

What is a clinical trial?



M/C, diagnosed with CF for 17 years

A clinical trial is a research study designed to test an investigational drug, medical treatment, or device in a group of human volunteers. Studies may measure a drug's ability to treat a condition, its safety, and its possible side effects. The information obtained from clinical trials helps doctors and the U.S. Food and Drug Administration (FDA) to decide if a drug is safe and effective for patients.

Clinical trials adhere strictly to the protocol or study design. Clinical trials are under the same ethical and legal codes that apply to medical practice and most clinical research is federally regulated.

Why are clinical trials important?

Clinical trials ensure that patients are prescribed medications that are safe and proven to work. Thanks to clinical trials, thousands of safe and effective treatments are available for thousands of medical conditions, enabling people to live longer and with improved quality of life. Survival rates for certain conditions (such as HIV, prostate cancer, and breast cancer) have increased because of recent research advances.

In addition, clinical trials help determine multiple uses for existing treatments. Increasingly, minority populations are being encouraged to actively participate in clinical trials, as evidence indicates that different ethnic groups respond differently to the same medications.

The TIGER-2 Research Study is seeking volunteers with mild CF lung disease age 5 and older. This research is being done to study the safety and effectiveness of an investigational drug for mild CF lung disease.

Summary of the TIGER-2 Research Study:

- This is a Phase 3 double-blind study.
- Volunteers may not need to change their current medication regimen to participate.
- The investigational medication is inhaled using a nebulizer, three times per day.
- The investigational medication may help improve moisture and mobility of mucus.
- The study lasts for about 6 months.
- During the 24-week treatment period, half of patients receive placebo* and the other half of patients receive investigational medication.
- Study participation includes as many as 7 office visits.
- Procedures include lung function test, chest x-ray, blood and urine tests, physical exams, and questionnaires.
- Volunteers must be age 5 or older, with mild CF lung disease (measured by a lung function test).

* A placebo is an inactive substance that should have no effect.

If you or a loved one has cystic fibrosis and would like to learn more about the TIGER-2 Research Study, please contact:

Dr. Pamela Zeitlin (Principal Investigator)
410-955-2795
Protocol #: NA_00015316



THE TIGER-2 RESEARCH STUDY FOR CYSTIC FIBROSIS

YOU CAN HELP ADVANCE CYSTIC FIBROSIS RESEARCH



A/M, diagnosed with CF for 6 years

J/D, diagnosed with CF for 20 years

D/J, diagnosed with CF for 46 years

M/C, diagnosed with CF for 17 years

Cystic Fibrosis Research and You

As you know, cystic fibrosis (CF) demands a lot of time, effort, and treatments to prevent infections, complications, and worsening of symptoms. CF patients typically use a combination of medications and other treatments to maintain wellness. However, each CF patient may have a different combination of symptoms and may respond to existing treatments differently or not at all. New treatments are always needed to improve quality of life. Each new medication or treatment adds to the number available to CF patients and their health providers.

Before new medications or treatments can be prescribed, they must be tested in human volunteers through research studies called clinical trials. The results of clinical trials are reviewed by the Food and Drug Administration (FDA). The FDA decides if the clinical trial proves that the medication is safe and effective. If so, doctors will be able to prescribe the treatment to patients. All medications must be tested in clinical trials before doctors can give them to patients.

You can help advance CF research. CF patients and their parents/caregivers may be able to volunteer for clinical trials. Through clinical trials, new treatments for CF may become available in the future.

The TIGER-2 Research Study for Cystic Fibrosis

The TIGER-2 Research Study is a clinical trial evaluating the safety and effectiveness of an investigational drug. The study is intended for individuals with mild cystic fibrosis (CF) lung disease.

The investigational drug being evaluated in the **TIGER-2 Research Study** is an inhaled medication which is delivered through a nebulizer. It is believed to work on the cells of the airways to improve the moisture and mobility of mucus. TIGER-2 is a Phase 3 double-blind study. The study drug has been evaluated in Phase 1 and Phase 2 studies with CF patient volunteers and non-CF volunteers. A Phase 3 study (TIGER-1) is currently ongoing in CF patients.

Volunteers for TIGER-2 may not need to change their current medication regimen in order to participate in TIGER-2.

Study Duration

Patients will be enrolled for as many as 28 weeks.

Run-In Period	2 to 10 days	Screening appointment (Visit #1) and the beginning of study drug (at visit #2)
Placebo-Controlled Study Drug Period	24 weeks	Patients receive either the investigational drug or placebo
Follow-Up	7-28 days	One follow-up visit (Visit #7) after stopping the study drug period

Eligibility

The TIGER-2 Research Study is seeking individuals who meet the following criteria:



- Age 5 and older
 - Confirmed diagnosis of CF
 - Mild CF as determined by lung function testing
 - Chest x-ray or other picture of your lungs in the past 12 months indicating no other lung disease
 - No acute respiratory tract infections or exacerbations within the past four weeks
 - No other anti-inflammatory medication, or other in-bronchodilator, corticosteroid, the past week
 - No changes in antibiotic use within the past 2 weeks
 - If using Pulmozyme®, must be using for 28 days or more
- Other criteria apply
- Study Procedures**
- Patients who qualify and enroll in TIGER-2 will take the investigational drug at home three times per day via nebulizer.
- Study participation includes as many as 7 study appointments, which may include any of the following:**
- Physical examinations
 - Questionnaires
 - Lung function testing
 - Chest x-ray

Benefits and Risks of Participating

- Participation in clinical trials contributes to knowledge about CF.
- Participation contributes to the knowledge about the investigational medication and how CF patients respond to it.
- Other CF patients may benefit from the investigational drug in the future if it is proven safe and effective in clinical trials.
- The investigational drug may not help the patient's CF at all.
- Clinical trials often include volunteers taking placebo, which could be a sterile saline (salt water) solution.
- There may be side effects, or undesired effects, to the investigational medication (the drug being studied) or placebo.
- The clinical trial may require more of the volunteer's time and attention than would a non-protocol treatment, including trips to the study site, more treatments, and dosing specifics of the investigational medication.

More information on risks can be provided to study participants by the study physician.

For more information on the TIGER-2 Research Study or to volunteer, contact the physician listed on the back of this brochure.

Terms You Should Know

Double-Blind: In double-blind clinical trial treatment periods, neither the patient nor the physician knows whether the active investigational drug or the placebo is being administered.

Phase 1: Clinical trials with a small number of healthy volunteers that are generally conducted to determine safety and the investigational drug's action in the body.

Phase 2: Clinical trials with a moderate amount of volunteers with the condition intended for treatment to determine both safety and effectiveness of the investigational drug.

Phase 3: Clinical trials with a larger amount of volunteers with the condition intended for treatment and help determine safety and effectiveness of the investigational drug. Drugs can be approved after completing Phase 3 trials.

Phase 4: Clinical trials occurring usually after a drug is approved to study new uses for a drug, compare the approved drug with other treatments, or study its long-term effects.

Placebo: A placebo is a pill, liquid, or powder that contains no medicine. In clinical trials, investigational drugs are often compared with placebos to assess the treatment's effectiveness.

For additional terminology and information on clinical trials, visit ClinicalTrials.com™.