

Investigating prion diseases with biocomputing and in vitro approaches

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Prion diseases are a group of invariably fatal neurodegenerative disorders without a known, effective therapeutical treatment. This class of maladies can exist as sporadic, genetic, or infectious. The genetic cases account for almost 15% of all cases and are characterized by almost 30 different missense point mutations in the open reading frame of the HuPrP. To date, there are no effective therapies for prion diseases; it is therefore imperative to develop new therapeutics for these maladies. We are carrying out a global drug screening of all known HuPrP mutations using two complementary approaches: (1) inhibition of in vitro amyloid formation assay; (2) develop new compounds using computational methods. Our approaches may help develop novel drugs for the treatment of a variety of human genetic prion diseases.