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## **New Molecular Tweezers Against Neurological Disorders And Viral Infections**

Tech ID: 30176 / UC Case 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	Туре	Number	Dated	Case
Switzerland	Issued Patent	3813841	08/13/2025	2018-906
European Patent Office	Issued Patent	3813841	08/13/2025	2018-906
Spain	Issued Patent	3813841	08/13/2025	2018-906
United Kingdom	Issued Patent	3813841	08/13/2025	2018-906
Unitary Patent	Issued Patent	3813841	08/13/2025	2018-906

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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

United States Of America Published Application 20210122771 04/29/2021 2018-906

#### **RELATED MATERIALS**

- ▶ Fokkens, M.; Schrader, T.; Klärner, F. G., A molecular tweezer for lysine and arginine. J. Am. Chem. Soc. 2005, 127 (41), 14415-14421.
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- P.Y.; Ehrmann, M.; Benedek, G. B.; Loo, J. A.; Klärner, F. G.; Schrader, T.; Wang, C.; Bitan, G., Lysine-specific molecular tweezers are broad-spectrum inhibitors of assembly and toxicity of amyloid proteins. J. Am. Chem. Soc. 2011, 133 (42), 16958-69.
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- ▶ 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-β Fiber Formation
- ▶ Treatment Of Lysosomal Storage Disorders

