



Treatment Burden: Weighing up the costs and benefits

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The 21st century continues to bring many new possibilities for treatment for children, young people and adults with cystic fibrosis and life expectancy has improved considerably over the last 20 years. Along with the huge potential benefits that these new therapeutic interventions offer, comes the substantial challenge of managing an increasing treatment burden.

Early intensive treatment and monitoring from the time of diagnosis has been associated with better clinical outcomes. Many treatments require chronic therapy interspersed with additional therapeutic interventions for management of acute exacerbations which may be difficult to sustain for individuals and families. This is especially difficult on a long term basis and particularly as therapeutic regimes become increasingly complex.

Treatment burden, as measured by health related quality of life tools such as the CFQ-R varies between populations. This burden appears to be considerable, particularly for parents.¹ Depression is also common in patients and parents of children with cystic fibrosis and may have a significant impact on health outcomes². While the consequences on depression of the increasing treatment burden are unknown, it is likely to further complicate management.

Adherence (or compliance) with treatment in cystic fibrosis varies depending on the type of therapy,³ and how adherence is measured.⁴ However, over all, there are relatively low levels of adherence- 50% or less, for many important therapies such as airway clearance regimes or inhaled therapies. New and more rapid and convenient delivery devices for inhaled therapies have become available which may improve adherence and reduce treatment burden for many patients. While these devices offer a huge potential benefit, they also need to be assessed from the perspective of comparison of treatment dose, because the original studies on which treatment is based may have used alternative delivery devices (and doses).

As new therapies are trialled, we will need to consider carefully the effect of therapies on health related quality of life and the ability of patients and families to sustain treatments. Also we will need to find ways to mitigate the ever increasing burden of treatment for patients and families. The relative benefits of treatments and the use of novel and new delivery devices will need to be carefully assessed to assure optimal health outcomes for individuals with cystic fibrosis.

1. Thomas et al. *J Pediatr.* 2006; 148:508-16.
2. Quittner et al. *Curr Opin Pulm Med.* 2008; 14: 582-8
3. Arias et al. *J Cyst Fibros.* 2008; 7:359-6
4. Modi et al. *J Cyst Fibros.* 2006; 5:177-85