



Mechanisms of Inherited Retinal Degenerative Diseases and Emerging Therapies

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Message from the Guest Editors

Inherited retinal degenerative diseases are a genetically and phenotypically heterogeneous group of visual disorders, which primarily affect the function of photoreceptor cells and are among the leading causes of clinical blindness in humans. The major challenge now is to functionally characterize these gene products to delineate the biological mechanisms of retinal disease pathogenesis using in vitro and in vivo models, with the goal to design gene-based treatments. While advances have been made in elucidating the pathophysiological mechanisms underlying the genetic causes of retinal dystrophies, therapeutic approaches are now being explored to mitigate vision loss in such patients. It is crucial to establish animal models to elucidate retinal and photoreceptor biology in retinal diseases and in the development of novel gene-based and cell-based therapeutic modalities. There is an evolving interest in developing nutrient based neuroprotective therapy in retinal diseases. Newer and improved therapeutic approaches will greatly enhance the quality of life of individuals living with these potentially blinding diseases and facilitate effective disease management.





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