Translational Medicine
ISSN: 2161-1025
OPEN ACCESS Freely available online
Opinion Article

Strategies in Neurodegenerative Disease Modelling Using Induced Pluripotent Stem Cells

Lucas Ferreira*

Department of Neuroscience, University of São Paulo, São Paulo, Brazil

DESCRIPTION

Neurodegenerative diseases such as alzheimer's disease, parkinson's disease, and amyotrophic lateral sclerosis present significant challenges due to their progressive nature and limited therapeutic options. A major obstacle in developing effective treatments has been the difficulty in studying disease mechanisms directly in human neural tissue. Translational medicine has addressed this limitation through the use of induced pluripotent stem cells, which allow researchers to generate patient-specific neural cells in laboratory settings and investigate disease processes in a controlled environment.

Induced pluripotent stem cells are generated by reprogramming adult somatic cells, such as skin fibroblasts or blood cells, into a pluripotent state. These cells can then differentiate into various cell types, including neurons and glial cells. This capability provides a unique opportunity to study neurodegenerative diseases using cells that retain the genetic background of the patient. As a result, researchers can observe disease-related changes at the cellular level and explore how genetic variations influence disease progression.

One of the key advantages of using induced pluripotent stem cells is their ability to model early disease stages. Neurodegenerative diseases often develop over many years before clinical symptoms appear. Traditional models, including animal studies, may not fully replicate human disease progression. In contrast, patient-derived neural cells can exhibit early pathological features, such as protein aggregation or altered cellular metabolism, allowing researchers to investigate the initial events that contribute to disease onset.

In Alzheimer's disease research, induced pluripotent stem cell-derived neurons have been used to study the accumulation of amyloid-beta and tau proteins. These models have revealed abnormalities in protein processing and cellular transport mechanisms. Similarly, in Parkinson's disease, dopaminergic neurons derived from patient cells have demonstrated mitochondrial dysfunction and increased oxidative stress. These findings provide insights into cellular vulnerabilities that may be targeted by therapeutic interventions.

Another important application of induced pluripotent stem cells is in drug screening. Traditional drug development relies heavily on animal models and immortalized cell lines, which may not accurately predict human responses. Patient-specific neural cells offer a more relevant platform for testing potential therapies. Researchers can evaluate the effects of compounds on disease-related cellular changes, identifying candidates that show beneficial effects. This approach can improve the efficiency of drug development by prioritizing compounds with higher likelihood of success in clinical trials.

Despite their potential, the use of induced pluripotent stem cells in translational research presents several challenges. One issue is the variability in differentiation protocols, which can lead to differences in cell characteristics between laboratories. Standardization of methods is necessary to ensure reproducibility and reliability of results. Additionally, generating mature neural cells that accurately reflect the aging process remains difficult, as many neurodegenerative diseases are associated with age-related changes.

Collaboration across disciplines is essential for advancing the application of induced pluripotent stem cells in neurodegenerative disease research. Neuroscientists, geneticists, bioengineers, and clinicians must work together to refine models, interpret data, and translate findings into clinical applications. Partnerships with pharmaceutical companies also play a role in moving discoveries from the laboratory to clinical trials.

CONCLUSION

Induced pluripotent stem cells have become a valuable tool in the study of neurodegenerative diseases within translational medicine. By enabling the generation of patient-specific neural cells, they provide insights into disease mechanisms, support drug discovery, and facilitate the development of targeted therapies. Continued efforts to address current challenges and refine methodologies will be essential for maximizing their impact on improving outcomes for individuals affected by these conditions.

Correspondence to: Lucas Ferreira, Department of Neuroscience, University of São Paulo, São Paulo, Brazil, E-mail: lucas.ferreira@uspneuro.br

Received: 18-Aug-2025, Manuscript No. TMCR-25-41452; **Editor assigned:** 20-Aug-2025, PreQC No. TMCR-25-41452 (PQ); **Reviewed:** 03-Sep-2025, QC No. TMCR-25-41452; **Revised:** 10-Sep-2025, Manuscript No. TMCR-25-41452 (R); **Published:** 17-Sep-2025, DOI: 10.35248/2161-1025.25.15.362

Citation: Ferreira L (2025). Strategies in Neurodegenerative Disease Modelling Using Induced Pluripotent Stem Cells. Trans Med.15:362.