European Cystic Fibrosis Society

Journal of Cystic Fibrosis
The Official Journal of the European Cystic Fibrosis Society

# Cystic Fibrosis Research News

## Title:

**Characterising burden of treatment in cystic fibrosis to identify priority areas for clinical trials**

## Authors:

Gwyneth Davies[1]*, Nicola J Rowbotham[2]*, Sherie Smith[2]*, Zoe C Elliot[3], Katie Gathercole[4,5], Oli Rayner[6], Paul A Leighton[7], Sophie Herbert[2], Alistair JA Duff[8], Suja Chandran[9], Tracey Daniels[10], Edward F Nash[11], Alan R Smyth[2].

*joint first authors

## Affiliations:

1.Respiratory, Critical Care and Anaesthesia Section, UCL Great Ormond Street Institute of Child Health, London, UK
2.Evidence Based Child Health Group, University of Nottingham, Nottingham, UK
3.Parent of Children with CF, Nottingham, UK
4.University of Leeds, Leeds, UK
5.Person with CF, Leeds, UK
6.Person with CF, Plymouth, UK.
7.Centre of Evidence Based Dermatology, University of Nottingham, Nottingham, UK
8.Leeds Teaching Hospitals NHS Trust, Leeds, UK
9.Paediatric CF Service, Kings College Hospital NHS Foundation Trust, London, UK.
10.Department of Physiotherapy, York Hull Adult CF Unit, York Teaching Hospital NHS Foundation Trust, York, UK
11.West Midlands Adult CF Centre, University Hospitals Birmingham NHS Foundation Trust, Birmingham, UK

## What was your research question?

We wanted to find out more about the burden that treatments can cause for people with CF and their families, helping focus future research aimed at simplifying it.

## Why is this important?

"What are effective ways of simplifying the treatment burden of people with CF?" was the CF community's top research question during the recent James Lind Alliance Priority Setting Partnership or "questionCF" as it was known on Twitter and Facebook. We felt that the term 'simplifying treatment burden' needed to be explored further, to make sure future research

in this area concentrates on the issues that are most important to the CF community. This led to the current study.

## What did you do?

We conducted an online survey of the CF community. In this we asked about the number of different treatments that people with CF are on, and how long these take each day. This included asking about their impact on daily life, and what makes things easier or more difficult. We asked healthcare teams whether they would support a trial of stopping or reducing some existing treatments for people with CF taking CFTR modulator drugs (e.g. Kalydeco, Orkambi and Symkevi). The results were then summarised into key topics relating to the many different aspects of treatment burden.

## What did you find?

Nearly 950 people took part. Two thirds were people with CF and their families and a third were healthcare professionals. On average people with CF were on ten treatments, taking two hours each day. Physiotherapy (airway clearance) and long-term nebulised antibiotics were the most burdensome treatments. Common reasons given for this were time taken, dislike, boredom, battles with children, cleaning equipment and no immediate benefit. Difficulty in getting regular medications was also a huge issue.

Almost 80% of healthcare professionals thought that a trial looking at stopping or reducing some existing treatments for people prescribed CFTR modulators was a good idea.

## What does this mean and reasons for caution?

We were able to find out what treatment burden really means to people with CF and their families, which will now allow the CF community to have a more focused approach to clinical trials to simplify treatment burden.

As with all anonymous online surveys, you need to be a little cautious with the results. What people say they do and what they actually do can be quite different. However, because the survey was anonymous people could be more honest without worry of their healthcare team finding out.

## What's next?

The CF community will now be able to focus the design of future clinical studies on the issues which are most important to people with CF and their families, to begin to answer the question of how to reduce treatment burden.



cfresearchnews@gmail.com

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**Original manuscript citation in PubMed**

https://pubmed.ncbi.nlm.nih.gov/31735561-characterising-burden-of-treatment-in-cystic-fibrosis-to-identify-priority-areas-for-clinical-trials/?from_single_result=Characterising+burden+of+treatment+in+cystic+fibrosis+to+identify+priority+areas+for+clinical+trials



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