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## **FIRST USE OF CORD BLOOD TO ALTER COURSE OF TYPE 1 DIABETES**

### **Yields Insights for Developing Future “Cocktail” to Treat the Disease**

*Chicago, IL (June 25, 2007)* – In a small pilot study, transfusion of stored, autologous (i.e. the person’s own), umbilical cord blood into a group of children newly diagnosed with type 1 diabetes appears to have reduced their disease severity, possibly re-setting the immune system and slowing the destruction of their insulin-producing cells, according to a report presented today at the American Diabetes Association’s 67th Annual Scientific Sessions.

“After only six months, it is too early to tell how long the children will benefit from this therapy, but early signs indicate that it may have helped enhance blood glucose control and management,” said Michael J. Haller, MD, Assistant Professor of Pediatric Endocrinology at the University of Florida College of Medicine and lead author of the study, in a recent interview.

“But more important than the potential benefit in these children, this first use of cord blood in diabetes will help us focus on what it is in the cord blood that yielded the benefit,” he said. “We then hope to isolate and grow that cell type to develop therapies for a larger pool of people, not just those who have stored cord blood.” He discussed how such a cellular therapy might be one component of a future immune-modulating “cocktail.”

Nearly 21 million Americans have diabetes, a group of serious diseases characterized by high blood glucose levels that result from defects in the body’s ability to produce and/or use insulin. Diabetes can lead to severely debilitating or fatal complications, such as heart disease, blindness, kidney disease, and amputations. It is the fifth leading cause of death by disease in the U.S.

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Type 1 diabetes is an immune-mediated disease that involves a failure of the body’s immune system to recognize cells – its “self” – as non-threatening (called a failure of “tolerance”), leading to progressive destruction of the insulin-producing beta cells in the pancreas. Type 1 diabetes usually strikes children and young adults, although disease-onset can occur at any age.

## **STUDY METHODOLOGY AND RESULTS**

The researchers recruited seven young (age 2 to 7 years at the time of infusion) children with type 1 diabetes who had their own stored cord blood and infused them with it. This group was matched with 13 randomly selected youngsters of similar age and diabetes duration who had been intensively treated with insulin and served as a control group.

A1C tests and total daily insulin use from diagnosis to six months after infusion were compared. (A1C is a measure of blood glucose control over a two- to three-month period.)

The children who received cord blood transfusions had lower average A1Cs, i.e. 7% vs. 8.04% in the intensively treated control group receiving insulin therapy alone. Children who had cord blood transfusions also required much lower average total daily insulin than the control group, 0.45 vs. 0.69 units of insulin per kilogram per day, respectively.

Over the six-month period there was little change in the stimulated C-peptide values of the group, indicating that they may have had retention of endogenous insulin production longer than expected for young children.

“Because of the cord blood infusion, these youngsters may retain endogenous insulin production for a longer period,” said Dr. Haller. “Therefore, they may be at lower risk for diabetes complications over the length of their lifetime, as was demonstrated by the groundbreaking Diabetes Control and Complications Trial.”

No adverse events associated with the transfusions were observed.

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## CONCLUSIONS

“Our preliminary data showing lower A1Cs, lower average insulin requirements, and possible preservation of C-peptide suggest a beneficial effect of autologous umbilical cord transfusion in youngsters with recent onset type 1 diabetes,” said Dr. Haller. “Considerable research today is seeking to delay complete beta cell loss, and this may be one effective approach for children who have their own cord blood, who are newly diagnosed with type 1, and who enter clinical trials.”

However, he emphasized that it would be costly and inefficient for everyone to save their cord blood as a possible type 1 therapy. Therefore, the goal is to determine the factor in the cord blood that is yielding the benefit.

## POSSIBLE MECHANISMS

Desmond A. Schatz, MD, Professor and Associate Chairman of Pediatrics at the University of Florida College of Medicine and senior author of the study, posited three potential mechanisms for the results in a recent interview.

“While cord blood contains stem cells capable of differentiating into insulin-producing cells, and infused cells could have stimulated islets to regenerate, it is most likely that infused regulatory T cells, known to be able to induce autoimmune tolerance, may have prompted a type of immune regulation,” he said.

“We think that cord blood is a very rich source of these regulatory T cells, and there was a measurable increase in these cells in patient’s blood through six months after the infusion,” said Dr. Schatz. “While we have not followed the participants long enough to determine how long these benefits will last, the improvement in blood glucose control appears to be related to the infusion of these cord blood cells.” The youngsters will continue to be followed long term.

While this study does not confirm a specific advantage for any particular type of cell therapy, it argues strongly in favor of expanded studies to better characterize any source of regulatory T cells (also known as T regs) that may eventually be used in type 1 diabetes.

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“Transfusing the cord blood may provide a bolus of T regs, a type of immune cell that can keep the immune system from attacking the pancreas,” said Dr. Haller. Based on the changes observed at six months, the researchers think the T regs may restrain the immune system.

“The idea of restoring tolerance is the holy grail of autoimmune research, and would make it possible to arrest and perhaps even prevent the development of diabetes,” he explained. “Our theory is that T regs are one of the key factors yielding benefit in cord blood,” he explained. “Eventually we might be able to take T regs out of cord blood in order to have a source from which to grow more.”

“The type 1 diabetes disease process may be altered with T regs plus a mild immunosuppressive or other immunomodulating drug or additional cell therapy, which is called “cocktail therapy,” in a manner similar to the breakthroughs made in treating HIV and cancer,” said Dr. Heller.

“We feel a cautious optimism for a role for cellular therapies in altering the natural history of type 1 as it relates to ameliorating the disease or preventing the disease, likely in combination with other agents,” said Dr. Schatz.

An editorial in the *Journal of the American Medical Association* in April 2007 on another approach to cellular therapy to interdict type 1 diabetes noted that umbilical cord cells are one of the many sources of such cells under consideration, as well as T-regulatory lymphocytes, embryonic or adult stem cells, and dendritic cells. The editorialist suggested that “Research in this field is likely to explode in the next few years...” and that “The time may indeed be coming for starting to reverse and prevent type 1.”<sup>1</sup>

Co-authors with Drs. Haller and Schatz were Mark Atkinson, PhD; Hilla-Lee Viener, BS; Todd Brusko, PhD; Clive Wasserfall, MS; Kieran McGrail, BS; Susan Staba, MD; and Chris Cogle, MD, all of the University of Florida College of Medicine.

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The American Diabetes Association is the nation's leading voluntary health organization supporting diabetes research, information and advocacy. Founded in 1940, the Association has offices in every region of the country, providing services to hundreds of communities. For more information, please call the American Diabetes Association at 1-800-DIABETES (1-800-342-2383) or visit [www.diabetes.org](http://www.diabetes.org). Information from both these sources is available in English and Spanish.

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Abstracts #313-OR and #314-OR

1. Skyler J. Cellular Therapy for Diabetes: Has the Time Come? *JAMA* 2007;297 1568-1576.

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