

Special Issue

Protein Folding Diseases— Molecular Mechanisms and Therapeutic Approaches

Message from the Guest Editors

Protein misfolding due to genetic, environmental or sporadic factors such as adverse intracellular conditions, is a hallmark of numerous pathologies, commonly referred to as protein folding diseases or conformational disorders. In these cases, a change in protein conformation may impair its biological function through functional deficiency or degradation (loss of function) or promote its accumulation as proteinaceous aggregates (toxic gain of function). Examples include metabolic diseases (phenylketonuria, cystic fibrosis), chaperonopathies (cataracts), and neurodegenerative conditions (Alzheimer's, Parkinson's). For most of these diseases, underlying molecular mechanisms remain scarcely understood, thus limiting the development of effective therapies. In this special issue, we look for contributions addressing the fundamental mechanisms and biochemistry of protein misfolding in a pathophysiological context. Research on approaches to mitigate protein misfolding, such as on proteostasis regulators (molecular chaperones), biologics (antibodies) and folding correctors (pharmacological chaperones), is also welcome.

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