Suppression of Proteotoxicity for the Treatment of Neurodegenerative Diseases

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OTHER INFORMATION

KEYWORDS

ER stress, protein, retinal, retinal degradation, alzheimer, Parkinson, dementia, ERAD, protein misfolding, proteotoxicity, cellular degeneration, Rhodopsin, photoreceptor, vision

CATEGORIZED AS

- **Biotechnology**
  - Health
- **Medical**
  - Disease: Ophthalmology and Optometry
  - Other
  - Therapeutics

RELATED CASES

2022-758-0

BACKGROUND

The accumulation of misfolded proteins can activate endoplasmic reticulum (ER) stress responses and cellular degeneration. For example, the most common cause of retinal degeneration in humans is a mutation in the gene that encodes rhodopsin, which causes the protein to fold improperly. Rhodopsin is a membrane protein that is essential for phototransduction in the visual system of all animals. Misfolded rhodopsin accumulates over time in photoreceptor cells, causing chronic proteotoxicity and ER stress that cannot be mitigated by the cell's homeostasis systems, which ultimately leads to photoreceptor cell death and vision loss. Preventing the accumulation of the misfolded rhodopsin and ER stress would have a direct and significant impact on treating retinal degradation and other neurodegenerative diseases in which protein misfolding leads to chronic proteotoxicity.

DESCRIPTION

Researchers at University of California, Santa Barbara have discovered that the overexpression of a specific protein turnover-enhancing factor is sufficient to degrade misfolded proteins and alleviate ER stress. This factor stimulates a cellular misfolded protein disposal mechanism known as ER associated degradation (ERAD), which ameliorates ER stress. This discovery underscores the potential applicability of a specific gene-therapy-based approach for treatment for retinitis pigmentosa, an inherited disease caused by mutations in rhodopsin. The findings also apply to therapeutic intervention in other protein misfolding diseases or in pathology characterized by insufficient ERAD, including neurodegenerative diseases such as Alzheimer's, Parkinson's, and frontotemporal dementia. As such, this invention has multiple applications for understanding and treating pathologies caused by protein misfolding, chronic proteotoxicity and ER stress in neurodegeneration and in normal aging.

ADVANTAGES

- Suppresses cell death induced by unrelenting ER stress caused by chronic proteotoxicity due to misfolded rhodopsin
- Applicable to multiple areas of therapeutic interest in protein misfolding disease

APPLICATIONS

- Therapeutics
- Retinal degradation
- Neurodegenerative diseases (Alzheimer's, Parkinson's, dementia)

PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Published Application	20250000945	01/02/2025	2022-758

RELATED MATERIALS

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- Stimulating Phagocytosis Of Cancer Cells By Activating Genes In Macrophages
- Methods of Treating Lymphoma with a Phagocyte Having a Chimeric Antigen Receptor

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