



Cystic Fibrosis Research News

Title:

Worldwide Rates of Diagnosis and Effective Treatment for Cystic Fibrosis

Lay Title:

Worldwide Estimates of Cystic Fibrosis Diagnosis and Treatment Access

Authors:

Jonathan Guo¹, Anna Garratt², Andrew Hill³

Affiliations:

¹School of Medicine, Faculty of Medicine, Imperial College London, United Kingdom

²Department of Medical Microbiology, Public Health Wales, United Kingdom

³Department of Pharmacology and Therapeutics, University of Liverpool, United Kingdom

What was your research question?

How many people around the world have cystic fibrosis (CF), and of those how many have been diagnosed and how many are receiving the best available treatment?

Why is this important?

Recently traditional preconceptions of cystic fibrosis as an “Anglo-Saxon” disease have been challenged, with it increasingly being viewed as a truly global condition. However, these preconceptions mean we know very little about how many people have CF outside of the western world. As such it is difficult to know where needs are not being met.

Recent innovations in CF care have led to huge improvements in the lives of patients, and if we cannot identify gaps in access to treatment, people with CF in the developing world risk being neglected from these benefits.

What did you do?

We used mixture of publicly available patient registry data, research articles, published sales summaries and an international survey of CF experts and patient organisations in order to make estimates of the total number of people with CF around the world, the proportion who have been diagnosed, and the proportion receiving the best available treatment (currently triple combination CFTR modulator therapy). We used multiple sources of data to maximise the amount of information in countries where high-quality registries don't exist.

Cystic Fibrosis Research News

cfresearchnews@gmail.com



Cystic Fibrosis Research News

What did you find?

Our analysis estimated that 162,400 people have CF worldwide in 94 different countries. Of these people, currently 65% (105,300) are diagnosed and 12% (19,500) are receiving triple combination therapy. In 64 countries no information could be found.

These estimates are higher than those currently quoted in CF science, and many people with CF were found in areas such as the Middle East, South America and Eastern Europe where no CFTR modulators are available.

What does this mean and reasons for caution?

A significant number of people have cystic fibrosis in countries where disease-modifying drugs are not available. Therefore, urgent action is needed to improve rates of diagnosis and treatment for CF, to ensure a higher proportion of patients receive the most effective treatments.

The overall accuracy of our estimates are limited because in many countries where patient registries were not available, very little high-quality information was found and indeed in many areas none at all.

What's next?

A significant number of people with CF live in countries where they have no access to the best treatments, therefore urgent action is needed to ensure they are not neglected. More high-quality research is needed in the developing world to better understand exactly how many people have CF.

Original manuscript citation in PubMed

<https://pubmed.ncbi.nlm.nih.gov/35125294/>

Cystic Fibrosis Research News

