Clinical VX-880 trial improves blood sugar control in all treated patients, with three achieving insulin independence

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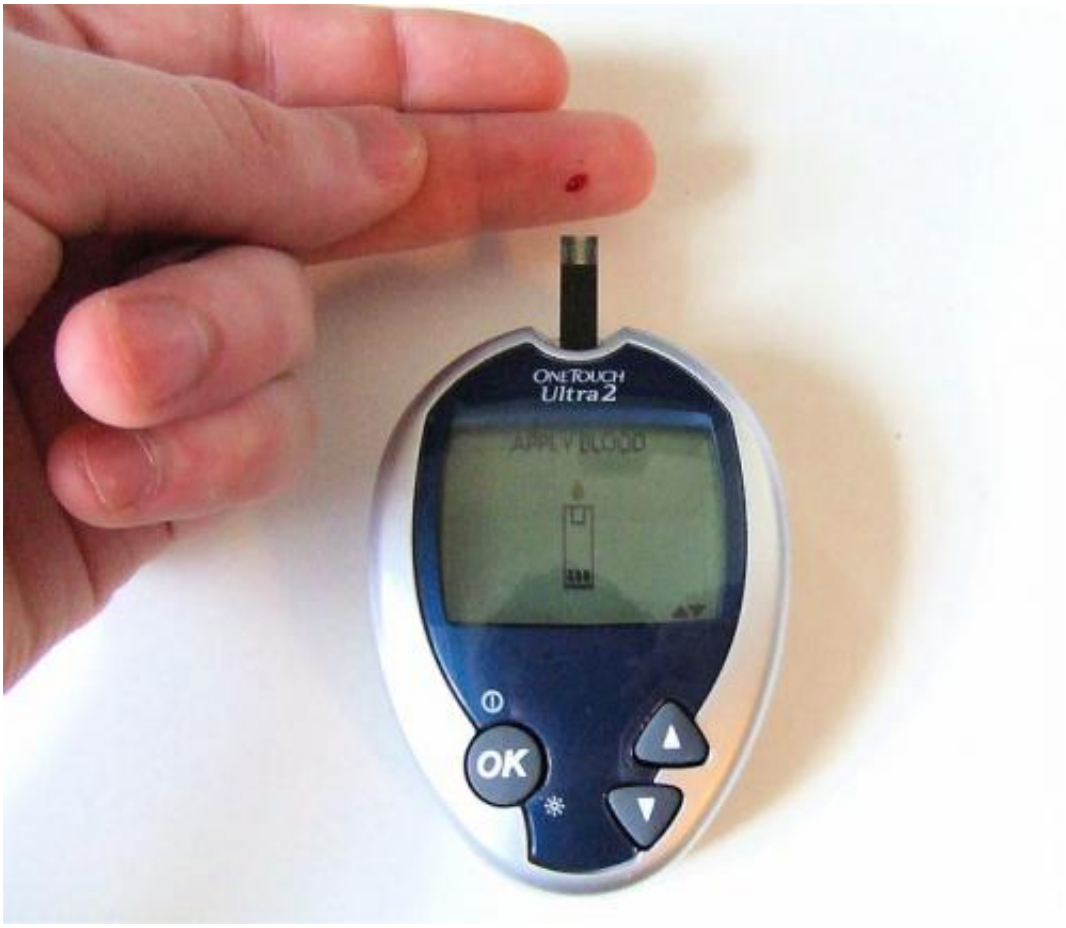

Blood glucose monitoring. Credit: Wikipedia

Six adults with type 1 diabetes (T1D) treated with stem cell-derived islet

cells (VX-880) have shown improved blood sugar control, with three participants achieving insulin independence, according to new research being presented at this year's Annual Meeting of The European Association for the Study of Diabetes (EASD) in Hamburg (2–6 Oct).

All patients treated with VX-880 have demonstrated improved glycemic control, as evidenced by elimination of severe hypoglycemia (low blood sugar), improvements in HbA1c (a measure of long-term sugar levels) and the amount of time their blood glucose levels were within the recommended range, as well as a reduction or elimination of their externally administered insulin needs.

The phase 1/2 trial results presented by Professor Trevor Reichman from Toronto General Hospital in Canada are from a study evaluating VX-880 in adults with impaired hypoglycemic awareness and a history of severe hypoglycemic events, where patients are unable to sense the symptoms of low blood sugar, and sugar levels that may drop so low that patients are unable to recover without assistance.

"These results are truly remarkable and offer hope of a life-changing therapy for people who suffer from the relentless life-long burden of type 1 diabetes," says Professor Reichman, M.D., Department of Surgery, University of Toronto, Canada. "All patients who have been treated with VX-880 have shown improvement across all measures of glucose control, including reduction or even elimination of external insulin use."

People with T1D don't have functional cells in the pancreas to produce the body's own insulin and require daily insulin injections or pumps to survive.

The ongoing trial is now in the final part of three. In Part A, two patients were enrolled sequentially and received half the target dose of VX-880;

Patient 2 subsequently withdrew consent and discontinued participation in the trial for personal reasons, not related to an adverse event. In Part B, five patients were enrolled sequentially and received the target (full) dose. Parts A and B of the study have completed enrollment, and Part C, in which 10 patients will be enrolled concurrently and will receive the target dose, is well underway.

Here, researchers report on all patients in Parts A and B, all of whom have been followed up for more than 90 days.

Following a single infusion of VX-880, patients were monitored for safety and tolerability (assessed by adverse events and clinical laboratory assessments), fasting and stimulated C-peptide (an indicator that the patient is making insulin), multiple measures of blood glucose control, and external insulin use.

In Part A, following VX-880 infusion at half the target dose, Patient 1 achieved insulin independence after 9 months, which was sustained at the most recent visit at 24 months. This is a patient with a nearly 42-year history of living with severe T1D, who was using an average of 34 units of insulin per day prior to trial enrollment.

In Part B, following VX-880 infusion at the target (full) dose, Patient 3 achieved insulin independence after 6 months, which was sustained at the most recent 12-month visit. This patient had a 19-year history of living with T1D and needed an average of 45 units of insulin per day prior to trial enrollment. Patient 3 was started on four units of daily insulin after 15 months. Within the last few weeks, Patient 4 reached day 180 and achieved insulin independence. All patients experienced improvements in HbA1c and the amount of time their blood glucose levels were in the recommended range.

All six patients demonstrated improved glycemic control, reduction in

HbA1c and an increase in the amount of time their blood glucose levels were in the recommended range, as well as a reduction in or elimination of their external insulin needs and elimination of severe hypoglycaemia for more than 90 days.

At 90 days post-procedure, all six patients in Parts A and B responded to a mixed-meal tolerance test by triggering production of insulin, which is exactly what a functioning pancreas would do, suggesting that the injected islet cells were acting as intended.

VX-880 has been generally safe and well tolerated in all patients dosed to date. The majority of adverse events (AEs) were mild or moderate, with two non-serious AEs related to VX-880; both were elevated transaminases (liver enzymes) that occurred shortly after VX-880 infusion, were transient and resolved. There were no serious AEs related to VX-880 treatment.

Despite the promising results, the new cells are at risk from the body's own immune system, therefore, immunosuppressants are needed to help prevent rejection, which may bring their own risk.

"The future goal is to create a version of the treatment that does not require immunosuppressive therapy," says Professor Reichman. "The makers of VX-880 are working on encapsulating the cells in a device that would allow them to evade the immune system as well as genetically modifying the cells so they won't initiate an autoimmune attack."

Provided by Diabetologia