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IDEAS TO LIFE RANGAHAUA KIA WHAI HUA

Direct Cell Reprogramming

A Novel Cell Replacement Therapy for Huntington's Disease

A novel approach for directly reprogramming human skin cells into brain stem cells.

Problem

Huntington's disease (HD) is a rare, autosomal dominant genetic disorder caused by a mutation in the gene encoding the huntingtin protein. HD is characterised by a loss of neuronal cells, mainly in the striatum and the cerebral cortex, resulting in cognitive, behavioural and motor decline. Ultimately fatal, there is currently no cure or disease-modifying treatment available for this disease. A range of different types of cells have been investigated for HD cell replacement therapy, including human foetal cells and pluripotent stem cells. However, these face challenges relating to ethics, cell rejection, growth of tumours and genetic mutagenesis.

Technology

We have developed a proprietary method for generating human brain stem cells (human induced neural precursors) directly from adult human skin cells without the need to generate a pluripotent intermediate state (Fig 1). This novel technology can be used to generate human striatal stem cells which can be differentiated to striatal neurons in vitro.

Our direct cell reprogramming technology can generate clinically safe, ethically acceptable cells for cell replacement therapy and overcomes many of the problems associated with other cell types used for cell replacement therapy.

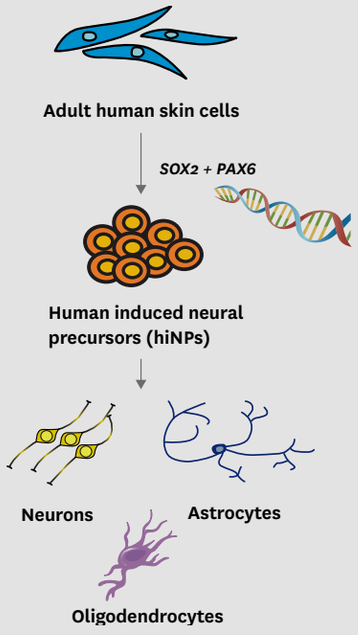

Figure 1:

Schematic demonstrating how adult human skin cells are directly reprogrammed to human induced neural precursor cells (hiNPs) using the genes SOX2 and PAX6. The hiNPs can then make the three cell types found in the brain: neurons, astrocytes, and oligodendrocytes.

Key Advantage

Direct cell reprogramming overcomes many of the problems associated with other cell types used for cell replacement therapy, including:

- Cells are ethically and easily sourced (patients' own skin cells).
- Cells are not pluripotent and therefore have no tumour potential (compared to pluripotent stem cells).
- Through the use of chemically modified RNA to reprogram skin cells, there is no risk of genome

integration and mutagenesis caused by DNA integrating technologies such as viral vectors.

- Cells are expandable, unlike directly induced neurons, allowing greater experimental flexibility with a limited skin cell source.
- The time required to generate our cells is greatly reduced compared to pluripotent stem cells, allowing for the generation of brain stem cells and subsequently a wide range of specific types of mature brain cells within 2-3 months.

Applications

We are currently focused on development of cells for treating Huntington's disease, but our technology can be expanded to:

- Parkinson's disease
- Multiple sclerosis
- Spinal cord Injury

UniServices by the numbers

Total external research funding:
$261.3M
(35% increase over 2020)

45
companies started in the past five years

$1.25BN
Total market capitalisation of companies formed

$73.5M
Net asset value of the University of Auckland Inventors' Fund

17,335 Covid-19 vaccinators trained by the Immunisation Advisory Centre in 2021

1,700
New Zealand teachers reskilled and upskilled through Tui Tuia | Learning Circle professional learning and development in 2021

3,000
clinical staff at 22 DHBs trained through teamwork-based acute care simulations designed by NetworkZ in the past five years

14,391 times that child and youth mental health workers attended Whāraurau e-modules, trainings and workshops in 2021

UniServices

UniServices is a not-for-profit company of the University of Auckland that champions research and ideas with the power to change the world. From seeking out and bringing together partners in academic institutions, industry and government to build new knowledge and solutions through research; to whakatupu (nurturing) and commercialising the ideas and intellectual property that arise from the University of Auckland's great minds, we act as the kaihono (those who join and link people to people, and people to projects) firmly entrenched in the ecosystem that bridges the world of academia with business, government and our communities.

University of Auckland

Waipapa Taumata Rau | The University of Auckland is New Zealand's largest and leading university. The name Waipapa Taumata Rau, gifted to the University by Ngāti Whātua Ōrākei, refers to the 'place of many peaks' – places to strive for, ascend to and succeed. We also rank in the top 10 globally for sustainable development impact. The University supports economic growth locally and nationally through innovation and entrepreneurship, creating quality jobs and high-value businesses, and producing graduates that contribute to our economy and society for the benefit of all.

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