Special Issue

Cystic Fibrosis and CFTR Interactions 2.0

Message from the Guest Editors

The Cystic Fibrosis (CF) community has witnessed remarkable advancement with the recent approval of modulators that target the underlying defect in mutant CFTR. Although modulators can now be used to treat about 90% of CF individuals, these therapies do not bring mutant CFTR to wild-type levels and the mechanisms of action for these drugs are still poorly understood. As some individuals do not yet have a therapeutic option available that is based on the molecular defects associated with their genotypes, there is a need for novel treatment approaches, i.e. gene therapy. Furthermore, CFTR interactions continue to perplex researchers as it is not clear which interactions are critical to rescue CFTR and how these are altered by modulators and cellular responses. Thus, there is continued interest in studying CF disease mechanisms to understand the root of the problem. Topics of interest include: CFTR interactions: Response to modulators or novel RNA and DNA therapeutics:

Change of cellular responses by CFTR therapeutics; Overcoming disease symptoms by restoring CFTR function;

Personalized therapies for rare CFTR mutations; Regulation of CFTR expression; Targeting other channels in CF.

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Deadline for manuscript submissions

closed (31 January 2024)



International Journal of Molecular Sciences

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Impact Factor 4.9 CiteScore 9.0 Indexed in PubMed



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Editor-in-Chief

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