Generation Of Human Beta Cell Equivalents From Pluripotent Stem Cells In Vitro

Tech ID: 25689 / UC Case 2016-019-0

INVENTION NOVELTY

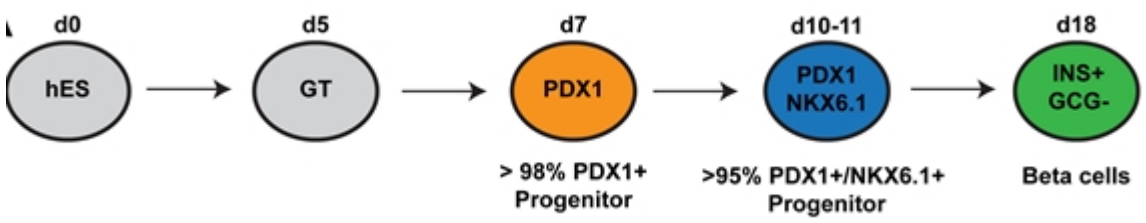
This invention describes a robust method to generate functional human beta cell equivalents from pluripotent stem cells in vitro for wide applications in basic research, drug and toxicology screens and as a diabetes cell therapy.

VALUE PROPOSITION

Islet cell transplantation holds great promise as a treatment or potential cure for patients suffering from diabetes. Yet until now, how to generate an abundant and reliable source of insulin-producing beta cells has remained an unsolved problem. Human cadaveric islets is one existing source of beta cells; however, their utility has been limited by a severe shortage of cadaveric organ donors in addition to the inherent variability between donors and islet preparations. Generating beta cells *in vitro* would get around these limitations of availability and reproducibility. However, current protocols do not accurately recapitulate the sequence of embryonic signals required for proper specification of beta cell precursors resulting in the generation of a mixed population of pancreatic progenitors and the development of unwanted cells. Researchers at UCSF have developed a simplified, fast, efficient and scalable system for generating functional human beta-like cells without unwanted, off-target differentiation by accurately reproducing the key steps of human pancreas development *in vitro*.

TECHNOLOGY DESCRIPTION

This invention describes how to generate insulin-producing beta cell equivalents from human pluripotent stem cells. The protocol has optimized the timing and components of a cocktail of known and novel factors. The cells remain functional after transplantation into mouse models and after short engraftment period, can reduce the blood glucose levels of diabetic mice. Additionally, this invention has improved the cells' survival upon transplantation by altering the differentiation conditions. This protocol is highly efficient generating up to 75% of functional pancreatic beta-like cells within 3 weeks.



LOOKING FOR PARTNERS

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OTHER INFORMATION

KEYWORDS

Diabetes, Type 1 diabetes, Beta cell, Insulin, Islet, Islet transplantation, Pancreas, Stem cell, Differentiation, Human pluripotent stem cells

CATEGORIZED AS

- **Medical**
  - Disease: Metabolic/Endocrinology
  - Stem Cell
- **Research Tools**
  - Screening Assays

RELATED CASES

2016-019-0

To develop and commercialize this technology not only as a system for generating insulin producing cells for transplantation for the treatment of diabetes, but also a:

- Screening tool for beta cell drug and toxicology studies

- Valuable basic research tool for studying beta cell biology *in vitro*

- Source of cells to test encapsulation devices

STAGE OF DEVELOPMENT

Pre-Clinical

RELATED MATERIALS

- Russ HA, Parent AV, Ringler JJ, Hennings TG, Nair GG, Shveygert M, Guo T, Puri S, Haataja L, Cirulli V, Blelloch R, Szot GL, Arvan P, Hebrok M. Controlled induction of human pancreatic progenitors produces functional beta-like cells in vitro. EMBO J. 2015 Jul 2;34(13):1759-72.
- Zhu S, Russ HA, Wang X, Zhang M, Ma T, Xu T, Tang S, Hebrok M, Ding S. Human pancreatic beta-like cells converted from fibroblasts. Nat Commun. 2016 Jan 6;7:10080.

DATA AVAILABILITY

Protocol and animal data under CDA / NDA

PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Issued Patent	11,332,716	05/17/2022	2016-019
European Patent Office	Published Application	3328404	06/06/2018	2016-019

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