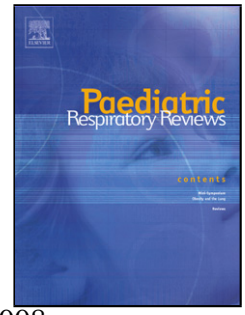

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Disease-modifying drug therapy in CF

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Summary

Whilst substantial progress has been made in the treatment of cystic fibrosis, the disease still carries a significant burden in terms of symptoms, requirement for treatment and early mortality. The last decade has witnessed a new era in the development of small molecule drugs targeting the CFTR protein, which for the first time may provide a truly disease-modifying approach to treatment. This article reviews progress and highlights some of the current and future challenges in CFTR modulator therapies.

Key words: potentiator, corrector, read-through, CFTR