Docket #: S14-396

Small molecule compounds as potential therapies for nucleotide repeat diseases

Dr. Stanley Cohen and colleagues have identified small molecular compounds that may be useful in the treatment of nucleotide repeat diseases. A well-known nucleotide repeat disorder is Huntington's disease. It is an inherited trinucleotide repeat (TNR) disorder characterized by lengthy polyglutamine (polyQ) repeats which lead to the production of abnormal proteins and result in severe neurodegeneration. Currently there is no treatment for TNR diseases. To help provide a treatment the inventors expanded on their previous technology which identified Supt4h as a therapeutic target for Huntington's disease and PolyQ disorders (see Stanford Docket S11-239). Here the inventors have identified compounds that target Supt4h function and which could be developed as possible therapeutics for TNR disorders.

Stage of research
Studies in cell lines have shown that the compounds reduce expression of genes containing expanded TNRs.

Applications

- Therapeutic development for TNR disorders

Advantages

- Unmet need- currently there is no effective therapeutic for NR diseases
- Specific- reduces expression of only the disease causing allele

Patents

- Published Application: STAN-1179PRV
- Published Application: WO2016196012
- Published Application: 20180064744
- Published Application: WO2018236910
- Published Application: 20200147069
- Issued: 10,675,293 (USA)

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