

Curing Type 1 Diabetes

Recent Research Progress

Cure

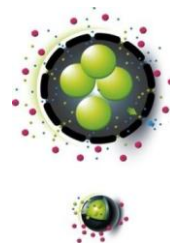
type 1 diabetes by replacing or regenerating beta cells, and halting the autoimmune process.

JDRF envisions the day when a cure will be available to people with T1D. We define a cure in patient terms as safe, durable, convenient, widely available, and cost-effective normal glucose control with insulin independence. To achieve this goal, JDRF is working to develop two distinct therapies. One is a replacement therapy - implanting encapsulated glucose-responsive, insulin-secreting cells. The other is regenerating a person's beta cells coupled with a therapy to block the autoimmune attack. Over the past roughly 5 to 7 years significant progress has been made towards achieving these goals. Read more to learn about the amazing progress of JDRF's priority programs to cure T1D.

JDRF Beta Cell Encapsulation Program – Insulin Independence via Cell Implantation

Why This Program Is Important

- Islet and pancreas transplantation proved T1D can be cured by cell replacement but it is not widely available due to limited human islet availability and the need for life-long immunosuppression
- JDRF's program is generating abundant insulin-producing cell sources and novel immune protective encapsulation materials to avoid immunosuppression



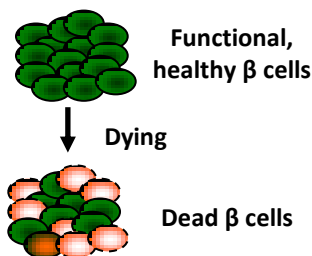
2005 Encapsulation Status

- Failure of early encapsulation attempts due to inadequate materials
- No human stem cell lines suitable for human use and limited insight on how to produce beta cells from human stem cells
- Early testing of pig islets as potential cell source for transplantation (for use with immunosuppression therapy)

Key Advances Since 2005

- Long-term animal studies demonstrate safety and effectiveness of pilot encapsulated cells
- Human stem cell lines established and embryonic stem (ES) cells differentiated into pancreatic progenitor cells
- LCT pig islet encapsulation completes 1st human study. Two companies start human stem cell encapsulation programs (ViaCyte & Betalogs)

JDRF Beta Cell Biomarkers Program – Tools to Accelerate Progress Towards A Cure



Why This Program Is Important

- Monitoring beta cells is difficult given their diffuse locations in the pancreas
- Detecting beta cell stress and death will allow more specific staging of people with the disease and targeting of new therapies to subgroups
- Detecting changes in beta cell numbers will more quickly reveal benefits of new therapies in clinical trials, speeding development.

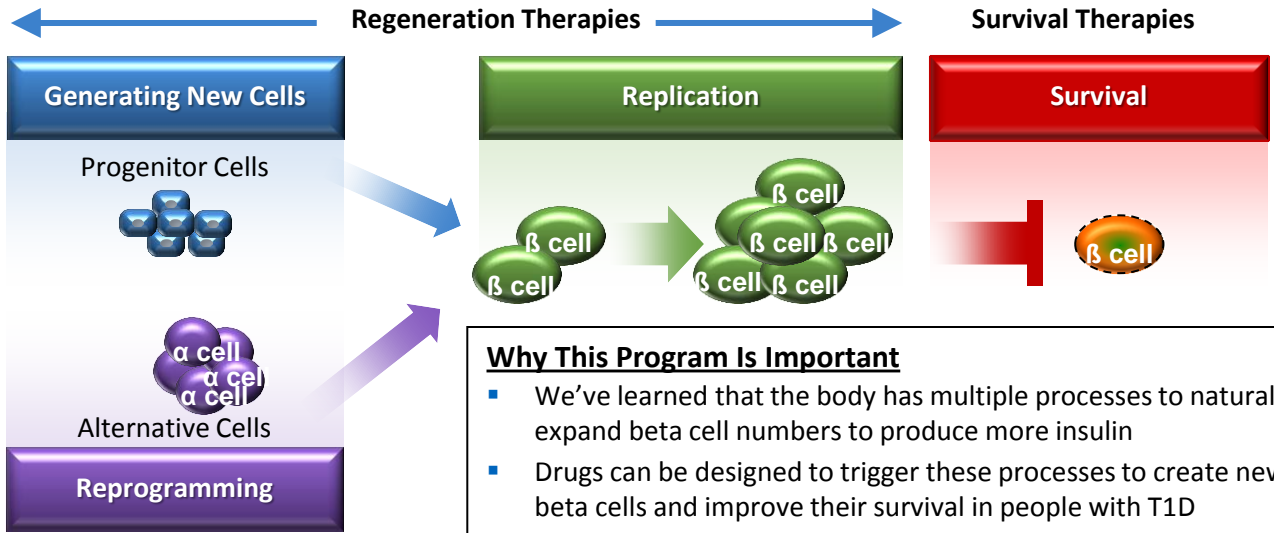
2005 Biomarkers Status

- Beta cell status can not be directly measured – only inferred indirectly from loss of blood glucose control and measurement of residual beta cell function (C-peptide)

Key Advances Since 2005

- Discovered several markers of beta cell death in lab studies – potential blood test and licensed to a company to develop further
- Discovered potential beta cell imaging concepts and tested in animals

JDRF Beta Cell Regeneration and Survival Program – Insulin Independence via Restored Native Beta Cells



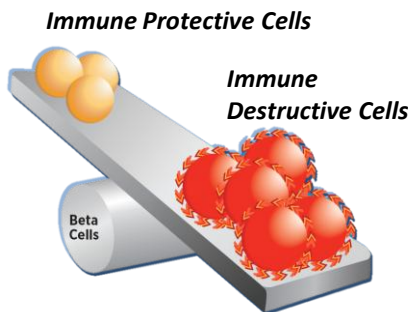
2005 Regeneration Status

- Beta cell replication concept still only a theoretical possibility
- Cell reprogramming to beta cells concept did not exist
- No beta cell survival strategies existed

Key Advances Since 2005

- Replication pathways identified & successfully tested in animals; Industry partner interest
- Discovered that alpha and other cell types can be converted to beta cells; Industry partners
- Discovered the role of beta cell stress in dying beta cells and launched first clinical study to improve beta cell survival and/or replication

JDRF Antigen-Specific Immune Therapies Program – Restoring Normal Balance to the Immune System



Why This Program Is Important

- T1D is an autoimmune disease so fixing the misdirected immune system is key to a cure
- Broad immunosuppression therapy shown not adequate alone for most people with T1D
- Targeted immune therapies will focus on the specific problems associated with the immune system in people with T1D

2005 Immune Therapies Status

- Clinical focus on therapies providing a broad suppression of the immune system (anti-CD3)
- Lacked an understanding of the difference between the healthy and T1D immune system

Key Advances Since 2005

- Broad immunosuppression therapy shown to preserve beta cell function in recent onset setting - but only transiently
- Discovered the role of specific components in balancing the immune system. Animal studies proved the potential for antigen-specific tolerance-inducing therapies
- Multiple industry partners launch unique R&D programs towards antigen-specific therapies