

CF Clinical Trials

Blaze a trail to better treatments and a cure



Marcus, age 19, is actively seeking a cystic fibrosis clinical trial.

Incredible breakthroughs in the treatment of cystic fibrosis have been achieved through clinical trials. Drugs and treatments that have added tomorrows — and make living with CF today better for thousands — are now available thanks to this research and everyone who volunteered to be a part of it. Paving the way for new treatments, we call clinical trial volunteers, and all those who support them, trailblazers.

Why participate

The research community can only develop new treatments for cystic fibrosis with the help of clinical trial volunteers. As a participant, you will be blazing a trail to new therapies and, ultimately, a cure. You may also gain early access to new drugs not available to the public.

Who can participate

With more than 50 trials currently underway across the country, there are plenty of opportunities to be part of a trial. Each clinical trial has guidelines about who can join, known as inclusion and exclusion criteria. Criteria are based on factors like age, CFTR mutation, previous treatment history, current state of health, and other medical conditions. You can search for trials you may be eligible for using the Clinical Trial Finder at [cff.org/find](https://www.cff.org/find).

In 1938, most people with CF didn't see their first birthday.

Today, more than half the CF population is 18 years or older.

There are many kinds of trials — one may be right for you

Trials vary in length, in number of visits (if visits are even required) and can be about a treatment, a drug, or simply involve reporting lifestyle and emotional states. Some may offer compensation. You can participate in many different trials, though not necessarily at the same time, and can leave a trial at any point if needed. If you choose to leave or withdraw from a trial, be sure to discuss this with your research team, as they may ask you to come for a final visit.

“Clinical trials made us feel in control of a disease that has often made us feel so powerless.”

—REBECCA, WHOSE DAUGHTER, CAROLINE, HAS CF

Potential benefits and risks

Like most things in life, participation in a clinical trial comes with possible benefits and risks.

POSSIBLE BENEFITS INCLUDE:

- Taking a more active role in your own health care
- Gaining access to new treatments not available to the public
- Getting expert medical care at leading health care facilities
- Helping others by contributing to medical research
- Receiving a treatment that works for you

POSSIBLE RISKS INCLUDE:

- Experiencing side effects or adverse reactions to medications or treatments
- Receiving a treatment that doesn't work for you

Your safety matters

Multiple regulatory groups, from the U.S. Food and Drug Administration to the Institutional Review Board for each particular study, help determine that every trial is safe and appropriate for patients. CF trials have an additional safeguard — your health is monitored in real time by the Data Safety Monitoring Board, an independent committee of experts in CF clinical care and research, as well as by the study research team and your regular care team. If you or those monitoring the study note any worsening of your health, you can be withdrawn, or the study may be halted altogether.

Visits and scheduling

Study visits vary based on the trial; some require only a quick blood draw, while others may involve multiple visits or more extensive testing. Most research teams are willing to coordinate with your CF care team so that some of your study visits may take place the same day as your or your child's regular clinic appointment. Ask your research team about the time commitment for a specific clinical trial.

Learning about trial results

It's only natural that after participating in a trial, you want to know the results. But it can take researchers several months or more to analyze the data. Once the trial results are available, they are posted on [cff.org/find](https://www.cff.org/find). You can also sign up for email alerts to have results sent to you directly. Your CF care team can help you learn more about what those results mean for you.

What you can do today

- Search for trials you may be eligible for at [cff.org/find](https://www.cff.org/find).
- Ask your care team about clinical trials that may be a good fit for you.
- Tell others about the importance of clinical trials and the critical role that all trailblazers — volunteers, researchers, and care team members — play in transforming the future of CF treatments.

Blaze your own trail at [cff.org/trailblazer](https://www.cff.org/trailblazer)



Dr. George Retsch-Bogart leads a research team that conducts clinical trials for cystic fibrosis.

Caroline, age 7, with her mother, Rebecca. Caroline has participated in five cystic fibrosis clinical trials.

