

Special Issue

Myotonic Dystrophies: An Update on Pathology, Molecular Pathogenesis and Therapeutic Approaches

Message from the Guest Editor

Myotonic dystrophies (DM) type 1 and type 2 are complex genetic diseases affecting many tissues, including the skeletal muscles, heart, and brain. DM1 and DM2 are caused by unstable expansions of CTG (DM1) and CCTG (DM2) repeats. Both diseases do not have a cure. Molecular studies of DM have identified the major mechanisms of these disorders, which are associated with the toxic effects of mutant RNAs containing long CUG and CCUG repeats. However, the mutant RNAs in DM1 and DM2 might affect additional intracellular pathways, thereby increasing the complexity of molecular pathogenesis. This Special Issue will summarize current findings regarding the molecular mechanisms of DM1 and DM2, and will discuss how these advances can be used for the development of clinical studies on DM1 and DM2.

Guest Editor

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