Examining the effects of Trikafta therapy on health-related fitness, physical activity and quality of life in children with cystic fibrosis

da Silva S.M.[1], Pedder L.[2], Timmons B.W.[1,2,3], Obeid J.[1,2,3]

[1]Child Health & Exercise Medicine Program, McMaster University. [2]Department of Pediatrics, McMaster University. [3]Department of Kinesiology, McMaster University

Background

- CF transmembrane conductance regulator (CFTR) modulators, such as Trikafta, have revolutionized Cystic fibrosis (CF) treatment, showing unparalleled improvements in clinical outcomes.[1,2]
- Our understanding of the effects of Trikafta therapy on important markers of health and well-being in children with CF remains limited.

Objective

- Examine the impact of 1-month and 6-months of Trikafta therapy on body composition, aerobic fitness, physical activity, exercise metabolism, and quality of life (QoL) in children with CF.

Methods

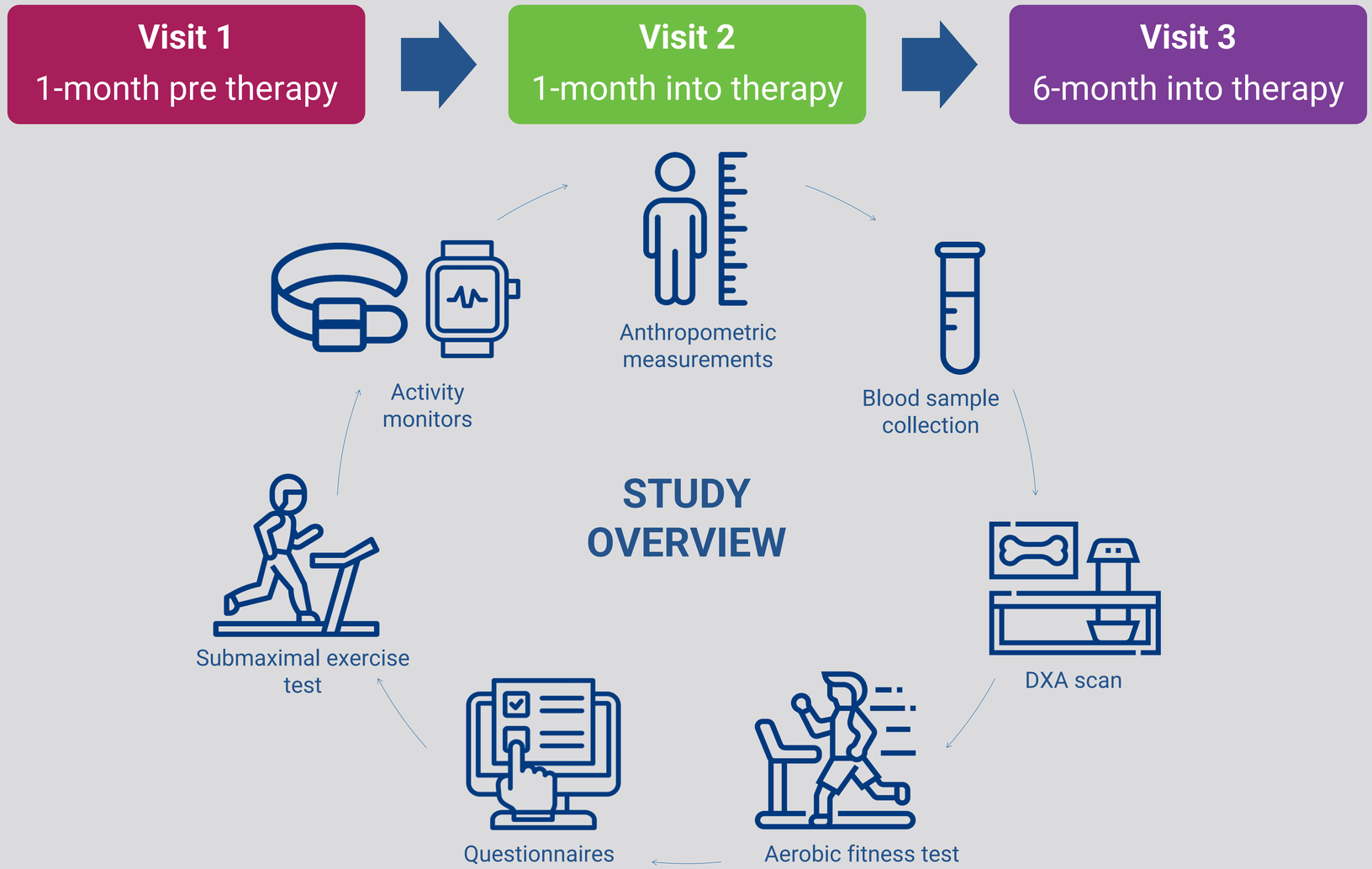
- We will recruit boys and girls aged 2-17 years with a confirmed diagnosis of CF, beginning Trikafta therapy.
- 3 identical study visits with measurements of:
  1. Body composition: Whole body and regional dual-energy X-ray absorptiometry to measure muscle, fat, and bone mass, and bone mineral density.
  2. Aerobic fitness and exercise metabolism: Treadmill testing with the Modified Bruce protocol and a submaximal steady-state protocol, respectively.
  3. Physical activity: Both a Garmin watch and an ActiGraph accelerometer will assess daily activity.
  4. QoL: Parent- or self-reported using Cystic Fibrosis Questionnaire-Revised (CFQR) and Peds QL.

Anticipated Results

- Based on the limited existing evidence, we expect:
  1. Body composition: ⇧ in weight, fat mass, and bone mineral density at 1 and 6 mos.[2,3,4]
  2. Aerobic fitness and exercise metabolism:
     - ⇧ in peak oxygen uptake at 1 and 6 mos.[5]
     - ⇧ fat oxidation matched to changes in fat mass.[7]
  3. Physical activity: ⇧ all intensities at 1 mo.[5,6]
  4. QoL: improve at 1 mo and sustained at 6 mos.[8,9]

Significance

- Expand our understanding of the effects of Trikafta therapy beyond traditional clinical outcomes.
- May offer evidence-based recommendations to support healthcare professionals caring for youth with CF initiating Trikafta therapy.



Examine the impact of **1-month** and **6-months** of Trikafta therapy on **changes in** body composition, aerobic fitness, exercise metabolism, physical activity, and QoL.

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