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Innovative Immune Cell Re-education Technique Holds Promise for Type 1 Diabetes

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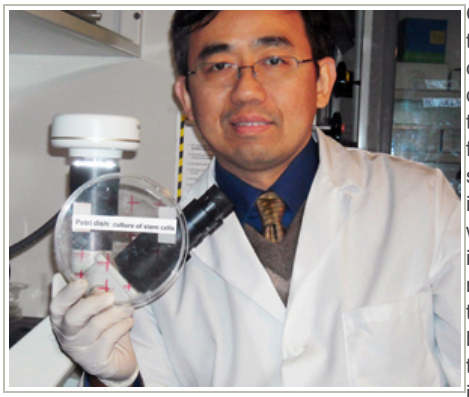

One of the biggest challenges that scientists face in aiming to cure autoimmune diseases is developing a lasting therapy targeted toward the body's faulty immune response, while safely preserving overall immunity and health. In people with type 1 diabetes, the immune system mistakenly recognizes its own pancreatic tissue as foreign and responds by sending destructive T cells to attack – just as it would for invading bacteria or a virus. As a result, vital insulin-producing beta cells are destroyed, and normal regulation of metabolism is lost.

In 2010, the ADA funded an innovative research project lead by **Yong Zhao, MD, PhD**, who proposed a novel use for stem cells, derived from human umbilical cord blood, to overcome autoimmunity in patients with type 1 diabetes and ultimately reverse the disease. Dr. Zhao's creative strategy to educate the immune system for long-term treatment impressed leading experts in the field, and he was named the winner of the Gail Patrick Innovation Award for his project's potentially high impact and advancement towards a cure.

Dr. Zhao saw that stem cell therapy could help replace lost beta cells. However, once an autoimmune response has occurred, attempts to replace them with either stem cells or transplanted tissue are generally susceptible to the same underlying autoimmune response that caused the original tissue destruction. For example, in the case of islet transplantation, long term immune suppression is required to prevent the new tissue from being destroyed.

Dr. Zhao's approach aimed to overcome this critical barrier. He and fellow colleagues at the University of Illinois at Chicago pioneered an *in vitro* technique called Stem Cell Educator therapy. In their study, patients with moderate to severe diabetes were connected to a blood-separating machine, which circulates blood and isolates the immune system's regulatory T cells, called lymphocytes. These lymphocytes are then exposed to cord blood stem cells derived from healthy donors, where they are re-trained to function normally. The newly "educated" lymphocytes are then reintroduced back into the blood circulation of the patient. Notably, no transplantation of donor stem cells or beta cells into the patient occurs in this technique.

Now, two years after embarking on his first independent grant, Dr. Zhao's approach shows successful control of the immune response, indicated by a lasting cell regeneration and restoration of beta cell function in diabetic patients who had very little remaining function prior to treatment. All of the study participants responded well to the therapy and experienced little pain and no adverse reactions. The researchers' initial results concluded that the therapy is feasible and safe for both the patient and the donor, and that one treatment is

effective in restoring normal immune function and insulin production for at least 10 months. Throughout this time, the participants showed significantly improved long-term blood-sugar control and less dependence on their daily insulin injections.

Further studies are necessary to determine the enduring effectiveness of the treatment and optimal dosage, but Stem Cell Educator therapy offers powerful evidence and potential for a cure. The impressive work was published in the journal *BMC Medicine* on January 10, 2012. Dr. Zhao's research findings may also benefit the treatment of other autoimmune or inflammatory diseases, in addition to providing considerable time and cost savings.

(Yong Zhao, Zhaoshun Jiang, Tingbao Zhao, Mingliang Ye, Chengjin Hu, Zhaohui Yin, et al. Reversal of type 1 diabetes via islet beta cell regeneration following immune modulation by cord blood-derived multipotent stem cells. *BMC Medicine*, 10:3, 10 January 2012.)