**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 patient.

**Development Stage**: Validated using patient blood samples for response to drug

**Brief Description & Differentiation**: 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).

- 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


**Future**: Development as a companion assay to evaluate drug efficacy of individual CF patients, as a tool for streamlining CF clinical trial candidates.

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**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.