




HomeLine

Brought to you by Cystic Fibrosis Services, Inc.

Guest Author Series

CF Patient Registry Serves as a Vital Resource to Improved Clinical Care

By Christopher H. Goss, M.D., M.Sc., and H. Worth Parker, M.D.

Christopher H. Goss, M.D., M.Sc., is an assistant professor in the division of pulmonary and critical care medicine and department of medicine at the University of Washington School of Medicine, Seattle. He serves as the associate medical director of the Therapeutics Development Network Coordinating Center (TDNCC), a CF Foundation-funded research group devoted to the study of new drugs for the treatment of CF. Goss uses the patient registry to look at the changing survival rates in patients with CF over time; to create a mathematical model to predict two-year survival; and to assess the impact of various airway bacteria on survival in CF. While working at the TDNCC, he participates in designing clinical trials for patients with CF and serves as a medical monitor for studies conducted within the Therapeutics Development Network.

H. Worth Parker, M.D., is an adult pulmonary specialist who has worked in the area of CF care and research for 20 years. He directs the CF care center in New Hampshire and is section chief of the pulmonary section at the Dartmouth Hitchcock Medical Center in Lebanon, N.H. He also serves on the CF Center Committee. Past and present research interests include using the CF patient registry to understand variation in clinical practice and to improve the use of the CF patient registry to measure compliance with the Clinical Practice Guidelines for Cystic Fibrosis document.

Q What is the purpose of a patient registry?

A Patient registries, in general, are important tools used to assess the course of a disease and how the disease has changed over time. A patient registry forms a database that contains information about the clinical characteristics (e.g., age, gender, lung function, hospitalization rate, etc.) of a patient population with a specific disease. Registries are particularly useful in diseases that are relatively rare, like cystic fibrosis (CF).

Rare, or “orphan,” diseases are difficult to study because of the limited number of patients. A registry can merge a geographically diverse patient population and encourage clinical and scientific investigation to advance patient care. Patient registries also give physicians and researchers a “window,” into the actual care of patients. In this way, important differences in patient care may be found that point to areas of improvement.

Q How does the registry impact the CF community?

A The *Cystic Fibrosis Foundation Patient Registry Annual Data Report* has been tremendously successful in describing the CF patient population to the clinicians that care for these patients and in describing the natural history of the disease as it evolves over time. The *CF Foundation Patient Registry Annual Data Report* first started collecting patient data in 1966. The registry has since grown extensively to its current comprehensiveness and breadth. More patients are included in the registry each year and the scope of clinical characteristics has increased. Details are now available regarding microbiology, for example, and lung function, complications, demographics, genotypes and the use of common treatments. The CF registry now contains information on more than 22,000 patients.

Q Why should/must information be linked to individuals?

A It is crucial that patient data remain linked and confidential. The CF Foundation has placed a premium on this aspect of the registry. Specific identifying information is not released to researchers, including the care center names. Because the natural history of CF is continually changing — due to improvements in care — it is vitally important that the CF Foundation continue to collect this information. Patients and clinicians have witnessed dramatic gains in the survival of people with CF — with the median age of survival improving from 14 years in 1969 to 32 years in

(continued on page 2)



CF Cystic
Fibrosis
Services, Inc.

PHARMACY SERVICES

**A Subsidiary of the
Cystic Fibrosis Foundation**



(CF Foundation Patient Registry Annual Data Report — continued from page 1)

2000. As the quest for a cure for this disease continues, the CF Foundation must keep track of where the health of the population is going.

Q How does the registry affect patient care?

A Most patient care is now informed by the study of a select subset of the CF population, those who participate in research studies. Differences between these patients and the CF population at large may significantly impact the interpretation of the results of these studies. Through the *CF Foundation Patient Registry Annual Data Report*, these differences can be adequately assessed and trends can be established. The registry also has been extremely important in regard to the effective design of CF clinical trials. In addition, the registry is used to calculate the number of patients needed for a research study to ensure statistically meaningful results. Through analysis of the registry data, CF researchers have been able to gain significant insight regarding the following:

- A comprehensive medical description of the CF population as a whole;
- Changing survival rates in patients with CF;
- Particular characteristics associated with better survival for those with CF;
- The frequency, and impact on health, of specific treatments;
- Lung function changes through time in patients with CF;
- Types of lung infections reported in patients with CF and suggested treatments;
- Development of new methods to help analyze this data.

Q How do CF Foundation-supported care centers benefit from this information?

A The availability of this data for care centers can be instrumental in allowing each care center to gauge their treatment of patients with CF compared to other centers. It allows them to ask the basic question: How are we doing and how can we improve our care? This information is available only to the clinicians at the care center so they can continually evaluate the quality of care they deliver to their patients. This ongoing evaluation fosters the advances in CF care — past, present and future — around the United States.

The *CF Foundation Patient Registry Annual Data Report* has evolved and now will be closely linked to the CF Foundation's Clinical Practice Guidelines Committee, and information related to specific screening procedures will be collected by the registry. Centers will know how they are doing in regard to the use of specific screening tools. An example of this is screening for diabetes. The CF Foundation's *Clinical Practice Guidelines for Cystic Fibrosis*, published in 1997, states that every person age 14 or older should be screened with a blood glucose determination each year. Data analysts are now able to see how many patients actually are screened and then provide feedback to the center.

Q How does the CF registry differ from other disease registries?

A The *CF Foundation Patient Registry Annual Data Report*, is unique because it is generated by the CF care centers. Since CF care centers provide treatments for the majority of the patients with CF in the United States, this makes the *CF Foundation Patient Registry Annual Data Report* one of the most comprehensive ongoing population studies in the United States. It is, and will remain, a vital tool to advance CF patient care. ❁

The medical contents of this newsletter express the opinions of the guest author(s) and are not necessarily the opinions of CF Services, or the Cystic Fibrosis Foundation. Please consult with your caregivers at your local CF care center for guidance.