Losses in the gains of children with cystic fibrosis who had to interrupt their modulator therapies: Time flies for half taken breaths!

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Abstract

Background: In this study, we aimed to evaluate the losses in the gains of children who had to discontinue their modulator therapies due to drug delivery procedures. **Methods:** Demographic, clinical, microbiologic, radiologic, and pulmonary function test parameters of twelve CF children, were evaluated. Parameters were divided into three groups as 'before treatment' (BT), 'during treatment' (DT) and 'after interruption of treatment' (AT) to show differences between. **Results:** There was a significant increase in forced expiratory volume in 1 s (FEV1) z-score, body mass index (BMI) z-score and Cystic Fibrosis Questionnaire-Revised respiratory domain score (CFQR-RS) of DT compared with the values BT ($p=0.001$, $p=0.012$, $p<0.001$; respectively). It was found that FEV1 z-score, BMI z-score and CFQR-RS of DT decreased significantly compared with the values AT ($p=0.003$, $p=0.01$, and $p<0.001$, respectively). When post and pre-treatment levels were compared, there was no significant difference between FEV1 z-score ($p=0.07$), BMI z-score ($p=0.56$), CFQR-RS ($p=0.7$). Half of patients had percent-predicted forced expiratory volume levels with a drop of more than 20%. It was also detected that *Pseudomonas aeruginosa* colonization was a significant factor in degradation of FEV1 z score to the lower pre-treatment levels. **Conclusion**: This is the first retrospective detailed study about discontinuation of modulatory therapies in children. Our study shows the importance of treatment continuation as well as the patients access to these drugs. We hope that this study will raise awareness about the regular long-term use of modulator therapies. To make these therapies available worldwide, immediate action is required.

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