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# Re-imagining cystic fibrosis care: next generation thinking

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Cystic fibrosis care has advanced dramatically over the past decade, with CFTR modulator therapy a game changer for some patients. With its increasing use, unexpected benefits and side-effects are being unmasked and must be managed accordingly. <http://bit.ly/2Ti8TYO>

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**ABSTRACT** Cystic fibrosis (CF) is a common multi-system genetically inherited condition, predominately found in individuals of Caucasian decent. Since the identification of the cystic fibrosis (CF) transmembrane conductance regulator (*CFTR*) gene in 1989, and the subsequent improvement in understanding of CF pathophysiology, significant increases in life-expectancy have followed. Initially this was related to improvements in the management and systems of care for treating the various affected organ systems. These cornerstone treatments are still essential for CF patients born today. However, over the last decade, the major advance has been in therapies that target the resultant genetic defect: the dysfunctional *CFTR* protein. Small molecule agents that target this dysfunctional protein *via* a variety of mechanisms have led to lung function improvements, reductions in pulmonary exacerbation rates and increases in weight and quality-of-life indices. As more patients receive these agents earlier and earlier in life, it is likely that general CF care will increasingly pivot around these specific therapies, although it is also likely that effects other than those identified in the initial trials will be discovered and need to be managed. Despite great excitement for modulator therapies, they are unlikely to be suitable or available for all; whether this is due to a lack of availability for specific *CFTR* mutations, drug-reactions or the health economic set-up in certain countries. Nevertheless, the CF community must be applauded for its ongoing focus on research and development for this life-limiting disease. With time, personalised individualised therapy would ideally be the mainstay of CF care.