News and Perspectives
Therapeutic Uses of Cord Blood Stem Cells

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Type 1 Diabetes: Landmark Clinical Trial Exploring Cord Blood Stem Cells as Therapy

Nearly 21 million Americans have some form of diabetes, a disease in which the body does not produce or properly use insulin, resulting in high blood glucose levels. In the case of type 1 diabetes, the body's immune system destroys insulin-producing beta cells in the pancreas. While disease onset can occur at any age, type 1 diabetes is particularly prevalent in children—one in 300 children is affected by diabetes, and the incidence is increasing three to five percent annually. To help address this startling trend, the University of Florida initiated a phase I/II clinical trial to examine how an infusion of autologous (or one's own) cord blood stem cells into children with diabetes will impact metabolic control over time, as compared to standard intensive insulin treatments. Researchers just presented compelling observational data from this trial, which show promising results, at the American Diabetes Association's 67th Scientific Sessions.

Cord Blood Stem Cells Have Distinct Clinical Benefits

Today, the three primary sources of stem cells for either scientific study or therapeutic use are: embryonic, adult (which includes bone marrow and peripheral blood) and cord blood.

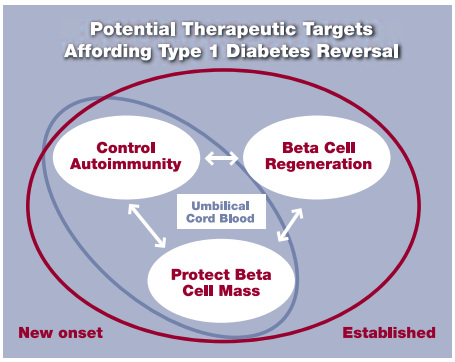
Used only experimentally in scientific study since 1998, embryonic stem cells remain highly controversial. Although research indicates they have significant therapeutic potential, researchers have yet to determine how to control their growth, guide their development into specific cell types or utilize them in human treatments.

Conversely, adult stem cells have been used in a variety of treatment areas for more than 40 years and have proven therapeutic value. Although effective, bone marrow stem cells are obtained in an invasive, complicated and painful procedure and do not grow or increase as rapidly as those found in cord blood. While the collection of peripheral blood stem cells is less invasive, these cells have many of the same properties as bone marrow stem cells, and thus many of the same potential limitations.

Umbilical cord blood, long considered a "waste product" of the birthing process, is a rich source of readily-available, easily-acquired stem cells. Cord blood stem cells have been used successfully for more than 20 years. Currently, they are used to treat approximately 70 diseases including immunodeficiences; genetic and neurological disorders; some cancers; and blood disorders, such as leukemia, lymphoma, sickle cell anemia, and aplastic anemia. Today, physicians have performed more than 8,000 cord blood stem cell transplants worldwide. These stem cells hold vast therapeutic promise to address major unmet medical needs and are increasingly being used in medical therapies to improve—and save—lives.

Children Infused with Autologous Cord Blood Stem Cells Show Marked Improvement

Preliminary results presented by the study's primary investigator, Dr. Michael J. Haller, demonstrate an infusion of cord blood stem cells reduced patients' disease severity, possibly re-setting the immune system and slowing destruction of insulin-producing beta cells in the pancreas. Children in the study who received the infusion of their own cord blood stem cells had lower blood sugar levels and required less insulin than the control group. Over a six-month observation period, these children continued producing their own insulin longer than expected and, therefore, may be at lower risk for diabetes complications over the length of their lifetime.



Researchers want to understand how autologous cord blood stem cell infusion impacts glucose control in children with type1 diabetes. The potential options may include any of the following: 1) regenerating islet-producing cells; 2) protecting existing insulin-producing cells; 3) controlling the body's immune response.

Introducing Cord Blood Stem Cells May Alter Course of Type 1 Diabetes

Diabetes can lead to debilitating or life-threatening conditions including heart disease, stroke, blindness, kidney disease, and amputations. Currently, it is the fifth leading cause of death by disease in the U.S. Consider this:

Cord blood is being considered a strong candidate for diabetes therapy for two reasons: 1) the stem cells in cord blood are capable of differentiating into insulin-producing cells and 2) cord blood contains cells that may help prevent the progressive "self-destruction" of the existing insulin-producing cells in the pancreas. Earlier animal studies using cord blood to treat diabetes also have delivered positive results. In an unrelated trial, mice with type 1 and type 2 diabetes that received an infusion of cord

blood stem cells had decreased glucose levels, improved survival curve, and reduced insulitis.

A New Era in Regenerative Medicine

Although further study is needed to determine just how cord blood yields benefits in treating diabetes in humans, it is just one of a growing number of regenerative stem cell therapies that are moving from the lab into the clinic. Regenerative medicine studies are underway for a variety of conditions including heart disease, stroke, Parkinson's disease, spinal cord injury, amyotrophic lateral sclerosis, muscular dystrophy and liver disease.

Each Year:	
1 in 7,000	Children develop type 1 diabetes
20.6 million	Americans are living with diabetes
12,000 - 24,000	New cases of blindness are caused by diabetes
In 2002:	
224,000	Deaths were associated with diabetes
44,000	People with diabetes were treated for end-stage renal disease