TECHNOLOGY OFFER

SERPIN inhibitors for the treatment of prion and prion like diseases

Brief Summary
To date, despite numerous active efforts, there are no drugs available for the cure of the neurodegenerative diseases collectively referred as prion and prion-like disorders. Symptomatic treatment is the only available option, including the administration to patients of antipsychotics, such as quetiapine and clonazepam, to treat myoclonus, and of selective serotonin re-uptake inhibitors (SSRIs) to treat depression.

Indeed, through this invention, we are presenting a new class of small molecules acting as SERPINA3 inhibitors, which are able to reduce PrPSc accumulation in prion-infected cell lines, thereby representing a new therapeutic treatment against prion and prion-like diseases.

The development of specific inhibitors of SERPINA3 with an activity in the nanomolar range is expected. The characterization of structural models of the binding of the small molecules with the protein should allow the identification of novel inhibitors with improved potency.

Value Proposition
So far there are no effective therapies for prion diseases. The proposed technology aims at developing novel molecules with improved efficacy on these devastating diseases.

Tests
The project is being developed at SISSA. Thus far the research team has characterized the system in vitro and partially in vivo. Further developments are expected in the coming months also depending on the identification of appropriate partnership.

Application Field
Treatment of neurodegenerative diseases

Keywords
serpina3, serpina3n, protease inhibitors, prion-like disease

Current Stage of Development
In vitro and in vivo (partial) proof of concept

Intellectual Property Rights and Priority
EP Application n. 18170092 - 30.04.2018

Type of collaboration
License for patent exploitation

Joint development

The Scientific Coordinator
Prof. Giuseppe Legname is Director of the Laboratory of Prion Biology at SISSA. He earned his Doctor of Philosophy at the University of Warwick, UK. After a long spell in industry in the field of immunotherapy, he moved to the National Institute for Medical Research (NIMR), Medical Research Council in London, UK as a Research Associate. In 1999 he became Assistant Adjunct Professor, at the Institute for Neurodegenerative Diseases (IND), University of California at San Francisco, USA, under the direction of 1997 Nobel Laureate Professor Stanley B. Prusiner. In 2006, he moved to SISSA. For more information: https://lpb.sissa.it/

Notes
Supplementary material and supporting data available upon request

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