ALZHEIMER'S DISEASE IN A DISH: PROMISES AND CHALLENGES OF HUMAN STEM CELL MODELS.

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Public Summary:
This review article highlights several studies that were published in 2011 and 2012 regarding the use of human stem cells to study Alzheimer's disease. Alzheimer's disease (AD) is the most common form of dementia and, despite many years of research, there is no cure. Human stem cells represent a new technology being used to study AD with the hope that new, effective treatments can be developed. Human stem cells can be differentiated into all cell types, including neurons, which are primarily affected in AD. Several studies have demonstrated that human stem cells made from AD patients are able to effectively differentiate into neurons and recapitulate cellular characteristics of AD in the laboratory. When these cells are treated with small molecules, these characteristics are altered, which shows the usefulness of this system for potentially screening new drugs. This review also discusses the current challenges facing this field of research, including examining other cells from the central nervous system, such as astrocytes, modifying stem cells to remove disease-causing genes, and analyzing stem cells from many different individuals with sporadic disease.

Scientific Abstract:
Human pluripotent stem cells can differentiate into disease-relevant cell types, which capture the unique genome of an individual patient and provide insight into pathological mechanisms of human disease. Recently, human stem cell models for Alzheimer's disease (AD), the most common neurodegenerative dementia, have been described. Stem cell-derived neurons from patients with familial and sporadic AD and Down's syndrome recapitulate human disease phenotypes such as amyloid beta peptide production, hyperphosphorylation of tau protein, and endosomal abnormalities. Treatment of human neurons with small molecules can modulate these phenotypes, demonstrating the utility of this system for drug development and screening. This review will highlight the current AD stem cell models and discuss the remaining challenges and potential future directions of this field.