Docket #: S25-471

Chaperone-inspired Biologics for Suppression of HTT Aggregation and Toxicity

Huntington's Disease and other ataxias are devastating diseases without any cure or treatment. They are caused by the formation of toxic oligomeric and the aggregation of the Huntintin (HTT) protein. A mutation in the HTT exon involving a polyglutamine expansion beyond 36 consecutive glutamines promotes the formation of amyloid-like fibers and results in disease. To date, there are no available treatments to prevent the aggregation and toxicity of the HTT protein or to treat the mutation.

The Frydman Lab at Stanford has synthesized a set of endogenous chaperones that can prevent HTT aggregation and toxicity. These biologics target the HTT-binding domains to prevent mutant HTT aggregation and toxicity. The binding regions of the constructs can also be modified so they can be used as small molecules to target different HTT regions. Delivery of the biologics can be achieved by using existing brain-penetrant strategies, such as adeno-associated virus vectors (AAV), nanoparticles, etc. The invention could also be applied to increase the chaperone capacity of cells and aid in other types of protein aggregation diseases.

Stage of Development
Research - in vitro

Applications

- Small molecule biologic
- Genetic disorders
- Protein-folding disorders

Advantages

- There are no currently available treatments that prevent Htt tocicity or aggregation in patients
- Compatible with existing delivery platforms
- Could be modified to target other genetic disorders involving protein aggregation

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