

Cystic Fibrosis Research News

Title:

Impact of One-Year Elexacaftor/Tezacaftor/Ivacaftor Treatment on Exercise Capacity in Cystic Fibrosis: A Prospective Observational Study

Lay Title:

Exercise capacity after one year of Kaftrio® treatment in people with Cystic Fibrosis

Authors:

Lue Katrine Drasbæk Philipsen, physiotherapist, MSc in health science^{1,4*}, Hanne Vebert Olesen, MD, PhD², Janne Hastrup Jensen, physiotherapist, MSc in health science³, Mette Frahm Olsen^{4,5}, Daniel Faurholt-Jepsen, MD, PhD^{4,6}, Frederik Buchvald, MD, PhD¹, Kim Gjerum Nielsen, professor, MD, DMSc^{1,6}, Marianne Skov MD, PhD¹, Tacjana Pressler, MD DMSc^{1,4}

Affiliations:

¹ Pediatric Pulmonary Service, Copenhagen CF-Center, Department of Pediatrics and Adolescent Medicine, Copenhagen University Hospital, Rigshospitalet, Copenhagen, Denmark; ² Department of Pediatrics and Adolescent Medicine, Aarhus University Hospital, Aarhus, Denmark; ³ Department of Physiotherapy and Occupational therapy, Aarhus University Hospital, Aarhus, Denmark; ⁴ Department of Infectious Diseases, Copenhagen University Hospital, Rigshospitalet, Copenhagen, Denmark; ⁵ Department of Nutrition, Exercise and Sports, University of Copenhagen, Copenhagen, Denmark; ⁶ Department of Clinical Medicine, University of Copenhagen, Copenhagen, Denmark

What was your research question?

What is the impact of Kaftrio® on physical functioning measured by exercise capacity using cardiopulmonary exercise test (CPET) in people with cystic fibrosis aged 12 and above?

Why is this important?

Commonly used indicators such as lung function and Body Mass Index (BMI) provide insights into the progression of the disease; however, they offer a limited perspective on the impact of the disease on physical functioning, exercise capacity and health-related quality of life. Numerous studies have established a correlation between exercise capacity, health related quality of life and life expectancy in people with cystic fibrosis. CFTR-modulators use has shown improved outcomes, especially in lung function and BMI. To date, only a limited number of studies have evaluated the effect of Kaftrio® on exercise capacity.

Cystic Fibrosis Research News

What did you do?

A large group of 229 Danish individuals with CF, eligible for Kaftrio® treatment, participated in this study conducted between Autumn 2020 and January 2023. Participants underwent cardiopulmonary exercise testing (CPET) before and after one year of treatment. The test was performed on an ergometer bike measuring direct oxygen uptake using a facemask. The test was performed until exhaustion. Various parameters such as peak oxygen uptake (VO₂ peak) was measured. Statistical analysis was performed to assess changes in exercise capacity and explore potential influencing factors.

What did you find?

Results indicated a significant improvement in VO₂ peak after one year of treatment, suggesting a positive response to the medication and improvements were observed in all other secondary outcome measures and parameters like maximum workload and other oxygen uptake variables.

Analysis revealed that factors like age, body mass index (BMI), lung function, and chronic infection status were associated with changes in exercise capacity, the higher the BMI the lower exercise capacity and the higher lung function the higher exercise capacity.

What does this mean and reasons for caution?

The small improvement in exercise capacity was not relevant for the group in total but for the individual patient it could be of relevance. Notably, more than half of the participants showed an increase in oxygen uptake after starting treatment. Increased BMI was also particularly notable, suggesting its potential influence on a decrease in exercise capacity.

This study provides valuable insights into the effects of one-year Kaftrio® treatment on exercise capacity in people with CF above 12 years of age. While improvements were observed, further research is needed to fully understand the short-term and long-term impacts of the treatment on exercise capacity and overall CF management.

What's next?

These findings offer guidance for healthcare professionals in optimizing treatment strategies and supporting physical activity in individuals with CF and helps to explain the effect of the new medication.

Original manuscript citation in PubMed

<https://pubmed.ncbi.nlm.nih.gov/38697864/>