

Lisa Jarvis, Columnist

[We Need More Way to Treat Diabetes. Enter Vertex.](#)

A world without insulin shots is looking like less of a fantasy.

Encouraging data suggest a new Type 1 diabetes treatment from Vertex Pharmaceuticals Inc. could allow patients to forgo insulin. While the data are early and come with caveats, they represent a crucial step forward in a field that has long struggled to move beyond insulin delivery.

Momentum in Type 1 diabetes is starting to hint at a day where new treatments release people from the mental load of constant, careful monitoring and worrying. It's too soon to talk about a cure for the roughly 2 million kids and adults in the US with the disease — but not to start to guess what a cure might look like.

In someone without diabetes, the body carefully orchestrates insulin production to respond to even the most minute variation in blood sugar levels. Getting that exquisite balance right is a lifelong challenge for people with Type 1 diabetes, which occurs when the immune system turns on beta cells, the factories that churn out insulin.

Achieving blood sugar control comes down to a million daily decisions about whether to eat or not, whether to take insulin or not, counting each bite of food or sip of soda. Take too much insulin and people suffer from dangerously low blood sugar; too little and sugar accumulates in the blood, over time, damaging the kidneys, heart, blood vessels, nerves and eyes.

Insulin levels can also be influenced by things that have nothing to do with what you put in your body, says Sanjoy Dutta, chief scientific officer of nonprofit research-funding organization Breakthrough T1D (formerly JDRF). “Stress can change our glucose levels. Sleeplessness can change your glucose levels,” he says. Some people develop disordered eating or become depressed or anxious trying to manage their disease. Some lose their vision or require amputation. Some lose their life.

While newer technologies like insulin pumps and continuous glucose monitors have improved the long-term health of many, the opportunity remains to improve the life and outlook for someone with the disease.

That's what makes this new swath of data from Vertex so encouraging. The biotech's therapy replaces the cells the immune system destroys in Type 1 diabetes with ones derived from stem cells. That sounds simple, but it took years to figure out the right cues and signals to transform stem cells into insulin-producing islet cells.

The goal of the small study was to answer two fundamental questions, explains Felicia Pagliuca, who leads the diabetes program at Vertex. Do these stem cell-derived islet cells actually work — that is, did they engraft and then behave like the healthy cells someone is born with? And then do they work well enough to change the course of the disease?

Each beta cell is intricately connected to the bloodstream, neural networks, neighboring cells, and more. “There's a lot of cross interactions, cross signaling required in harmony to orchestrate,” Dutta says.

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(Breakthrough T1D funded some of the basic science and early companies developing this technology). To create that in a petri dish, put it in a human and hope the cells will emulate that “is a tall feat.”

So far, it seems to be working. The data are preliminary and have yet to undergo peer review, but Vertex has reported that the cells engrafted in all 12 volunteers who received full doses of its treatment. Three months out and the cells were producing insulin in every patient, all had seen improvements in their blood sugar levels, and 11 were able to reduce or eliminate their insulin intake. Three patients who were a year out from treatment had stopped taking insulin altogether.

Twelve is a small number of test subjects, but in the world of cell therapy studies, it’s meaningful — especially when the therapy seems to be working in everyone. It gives hope that this approach is viable, albeit with much more work to come.

The next questions are whether that effect will last and what being freed from insulin could mean for someone’s long-term health. Answers can only come with time and careful monitoring of the trial volunteers, but Pagliuca sees reason for optimism. Of the many types of cells in our body, islet cells are known to have an incredible lifespan.

Still, this advance comes with a big asterisk: Because these engineered islet cells started out as stem cells that came from a donor, people receiving them need to take powerful drugs to prevent their immune system from attacking the cells. That trade off — potential insulin independence to lifelong immunosuppressive therapy — means that even this therapy proves highly effective, it will likely only appeal to people struggling to keep their disease in check. (Vertex is testing the product in people experiencing severe hypoglycemia.)

The next big goal is to come up with a stem cell therapy that obviates the need for that after-care. That’s a more distant aspiration, but a few good ideas are already being studied. Vertex is working on an implantable device that encapsulates those little insulin factories, shielding them from immune attack, while also allowing for things like insulin and nutrients to waft in and out.

And the ultimate goal would be to use Crispr gene-editing tools to modify those stem cell-derived islet cells to be invisible to the immune system. That’ll be complicated — they need to not just skirt the immune cells’ surveillance for foreign invaders, but also get around the autoantibodies that cause islet cells to be attacked in Type 1 diabetes.

While there’s a lot of work to be done to get there (and it’s not too early to worry about the [cost of a one-time transformative treatment](#)), it’s also incredible to see the rapid transformation in the field as years of basic research coincide with advances in technology.

For too long, talk of a cure for Type 1 diabetes felt like science fiction. As filmmaker Lisa Hepner, who has Type 1 diabetes, says in the opening of her 2022 documentary *The Human Trial*, “It’s become a joke in the community that the cure is always 5 years away.”

Five years is still a stretch, but at least there’s finally a reasonable roadmap to get there. That’s something to celebrate.

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