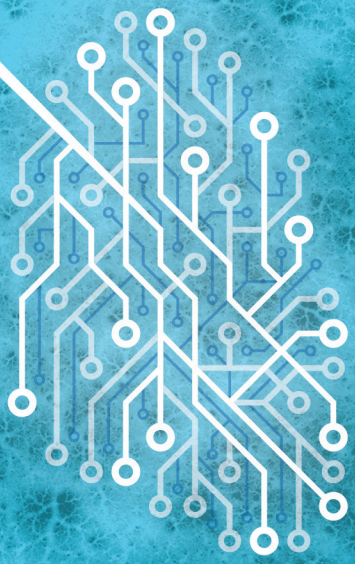
TRANSFORMING HOPE



Great advances have been made in cystic fibrosis (CF) treatment in our lifetime. Join Cystic Fibrosis Canada and SickKids as we transform the future for people living with CF.

We have never been closer to a cure for CF. Advances made in CF genetics—from the discovery of the CF gene 25 years ago to the development of targeted treatments within the past 5 years—have created tremendous hope that a child diagnosed with CF today will live a long, healthy life.

Together, Cystic Fibrosis Canada and SickKids are creating a national resource to rapidly develop effective, personalized treatments for every person with CF. Help us bring the dream of a cure closer to reality.

Cystic Fibrosis Canada™

SickKids®

HOW MATTHEW, A CYSTIC FIBROSIS PATIENT, IS BRINGING HOPE TO OTHERS WITH THE DISEASE

Matthew is a happy toddler who brings joy to everyone he meets. And for such a small person, though he doesn't yet know it, he's already having a big impact on the future for CF patients.

When Matthew's family heard about CF research being done through the **Program for Individualized Cystic Fibrosis Therapy**, they jumped at the chance to help researchers develop new treatments for their son and other children with CF.

Stem cells were harvested from a sample of Matthew's skin and used to create lung cells so researchers can see how different drugs work on his specific form of the disease.

"There were really two reasons for joining this research study: a little hope and a lot of fear," says Gillian, Matthew's mom. "My optimistic side would say a lot of hope."

Looking at the improvements in CF care within our lifetime, it's incredible how far we've come. With your help, there's so much more we can do.

Join Cystic Fibrosis Canada and SickKids to transform hope for children like Matthew and their families.

THERE IS NO BETTER TEAM TO TACKLE CYSTIC FIBROSIS

Together, Cystic Fibrosis Canada and SickKids have driven a number of landmark discoveries in cystic fibrosis (CF) research, including identification of the gene responsible for CF in 1989. We were also the first to show that CF is a defect of a chloride channel—the process by which salt is drawn to the surface of mucus membranes. Together, these discoveries led to the development of KALYDECO, the first drug that repairs the defective CF gene instead of relieving disease symptoms.

KALYDECO has dramatically improved quality of life for approximately 5 per cent of CF patients. However, no similar treatment exists for the majority of people affected by CF. We have made incredible progress, but we still have more work to do to ensure every CF patient can control their disease and live a long, healthy life—and we need your help.

The work of Cystic Fibrosis Canada and SickKids is testament to what can be accomplished when communities join together to improve the lives of the thousands of Canadians affected by CF. With your support, we will revolutionize the way CF is treated and change the future for people affected by this disease.

THE PROGRAM FOR INDIVIDUALIZED CYSTIC FIBROSIS THERAPY

Drawing from leading programs and infrastructure in stem cells, genetics, and cystic fibrosis, SickKids is the world's most advanced centre for research into the genetic basis of CF and the use of stem cells to identify new treatments. Now, Cystic Fibrosis Canada and SickKids are bringing our work to the next level by establishing the **Program for Individualized CF Therapy**, a national resource dedicated to individualized CF drug discovery. With two thousand different CF gene mutations, each patient is different. Our goal is to ensure every CF patient has access to the life-changing treatments that will work best for them, as quickly as possible.

We have never been closer to being able to treat cystic fibrosis as a chronic, yet controllable illness. SickKids researchers now use lung cells, grown from stem cells harvested from a patient's skin or blood, to test the effectiveness of a potential therapy on a specific patient. New technology is allowing scientists a better understanding of complex genetic factors that affect an individual's response to treatment. And processes that once took years—like screening drugs before they can be tested in humans in a clinical trial—can now be performed in the lab in a fraction of the time.

Cystic Fibrosis Canada has committed to raising **$7.5 million** over five years towards the development of more effective, personalized therapies for CF. With your support, we will accelerate the discovery of new treatments and determine the best treatments available on an individual basis, ensuring there is no patient without a treatment option to live a healthy life.

PROGRAM OVERVIEW

The Program for Individualized CF Therapy will be the world's first resource dedicated to the discovery of more effective, personalized treatments for CF.

Investments in this initiative will create the platform necessary to harness technological and scientific advances through:

STEM CELL COLLECTION

Samples from at least 100 CF patients that reflect the diversity of genetic mutations within the Canadian population are required to build a database of stem cells, which will be made available to researchers around the world.

GENETIC ANALYSIS

Each patient will help researchers understand the genetic factors affecting an individual's disease severity and response to therapy, and allow them to predict which therapies will work best for each patient.

PATIENT-SPECIFIC, PRE-CLINICAL TRIALS

Researchers will be able to rapidly test new and existing drug compounds on multiple cell samples at the same time, predicting a drug's effectiveness before it is tested on patients.

CLINICAL TESTING

This will allow patients who might benefit from a specific therapy to receive treatment as soon as possible, and rapidly bring new drugs to market.

COLLABORATION & INNOVATION

Catalyst and travel grants will position the Program as a hub for global collaboration and enable the world's most innovative ideas in CF research to come to fruition.

HOW YOU CAN HELP

Your gift to this initiative will help us develop a platform that will enable the rapid discovery of new drugs to tackle CF. More than that, you will help us bring hope to affected families—and the possibility of a cure. Funding opportunities include the following:

Research Director **$1M**

This globally recognized CF researcher will provide leadership to the Program and seed collaborations with other institutions around the world.

Resource Platforms **$500K** (4)

Investments are needed to create four critical components of our Resource Platform, the infrastructure necessary to discovery and bring to market more effective, personalized CF therapies:

- **Stem Cell & Tissue Resource**
- **Genetic Database & Patient Biobank**
- **Pre-Clinical Trial Resource**
- **Clinical Testing Resource**

Cell Line Partner **$250K** (4)

In order to ensure that we have the greatest chance to help the most patients, we need to recruit at least 100 patients representative of the Canadian CF patient population to contribute tissue samples.

Genetics Analysis Partner **$100K** (∞)

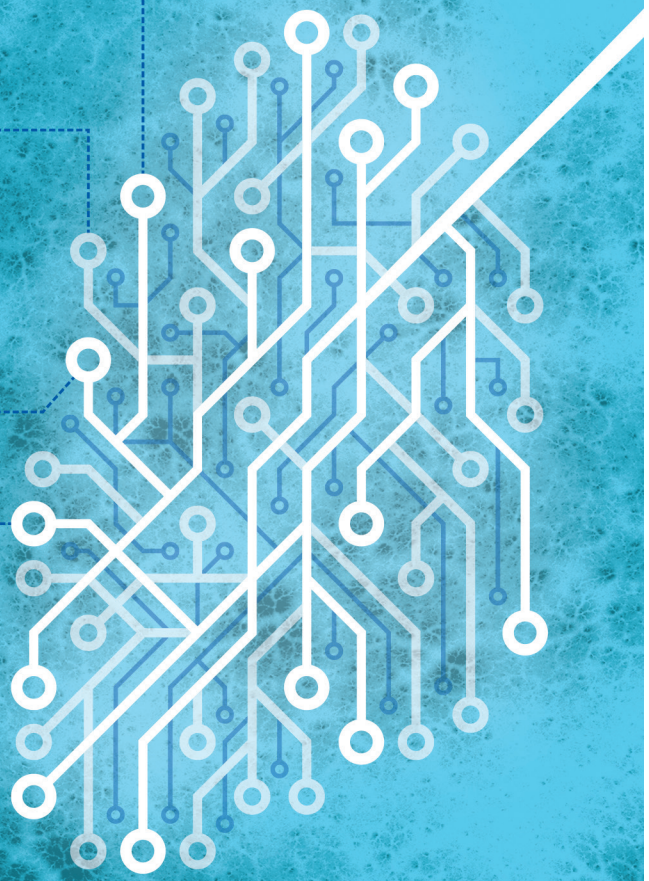
Analyzing the genetic factors that affect a patient's potential response to specific therapies will enable researchers to identify optimal treatment approaches for each patient.

Catalyst Grants **$50K** (∞)

Collaboration is critical to scientific discovery. Catalyst Grants will create an international hub of collaboration and provide incentive for researchers to work with scientists from other disciplines on innovative CF projects.

Travel Grants **$25K** (∞)

Travel Grants will allow our researchers to share expertise and collaborate with scientists from around the globe, multiplying the impact of our efforts.



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