Dornase alpha use in patients with cystic fibrosis

O uso de alfadornase em pacientes com fibrose cística

El uso de alfadornasa en pacientes con fibrosis quística

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Cystic Fibrosis (FC) is an autosomal recessive disorder with an estimated incidence of one in 7,500 live births in Brazil, varying in different states[1]. The gene in question encodes a protein called cystic fibrosis transmembrane conductance regulator (CFTR), involved in the transepithelial transport of ions and water. It affects, thus, the epithelial organs whose secretions are thick, with consequent obstruction and development of classical clinical changes.

In the bronchial tubes, thickened secretions predispose to the onset of infection, which leads to a vicious cycle of increased clogging and tendency of recurrent infections. About 10% of sputum in these patients is made up of DNA, primarily from leukocytes that migrate into the bronchial lumen to fight infection[2].

Within this perspective, the use of effective mucolytics was always sought for a better management of CF, aiming at improving the quality of life and reducing the associated morbidity.

From about two decades ago, dornase alfa has been recommended for use in patients with CF. Its action, cleaving DNA, dilutes secretions, being effective in reducing the decline in lung function and decreasing the number of pulmonary exacerbations, according to a randomized clinical trial published in 1994[3]. Later, other studies showed that the drug was effective in reducing pulmonary exacerbations and improving other functional outcomes, even in patients with incipient pulmonary disease, still with normal functional parameters[4-9]. The proven effectiveness of dornase alfa in such conditions led to its inclusion in consensus guidelines on the management of CF lung disease for children over 6 years of age[10,11].

In this issue of Revista Paulista de Pediatria, Rozov *et al* present an elegant study, in which they aggregate evidence for the use of dornase alfa in children and adolescents with CF[12]. The Brazilian multicenter study evaluated patients over 5 years of age who had not received the drug. CF is a genetic disorder whose development is influenced by environmental factors. Therefore, the merit of this study lies in the fact that it demonstrates the benefits of dornase alfa in Brazilian patients, regarding the reduction of visits to the emergency room, an outcome associated with quality of life. Previously, the authors had already published results of quality of life for these patients, showing improvements in many areas after the introduction of this medication[13].

A limitation of the study that should be mentioned is its uncontrolled design. There are inherent limitations to studies with a design of "before and after". Patients monitored in surveys tend to be more compliant and subject to the placebo effect. However, ethical constraints absolve the authors as to the choice of the design, since there was already evidence of the benefit of the drug in children older than 6 years, a fact that advised against a control group with placebo.

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