Trends in the use of outcome parameters for Respiratory Drug Research

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Abstract

Substantial success has been realized within the treatment respiratory diseases due to more insight within the mechanisms of disease like inflammatory pathways and new targets for treating these diseases. However, there is a transparent discrepancy between the outcome parameters, currently utilized in clinical studies, and clinical outcome and survival. So, there is an unmet need for additional more sensitive outcome parameters that might assist in understanding the mode of action and subsequent pharmacodynamics effects. The ever-rising costs and rate of attrition of developing new respiratory drugs, makes it mandatory to extend the performance of clinical trials by using these methods and by more efficient study designs. Patient-reported outcome (PRO) measures that quantify disease impact became important measures of outcome in COPD research and treatment. The development of COPD may affect several aspects of a patient's health. These consequences of illness are often considered a process of illness progression, which normally starts at the event of physiological or biological abnormalities, leading to symptoms and physical limitations that are noticed and reported by the patients. Eventually, patients will need to face their inability to take part in their usual activities, which can influence their perception of their health and ultimately their general well-being.

A wide range of outcome measures or endpoints are utilized in clinical trials to assess the consequences of treatments in pediatric respiratory diseases. This will make difficult to match treatment outcomes from different trials and also to know whether new treatments offer a true clinical benefit for patients. Clinical trials in respiratory diseases evaluate three kinds of endpoints: subjective (i.e., symptom scores, need for rescue medication, quality-of-life measures), objective (i.e., lung function tests, markers of inflammation) and health-related outcomes (i.e., reduction in need for drugs, reduction in need for hospitalizations, reduction in absence from school, reduction in deaths).

The field of respiratory medicine, especially, is plagued by unmet needs. First of all, there's little or no awareness of respiratory illness at all. For chronic obstructive pulmonary disease (COPD), for instance, diagnosis rates are often as low as 30 percent leading to unnecessary delays in treatment initiation. Even when a patient is correctly diagnosed, treatment is often less than ideal. Moreover, there are clearly unmet needs within the treatment of COPD, like exacerbation and symptom control, improving health status, and slowing the decline of lung function and disease progression. Clinical trials in chronic obstructive pulmonary disease (COPD) normally include forced expiratory volume in one second (FEV1), the principal measure of lung function, as an outcome, mainly because the COPD research community and regulatory agencies have recognized its importance as an objective index of airflow obstruction that measures both symptomatic relief and disease progression.

Patients with respiratory diseases need new medicines that alleviate symptoms and modify the course of the disease without, at an equivalent time, causing undue side effects and/or non-adherence to medication regimens. additionally, to pulmonary manifestations, patients with COPD may develop other systemic problems and comorbidities that contribute significantly to reduced exercise capacity and HRQoL. Therefore, recent treatment guidelines for COPD have recommended the inclusion of symptom assessments like the COPD Assessment Test.

The use of a patient reported outcomes as a primary or a secondary endpoint in clinical trials has become more widespread in recent years, and guidance on the utilization of PROs in clinical trials has been recently published. Typically, PROs present the patient perspective of treatment benefit and may be wont to assess and monitor disease progression, exacerbation of symptoms, or adverse effects of treatment. Key concepts in COPD that have significant impact on a patient's HRQoL include breathlessness/dyspnea, fatigue, cough and sputum production, physical functioning, social functioning, and exacerbations.

Respiratory agents is a term used to describe a wide sort of medicines used to relieve, treat, or prevent respiratory diseases like asthma, bronchitis, chronic obstructive pulmonary disease (COPD), or pneumonia. Respiratory agents are available in various forms, like oral tablets, oral liquids, injections or inhalations. Inhalations deliver the specified medicine or medicines directly to the lungs, which suggest the medicine(s) can act directly on the lung tissues, minimizing systemic side effects. Some products contain more than one medicine (example: few inhalers that combine a long-acting bronchodilator with a glucocorticoid). Bronchodilators are the most frequently used inhaled medications. They can be subdivided into sympathomimetic (adrenergic) drugs and parasympatholytic (anticholinergic) drugs, as well as being classified as short acting or long acting. The adrenergic drugs stimulate the sympathetic nervous system, while anticholinergic drugs block the parasympathetic system. Adrenergic agents are used to cause bronchodilation; anticholinergic drugs block bronchoconstriction. Short-acting drugs are effective for 4 to six hours and long-acting bronchodilators generally last about 12 hours.

In selecting a treatment plan, the advantages and risks to the individual and the costs, direct and indirect, to the community must be considered. Patients should be identified before the end stage of the illness when disability is substantial. However, the advantages of spirometric screening, of either the overall population or smokers, are still unclear. Educating patients and physicians to acknowledge that cough, sputum production, and particularly breathlessness aren't trivial symptoms is an important aspect of the general public health care of this disease.