# Exploring the challenges of accessing medications for patients with cystic fibrosis

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**Abbreviations:** JLA: James Lind Alliance, PSP: Priority Setting Partnership, MDT: Multi-disciplinary Team, PwCF: person with CF, CF: Cystic Fibrosis, Cystic Fibrosis Transmembrane Conductance Regulator (CFTR)

## Background

Cystic Fibrosis (CF) is a genetic condition affecting 1 in 2,500 people. CF is an autosomal recessive disorder, caused by a mutation in the gene coding for the Cystic Fibrosis Transmembrane Conductance Regulator (CFTR). CFTR is a chloride channel, which is found in many body systems including the respiratory tract, the gut and reproductive system. In the apical membrane of respiratory epithelial cells, CFTR function is essential to ensure that cilia lining the respiratory tract are bathed in an aqueous layer. This layer enables cilia to beat in a coordinated fashion, propelling mucus, particulate matter and micro-organisms up the respiratory tract. Lack of CFTR function leads failure of mucociliary clearance and accumulation of

mucus and micro-organisms in the respiratory tract. This in turn leads to infection, inflammation and bronchiectasis. (1)

**Rationale**

CF predominantly affects the lungs; however, the multi-organ disease process can result in CF related diabetes, liver disease, infertility and digestive disruption. (2) Due to the high burden of disease on many organs and the increasing life expectancy, the average CF patient is on eight or more medications daily. (3) The complex treatment regimen in CF patients can include time consuming treatments for example airway clearance techniques, inhaled antibiotics, and enzyme replacements resulting in treatment regimens necessitating on average 137 minutes per child or 150 minutes for an adult every day. (2) (4)

The James Lind Alliance Priority Setting Partnership in Cystic Fibrosis explored the research priorities of the CF community in order to produce a “top 10” list of priorities for clinical research in CF. The first priority was investigating ways to reduce treatment burden. (5) High treatment burden is well recognised within the CF community and is associated with a poorer quality of life. (6) Reduced compliance and subsequent wasted medication, and a potential deterioration in health are consequences of high treatment burden. (7)

‘Treatment burden’ is non-specific terminology used to describe the increased work load associated with health care consequently affecting an individual’s physical and psychological wellbeing. (8) Aspects of treatment burden can include the volume of medication, time taken to administer medications, or how patients access their

prescriptions or medications. Furthermore, access to medications is documented to be a problem for patients with a variety of chronic diseases. (9) (10) Common difficulties include pharmacy stock, medication errors and short durations of prescriptions. (9) (10) (11)

We propose a qualitative observational study to explore how patients with CF access their medications to examine obstacles contributing to their treatment burden and explore potential solutions. We will investigate whether barriers are similar to the non-CF community. The data will be assessed alongside the views and perceptions of both general practitioners (GPs) and community pharmacists.

**Objectives**

1. Review the current system for accessing medications in Primary Care.
2. Explore the difficulties patients with CF experience when accessing their medications in primary care.
3. Review the perception of general practitioners when prescribing medications for patients with CF.
4. Review the perceptions of community pharmacists when reviewing and dispensing medications for patients with CF.

**Outcome**

1. To better understand the number one research priority of reducing treatment burden for a patient with CF.
2. To document the barriers to accessing medications for patients with CF and generate potential solutions.

3. To represent the views of pharmacists and general practitioners when prescribing, reviewing and dispensing medications for patients with CF.

**Study Design**

The James Lind CF2 launched a SurveyMonkey questionnaire in March 2018 focussing on how to simplify treatment burden for patients with CF. A wealth of qualitative and quantitative data were collected from the CF community on treatment burden including their problems of accessing medications. The data relating to accessing medication will be analysed prior to completing the GP and community pharmacists survey.

A SurveyMonkey questionnaire will be developed for both GPs and community pharmacists. The questionnaire for the GPs and pharmacists will be developed with the analysed James Lind CF2 data and guidance of both professions within a small focus group. A mixture of both qualitative, 'free text' and quantitative 'single answer' questions will be incorporated. The questionnaires will be available for 4 weeks from June 2019 or until 100 participants have completed the study.

**Ethics approval**

Faculty of Medicine & Health Sciences Research Ethics Committee deemed this work not to require ethical approval on 30/4/2019.

**Consent**

A privacy policy will be made available within the survey. The questionnaire is anonymous, and no personal data will be stored or financial incentive given to

complete the questionnaire. Completing the questionnaire is optional and can be terminated at any point.

**Recruitment**

Questionnaire participants will be invited to take part in the study on social media, including Twitter and Facebook platforms. The link will be tweeted from the @questioncf account and groups on Facebook such as Women in Pharmacy, NASGP group will be invited to complete the questionnaire. The Royal college of Pharmacists and Royal College of GPs will be contacted to advertise the questionnaire.

**Inclusion and exclusion criteria**

Inclusion criteria for questionnaire specific to that population group:

- GP
- Community Pharmacist
- Not restricted by country

Exclusion criteria:

- Any healthcare professional that is not a GP or Community pharmacist

**Data management and analysis**

The survey data will be downloaded from Surveymonkey into Excel for quantitative analysis and then into NVivo for qualitative analysis.

Quantitative analysis

We will use Excel to present frequency data for the closed questions in the survey to describe the responses and basic demographics of both community pharmacists and GPs.

Qualitative analysis

Free text responses will be analysed in NVivo to identify themes across the data. We will describe the data using word frequencies, word clouds and free text quotes to support our findings.

Comparative analysis

Data from the patient survey previously collected as part of the James Lind CF2 work will be used to compare with views of the GP and community pharmacist responses to highlight any common barriers of accessing medications in the CF community.

**Timeline**

Proposed timeline is to launch the questionnaire in June 2019 with analysis of the data to be conducted in July 2019.

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