



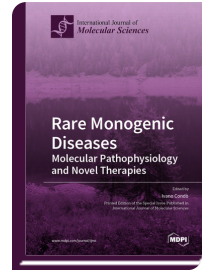
Special Issue Reprint

Rare Monogenic Diseases: Molecular Pathophysiology and Novel Therapies

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The reprint, “Rare Monogenic Diseases: Molecular Pathophysiology and Novel Therapies”, provides a collection of original research articles and systematic reviews focused on diverse conditions stemming from a pathogenic single gene mutation. The articles are focused on the molecular mechanisms that are affected within a rare monogenic disorder, such as those involving gene expression, molecular pathways and cell death. Moreover, specific experimental therapies including drug discovery, drug repositioning, gene therapy and protein-based therapeutics are discussed.



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