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Therapeutic Approaches for Cystic Fibrosis

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Message from the Guest Editors

Cystic fibrosis (CF)-causing mutations have complex effects on the CF transmembrane conductance regulator (CFTR) protein, including disrupting its processing to and stability at the plasma membrane, as well as its chloride channel activity. Because of this complex disease etiology, a combination of CFTR modulators is required to rescue the trafficking and functional defects of disease-associated CFTR variants. Despite the success of these combinatorial therapeutics, some CF-causing mutations appear less responsive. For these CF variants, mutation-agnostic therapies have to be developed, such as ENAC inhibitors, activators of alternative chloride channels, and artificial anion transporters (anionophores), as well as cell-based and gene therapies.

This Special Issue on "Cystic Fibrosis" will gather reviews and original articles focused on novel therapeutic approaches to this disease at basic, translational, and clinical levels to provide expert insights and perspectives on advances in the field.









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Message from the Editor-in-Chief

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