

Special Issue

Latest Advances in Targeted Molecular Therapies for Genetic Disease

Message from the Guest Editor

This Special Issue aims to provide a comprehensive and critical overview of the latest advances in targeted molecular therapies for genetic diseases, highlighting their translational relevance and clinical impact. It brings together state-of-the-art research and authoritative reviews that explore innovative therapeutic strategies designed to address the underlying molecular and genetic mechanisms of inherited disorders. By directly addressing disease-causing molecular defects, these approaches represent a paradigm shift from symptomatic management toward mechanism-based intervention in genetic disorders. The scope of the Issue encompasses both rare and common genetic diseases, highlighting how advances in molecular biology, genomics, and bioinformatics have accelerated the development of personalized and mechanism-driven treatments. Targeted molecular therapies seek to elucidate and therapeutically exploit the genetic and biochemical alterations underlying inherited diseases, with the overarching goal of developing more precise, effective, and durable treatment strategies.

Guest Editor

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Message from the Editor-in-Chief

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