**Title:**

Cystic fibrosis year in review 2024

**Lay Title:**

What are the latest advances in Cystic fibrosis literatures in 2024?

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**What was your research question?**

Our aim was to assess the current state of knowledge on cystic fibrosis. In this context, we conducted a review of the scientific literature on cystic fibrosis (CF) published in 2024. Since we’re witnessing a changing landscape, particularly in the aspect of new therapies, we focused on current priorities in CF care, the challenges those involved in the care of people with CF are facing

**Why is this important?**

This review addresses the progress in the treatment of CF through CFTR modulators (CFTRm). As these therapies continue to revolutionize clinical care for patients with CF, it is essential to track their effectiveness and assess best standards for their use. Moreover, key challenges are highlighted, as the global disparities in access to CFTRm. By exploring the benefits and accessibility issues related to CFTRm, this review provides valuable insights for policymakers and healthcare professionals aiming to optimize CF management. Novel therapies and ongoing efforts to make these advancements available to patients worldwide are also discussed.

**What did you do?**

This review offers a focused overview of the most impactful articles on the topic of CF published in 2024. The literature search was systematically performed and authors discussed priority topics and selected the most impactful articles by unanimous judgment.

The selection of impactful articles was based on a literature search followed by a rigorous evaluation by the authors. Criteria included citation potential, novelty, clinical relevance, methodological robustness, and contribution to advancing the understanding or management of CF. Articles were selected through unanimous agreement among the authors, prioritizing those with the highest scientific and clinical significance

**What did you find?**

The latest Standards of Care documents for CF cover key updates about evolving care models, challenges faced by adults with CF, global health concerns, disorders related to CFTR dysfunction (called CFTR related disorders). CFTRm were approved for more genetic variants and have shown benefits across multiple organs, improving digestion, liver health, body composition, and blood sugar levels. De-escalation of therapies is a topic of great interest. CFTRm users reported better overall health and quality of life, but mental health issues are emerging. Parenting showed to be challenging due to the need to balance personal care and family management. New therapies as gene therapies offer hope for the future.

All the studies published this year have been summarized and their key findings highlighted under the main topic discussed in the primary paper.

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

The scientific literature in 2024 highlights the CFTRm efficacy in responsive genotypes and makes us aware of the necessity to update the systems of care and the indications for support therapies in this evolving landscape. Additionally, efforts should be made to monitor patients-reported outcomes and strategies to cope with mental health should be a priority in the coming years. Research should also address inequities and disparities in terms of care and the availability of therapies

**What’s next?**

This review highlights the need for a transformation in care models, incorporating more specializations to address emerging health issues in the ageing population and leveraging digital health advancements. Given that a single treatment model may not be suitable for every patient, new therapeutic approaches and personalized therapy will become one of the primary goals in CF care.

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