# CF FOUNDATION PRIORITY CLINICAL STUDIES

Key multi-site clinical studies funded through grants from the Cystic Fibrosis Foundation.

## STATUS KEY

PLANNING | ENROLLING | DATA ANALYSIS | COMPLETED/RESULTS

## GENETIC THERAPIES

- REACH — AGES 12+

## CFTR MODULATORS

- PROTECT* — AGES 18+
- RETRIAL* — AGES 6+
- BEGIN* — AGES <6
- MAYFLOWERS* — AGES 16+
- PROMISE* — AGES 6+

## MUCUS CLEARANCE

- REACT — AGES 3-16
- SIMPLIFY — AGES 12+

## INFLAMMATION

- BEGIN* — AGES <6
- PROMISE* — AGES 6+

## INFECTION

- SEND-CF — AGES 16+
- SPARC — AGES TBD
- ABATE — AGES 18+
- POSTSTAMPS — AGES 6+
- PREDICT /PATIENCE — AGES 6+
- STOP360 — AGES 6+
- STOP PEDS 2.0 — AGES 3-18
- BEGIN* — AGES <6
- PROMISE* — AGES 6+

## DIGESTIVE SYSTEM

- RETRIAL* — AGES 6+
- STRONG-CF — AGES 18+
- BEGIN* — AGES <6
- PROMISE* — AGES 6+
- GALAXY — AGES 2+
- NICE-CF — AGES 18+

## CFRD

- BIONIC CF — AGES 14+
- SPECTRUM — AGES 12+
- BEGIN* — AGES <6
- PROMISE* — AGES 6+

## REPRODUCTIVE HEALTH & PARENTING

- PROTECT* — AGES 18+
- HOPE CF* — AGES 18+
- MAYFLOWERS* — AGES 16+

## MENTAL HEALTH

- HOPE CF* — AGES 18+
- RETRIAL* — AGES 6+
- TIDES 2.0 — AGES 1.5-11

## ADVANCED LUNG DISEASE & TRANSPLANT

- ABC STUDY — AGES 18+

To learn more about these and other CFF-supported studies, visit **cff.org/finder**.

**Study is included in more than one category.*
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CYSTIC FIBROSIS FOUNDATION®

# CF FOUNDATION PRIORITY CLINICAL STUDIES

The CF Foundation supports a wide range of research to address the complex needs of people with CF, from collaborating with industry partners to develop new therapies to funding academic researchers at universities and hospitals who are focused on critical questions in CF treatment and care.

This document highlights a selection of key, multi-site clinical studies funded through CF Foundation grants in areas of high priority to the CF community. Most of these academic studies are observational or focused on improving aspects of care — like optimizing how we treat pulmonary exacerbations or reducing treatment burden — meaning they are not testing a new drug. For a list of studies that involve testing new drugs, readers can refer to the Drug Development Pipeline, a separate resource which tracks CF therapies in development. Together, these resources reflect different but complementary parts of the Foundation's research efforts.

*This list of priority clinical studies is **not comprehensive** and does not include every study the Foundation funds.*

**Studies that appear in multiple categories are denoted with an asterisk.*

## GENETIC THERAPIES

**REACH: This study is enrolling adults and children with CF ages 12 years and older.** REACH is a one-of-a-kind research study for people with CF who are not on modulators, regardless of the reason. The goal of REACH is to collect health information and specimens (like blood) from people who do not take modulators. The data collected in REACH will be used in multiple ways to advance science and move towards new and better treatments for people with CF. REACH is not testing a new drug, but the data we collect in the REACH study will be used to help evaluate the next generation of CF therapies and treatments. REACH research participants will have four study visits over a year at a research site. Also, participants will complete at-home surveys and home spirometry monthly.

## CFTR MODULATORS

**PROTECT*: This study is in the planning stage and is not yet open to enrollment.** The PROTECT study will evaluate the impact of modulator therapy in utero. The study is for pregnant mothers who do not have CF and are carrying a fetus with CFTR mutations that are eligible for Trikafta. Trikafta must be separately prescribed by a clinician (it will not be prescribed through the study). To learn more, contact the Clinical Trial Navigator.

**RETRIAL*: This study is enrolling adults and children with CF ages 6 years and older.** RETRIAL is an observational study for people with CF who had to stop taking or change how they took Trikafta because of new or worsening mental health symptoms (such as depression, anxiety, mood, sleep, and brain fog/memory problems) and/or liver symptoms (elevated liver enzymes), and who plan to start taking Alyftrek. To participate, people with CF need to enroll before they start taking Alyftrek, and they must receive the prescription from their CF doctor (Alyftrek will not be prescribed through the study). Researchers will collect information regarding changes in health, including mental health and liver health, both before participants start taking their clinically prescribed Alyftrek and for 12 months after. This study involves two visits over 15 months and will require questionnaires, which can be completed remotely. In-person blood draws and other sample collections, as well as a neurocognitive sub-study, are optional.

**BEGIN*: This study is closed to enrollment and undergoing data analysis.** BEGIN is an observational study to look at the impact of CFTR modulators in young children and infants with CF. Researchers collected information on growth, GI symptoms, liver health, and lung function before and after starting modulator therapy. Additional sub-studies evaluated infection, inflammation, blood glucose, and sinus disease.

# CF FOUNDATION PRIORITY CLINICAL STUDIES

**MAYFLOWERS*: This study is closed to enrollment and undergoing data analysis.** MAYFLOWERS is an observational study to evaluate the effects of CFTR modulators on women with CF during and after pregnancy. Women with CF were enrolled early in pregnancy and were assessed every three months during pregnancy into the first year after delivery, and then every six months for an additional year. Researchers evaluated changes in lung function to better understand how CFTR modulators affect maternal health during and after pregnancy. The study also tracked the health of the newborns.

**PROMISE*: This study is closed to enrollment and undergoing data analysis.** PROMISE is a real-world observational study designed to understand the effects of Trikafta in adults and children with CF ages 6 years and older. This study evaluated participants before and after they began taking Trikafta and collected information on lung function, sweat chloride, respiratory microbiology, inflammation, gastrointestinal symptoms and function, liver function, body composition/growth, and CF-related diabetes. Blood and urine samples were collected throughout the study to enable future research.

## MUCUS CLEARANCE

**REACT: This study is in the planning stage and is not yet open to enrollment.** The REACT study will evaluate the use of hypertonic saline or dornase alfa (Pulmozyme) in kids with CF ages 3-16 years. To learn more, contact the Clinical Trial Navigator.

**SIMPLIFY: This study is completed with results.** The SIMPLIFY study evaluated the effects and safety of stopping inhaled hypertonic saline or dornase alfa (Pulmozyme) in teens and adults with CF who were also taking Trikafta. Researchers tracked lung function and changes in respiratory symptoms and monitored for adverse events. In adolescents and adults with relatively good lung function on Trikafta, discontinuing daily use of either hypertonic saline or dornase alfa for six weeks did not result in a loss of lung function. Several other studies assessing longer-term effects on health are underway to provide additional insight into the implications of stopping medications and therapies while on Trikafta, including HERO-2 and CF-STORM.

## INFLAMMATION

**BEGIN*:** This study looks at how CFTR modulators affect different aspects of health in young children and infants with CF, including inflammation. See the "CFTR Modulators" section for more details.

**PROMISE*:** This study looks at how Trikafta affects different aspects of health in people with CF ages 6 years and older, including inflammation. See the "CFTR Modulators" section for more details.

## INFECTION

**SEND-CF: This study is in the planning stage and is not yet open to enrollment.** SEND-CF is an observational study to identify new ways to detect harmful bacteria in the lungs of people with CF. Participants will be asked to provide samples including sputum, saliva, blood, and urine at a single study visit. Participants must be able to produce sputum to participate.

**SPARC: This study is in the planning stage and is not yet open to enrollment.** SPARC, or Special Pathogens Analysis and Research Consortium, will include multiple studies focused on difficult-to-treat infections like *Achromobacter*, *Burkholderia cepacia*, and nontuberculous mycobacteria (NTM). To learn more, contact the Clinical Trial Navigator.

# CF FOUNDATION PRIORITY CLINICAL STUDIES

**ABATE: This study is enrolling adults with CF ages 18 years and older.** ABATE is an open-label interventional study to evaluate the safety and effectiveness of intravenous (IV) gallium in adults with CF who have nontuberculous mycobacteria (NTM). Researchers will measure whether IV gallium reduces NTM levels in sputum cultures. This study involves eight visits over 20 weeks and may require sputum samples and other measurements.

**POSTSTAMPS: This study is enrolling adults and children with CF ages 6 years and older.** The POSTSTAMPS study will evaluate the compassionate use of phage therapy in people with CF who have treatment-refractory *Mycobacterium abscessus*, a type of nontuberculous mycobacteria (NTM). To learn more, contact the Clinical Trial Navigator.

**PREDICT/PATIENCE: These studies are enrolling adults and children with CF ages 6 years and older.** PREDICT and PATIENCE are observational studies to evaluate current standards for diagnosing and treating nontuberculous mycobacteria (NTM) in people with CF. In PREDICT, researchers will track how often individuals with positive NTM cultures are diagnosed. They will also monitor clinical outcomes and NTM types across regions of the country. The study involves 20 visits over five years. Participants in PREDICT will have the opportunity to enroll in PATIENCE, which will collect data related to treating NTM.

**STOP360: This study is enrolling adults and children with CF ages 6 years and older.** STOP360 is an interventional study to standardize and optimize treatment in people with CF who require IV antibiotics for a pulmonary exacerbation. Participants will be randomized with half receiving treatment with one antibiotic (a beta-lactam) and half receiving treatment with two antibiotics (tobramycin and a beta-lactam). Researchers will measure lung function and symptom improvement to determine whether there is a difference between the two groups. This information will help us understand whether pulmonary exacerbations can be treated with fewer antibiotics. This study is open label, so all participants will receive antibiotics and will know which antibiotics they are receiving. This study will involve three visits over 48 days.

**STOP PEDS 2.0: This study is enrolling children with CF ages 3 to 18 years.** STOP-PEDS 2.0 is an interventional study evaluating the safety and effectiveness of two antibiotic treatment approaches for mild pulmonary exacerbations, which are treated at home, in children with CF. Participants will be randomized to receive either "immediate antibiotics" or "tailored therapy," where antibiotics begin only if symptoms worsen or fail to improve over time. Researchers will track lung function and antibiotic use to learn if one of these approaches is better than the other. This information will help us understand whether mild pulmonary exacerbations in kids can be treated with fewer oral antibiotics. This study involves six study visits and may require pulmonary function testing, lung clearance index, and other measurements.

**BEGIN*:** This study looks at how CFTR modulators affect different aspects of health in young children and infants with CF, including infection. See the "CFTR Modulators" section for more details.

**PROMISE*:** This study looks at how Trikafta affects different aspects of health in people with CF ages 6 years and older, including infection. See the "CFTR Modulators" section for more details.

## DIGESTIVE SYSTEM

**RETRIAL*:** This study looks at how Alyftrek affects different aspects of health in people with CF ages 6 years and older, including liver health. See the "CFTR Modulators" section for more details.

# CF FOUNDATION PRIORITY CLINICAL STUDIES

**STRONG-CF: This study is enrolling adults with CF ages 18 years and older.** STRONG-CF is an observational study that assesses strength and nutrition in adults with CF. Researchers will look at physical measurements, including body mass index, body composition, bone density, arm circumference, hand-grip strength, six-minute walk distance, and lung function. This information will help researchers better understand nutrition in people with CF, as well as what contributes to malnutrition in some people with advanced lung disease. This study involves five visits over 12 months and may require blood draws, lung function tests, nutritional assessments, and other measurements.

**BEGIN*:** This study looks at how CFTR modulators affect different aspects of health in young children and infants with CF, including GI symptoms. See the "CFTR Modulators" section for more details.

**PROMISE*:** This study looks at how Trikafta affects different aspects of health in people with CF ages 6 years and older, including GI symptoms and function. See the "CFTR Modulators" section for more details.

**GALAXY: This study is completed with results.** GALAXY was an observational study that used questionnaires to characterize GI symptoms and treatments in adults and children with CF. The most common symptoms experienced were straining, fullness, incomplete bowel movements, bloating, distension, and lower and upper abdominal pain. The study found that these GI symptoms were more common in adults than in children and more prevalent in females. Participants reported a high overall dissatisfaction with existing therapies. The results from this study established a baseline picture of common upper and lower GI symptoms and medication use in the CF population and helped identify areas for further research.

**NICE-CF: This study is completed with results.** NICE-CF was an observational study to determine whether stool-based testing could substitute for a colonoscopy to screen for colorectal cancer in adults with CF. The study found that colonoscopy remains the most reliable way to prevent and detect colorectal cancer in people with CF.

## CYSTIC FIBROSIS-RELATED DIABETES (CFRD)

**BIONIC CF: This study is enrolling adults and teens with CF ages 14 years and older who have CFRD.** BIONIC CF is an interventional study to evaluate the safety and effectiveness of using the insulin-only Bionic Pancreas system compared to using one's usual insulin delivery method. During the first part of the study, participants will be randomized to use the Bionic Pancreas system or their usual insulin delivery method. During the second part of the study, all participants will use the Bionic Pancreas system. This study involves four visits over seven months and will require continuous glucose monitoring for the duration of the study. It may also require blood draws and questionnaires.

**SPECTRUM: This study is enrolling adults and children with CF ages 12 years and older who have CFRD.** SPECTRUM is an observational study to monitor for diabetes complications, such as retinopathy, neuropathy, and high blood pressure, in a racially and ethnically diverse group of adolescents and adults with CF. Researchers will collect information on diabetes management and problem areas, management of blood sugar levels (glycemic control), fluctuations in blood sugar (glucose variability), kidney function, and liver function. This study involves a single visit and will require questionnaires, continuous glucose monitoring, blood draws, urine specimens, and other sample collections and tests.

**BEGIN*:** This study looks at how CFTR modulators affect different aspects of health in young children and infants with CF, including blood glucose. See the "CFTR Modulators" section for more details.

# CF FOUNDATION PRIORITY CLINICAL STUDIES

**PROMISE*:** This study looks at how Trikafta affects different aspects of health in people with CF ages 6 years and older, including CFRD. See the "CFTR Modulators" section for more details.

## REPRODUCTIVE HEALTH AND PARENTING

**PROTECT*:** This study will evaluate the impact of modulator therapy in utero. See the "CFTR Modulators" section for more details.

**HOPE CF*: This study is enrolling adults with CF ages 18 years and older.** HOPE CF is an observational study evaluating the impact of parenthood on the health of people with CF. Participants will complete questionnaires that assess their physical and mental health over a period of five years after becoming a parent. Participation in the study can be completely remote, and there are no study visits.

**MAYFLOWERS*:** This study looks at the effects of CFTR modulators on women with CF during and after pregnancy. See the "CFTR Modulators" section for more details.

## MENTAL HEALTH

**HOPE CF*:** This study will evaluate the impact of parenthood on the health of people with CF. See the "Reproductive Health and Parenting" section for more details.

**RETRIAL*:** This study looks at how Alyftrek affects different aspects of health in people with CF ages 6 years and older, including mental health. See the "CFTR Modulators" section for more details.

**TIDES 2.0: This study is enrolling children with CF ages 18 months to 11 years.** TIDES 2.0 is an observational study aiming to learn more about anxiety, depression, and behavior concerns in children with CF under age 12. Participants (and/or their parents) in this study will complete questionnaires at three study visits over a 12-to-18-month period.

## ADVANCED LUNG DISEASE AND TRANSPLANT

**ABC STUDY: This study is enrolling adults with CF ages 18 years and older.** The Airway Brush in CLAD (ABC) study is examining whether certain cells in the lung can be used to identify the beginning stages of chronic lung allograft dysfunction, or CLAD. The study uses lung tissue samples from the Cystic Fibrosis Lung Transplant Consortium Biorepository and Registry as well as data from the CF Foundation's Patient Registry. To learn more, contact the Clinical Trial Navigator.