CYSTIC FIBROSIS FOUNDATION®

PLACEBOS IN CF CLINICAL RESEARCH

Find out how placebos work, how they are used in clinical trials, and why they are so important in developing new therapies.

FAST FACTS

- ✓ Placebos do not provide benefit or cause harm.
- ✓ Placebos are necessary to study the effectiveness of potential therapies.
- ✓ Your health is monitored closely during a clinical trial.

WHY ARE PLACEBOS USED IN CLINICAL TRIALS?

Placebos allow researchers to demonstrate whether a potential therapy is safer and more effective than no treatment at all.

It is not always easy to understand how effective a potential therapy is, because some people may get better during a clinical trial even when they aren't taking the active treatment.

That is why researchers design trials with both an **active treatment group** and a **placebo group**. Comparing results from the two groups shows researchers whether changes in the active treatment group were related to taking the treatment, or if they occurred by chance or other factors.

ARE PLACEBOS SAFE?

Unlike the active treatment, a placebo contains no medication. It does not provide benefit or cause harm.

Nothing is more important than patient safety in developing new CF treatments. Your health is monitored closely in real time throughout a trial. If your health worsens at any point during the trial, you and the study doctor will discuss stopping the treatment you were assigned to and whether to continue in the trial. You can choose to stop your participation in a clinical trial at any time.

For more information about safety in CF clinical trials, visit **CFF.org/safety**.

KEY TERMS

Placebo: a product that looks and tastes like the active treatment being tested but contains no medication

Active treatment group: study participants who receive the active drug or intervention being tested

Placebo group: study participants who receive a placebo, or inactive product containing no medication

Randomization: assigning study participants to the active treatment and placebo groups by chance, rather than choice

Blinding: the study participant and/or the research team are unaware of who is receiving the active treatment and who is receiving a placebo

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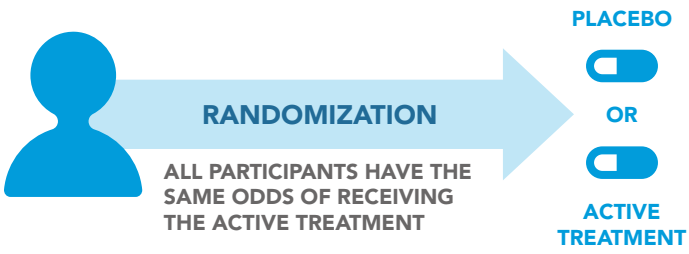
CAN I CHOOSE WHETHER I RECEIVE A PLACEBO OR THE ACTIVE TREATMENT?

The decision about who will receive the active treatment and who will receive a placebo is randomized. This means that participants are assigned to either the placebo group or the active treatment group by chance, rather than choice.

Randomization helps to make the different groups in a trial comparable. Researchers want to ensure that participants in one group have no significant differences from those in another group. For example, if doctors could choose where to assign patients in a trial, some might assign sicker patients to the active treatment group and healthier patients to the placebo group. The doctors might not even realize they are doing this, and it could affect the trial results. Randomization avoids this problem and gives all study participants equal chances of receiving the active treatment.



Many trials are also double-blinded. In a double-blinded clinical trial, you and your research team won't know whether you are in the active treatment group or the placebo group until the trial is over.

IS RECEIVING A PLACEBO INSTEAD OF THE ACTIVE TREATMENT A WASTE OF TIME?

Active treatment groups and placebo groups are equally important in clinical trials.

Every single clinical trial provides crucial information about new potential therapies, and every trial participant is equally important.

When you volunteer for a clinical trial, you are helping others by blazing the trail to new treatments, regardless of whether you receive the active treatment or a placebo. To search for trials that may be right for you, visit CFF.org/find.

"WHETHER OR NOT THE MEDICATION WAS A PLACEBO DURING MY TRIAL, I HELPED GET A NEW DRUG APPROVED."

— Steve, age 50, has participated in seven cystic fibrosis clinical trials.

WHAT TO ASK WHEN CONSIDERING A TRIAL

- What is the purpose of the clinical trial?
- Why do researchers think this particular CF drug or treatment might work?
- How long will the trial last?
- What are the odds of receiving a placebo?
- How long would I be on the placebo?
- Do I need to stop any of my current CF medications?
- How do the possible risks, side effects, and benefits compare with my current treatments?
- Will I be compensated for my participation in the clinical trial and for my travel expenses?
- Will results of the clinical trial be given to me and, if so, when?
- Who should I contact during the trial: the research team, my CF care team, or both?