PC12 Cells Engineered for Screening Drugs to Treat Huntington's Disease

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SUMMARY

UCLA researchers have developed a cell-based screening assay to identify chemical compounds that protect against Huntington's Disease-mediated cell death.

BACKGROUND

Huntington's disease (HD) is characterized by progressive deficits in motor control and cognitive deterioration. It is caused by an increase in CAG nucleotide repeats in the Huntingtin gene, resulting in an expansion of a polyglutamine region in the encoded Huntingtin protein (Htt). This mutant Htt protein is highly toxic to neurons and results in substantial cell death in the brain, but its mechanism of action is unknown. There is currently no treatment to prevent or palliate the progress of HD. Identification of clinically effective drugs that would decrease symptoms or increase survival would represent a major advance.

INNOVATION

Researchers at UCLA have developed a cell-based screen to test chemical compounds for their ability to protect against Huntington's Disease (HD)-related cell death. The researchers have engineered neuronal cell lines (PC12/pBWN:Htt ex1(Q103)-EGFP cells and PC12/pBWN:Htt ex1(Q25)-EGFP) that expresses mutant Huntingtin protein (Htt) in an inducible manner. These cells exhibit rapid and extensive cell death after induction of mutant Htt. This assay provides an important tool for the discovery of drugs to treat HD and to increase our understanding of the pathogenic mechanisms that underlie disease progression.

APPLICATIONS

- Screening drugs for the treatment of Huntington's Disease (HD).
- Studying the cell biological mechanisms responsible for the pathogenesis of HD.
- Assay system can be modified to screen for drugs to treat other neurodegenerative diseases.

ADVANTAGES

- High-throughput design permits screening of drugs in 96-well microplate format.
- Inducible system allows for rapid expression of mutant Huntingtin protein (Htt) and cell death within 48 hours.
- Utilizes a relatively hypothesis-independent strategy for drug discovery

STATE OF DEVELOPMENT

The researchers have tested and validated the system for its ability to identify compounds that protect against mutant Htt-mediated cell death.

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OTHER INFORMATION

KEYWORDS

Drug Discovery, Cell Assay, CNS

CATEGORIZED AS

- **Medical**
  - Screening
- **Research Tools**
  - Screening Assays

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