**Title:**

CHALLENGES AND OPPORTUNITIES OF ADDING CFTR MODULATORS FOR CYSTIC FIBROSIS TO THE WHO ESSENTIAL MEDICINES LIST

**Lay Title:**

ARE CFTR MODULATORS ESSENTIAL MEDICINES FOR LOW-INCOME COUNTRIES?

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**What was your research question?**

This paper explores the challenges and opportunities of including CFTR modulators, specifically the triple combination elexacaftor/tezacaftor/ivacaftor, in the World Health Organization's Essential Medicines List for patients, policymakers, and healthcare systems.

**Why is this important?**

Listing a medicine on the Essential Medicines List encourages a series of actions, such as prioritising national public health policies, driving international donations, streamlining medicines procurement, promoting subsidies, improving healthcare literacy, and lowering costs to improve access. However, the inclusion of highly-priced medicines addressing the health needs of a limited number of patients can discourage policymakers from using the Essential Medicines List to prioritise the selection of treatments to target the therapeutic needs of the population in low-resource settings, especially in countries characterised by high childhood mortality rates due to infectious diseases. A balancing act is therefore essential for CFTR modulators.

**What did you do?**

We have critically discussed the opportunities and challenges of adding CFTR modulators to the Essential Medicines List.

**What did you find?**

While CFTR modulators represent a significant advancement in the treatment of cystic fibrosis, their high costs and complexity may limit their adoption in clinical practice. To address this issue, we explored various scenarios and identified the pros and cons of different strategies to improve accessibility. These strategies include voluntary (even compulsory) licensing, as well as the necessity of enhancing cystic fibrosis awareness and education in low-income countries.

**What does this mean and reasons for caution?**

Incorporating CFTR modulators into the Essential Medicines List could present a significant opportunity for people living with Cystic Fibrosis worldwide. However, while medicine costs are a major barrier to access, other factors are equally important in ensuring proper use. For CFTR modulators that require genetic testing for diagnosis and eligibility, the availability of the necessary technological infrastructure and tools for correct administration is also essential.

**What’s next?**

Although the availability of CFTR modulators may enhance Cystic Fibrosis awareness globally, other medicines, such as antibiotics or anti-inflammatories, are routinely used in clinical practice. A comprehensive review of treatments for Cystic Fibrosis, adopting a holistic disease-based approach, would therefore improve the core operations and expertise of Cystic Fibrosis Centres.

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