

Stem cell transplantation helps patients with diabetes become insulin free

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The majority of patients with type 1 diabetes who underwent a certain type of stem cell transplantation became insulin free, several for more than three years, with good glycemic control, and also increased C-peptide levels, an indirect measure of beta-cell function, according to a study in the April 15 issue of *JAMA*, a theme issue on diabetes.

Richard K. Burt, M.D., of the Northwestern University Feinberg School of Medicine, Chicago, presented the findings of the study at a *JAMA* media briefing at the National Press Club in Washington, D.C.

Clinical evidence indicates that there is an inverse association between beta-cell (a type of cell in the pancreas that secretes insulin) preservation and function and chronic complications of [type 1 diabetes](#) mellitus (DM), and the higher the C-peptide levels (a byproduct of insulin production, made up of [amino acids](#)), the lower the incidence of some types of complications of type 1 DM. A previous study found that autologous nonmyeloablative hematopoietic stem [cell transplantation](#) (HSCT) in 15 patients with newly diagnosed type 1 DM resulted in the majority of patients becoming insulin free during the follow-up, which averaged about 19 months. "However, it was suggested that subsequent insulin independence was a prolonged honeymoon period due to dietary and exercise changes associated with close posttransplant medical observation," the authors write, and it was not known if this change was because of an improvement in beta-cell preservation.

HSCT, which uses a patient's own blood stem cells, involves the removal

and treatment of the [stem cells](#), and their return to the patient by intravenous injection.

Dr. Burt and colleagues conducted a study to determine if posttransplant insulin independence was due to improved beta-cell function by monitoring the C-peptide levels of 23 patients who underwent stem cell transplantation. The patients, with type 1 DM, were ages 13-31 years.

Of the 23 patients, 20 experienced time free from insulin (12 continuously and 8 transiently). Patients remained continuously insulin free for an average time of 31 months (range, 14-52 months). One patient had more than 4 years with no exogenous (produced outside the body) insulin use, 4 patients for at least 3 years, 3 patients for at least 2 years, and 4 patients for at least 1 year. Eight patients relapsed and resumed insulin use at low doses. The majority of patients achieved good glycemic control.

In the continuously insulin-free group, average area under the curve (AUC; a type of measurement) of C-peptide levels before transplantation (225.0 ng/mL per 2 hours) showed a significant increase at 24 months after transplantation (785.4 ng/mL per 2 hours) and at 36 months after transplantation (728.1 ng/mL per 2 hours). In the transient insulin-independent group, average AUC of C-peptide levels also increased from 148.9 ng/mL per 2 hours pretransplantation to 546.8 ng/mL per 2 hours at 36 months, which was sustained at 48 months. In this group, 2 patients regained insulin independence after treatment with the antihyperglycemic drug sitagliptin, which was associated with an increase in C-peptide levels.

Two patients developed pneumonia in the hospital, 3 patients developed late endocrine dysfunction, and 9 patients developed oligospermia (sperm deficiency). There were no deaths.

"In conclusion, autologous nonmyeloablative HSCT was able to induce prolonged and significant increases of C-peptide levels associated with absence of or reduction of daily [insulin](#) doses in a small group of patients with type 1 DM," the researchers write. "At the present time, autologous nonmyeloablative HSCT remains the only treatment capable of reversing type 1 DM in humans. Randomized controlled trials and further biological studies are necessary to confirm the role of this treatment in changing the natural history of type 1 DM."

More information: JAMA. 2009;301[15]:1573-1579.

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