REGIONAL CENTRE FOR BIOTECHNOLOGY
Seminar series

Molecular Tweezers: Novel Therapeutic Strategies for the Treatment of Dementia Related Diseases

Sharmistha Sinha, PhD
Iowa State University, USA

Thursday, October 18, 2012
11:00 am
Seminar Room

Abstract

Anomalous protein aggregation causes nearly two-dozen human diseases, generally called amyloidosis. Alzheimer's, Parkinson's, Huntington's, and prion diseases are notable examples of such diseases. At present there is no cure for this genre of diseases. Molecules that inhibit the aberrant aggregation are good therapeutic targets for amyloidosis. Though there are several proteins that cause these diseases, the toxic oligomers and mature fibrillar structures have structural similarity, suggesting that common inhibitors of aggregation and toxicity for all these proteins may exist.

Using a rational approach, I have identified lysine specific "molecular tweezers," which inhibit the aggregation and toxicity in amyloidogenic proteins. The lead compound, CLR01, inhibits the aggregation and toxicity of multiple amyloidogenic proteins, including amyloid β- peptide (Aβ), **α-synuclein** (αS), β2-microglobulin (β2m), calcitonin, insulin, transthyretin and prion (106-126) fragment. CLR01 is effective *in vivo* in ameliorating toxic insults of Aβ and **αS.**