The importance of incorporating CFTR modulator therapy into the Brazilian public health system: one year of advances in cystic fibrosis care.

Renata Wrobel Folescu Cohen[1,2], Patrícia Fernandes Barreto Machado Costa[1], Katty Anne Carvalho Marins[1], Célia Regina Moutinho de Miranda Chaves[1], Tania Wrobel Folescu[1]

TO THE EDITOR,

May 2025 marks one year since the incorporation of elexacaftor/tezacaftor/ivacaftor (ETI), a Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) modulator therapy commercially known as Trikafta®, into the Brazilian Unified Health System (SUS)—a historic milestone for individuals with cystic fibrosis (CF) and for the entire community dedicated to the care and research of this disease in Brazil. This decision was formalized through Ordinance No. 41 of April 27, 2024, published in the Official Gazette of the Union on April 30, 2024,[1] and its use is regulated by the Clinical Protocol and Therapeutic Guidelines (PCDT) for Cystic Fibrosis, established by Joint Ordinance SCTIE/MS No. 17 of April 10, 2024.[2]

Through this letter, we would like to emphasize the importance of this incorporation in reducing CF-related morbidity, as evidenced by real-world data from a national reference center. These data were obtained from the Cystic Fibrosis Reference Center (CRFC) of the Fernandes Figueira National Institute of Women's, Children's, and Adolescents' Health (IFF/FIOCRUZ), located in Rio de Janeiro, Brazil. The CRFC currently provides specialized care to 215 children and adolescents from across the state of Rio de Janeiro, reinforcing its role as a leading institution in the multidisciplinary management, research, and support of individuals living with cystic fibrosis. In the 12 months preceding ETI introduction, a total of 84 CF-related hospitalizations were recorded. In the 12 months following ETI implementation, this number fell to 44, representing a 47.6% reduction in hospital admissions.

When analyzing exclusively the patients who initiated ETI therapy, the benefits were even more pronounced: hospitalizations decreased from 33 in the 12 months prior to treatment initiation to only 5 in the subsequent 12 months, representing an 84.8% reduction within this group. Moreover, no deaths occurred in the 12 months following ETI introduction, compared to 3 deaths registered in the previous period.

These data clearly demonstrate the positive impact of ETI on the lives of individuals with CF, as reflected by the substantial reduction in hospitalizations and the improvement in clinical outcomes, with potential to extend survival and enhance quality of life.

The purpose of this letter is to emphasize the significance of ETI's incorporation into SUS, recognizing it as a collective achievement of the medical and scientific communities, patients, and their families. Furthermore, it is important to highlight the marked reduction in morbidity and hospital admissions within the Brazilian public health system, contributing both to improved health outcomes and more efficient use of healthcare resources.

AUTHOR CONTRIBUTIONS

All authors contributed equally to the conceptualization, data curation, and writing of this letter.

REFERENCES

1. Brasil. Ministério da Saúde. Portaria nº 41, de 27 de abril de 2024. Torna pública a decisão de incorporar, no âmbito do Sistema Único de Saúde - SUS, o medicamento elexacaftor/tezacaftor/ivacaftor (Trikafta®) para o tratamento de fibrose cística. Diário Oficial da União. 2024 abr 30.
2. Brasil. Ministério da Saúde. Portaria Conjunta SCTIE/MS nº 17, de 10 de abril de 2024. Aprova o Protocolo Clínico e Diretrizes Terapêuticas da Fibrose Cística. Diário Oficial da União. 2024 abr 11.

1. Instituto Fernandes Figueira, Fundação Oswaldo Cruz, Rio de Janeiro (RJ), Brasil.
2. Universidade do Estado do Rio de Janeiro, Rio de Janeiro (RJ), Brasil.