# PROGRAMA DE EXERCÍCIOS DOMICILIÁRIOS EM CRIANÇAS E ADOLESCENTES COM FIBROSE QUÍSTICA

# HOME- BASED EXERCISE PROGRAMME FOR CHILDREN AND ADOLESCENTS WITH CYSTIC FIBROSIS

A presente dissertação académica foi submetida com a proposta de obtenção do grau de doutor do curso de doutoramento em Fisioterapia da Faculdade de Desporto da Universidade do Porto considerando o Despacho n.º 13169/2011, publicado no Diário da República, 2.ª série, n. º189, de 30 de setembro de 2011

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Porto, 2025

Gagulic, S (2025). Programa de exercícios domiciliários em crianças e adolescentes com fibrose quística.
Dissertação académica apresentada com o objetivo de obtenção do grau de doutor em Fisioterapia. Faculdade de Desporto da Universidade do Porto, Porto, Portugal

PALAVRAS-CHAVE: FIBROSE QUÍSTICA, EXERCÍCIO FÍSICO; PROGRAMAS DOMICILIÁRIOS, QUALIDADE DE VIDA RELACIONADA COM A SAÚDE, CUIDADORES, BARREIRAS E FACILITADORES.

**Funding:** This research received no external funding.

Dedico este trabalho a mim,
à minha família
e a todos os que sempre acreditaram que era possível.

# Agradecimentos

Ao concluir, sinto que este trabalho não é apenas o resultado das minhas mãos, mas também de tantas outras que, direta ou indiretamente, o tornaram possível.

A todas as crianças e pais que aceitaram partilhar um pedaço das suas vidas comigo, bem como a todos os médicos e profissionais de saúde que abriram as portas dos seus serviços, o meu mais sincero agradecimento. A vossa generosidade e disponibilidade foram fundamentais para que esta investigação pudesse ganhar forma.

À Professora Doutora Alda Marques, pela orientação inigualável, pela exigência que sempre me desafiou a ser melhor e pela confiança depositada em mim, mesmo nos momentos em que eu própria duvidava. O seu apoio e os seus ensinamentos ultrapassam em muito as fronteiras desta tese.

À Ana Bártolo, por ter estado lá nos momentos mais difíceis, com palavras de conforto e um ombro amigo. A sua ajuda, em particular quando eu pensava em desistir, teve um impacto que nunca esquecerei.

Ao Daniel Martins, que foi essencial na reta final deste processo. A sua paciência e dedicação não só fizeram a diferença no trabalho, mas também me deram forças para acreditar que era possível chegar ao fim.

Aos amigos que, em silêncio, souberam estar presentes. Que entenderam a minha ausência, respeitaram o meu espaço e, ainda assim, me apoiaram incondicionalmente.

Aos meus pais, pelo amor inabalável e por compreenderem que, em tantos momentos, não pude estar tão presente como gostariam. Agradeço-vos por nunca me deixarem esquecer que, não importa o quão longe esteja, há sempre um lugar onde pertenço.

Ao António e ao Damir, o meu porto seguro. Por estarem ao meu lado, por aturarem o meu mau humor nos dias em que a exaustão tomava conta de mim e por nunca me deixarem sentir sozinha, mesmo quando eu não conseguia retribuir da forma que mereciam. Obrigada por serem âncora e farol ao mesmo tempo.

À minha avó, que apesar de não estar presente, esteve sempre comigo no coração. A sua força e sabedoria continuam a guiar-me, como um farol que nunca se apaga.

Esta tese é também vossa.

Com gratidão,

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# Resumo

**Enquadramento**: A fibrose quística é uma doença hereditária crónica e progressiva que afeta profundamente a qualidade de vida das crianças e das suas famílias. O exercício físico é essencial para preservar a função pulmonar, melhorar a aptidão física e promover o bem-estar emocional. Contudo, barreiras logísticas, financeiras e emocionais dificultam a adesão a esta intervenção. Os programas domiciliários de exercício físico destacam-se como uma abordagem prática e efetiva, ajustando-se às rotinas familiares e proporcionando benefícios significativos à saúde e qualidade de vida. Contudo, a sua aplicação em indivíduos com fibrose quística ainda carece de estudos mais robustos, especialmente no que diz respeito ao impacto na capacidade aeróbica, função pulmonar e qualidade de vida.

**Objetivos**: Esta tese teve como objetivos principais: **i)** mapear o impacto multifatorial da fibrose quística, identificando barreiras e facilitadores percebidos pelos cuidadores parentais na gestão da doença em crianças e adolescentes; e **ii)** avaliar a eficácia de um programa domiciliário de exercício físico supervisionado pelos pais, denominado KidMove, na melhoria da capacidade aeróbica, aptidão física, função pulmonar e qualidade de vida relacionada com a saúde.

**Métodos**: Foram realizados dois estudos complementares. O primeiro estudo, qualitativo e exploratório, envolveu entrevistas semiestruturadas. A análise temática indutiva foi utilizada para explorar as perceções dos cuidadores sobre o impacto emocional da doença, os facilitadores e barreiras na gestão da doença e o papel da atividade física. O segundo estudo consistiu num estudo quase-experimental que avaliou a eficácia do programa domiciliário KidMove. Este programa decorreu ao longo de 12 semanas e incluiu 35 exercícios (endurance, força, flexibilidade e equilíbrio), realizados em casa e supervisionados pelos pais. Os participantes foram distribuídos em grupo de intervenção e grupo de controlo com *waiting list*, a capacidade aeróbica foi avaliada através do *modified shuttle walking test*, variável primária, as variáveis secundárias incluíram a avaliação da composição corporal, força muscular, flexibilidade, controlo postural, função pulmonar e qualidade de vida relacionada com a saúde. A análise estatística foi realizada através de modelos de equações de estimativas generalizadas para potenciais fatores de confusão.

**Resultados**: No primeiro estudo, participaram 20 cuidadores (6 pais e 14 mães) de 15 crianças e adolescentes com fibrose quística (6 a 18 anos), que relataram um impacto emocional significativo da doença, particularmente em diagnósticos tardios, sublinhando o papel do conhecimento na adaptação e aceitação desta. Barreiras financeiras, logísticas e emocionais foram apontadas como os principais desafios na adesão ao tratamento e às rotinas exigentes. Contudo, facilitadores como o suporte social, o acesso a informação e a normalização da doença na dinâmica familiar ajudaram a mitigar estas dificuldades. A atividade física foi unanimemente reconhecida como essencial para a saúde das crianças, mas surgiram barreiras importantes, como dificuldades na integração nas rotinas familiares e os custos associados. Estes achados sublinham a importância de intervenções que apoiem toda a família, promovendo estratégias práticas e sustentáveis para a gestão da fibrose quística. No segundo estudo, participaram 46 crianças com 10±4 anos (6 a 18 anos), maioritariamente do sexo masculino (n=24; 52,2%), o programa KidMove demonstrou melhorias na capacidade aeróbica no grupo de intervenção, evidenciada pelos resultados no *modified shuttle walking test* ($\chi^2 = 14{,}24$; $p < 0{,}001$). Melhorias adicionais foram observadas no controlo postural ($\chi^2 = 3{,}89$; $p = 0{,}048$), na flexibilidade dos flexores do joelho ($\chi^2 = 5{,}58$; $p = 0{,}018$) e na funcionalidade emocional das crianças ($\chi^2 = 9{,}34$; $p = 0{,}002$). Apesar de não terem sido registadas mudanças significativas na composição corporal e na função pulmonar, os resultados sugerem que intervenções mais prolongadas ou complementares poderão potenciar estes domínios.

**Conclusão**: Os resultados desta tese sublinham a importância de intervenções personalizadas e centradas nos indivíduos e na família para otimizar a gestão da fibrose quística. O mapeamento do impacto multifatorial da doença destacou a necessidade de apoiar não só as crianças, mas também os seus cuidadores, através de estratégias que reduzam barreiras financeiras e logísticas e promovam o acesso a informação de qualidade. O programa KidMove revelou-se uma abordagem eficaz para melhorar a capacidade aeróbica, aptidão física e funcionalidade emocional, apresentando-se como uma solução prática e acessível para as famílias. Este trabalho destaca ainda a relevância de intervenções domiciliárias, que podem representar uma alternativa viável aos modelos tradicionais de reabilitação realizados em hospitais ou clínicas, promovendo benefícios significativos para a saúde física e emocional das crianças e para o bem-estar global das suas famílias.

**Palavras-chave**: fibrose quística; exercício físico; programas domiciliários; qualidade de vida relacionada com a saúde; cuidadores; barreiras e facilitadores.

# Abstract

**Background**: Cystic fibrosis is a chronic and progressive hereditary disease that profoundly affects the quality of life of children and their families. Physical exercise is essential for preserving lung function, improving physical fitness, and promoting emotional well-being. However, logistical, financial, and emotional barriers hinder adherence to this intervention. Home-based exercise programs are a practical and effective approach, fitting into family routines and providing significant health and quality of life benefits. However, its application in individuals with cystic fibrosis still lacks more robust studies, especially about the impact on aerobic capacity, lung function, and quality of life.

**Objectives:** The main objectives of this thesis were: i) to map the multifactorial impact of cystic fibrosis, identifying barriers and facilitators perceived by parental careers in the management of the disease in children and adolescents; and ii) to evaluate the effectiveness of a home-based physical exercise program supervised by parents, called KidMove, in improving aerobic capacity, physical fitness, lung function and health-related quality of life.

**Methods**: Two complementary studies were carried out—the first study, which was qualitative and exploratory, involved semi-structured interviews. Inductive thematic analysis was used to explore careers' perceptions of the emotional impact of the disease, the facilitators and barriers in managing the disease, and the role of physical activity. The second study consisted of a quasi-experimental study that assessed the effectiveness of the KidMove home-based program. This program ran for 12 weeks and included 35 exercises (endurance, strength, flexibility, and balance), performed at home and supervised by parents. Participants were divided into an intervention group and a control group with a waiting list, aerobic capacity was assessed using the modified shuttle walking test, and the primary outcome and secondary outcome included an assessment of body composition, muscle strength, flexibility, postural control, lung function and health-related quality of life. Statistical analysis was carried out using generalized estimating equation models for potential confounding factors.

**Results**: In the first study, 20 careers (6 fathers and 14 mothers) of 15 children and adolescents with cystic fibrosis (6 to 18 years old) took part. They reported a significant emotional impact of the disease, particularly in late diagnoses, underlining the role of knowledge in adapting to and accepting it. Financial, logistical, and emotional barriers were identified as the main challenges in adhering to treatment and demanding routines. However, facilitators such as social support, access to information, and the normalization of the disease in the family dynamic helped to mitigate these difficulties. Physical activity was unanimously recognized as essential for children's health, but important barriers emerged, such as difficulties in integrating it into family routines and the associated costs. These findings emphasize the importance of interventions that support the whole family, promoting practical and sustainable strategies for managing cystic fibrosis. In the second study, which involved 46 children aged 10±4 years (6 to 18 years), mostly male (n=24; 52.2%), the KidMove program showed improvements in aerobic capacity in the intervention group, as evidenced by the results of the modified shuttle walking test ($\chi^2 = 14.24$; $p < 0.001$). Additional improvements were seen in postural control ($\chi^2 = 3.89$; $p = 0.048$), knee flexor flexibility ($\chi^2 = 5.58$; $p = 0.018$) and the children's emotional functioning ($\chi^2 = 9.34$; $p = 0.002$). Although there were no significant changes in body composition and lung function, the results suggest that longer or complementary interventions could enhance these areas.

Conclusion: The results of this thesis highlight the importance of personalized, individual- and family-centered interventions to optimize the management of cystic fibrosis. Mapping the multifactorial impact of the disease highlighted the need to support not only children but also their careers, through strategies that reduce financial and logistical barriers and promote access to quality information. The KidMove program proved to be an effective approach to improving aerobic capacity, physical fitness, and emotional functioning, presenting itself as a practical and accessible solution for families. This work also highlights the relevance of home-based interventions, which can represent a viable alternative to traditional rehabilitation models carried out in hospitals or clinics, promoting significant benefits for the physical and emotional health of children and the overall well-being of their families.

**Keywords:** cystic fibrosis; physical exercise; home programs; health-related quality of life; caregivers; barriers and facilitators.

## List of abbreviations and symbols

| | |
|---|---|
| % | Percentage |
| ADF-HHD | Ankle dorsiflexors maximum voluntary isometry strength- hand-held dynamometry |
| BMI | Body mass index |
| CF | Cystic fibrosis |
| CFQ-R | Cystic fibrosis questionnaire-revised |
| CFTR | Cystic fibrosis transmembrane conductance regulator |
| CG | Control group |
| CIAFEL | Centro de Investigação em Actividade Física, Saúde e Lazer |
| CINTESIS | Centro de Investigação em Tecnologias e Serviços de Saúde |
| CONSORT | Consolidated Standards of Reporting Trials |
| COREQ | Consolidated Criteria for Reporting Qualitative Research |
| EE, HHD | Elbow extensors maximum voluntary isometry strength- hand-held dynamometry |
| EF-HHD | Elbow flexors maximum voluntary isometry strength- hand-held dynamometry |
| ESSUA | Escola Superior de Saúde da Universidade de Aveiro |
| FADEUP | Faculdade de Desporto da Universidade do Porto |
| FCV | Forced vital capacity |
| $FEV_1$ | Forced expiratory volume in one second |
| GEE | Generalized estimating equations |
| HAB-HHD | Hip abductors maximum voluntary isometry strength- hand-held dynamometry |
| HAD-HHD | Hip adductors maximum voluntary isometry strength- hand-held dynamometry |
| HE-HHD | Hip extensors maximum voluntary isometry strength- hand-held dynamometry |
| HF-HHD | Hip flexors maximum voluntary isometry strength- hand-held dynamometry |
| HR | Heart rate |
| HRQoL | Health-related quality of life |
| iBiMED | Instituto de Biomedicina |
| IG | Intervention group |

| | |
|---|---|
| INSIGHT | Research Center for Ecological Human Development |
| KE- HHD | Knee extensors maximum voluntary isometry strength- hand-held dynamometry |
| KF-HHD | Knee flexors maximum voluntary isometry strength- hand-held dynamometry |
| Kg/F | Kilogram-Force |
| $Kg/m^2$ | Kilogram per square meter |
| KidMove | Tailored home-based exercise program for kids with cystic fibrosis |
| Lab3R | Respiratory Research and Rehabilitation Laboratory of the School of Health Sciences, University of Aveiro |
| MSRT | Modified sit and reach test |
| MSWT | Modified Shuttle Walking Test |
| *p* | p-value |
| QIC | Quasi-likelihood information criterion |
| RM | Repetition maximum |
| RNA | Ribonucleic acid |
| SAB-HHD | Shoulder abductors maximum voluntary isometry strength- hand-held dynamometry |
| SD | Standard deviation |
| SEBT | Star excursion balance test |
| SF- HHD | Shoulder flexors maximum voluntary isometry strength- hand-held dynamometry |
| SPSS | Statistical package for the social sciences |
| T0 | Baseline |
| T1 | After intervention |
| TIDiER | Template for Intervention Description and Replication |
| UPT | Universidade Portucalense |
| $VO_2$ | Peak oxygen consumption |
| WE-HHD | Wrist extensors maximum voluntary isometry strength- hand-held dynamometry |

# Chapter 1. General introduction

## Introduction

Chapter I aims to provide a general introduction to the state of the art and the main topics needed to write this thesis.

## Cystic fibrosis: overview of the disease and its impacts

Cystic fibrosis (CF) is a severe genetic disease affecting 70.000-90.000 individuals worldwide (Rey et al., 2019), with varying prevalence in different populations (Gramegna et al., 2024). In 2018, the estimated number of diagnosed individuals in Europe was 47,650 diagnosed individuals in 40 countries with a mean prevalence of 0.548±0.48 in a population up to over 513 million. In Portugal, the prevalence rate is 2.97, with a prevalence of around 307 individuals and an incidence rate of less than 14.3.

CF is caused by mutations in the CF transmembrane conductance regulator (CFTR) gene in chromosome 7 (Hanssens et al., 2021). The CFTR gene encodes a protein essential for regulating chloride ion transport across epithelial cells, critical for maintaining fluid balance in the lungs and digestive system (Chen et al., 2021). There are six classes of CF based on the type of CFTR gene mutations. Class I mutations produce no CFTR protein, such as in the case of mutations like G542X. Class II mutations, exemplified by the ΔF508 mutation, lead to the production of CFTR protein that is improperly processed and subsequently degraded. Class III mutations allow the CFTR protein to reach the cell surface but render it dysfunctional, such as with the G551D mutation. Class IV mutations produce CFTR proteins that reach the cell surface but function at reduced efficiency (e.g., R117H), while Class V mutations result in reduced amounts of CFTR protein being produced (e.g., A455E). Finally, Class VI mutations cause CFTR proteins to be unstable at the cell surface, leading to various clinical symptoms (Bell et al., 2020; Shteinberg et al., 2021).

The diagnosis of CF is a systematic process that begins with clinical evaluation and newborn screening, followed by confirmatory tests like the sweat test and genetic analysis (Bell et al., 2020). Early diagnosis is crucial for effective management and improved clinical outcomes, such as respiratory infections, hospitalization, and nutritional status for individuals with CF (Coverstone & Ferkol, 2021).

CF is commonly divided into classical and non-classical forms. Classical CF is characterized by severe involvement of the pulmonary and pancreatic systems, resulting

in frequent respiratory infections and malabsorption problems (Hanssens et al., 2021). In contrast, non-classical CF tends to have milder symptoms, often limited to the lungs or other organs, without pancreatic insufficiency (Coverstone & Ferkol, 2021; Dickinson & Collaco, 2021).

Pulmonary and extrapulmonary manifestations have a significant impact on the functional status of individuals with CF, which is an important aspect of their health-related quality of life (Sergeev et al., 2020). Functional status is defined as an individual's ability to perform daily activities that are essential to meet basic needs, maintain usual roles, and maintain overall health and well-being (Dickinson & Collaco, 2021).

Therefore, a comprehensive understanding of the clinical manifestations of CF is essential as they directly affect the functional capacity performance and overall quality of life of affected individuals (Gramegna et al., 2024; Sreenivasulu et al., 2023). Declining functional status in individuals with CF is associated with increased dependence on caregivers, substantial productivity losses, and an increased burden of disease-related costs (Daly et al., 2022). However, the literature on strategies to support sustained parental involvement and prevent caregiver burnout remains limited (Daly et al., 2022; Fitzgerald et al., 2018).

CF significantly reduces individuals' functional status, affecting their ability to perform daily activities and diminishing their overall quality of life due to both pulmonary and extrapulmonary complications (Shteinberg et al., 2021). Impaired CFTR activity reduces airway surface liquid and increases the viscosity of mucociliary secretions, creating conditions that encourage obstruction and inflammation (Shteinberg et al., 2021). As a result, thick, sticky mucus accumulates, blocking airways and ducts in various organs, particularly the lungs and pancreas (Morrison et al., 2019). In the lungs, this mucus buildup leads to chronic infections and inflammation, progressively diminishing lung capacity and respiratory function over time (Ahmed & Mukherjee, 2018). Individuals often experience persistent cough, shortness of breath, and fatigue, all of which limit their ability to engage in physical activities like exercise, sports, or even routine tasks such as climbing stairs (Bell et al., 2020). These respiratory issues are further compounded by recurrent infections, which frequently require hospitalization and extended antibiotic treatments. The disruptions from these hospitalizations interfere with daily life, education, or work, often isolating individuals socially due to missed interactions and the demands of ongoing medical care (Prieur et al., 2021).

In addition to pulmonary complications, CF also severely impacts the digestive system (Castellani et al., 2023). The disease affects the pancreas and other digestive organs, impairing nutrient absorption and leading to malnutrition, poor growth, and gastrointestinal issues like constipation and abdominal pain. Infants with CF may experience intestinal blockages (meconium ileus), and as they age, individuals often contend with conditions such as gastroesophageal reflux disease and distal intestinal obstruction syndrome. These digestive issues make it challenging to maintain adequate nutrition, which exacerbates fatigue and physical weakness (Chen et al., 2021). Other systemic complications include CF-related diabetes, which requires careful blood sugar management, and liver disease, adding complexity to the daily care regimen and increasing the risk of severe health problems (Granados et al., 2019).

CF also imposes significant physical limitations, as many individuals experience reduced bone density, muscle wasting, and musculoskeletal issues (Southern et al., 2024; VanDevanter et al., 2016). Due to malnutrition and limited physical activity, CF individuals are prone to osteoporosis, fractures, and posture-related problems such as kyphosis (Gruet et al., 2017). These musculoskeletal complications decrease mobility and independence, particularly as individuals become more vulnerable to injuries and find it increasingly difficult to stay active (Southern et al., 2024). Furthermore, physical exhaustion and reduced exercise tolerance are persistent challenges for CF individuals. Though exercise is beneficial, fatigue, shortness of breath, and muscle weakness make it challenging to meet recommended activity levels, limiting opportunities for social engagement, personal fitness, and a sense of normalcy in everyday life (Radtke et al., 2022).

Beyond physical impacts, CF takes a toll on mental health, with individuals frequently experiencing anxiety, depression, and isolation stemming from the chronic and unpredictable nature of the disease (Quittner et al., 2016). The daily burden of managing treatments, fear of disease progression, and potential social stigma can strain mental well-being, further limiting engagement in social and recreational activities. Additionally, the time-intensive nature of CF care — including frequent nebulizer treatments, physiotherapy, and complex medication schedules — restricts individuals' and their families' abilities to engage in spontaneous activities or social interactions (Castellani et al., 2023). Teenagers may feel self-conscious about their condition, avoiding social settings where symptoms may be visible or misunderstood (Denford et al., 2020).

The cumulative impact of these pulmonary, digestive, and psychosocial challenges leads to a substantial reduction in the quality of life (Giannakoulakos et al., 2022) balancing health needs with education, career, and personal aspirations is a continuous challenge for individuals and caregivers alike (Daly et al., 2022; Lum, 2017). The physical constraints imposed by respiratory and digestive issues, combined with emotional and social stressors, diminish independence and limit opportunities for a fulfilling life, leading to lower overall well-being (Gruet et al., 2022). Consequently, comprehensive CF management aims not only to address symptoms but also to enhance individuals' functional status, quality of life, and emotional resilience through holistic, person-centered care (Southern et al., 2024).

Despite advances in pharmacotherapy, including CFTR modulators such as ivacaftor, gene editing technologies, and RNA-based therapies, CF remains a chronic multisystem disease with significant morbidity. While these therapies improve chloride transport and reduce mucus viscosity, they are not curative (Tewkesbury et al., 2022).

As a result, individuals continue to experience progressive lung damage, frequent infections, and comorbidities such as obesity, hypertension, CF-related diabetes, liver disease, and bone disease (Bell et al., 2020; Sergeev et al., 2020).

In addition to the physical limitations, the financial, social, and emotional burden of CF is significant (Desai et al., 2023). The annual costs of managing CF range from €18,745 to more than €34,530 per person (Angelis et al., 2015; Daly et al., 2022) and include the costs of medications, frequent hospitalizations, outpatient care, and lost productivity (Desai et al., 2023). These costs, combined with the need for frequent doctor visits, hospitalization, and costly therapies, place a heavy burden on individuals and their families. Indirect costs from time off work and school further impact quality of life, relationships, and career opportunities, highlighting the need for a comprehensive support system (Daly et al., 2022; Fitzgerald et al., 2018). Balancing complex treatment regimens with work and family responsibilities, along with the anxiety about the unpredictability of the disease and the potential for caregiver burnout can be challenging (Daly et al., 2022; Quittner et al., 2016).

Effective long-term management of CF, therefore, requires a holistic approach that integrates both pharmacological treatments and non-pharmacological strategies, with exercise playing a particularly key role in improving health outcomes and overall well-being (Southern et al., 2024). In this context, personalized exercise programs are

increasingly seen as an important complement to traditional therapies, helping to address the physical and mental challenges associated with CF (Gruet et al., 2022). Given the profound impact of CF on physical and mental health, integrating strategies such as exercise into its management is crucial for improving both functional status and quality of life.

## The role of physical exercise in cystic fibrosis management

Physical exercise is an essential component of CF management, complementing pharmacological treatments and contributing to improved health outcomes (Gruet et al., 2022). Initially, exercise was recommended primarily to enhance mucus clearance; however, its benefits have expanded to encompass various physiological and psychosocial dimensions (Radtke et al., 2022).

Regular endurance and resistance exercise training is essential for preserving lung function, respiratory muscle strength, and exercise capacity (García-Pérez-de-Sevilla et al., 2022; Gruet et al., 2022; Radtke et al., 2022) health-related quality of life (HRQoL) (Giannakoulakos et al., 2022) by promoting social integration, enhancing self-esteem, and fostering independence.

A systematic review of 15 randomized controlled trials with a total of 456 participants with CF highlighted that physical exercise training, including both aerobic and anaerobic exercises, can positively influence exercise capacity and pulmonary function in individuals with CF (Radtke et al., 2017). Although limited evidence exists for specific outcomes (e.g., exercise capacity, lung function, hospitalization frequency), exercise has been established as a crucial element of multidisciplinary CF care due to its overarching benefits (Kinaupenne et al., 2022). Importantly, adverse effects related to physical activity are infrequent, and there is no rationale for discouraging regular exercise (Radtke et al., 2022).

The benefits of physical exercise may depend on factors such as the type and duration of the program, the presence of CF-related complications, and personal preferences or barriers to exercise (Giannakoulakos et al., 2022). However, the World Health Organization guidelines for physical activity and sedentary behaviour apply to individuals with CF the optimal type, duration, and intensity of exercise for individuals with CF is still unknown (Gruet et al., 2022; Radtke et al., 2022). Some authors suggest that it is recommended to include aerobic exercise at least three times a week, ideally for

60 minutes daily at a minimum intensity of 70% of maximal heart rate, and resistance training 2–3 times a week, targeting both limb and trunk muscles with 1–3 sets of 8–12 repetitions at 70–85% of 1-RM (Cox et al., 2023; Del Corral et al., 2018; Denford et al., 2020; Gruet et al., 2022; Hommerding et al., 2015; Puppo et al., 2020; Radtke et al., 2017). Incorporating these principles is crucial for designing effective exercise programs aimed to optimize health and performance.

Exercise programs should be personalized and developed collaboratively with the person with CF and their family, accommodating individual preferences for environment, type, duration, intensity, and frequency (Shelley et al., 2022). Exercise not only supports lung function and physical fitness but also contributes positively to mental well-being, helping individuals build resilience and cope with the ongoing challenges of CF (Radtke et al., 2017, 2022). By prioritizing both physical and emotional needs, such person-centered, non-pharmacological strategies provide an accessible and holistic approach to CF management, improving quality of life and enabling a more balanced, sustainable approach to living with the disease (Gramegna et al., 2024). Providing choice, variety, and flexibility can enhance engagement, enjoyment, and long-term adherence, fostering self-efficacy and resilience in managing unexpected challenges (Gruet et al., 2022; Shelley et al., 2022).

Given the multisystemic impact of CF, particularly on lung function and muscle strength, exercise should be considered a vital component of comprehensive CF management (Radtke et al., 2022). However, despite the physiological benefits of exercise, many individuals with CF may find it challenging to incorporate such an exercise commitment into their daily routines due to the level of dedication required (Gruet et al., 2022). This emphasizes the importance of balancing ideal physiological recommendations with realistic, adaptable approaches that fit seamlessly into everyday life for individuals with CF (Radtke et al., 2022).

However, despite its recognized benefits, children with CF often face unique challenges that limit their participation in regular physical activity, as explored in the following section.

**Barriers to exercise in children with CF**

Despite the well-established benefits of exercise, many children with CF face significant barriers to regular participation in exercise activity. These barriers are multifaceted and can be broadly categorized into physical, psychological, and logistical challenges.

Although physical activity is widely recognized for its health benefits, many children, teenagers, and young adults with CF do not meet recommended activity levels (Radtke et al., 2022). Barriers to physical activity in children, teenagers, and young adults included a lack of enjoyment, low awareness of perceived health benefits, and insufficient social support from family and peers (Southern et al., 2024). Barriers to participation involved changing the state of health, competing medical treatments, physical discomfort (e.g., fatigue and breathlessness), and psychological factors such as self-consciousness and negative perceptions of exercise. Parental involvement and access to exercise facilities were also deemed crucial for fostering consistent physical activity, understanding the role of parental support in encouraging active lifestyles, as well as the impact of an accessible and safe exercise environment, is essential for promoting long-term exercise habits in CF (Gruet et al., 2022).

Physical limitations such as chronic fatigue, dyspnoea, and exercise-induced bronchoconstriction are common in children with CF and may inhibit regular exercise participation (Radtke et al., 2017). Additionally, the fear of exacerbating respiratory symptoms or sustaining injury may lead both children and caregivers to avoid physical exercise (Radtke et al., 2022).

Psychologically, children with CF may feel embarrassed or self-conscious about their condition, particularly in social settings that involve physical activity, such as sports or physical education classes (Denford et al., 2020).

Logistic challenges also present barriers to regular exercise (Radtke et al., 2017). The time-consuming nature of CF treatments, such as airway clearance techniques and nebulized medications, can significantly reduce the time available for physical activity (Daly et al., 2022). In addition, access to specialized rehabilitation facilities, and structured exercise programs tailored to the needs of children with CF and based on a conceptual health promotion model may be limited, particularly in rural or underserved areas.

These barriers underscore the importance of innovative solutions such as home-based exercise programs, which are tailored to address these challenges while promoting long-term adherence.

**Home-based exercise programs: an emerging solution**

In response to the challenges faced by children with CF, home-based exercise programs have emerged as a promising alternative to traditional clinic-based rehabilitation (Gruet et al., 2022; Junior et al., 2023)

These programs offer numerous advantages, including greater flexibility, reduced logistical burdens, and the ability to tailor exercise regimens to individual needs and preferences (Uzzaman et al., 2022). Tailored home-based exercise programs, designed to be adaptable and enjoyable, allow CF individuals and their families to integrate physical activity into their daily routines, reducing the logistical and psychological barriers associated with clinic-based exercise (Gruet et al., 2022). These programs also foster social integration and enhance family involvement, as they often encourage caregivers and family members to participate actively in the exercise regimen, which strengthens support networks and reinforces the patient's commitment to maintaining health routines (Barnes et al., 2020a).

The rationale behind implementing home-based exercise programs for children with CF is multifaceted. Primarily, the psychological burden associated with CF management can lead to feelings of isolation, anxiety, and depression among young individuals (Daly et al., 2022). Engaging in home-based exercise provides an opportunity for autonomy, allowing children and teenagers to take an active role in their care (Gruet et al., 2022). This empowerment is crucial for enhancing self-esteem and fostering a positive self-image, particularly as children navigate the complexities of their condition (Sreenivasulu et al., 2023). Moreover, the flexibility of home-based programs allows families to integrate physical activity into their daily routines, overcoming logistical barriers associated with traveling (Junior et al., 2023). For many families, the time and energy required to attend regular rehabilitation sessions can be overwhelming, especially when compounded by the demands of daily CF treatments. Home-based programs alleviate this burden, enabling families to prioritize physical activity without sacrificing essential treatment time (Uzzaman et al., 2022).

Effective home-based exercise programs should incorporate a variety of activities tailored to individual fitness levels and preferences. Aerobic exercises, such as cycling, jogging, and swimming, are essential for enhancing cardiovascular endurance and promoting mucus clearance. Resistance exercises, utilizing bodyweight movements and resistance bands, are crucial for strengthening muscles and supporting bone density (Del Corral et al., 2018; Gruet et al., 2022; Southern et al., 2024). Additionally, breathing exercises play a vital role in improving respiratory function (Williams & Stevens, 2013).

To enhance adherence to home-based exercise programs, it is essential to involve individuals in the design of their exercise regimens, ensuring that activities are enjoyable and aligned with their preferences. Gamification strategies, such as fitness mobile software and interactive platforms, have shown promise in increasing engagement among younger individuals. For instance, applications that track progress, set goals, and provide rewards for achievements can motivate children to participate consistently (Ambros-Antemate et al., 2023).

Educating families about the importance of physical activity and the benefits of home-based programs is critical to fostering a supportive environment conducive to adherence. Family involvement not only increases motivation but also facilitates supervision, which is essential to ensure that children adhere to prescribed exercises (Barnes et al., 2020a; Prieur et al., 2021). This integration of exercise and family-centered support reflects a shift towards comprehensive CF care that values patient empowerment and long-term well-being as central outcomes (Gruet et al., 2022). Parents play a central role in supporting their child's adherence to exercise routines to ensure maximum therapeutic benefit. Research showed that parental engagement, combined with virtual supervision, is associated with significant improvements in pulmonary function and a reduction in respiratory exacerbations (Adair et al., 2022).

Home-based exercise programs offer practical benefits for individuals with CF, but adherence can be challenging due to various psychological, physical, and logistical factors (Gruet et al., 2022). The demands of CF, including its psychological toll, logistical challenges, and low motivation levels, can make consistent exercise difficult. Additionally, effective home-based exercise may require equipment or technology that might not be accessible to all families (Uzzaman et al., 2022). To address these barriers, providing guidance on affordable exercise options and encouraging families to leverage community resources is essential (Dillenhoefer et al., 2022).

Research on tailored home-based exercise programs for individuals with CF is currently limited, particularly regarding their impact on critical health outcomes such as aerobic capacity, lung function, and overall quality of life (Goetz et al., 2024; Radtke et al., 2022). There is also a lack of understanding of the specific barriers and facilitators that influence caregiver engagement in these programs. Maintaining sustained engagement in home-based exercise programmes is challenging and strategies to ensure consistent participation are needed. Socioeconomic and cultural factors are important determinants of parental support for these programs, but their influence on caregiver participation has not been adequately explored.

Beyond these logistical and practical issues, the psychosocial impact on parents of managing CF routines and supporting home-based exercise programs is largely overlooked. The emotional and mental demands placed on caregivers may influence their ability to consistently engage in and support these routines for their children. Addressing these research gaps and providing comprehensive support for both individuals and caregivers are essential steps toward fully realizing the benefits of home-based exercise programs in CF management.

While home-based exercise programs have shown promise in overcoming logistical barriers, their efficacy in improving health outcomes in children with CF, particularly when combined with parental supervision, remains underexplored. Moreover, the psychosocial impact on caregivers supporting these interventions warrants further investigation.

Research on tailored home-based exercise programs for individuals with CF reveals significant gaps that require further exploration. These programs show potential in addressing logistical barriers to exercise, yet their effectiveness in improving critical health outcomes, including aerobic capacity, lung function, and HRQoL, remains underexplored. Factors influencing caregiver engagement, such as barriers, facilitators, and the role of socioeconomic and cultural determinants, are not well understood.

Long-term participation in home-based exercise programs continues to be a challenge, highlighting the need for strategies to promote consistent adherence. Caregivers face substantial emotional and mental demands when managing CF routines and supporting these programs, but the psychosocial impact of these demands has

received little attention. This lack of focus on caregiver well-being and its effects on engagement creates a crucial research gap.

Unresolved issues related to caregiver involvement, parental supervision, and cultural and psychosocial influences must be addressed to enhance the design and implementation of these programs. Bridging these gaps is essential to fully realize the benefits of home-based exercise interventions in CF management.

## Objectives

The relevance of this thesis lies in addressing the need to develop effective and sustainable interventions to manage the multifactorial impact of CF on children and their families. It focuses on overcoming barriers to treatment adherence, particularly regarding exercise, which is crucial for improving health outcomes.

Thus, this thesis aimed to map the multifactorial impact of CF and identify the barriers and facilitators perceived by parental caregivers in managing the disease in paediatric individuals, and explore the effectiveness of a tailored home-based exercise program supervised by parents in improving aerobic capacity, physical fitness, lung function, and HRQoL in children with CF.

These objectives were achieved through two original studies presented in Chapters II and III.

The thesis is organized into seven chapters. Chapter I, provides a general introduction, including an overview of CF and its impacts, the role of physical exercise in CF management, barriers to exercise in children with CF, and the potential of home-based exercise programs as an emerging solution. Chapter II, titled *"Mapping perceived impact, facilitators and barriers of cystic fibrosis management in children and adolescents: a qualitative study from the parents' perspective"* presents the findings of a qualitative study that explores caregivers' experiences and perceptions of managing CF in children. Chapter III, titled *"Effects of a tailored home-based exercise program KidMove in children with cystic fibrosis: a quasi-experimental study,"* evaluates the effectiveness of a supervised home-based exercise program in improving physical and emotional outcomes in children with CF. Chapter IV presents the general discussion and critical reflection, integrating the findings from both studies and situating them within the broader literature. Chapter V outlines the main conclusions, practical recommendations, and perspectives for future research. Chapter VI compiles the bibliographical references supporting Chapters I and IV. Lastly, chapter VII contains the appendices and annexes respectively, relevant to the thesis.

## Chapter 2. Original study I

# Mapping perceived impact, facilitators and barriers of cystic fibrosis management in children and adolescents: a qualitative study from the parent's perspective

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Submitted to: Scandinavian Journal of Caring Sciences

Waiting for editor's decision after response to reviewers

**Abstract**

**Background:** Cystic fibrosis imposes a significant treatment burden on children and their informal caregivers, who have to change their routines to carefully adhere to medication and exercise as treatment regimes. Although informal caregivers are known to be key players in the daily management of these children, their voice is scarcely explored, often hindering the personalization of care. The main objective of the study was to map the multifactorial impact of cystic fibrosis, as well as identify barriers and facilitators perceived by parental caregivers in managing the disease at the paediatric age.

**Methods:** A qualitative exploratory study was conducted involving six fathers and 14 mothers of 15 children and adolescents (6-18 years; 40% male) diagnosed with cystic fibrosis. Semi-structured individual interviews were conducted. Data were analyzed using inductive thematic analysis.

**Results:** Four main themes emerged from the analysis: 1) perceived impact of the disease; 2) facilitators of disease management; 3) adherence to treatment; and 4) physical activity. Findings emphasized the emotional impact of the disease, especially when diagnosed at a later stage of development. Increased knowledge seemed to facilitate adaptation and daily management, as well as a normalization of attitudes by parents. All parents recognized physical activity as an important part of treatment, although financial and logistical factors (e.g. reconciling schedules) were important barriers to adherence.

**Conclusions:** Our findings suggest that disease management and specifically adherence to treatment recommendations is impacted by early diagnosis, attitudes towards the disease, social support, and financial constraints. Future interventions should focus on identifying the needs and supporting the whole family to cope with the demands of the disease, namely by improving knowledge about the benefits of different intervention approaches.

**Keywords:** family caregivers, paediatric, chronic respiratory disease, cystic fibrosis, adherence.

## Introduction

Cystic fibrosis (CF) is a rare multifactorial genetic disease (1). According to the European Cystic Fibrosis Society (2), 54043 cases of CF have been registered in Europe. Of these, 46% are children with an average age of diagnosis of three and a half months. Despite the advances in understanding the disease and its associated therapies resulting in individuals' lung function and survival improvements (3), quite complex and time-consuming treatments need to be undertaken on a daily basis (4).

Parents assume, in most cases, the role of main informal caregivers (5) and have to deal with the demands of adhering to, delivering, and supervising CF-specific therapies, medical appointments, hospital admissions, financial difficulties, and uncertainty about their children's future (6,7). Taken together, these aspects add more stress to their roles and increase conflict with adolescents (8). Moreover, children and adolescents deal daily with the need to manage the challenges of disease and developmental transitions (9). Children and parents are, therefore, both at a considerable risk of developing psychological symptoms, such as depression and anxiety, and a poor quality of life as a result of these responsibilities (9,10).

Recent research has indicated an association between depressive symptoms in children with CF and their parents and low confidence in medical treatments, which may have an impact on treatment compliance (11). Adherence to physical exercise in CF is not always considered a priority by family caregivers (12-14), although it is currently seen as a crucial part of integrated CF treatment (15). Thus, non-adherence to the different CF intervention regimens can have serious implications for children's health, leading to an increase in respiratory exacerbations, hospitalizations, and loss of lung function, which are leading causes of premature death related to the disease (16). It is, therefore, important to understand the perceived impact of the disease from the caregiver's perspective, playing a fundamental role at this stage of development, and the factors that affect management and consequent adherence to different medical recommendations, such as medication and physical exercise.

Several studies have already focused on understanding parents' perceptions and experiences of the physical and emotional impact of CF (5,7,9), as well as the changes caused in family dynamics (17). However, to date and the best of our knowledge, the barriers and facilitators of disease management and the reasons for non-adherence to an integrated treatment regimen, from the perspective of caregivers, are largely unexplored.

This knowledge can contribute to identifying factors that should be targeted when personalizing the interventions to improve disease management and adherence in this context. The main objective of the study was to map the multifactorial impact of CF, as well as identify barriers and facilitators perceived by parental caregivers in managing the disease at the paediatric age.

## Methods

### Study design and ethics

An exploratory qualitative study was carried out to map the multifactorial impact of CF from the parents' perspective (18). This study is a subset of a larger project that adheres to the Declaration of Helsinki (19) and has received ethics committee approval. All participants were informed that their involvement in the study was voluntary and confidential. Written informed consent was obtained before any data collection. This study is reported in accordance with the Consolidated Criteria for Reporting Qualitative Research (COREQ) checklist (20) to ensure transparency, rigor, and comprehensiveness of the study context and its findings.

### Research team and reflexivity

Three authors were involved in the analytical process to ensure trustworthiness, confirmability (i.e., researcher objectivity), and reflexivity. The team's diverse expertise and professional backgrounds informed the authors' pre-understandings, directly influencing decisions from the selection of the research topic to data interpretation. The first author, a physiotherapist and PhD student with extensive clinical experience in CF, contributed to a practical, patient-centered perspective that guided the development of the interview guide and facilitated a sensitive approach to data collection. This expertise necessitated reflexivity to mitigate potential bias stemming from her familiarity with CF care. The second author, an experienced qualitative researcher with a PhD, systematically reviewed transcripts and contributed to the analytical and interpretive phases. Her expertise in qualitative methodologies ensured analytical rigor, facilitating robust theme identification and balancing the first author's clinical perspective with methodological objectivity. The third author, a PhD researcher specializing in respiratory health, reviewed thematic coding and engaged in interpretive discussions with other authors. Her scientific background in respiratory care brought depth to the CF-specific analyses while

upholding scientific rigor. The absence of pre-existing relationships between researchers and participants minimized interpersonal bias. Through regular reflexive discussions, the team critically engaged with their pre-understandings, reducing personal influence on the analytical process. Each researcher's unique expertise enriched the interpretive framework, strengthening both the depth and validity of the study's findings (21).

**Participants and recruitment**

Recruitment was performed in a Paediatric hospital in Portugal, using a purposive sampling technique. A subset of parents included in a larger project aiming at investigating the effects of a home-based exercise intervention on CF children that accepted to participate were included in this study. Children were eligible for inclusion in the study if they (i) were 18 years of age or younger; (ii) were not mechanically ventilated (iii) had a respiratory function test result of $FEV^1 > 40$ (22); and (iv) regularly attended their child's hospital appointments. All parents whose children had a current acute respiratory exacerbation identified by an attending physician were excluded. Clinicians at the institution identified and contacted eligible individuals explained the purpose of the study, checked their willingness to participate, and referred those interested in taking part to the main researcher. All parents of individuals who met the inclusion criteria accepted to participate in the study.

**Data collection**

In-depth individual interviews were conducted to explore the perceptions of parents of children or adolescents diagnosed with CF. A semi-structured interview guide with open-ended questions was developed and pilot-tested with two caregivers before collecting data. After small adjustments in the language, the questions focused on mapping parents' perceptions of the impact of the disease on their child's life and their own, such as daily management and treatment adherence, as well as their relationship with physical activity (Table 1). A researcher trained in qualitative research conducted the interviews face-to-face in a private room at the hospital. Field notes were taken to register any observations or non-verbal responses of participants during the interviews which could contribute to inform future data analysis. The interviews lasted between 20 and 60 minutes, were

audio-recorded (Tascan DR-05X (TEAC America Inc., Santa Fe Springs, CA), and transcribed verbatim.

Table 1 – Main questions included in the semi-structured interview script directed to parents of children/adolescents with cystic fibrosis.

| Question number | Interview script |
|---|---|
| **1** | What impact does the disease have on your family? |
| **2** | How do you manage time to care for your child? |
| **3** | Do you mind sharing with me the organization of your day? |
| **4** | What do you think about your child's medical condition? |
| **5** | What is your opinion on your child's possible participation in sports or other activities involving physical activity? |
| **6** | What is your child's opinion of physical activity? Any favourite? If yes, why? |

**Data processing and analysis**

Data were analyzed using an inductive thematic analysis grounded in constructivist epistemology, which asserts that knowledge is co-constructed through participants' subjective experiences. Themes and sub-themes were derived directly from these experiences, with codes assigned to relevant segments of text (Figure 1). Two researchers independently analyzed the data, following Braun & Clarke's six-step procedure: (i) familiarization with the data, (ii) initial coding, (iii) theme identification, (iv) theme review, (v) theme definition and naming, and (vi) reporting (21). This structured approach allowed for reflexive interpretation of participants' narratives. The ATLAS.ti software (version 22) facilitated the organization of codes into potential themes, ensuring rigorous analysis aligned with our research objectives. To enhance the study's rigor, we followed Lincoln and Guba's guidelines (23). Credibility was sought by using triangulation, where multiple researchers analyzed and discussed themes to reach a consensus. Transferability

was achieved via purposive sampling to ensure diverse representation across demographic variables. Dependability was reinforced through regular team discussions and systematic coding processes, while confirmability was ensured through transparent documentation, reflective journaling, and an audit trail. Data saturation was defined as the point where additional data ceased to yield new themes (24). By situating our analysis within a constructivist framework, we emphasized that participants' realities and social contexts are crucial for understanding the complexities of managing CF, enriching our findings and contributing to a deeper comprehension of the lived experiences of parental caregivers.

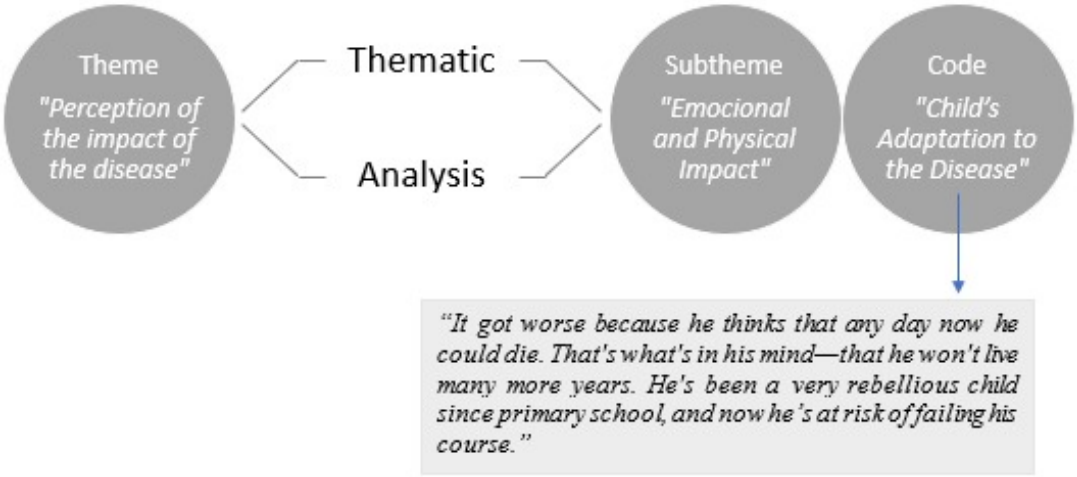


**Figure 1** – Data analysis process example: thematic analysis.

## Results

### Participants' characteristics

Thirty-two eligible individuals were identified. Of these, 20 (14 mothers and six fathers) of 15 children were available to take part in the study and be interviewed. Their children were between 6 and 18 years old (children: 6-8 years [n=6]; adolescents: 10-18 years [n=9]). Parents were on average 38 years old (38±5), most were married (90%; n=18), had secondary education (65%; n=13), and were employed (80%; n=16).

## Qualitative findings

Figure 2 shows the themes and sub-themes that emerged from the thematic analysis. Four main themes emerged, (i) perception of the impact of the disease; (ii) facilitators of disease management; (iii) adherence to treatment; and (iv) physical activity. A summary of these findings is presented below, followed by the most illustrative quotations.

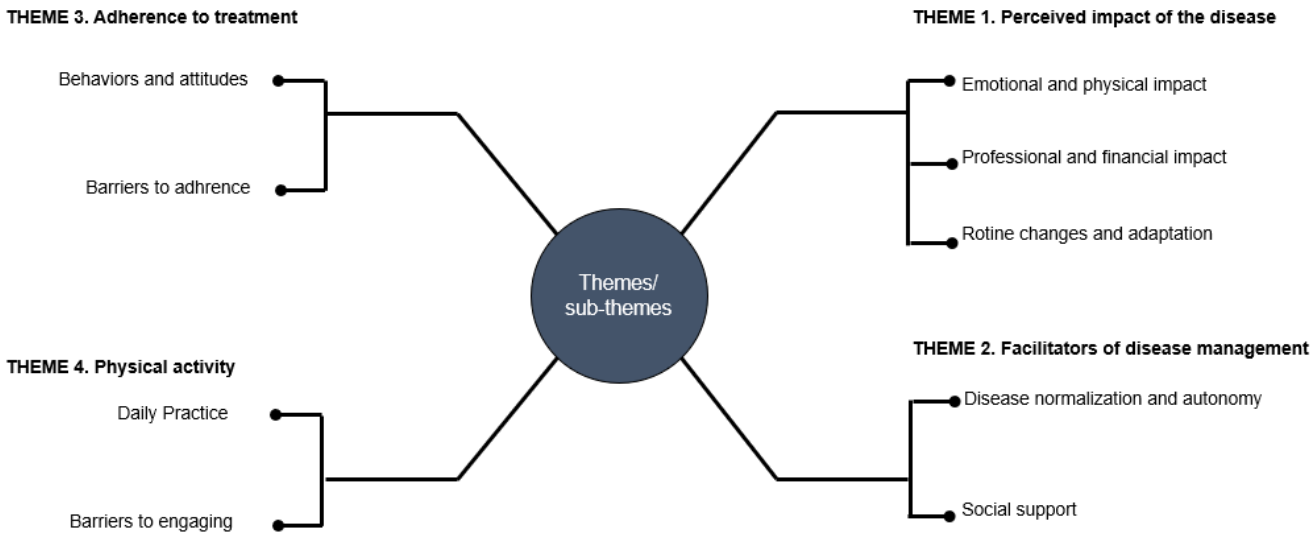


**Figure 2** – Themes and sub-themes obtained from the qualitative data analysis (n=20).

### Theme 1: Perception of the impact of the disease

Caregivers reported that the period before diagnosis was the phase of greatest impact and family disorganization. Most parents had no prior knowledge of the disease. In some cases, difficulties were reported in getting the right diagnosis (sometimes mistaken for asthma), which seems to have had a significant emotional impact. For instance, one mother shared: *"We didn't know (...) we fed her, but because of the illness she lost weight (...) and they said we didn't feed her..."* [mother, 6-year-old child]; This perception of misjudgement created feelings of guilt and frustration among caregivers, exacerbating their emotional struggles. Another caregiver highlighted her son's physical exhaustion, explaining: *"My daughter was coughing so much that tears were running down her face. She wasn't crying. It was the coughing, the effort (...). We had a traumatic time until we got the diagnosis"* [mother, 11-year-old adolescent]. Such accounts underscore the prolonged negative impact that both parents and children endured as they struggled with

persistent symptoms and sought clarity, ultimately amplifying the emotional challenges they faced in the absence of a clear diagnosis.

Hospital admissions, associated with periods of exacerbation of the child's and/or adolescent's symptoms, were pointed out as major factors of negative emotional impact and with significant implications for family and professional management. Some mothers reported having to quit their jobs to support their children, especially the younger ones: *"I was working and I had to quit because they didn't want to look after my son at nursery school because of the medication and all that stuff..."* [mother, 6-year-old child]: *"... I'm a beautician, and I had my own office, but when they told me to wait three months at home, one in hospital, there's no one who can keep a business open (...)"* [mother, 11-year-old child]. The demands related to frequent trips to the hospital were also highlighted, which meant changes to the daily routine (e.g., professional flexibility, reorganization of other parental tasks), as well as a greater financial burden: *"(...) But there was a phase when the hospital allowed us to take the medication home for three months. But then, at some point, they banned it, which forced me to travel here [to the hospital] often to get the medication."* [mother, 18-year-old adolescent]. This is suggestive of the continued pressure on families as they overcome logistical and financial hurdles to secure necessary medical care for their children, emerging as a double burden to the already demanding role of caregiver.

From the perspective of most parents, their children were well adapted to the disease, especially when the diagnosis occurred at an early stage of development. Most maintained a daily routine similar to their peers, although they did report greater during some activities or loss of appetite: *"She feels a bit tired. And if she gets too tired if she eats, she can throw it all out"* [mother, 6-year-old child]. The main impacts, especially on an emotional level, were more noticeable in adolescents diagnosed later, with parents reporting symptoms of hopelessness and challenging behaviour at school: *"It's gotten worse because he thinks that from "today to tomorrow" he could die. That's what's in his head. That he won't live for many years. He has been a very rebellious child since elementary school and now he is in danger of failing his course."* [mother, 17-year-old adolescent]. Older children are thus more vulnerable to psychological distress and behavioural changes, which also impact family dynamics.

**Theme 2: Facilitators of disease management**

Parents' reports suggested that the passage of time and the consequent increase in knowledge about the disease led to better adaptation and acceptance by themselves and their children. Parents of adolescents diagnosed in early childhood highlighted their children's autonomy in managing symptoms and therapies, which seemed to help ease the burden on parents and normalize family routines. For instance, one father shared, *"She has a symptom, gets up, coughs, vomits and that's it"* [father, 16-year-old adolescent]; while other mothers similarly expressed confidence in their daughters' abilities to manage their medication: *"I trust her with the medication. For example, when she eats at school she takes it, I know she doesn't hide it."* [mother, 11-year-old adolescent]; *"She knows she has to take her medication, she knows the care she has to take"* [mother, 18-year-old adolescent]. This sense of independence in children allowed parents to feel more secure about their child's care and facilitated a greater balance within family life.

In addition, the social support network (e.g., family, friends, health professionals) was shown to be an important facilitator in managing the illness, especially for parents. However, conflicting perceptions existed regarding the support provided by formal structures such as the school. Some parents reported an understanding attitude from teachers and educators, while others pointed to this as an additional stress factor: *"(...) we don't have any support at school for her, because she misses a week, but nobody tells her, take this to make up for it"* [mother, 16-year-old adolescent]." These mixed experiences suggest that while informal support networks were generally effective, formal support systems could either mitigate or amplify parental challenges, depending on the level of cooperation provided.

Finally, an encouraging and normalizing attitude from parents also appeared to have a positive impact on the children and adolescents dealing with their chronic condition: "*Our initial goal was to create a sense of normalcy while safeguarding the extra care and activities they have to accomplish. As they grow up, we now strive to ensure that they lead perfectly normal lives."* [mother, 16-year-old adolescent]*; "She has never been a girl who has rebelled against her illness, she takes everything very calmly and always with a smile."* [father, 16-year-old adolescent]. These perspectives reinforce the value of normalization strategies, which can strengthen children's resilience and contribute to their overall well-being as they face health challenges.

**Theme 3: Adherence to treatment**

Parents reported adherence to treatment by children and adolescents as being good, however, a later diagnosis also appeared to be a barrier to adherence, particularly to the use of airway clearance devices such as "*Flutter*." Emotional factors, such as hopelessness due to the health condition, were pointed out as a contributor to this behaviour: *"Bad, bad and continues to deal badly, doesn't want to do the flutter, every day is a massacre. (...) I have to force him every day. He says it's not worth doing because he's going to die."* [mother, 17-year-old adolescent]. Reports also seemed to suggest that the period of adolescence may lead to more feelings of shame and conditional use of medication in social contexts. Moreover, a negative reaction from peers may further aggravate the child's behaviour and adherence to medical recommendations: "*She talks more about the kids... They make fun of her when she wants to expel it. Sometimes she swallows it if she can.* [father, 8-year-old child]. These insights suggest that the complexities of adolescence, coupled with emotional struggles and social dynamics, significantly influence treatment adherence.

**Theme 4: Physical activity**

All parents reported that their children practiced some kind of physical activity, however, the frequency of these activities depended on financial and logistical factors. Most children practiced sports activities such as swimming (the most common on medical advice), ballet, and/or soccer; however, families with a greater financial burden only reported involvement in more structured activities carried out at school (e.g., physical education classes). Free activities such as jumping rope and cycling were commonly reported by parents of young children.

Among the main barriers to participation in physical activities, parents highlighted the difficulty of reconciling schedules due to family and/or professional demands, the overload of school activities (especially for adolescents), and the weather conditions in outdoor activities *"He says he loses a lot in training and has to do his homework, which then affects his school* [mother, 10-year-old adolescent]). Parents' reports also showed that children and adolescents have difficulties in managing activities and the associated tiredness: "*I think he does too much physical exercise, sometimes blood appears in his secretions and that's why I don't insist too much either and I've been walking with him, so he doesn't play ball so much and run around so much*" [father, 16-year-old adolescent].

Finally, another important aspect of managing physical activity seemed to be the involvement of parents in the dynamics, especially for more dependent children. Lack of engagement can discourage exercise: "*She asks to go for a walk at night, but the person wants to get home and rest a little and doesn't feel like doing anything*" [mother, 6-year-old child]. These findings suggest that while physical activity is valued by families, various obstacles—including financial constraints, scheduling conflicts, and parental involvement—play significant roles in shaping children's engagement in exercise. Addressing these barriers may require a multifaceted approach that considers not only the children's needs but also the logistical and emotional support systems within the family.

## Discussion

Our findings suggest that managing CF in children is demanding and has a multidimensional impact, affecting the emotional, physical, and social well-being of children and their informal caregivers. Overall, participants identified different modifying factors, that affected the management of the disease and adherence to an integrated treatment, directly related to the child's characteristics perceptions, and attitudes (e.g., normalization) toward the disease in the family context. According to the reports, knowledge, the child's greater autonomy in medication management and social support emerged as the main factors that facilitate adherence to therapeutic recommendations. This study confirms earlier research (4,10,25) indicating that parents of children diagnosed with CF face significant emotional and job-related challenges. Hospitalizations due to exacerbations particularly disrupt family life and cause emotional distress. The potential for a terminal illness and various stressors like fear, limited social time, and financial strain significantly affect caregivers' quality of life (5).

Children with chronic illnesses, as previously reported (6), often face higher educational demands compared to their healthy peers, being absent from school an average of 16 days yearly versus three days for healthy peers (27). Our study corroborates these findings, highlighting school support as a key factor in managing these challenges, according to parents.

Late diagnosis exacerbates symptoms and family disruption, hindering adaptation and treatment adherence due to shame and hopelessness about the future.

Early CF diagnosis provides the opportunity to improve illness management and avoid early repercussions by adopting more peer-like habits (28). Our study supports findings

from earlier literature showing parents prefer an early diagnosis, even when their child has an incurable disease (29). The majority of kids are better able to accept daily routines and cognitively adjust to changes with a posture of control and normality when they receive an early diagnosis (30).

In addition, families appear to be still challenged by the need to make daily choices about what to prioritize in terms of CF management (academic vs. therapeutic). For example, the inclusion of physical exercise in the routine, as part of the treatment of the disease, was recognized as a benefit for the management of CF symptoms, as the literature has shown, but uncertainty and concern about overdoing it were also reported. Adolescents with greater autonomy also seem to experience this pressure, with the difficulty in managing schedules being one of the main barriers to non-compliance with physical activity. This reflects the importance of health professionals being more aware of the need to adjust therapies to families' routines (31). Physical activity must be properly incorporated into daily routines so that individuals with CF, and their parents, feel satisfied with their lives (15). Patients who incorporate exercise into their routine appear to experience improvements in their physical and mental well-being, feelings of control, and sense of normality (13).

**Limitations**

This study acknowledges several limitations due to its exploratory nature. Firstly, variability in interview durations was observed, influenced by participant availability, which may have affected the depth and richness of the data collected. Secondly, the research was conducted within a single hospital context, limiting the generalizability of the findings to other settings and populations. Additionally, the absence of data triangulation through interviews with both children and health professionals presents a limitation, as it restricts a comprehensive understanding of the complexities involved in managing CF from multiple perspectives. Furthermore, there is the possibility that social desirability bias could affect the authenticity of the data collected. To strengthen the validity of our findings, future research should consider the role of developmental transition phases in treatment adaptation and adherence in the context of CF.

Despite these limitations, our study underscores the multifaceted impact of CF on children and parents from parents' perspective, advocating for flexible and personalized care models tailored to families' realities. Collaboration between specialized CF services and public health programs, alongside technological innovations, can enhance

personalized management and adherence. Normalizing routines, as per our findings, is crucial for effective family disease management.

## Conclusions

This study underscores the significant emotional and logistical challenges parents face in managing CF in their children. Key findings highlight the importance of early diagnosis and increased parental knowledge in facilitating effective disease management. To enhance clinical practice, healthcare providers should implement tailored educational programs for families and establish multidisciplinary support teams to address their diverse needs. Additionally, personalized, and flexible treatment plans should be developed to accommodate individual family dynamics. Engaging community resources and schools can further support physical activity and overall well-being for children with CF. These recommendations aim to improve treatment adherence and, in the end, improve the lives of impacted families.

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# Chapter 3. Original study II

# Effects of a Tailored Home-Based Exercise Program, "KidMove", on Children with Cystic Fibrosis: A Quasi-Experimental Study

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Submission received: 11 November 2024 / Revised: 7 December 2024 / Accepted: 21 December 2024/ Published: 24 December 2024

Open Access Article

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*Healthcare* **2025**, *13*(1), 4; **https://doi.org/10.3390/healthcare13010004**

**Submission received: 11 November 2024 / Revised: 7 December 2024 / Accepted: 21 December 2024 / Published: 24 December 2024**

**Abstract**

Exercise for children with cystic fibrosis leads to well-known health benefits, however, maintaining regular activity is challenging due to the daily demands of academics, clinical care, and family tasks. Home-based exercise programs offer a more adaptable alternative, fitting into family schedules. This study evaluated the effectiveness of the "KidMove" program, a parent-supervised, tailored home exercise regimen. A quasi-experimental study was conducted with an intervention group (IG) and a wait-list control group (CG). The "KidMove" lasted 12 weeks and included 35 exercises targeting endurance, resistance, flexibility, and neuromotor training. The primary outcome, endurance, was measured with the Modified Shuttle Walking Test, while secondary outcomes included body composition, resistance, flexibility, postural control, respiratory function, and health-related quality of life. Data were collected at baseline and post-intervention. A per-protocol analysis was conducted with generalized estimating equations (GEE). Forty-six children aged 10±4 years (6 to 18 years), mostly male (n=24; 52.2%) participated. Significant improvements were found in the Modified Shuttle Walking Test [Wald $\chi2 = 14.24$, $p < .001$], postural control [Wald $\chi2 = 3.89$, $p = .048$], knee flexibility [Wald $\chi2 = 5.58$, $p = .018$], and emotional functioning [Wald $\chi2 = 9.34$, $p = .002$]. The "KidMove" program offers a practical, family-friendly alternative to center-based exercise by empowering parents to support their children's physical activity at home, endurance, flexibility, and emotional well-being while reducing logistical challenges.

**Keywords:** paediatrics; adherence; physical activity; endurance; quality of life

## Introduction

Cystic Fibrosis is a complex, multi-systemic disorder primarily characterized by pulmonary complications that significantly impact morbidity and mortality rates (Bell et al., 2020; Shteinberg et al., 2021). It is characterized by progressive chronic airway obstruction and infections since infancy and childhood (Goss & Burns, 2007) as well as lung function decline in adolescence (VanDevanter et al., 2016) which lead to reduced exercise capacity, muscle resistance, balance, shoulder and chest mobility, bone density, and an increased risk of fractures (Bayfield et al., 2024).

These complications contribute to limitations in daily activities, loss of independence, and a decline in overall quality of life (Bell et al., 2020; Radtke et al., 2022).

Exercise programs are recognized as vital non-pharmacological interventions for individuals with cystic fibrosis, ideally beginning in early childhood. They help mitigate cystic fibrosis-related morbidity by improving physical health, fitness levels, and social engagement (Gruet et al., 2022) although minor effects on lung function and health-related quality of life have also been observed (Bayfield et al., 2024). Maintaining adequate physical fitness is associated with favourable disease prognoses and reduced hospitalizations (Bayfield et al., 2024; Cox & Elkins, 2011; Burnett et al., 2020).

However, children with CF often face physical challenges, such as reduced pulmonary function, muscle weakness, and fatigue, which can create significant social barriers, including feelings of inadequacy and withdrawal from group activities. These challenges are further amplified by the absence of tailored exercise opportunities in schools and community programs (Bayfield et al., 2024). Tailored exercise programs that are specifically designed to address individual needs and preferences are more effective than generic approaches in improving both physical and psychological outcomes, highlighting the importance of personalized strategies in supporting this population ((Burnett et al., 2020; Del Corral et al., 2018; Conway et al., 2014). These programs can be designed to address specific postural control issues, incorporating balance, resistance, and flexibility training that are personalized for the individual (Bayfield et al., 2024; Gruet et al., 2022).

Nevertheless, most treatments for cystic fibrosis are complex and require a significant amount of time to administer. Even when children receive the most suitable treatment and appropriate guidance, adherence to treatment recommendations in children with cystic fibrosis is reported to be less than 50% (Goodfellow et al., 2015). A recent meta-analysis has found that support from parents and their modelling behaviours were related to

children's physical activity levels (Yao & Rhodes, 2015). Therefore, balancing the benefits and burden of exercise is crucial not just for individuals, but also for families and health professionals, ensuring adherence to exercise as a standard therapeutic approach (Conway et al., 2014; Junior et al., 2023. Community or home-based exercise programs have been suggested as promising alternatives since they can be more flexibly administered and minimize the risk of cross-infection (Fielding et al., 2015).

Nevertheless, their effects on physical fitness, lung function, infection status, antibiotic usage, health-related quality of life, adherence, and satisfaction remain limited and inconsistently documented (Bayfield et al., 2024). A recent systematic review reported the effectiveness of home-based rehabilitation on pulmonary function, functional capacity, and health-related quality of life in children and adolescents (6 to 20 years) with cystic fibrosis, emphasizing that home-based rehabilitation may be an alternative when conventional center-based programs are not feasible (Junior et al., 2023). Exercise interventions, especially those tailored to individual needs, have been proposed as crucial for managing these complications, yet adherence remains a major issue, especially in children. Given the potential of home-based exercise programs to provide a more flexible and feasible alternative to traditional center-based approaches, this study aimed to assess the effectiveness of a tailored home-based exercise program "KidMove" in children with cystic fibrosis with parents' supervision. We hypothesized that a home-based exercise program will improve endurance (primary outcome) and other health-related physical fitness components (body composition, muscle resistance, flexibility, neuromotor), as well as lung function and health-related quality of life in children with cystic fibrosis.

## Materials and methods

### *Study design*

A quasi-experimental study with two arms (intervention group *vs.* wait-list control group) was conducted to assess the effects of a home-based exercise program "KidMove." Ethical approval was obtained from the Ethics Committee of the Centro Hospitalar de Coimbra and the Ethics Committee Centro Hospitalar do Porto (CHUC-004-13), where recruitment took place. Informed consent was obtained from the parents or guardians of each participant before any data collection, and privacy was ensured by European

regulation (EU 2016/679). All procedures were conducted by the ethical standards of the National Research Committee, the 1964 Helsinki Declaration, and its subsequent amendments or comparable ethical standards. The study was unblinded, as participants were aware of the intervention received. This study adheres to the Consolidated standards of reporting trials (CONSORT) statement for non-randomized studies (Ruf & Hebestreit, 2009) and the Template for intervention description and replication (TIDiER) guidelines (Gribble et al., 2012).

*Participants and procedure*

Participants were eligible for inclusion in the study if they were 18 years of age or younger, had a respiratory function test result of FEV1 > 40%; and regularly attended their routine hospital appointments. Individuals were excluded if they were mechanically ventilated, had a musculoskeletal condition that could interfere with their physical assessment, or experienced a recent (within the last month) acute respiratory exacerbation (Goss & Burns, 2007).

Recruitment was conducted face-to-face using a convenience sampling method. Children and adolescents were initially identified by their attending paediatricians, who provided a brief explanation of the study to the whole family. Those who met the eligibility criteria and expressed interest in participating were subsequently contacted by a member of the research team (SG), who provided detailed information about the study and addressed any remaining questions.

A statistical power analysis was conducted using G*Power 3 (University of Düsseldorf, Germany) to determine the required number of participants for the study. This calculation was based on data from previous studies that evaluated the impact of a home-based exercise program on the modified shuttle walking test (mswt) (Urquhart et al., 2012). The sample size analysis indicated that 13 participants per group would be needed to detect a large effect size in the mswt (m) with 80% power and a 5% significance level. To account for this, a target of 26 participants per group was set for the study (Kang, 2021). Additionally, Whitehead and colleagues recommend that for a study aimed at achieving 90% power with a two-sided 5% significance level and considering medium standardized effect sizes, the pilot sample sizes should include a minimum of 15 participants per arm

(Whitehead et al, 2016). This guideline further supports the rationale behind our sample size decision, ensuring that our study is adequately powered to detect meaningful effects.

*Intervention*

Participants in the intervention group completed a personalized home-based exercise program - "KidMove," alongside standard treatment, which included antibiotics, bronchodilators, pancreatic enzyme supplements, and airway clearance techniques.

The intervention began with a face-to-face baseline assessment, during which researchers provided individualized guidance on appropriate exercise intensities and instructed participants on monitoring key physiological parameters, including heart rate (targeting 70%-80% of maximum heart rate) and perceived exertion using the modified Borg Scale (target range: 4–6). Participants were also educated on recognizing warning signs and symptoms, emphasizing the importance of stopping exercise immediately if their heart rate exceeded the recommended maximum or if they experienced extreme coughing or a dyspnoea perceived exertion rating of 8–10 on the modified Borg Scale (Borg, 1982).

Caregivers were actively involved in the intervention. An initial training session was delivered, where caregivers were instructed on their role in supporting and supervising the exercise program. This included strategies for encouraging and motivating participants, as well as participating in some exercises themselves to foster engagement. Caregivers were also trained to monitor and document key details of the intervention, such as the type, duration, and frequency of physical activities, using a structured activity diary. These diaries were periodically reviewed by the research team to ensure adherence to the exercise program and to address any challenges or concerns raised by participants or caregivers. By integrating caregiver involvement and structured monitoring, the "KidMove" program sought to create a supportive environment, reinforcing adherence to the intervention while maintaining a focus on participant safety.

The program lasted 12 weeks, with the recommended frequency of exercise 3 to 5 times per week and consisted of a total of 35 exercises targeting endurance, resistance, balance, and flexibility training, supervised by parents. Exercises were selected based on each participant's preferences and strategies to enhance adherence were based on previous studies such as the Fitness Challenge Program (FitKitTM) (Mandrusiak & Watter, 2012). Participants were instructed to choose exercises from each category according to their preference and that they should progress if the exercises were too easy (<4) in perceived

exertion in the modified Borg scale. Physical activity levels were documented using self-reported diaries, detailing exercise type, duration, supervision status, and associated symptoms (Bradley et al., 2015). Before the intervention, education on hydration and breathing techniques during exercise was implemented by one member of the research team (SG), aiming to enhance awareness and facilitate sustained engagement in physical activities.

The exercise regimen was carefully designed to provide a comprehensive and structured program, incorporating a warm-up phase (5-10 minutes) comprising range-of-motion exercises, stretching, low-intensity aerobic exercises, and breathing techniques. Endurance training sessions lasting 20-30 minutes, with activities such as swimming, running, cycling, skipping, aerobic classes, step aerobics, and trampolining. The intensity of endurance training was individually prescribed using the mswt (Holland et al., 2014), targeting a perceived exertion of 4-6 on the modified Borg scale (Borg, 1982). Exercises were tailored to match participants' fitness levels and preferences, ensuring both engagement and safety. Resistance training sessions (15 minutes) started with one set of 10 repetitions and progressed to six sets of 10-15 repetitions per set, with a 60-second rest between sets. Resistance exercises included bodyweight activities such as push-ups, squats, lunges, and the use of free weights (e.g.: a packet of rice, and bottles). Balance and flexibility training (5 minutes each) included static and dynamic exercises arranged at increasing difficulty levels (Muehlbauer et al., 2012), such as tandem walking and one-leg stands. Flexibility training, which also lasted 5 minutes and focused on major muscle groups. These exercises included yoga-inspired stretches and static holds. The cool-down period (10 minutes) mirrored activities from the warm-up phase (Boas, 1997; Garber et al., 2011). The intensity of all exercises was monitored and adjusted according to the participant's perceived fatigue and dyspnoea, targeting a range of 4-6 on the modified Borg scale.

Participants and parents were instructed to accumulate 150 minutes of exercise per week, aligning with recommendations from the American College of Sports Medicine (Garber et al., 2011), and report the type of exercise and adverse events in the activity diary. To improve program adherence, parents and teenagers received biweekly mobile messaging and weekly phone calls from researchers. Adverse events during exercise were reported in the activity diary. A structured guide was followed during telephone calls consisting of gathering data on encountered challenges and adjustments needed in the exercise. Participants were advised to promptly consult their attending physician or researcher

regarding concerns about exacerbations, signs of respiratory distress, monitoring, or exercise-related issues.

*Outcome measures*

Data were collected at baseline and after 12 weeks by the same assessor (SG; physiotherapist) who was experienced in administering the selected outcome measures.
The primary outcome was the endurance component, assessed using the MSWT. The mswt involves participants walking or running on a 10-meter course with cones, guided by auditory cues that progressively increase speeds from 0.5 m/s, escalating by 0.17 m/s per minute until exhaustion or inability to complete within the specified time frame (Urquhart et al, 2012; Saynor et al, 2023; Selvadurai, 2002). Participants completed the mswt twice with a minimum mandatory 30-minute rest period between tests (until returning to their baseline vital signs), to mitigate learning effects. The best performance was kept for analysis. Key metrics recorded included total distance covered, maximum speed achieved, number of shuttle runs completed, as well as reasons for test cessation to ensure safety and accurate interpretation of results. This test is well-known for its dependability in young people with cystic fibrosis (Del Corral et al., 2020). It also shows good test-retest reliability ($r=0.99$, $p<0.01$), sensitivity to change in aerobic fitness, and a substantial correlation with $VO_2$max in cystic fibrosis. Physiological responses including heart rate and peripheral oxygen saturation were monitored using a pulse oximeter (Moretti, FS10) before and after each mswt. Fatigue and dyspnoea levels were assessed using the modified Borg Scale, which closely correlates (0.6-0.8) with $VO_2$ peak (Nashimoto, 2021). The minimal clinically significant difference of the mswt is 97 m (Del Corral et al., 2020).
The remaining health-related physical fitness components were included as secondary outcomes and were assessed with body mass index (BMI) (weight/height2) for body composition (SECA® mechanical scale and WHO AnthroPlus software); handheld dynamometer (microFET2, Hoggan Health, The Best Salt Lake City, Utah) in kilogram-force (KgF) for muscle strength (Beenakker et al., 2001); the modified sit and reach test (msrt) (Hui & Yuen, 2000) for flexibility; the star excursion balance test (sent) (Gribble et al., 2012) for neuromotor component. Additionally, a spirometry (Spirobank II USB R ©, MIR, Rome, Italy) was conducted, according to the American Thoracic Society/European Respiratory Society guidelines (Graham et al., 2019), to assess lung

function and the values obtained for the forced expiratory volume in one second ($FEV_1$) and the forced vital capacity in litters and percentage predicted were registered (FCV). Finally, the cystic fibrosis questionnaire-revised (CFQ-R) was used to assess health-related quality of life (Quittner et al., 2009).

Sociodemographic and clinical data were collected to characterize the sample, as well as recruitment and retention indicators through 1) participation rate: participants volunteering to participate/eligible participants × 100; and 2) study dropout (post-test): participants who did not complete the post-test/enrolled participants × 100. Adverse events during exercise were reported in the activity diary.

*Data analysis*

Descriptive statistics were used to describe the sample and to evaluate retention and effectiveness indicators. Absolute and relative frequencies, measures of central tendency, and/or dispersion were utilized. To evaluate the intervention's effectiveness (examining both between-group and within-group effects for each outcome), data were analyzed using methods appropriate for correlated panel data, specifically generalized estimating equations (GEE) modelling. The quasi-likelihood information criterion (QIC) was used to determine the optimal correlation structure and best-fitting model within the GEE analysis. For all outcomes, a Gamma distribution was chosen, as it yielded the lowest QIC. Statistical analyses were conducted using IBM SPSS Statistics for Windows, version 28.

## Results

*Characterization of study participants at baseline*

Fifty-two children and/or adolescents with cystic fibrosis were eligible and were referred to participate in the study, 48 of whom agreed to take part. Two participants allocated to the intervention group dropped out of the study and did not complete the intervention. Based on a per-protocol approach, 46 participants were included finally (23 participants in each group; see Figure 1). The participation rate in the study was 92.3% and the dropout rate was 4.3%. No adverse events were reported.

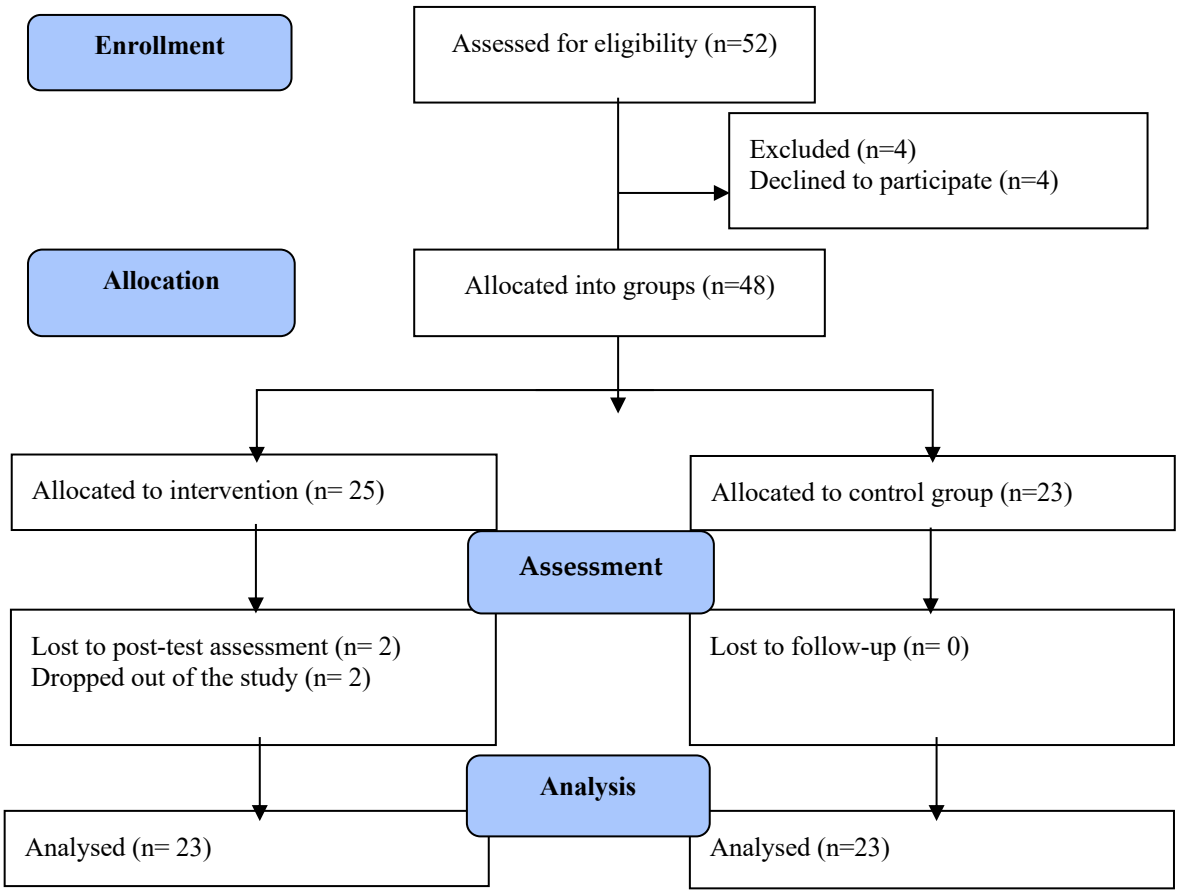


**Figure 1.** Consolidated standards of reporting trials (CONSORT) diagram of the included participants with cystic fibrosis in the home-based exercise program ("KidMove").

Participants were 10±4 years old (6 to 18 years), mostly male (n=24; 52.2%), and were attending preschool and primary school (n=25; 54.3%). Approximately 54.3% (n=25) did not have physical education classes in a school context. At the time of recruitment, 13% (n=6) of the children had experienced an exacerbation, requiring hospitalization, within the past 3 months. Detailed characteristics of the participants for each group are presented in Table 1. No differences were found between groups at baseline.

**Table 1.** Baseline characteristics of participants with cystic fibrosis included in the study (n=46).

| Variable | Total sample | Intervention Group (IG; n=23) | Control Group (CG; n=23) | t-value/$\chi^2$ | p |
|---|---|---|---|---|---|
| | n (%) | n (%) | n (%) | | |
| Age, years (M(SD)) | 10.37 (3.86) | 10.3 (4.09) | 10.3(3.70) | .03 | .970 |
| Sex, n (%) | | | | | |
| Male | 24 (52.2) | 11 (47.8) | 13 (56.5) | | |
| Female | 22 (47.8) | 12 (52.2) | 10 (43.5) | 3.0 | .223 |
| Education | | | | .18 | .980 |
| Elementary school | 25 (54.3) | 12 (52.2) | 13 (56.5) | | |
| 5th-6th grade | 8 (17.4) | 4 (17.4) | 4 (17.4) | | |
| 7th-9th grade | 6 (13.0) | 3 (13.0) | 3 (13.0) | | |
| Secondary school | 7 (15.2) | 4 (17.4) | 3 (13.0) | | |
| Physical exercise at school (yes) | 21 (45.7) | 13 (56.5) | 8 (34.8) | 2.19 | .139 |

*Effectiveness of the intervention*

A group-time effect following a per-protocol approach, a significant group-by-time interaction effect favouring the intervention was found for the MSWT, highlighting the positive effects of the "KidMove" program on the endurance of participants (Table 2). In the intervention group, the increase in distance achieved during the MSWT was 99.56 m, indicating a real and clinically meaningful improvement whereas, no significant improvement was observed in the control group. The data are presented as mean ± standard deviation (SD), along with frequency and percentage.

A significant increase in scores from pre-test to post-test was observed in the intervention group while in the control group, these scores remained similar (although with a slight improvement) from baseline to post-test. Regarding the quality of life, an interaction effect was identified in the dimension in the intervention group, while no significant changes were noted in the control group (Table 2).

**Table 2.** Results at baseline and 12 weeks after tailored home-based exercise program in children with cystic fibrosis with parents' supervision "KidMove", according to the per-protocol analyses (n=46).

| Outcome | Outcome measure | Intervention Group (IG) (n=23) | | Control Group (CG) (n=23) | | Group-time effect | | |
|---|---|---|---|---|---|---|---|---|
| | | T0 | T1 | T0 | T1 | $\chi$2 | p* | B (95% CI)† |
| | | M(SE) | M(SE) | M(SE) | M(SE) | | | |
| BMI, Kg/m$^2$ | | 17.4 (.68) | 17.1 (.6) | 17.6 (.5) | 17.5 (.52) | 2.2 | .131 | .261 (-.078, .600) |
| | FEV1, % predicted | 78.8 (2.5) | 79.2 (2.4) | 85.8 (2.3) | 85.6 (2.3) | 1.7 | .184 | -.578 (-1.431, .274) |
| | FVC, % predicted | 77.0 (2.1) | 78.2(1.9) | 85.4 (1.7) | 85.1 (1.8) | 3.1 | .076 | -1.583 (-3.33; .165) |

| Outcome | Outcome measure | Intervention Group (IG) (n=23) | | Control Group (CG) (n=23) | | Group-time effect | | |
|---|---|---|---|---|---|---|---|---|
| | | T0 | T1 | T0 | T1 | $\chi 2$ | p* | B (95% CI)† |
| Neuromotor component | Modified Shuttle Walking Test (m) | 515.2 (24.8) | 614.7 (26.4) | 495.2 (18.9) | 530.4 (20.3) | **14.2** | **<.001** | **-64.35(-97,775; -30,92)** |
| | Star excursion balance test-Posteromedial right | 79.9 (1.1) | 85.2 (1.0) | 72,69 (2.2) | 77.6 (1.7) | .12 | .720 | -.378(-2.449, 1.692) |
| | Star excursion balance test-medial | 65.2 (2.0) | 71.0 (2.1) | 38.8 (2.2) | 45.3 (2.2) | .24 | .623 | .630 (-1.880, 3.141) |
| | Star excursion balance test - anterior right | 70.4 (1.6) | 75.6 (1.6) | 69.4 (2.7) | 73.2 (2.7) | **3.8** | **.048** | **-1.383 (-2,756, -.009)** |
| Muscle Strength | HHDMVS-HHD, Kg/F | | | | | | | |
| | HFMVS-HHD Kg/F) | 17.5 (1.1) | 18.9 (1.2) | 17.1 (1.0) | 18.4 (1.0) | .25 | .613 | -.130 (-.636, .375) |
| | HABMVS-HHD Kg/F | 15.6 (1.3) | 16.9 (1.4) | 15.7 (1.3) | 16.9 (1.2) | .07 | .788 | -.061(-.504, .382) |
| | KEMVS-HHD Kg/F | 19.5 (1.7) | 21.3 (1.8) | 19.4 (1.5) | 20.8 (1.6) | 2.7 | .097 | -.474 (-1.034, .087) |
| | KFMVS-HHD Kg/F | 18.3 (1.4) | 19.7 (1.5) | 17.0 (1.0) | 17.8 (1.0) | **5.5** | **.018** | **-.617 (-1.130, -.105)** |
| | ADFMVS-HHD Kg/F | 13.9 (.9) | 15.4 (1.0) | 13.9 (.6) | 15.2 (.6) | .48 | .487 | -.187(.2688, -.714) |
| | SFMVS-HHD Kg/F | 11.5 (.9) | 12.4 (1.0) | 11.9 (.8) | 12.7 (.8) | .48 | .487 | -.135(-.515, .245) |
| | SABMVS-HHD Kg/F | 11.7 (1.1) | 12.2 (1.2) | 11.9 (1.1) | 12.3 (1.1) | 1.8 | .180 | -.170(-.417, .078) |
| | EFMVS-HHD Kg/F | 13.7 (1.1) | 14.8 (1.1) | 14.2 (.8) | 15.0 (.8) | 2.7 | .095 | -.270(-.586, .047) |
| | EEMVS-HHD Kg/F | 10.3 (1.0) | 10.8 (.9) | 11.8 (.9) | 12.5 (.9) | 2.5 | .108 | .274 (-.060, .608) |
| | WEMVS-HHD Kg/F | 9.2 (.91) | 9.7 (.95) | 9.4 (.6) | 9.8 (.6) | **4.0** | **.044** | **-.187 (-.369, -.005)** |
| Flexibility(m) | Modified Sit Reach Test | 15.7 (.93) | 18.5 (1.0) | 14.2 (.7) | 16.6 (.8) | .60 | .437 | -.387(-1.362, .588) |
| HRQoL | CFQ-R Physical | 71.2 (3.1) | 74.6 (3.2) | 63.7 (3.2) | 66.7 (3.3) | .01 | .904 | -.387 (-6.664, 5.890) |
| | CFQ-R Emotion | 71.7 (3.2) | 77.3 (2.8) | 84.4 (2.1) | 83.6 (2.8) | **9.3** | **.002** | **-6.435(-10.562, -2.308)** |
| | CFQ-R Eating | 52.6 (4.2) | 54.1 (3.8) | 57.0 (3.0) | 57.5 (2.9) | .10 | .741 | -.756(-5.246, 3.734) |
| | CFQ-R Treatment Burden | 66.2 (3.3) | 70.5 (2.7) | 73.9 (2.5) | 74.9 (2.3) | 2.2 | .130 | -3.378 (-7.754, .997) |
| | CFQ-R Social | 54.9 (3.4) | 58.4 (3.0) | 58.6 (2.3) | 61.3 (1.6) | .20 | .654 | -.837 (-4.497, 2.822) |
| | CFQ-R Body image | 72.7 (4.3) | 78.3 (3.3) | 69.6 (2.5) | 73.9 (2.3) | .25 | .612 | -1.207 (-5.863, 3.450) |
| | CFQ-R Respiratory | 73.5 (1.9) | 82.8 (1.3) | 76.2 (2.1) | 80.9 (2.2) | 3.0 | .079 | -4.600(-9.737, .537) |
| | CFQ-R Digest | 74.8 (4.3) | 82.6 (3.1) | 89.8 (3.1) | 91.3 (3.0) | 2.1 | .144 | -6.287 (-14.726, 2.152) |

Abbreviations: BMI: body mass index; FEV: forced expiratory volume; FVC: forced vital capacity; MSWT: Modified Shuttle Walking test; HF-HHD: hip flexors maximum voluntary isometry strength- hand held dynamometry; HAB-HHD: hip abductors maximum voluntary isometry strength- hand held dynamometry; KE- HHD: knee extensors maximum voluntary isometry strength- hand held dynamometry; KF-HHD: knee flexors maximum voluntary isometry strength- hand held dynamometry; ADF-HHD: ankle dorsiflexors; maximum voluntary isometry strength- hand held dynamometry; SF- HHD: Shoulder flexors maximum voluntary isometry strength- hand held dynamometry; SAB-HHD: shoulder abductors maximum voluntary isometry strength- hand held dynamometry; EF-HHD: elbow flexors maximum voluntary isometry strength- hand held dynamometry; EE-HHD: elbow extensors maximum voluntary isometry strength- hand held dynamometry: HAD-HHD: hip adductors maximum voluntary isometry strength- hand held dynamometry; HE-HHD: hip extensors maximum voluntary isometry strength- hand held dynamometry; WE-HHD: Wrist extensors maximum voluntary isometry strength- hand held dynamometry; MSRT: modified sit and reach; Cystic Fibrosis Questionnaire – Revised. * P-values for type III GEE model effects tested using the Wald Chi-Square test; Values in bold represent the statistically significant differences at $p < .05$; † Unstandardized coefficients and 95% confidence intervals values under group-time effect corresponding to group 1 * time 1 (upper and lower values, respectively).

**Discussion**

The present study showed that the tailored home-based exercise program— "KidMove" is safe and effective in improving the endurance of children with cystic fibrosis.

Improvements in secondary outcomes, including neuromotor (excursion distance and anterior right stance), muscle resistance (knee flexion, wrist extensor resistance), and health-related quality of life (emotional functioning), highlighted the potential of this approach.

Overall, the findings underscore the benefits of structured exercise programs for children with cystic fibrosis. Participants demonstrated enhancements in endurance, muscle strength, balance, and flexibility, contributing to improved functional and emotional health. These results align with the existing literature, as exercise interventions have been linked to improvements in endurance and affective responses associated with the disease, particularly in paediatric contexts (Radtke et al., 2022; Del Corral et al., 2018; Urquhart et al., 2012; Burtin & Hebestreit, 2015; Hommerding et al., 2015; Schindel et al., 2015).

Home-based exercise programs overcome barriers related to the daily routine overload of families, leading to greater adherence and promising results in improving endurance, resistance, and overall physical functioning. The "KidMove" program demonstrated consistency with previous evidence, as the retention rate was 95.7%, and a group-by-time interaction effect was observed for the primary outcome, with the intervention group showing a greater change in MSWT from baseline to post-intervention (Del Corral et al., 2018; Urquhart et al., 2012).

In addition to this statistical difference, the minimal clinically important difference mswt ≥ 97.08 m (Del Corral, 2020) was reached. These findings suggest that the proposed intervention conferred advantages to children and adolescents with cystic fibrosis, producing outcomes like those attained by center-based methods (Selvadurai et al., 2002; (Santana et al., 2011).

Moreover, the "KidMove" program outperformed other home-based approaches which have demonstrated smaller changes in endurance, without evidence of changes in minimal clinical difference (Hebestreit et al., 2022). This difference may be attributed to the greater flexibility of the intervention proposed in this study, which allowed participants to select exercises based on their preferences, thereby enhancing adherence, and increasing engagement in the exercises.

It is also important to highlight that “KidMove” distinguishes itself by integrating a multimodal intervention option, which includes flexibility, balance/neuromotor, and endurance exercises. This may further explain its positive outcomes in secondary indicators, such as postural control and knee flexion. Flexibility exercises have not been a major focus in most interventions (Schindel et al., 2015), yet they may also positively impact the circulatory system's postural control and balance ability (Leung et al., 2013).

The positive impact of structured exercise programs on health-related quality of life across multiple domains—including physical, emotional, social, and academic performance—has been well-documented in children with cystic fibrosis (Quittner et al., 2009). The tailored training program provided substantial benefits, such as enhanced endurance, muscle resistance, balance, flexibility, and postural control, which collectively improved functional mobility and reduced fall risks. Additionally, flexibility exercises contributed to better joint mobility and circulation, while emotional well-being improvements alleviated psychological burdens and fostered adherence to the program (Hakim et al., 2022). This study replicated findings at the level of health-related quality of life emotional functioning, demonstrating improvements in the intervention group and a decline in the control group.

This is particularly relevant given that cystic fibrosis has been recognized as a disease with a significant emotional impact on children and their families, further underscoring the broad applicability of this intervention approach.

Despite these results, some limitations are worth noting. Although the study included a control group, its quasi-experimental design limits the ability to establish a direct causal relationship between the “KidMove” program and the observed outcomes. The lack of randomization, a key feature of randomized controlled trials, may introduce selection bias, affecting the comparability between groups. Additionally, the sample size may have been insufficient to detect effects in some secondary outcomes, limiting the generalizability of the results. Although the program's flexibility, which permits participants to select their exercises daily, can be seen as a strength, it also introduces a methodological bias that may result in varied outcomes, complicating the standardization of the intervention. Additionally, the lack of long-term follow-up hinders the assessment of the program's sustainability and its enduring impacts on both primary and secondary outcomes.

Future studies should consider adopting more rigorous designs, such as randomized controlled trials, along with long-term follow-ups to assess the sustainability of the

intervention's benefits. Including qualitative data from participants and their families could also provide valuable insights into the intervention process, helping refine and optimize the program for better outcomes.

## Conclusions

This study demonstrated that the personalized home-based exercise program, "KidMove," is both safe and effective in enhancing the endurance of children with cystic fibrosis. Moreover, the study also supports the idea that combining different types of exercises—such as endurance, resistance, and neuromotor control, can significantly improve physical and emotional health in young cystic fibrosis individuals. The intervention enhanced emotional health, particularly in the CFQ-R Emotion domain, suggesting a link between physical activity and mental well-being in patients with chronic illnesses like cystic fibrosis. The study emphasizes home-based personalized exercise programs as a safe and effective alternative to the one-size-fits-all approaches to improve health outcomes in cystic fibrosis.

**Author Contributions:** Conceptualization, S.G., and A.M.; methodology, S.G., A.M., and A.B.; formal analysis, A.B., and S.G.; resources, S.G.; Writing—original draft preparation, S.G., A.B., and A.M.; writing—review and editing, S.G., A.M., and A.B. All authors have read and agreed to the published version of the manuscript.

**Funding:** This research received no external funding.

**Institutional Review Board Statement:** The study was conducted by the Declaration of Helsinki and received a favourable review from the Ethics Committee of the Centro Hospitalar de Coimbra and the Ethics Committee Centro Hospitalar do Porto (CHUC-004-13)

**Informed Consent Statement:** Informed consent was obtained from the legal guardians of all subjects involved in the study.

**Data Availability Statement:** The data presented in this study are available on request from the corresponding author due to confidentiality agreements.

**Acknowledgments:** The authors wish to thank the nurses and doctors of Centro de Referência de Fibrose Quística – Centro Hospitalar de Coimbra and a special thanks to Dr. Miguel Félix e Dra. Teresa Reis Silva and nurses and doctors of Centro de Referência de Fibrose Quística – Hospital Santo António and a special thanks to Dr. Herculano Rocha.

**Conflicts of Interest:** The authors declare no conflicts of interest.

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# Chapter 4. General discussion

## General discussion

This thesis has investigated three core areas of research and clinical interest in CF: 1) understanding the multifaceted impact of CF on children and their caregivers, 2) evaluating the effectiveness of personalized home-based exercise programs, and 3) identifying factors that influence adherence to such programs. Together, these studies provide a comprehensive framework for family-centered CF care.

Chapters 2 and 3 make a substantial contribution to the non-pharmacological management of CF.

Chapter 2 (Study I) explored the psychological and logistical challenges faced by caregivers, offering valuable insights into the barriers and facilitators of CF management. Chapter 3 (Study II) demonstrated the efficacy of a tailored home-based exercise program, KidMove, in improving physical and emotional well-being, presenting a viable model for integrating exercise into daily routines.

This chapter presents an integrated discussion of all findings based on the most recent research. It provides a comprehensive overview, emphasizing the two primary areas of knowledge advancement: the impact of disease and physical exercise, and treatment adherence. A review of general limitations and suggestions for further study and application concludes this chapter.

### Managing the burden of cystic fibrosis: challenges and solutions

Chapter 2 (original study I) addressed an important standard in healthcare, empowering individuals with CF and their families to collaborate with healthcare teams to define their goals and aspirations. It has been established in the literature that CF teams should take a proactive approach to recognizing and addressing CF-specific health issues. This provides vital support for the mental and emotional well-being of individuals with CF and their families, facilitating their journey toward achieving a balance between quality of life, treatment burden, and clinical outcomes (Southern et al., 2024). Additionally, it demonstrated that CF has a profound impact not only on affected children but also on their families, with caregivers bearing significant emotional and logistical burdens (Daly et al., 2022). The primary emotional strain on parents arises from the demanding and complex nature of CF treatments, frequent hospitalizations, and the

unpredictable nature of exacerbations (Prieur et al., 2021) These challenges are especially pronounced during the initial period following a CF diagnosis when caregivers face high levels of stress, anxiety, and depression due to the need to balance daily treatments and hospital visits alongside family and work responsibilities (Quittner et al., 2016).

Chapter 2 (original study 1) emphasizes the importance of attitudes toward the disease and social support that findings are consistent with previous research, which similarly emphasizes the psychological toll associated with caring for children with chronic illnesses, such as CF (Daly et al., 2022; Quittner et al., 2016). Financial constraints were also raised as a problem, particularly when treatments are not fully covered by insurance and require out-of-pocket expenses, further exacerbating caregivers' emotional stress (Southern et al., 2024).

Moreover, Study I (Chapter 2) indicated that, over time, parents develop enhanced coping mechanisms, particularly when they have access to robust social support networks and adequate healthcare resources. This strategy is aligned to adopt a more person-centered approach and emphasizes the vital importance of incorporating individuals into collaborative decision-making processes (Shelley et al., 2022). However, Chapter 2 revealed the significance of the timing of diagnosis. Parents whose children were diagnosed early in life reported feeling progressively more capable of managing the disease (Coverstone & Ferkol, 2021). This may result from the gradual normalization of treatment routines and an enhanced understanding of the disease (Castellani et al., 2023). An accurate and prompt diagnosis is crucial for minimizing uncertainty and ensuring optimal care. These findings align with existing literature, which suggests that early diagnosis, combined with effective medical and social support, promotes superior long-term adaptation (Southern et al., 2024) and disease management (Castellani et al., 2023).

Treatment adherence, particularly physical exercise, was identified as a significant challenge. Caregivers expressed concerns regarding potential exercise-related risks, including fatigue and respiratory exacerbations. Nonetheless, they recognized the role of physical exercise in supporting lung function and overall well-being. Barriers to exercise, such as logistical challenges and time constraints, reflect findings in the literature, where limited time and insufficient support hinder engagement in physical exercise (Denford et al., 2020). This dual role of exercise—as both a barrier and a facilitator—illustrates the inherent complexity of managing CF in a home setting.

Furthermore, caregivers stated that managing CF became harder as children grew older because of the growing involvement of social and scholastic obligations and disease

management. In keeping with previous research highlighting the difficulties of managing CF during adolescence, caregivers of older adolescents frequently reported greater difficulty sustaining adherence to both medicines and physical exercise (Narayanan et al., 2017). Because CF is a progressive disease, treating it during crucial developmental phases emphasizes the need for flexible, kid-centered therapies, like physical exercise regimens that can be tailored to each person's requirements and preferences. This qualitative study provided valuable information for the design of the interventions, including the context, the type of activities, and flexibility in the choice of exercises.

**Home-based exercise: a promising approach to sustaining physical exercise**

The original Study II (Chapter 3) demonstrated the effectiveness of the KidMove home-based exercise program, presenting strong evidence that such interventions can improve both aerobic capacity and emotional well-being in children with CF. The improvement in aerobic capacity, assessed by the mswt, aligns with findings from previous research demonstrating the positive impact of structured exercise programs on the physical fitness of CF individuals (Radtke et al., 2022). These findings underscore the potential of home-based exercise programs as a valuable alternative to traditional center-based exercise programs, benefiting individuals in terms of exercise capacity and symptom management when adequately supervised (Uzzaman et al., 2022).

The flexibility of a home-based setting, combined with tailored activities that were both functional and enjoyable, and active parental supervision contributed to adherence to the KidMove program. These elements not only promoted sustained engagement but also demonstrated that well-designed home-based programs can effectively improve physical exercise capacity and enhance quality of life (Cecins et al., 2017). This approach offers a practical and accessible alternative to traditional center-based interventions, making it easier for families to integrate physical exercise into their daily routines and meet the unique needs of children with CF (Junior et al., 2023; Uzzaman et al., 2022).

Providing an accessible environment in the KidMove program reduces barriers related to travel and facility access, making it easier for children to participate in regular physical exercise. Customization of activities ensures that the program is tailored to each child’s specific abilities and needs, thereby maximizing individual relevance and effectiveness (Del Corral et al., 2018; Gruet et al., 2022; Radtke et al., 2022).

Incorporating functional and enjoyable activities helps maintain participants' interest and engagement by making the exercises practical and appealing (Radtke et al., 2017).

Comprehensive support systems, including in-person and telephone assistance, further enhance adherence by offering educational and psychosocial resources, such as informational worksheets and a manual (Hommerding et al., 2015). Additionally, emphasizing parental involvement through supervision and guidance plays a crucial role in fostering consistent participation and motivation, which are essential for the program's success (Stafinski et al., 2022).

By empowering children to engage in physical exercise at home, the KidMove program fostered a sense of control and normalcy, contributing to the observed improvements in emotional well-being (Prieur et al., 2021). Although no significant changes were noted in lung function, the observed enhancements in endurance and emotional well-being highlight the importance of including physical exercise as a core component of CF management. Previous studies have indicated that sustained physical exercise can help preserve lung function over time, even if immediate changes are not always detectable (Radtke et al., 2017).

Therefore, these results highlight KidMove as a straightforward, meaningful, and person-centered intervention that is effective and efficient in adherence to physical exercise in daily routines without overly weighted time schedules by utilizing commonplace resources (such as water bottles or body weight) and integrating activities into individuals' daily routines. The physical exercise manual has been designed to provide a comprehensive range of exercises across various training modalities, enabling individuals and their families to tailor the intervention according to their preferences and scientific knowledge. This manual can be used by children, parents, and health professionals to optimize the long-term benefits of exercise. In alignment with the European Cystic Fibrosis Society recommendations, this manual encourages the involvement of all stakeholders in the formulation of health objectives to enhance health-related variables (Saynor et al., 2023; Southern et al., 2024).

A distinctive feature of the KidMove program is its strong emphasis on parental involvement. Parents were encouraged to engage in two key roles: firstly, to supervise their children during exercise sessions, and secondly, to provide encouragement and motivation to their children. This family-centered approach contributed to the high adherence observed, as caregiver involvement is a critical factor in exercise adherence among children with chronic illnesses (Prieur et al., 2021). By actively engaging parents,

the program enhanced its effectiveness, ensuring that children remained engaged and motivated throughout the intervention (Happ et al., 2013). Adherence to the KidMove program can indeed be attributed to behaviour changes, which are essential for sustaining long-term engagement in any exercise or health intervention. Behaviour change theories, like the COM-B model (Capability, Opportunity, and Motivation-Behaviour) (West & Michie, 2020), provide a structured framework for understanding how adherence to a program like KidMove can be facilitated through targeted changes in behaviour (figure 3). By equipping children and their families with the skills, knowledge, and confidence to perform exercises at home, the program enhances participants' physical and psychological capabilities (Denford et al., 2020). This was achieved through customized exercises, instructional materials, and supportive guidance, which helped children feel more competent in managing their activities, increasing the likelihood of sustained engagement (Narayanan et al., 2017). The program's home-based format reduced the logistical and environmental barriers, creating an accessible environment that makes participation easier. KidMove incorporated functional and enjoyable activities, providing children with an ideal opportunity to integrate physical exercise into their daily routines. This key behaviour change supports consistent adherence to the program (Gruet et al., 2022). The focus on family-centered support, particularly parental involvement, further fostered motivation and sustained engagement (Barnes et al., 2020b). When parents were actively engaged in supervising and encouraging their children, it helped sustain the children's motivation over time. Additionally, support mechanisms such as regular check-ins, educational resources, and goal setting enhance intrinsic motivation, helping children see the benefits of regular exercise beyond short-term gains. Sustained adherence to the KidMove program could be further reinforced through continued behaviour change.

As children and their families establish lasting routines, build confidence, and experience ongoing physical and emotional benefits, these positive outcomes are likely to encourage continued engagement with the program. This approach aligns with evidence suggesting that behaviourally driven programs that emphasize capability, opportunity, and motivation are highly effective in supporting sustainable health behaviours (Jerebine et al., 2024).

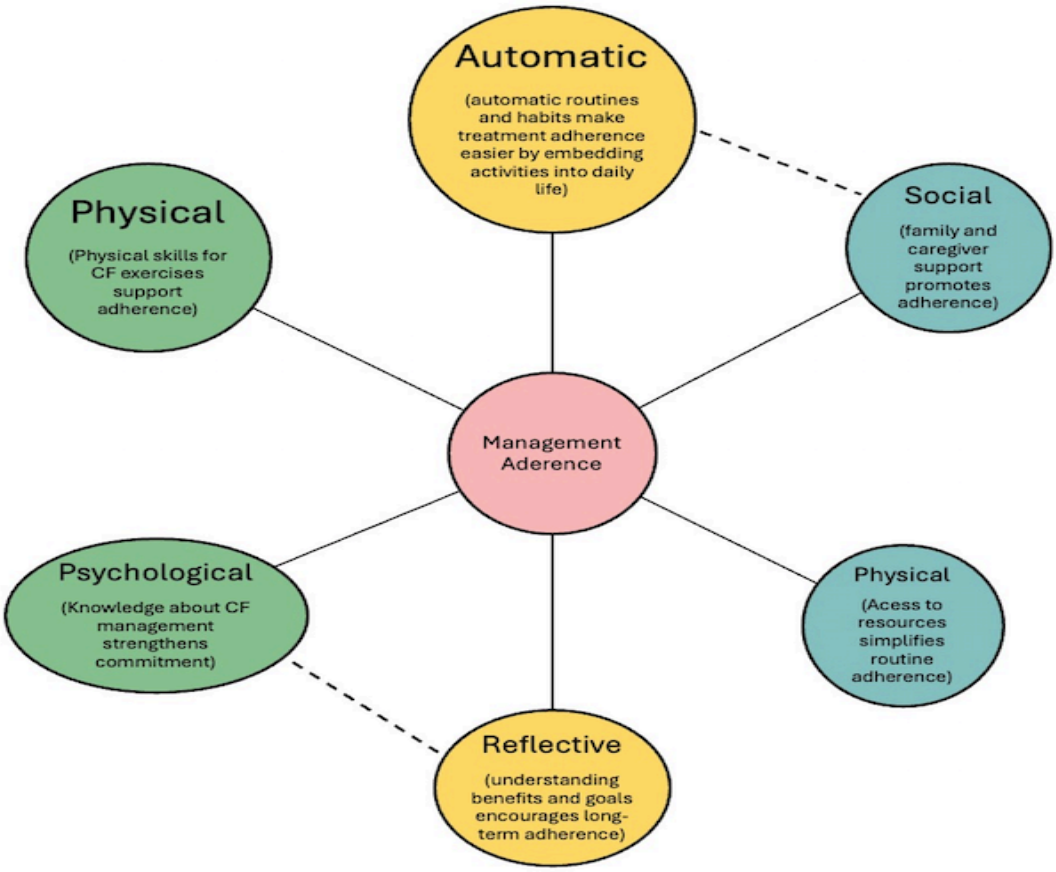


**Figure 1** – Diagram illustrating the main factors of KidMove that promote adherence to exercise and maintenance of physical exercise.
**Legend**: capability–skills, and knowledge; opportunity and access; management adherence (behaviour); motivation–habits and goals; direct influence on adherence; - - - support influence on components

Fostering sustainable health behaviours, such as regular exercise, is vital for managing chronic conditions like CF. These behaviours must be integrated into daily life, balancing personal and disease-related demands.

The success of this approach depends on addressing **capability**, by equipping individuals and families with the necessary knowledge and skills; **opportunity**, by removing barriers and creating supportive environments like home-based interventions; and **motivation**, by reinforcing benefits, building confidence, and ensuring sustained engagement. Together with tailored professional support, these elements empower patients, promote long-term adherence, and enhance autonomy, resilience, and overall health outcomes.

**Limitations**

It is essential to critically acknowledge the limitations of this Thesis when analyzing its findings. In Study I, data triangulation among parents, children, and health professionals was not employed, which limits the scope of perspectives and potentially reduces the comprehensiveness of insights related to the facilitators and barriers in CF management. Furthermore, the sample in this initial study was restricted to a single reference center in Portugal, limiting the generalizability of the findings. Regional variations could influence the facilitators and barriers to program adherence, suggesting that further research across different regions would provide a more comprehensive understanding. To address this limitation, a subsequent study was conducted with a larger and more geographically diverse sample, employing an alternative methodology to broaden the findings' contextual relevance.

Additionally, interviews in the first study were conducted in Portuguese, which introduces the potential for translation loss, impacting the nuanced interpretation of participants' experiences. This constraint limits the applicability of findings to individuals with more severe CF manifestations.

In study II, the intervention's evaluation period was short, spanning only three months, which may not capture the full scope of long-term adherence or efficacy. Consequently, caution is warranted in interpreting these results, as they may not fully represent long-term effects or less apparent outcomes. Additionally, the sample was limited to individuals with a $FEV_1$ greater than 40%, which restricts the applicability of these results to children and young individuals with milder CF symptoms. Findings may not extend to individuals experiencing more advanced disease progression or lower $FEV_1$ values.

Moreover, the nature of the intervention precluded participant blinding, which could introduce bias in self-reported adherence and outcome measures. Furthermore, the adoption of a quasi-randomized controlled trial design, dictated by the inherent difficulty of recruiting a sufficiently large sample size within this population, limits the robustness of the findings. These limitations underscore the need for future research to address these methodological constraints, thus enhancing the broader applicability and depth of understanding of the KidMove program's effectiveness in CF management among younger populations.

Despite these limitations, this Thesis provides a valuable contribution to person-centered care by introducing a home-based physical exercise program specifically tailored for individuals with CF. Through various methodological approaches, this Thesis presents robust evidence to support the effectiveness of this intervention. Further investigation is essential to expand on these promising results and validate the program's potential benefits for improving CF care.

# Chapter 5. Conclusion, implications for practice, and future research

## Conclusion and implications for practice

In conclusion, the studies presented in this thesis provide valuable insights into the multifactorial impact of CF on children and their caregivers, and the efficacy of tailored home-based interventions. By addressing both the physical and psychological needs of CF individuals and their families, these findings offer a more holistic approach to CF management, with the potential to improve quality of life and long-term health outcomes.

The findings from both studies have important clinical implications. The emotional and logistical burden on caregivers highlights the need for comprehensive, family-centered care models that provide both medical and psychosocial support. Healthcare providers should prioritize interventions that consider the unique challenges faced by families, particularly in the early stages of diagnosis. Additionally, support systems, such as school programs and community resources, should be strengthened to provide a more supportive environment for children with CF and their caregivers.

The effectiveness of the KidMove program highlights the potential of home-based interventions as a viable and effective alternative to clinic-based rehabilitation for patients with moderate disabilities. These programs' inherent adaptability and accessibility facilitate their integration into daily routines, fostering sustained engagement and adherence over time. In the context of care provision, this emphasizes the importance of adopting a model where users and their families actively participate in disease self-management, supported by health professionals through structured monitoring and evidence-based counselling. This approach reduces reliance on traditional, resource-intensive hospital-based care models, often requiring multiple weekly visits, and underscores the feasibility of remotely supervised interventions. Such strategies enhance treatment adherence, improve clinical outcomes, and alleviate the logistical and financial burdens typically associated with hospital-based care, thereby promoting a more sustainable and patient-centered healthcare model.

## Implications for future research

Future research should build upon the findings of this thesis to refine and expand care strategies for individuals with cystic fibrosis and similar conditions. Future studies should focus on improving home-based exercise programs by incorporating elements tailored to each patient’s individual needs and preferences, thereby further increasing their efficacy. Larger randomized controlled trials are required to validate these findings

on a broader scale and in diverse settings, while also investigating the long-term benefits of home-based exercise therapy on lung function, quality of life, and overall disease progression. Additionally, exploring e-Health monitoring strategies could enhance remote supervision, providing real-time feedback and increasing adherence to exercise programs.

Another critical area involves analyzing the effects of exercise in specific subgroups, such as those with different classes of genetic mutations or severe disease classifications (e.g., $FEV_1$<40%), to create interventions tailored to unique patient profiles. Multicenter randomized control trials could further validate the benefits of home-based interventions across diverse populations and healthcare contexts. It is equally important to assess the medium- and long-term effects of these interventions on clinical, functional, and psychosocial outcomes to determine their sustainability and broader impact over time.

Carrying out cost-effectiveness analyses is essential to evaluate the economic feasibility of implementing such programs on a larger scale, particularly within resource-constrained healthcare systems. Engaging the educational community to address the needs of children with cystic fibrosis and their parents could foster increased awareness, reduce stigma, and provide targeted support. Exploring the needs of informal caregivers and assessing the impact of home-based programs on their well-being and caregiving burden would further promote a family-centered approach to care. Finally, establishing minimal clinically important differences for key outcomes in this population would enhance the interpretation of results and improve the design of future trials.

These research directions aim to optimize the design, delivery, and sustainability of home-based interventions, improving health outcomes, quality of life, and caregiving experiences for individuals with chronic conditions and their families.

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# Chapter 7. Appendices

**Appendix 1**. Scientific outputs with the scope of this thesis

Publication in peer-reviewed journals

Gagulic, S.; Bártolo, A.; Marques, A. Effects of a Tailored Home-Based Exercise Program, "KidMove", on Children with Cystic Fibrosis: A Quasi-Experimental Study. *Healthcare* **2024**, *13*, x. https://www.mdpi.com/2227-9032/13/1/4

Peer-reviewed oral communications

Gagulic, S.; Bártolo, A.; Marques, A. (2024) " Effectiveness of a tailored home-based exercise program ["KIDMOVE"] on the aerobic capacity and quality of life of children with cystic fibrosis" 40th Congress of Pulmonology of the Portuguese Pulmonology Society, 14th-16th November 2024, Lisboa, Portugal

## **Appendix 2.** Informed consent forms

**Programa de exercício domiciliário: Efeitos na qualidade de vida, aptidão física e função pulmonar em crianças e jovens com Fibrose Quística.**

**Formulário de Consentimento Informado**

*Por favor leia com atenção o texto seguinte, que pretende descrever pormenorizadamente o estudo a desenvolver, de modo a que possa ficar bem informado(a) e, se assim o entender, autorizar a participação no mesmo. Ao terminar esta leitura, se tiver qualquer dúvida sobre qualquer aspecto, não hesite em pedir o seu esclarecimento ao médico da Consulta de Fibrose Quística.*

Este estudo pretende investigar os efeitos de um programa de exercícios domiciliário na qualidade de vida, aptidão física e função pulmonar de crianças e jovens com diagnóstico de fibrose quística de gravidade ligeira e moderada.
A investigação sobre exercício na fibrose quística mostra que a prática de um plano de exercícios domiciliário contínuo ajuda a melhorar a condição óssea, muscular e cardio-respiratória, bem como a qualidade de vida das crianças e jovens com esta doença.

Os principais objectivos do estudo são:
1) Caracterizar as crianças e adolescentes com fibrose quística (e os pais) de acordo com a Classificação Internacional de Funcionalidade (CIF),
2) Elaborar, de forma adaptada a cada participante, e implementar um programa de exercícios domiciliário baseado nas necessidades e interesses das crianças e adolescentes com fibrose quística ligeira e moderada,
3) Avaliar os resultados da aplicação desse programa, de acordo com parâmetros da qualidade de vida, aptidão física e função pulmonar.

**Descrição:**
Serão convidados a participar no estudo apenas as crianças e adolescentes que os médicos responsáveis pela consulta de fibrose quística considerem ter uma situação clínica que possibilite a participação no estudo sem riscos acrescidos.
O programa completo de exercício terá uma duração total de 12 semanas.
Antes do início do estudo, num dia em que vier a uma consulta já agendada, para além da consulta habitual com o médico responsável, serão realizados alguns testes físicos e uma entrevista.
Nesta consulta, serão informados pelo médico responsável e pela investigadora de quais os exercícios a realizar em casa, de como será efectuado o acompanhamento, das situações em que pode ser necessário interromper o exercício, bem como da atitude a tomar se houver agudização da doença durante o programa.
Esta consulta, antes do início do programa de exercício, será mais demorada que a consulta habitual. As consultas seguintes serão as habitualmente programadas, não sendo marcadas consultas adicionais pela participação no estudo.
A participação no estudo não implica qualquer tipo de encargo financeiro adicional.
A entrevista tem como objetivo partilhar necessidades/dificuldades em lidar com a doença, explicar a importância da prática de exercício domiciliário e perceber as preferências de exercícios de cada uma das crianças/adolescentes, bem como as limitações sentidas.
Para avaliar a actividade física habitual, durante a semana inicial do estudo deve utilizar um pequeno aparelho que regista a actividade física todos os dias. A utilização do aparelho não apresenta qualquer risco ou desconforto para a criança/adolescente. Deverá ser retirado apenas quando está a dormir e tomar banho. O aparelho é colocado à cintura, por baixo da roupa.
O programa de exercícios será programado para cada participante com base na avaliação inicial da sua capacidade física e será aprovado pelo médico responsável. Deve ser realizado três vezes por semana, com uma duração de cerca de 60 minutos cada dia. A supervisão e participação dos pais são extremamente importantes. Todas as outras atividades

habituais do seu filho devem ser mantidas. A realização do programa de exercícios indicado, e validado pelo médico responsável, não acarreta risco físico; no entanto, pode ser interrompido em qualquer altura, se decidir abandonar o programa, ou em caso de agudização respiratória ou outra, sem qualquer modificação posterior dos cuidados habituais. A investigadora contactará por via telefónica duas vezes por semana para acompanhamento e esclarecimento de dúvidas e informará o médico sobre o resultado do contato, mas, caso tenha necessidade de contactar a investigadora ou o médico assistente, serão disponibilizados meios de contacto, que pode utilizar quando for necessário. A investigadora informará o médico assistente sobre o decorrer

Após as doze semanas do programa será realizada uma avaliação semelhante à avaliação inicial na consulta de fibrose quística. Todos os resultados do estudo ser-lhe-ão comunicados. Toda a confidencialidade e anonimato serão respeitados no tratamento e eventual apresentação e publicação dos resultados do estudo.

Verifique se compreendeu todas as informações. Se não tiver dúvidas por esclarecer e se concordar com a participação no estudo, deverá ser assinado o seguinte consentimento pelo participante e/ou seu representante legal.

**CONSENTIMENTO INFORMADO**

Declaro ter compreendido os objectivos que foram propostos e explicados pelo médico e pela investigadora que assina este documento, tendo tido oportunidade de fazer todas as perguntas sobre o assunto e para todas elas ter obtido resposta esclarecedora. Foi garantido que não haverá prejuízo nos cuidados clínicos e direitos assistenciais prestados, caso recuse esta solicitação, ou caso haja interrupção do programa por qualquer razão. Toda a confidencialidade dos meus dados foi garantida, sendo apenas utilizados no âmbito da investigação.

Foi-me dado tempo suficiente para reflectir sobre esta proposta.

- Autorizo a participação no estudo, incluindo os procedimentos de diagnóstico necessários para a avaliação inicial e para a avaliação dos resultados do estudo.
- Autorizo que o médico responsável pela consulta de Fibrose Quística forneça à investigadora dados do processo clínico necessários para a realização deste estudo.

Nome do participante no estudo:

_____

Nome da pessoa que autoriza (se não for o doente):

_____

Assinatura:_____

Doente ___ Pai/Mãe ___ Outro (especificar) _____

Data _____

Médico Responsável da Consulta de Fibrose Quística:_____

Investigadora: _____

# Chapter 8. Annexes

**Annexe 1 – KidMove: O meu diário de atividade física – registo individual**

KidMove

*O Meu Diário de Actividade Física*

Registo Individual

KidMove

*O que precisas de saber...*

O exercício físico é muito importante para te sentires com mais energia e mais força.

Tenta fazer exercício pelo menos três vezes por semana até uma hora, vai ajudar te a ficares mais ativo.

Quando te sentires cansado ou com muita tosse, pára um bocadinho bebe água, escolhe uma das posições abaixo e respira calmamente, tentando inspirar pelo nariz e expirar pela boca e depois podes continuar. Caso estejas muito cansado, amanhã tentas outra vez.

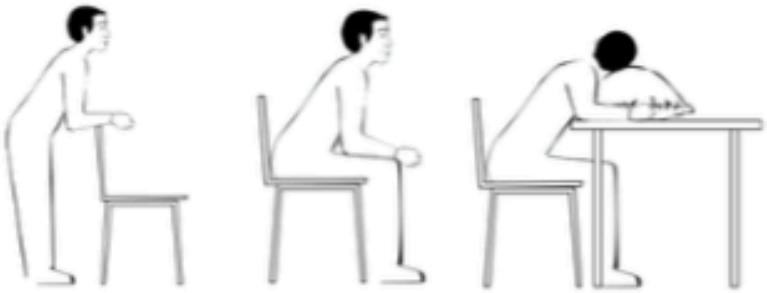
Não te esqueças de beber água diariamente.

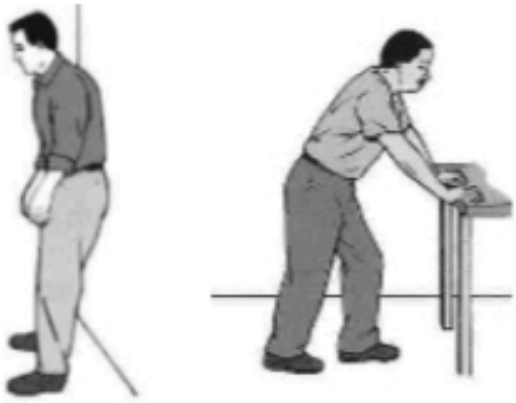
DIVERTE TE

KidMove

## Posições de alívio de dispneia

Inspira lentamente pelo nariz contando até 2 e expira lentamente pela boca, com os lábios semi-cerrados contando até 4





KidMove

## Cuidador

### *O que precisa saber...*

Deve incentivar o seu filho (a) à prática de exercício, pois faz bem aos seus pulmões, músculos e ossos. O exercício deve ser feito pelo menos três vezes por semana durante 1 hora, devendo fazer se possível todas as categorias que estão no plano de exercícios e que ele goste.

Se for possível fazer o plano de exercícios com ele/ela seria o ideal, pois também faz bem à sua saúde e ajuda que ele/ela mantenha a vontade de continuar a fazer exercício.

É importante que registe o tipo de exercícios e o tempo que fez para conseguir ver a sua progressão.

KidMove

# Registo Individual

## KidMove Semana 1

| Data | Hora | Exercício Escolhido | Duração | Supervisão | | | Sinais e Sintomas | | | | |
|---|---|---|---|---|---|---|---|---|---|---|---|
| | | | | Sim | Não | Quem? | Dor | Dispneia | Esforço | Tosse | Vómitos |
| | | | | | | | | | | | |
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