

The Human Model: Changing Focus on Autism Research.

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Public Summary:

This is a critical review on the use of stem cells models to re-create "mini-brains" and neuronal networks derived from people suffering with autism spectrum disorders. I propose a novel paradigm for autism research, with the potential to accelerate drug discovery and biomarkers.

Scientific Abstract:

The lack of live human brain cells for research has slowed progress toward understanding the mechanisms underlying autism spectrum disorders. A human model using reprogrammed patient somatic cells offers an attractive alternative, as it captures a patient's genome in relevant cell types. Despite the current limitations, the disease-in-a-dish approach allows for progressive time course analyses of target cells, offering a unique opportunity to investigate the cellular and molecular alterations before symptomatic onset. Understanding the current drawbacks of this model is essential for the correct data interpretation and extrapolation of conclusions applicable to the human brain. Innovative strategies for collecting biological material and clinical information from large patient cohorts are important for increasing the statistical power that will allow for the extraction of information from the noise resulting from the variability introduced by reprogramming and differentiation methods. Working with large patient cohorts is also important for understanding how brain cells derived from diverse human genetic backgrounds respond to specific drugs, creating the possibility of personalized medicine for autism spectrum disorders.

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