Treatment Of Lysosomal Storage Disorders

Tech ID: 30307 / UC Case 2018-726-0

SUMMARY

UCLA researchers in the Departments of Neurology have developed a novel treatment for Lysosomal-storage diseases (LSDs) with neurological impairment.

BACKGROUND

Lysosomal-storage diseases (LSDs) cause abnormal accumulation and aggregation of proteins due to insufficient clearance of proteins by the lysosomes. Although individually rare, LSDs as a group occur in at least 1/7700 live births. However, to date, there are no approved products for the treatment of LSDs with neurologic impairment. Therefore, there is an urgent need for improved treatments for LSDs with neurological impairment.

Current therapies for LSDs, such as enzyme replacement therapy (ERT), hematopoietic stem cell transplantation (HSCT) and experimental gene therapy, are some of the most expensive in medicine. Moreover, the neurological degradation accompanied by multiple complications requires a multidisciplinary management to allow adapted symptomatic treatment.

INNOVATION

Professor Bitan and coworkers have demonstrated that molecular tweezer, such as CLR01, covered by UCLA case 2008-489, are capable of improving memory deficits, attenuating neuronal loss, and reducing inflammation in LSDs, thus providing a novel treatment for LSDs with neurological impairment. Molecular tweezers are demonstrated to both prevent the formation of amyloid deposits and reduce the existing deposits in LSD mice. In addition, the treatment led to improvement of neuroinflammation in the brain, as both microgliosis and astrocytosis were reduced significantly following the treatment.

APPLICATIONS

- Treatment of Mucopolysaccharidosis type III (MPS III)
- Treatment of other lysosomal-storage diseases (LSDs), such as Krabbe disease, metachromatic leukodystrophy, Batten disease, Sandhoff disease, Niemann-Pick disease

ADVANTAGES

- No previous therapeutic approaches
- Non-cytotoxic
- Efficacious for both therapeutics and prevention
- Improved neuroinflammation in the brain

PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Issued Patent	12,076,332	09/03/2024	2018-726
Germany	Issued Patent	602019055850.3	07/24/2024	2018-726
European Patent Office	Issued Patent	3784794	07/24/2024	2018-726
France	Issued Patent	3784794	07/24/2024	2018-726
United Kingdom	Issued Patent	3784794	07/24/2024	2018-726
Italy	Issued Patent	502024000046932	07/24/2024	2018-726

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INVENTORS

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OTHER INFORMATION

KEYWORDS

lipofuscin aggregation, molecular tweezer, macular degeneration, lysosomal-storage diseases (LSDs)

CATEGORIZED AS

- **Biotechnology**
  - Health
- **Materials & Chemicals**
  - Biological
- **Medical**
  - Disease: Central Nervous System
  - New Chemical Entities, Drug Leads
  - Therapeutics

RELATED CASES

2018-726-0

Sweden	Issued Patent	3784794	07/24/2024	2018-726

RELATED MATERIALS

- Fokkens, Michael, Thomas Schrader, and Frank-Gerrit Klärner. "A molecular tweezer for lysine and arginine." Journal of the American Chemical Society 127.41 (2005): 14415-14421.
- Attar, Aida, and Gal Bitan. "Disrupting self-assembly and toxicity of amyloidogenic protein oligomers by “molecular tweezers”-from the test tube to animal models." Current pharmaceutical design 20.15 (2014): 2469-2483.
- Schrader, Thomas, Gal Bitan, and Frank-Gerrit Klärner. "Molecular tweezers for lysine and arginine–powerful inhibitors of pathologic protein aggregation." Chemical Communications 52.76 (2016): 11318-11334.

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- Inhibition Of Lipofuscin Aggregation By Molecular Tweezers
- 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

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