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### SERPIN INHIBITORS FOR THE TREATMENT OF PRION AND PRION LIKE DISEASES

Ref-No: TA-PT-1.2816G

#### BACKGROUND

To date, despite numerous active efforts, there are no drugs available for the cure of the neurodegenerative diseases collectively referred as prion and prionlike 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.

#### PROBLEM

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.

#### SOLUTION

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

#### SCOPE OF APPLICATION

Compounds for use in the therapeutic treatment of a prion or prion-like disease and to pharmaceutical compositions comprising these compounds.

#### SERVICE

The project is being developed at Scuola Internazionale Superiore di Studi Avanzati (SISSA). Thus far, the research team has characterized the system in vitro and partially in vivo. Further developments are expected in the coming



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#### **DEVELOPMENT STATUS**

Trial

### CATEGORIES

//Medicine and pharmaceutics //Therapy and therapeutics

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months also depending on the identification of appropriate partnership.

Supplementary material and supporting data available upon request.