PERSONALIZING MEDICINE FOR CYSTIC FIBROSIS

Cystic Fibrosis Canada

SickKids

BECAUSE EACH PERSON IS DIFFERENT

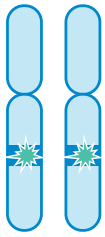
> "**Personalized medicine brings hope** to our family, and to our sons, Stephen and Ryan, who have the same CF mutations but different CF symptoms. **Learning which treatments work best** for each of them would lead to **better care** and help each of them to **lead healthier lives**." ANNE MARIE DRAKE, CF PARENT

HOW DO PEOPLE WITH CF DIFFER FROM EACH OTHER?

1. TYPE OF CF MUTATION

CF is caused by mutation of the *cystic fibrosis transmembrane conductance regulator (CFTR)* gene. Over 2000 mutations have been discovered in the gene that causes CF.

2000 + MUTATIONS



CFTR gene

2. ENVIRONMENT

Food, exposure to second-hand smoke, outdoor pollution, amount of exercise, exposure to bacteria, living conditions and stress levels can directly affect CF disease and treatment response.

3. GENETIC BACKGROUND

Secondary, non-*CFTR* genes (modifier genes) are being identified and studied as potential targets for treatment.

CF DISEASE SEVERITY CAN VARY WIDELY EVEN AMONG PEOPLE WITH THE SAME DISEASE-CAUSING MUTATIONS.

STEP ONE

Recruit 100 people with CF and **collect blood and nasal cell samples** from them.

STEP TWO

Catalogue each person's **genetic makeup**.

STEP THREE

Grow stem cells from each person's blood sample.

A stem cell is a cell that can develop into many different cell types in the body.

STEP FOUR

Use each person's stem cells to **grow different types of cells**, such as lung and intestinal cells.

STEP FIVE

Use these cells to **test existing and new drugs and combinations** to find out their effectiveness for that individual, enabling personalized treatment.