

Arizona Public Cord Blood Banking Program

When did the Arizona Biomedical Research Commission (ABRC) establish the Arizona Public Cord Blood Banking Program (APCBBP)?

Five years ago the Commission proposed and the legislature approved funding to create a public cord blood bank. Two years ago the ABRC conducted a national survey looking for a partner to create an Arizona public cord blood collection and banking. The ABRC identified ClinImmune Labs at the University of Colorado Denver as its collaborator. ClinImmune has a nationally accredited cord blood bank with experience in banking cord blood collected at remote sites.

Where is the funding for the APCBBP coming from?

Support for the APCBBP is composed of funds directed by the voters from the state lottery to the Commission. Additional funding comes from the Health Resources Services Administration (HRSA a federal agency). ClinImmune will provide the services for processing and storage of the cord blood collected from the Arizona participating institutions. In addition, 50% of the proceeds of cord blood from Arizona that are shipped to transplant centers will come back to the APCBBP to perpetuate funding of this project for many years to come.

How much money is involved?

The ABRC has set aside \$560,000 a year for the next three years (\$1.68 million) to support the APCBBP. The money is divided between ClinImmune and the three institutional partners. In addition, HRSA has provided a \$6.2 million three year grant for this initiative with half of the units being collected in Arizona.

Who are the participants in the APCBBP?

The ABRC has created a collaboration of ClinImmune, St. Joseph's Hospital and Medical Center, Maricopa Integrated Health System, and Phoenix Baptist Hospital. In addition, the Commission has contracted for courier and air delivery services to transport the collected cord blood to Colorado for banking.

How many people are involved?

The APCBBP is not an economic development project. However, the APCBBP supports six clinical technicians and clinical coordinators, as well as the courier services in Arizona plus technicians located at ClinImmune.

How long will the project last?

ABRC financial support will continue for an additional three years with possibility of renewal. It is the hope of the ABRC that the APCBBP will continue as long as there is a need for cord blood.

What is cord blood used for?

Cord blood transplants are an established therapy for blood and bone marrow related disorders such as lymphomas, leukemia, and anemia. For a further explanation see the attached

materials. Some of the cord blood units that are deemed not suitable for banking will be available for clinical research to Arizona scientists for only the cost of shipping the unit.

Why is the focus of the APCBBP on collecting cord blood from under-represented populations?

Nationally there is a shortage of cord blood being collected from Native American and Hispanic populations. If a patient is Native American and does not have a family member match, the chance of finding matching cord blood is 1 percent. For Hispanics the chance of a match is 7 percent. For life threatening diseases the lack of matches is in effect currently a death sentence. That is why we are focusing on collecting cord blood from minorities.

Why is public cord blood banking better than private?

Public cord blood banking is by itself not better than private. The advantage is for those who cannot afford private banking, the APCBBP is a no cost option. In return, those who donate cord blood agree to make their cord blood available for others in need of a match. The cost of the APCBBP cord blood to the Transplant Center is approximately \$25,000 per unit.

How long do cord blood cells stay viable?

Once the cord is collected from the mom and child, it is kept at room temperature as it is transported by air within 44 hours to ClinImmune where it will be processed and stored in liquid nitrogen. The cord blood will remain viable for at least 25 years and perhaps longer.

What is the status of APCBBP today?

The APCBBP is one of the first regional cord blood collection and banking ventures in the United States. HRSA believes that this Arizona/Colorado initiative will serve as a national model to promote public cord blood banking and significant cost savings compared to stand alone facilities.

The first Arizona cord was collected from a Hispanic child at Phoenix Baptist Hospital on June 29, 2011.

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Standard Cord Blood Treatments for Diseases

Cord blood transplants are an established therapy for blood- and bone marrow-related disorders such as lymphomas, leukemia and anemia. As treatment for other diseases, cord blood transplants are less common and could be considered more experimental. **Table 1** provides an overview of the current therapeutic applications of cord blood, ranging from blood and bone marrow diseases to immune disorders, metabolic disorders, and specific cancers.

Table 1. Cord Blood Applications

Disease	Example of cord blood treatment	References
Bone Marrow Failure Disorders (amegakaryocytosis, aplastic anemia, Blackfan-Diamond anemia, congenital cytopenia, congenital dyserythropoietic anemia,	First use of cord blood was a sibling with Fanconi anemia. Several subsequent studies have verified that cord blood is an effective alternative to marrow for the treatment of this disease	Gluckman et al., 1990; Auerbach et al., 1990; Kohli-Kumar et al., 1993; Aker et al., 1999; Guardiola et al., 2003, 2004.
Blood Protein Disorders (sickle cell disease, beta thalassemia)	Sibling cord blood transplantation has been an effective treatment for sickle cell disease. Recent research is focused on the use of nonmyeloablative preparatory regimens.	Brichard et al., 1996; Vermeylen and Cornu, 1997; Vermeylen et al., 1998; Miniero et al., 1998; Gore et al., 2000; Locatelli et al., 2003; Barker et al., 2005
	Sibling cord blood or mixed marrow and cord blood transplantation have been successful for treatment of beta thalassemia. Research on unrelated cord blood transplantation is in the beginning stages.	Issaragrisil et al., 1999, 1995; Gedikoglu, 2001; Goussetis et al., 2000; Orofino et al., 2003; Locatelli et al., 2003; Miniero et al., 1998
Inherited Metabolic Storage Disorders (adrenoleukodystrophy, fucosidosis, Guacher Disease, Hurler Syndrome, Krabbe disease, Lesch-Nyhan Syndrome, mannosidosis, Maroteaux-Lamy syndrome, metachromatic leukodystrophy, mucopolipidosis (I-cell Disease), Batten disease, Niemann-Pick disease, Sandhoff disease, mucopolysaccharidoses, Sheie syndrome, Sly syndrome, Tay Sachs, Wolman Disease)	Unrelated transplant trials involving 20 Hurler Syndrome patients at Duke University have been successful. The stage of the disease at time of transplant has been shown to affect outcome. Larger clinical trials are needed to better understand the full range of cord blood's potential as a treatment.	Staba et al., 2004; Muenzer and Fisher, 2004
Inherited Immune Deficiency Disorders (chronic granulomatous disease, congenital neutropenia, leukocyte adhesion deficiency, severe combined immunodeficiencies, Wiskott-Aldrich Syndrome, X-linked lymphoproliferative disorder)	Long-term engraftment has been demonstrated in mice with severe combined immunodeficiency. A 2-month-old female was successfully treated with no pretreatment with a transplant from a fully matched donor.	Hogan et al., 1997
	In a data set involving 33 Wiskott-Aldrich patients transplanted with units provided	New York Blood Center, unpublished <i>IOM</i> , 2005

	by the New York Blood Center, 90 percent engrafted, and 63 percent achieved 5-year survival.	
Other Inherited Disorders (cartilage-hair hypoplasia, osteoporosis, Gunther Disease, DiGeorge Syndrome)	Bone marrow transplantation is the only fully proven treatment for osteoporosis. However, bone reabsorption has been achieved with cord blood transplantation. Due to the strain of conditioning regimens, this treatment is generally only reserved for the most severe cases.	Locatelli et al., 1997; NIH, 2000
Other Diseases Treated with Cord Blood		
Leukemias and Lymphomas (acute biphenotypic leukemia, acute lymphocytic leukemia, acute myelogenous leukemia, acute undifferentiated leukemia, adult T cell leukemia/lymphoma, chronic lymphocytic leukemia, chronic myelogenous leukemia, Hodgkin's lymphoma, juvenile chronic myelogenous leukemia, juvenile myelomonocytic leukemia, myeloid/natural killer cell precursor acute leukemia, non-Hodgkin's lymphoma, polymorphocytic leukemia)		
Myelodysplastic/myeloproliferative Disorders (acute myelofibrosis, agnogenic myeloid metaplasia, amyloidosis, chronic myelomonocytic leukemia, essential thrombocythemia, polycythemia vera, refractory anemias)		
Plasma Cell Disorders (multiple myeloma, plasma cell leukemia, Waldenstrom's macroglobulinemia)		
Histiocytic Disorders (familial erythrophagocytic lymphohistiocytosis, hemophagocytosis, Langerhans' cell histiocytosis)		
Malignancies (brain tumors, Ewing sarcoma, neuroblastoma, ovarian cancer, renal cell carcinoma, rhabdomyosarcoma, small cell lung cancer, testicular cancer, thymic carcinoma)		
Other Disorders – Not Inherited (chronic active Epstein Barr, Evans Syndrome, multiple sclerosis, rheumatoid arthritis, systemic lupus erythematosus, thymic dysplasia)		

Sources:

- 1) **Cord Blood: Establishing a National Hematopoietic Stem Cell Bank Program.** Institute of Medicine of the National Academies. Committee on Establishing a National Cord Blood Stem Cell Bank Program, Emily Ann Meyer, Kathi Hanna, and Kristine Gebbie, Editors, National Academies Press, Washington, D.C., 2005.
- 2) **CBR Systems, Inc.** www.cordblood.com/cord_blood_banking_with_cbr/banking/diseases_treated.asp
- 3) **Stem Cell Therapies** www.stemcelltherapies.org/treatable_conditions.htm

Experimental Cord Blood Therapeutic Research

With recent findings that stem cells from cord blood—once thought capable only of turning into blood cells—may be able to grow into other kinds of cells, research on cord blood as treatment for other diseases has ramped up. **Table 2** shows areas of current research using cord blood transplants to treat a wide range of diseases from cardiac and neurological diseases to autoimmune disorders and advanced cancers.

Table 2. Cord Blood Research Focus Areas

Disease	Status of cord blood research	Reference
Heart disease	Capillary-like tubes are grown in culture Perry and Roth (2003)	Perry and Roth (2003)
	Transplants in animals have led to improved cardiac function.	Vanelli et al. (2004)
Central nervous system disease	Mice with amyotrophic lateral sclerosis improved after transplantation	Newman et al. (2004)
Spinal cord injury	Hematopoietic progenitor cells engrafted in the area of injury in rats	Saporta et al. (2003)
Stroke	Vascular activity in damaged area in mice increased post-transplantation	Taguchi et al. (2004)
	Motor improvement was noted in mice post-transplantation	Willing et al. (2003)
Brain damage	Hypoxic mice showed improvement posttransplantation	Jensen et al. (2003)
Parkinson's disease	N/A	Cord Blood Registry®
Liver injury	Potential for transdifferentiation was first noted in humans post-transplantation	Di Campi et al. (2004)
Gastrointestinal disorders	Minimal transdifferentiation for intestinal tissue was noted	Ishikawa et al. (2004)
Muscular dystrophy	N/A	Cord Blood Registry® ISCCR
Diabetes	N/A	Cord Blood Registry®
Chronic inflammatory diseases (lupus)	N/A	ISCCR
Metabolic disorders (amyloidosis)	N/A	ISCCR
Advanced cancers	N/A	ISCCR

Sources:

- 1) **Cord Blood: Establishing a National Hematopoietic Stem Cell Bank Program.** Institute of Medicine of the National Academies. Committee on Establishing a National Cord Blood Stem Cell Bank Program, Emily Ann Meyer, Kathi Hanna, and Kristine Gebbie, Editors, National Academies Press, Washington, D.C., 2005.
- 2) **CBR Systems, Inc.** www.cordblood.com/cord_blood_banking_with_cbr/banking/diseases_treated.asp
- 3) **International Society for Stem Cell Research (ISCCR)** <http://www.isscr.org/science/faq.htm>

Cord Blood in Clinical Trials

We queried the FDA's website, clinicaltrials.gov, to determine the range and types of conditions being treated experimentally with cord blood. At present, 138 active or completed studies use cord blood as the intervention. The conditions listed most frequently in these studies are almost entirely related to disorders of the lymph and blood. **Table 3** shows the conditions listed most frequently (many studies address more than one type of condition, resulting in the sum of all conditions far exceeding the total number of studies). Conditions that were listed in only a few instances are not shown.

Table 3. Conditions under Evaluation for Treatment with Cord Blood

Condition	Approximate Number of Times Listed among 138 Trials
Leukemia	760
Lymphoma	167
Anemia	119
Neoplasms/Cancer	110
Congenital Abnormalities	35
Vascular Disorders	35
Blood Coagulation Disorders	29
Metabolic Disorders	22

Source: <http://www.clinicaltrials.gov/ct2/search/advanced>