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## New Molecular Tweezers Against Neurological Disorders And Viral Infections

Tech ID: 30176 / UC Case 2018-906-0

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### INVENTORS

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### OTHER INFORMATION

#### KEYWORDS

molecular tweezer, amyloid,  
therapeutics, Alzheimer's disease,  
Parkinson's disease, protein  
aggregation, neurodegenerative  
diseases, anti-viral, orphan diseases,  
enveloped viruses, ZIKA

#### CATEGORIZED AS

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

#### RELATED CASES

2018-906-0

## SUMMARY

UCLA researchers in the Department of Neurology with an international team of scientists have developed several new molecular tweezer derivatives with novel synthesis methods that significantly improved the therapeutic efficacy and pharmacokinetic characteristics of the drug candidates.

## BACKGROUND

Protein aggregation is critical in the pathologic mechanism of various neurodegenerative diseases, such as Alzheimer's and Parkinson's, as well as rare orphan diseases, such as multiple system atrophy, amyotrophic lateral sclerosis, and familial amyloidotic polyneuropathy. These diseases bring high financial burdens globally and effective therapeutics are gravely needed.

Molecular tweezers are a type of small molecule drug candidates that efficiently inhibit abnormal protein aggregation, toxicity, and cell-to-cell spreading. The earlier lead compound, CLR01, covered by UCLA case 2008-489, has shown great therapeutic effects in various animal models, preventing the formation of protein aggregates while enhancing the clearance of already formed aggregations. Continuous efforts have been made since then to further improve CLR01 for FDA testing and clinical trials.

## INNOVATION

UCLA researchers in the Department of Neurology with an international team of scientists have developed several new molecular-tweezer derivatives using novel synthetic methods. The versatility of the molecular tweezers is significantly increased with the new synthesis methods. Several of the new molecular tweezer derivatives have shown improved pharmacokinetic characteristics, including improved oral bioavailability and blood-brain barrier penetration.

## APPLICATIONS

- ▶ Therapeutics in Alzheimer's disease
- ▶ Therapeutics in Parkinson's disease
- ▶ Therapeutics in other neurodegenerative diseases
- ▶ Therapeutics in rare orphan diseases
- ▶ Anti-Viral therapeutics against enveloped viruses

## ADVANTAGES

- ▶ Improved lysine and arginine affinities
- ▶ Improved blood-brain barrier (BBB) penetration
- ▶ Improved oral bioavailability

## PATENT STATUS

Country	Type	Number	Dated	Case
European Patent Office	Published Application	EP 3 813 841	05/05/2021	2018-906
United States Of America	Published Application	20210122771	04/29/2021	2018-906

## RELATED MATERIALS

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- ▶ Schrader, T.; Bitan, G.; Klärner, F. G., Molecular tweezers for lysine and arginine - powerful inhibitors of pathologic protein aggregation. *Chem Commun (Camb)* 2016, 52 (76), 11318-34.
- ▶ Röcker, A. E.; Müller, J. A.; Dietzel, E.; Harms, M.; Kruger, F.; Heid, C.; Sowislok, A.; Riber, C. F.; Kupke, A.; Lippold, S.; von Einem, J.; Beer, J.; Knoll, B.; Becker, S.; Schmidt-Chanasit, J.; Otto, M.; Vapalahti, O.; Zelikin, A. N.; Bitan, G.; Schrader, T.; Munch, J., The molecular tweezer CLR01 inhibits Ebola and Zika virus infection. *Antiviral Res* 2018, 152, 26-35.

## ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- ▶ [Inhibition Of Lipofuscin Aggregation By Molecular Tweezers](#)
- ▶ [Preventing Synuclein Accumulation as a Strategy for Improving Neuronal Survival and Regeneration after Spinal Cord Injury](#)
- ▶ [Small Molecule "Molecular Tweezers" that Inhibit Amyloid- \$\beta\$  Fiber Formation](#)
- ▶ [Treatment Of Lysosomal Storage Disorders](#)

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