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

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HINTERGRUND

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.

PROBLEMSTELLUNG

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.

LÖSUNG

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.

ANWENDUNGSBEREICHE

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
months also depending on the identification of appropriate partnership.

Supplementary material and supporting data available upon request.