

As featured in



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sickle cell SUCCESS

Promising rays of hope amid the
continuing crusade for a

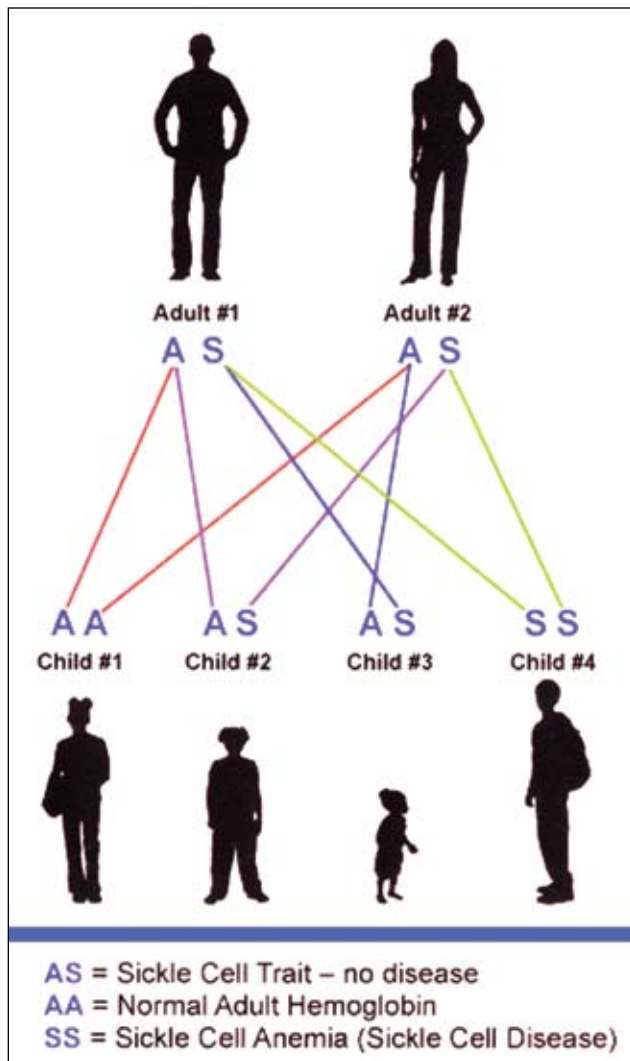
CURE

BY JOY T. BENNETT

NEARLY TWO YEARS AGO, PAMELA NEWTON WAS NEAR DEATH. Her doctors told her she was close to the end of her lifelong battle with sickle cell anemia. But today she's alive and planning a future she never expected because of promising yet risky bone marrow transplant. Her doctors say she is the first adult cured of sickle cell disease, yet doctors, scientists and parents of children with this very serious blood disorder are still searching for a universal cure.

Sickle cell disease (SCD) is an inherited blood disorder that affects red blood cells, causing them to change from the normal disc shape to a sickle shape. Those sickled cells can cause blockages in blood vessels, stopping oxygen from reaching organs. They also cause excruciating pain and even death from stroke, respiratory failure or infection. The disease affects 1 in 375 African-Americans, and more than 80,000 Americans have sickle cell disease. More than 2 million people carry the trait that can be passed on to their children, according to the Sickle Cell Disease Association of America. Millions more are affected around the world.

The disease is most common in African-Americans, Hispanics and people whose ancestors come from Africa, Saudi Arabia and other countries where malaria is or was prevalent. Inheriting one copy of the trait is protective against malaria, says Dr. Betty Pace, chief medical officer of the Sickle Cell Disease Association. The problem comes when a child inherits the sickle cell trait from both parents. "It's the most common genetic disease in the United States, says Pace.



Until recently, drug therapies were the only method to treat the disease, which can be fatal. But a high-risk bone marrow transplant is now showing promise as a cure. Bone marrow treatments have been used to treat sickle cell disease for years, but most of the 200 patients cured with this method have been children. Medical experts in general believe that adults are not likely to survive the toxic treatments that involve high doses of chemicals to the patient's own bone marrow.

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However, Newton, 35, of Capitol Heights, Md., owes her life to the procedure. Her doctors believe she is the first adult cured of sickle cell disease. After having several illnesses as a child, Newton was formally diagnosed with the disease at age 5. By 2006 she was having severe internal bleeding and was receiving daily transfusions of blood platelets. She also had been hospitalized twice in intensive care with bleeding on her brain. “They gave me six months to live,” she says. Her

Howard University Hospital doctor referred her to Dr. Robert Brodsky, director of hematology at Johns Hopkins Hospital in Baltimore, to consider a bone marrow transplant as her last option to stay alive.

A big problem with the transplant in the past is that you needed a perfect bone marrow match. Brodsky has developed a procedure that requires only a half-match, meaning that children and parents of the patient could be suitable donors. Newton’s mother was her bone marrow donor. “It’s a horrible disease,” Brodsky says. “The thing that has been so incredible is that to do a half-match is incredible with no “graft vs. host” disease [where the transplanted marrow causes a serious complication to the patient], and now she’s basically on no medications at all.”

At the time of the transplant in December 2006, Newton says she was taking 15 high-dose narcotic pain pills a day, and that doesn’t include other medications. She says because of the excruciating pain, sickle cell patients can become “legal addicts” to pain medications, complicating an already brutal ailment.

Her doctors are very optimistic about this procedure. “We’re very, very excited about this,” Brodsky says. “There’s no question that this [bone marrow transplant] can cure sickle cell. This also makes the availability much more in reach, because before, really only less than 5 percent of patients could even entertain it as an option because they didn’t have a suitable match and/or they weren’t in good enough shape to tolerate it.”

More clinical trials are necessary to determine the actual mortality rate from this procedure. Brodsky says he hopes to get enough patients for a more extensive clinical trial to be conducted at Johns Hopkins in the next year.

“Do we take a risk to cure? That’s a very, very personal decision,” Brodsky adds. “Pam knew she was going to die of this disease in the next year. She felt she had absolutely no quality of life, which she didn’t.”

Adds Newton: “I’ve dreamt about being ‘normal.’ I can’t remember a time [before the transplant] when I woke up and I wasn’t in pain,” she says. “Now my energy level is up and my mind is so much clearer. I would recommend that anybody with sickle cell anemia look into this.”

Now Newton is looking toward a future she never thought she would have. She’s planning to enroll in a Bible college, enter the ministry and possibly get married and have children. A woman of deep religious faith, she also believes that faith played a role in her cure. “I’ve always felt like God had a calling on my life,” she says. “I grew closer to God through all that I’ve been through. I knew He was going to heal me and I told God to have His way. I believe in God, and I believe one option is better than none.”

SICKLE CELL FAQ

What is sickle cell disease?

It is an inherited disorder that affects red blood cells that become hard and pointed rather than the normal soft and round. Sickle cells cause anemia, pain and many other problems.

What is sickle cell trait?

It is the inherited gene for sickle cell disease. Having the trait does not mean you will get the disease. A man and woman who have the sickle cell trait may produce a child with sickle cell disease. About 2.5 million people in America carry the sickle cell trait. (See the illustration at far left.)

How many people have sickle cell disease? It’s estimated that more than 80,000 people in the U.S. have sickle cell disease. About 1,000 U.S. babies are born with the disease each year.

Are people of African descent the only group affected? No. The sickle cell trait is also present in Portuguese, Spanish, French, Corsicans, Sardinians, Sicilians, mainland Italians, Greeks, Turks and Cypriots. Sickle cell disease also appears in Middle Eastern countries and Asia.

Information provided by the Sickle Cell Disease Association of America Inc., For more information, visit

sicklecelldisease.org

One other option for curing sickle cell anemia—using stem cells from umbilical cord blood—saved the life of Joseph Davis Jr. Born with severe sickle cell anemia eight years ago, the baby needed frequent blood transfusions to survive. “We didn’t expect to have him around very long,” says his father, Joseph Sr., a nurse who has taken care of a number of sickle cell patients.

Determined to help their son, he and his wife, Darlene, spent more than a year searching for a bone marrow or cord blood donor and could not find a match—all while their son’s condition worsened. But they soon experienced not one but two miracles. Darlene unexpectedly became pregnant. “Joseph was a fertility baby; but Isaac was not,” says Darlene Davis. The prenatal test amniocentesis revealed that the baby was a perfect tissue match for Joseph Jr. Minutes after Darlene gave birth to baby Isaac, doctors collected the blood from his umbilical cord. The Davis’ were able to save Isaac’s cord blood through Cord Blood Registry’s Designated Transplant Program, which offers free cord blood processing and storage to families with existing medical needs. “It was so easy and there is no harm to the mother or baby,” Davis says.

Joseph was 2 years old when he had his transplant. Today, doctors say the boy, now 8 years old, is completely cured of sickle cells disease. “I think the work is there and moving forward. What’s nice about umbilical cord cells is that because they are so young, the body will accept them more readily, even if it’s from someone who is unrelated,” says Pace, who is also director of the Sickle Cell Disease Research Center at the University of Texas at Dallas. It is vitally important that treatment begin as early as possible.

Doctors at the University of Chicago Hospitals concur. “It’s becoming clear that it is highly important that children with SCD are seen at the earliest possible moment after they are delivered and the diagnosis is made and that they are followed aggressively in the first three to five years of life, particularly to ensure that we minimize the number of very severe events that could occur in those first five years of life,” says Dr. John Cunningham, section chief of pediatric hematology/oncology at the University of Chicago. Cunningham and the University of Chicago Pediatric Sickle Cell Disease Program plan a series of new clinical trials of new therapies for sickle cell disease.

Pace and her colleagues are also focusing on research to develop new treatment strategies and ultimately develop a universal cure for the disease. Because of the risks of the bone marrow transplant,



Joseph Davis Jr., 8 (l.), who was diagnosed with sickle cell anemia at birth, poses with his younger brother, Isaac, 6, whose birth provided the cord blood needed to save Joseph’s life. Their parents worked with Cord Blood Registry’s Designated Transplant Program.

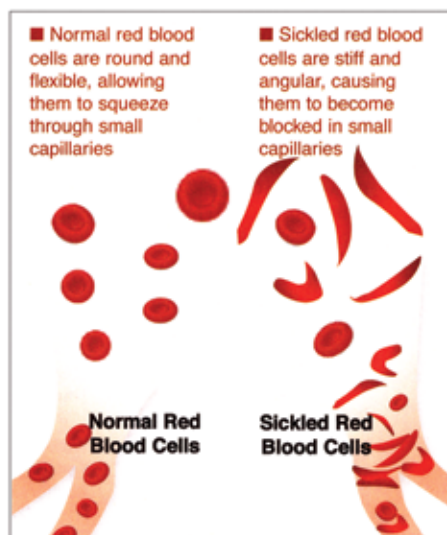
some patients (or their parents) will not choose that procedure. “Ultimately you work toward a gene therapy cure that would be universal and would be available to all individuals,” she says.

Increasing awareness about the disease and its impact is a major goal of the Sickle Cell Disease Association of America. “Finding a universal cure is the primary mission,” says Dr. Willarda V. Edwards, president and COO of association. “[Our goal is] to improved the quality of life and treatment for the community we serve and to encourage and support research that will one day find a cure for sickle cell disease.” Edwards says life expectancy for patients with sickle cell disease has risen since the 1960s and 1970s, when patients seldom lived beyond their 20s.

“Now at our conventions we see people in their 50s and 60s,” she adds.

“Today, the official life expectancy for people with sickle cell disease is 42 years for males and 48 years for females,” says Pace. She and other researchers are very excited about the groundbreaking work at Johns Hopkins, but as a scientist and researcher, she and her other medical colleagues feel that there has not been enough progress made to find a universal cure for sickle cell disease or even enough treatment options.

Cunningham agrees there is a need to expand resources in public health care. “We need more money just for laboratories and for clinical trials, and we also need to develop the trust in the community, more than we have, to work with us. This is a disease that affects people of different ethnicities, so this is a disease that should be cared about by all of society no matter what the ethnic origin of the patient,” he says. □



SICKLE CELL DISEASE SYMPTOMS

- Fatigue
- Jaundice (yellowing of the skin)
- Shortness of breath
- Pain in body organs or joints