CYSTIC FIBROSIS CANADA

Accelerating Clinical Trials
CF CanACT

ANNUAL REPORT 2021

clinicaltrials@cysticfibrosis.ca

Cystic Fibrosis Canada
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TABLE OF CONTENTS

KELLY GROVER
PRESIDENT AND CE0
CYSTIC FIBROSIS CANADA

MESSAGE FROM THE PRESIDENT AND CEO, CYSTIC FIBROSIS CANADA

Three years ago, Cystic Fibrosis Canada established the Cystic Fibrosis Canada Accelerating Clinical Trials Network (CF CanACT). World-class clinical trials are an integral part of the process that bring new therapeutics and better care to those living with cystic fibrosis. CF CanACT was established to facilitate the development of new treatments for cystic fibrosis as well as to increase the capacity of clinical research in Canada. The network has seen wonderful success over the three years, even with the impact of a global pandemic.

Starting with six sites, the network expanded to ten sites and now encompasses 60% of people living with cystic fibrosis in Canada. There has been a great increase in the number of patients enrolled in clinical trials. In 2018, prior to the network being formed there were 63 patients enrolled in clinical trials in Canada. By July 2021, this number had increased to 227. We know this success is due in part to the referral system put in place by CF CanACT which allows patients attending other CF clinics who are not part of the network to be referred to a CF CanACT site for a clinical trial.

The engagement of people diagnosed with cystic fibrosis and their families is especially important to Cystic Fibrosis Canada and we are pleased to see how patient engagement is a key element of CF CanACT. Adults with cystic fibrosis (CF) and parents of children with CF sit on the executive and protocol review committees and provide input and direction not only on which trials are important to them, but also on how feasible the trial would be for patients.

Partnerships are also an important part of the success of CF CanACT. The network works closely with the CF Foundation's Therapeutics Development Network (TDN) and the European Cystic Fibrosis Society's Clinical Trial Network (ECFS-CTN), and we thank them so much for the support they have provided to CF CanACT.

Cystic Fibrosis Canada is proud of the work of the CF CanACT team and thankful for the achievements made. We look forward to the next chapter for the network as we continue to go further for all those diagnosed with cystic fibrosis in Canada.

Sincerely,

Kelly Grover, President and CEO
Cystic Fibrosis Canada

DR. BRAD QUON
MEDICAL LEAD OF CF CanACT

Q&A WITH DR. BRAD QUON, MEDICAL LEAD OF CF CANACT

As Medical Lead of the research network, I work closely with clinician researchers and research coordinators from each of the 10 Cystic Fibrosis Canada Accelerating Clinical Trials (CF CanACT) sites across Canada, along with pediatric/adult patient representatives. Collectively, we influence the strategic direction of cystic fibrosis (CF) clinical trial activity in Canada and review clinical trial protocols to ensure they are feasible and aligned with the priorities of Canadians living with CF. I also meet regularly with the U.S. CF Foundation Therapeutic Development Network (CFF-TDN) and the European CF Society Clinical Trial Network (ECFS-CTN) to ensure that CF clinical trials are performed efficiently and in a coordinated manner globally. Lastly, I meet regularly with pharmaceutical companies to attract new clinical trials to Canada.

WHAT IS THE MAIN GOAL OR PURPOSE OF CF CANACT?

The goal of the CF Canada Accelerating Clinical Trials (CF CanACT) network is to support global efforts to advance the clinical development of new therapies for CF. Cystic Fibrosis is a rare disease and therefore we as a global CF research community can achieve much more and faster if we work together in a coordinated manner.

WHAT ARE THE BENEFITS YOU ARE SEEING WITH THE CF CANACT NETWORK? WHY IS THIS NETWORK IMPORTANT?

By having a coordinated network of very experienced clinical trial sites, we are able to attract more pharmaceutical companies to conduct their studies here in Canada. While we celebrate the tremendous success of CFTR modulators such as elexcaftor/tezacaftor/ivacaftor (Trikafta) for our patient population, we are still in pursuit of a cure for CF and there remain several unmet needs for our patient population that can be more readily addressed by having a network that executes these studies efficiently.

What is the significance of expanding to 10 sites (4 new sites) in 2020? How does it affect the CF community? What other achievements were there in 2020?

By expanding from 6 to 10 participating sites across Canada, we now represent over 60% of the Canadian CF population and have clinical trial sites from coast to coast. The geographic spread of sites across the country makes participating in clinical trials more accessible for our patients.

In addition to expanding our network in 2020, we supported 21 clinical trials with a mix of industry-sponsored pharmaceutical studies and protocols initiated by Canadian CF researchers. As an example of the latter, we supported the start-up activities of a very important research initiative referred to as CAR-CF. This study is aimed at evaluating antibody responses to COVID-19. While it was initially designed to examine COVID-19 infection rates in the Canadian CF population and its impact on health outcomes, this study is also poised to examine the durability of COVID-19 vaccine responses in the CF population which remains unknown.

What are you most proud of with what CF CanACT has been able to accomplish either this year or overall?

Despite the curtailment of clinical trial activity brought on by the COVID-19 pandemic, CF CanACT has demonstrated steady growth in the number of clinical trials conducted and the number of new participants enrolling in clinical trials each year. Over this past year, I am extremely proud that the research teams across the country were able to rally together and support each other during these challenging and unprecedented times through town hall meetings as we all quickly pivoted and adapted to new ways of working. Our clinical trial sites were able to strike the right balance in terms of adhering to clinical trial protocols to produce high quality data for our study sponsors to support new drug applications to the regulatory agencies and at the same time keep our research participants and study staff safe.

Where do you see CF CanACT heading in the next 3- 5 years?

Although we have already accomplished a lot as a network, the next few years will be important for the growth of CF CanACT. In addition to continuing to grow our participation in industry-sponsored clinical trials, CF CanACT has brought us together as a CF research community across the country and I hope we can leverage this opportunity and expertise to answer questions that are important and clinically relevant to Canadians living with CF. As one example, we are working together to study the long-term impact of Trikafta on quality of life, work productivity and physical activity of the Canadian CF population. I also hope that CF CanACT can play a bigger role over the next few years influencing clinical trial design and the regulatory approval process for new CF therapies in Canada.

How has Covid-19 affected CF CanACT network operations, if applicable? Do you predict these changes to continue?

Most new CF clinical trials during the pandemic were delayed in start-up as COVID-related studies took precedence. Patient recruitment was also sluggish as most of our clinical trial units are located in hospitals and therefore activities such as research were put on pause by our institutions to reduce non-essential visitors. Many of our patients were also hesitant to visit the hospital to attend clinic or participate in studies when case numbers in the hospital were high. However, I remain optimistic that operations and clinical trial activity will eventually return to pre-pandemic levels with increased uptake of the COVID-19 vaccine and the welcoming back of our patients to clinic for in-person visits.

Do you have any parting wishes, acknowledgements or information?

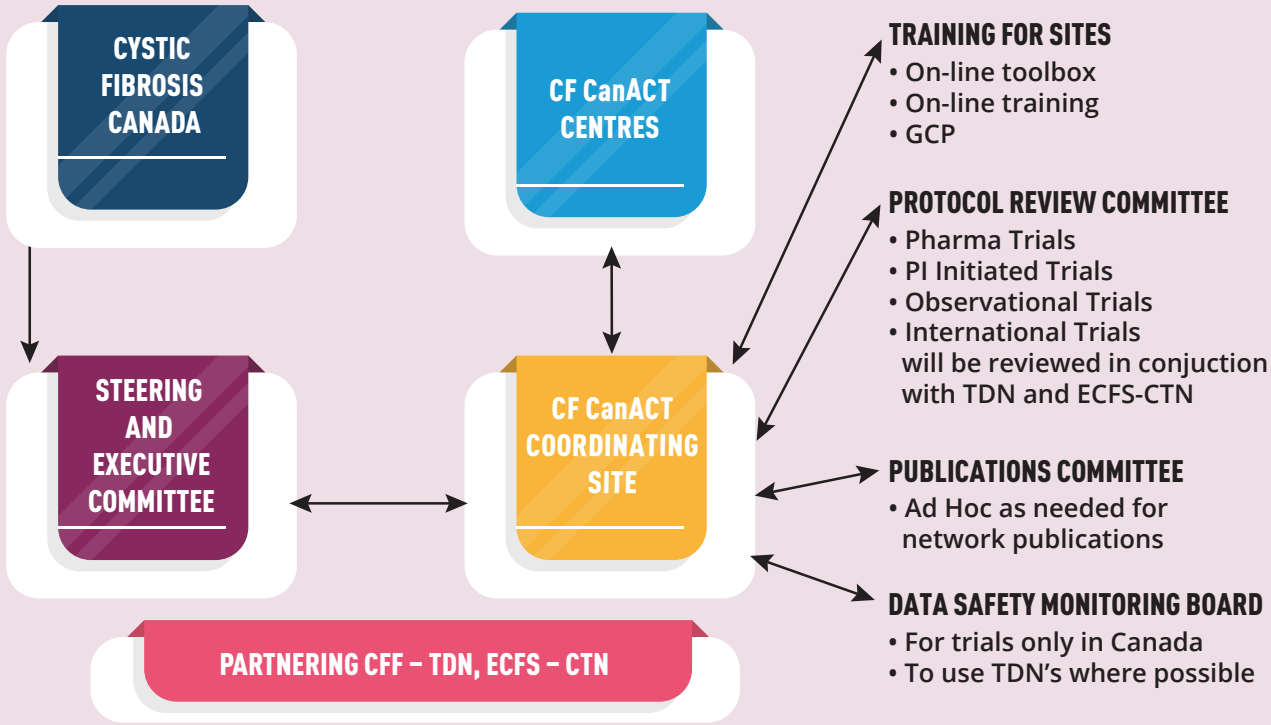
I would like to acknowledge Cystic Fibrosis Canada and the Cystic Fibrosis Foundation (CFF) for their continued financial and operational support of CF CanACT. Dr. John Wallenburg (Cystic Fibrosis Canada's Chief Scientific Officer) and Dr. Maggie McIlwaine (CF CanACT Research Manager) have provided tremendous leadership to CF CanACT and are the engines that keep driving us forward. As a relatively nascent network, we have received valuable mentorship from the executive team members of the CFF-TDN and ECFS-CTN and I would like to acknowledge them for their continued support. Members of our network including the clinical researchers, research coordinators, and patient representatives have dedicated their time and energy to make CF CanACT successful. Last but not least, I would like to thank our patient community for their strong involvement in CF research as their participation, enthusiasm, and altruism are contributing to a better and brighter future for individuals living with CF.

HISTORY OF CF CanACT

Cystic Fibrosis Canada works to achieve its vision of a world without cystic fibrosis (CF) by funding world-class research and clinical care. As world-class clinical trials are integral to bringing new therapeutics and better care to CF patients, in 2018, Cystic Fibrosis Canada launched the Cystic Fibrosis Canada Accelerating Clinical Trials (CF CanACT) network.

CF CanACT started with six experienced sites to establish a strong foundation to build upon. Those six sites collectively represented 37% of all Canadian CF patients. The network has established an Executive/ Steering committee to provide advice on overall cystic fibrosis clinical trials strategy and direction, and is charged with developing training programs, protocol review processes, and standard operating procedures. CF CanACT has established global protocol review agreements and processes in conjunction with the Cystic Fibrosis Foundation (CFF) Therapeutics Development Network (TDN) and the European CF Society (ECFS) Clinical Trial Network (CTN) to review industry-sponsored international trials.

CF CanACT NETWORK STRUCTURE



The network's start-up phase was supported through a generous grant from the CFF.

The network is tasked with increasing recruitment in clinical trials by supporting patients and families with CF through education, and community engagement. Cystic Fibrosis Canada is also levering the CF Patient Registry to allow identification of potential subjects by directly querying the registry. Another goal of CF CanACT is to ensure that patients are engaged in all aspects of clinical trials, including prioritization and selection of studies that are important to them, shaping the design of trials, and as participants. Increased engagement and improved communication amongst the clinics, and throughout the community, will lead to faster starts and faster enrollments, and result in recognition for Canada as a preferred site for CF clinical trials.

REFERRAL MAKES CLINICAL TRIAL POSSIBLE FOR VICTORIA FAMILY

Three-year-old Jordan loves to watch the float planes come in and take off at the Inner harbour in Victoria. His mom, Anna, brings him there along with his dad, Norm and younger brother Henry. By all accounts Jordan is a typical toddler; he loves being a big brother and playing in the dirt.

But in addition to his typical toddler activities, every few months the family finds themselves on a ferry or plane, heading to Vancouver for one of Jordan's clinical trial appointments.

Jordan was diagnosed with cystic fibrosis (CF) through the newborn screening program. Like many families affected by the disease, it came as a shock. Cystic fibrosis was not on their radar. It wasn't until Jordan's diagnosis that they discovered a distant relative who also had CF.

When Jordan was six months old, his pediatrician in Victoria mentioned that there would be a clinical trial in Vancouver for a modulator therapy. The trial was recruiting 12–24-month-olds with a Delta F508 mutation. Anna didn't think it would work out, since Jordan was too young at the time, "I learned it takes a while for a trial to get off the ground. By the time it started, Jordan was 18 months and was eligible."

As a result of the referral from his CF team in Victoria, Jordan ended up joining the six-month trial at BC Children's Hospital in October 2019 when he was around 20 months old.

Anna notes that the family had no hesitation when it came to deciding to participate in the clinical trial, "We felt confident, probably because of the care we received in Victoria since he was born. I trust our team. They were very encouraging of us participating in the trial. They were great at walking me through what the trial would look like."

Jordan is participating in a modulator trial, which means he has to take extra medication, and in addition to his regular CF care in Victoria, every few months the family must travel to Vancouver to meet with the clinical trial team at BC Children's Hospital.

"Although we don't attend the Vancouver clinic for Jordan's CF care, everything has been really smooth. The team in Vancouver has been awesome and we've felt really supported throughout the process," said Anna. "We were and still are well taken care of."

The family's travel to Vancouver is covered by the pharmaceutical company. Pre-COVID, they would stay a night in Vancouver and try to make the most out of their trip. Now, Jordan and his dad will go for the day.

At the six-month mark, Jordan's trial was lengthened for another two years. "With Canada being so slow to cover some of the CF drugs, the clinical trial is a way to get him on a treatment," said Anna. "In addition to knowing that Jordan is on a drug that is getting at the root cause of his disease, there are other fringe benefits like having a relationship with another doctor, offering another perspective. Our treatment hasn't changed but having another expert in my life feels good."

The study is blind, which means the family does not receive information about if the medication is positively impacting Jordan's health, but there was a two-week period between the original trial and the two-year lengthening when Jordan was taken off of the medication. During this time Jordan's stools became greasy again, which the family believes could be an indicator of what Jordan's normal would be if taken off the drug.

Cystic Fibrosis Canada's Clinical Trial Network is directly accessible to 60% of the CF population in Canada at their CF clinics, but is available to all Canadians, like Jordan, who meet the requirements of specific trials. Canadians with cystic fibrosis who are interested in participating in a trial are encouraged to speak with their healthcare team or check out CF Canada's clinical trial finder for a list of CF related clinical trials in Canada.

Please note that names have been changed.

NETWORK EXPANSION

The Cystic Fibrosis Canada Accelerating Clinical Trials (CF CanACT) network brings together world-class researchers and the Canadian cystic fibrosis community to facilitate the development of new medicines and treatments and to encourage the participation of people living with cystic fibrosis in clinical trials. Improving the quality of care for CF patients lies at the heart of the network.

To expand this integral work, in 2019 CF CanACT issued a call for applications from CF clinics across Canada. The goal was to build upon the network's established foundation by welcoming in new sites. Following careful consideration of all proposals, CF CanACT has officially expanded in 2020-2021 to include four new sites and two new provinces with one site in Halifax, Québec City, Saskatoon, and Montréal.

This brings the total number of sites across Canada to 10:

Halifax, Nova Scotia (Adults)

Québec City, Québec (Adults & Pediatrics)

Montréal, Québec (Adults & Pediatrics)

Montréal, Québec (Adults)

Toronto, Ontario (Pediatrics)

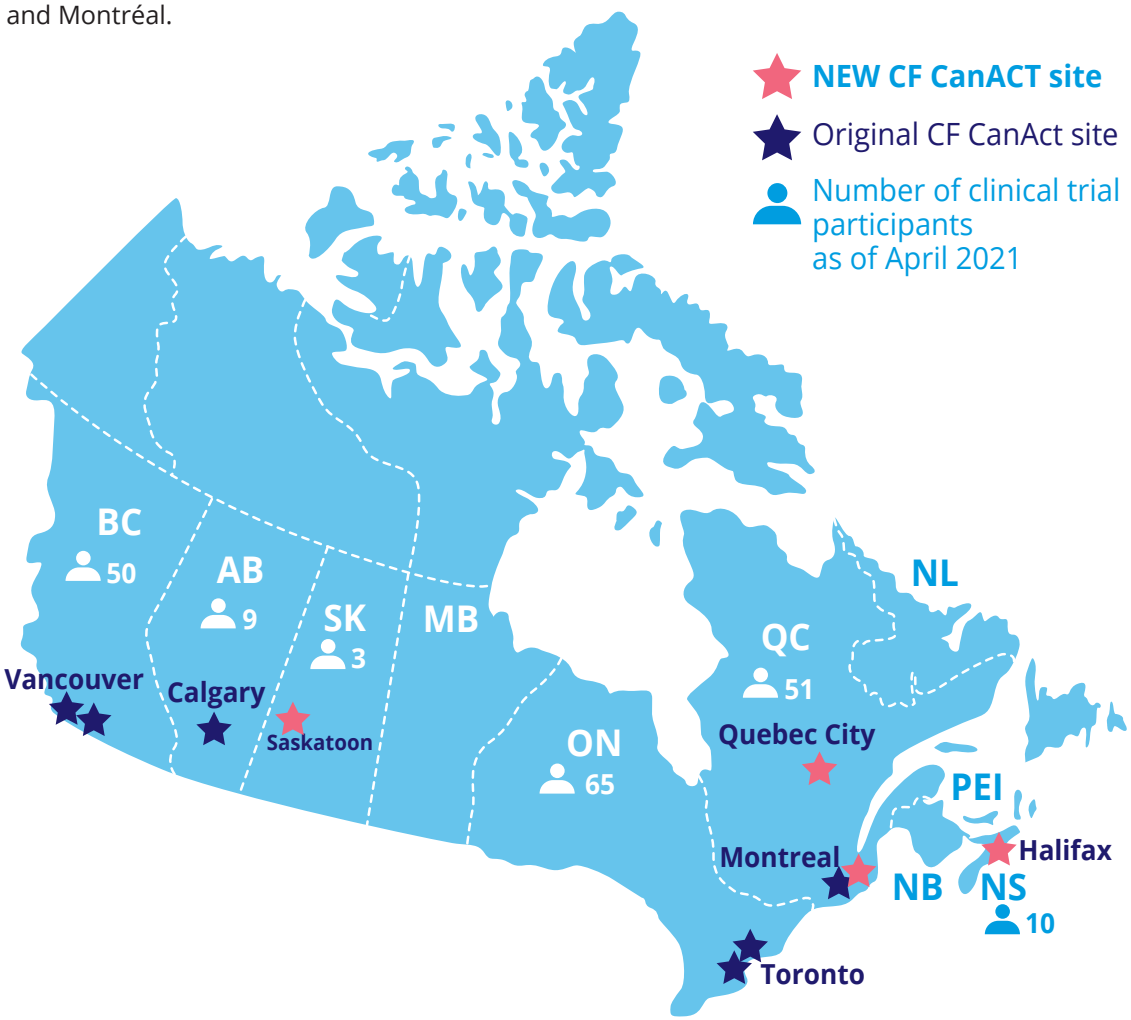
Toronto, Ontario (Adults)

Saskatoon, Saskatchewan (Adults & Pediatrics)

Calgary, Alberta (Adults)

Vancouver, British Colombia (Adults)

Vancouver, British Colombia (Pediatrics)



MEET A PRINCIPAL INVESTIGATOR

Lara Bilodeau is the Director of the Cystic Fibrosis clinic at the Québec Heart and Lung Institute (IUCPQ).

It was during her years as a pulmonology resident at IUCPQ that Lara discovered her interest in cystic fibrosis. She describes herself as being in the right place at the right time. Adults with CF were still attending the Pediatric Clinic in Quebec City and the need to develop an adult clinic was obvious. For Lara, it was "a great opportunity to express interest, receive training on cystic fibrosis and set up a brand-new clinic."

Why do you want to work with people with cystic fibrosis?

For several reasons, including the fact that we follow these young people for many years and therefore establish very special bonds with them. The partnership between the care provider and the patient is different from that of all our other clients. I also knew that there was a lot of research being done, and I wanted to be part of it. I saw the field as dynamic. I know we can't yet cure people living with CF, but we hope to get there one day. In the meantime, we must continue to invest in research, so that these people are less sick. That's why I think it's so important to participate in research and give our patients access to clinical studies.

What prompted you to join the CF CanACT network?

A few years ago, we started doing research in Quebec City, at the IUCPQ, gaining more experience with each study that we did. Being part of a Canadian research network facilitates participation in clinical studies, and so when the opportunity to join CF CanACT arose, we jointly applied with the pediatric centre in Québec City, and included peripheral centres such as the Rimouski, Chicoutimi and Sherbrooke clinics in order to reach a larger population of patients and offer them the opportunity to take part in clinical studies.

What are the advantages of being part of the CF CanACT network?

After our acceptance into the Canadian network, the funds allocated to us allowed us to hire a second nurse. We very much appreciate having more time for the coordination of studies, as it helps us to engage in more research projects. As an example, we are in the process of establishing a database including all patients in peripheral centers, which will facilitate the identification of those who could participate in research projects. We are particularly interested in collaborating with other Canadian centres and appreciate our mutual support with all other researchers across the country.

I also believe that Cystic Fibrosis Canada ensures the smooth running of all research projects by ensuring the quality of the participating centres. And if we're too busy, we know we can ask them for help. In addition, if a more distant patient needs help, the CF CanACT network can also provide support; it ensures the well-being of patients and ensures fair treatment for all.

Why is the research you do important for people with cystic fibrosis and how do they respond?

My research projects focus mainly on clinical trials of new drugs. Of course, we still need new tools to treat people with CF, so developing new drugs, in a safe environment, is important for these people. I am happy to be part of these projects, sometimes it's a way to give patients access to new treatments, for example the new modulators that will one day be on the market. Our patients are very open to participation in clinical trials. We are sometimes pleasantly surprised by the enthusiasm of our patients to participate in studies. We should not hesitate to offer these research projects to all eligible individuals.

What do you like about your job?

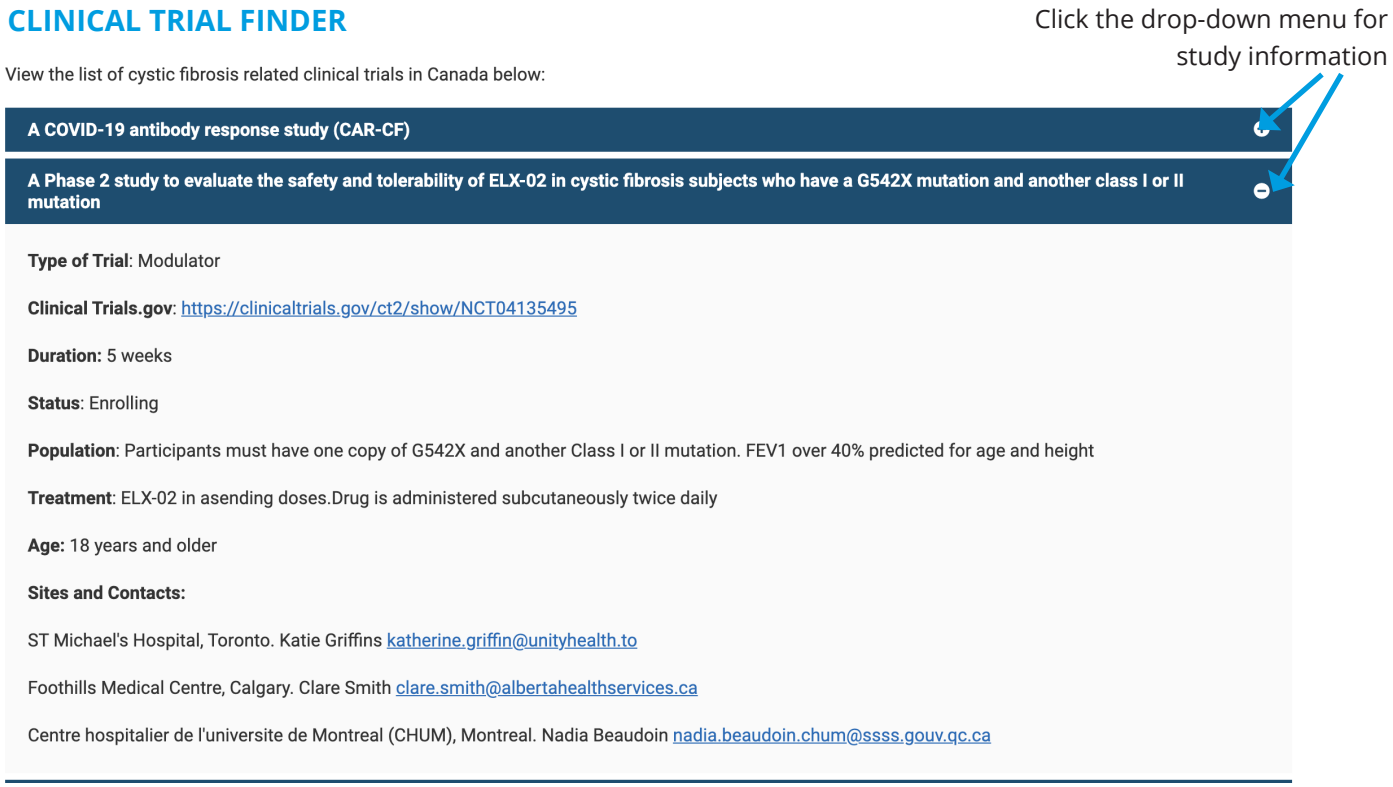
I love working with people with cystic fibrosis and my team. I am also happy and proud of the studies we have added over the past few years. In my opinion, we are at a particularly exciting time when it comes to cystic fibrosis and all our patients have excellent reasons to keep hope for the future. I look forward to continuing my work for many years to come.

CF CanACT SANCTIONED STUDIES AND CLINICAL TRIAL FINDER

To be deemed a CF CanACT-sanctioned study, study protocols must be jointly reviewed by the CF CanACT executive committee and the CF CanACT Protocol Review Committee (PRC). Reviews are conducted alongside an adult person with cystic fibrosis (CF) or a CF parent for pediatric studies. Critical protocol evaluation ensures feasibility, appropriate study design, and that study goals address the priorities of CF CanACT and the CF community, as these clinical trials have the power to impact the future of cystic fibrosis care.

Clinical Trial Finder

Interested in participating in a clinical trial? The Clinical Trial Finder featured on Cystic Fibrosis Canada's website lists Canadian CF-related clinical trials being conducted. You can find information about the type of study, trial duration, study eligible population, treatment, site locations and contact information by clicking the drop-down menu.



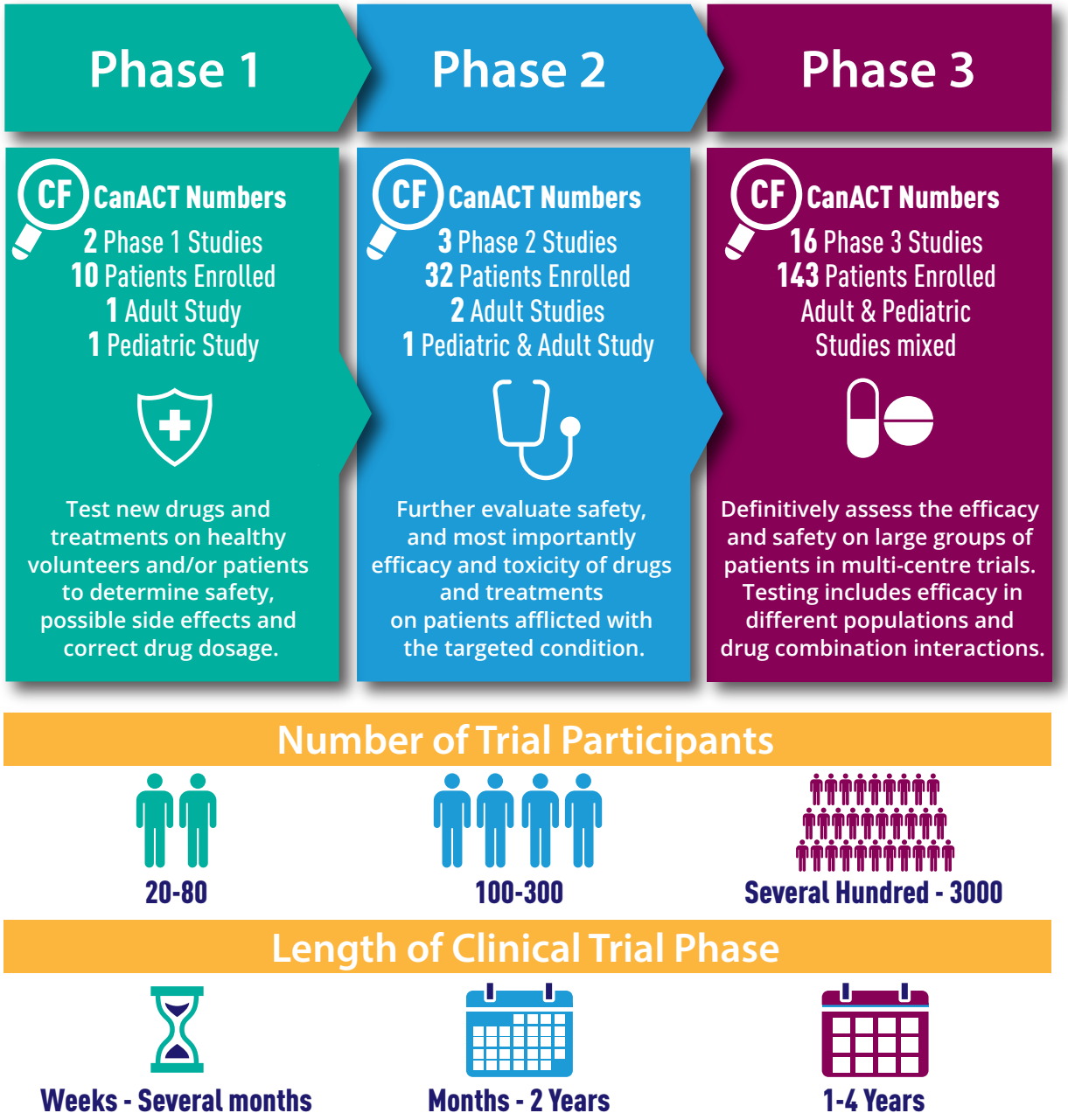
Individuals from any Canadian cystic fibrosis clinic may participate in a clinical trial provided they meet all selection criteria for that specific trial. Please note that if you are referred to a different site to participate in a clinical trial, regularly scheduled CF care will continue at your local CF clinic.

To be referred to a clinical trial:

- Visit www.cysticfibrosis.ca/clinicaltrials to view the Clinical Trial Finder for studies you are eligible for and interested in. The www.clinicaltrials.gov links provide further details about a clinical trial
- Speak with your CF clinic physician or nurse about clinical trials and how you may be referred to a clinical trial
- Contact the Research Coordinator of a clinical trial you are interested in or a study site you are located nearest to for additional information as listed in the Clinical Trial Finder

CF CanACT PARTICIPATION IN THE VARIOUS PHASES OF CLINICAL TRIALS

Clinical trials allow potential new medicines and treatments which have had positive results in laboratory and animal studies to be safely tested in healthy volunteers and patients. Before they can be distributed to the larger community, they must pass through three consecutive phases that further test the safety and efficacy of the drug/treatment.



If trial results after Phase 3 are deemed to be positive, the experimental drug/treatment will be approved for use. At that point, Phase 4 of a clinical trial begins. Safety and effectiveness continue to be studied in large and diverse populations. This process ensures that high quality and thoroughly tested drugs and treatments reach the people who need it the most.

A JOURNEY TO LEARNING ABOUT THE DISEASE THROUGH CLINICAL TRIALS

Two clinical trials, two different treatments, one journey to learn more about cystic fibrosis.

Back in 2015, Jeff was notified by his doctor at St. Mary's Hospital about the off-label drug Riociguat used to treat pulmonary hypertension. Preclinical studies suggested it might help those with a delta F508 mutation by improving chloride channel function and a clinical trial was underway to determine its effectiveness. That is when Jeff received the call. "I think my CF doctors were probably approached by Katie Griffin, who's the Research Coordinator at St. Michael's Hospital. She called me and I said, *Yeah, why not?* At that point I wasn't on any of the cystic fibrosis (CF) modulators, one of the requirements for the trial. It actually worked out well for me."

And so Jeff began the journey of learning more about his disease. "When I did the Riociguat study," Jeff recalls, "what I discovered was it was exciting to take part in it, as I got closer to the information stream of what other drugs were in the pipeline in addition to that particular drug. And at least Katie, as much as she could, provided me with a bit of a preview of what was coming. It's pretty exciting and motivating to stay healthy when you know that there are more things coming down the pipeline that might help you as well. I could only glean so much of that from my local clinic since they're not as close to the research as someone at St. Mike's is. I really value St. Mike's reaching out to other clinics who are not part of CF CanACT."

Part of CF CanACT's work is to inform clinical trial participants and ensure the process is comfortable and understandable. Jeff received scientific literature relating to the drug and what was being studied, detailed time commitment sheets and a study timeline. Clinical trial visits to St. Michael's Hospital in Toronto were incorporated into Jeff's work week. And the clinical trial staff were readily available to bounce questions off, even following the completion of a trial. "We were well prepared so there were no surprises. I think that's probably why I didn't have any misconceptions about the clinical trial."

JEFF ALLEN WITH HIS WIFE, LISA.

Then in 2017, the referral call rang again. This time, Jeff was introduced to a pharmaceutical inhaled drug that targets sodium channels in the airways. With the same excitement as in 2015, Jeff agreed to participate in the 4-week clinical trial with additional follow-up appointments. His main reason for joining a CF CanACT trial a second time?

"TO PARTICIPATE IN THE PROGRESSION OF THE RESEARCH AND DEVELOPMENT CYCLE. WE CAN'T GET PROGRESSION IN THESE CF TREATMENTS WITHOUT PEOPLE PARTICIPATING."

Now in 2021, Jeff is interested in current and upcoming CF CanACT clinical trials, he noted, "It sounds exciting. The more ammunition [against CF] that's coming out is encouraging."

CF CanACT is currently running observational trials for Covid-19 antibody presence in CF and following people on Trikafta. In addition, the network is running mRNA trials for those who do not produce any CFTR protein, and new anti-bacterial medicines based on phage therapy are also in the works.

For those considering participating in clinical trials, Jeff has a message- "Looking at the risks, and looking at the potential benefits, just being part of the process is worth it. I would recommend it to others with CF. If they asked me, I would say go for it."

2020–2021 CF CanACT Year in Review

CF CanACT network expanded to include **4 new sites**

10 sites represent **60%** of Canadian CF population

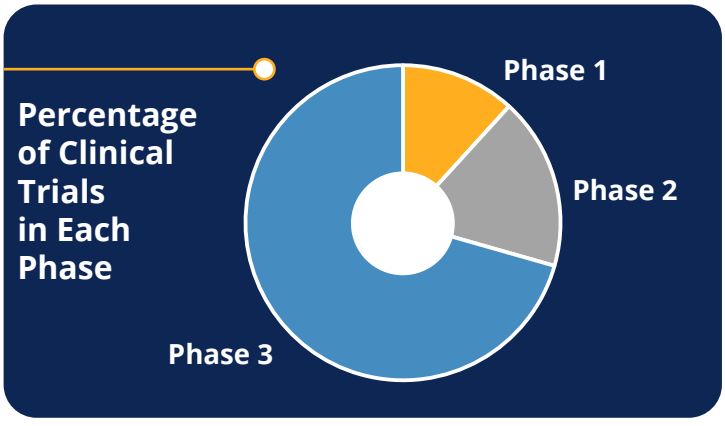
185 people with CF enrolled in clinical trials

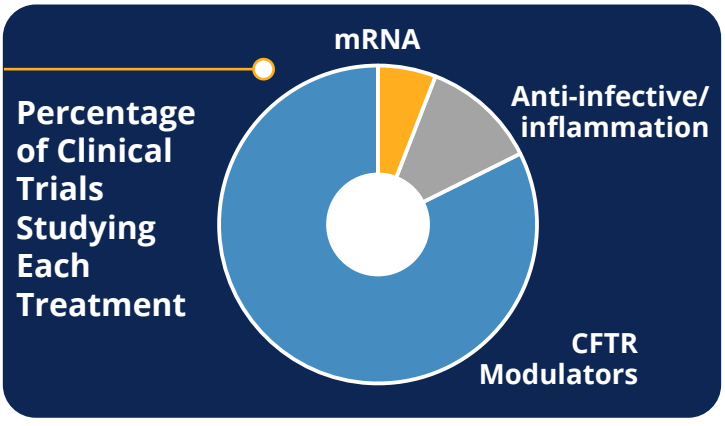
Supported **21** active clinical trials

3 protocol reviews

1 feasibility study

2 abstracts accepted to the North American CF conference





KATIE GRIFFIN
RESEARCH COORDINATOR

BEHIND THE SCENES WITH A RESEARCH COORDINATOR

Today, Katie Griffin, Research Coordinator at the St. Michael's Hospital CF CanACT site sits in meetings with her cystic fibrosis (CF) care team and respirology research group discussing the clinical trials at hand. Tomorrow, she might be negotiating the language for a 20-odd page informed consent form or reviewing in detail that same consent form with trial participants. The next day might involve coordinating with other hospital departments for the testing needed for a specific trial, like an ophthalmologist to perform eye exams for clinical safety monitoring. All the while navigating budgets, contracts, and policies.

Katie describes it with a chuckle as, "Definitely there is no typical coordinator's day. It depends on what's going on at the time. There are lots of moving pieces."

The first moving piece is determining which clinical trials to undertake. The trial only starts, "if the patients are interested in doing the trial. We get to know what patients have said about wanting to participate in certain trials over the years." The benefit of this system is to drive needed innovation in CF and "hope for the future," Katie remarks.

"YOU GO INTO IT KNOWING THAT NOT EVERY DRUG IS GOING TO BE A SUCCESSFUL CANDIDATE FOR FUTURE DEVELOPMENT. BUT YOU SEE THE PATIENTS FEEL LIKE THEY'RE BEING PART OF SOMETHING BIGGER AND HELPING A LOT. IT'S NOT FOR THEM INDIVIDUALLY, THEY REALLY DO IT FOR THE COLLECTIVE CF COMMUNITY. I THINK, TO SEE NEW TREATMENTS AND NEW PROCESSES COME UP AS A RESULT OF THE WORK THAT WE HAVE DONE IS QUITE HUMBLING AND INSPIRING."

"As a CF team, we're aware of some of the barriers that people experience in staying healthy. Drugs are definitely one component of improving that, while clinical trials are another component of helping people achieve optimal health."

Gaining equitable access to these newly developed drugs after a clinical trial has driven Katie to pursue her Master of Public Health. Exchanging meetings for classrooms, her non-typical day continues by learning health promotion and protection strategies for a population like CF. "I am taking the needs that I see in the clinic and trying to apply them in my Masters, and hopefully bring some of that information and knowledge back to the clinic."

In the clinic, that knowledge helps in identifying additional ways of improving health. Aside from clinical trials there are quality of life and burden of disease studies. New ways to improve testing or monitoring of certain characteristics are topics discussed with the care team. "All of that brings about changes in our practice, and I think new knowledge allows for, hopefully, less burdensome clinic visits or therapies. Being a part of these initiatives that change the way that care is delivered is also very important and a big part of our clinic, and what we do as research coordinators when we're not focusing on the clinical trials."

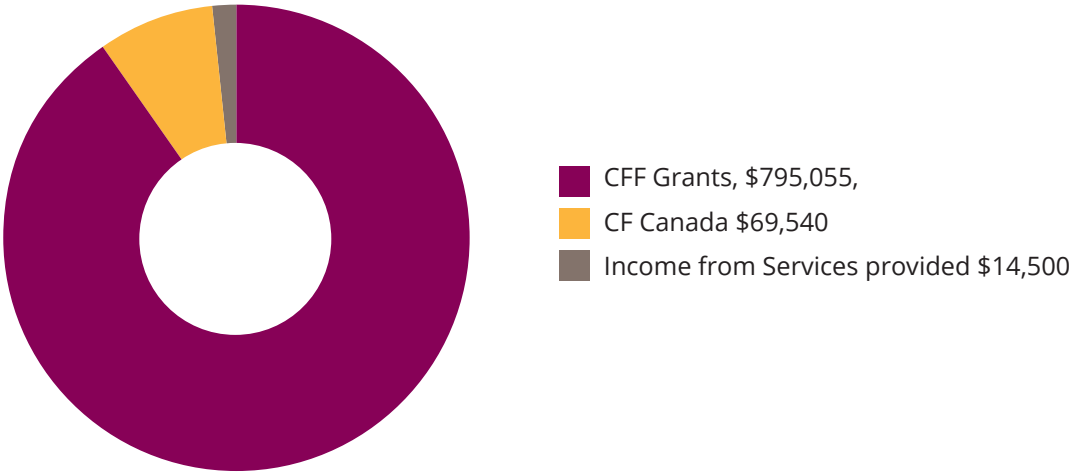
The supportive team at St. Michael's Hospital enables clinical trials to bring about new drugs and treatments. But behind the scenes, the team tackles all areas of care to with the goal of achieving a world without cystic fibrosis.

FUNDING

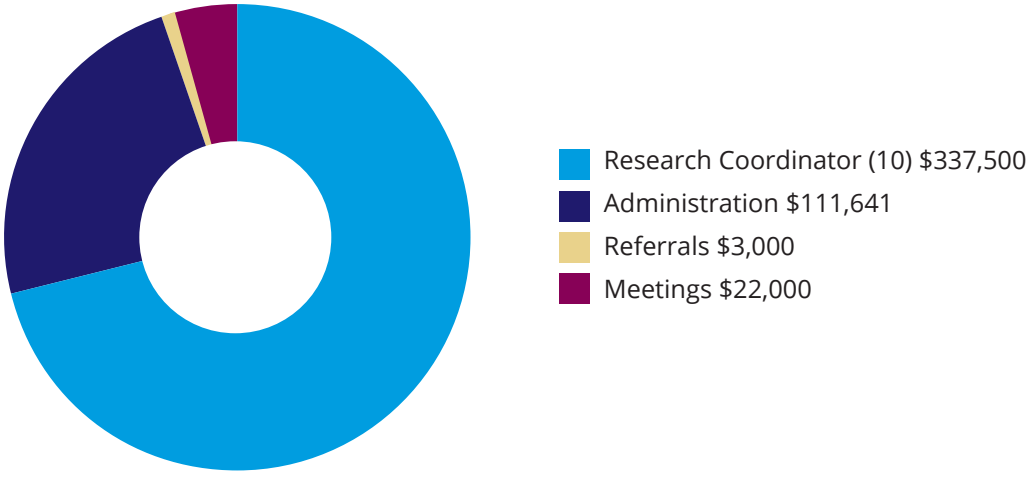
Cystic Fibrosis Canada Accelerating Clinical Trials (CF CanACT) network is partially funded by grants from the Cystic Fibrosis Foundation in the US and Cystic Fibrosis Canada. Additional income is generated through scientific services including performing protocol reviews or feasibility studies across CF CanACT sites.

It is important for the network to be financially independent of pharmaceutical companies so that there is no conflict of interest when providing scientific advice on clinical trials.

Income 2020-2021*



Expenditures 2020-2021*

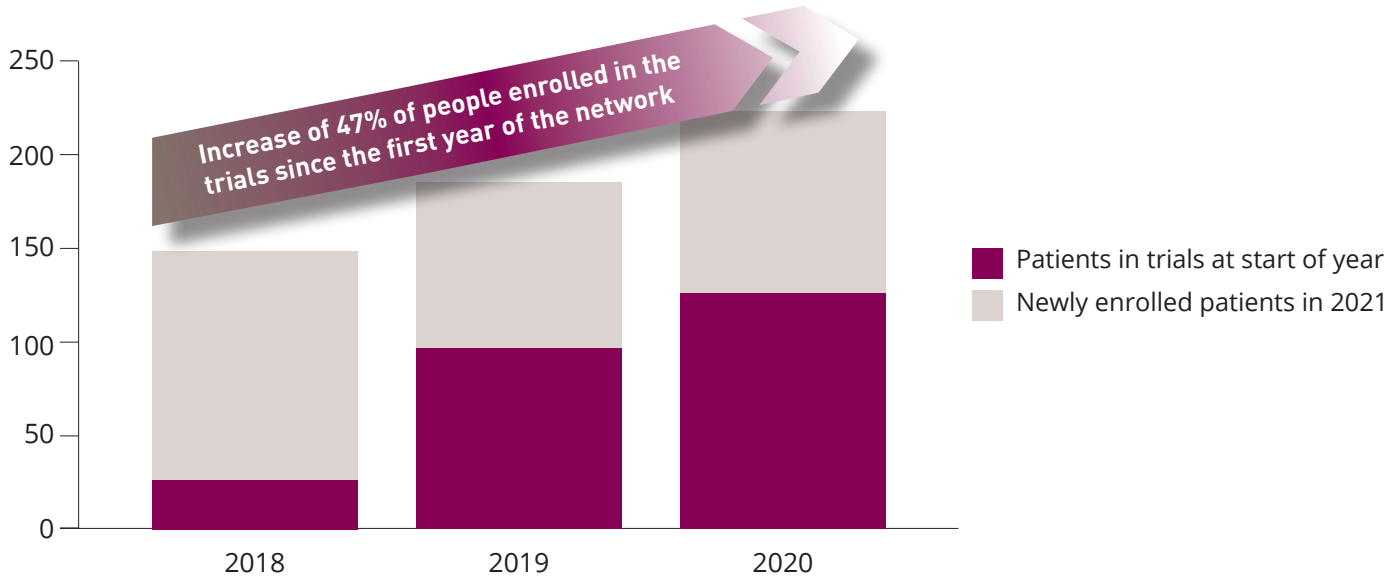


*In 2020 CF Canada's funding was decreased due to lower fund-raising revenue during COVID-19.

*There was no travel during 2020-2021 due to COVID, but increased virtual conferencing fees.

CF CanACT METRICS SINCE INCEPTION IN 2018

Total number of people with CF enrolled in a clinical trial (In 2021, 11.3% of people with CF attending a CF CanACT site are participating in a clinical trial)



During 2020, four additional sites joined CF CanACT. These new sites contributed 47 clinical trial participants. One of these sites in still in the start-up phase and has not yet enrolled any person with CF into a Pharma trial.

One site was closed to enrolling new people with CF to a clinical trial due to COVID.

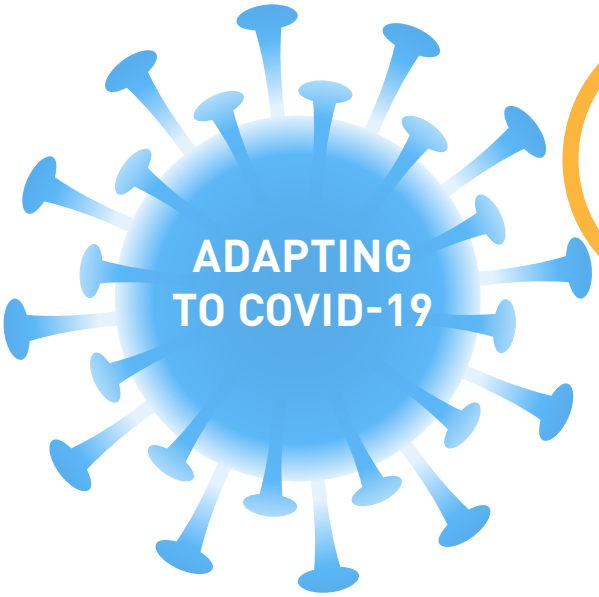
In 2020-2021 there were 26 active clinical trials within CF CanACT

21 Pharma, **5** Investigator trials	**3** Anti-infectives	**1** mRNA
20 Modulator trials	**1** Anti-inflammatory	**1** COVID antibodies

THE IMPACT OF COVID-19

The Covid-19 pandemic posed new challenges for CF CanACT. Restricted clinic capacity hindered the initiation of new studies. Regional lockdowns made it difficult to recruit new participants and limited in-person consultations impacted clinical trial enrolment. To continue the quality research performed in the network, CF CanACT practices had to change in new ways according to the prevalence and spread of the Covid-19 virus in different Canadian regions. Public health was placed at the forefront to safeguard the health and well-being of individual patients and the CF community.

Read below to learn just some of the creative ways CF CanACT adapted to the everchanging Covid-19 pandemic.



Home sweet home

With clinics closed for essential visits only, various components of clinical trials were completed at home. Drugs were delivered directly to trial the participants' home and training videos were made available for at-home spirometry tests. Sputum collections could also be done at home prior to a clinic visit.

The virtual care boom

Both regular CF care visits and clinical trials were shifted to online platforms. This enabled individuals to still participate in trials despite regional lockdowns and without having to travel to clinic sites.

Changing up old ways

Traditional methods were revamped to address infection control policies. Pulmonary function tests were performed in patients' rooms with personal protective equipment. Nurses could perform home visits to collect lab work which was then sent to local labs for testing whenever possible.

CF CanACT TRIALS

MODULATOR TRIALS

Phase 2 study of ABBV-3067 alone and in combination with ABBV-2222 in people with CF aged 18 and older with 2 F508del mutations. (Abbvie M19-530).

Phase 2 study to evaluate the safety and tolerability of ELX-02 in cystic fibrosis subjects who have a G542X mutation and another class I or II mutation (Eloxx EL-012).

Phase 1 study to evaluate the safety and drug tolerability of PTI-801 in healthy volunteers and in adults with CF (Proteostasis PTI-801-01).

Phase 3 open-label extension study of Trikafta in people 12 years and older who have one copy of the F508del mutation and one copy of a minimal function mutation (VX17-445-105, parent study VX17-445-102).

Phase 3 open-label extension of long-term ivacaftor in children with CF aged under 2 years with a gating mutation (Vertex VX15-770-126; parent study VX15-770-124).

Phase 3 open-label extension study of VX-659 in triple combination with ivacaftor and tezacaftor in people 12 years and older with 1 or 2 F508del mutations (Vertex VX 445-113; parent study Vx17-659-105) .

Phase 3 open-label extension study of long-term treatment of Symdeco in people aged 12 years and older with 1 or 2 F508del mutations (Vertex VX14-661-110).

Phase 3 open-label extension study of long-term treatment with Symdeco in children aged 6 years and older with 1 or 2 F508del mutations (Vertex VX16-661-116; parent study: VX16-661-113).

Phase 3 open-label extension study of Orkambi in subjects 1 to less than 2 years of age homozygous for F508del (VX 809-124; parent study VX 809-122).

Phase 3 study of VX-659 in combination with ivacaftor and tezacaftor in children with CF ages 6-11 who have at least one copy of the F508del mutation (VX18-445-106).

Phase 3 open-label extension study evaluating the long-term safety and efficacy of VX-659 in combination with ivacaftor and tezacaftor in children with CF ages 6-11 who have at least one copy of the F508del mutation (VX-445-107; parent study VX18-445-106).

Phase 3 open-label extension study evaluating the long-term safety and efficacy of Trikafta in people with CF with 1 F508del mutation and 1 gating or residual function mutation (VX18-445-110; parent study VX18-445-104).

Phase 3 study to evaluate the safety and efficacy of Trikafta in children with CF aged 6-11 year with 1 F508del mutation and 1 minimal function mutation. (Vertex VX19-445-116).

Phase 3 open-label extension study to evaluate the long-term safety and efficacy of Trikafta in children with CF aged 6-11 year with 1 F508del mutation and 1 minimal function mutation. (Vertex VX19-445-119; parent study VX19-445-116).

Phase 3 safety and efficacy testing of VX-121 combination triple therapy in people aged 12 years and older with CF. (Vertex VX21-121-103).

ANTI-INFECTIVE/ANTI-INFLAMMATORY TRIALS

Phase 1b study to evaluate CB-280 in adults with cystic fibrosis and chronic Pseudomonas aeruginosa (Calithera CX-280-202).

Phase 2 study of Lenabasum (AU-7b), an anti-inflammatory drug in people with cystic fibrosis over the age of 18 years (LAU-14-01).

MUCOCILLIARY CLEARANCE TRIALS

Phase 2 safety and efficacy testing of inhaled BI 1265162 when added to standard care in people with CF aged 12 years and older. (BI 1399-0003)

INVESTIGATOR TRIALS

Phase 3 study to evaluate the effectiveness of prednisone as an adjunctive treatment for patients who do not respond to regular intravenous antibiotics in cystic fibrosis pulmonary exacerbations (PIPE).

Phase 3 antibiotic treatment of Staphylococcus aureus in stable people with CF (ASAP-CF).

Phase 4 study examining the use of Hyperpolarized imaging in children with CF between 6 and 18 years of age before and after initiating Trikafta (HyPOINT).

Observational study to examine antibody response to COVID-19 before and after receiving the COVID-19 vaccine (CAR-CF).

PRINCIPAL INVESTIGATORS AND LEAD RESEARCH COORDINATORS

Vancouver Adults, ST Paul's Hospital

Principal Investigator: Dr. Brad Quon

Research Coordinator: Taryn Leach

Vancouver Paediatrics, BC Children's Hospital

Principal Investigator: Dr. Mark Chilvers

Research Coordinator: Alam Lakhani

Calgary Adults, University of Calgary, Foothill's Hospital site

Principal Investigator: Dr. Mike Parkins

Research Coordinator: Clare Smith

Saskatoon Adults and Pediatrics, Royal University Hospital

Principal Investigator: Dr. Julian Tam and Dr. Martha McKinney

Research Coordinator: Dawn Johnson

Toronto Adults, St Michael's Hospital

Principal Investigator: Dr. Elizabeth Tullis

Research Coordinator: Katherine Griffin

Toronto Paediatrics, SickKids Hospital

Principal Investigator: Dr. Felix Ratjen

Research Coordinator: Stephanie Jeanneret-Manning

Montreal McGill site Adults and Pediatrics, Montreal Children's Hospital

Principal Investigator: Dr. Larry Lands

Research Coordinator: Tracey Mercier and Paul Edmonds-Brunache

Montreal Adults, Centre Hospitalier Universitaire de Montreal (CHUM)

Principal Investigator: Dr. François Tremblay

Research Coordinator: Nadia Beaudoin

Quebec City Adults, Institut universitaire de cardiologie et de pneumologie de Québec Paediatrics, Centre Mere-Enfant du CHU de Québec

Principal Investigators: Dr. Lara Bilodeau and Dr. Patrick Daigneault

Research Coordinator: Andrée-Anne Therrien

Halifax -Adults Queen Elizabeth II Hospital

Principal Investigator: Dr. Nancy Morrison

Research Coordinator: Andrea Dale

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