Reprogramming technology differentiates specialized cells of a specific cell type to be converted to another cell type with different functions, either through the production of induced pluripotent stem cells (iPSCs) or through direct reprogramming. This technology has the potential to generate models of diseases, allowing researchers to study disease mechanisms in an in vitro setting.

Understanding the applications of iPSC-derived neural cells

- **Neurons and hippocampal iPSCs**
- **Motor neurons**
- **Forebrain neurons**

**Phenotype**

- EXPAND NPCs with STEMdiff™ Neural Progenitor Medium (Catalog #05833)
- SMN1
- FXN

More representative of the brain's extracellular environment

iPSC-derived progeny

- ATXN3

**Gene Expression**

- **RefSeq**
- **HGNC**
- **LocusLink**

**Gene Regulation**

- epigenetic changes influence gene expression

**Cell Culture**

- iPSC-induced neurons versus other non-human primates

**Clinical Trials**

- iPSC-derived neurons for some neurological diseases

**Cell-Reprogramming Technology**

- Can be adapted to study related pathways and cellular phenotypes in reproducible and scalable bioassays

**Human iPSC-derived Models of Neurodegenerative and Psychiatric Disorders**

<table>
<thead>
<tr>
<th>Disease</th>
<th>Mutated genes</th>
<th>iPSC-derived phenotype</th>
</tr>
</thead>
<tbody>
<tr>
<td>Alzheimer disease</td>
<td>APP, PS1, PS2</td>
<td>Neuronal cell death, amyloid plaques, hyperphosphorylated tau</td>
</tr>
<tr>
<td>Parkinson disease</td>
<td>SNCA, LRRK2</td>
<td>Dopaminergic neuron loss, Lewy body formation</td>
</tr>
<tr>
<td>Huntington disease</td>
<td>ITT</td>
<td>Neuron loss, atrophy, striatal atrophy</td>
</tr>
<tr>
<td>Cerebellar ataxia</td>
<td>ATXN3, ATXN7</td>
<td>Neuron loss, atrophy, cerebellar atrophy</td>
</tr>
</tbody>
</table>

**Challenges and future directions**

- Modelling polygenic and multifactorial CNS disorders
- Integrating methods to differentiate iPSCs into the relevant cell types involved in neurological disease with reproducible and scalable platforms

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**References**


**High-throughput phenotypic screens using iPSC-derived cells**

- Based on a novel platform for data analysis that allows the generation of disease models in different species

**Identiﬁcation of quantifiable disease biomarkers in iPSCs derived from patients and controls**

**Ongoing trials**

- Based on human iPSC-derived cell models