Module 2 - Case Study: Methods In Neurobiology **09/10/2021**

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Biological Problem: Over 32.4 million people suffer from Diabetes in the United States of America [1]. Diabetes is caused when cells in the human body are unable to use the sugar present in the blood, this happens because the body of a diabetic patient is unable to produce enough or any Insulin. Insulin is a peptide hormone which is produced in the pancreas by a cluster of cells called the islets. In a diabetic patient either the immune system attacks the Islets making insulin production difficult or ceasing it completely, or islets cannot produce enough insulin to overcome insulin resistance [5].

Aim: Induced Pluripotent Stem cells (IPSCs) are cells that have multiple potential. Using IPSCs model of cell generation I will promote regeneration of Insulin in the body. We will achieve this by stably integrating of *PDX1* and *NKX6.1* the two transcription factors that are indispensable precursors of functional pancreatic beta cells [4].

Research Model and Plan:

1. To promote generation of insulin in a regular manner I will have to first collect the patients somatic cells from the affected organ i.e.the pancreas in this case.
2. The process of generating IPSCs means reprogramming of somatic cells. Which will involve introduction of genes such as the Oct4, Sax2, Klf4 and c-Myc in adult somatic cells which will lead to the generation of Induced pluripotent stem cells.

   Note: Reprogramming of somatic cells will dedifferentiate adult somatic cells to produce patient-specific stem cells [3].

3. Once my stem cells have been cultured, I'll have to introduce *PDX1* and *NKX6* transcriptional factors stably in the IPS cell line.
4. Firstly, to achieve an enhanced population of *PDX1* and *NKX6* I'll manipulate in-vitro conditions during differentiation by dissociating densely formed endodermal cells and re-plating them at different densities.
5. Now the final step for me would be to incorporate *PDX1* and *NKX6* in my stem cells. I'll achieve that by performing Ex vivo gene therapy, I'll use vectors to introduce my desired modification in the cells and reintroduce it back into the patient's body.
6. To determine the progress and success rate of my procedure I'll perform quantitative gene expression measurements, immunocytochemical staining and functionals assays[7].
7. Ex vivo method has already proved its efficacy in treating major neurological diseases such as Alzheimers, Parkinson and Huntington disease. I think with prior knowledge and success on this technique and IPSCs, this model would be able to gain success in treating patients living with diabetes.

References:

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