

Cystic Fibrosis Pulmonary Guidelines

Chronic Medications for Maintenance of Lung Health

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Rationale: Cystic fibrosis is a recessive genetic disease characterized by dehydration of the airway surface liquid and impaired mucociliary clearance. As a result, individuals with the disease have difficulty clearing pathogens from the lung and experience chronic pulmonary infections and inflammation. Death is usually a result of respiratory failure. Newly introduced therapies and aggressive management of the lung disease have resulted in great improvements in length and quality of life, with the result that the median expected survival age has reached 36 years. However, as the number of treatments expands, the medical regimen becomes increasingly burdensome in time, money, and health resources. Hence, it is important that treatments should be recommended on the basis of available evidence of efficacy and safety.

Objectives: The Cystic Fibrosis Foundation therefore established a committee to examine the clinical evidence for each therapy and to provide guidance for the prescription of these therapies.

Methods: The committee members developed and refined a series of questions related to drug therapies used in the maintenance of pulmonary function. We addressed the questions in one of three ways, based on available evidence: (1) commissioned systematic review, (2) modified systematic review, or (3) summary of existing Cochrane reviews.

Conclusions: It is hoped that the guidelines provided in this article will facilitate the appropriate application of these treatments to improve and extend the lives of all individuals with cystic fibrosis.

Keywords: antibiotics; antiinflammatory agents; bronchodilators; mucolytics; saline solution, hypertonic

Cystic fibrosis (CF) is a complex multiorgan disease in which lung disease accounts for nearly 85% of the mortality (1). Lung destruction is caused by obstruction of the airways due to dehydrated, thickened secretions, resultant endobronchial infection, and an exaggerated inflammatory response leading to development of bronchiectasis and progressive obstructive airways disease. Physicians treating patients with CF are faced with a growing number of treatment options for the maintenance of lung health for children

and adults with CF. At present, the Cystic Fibrosis Foundation has published consensus guidelines for only two of these therapies: aerosolized tobramycin (2) and recombinant human DNase (deoxyribose nuclease) (3), and additional studies have been published since the release of those guidelines. To provide guidance to the physician who must choose from an ever-expanding arsenal of treatments for chronic CF lung disease, the Cystic Fibrosis Foundation established the Pulmonary Therapies Committee. This document represents the committee's recommendations, based on available evidence, for the use of medications intended to maintain lung health. The guidelines are designed for general use in most patients, but may need to be adapted to meet individual needs as determined by the patient's health care provider. In addition, because of the limited number of studies available involving very young children, unless otherwise noted, recommendations are intended for individuals at least 6 years of age.

METHODS

A preliminary meeting was held in October 2005 to initiate the process of identifying and prioritizing topics to be covered in these guidelines. The committee was composed of 15 members, all from the United States, representing internal medicine, pediatrics, nursing, respiratory therapy, systematic review procedures, and the Cystic Fibrosis Foundation. Each member of the committee was involved in developing the recommendations and reviewed and commented on the final version of the guidelines. The committee members developed and refined a series of questions related to drug therapies used in the maintenance of pulmonary function. We addressed the questions in one of three ways, based on available evidence: (1) commissioned systematic review, (2) modified systematic review, or (3) summary of existing Cochrane reviews. See the online supplement for details on these methods.

Members of the committee were provided with summaries of the evidence. Subcommittees, formed to review the evidence for each specific treatment, drafted recommendations. Their assessment of the evidence and draft statements was then presented to the full committee for discussion. For each treatment under question, the body of evidence was evaluated by the full committee and recommendations were made on the basis of the U.S. Preventive Services Task Force grading scheme (4) (see Table 1). A draft of the recommendations was presented in November 2006 at the North American Cystic Fibrosis Conference and the committee solicited public commentary for 1 month after the presentation. This input was considered by the committee in the preparation of these guidelines.

(Received in original form May 4, 2007; accepted in final form August 24, 2007)
Supported by the Cystic Fibrosis Foundation.

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This article has an online supplement, which is accessible from this issue's table of contents at www.atsjournals.org.

Am J Respir Crit Care Med. Vol 176, pp 937-949, 2007

Originally Published in Print as DOI: 10.1164/rccm.200705-0640OC on August 29, 2007
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