chemotherapy used to treat cancer
3. Treatments for other autoimmune diseases, such as lupus and rheumatoid arthritis
4. Pregnancy - sometimes, this aplastic anemia improves on its own after the woman gives birth
5. Infectious dieases, such as hepatitis, Epstein-Barr virus, cytomegalovirus, parvovirus B19 and HIV.
6. Sometimes, cancer from another part of the body can spread to the bone and cause Aplastic anemia.

EPIDEMIOLOGY

- Acquired Aplastic anemia affects males and females in about equal numbers.
- Most cases affect older children, teenagers or young adults.
- The incidence of Aplastic anemia in Europe and Israel is two new cases among 1 million people per year.
- The exact incidence rates exist for the United States is unknown although some sources say that approximately 500-1,000 new cases of Aplastic anemia are diagnosed each year.
- The incidence rate is two or three times greater in Asia.[1]
- Aplastic anemia is a common hematological abnormality among peripheral pancytopenia in North Bengal region. Males were affected more than females in this study. [2]

1. https://rarediseases.org/rare-diseases/acquired-aplastic-anemia/
2. Haldar B, Pal Partha Pratim et al., Aplastic Anemia: A Common Hematological Abnormality Among Peripheral Pancytopenia. N Am J Med Sci. 2012 Sep; 4(9): 384–388.

SIGNS AND SYMPTOMS [1]

- The symptoms of Aplastic anemia are caused by low blood cell counts. The symptoms depends on which type of blood cells is affected.

1. Low Red blood cell count- Anemia.

- The most common symptom of a low red blood cell count is fatigue. It can also cause shortness of breath, dizziness, headaches, coldness in hands or feet, pale skin and chest pain.

2. Low White blood cell count- Neutropenia.

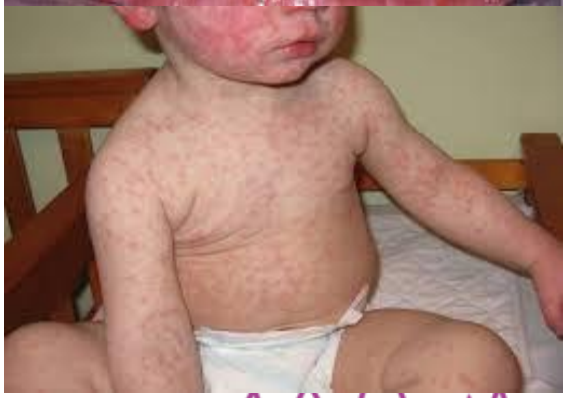
- It can increase the risk of infections.

1. http://www.aamds.org/about/aplastic-anemia/symptoms

3. Low Platelet Count- thrombocytopenia.

- It can lead to bleeding problems. Common types of bleedings include nosebleeds, bleeding gums, pinpoint red spots in the skin and blood in stool.
- Women may also have heavy menstrual bleeding.

Aplastic anemia can cause signs and symptoms that aren't directly related to low blood cell counts. Examples include nausea (feeling sick to your stomach) and skin rashes.[1]



1. http://www.nhlbi.nih.gov/health/health-topics/topics/aplastic/signs

DIAGNOSTIC TESTS [1]

- Aplastic anemia are diagnosed based on medical and family histories, a physical exam, and test results.
- **Diagnostic Tests-**

Many tests are used to diagnose Aplastic anemia:

1. Complete Blood Count

- Often, the first test used to diagnose Aplastic anemia is a complete blood count (CBC).
- The CBC also checks the number of red blood cells, white blood cells, and platelets in your blood.

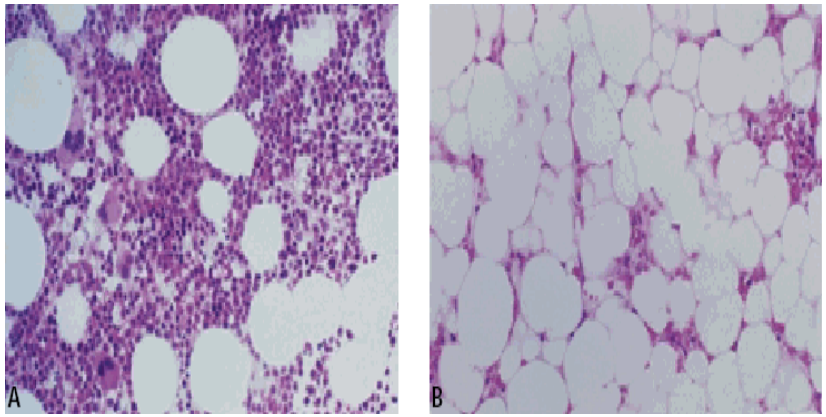
1. http://www.nhlbi.nih.gov/health/health-topics/topics/aplastic/diagnosis

2. Reticulocyte Count

- A reticulocyte count measures the number of young red blood cells in your blood.
- The test shows whether your bone marrow is making red blood cells at the correct rate. People who have aplastic anemia have low reticulocyte levels.

3. Bone Marrow Tests

- Bone marrow tests show whether your bone marrow is healthy and making enough blood cells. The two bone marrow tests are aspiration and biopsy.
- The tissue is checked for the number and types of cells in the bone marrow. In Aplastic anemia, the bone marrow has a lower than normal number of all three types of blood cells.



Bone marrow biopsy specimen from (A) healthy patient
(B) Aplastic anemia patient

TREATMENTS

- Aplastic anemia tends to get worse over time, unless its cause is found and treated. Treatments for aplastic anemia include blood transfusions, blood and marrow stem cell transplants, and medicines.
- Blood and marrow stem cell transplants may offer a cure for some people who have Aplastic anemia.

1. Blood Transfusion-

- Platelet transfusions are the first course of treatment for any Aplastic anemia patient who is severely deficient in these blood cells.
- The transfusions reduce the high risk these patients have of suffering a fatal hemorrhage.
- Transfusions of red blood cells can help combat the fatigue and shortness of breath experienced by Aplastic anemia patients.
- The process may quickly stabilize Aplastic anemia patients with severe blood cell deficiencies, but it is very rare for a patient to achieve a long-term recovery using this form of treatment alone. [1]
- Blood transfusions help relieve the symptoms of Aplastic anemia, but they're not a permanent treatment.

1. http://www.hopkinsmedicine.org/kimmel_cancer_center/types_cancer/aplastic_anemia.html

2. Drug therapy-

- To suppress immune system: Anti-thymocyte Globulin (ATG), Anti-lymphocyte Globulin (ALG), cyclosporine, Methylprednisolone which stimulates the production of blood cells by suppressing the immune system, may be recommended for these patients.
- While drug therapy is not a cure for Aplastic anemia, it may partially restore blood cell production. However, as many as 50 percent of patients relapse or develop other blood forming malignancies or disorders.
- For prevention and control of infection: Antibiotics
- To stimulate bone marrow: Growth factors such as Granulocyte-Macrophage Colony-Stimulating Factor (GM-CSF), Granulocyte Colony-Stimulating Factor (G-CSF) and Cytokines, may help stimulate the bone marrow to produce new blood cells, especially disease-fighting white blood cells. [1]

1. http://www.hopkinsmedicine.org/kimmel_cancer_center/types_cancer/aplastic_anemia.html

3. Bone Marrow Transplantation-

- Aplastic anemia was one of the first diseases for which bone marrow transplantation was found to be effective.
- The donor marrow must match the patient's immune system in order to avoid graft rejection and other serious complications.
- When successful, bone marrow transplant is often a cure for aplastic anemia with few incidences of relapse.
- However, it is a very intensive therapy, and serious immune complications and infections are not unusual.

CORD BLOOD TRANSPLANTATION

- Cord blood transplantation (CBT) is an alternative option that has been successfully explored in patients with hematologic malignancies.

1. However, a recent study from the Japanese group[1] reports 31 CBT with an overall survival of 80% was seen. Thus, CB may not be the first option in Aplastic Anemia patients lacking a family donor, but some investigators are exploring this stem cell source, and results may be encouraging.

2. *Regis Peffault de Latour (2011)* retrospectively analyzed 71 patients with Severe Aplastic Anemia who received a single-unit (n = 57; 79%) or double-unit UCBT (n = 14; 19%) in 32 centers between 1996 and 2009 [2].

At a median follow-up of 35 months, the estimated probability of 3-year overall survival was 38% ± 6%. Significantly improved overall survival was seen in recipients of total nucleated cell (TNCs) /kg prefreezing.

1. A. Yoshimi, S. Kojima, S. Taniguchi, *et al.* Unrelated cord blood transplantation for severe aplastic anemia. Biol Blood Marrow Transplant, 14 (2008), pp. 1057–1063.
2. Regis Peffault de Latour, *et al.*, Influence of Nucleated Cell Dose on Overall Survival of Unrelated Cord Blood Transplantation for Patients with Severe Acquired Aplastic Anemia: A Study by Eurocord and the Aplastic Anemia Working Party of the European Group for Blood and Marrow Transplantation. Biology of Blood and Marrow Transplantation. Volume 17 (1), 2011, pp 78–85.

3. A 20 month-old boy presented Severe Aplastic anemia secondary to liver transplantation. A living related liver transplantation from his haploidentical father was performed. Three months later, the patient developed pancytopenia.

It was decided to use patient's stored cord blood for transplantation.

The white blood cell engraftment (> 1000 µl) and platelet engraftment occurred by day 11 and 14 respectively. The patient's recovery was remarkable.

At 3 years of follow-up, the patient remains on immunosuppressive therapy for his immunosuppressive regimen after living related liver transplantation and is in complete hematologic remission with normal complete blood count. [1]

1. Steven M. Fruchtman *et al*., The successful Treatment of Severe Aplastic Anemia with Autologous Cord Blood Transplantation. Biology of Blood and Marrow Transplantation. 10: 741-742 (2004).

CORD BLOOD SUCCESS STORIES

ONE-MONTH-OLD GIRL COMES TO BROTHER'S AID [1]

By caiwenjun | november 26, 2014, Wednesday

- Three-year-old boy with a life-threatening condition received a stem cell transplant from his baby sister's umbilical cord blood at the shanghai children's medical center. In helping her brother, the one-month-old girl known as Tongtong, became the youngest donor of umbilical cord blood in the city.
- The little boy, An'an, suffering from Aplastic Anemia faced a setback 10 days ago when a matched volunteer from the Chinese marrow donor program backed out.
- The family is hopeful that their daughter's gift will bring better times.

1. http://www.shanghaidaily.com/metro/health-and-science/onemonthold-girl-comes-to-brothers-aid/shdaily.shtml

BLOOD TAKEN FROM UMBILICAL CORD SAVES A BOY'S LIFE [1]

By diana guevara on jun 27, 2012

- At only 10 years old, Ricky Martinez for more than a year he had been battling an extremely rare disease known as Aplastic Anemia. His only hope had been to get a bone marrow transplant. His family was searching for a donor since.
- But in an unexpected find, they were able to locate blood from his own umbilical cord through a national database in Washington D.C. Ricky's mother had donated the umbilical cord to science after he was born. "we couldn't afford to bank it ourselves [so] I figured let me help somebody else," said Martinez. But in an ironic twist of fate, "it came back," she added.
- It was an experimental potentially lifesaving blood transfusion that is already showing signs of promise.

1. http://www.nbcsandiego.com/news/local/blood-taken-from-umbilical-cord-saves-a-boys-life--160623165.html

HOW A BABY HELPED SAVE HIS BIG BROTHER'S LIFE [1]

By the Vancouver Sun May 9, 2007

- When Coquitlam toddler Joseph was born about 18 months ago, doctors at B.C. children's hospital took the extremely rare step of retrieving the stem cells in his umbilical cord blood and infusing them into his then-five-year-old brother, who was suffering from severe Aplastic anemia.
- "I made a note that his mother was pregnant, but I didn't count on an umbilical cord blood transplant at that point because there was only a 25-per-cent chance of a match. Then we had started a search for a bone marrow transplant donor, but we couldn't find any matches," Dr. Dix had said.
- When Joseph was born, Daniel's family and medical team were thrilled to learn that he was a 100-per-cent match.
- "We hadn't done a cord blood transplant for this [condition] at the hospital before this but we knew of a case elsewhere so we made a decision to go ahead since it was too risky for Daniel to wait any longer. He responded incredibly well." said Dr. Dix.

1. http://www.canada.com/story_print.html?id=08ad2a98-0568-4b6c-b0f6-d5c388da04e1&sponsor=

Thank you