

Patient stratification blood test for cystic fibrosis

DIAGNOSTIC

Product Type	Blood-based diagnostic assay to provide personalized treatment pathways for CF
Indication / ROA	Cystic fibrosis (CF)
Target / MoA	Mitochondrial function (oxygen consumption rate); this assay evaluates the efficacy of CF drugs by measuring the oxygen consumption rate of the mitochondria derived from biological samples (blood) of a CF patients.
Development Stage	Validated using patient blood samples for response to drug
Brief Description & Differentiation	<p>Individual CF patient have distinct CF phenotypes, wherein not all drugs will be efficacious for treating all CF patients. Unfortunately, the current approach in determining the efficacy of a proposed CF-drug is to conduct lengthy clinical trials and CF patients are subjected to a range of pharmacological agents prior to obtaining clinical benefit, which can be costly and inconvenient. As such, the present invention provides a method for evaluating the efficacy of an agent for treatment CF using just the biological sample (i.e. blood) from a CF patient based on the difference on mitochondrial function observed in CF patients (Figure 1). Increase in mitochondrial function (oxygen consumption rate) in the biological sample upon contact with a marketed drug for CF indicates patient response to drug (Figure 2).</p> <ul style="list-style-type: none"> Information on patient responders can be acquired rapidly (within hours), based on a single blood test in individual patients Repeatable for rapid verification of results Low cost method of implementing personalised medicine
Research Team	Prof John Wilson, A/Prof Tom Kotsimbos
Intellectual Property	Provisional patent application has been filed covering the assays and methods for determining the efficacy of one or more pharmaceutical agents for improving mitochondrial function in an individual
Key Publications	Allen-Graham J. <i>et al.</i> Mitochondrial dysfunction in cystic fibrosis. <i>Journal of Cystic Fibrosis</i> . 2017, 1: s72-73.
Future	Development as a companion assay to evaluate drug efficacy of individual CF patients, as a tool for streamlining CF clinical trial candidates.

➤ Key Data

Demonstrates increased mitochondrial function in CF patient blood sample upon treatment with approved CF drug, Orkambi® (lumacaftor/ivacaftor), indicative of patient response to drug.

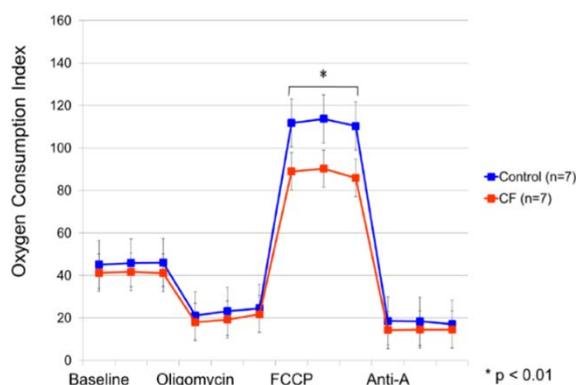


Figure 1. Measurement of mitochondrial respiration (oxygen consumption rate) in the peripheral blood mononuclear cells (PBMCs) derived from CF patients compared to control.

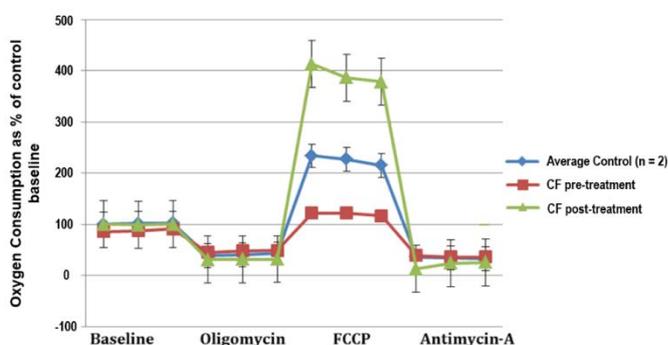


Figure 2. Measurement of maximal oxygen consumption in the PBMCs derived from CF patient pre- and post-treatment with a marketed CF drug.