Inhibition Of Lipofuscin Aggregation By Molecular Tweezers

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SUMMARY

UCLA researchers in the Departments of Neurology and Molecular Therapy & Medical Genetics have developed a novel approach toward broad inhibition of lipofuscin aggregation.

BACKGROUND

Lipofuscin is an age-related pigment compound that can be found throughout the body and its aggregation is associated with conditions ranging from macular degeneration to Alzheimer's disease. Excessive accumulation of lipofuscin is also associated with a group of neurodegenerative disorders known as neuronal ceroid lipofuscinosis (NCL). There are a number of treatments for NCL, including an FDA-approved enzyme replacement therapy, and gene therapy and small molecule approaches in clinical trials. However, despite the widespread association of lipofuscin aggregation with human disease, there are no general therapeutic approaches which inhibit its aggregation.

INNOVATION

Professor Bitan and coworkers have demonstrated that the molecular tweezer CLR01 may be used to inhibit lipofuscin aggregation. CLR01 is a large organic compound with an open cavity that is able to bind and sequester guest molecules and has been shown to be non-toxic in multiple cell culture and animal tests. In human retinal pigment cells, treatment with CLR01 inhibits the formation of lipofuscin aggregates when administered pre- or post- lipofuscin addition.

APPLICATIONS

- Treatment of neurodegenerative disorders (Alzheimer's, Parkinson's, amyotrophic lateral sclerosis)
- Treatment ofneuronal ceroid lipofuscinosis
- Treatment of lipid myopathy and centronuclear myopathy
- Treatment of melanosis coli
- Treatment of inherited juvenile macular degeneration

ADVANTAGES

- No previous therapeutic approaches for prevention of lipofuscin aggregation
- Non-cytotoxic
- Efficacious when tweezer is administered pre- or post-lipofuscin aggregation

STATE OF DEVELOPMENT

Tweezers have been used to prevent lipofuscin aggregation in human retinal pigment cells. Pre-treatment of cells with the molecular tweezer and subsequent administration of lipofuscin resulted in 60% reduction in aggregation. However, when the lipofuscin was administered first and subsequently treated with the molecular tweezer, aggregation was reduced almost to negative-control level.

PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Issued Patent	12,076,330	09/03/2024	2018-455
European Patent Office	Published Application	3784795	03/03/2021	2018-455

CONTACT

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INVENTORS

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OTHER INFORMATION

KEYWORDS

Lipofuscin aggregation, molecular tweezer, macular degeneration

CATEGORIZED AS

- **Materials & Chemicals**
  - Chemicals
- **Medical**
  - Disease: Central Nervous System
  - Disease: Genetic Diseases and Dysmorphic Syndromes
  - Disease: Ophthalmology and Optometry
  - Other
  - Therapeutics

RELATED CASES

2018-455-0

RELATED MATERIALS

- Attar, A., and Bitan, G, Disrupting self-assembly and toxicity of amyloidogenic protein oligomers by "molecular tweezers" – from the test tube to animal models, Curr. Pharm. Des., 2014.
- Schrader, T., Bitan, G., and Klärner, F. G., Molecular tweezers for lysine and arginine - powerful inhibitors of pathologic protein aggregation, Chem Commun (Camb), 2016.

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- New Molecular Tweezers Against Neurological Disorders And Viral Infections
- Preventing Synuclein Accumulation as a Strategy for Improving Neuronal Survival and Regeneration after Spinal Cord Injury
- Small Molecule "Molecular Tweezers" that Inhibit Amyloid-β Fiber Formation
- Treatment Of Lysosomal Storage Disorders

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