San Diego company's stem cells treat diabetes in mice

by Terri Somers

SAN DIEGO - A tiny San Diego biotechnology company has coaxed human embryonic stem cells to evolve into cells that produce insulin to control blood sugar when injected into mice with diabetes, according to a study published online Wednesday in the journal Nature Biotechnology.

The study shows that Novocell, a privately held company with 37 employees, is moving one step closer to its goal of creating a human embryonic stem cell therapy for diabetes.

"I think this validates human embryonic stem cells as something that can be very valuable and used to produce islet-like cells for diabetes treatment, a very specific, very differentiated cell type that would be very difficult to get otherwise," said Emmanuel Baetge, Novocell's chief scientist and the senior author of the study.

Novocell is one of a handful of companies delving into the controversial world of human embryonic stem cells in the hopes of developing therapies to treat a number of diseases. Embryonic stem cells, which exist just days after fertilization, evolve into the more than 200 different cell types in the body. But the field is controversial because it requires the destruction of a days-old human embryo.

Novocell uses stem cells derived from embryos left over from the in-vitro fertilization process and no longer wanted by people attempting to have children.

In November 2001, when Baetge joined the company, it had not made even one stem cell line from embryonic stem cells. So Baetge and his team started from scratch.

In 2005, the team published a study showing they could coax human embryonic stem cells to become endoderm cells, a lineage of cells that eventually turn into islet cells and other cell types.

In 2006, the team showed it could develop these cells further into fetal endocrine cells.

But then they had trouble trying to get them to evolve further into the islet cells, Baetge said. So, they backed up the process to one step before the development of endocrine cells, he said.

They took those cells, the progenitors of the endocrine cells, and injected them into mice and eventually got the insulin-producing cells.

There is something in the living organism that signals these cells to develop into islet cells, Baetge said.

The cells were injected into 105 mice. Some were chemically induced to have diabetes and others were not. Ninety-two percent of the animals made functional islet cells, a very high percentage of success, Baetge said.

"We've finally gotten to a point where we can say that we can make this function well and can now work on how this can be turned into a product," Baetge said.

At least 170 million people globally have diabetes, with numbers expected to double by 2030. Five percent to 10 percent have Type 1 diabetes, caused when the immune system mistakenly destroys the insulin-producing cells in the pancreas.

In both Types 1 and 2 diabetes, the body cannot use insulin properly or make enough of it to regulate glucose in the blood. Patients must monitor their blood sugar and often must take insulin as needed.

A Canadian team is having some success transplanting purified islet cells harvested from cadavers into diabetics. But that supply of islet cells is limited.

Novocell's plan is to use human embryonic stem cells to create a plentiful and ongoing pool of cells that can be created under standardized processes and then injected into humans. The company's technology involves a process of encapsulating they cells in a polymer that would make them invisible to the immune system, and therefore avoid the need for recipients to take immune-suppression drugs to prevent rejection of the cells.

The encapsulation procedure was not used on the cells injected into the mice for this study. But that process is now under way in further mice studies at the company, Baetge said.

Meanwhile, the company is also working on a process of purifying the cells before transplantation, he said.

Novocell then plans to begin meeting with the Food and Drug Administration to determine its pathway

toward approval to begin clinical trials using its cells, he said.

"Obviously that kind of research is expensive and requires probably more financial resources than Novocell's current shareholder base has the ability to provide," said Chief Executive Alan Lewis.

In the next 12 months, Novocell is hoping to find a corporate partner with those resources as well as experience in manufacturing and commercialization to help move its product closer to market, Lewis said.

San Diego company's stem cells treat diabetes in mice by Terri Somers