Huntington's Disease

Neurological | Umbilical Cord Blood

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Huntington's disease is a progressive brain disorder resulting in the slow loss of brain cells. Mutation results in a longer than normal protein, which accumulates in the brain cells, specifically those controlling motor function. Cognition and mental disease issues may also be involved. Onset usually occurs in middle age and reduces expected lifespan, with an average time from disease onset to death of 15 to 25 years. However, the earlier the onset of disease, the shorter the life expectancy. Death is usually due to infection, injuries relating to a fall or other complications.

Between 6,500 and 8,000 people in the UK are affected, costing £12 million per year for treatment and support. It appears to be more common in European ancestry populations.

Cell Line

A stem cell line with the genetic defect has been created to help model the disease and test possible treatments. Genes that combat the effects of the disease could be introduced to cell lines to stimulate production of brain cells, or stem cells may be introduced to repair the damaged brain cells in situ.

Animal Studies

Present treatments slow the disease progression, and some symptomatic treatments are available. Stem cell-based therapies are currently in animal model phase only. This includes formation of GABA neurons that can be implanted into the brain to effect a restoration of function. This has been successfully undertaken in mice, but the GABA neurons were created from embryonic stem cells, which are ethically difficult.

Future Work

Future work may include transplantation of specific cell types such as neurons into the damaged area. All of these are possible avenues that are being investigated. It is anticipated that cell-based therapies may be available in 5 to 15 years.

Summary

A stem cell-based treatment for Huntington's disease is many years away, yet current research aims to use stem cell technologies to understand and work on gene therapy and symptomatic treatment.

References

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