

Cell Therapy for Type 1 Diabetes

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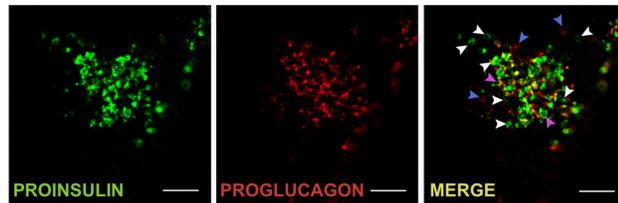
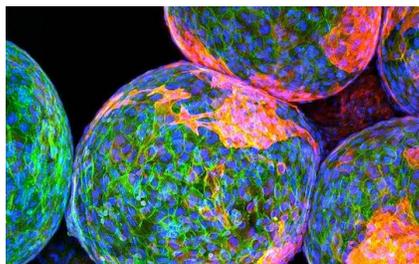
Intellectual Property:

Pending Applications:
- PCT/US2019/047735

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Human pluripotent stem cell-derived beta cells

Clinical Need:

Type 1 Diabetes (T1D) is an autoimmune disease in which beta cells are mistakenly destroyed by the body's immune system. The cause is not fully known and there currently is no cure. T1D patients are dependent on injected insulin to survive. Less than one-third of people with T1D in the U.S. are consistently achieving target blood-glucose control levels.

Value Proposition:

To produce functional beta cells capable of insulin production for T1D patients using a novel process that is more efficient, cost-effective and consistent than current stem cell therapy methods in development, which rely on growth factors and/or animal products.

Technology Solution:

Stem cell therapy is an emerging biotechnology that offers potential therapeutic solutions in diseases where current pharmacological and surgical treatments are not effective. Human pluripotent stem cell (HPSC)-derived beta cells could be used as a cure for treating T1D. Penn State researchers in the Department of Biomedical Engineering have developed a process for converting HPSCs to pancreatic progenitor cells, which are precursors to functional pancreatic beta cells. This novel method uses small molecules, rather than growth factors or animal products, resulting in a cost-effective and more consistent process. Preliminary in vitro results with various stem cell lines have shown beta cell differentiation efficiencies near 90% using this method and resulting beta cells are capable of producing insulin in response to fluctuating glucose levels.

Market Opportunity:

The economic impact of T1D is staggering – in the U.S., more than \$16 billion is spent annually on direct medical costs alone. The prevalence of T1D is increasing year-over-year in all major markets. Virtually all marketed drugs to treat T1D are insulins; however, there is a wide array of drug types in the pipeline, including several stem cell therapy candidates, most of which are in pre-clinical development. Early experimental success will create significant value and increase the possibility of a cure for T1D.

Path Forward:

Currently, differentiated cells are being tested in T1D animal models, including mice and monkeys; successful results will build the foundation for human clinical trials.



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