Understanding Clinical Trials

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What Are Clinical Trials?

The term clinical trial is used to describe many different types of research studies on people ("human subjects"). There are five main types:

- **Treatment trials**
  Study new medications, new combinations of existing medications, or new types of therapies
- **Prevention trials**
  Look for better ways to prevent disease — sometimes with medicines, sometimes by changing people's behavior
- **Diagnostic trials**
  Test the best way to find disease or changes within the body
The Basic Components of Clinical Trials

The scientists and health care providers who manage clinical trials are usually called investigators. The people who join clinical trials are usually called participants but may also be called volunteers or study subjects. ALL clinical trials are voluntary – you never have to participate in a clinical trial unless you want to; this is a human right protected by international laws.

In 2002, the World Health Organization (WHO) released a Handbook for Good Clinical Research Practice [3]. It is intended to help each country develop its own laws about clinical trials. The WHO’s handbook is based in part on the recommendations for good clinical practice developed by the International Conference on Harmonisation (ICH) in 1996. In the US, for example, the Food and Drug Administration (FDA), which approves drugs for public use, has adopted the ICH’s recommendations for clinical trials.

Each clinical trial has a written plan, or study protocol, that describes the goals of the study, how long the study will last, who can participate (also called inclusion and exclusion criteria), and what tests and procedures will be conducted for each participant.

Inclusion and exclusion criteria are the guidelines for determining who can and cannot participate in a specific clinical trial. Inclusion criteria are requirements that a person must meet to participate (such as HIV drug-naïve — never having taken HIV drugs — CD4 cell [4] count, viral load [5], age, or many others). Exclusion criteria are factors that prevent a person from participating, either for his or her safety or to make it easier to understand the study results. For example, people might be excluded for having liver problems, or if they have already taken a drug that is being studied.

Each participant in a clinical trial must sign a document called “informed consent”. When you sign this document, it means that you understand the details of the study and you agree to participate. You may want to take the document with you and talk about the study with your health care provider, family, or friends before you decide to participate. If your native language is not the language spoken by the people describing the study, you should ask if a translation is available. It is important that you truly understand the study and what you will be asked to do. If you have any questions, be sure to ask the study staff. It is their job to make sure that you understand what you are agreeing to do when you sign this document.

You may be paid for travel expenses to and from the study site and for your time; this payment is sometimes called compensation. Childcare may also be provided. You can choose to leave (drop out of) a study at any time and for any reason.

The Stages of Treatment Trials

The most common type of clinical trial in HIV is a treatment or drug study. There are four stages, or phases, of clinical trials for new treatments. With each phase, a little more is known about the treatment being studied. Each phase has a different purpose and helps researchers answer specific questions about the drug in the trial.
Phase I: Is the drug safe?
A Phase I trial tests a drug in a small number of participants (usually fewer than 100) to find a safe dose and to document the side effects [6] of the drug. Phase I trials are usually short, lasting a few days to a few weeks.

Phase II: Is the drug effective?
A Phase II trial tests a drug in a larger number of participants (on average 100 to 300) to see if the drug works. Phase II trials may also test different doses of the drug to find the dose that works best. The safety of the drug also continues to be evaluated. A Phase II trial usually lasts six months to one year.

Phase III: Is the drug safe and effective in larger groups of people for longer periods of time?
A Phase III trial tests a drug in a very large group of participants (typically 1,000 to 3,000). Phase III trials compare the drug to an existing medication or treatment. This lets researchers gather more information about the safety and effectiveness of the drug. Phase III trials generally last two to three years.

Phase IV or Post-Marketing Studies:
What are the long-term results of using the drug? These trials are done after the US Food and Drug Administration (FDA) has approved the drug. They let researchers get more information about the drug's best use and learn more about long-term side effects [6].

Once a drug trial has completed the first three phases of research, the company that makes the drug submits the study data to the organization that regulates drugs in that country (e.g., the FDA in the US) for approval. Because the need for treatments for HIV is so great, a drug company can apply for something called accelerated, or quicker, approval if the drug offers something new or meets a need for people living with HIV (HIV+).

Accelerated approval may put special restrictions on how the drug can be used. However, even if a drug gets accelerated approval, the drug company must continue to do long-term research on the drug to get full approval.

Why It's Important to Consider Participating in a Clinical Trial
To date, women have been drastically underrepresented in HIV clinical trials. This means that many decisions about women's HIV care and treatment are based on research studies in men. Women are not simply smaller versions of men. Women's bodies are different; consequently, we need women's participation in clinical research to help us understand how HIV acts in the female body.

What are the correct HIV drug dosages for women? How do opportunistic infections [7] and gynecologic problems [8] affect women living with HIV? What side effects [6] are more likely to occur in women than in men? Women who participate in clinical trials can help answer many questions specific to women living with HIV while also benefitting the field of HIV research as a whole.

There are many reasons for participating in a clinical trial. You may:

- Get access to new treatments not available to the public
- Receive expert medical care at leading healthcare facilities
- Get access to new experimental medications
- Have a chance to help others by contributing to medical research

Should I Participate in a Clinical Trial?
Choosing to participate in a clinical trial is a big decision. There are risks and benefits to consider.
Risks

- You may experience unpleasant or serious side effects
- You may have to stop all current medications, including any HIV medications
- You may receive a placebo (a pill containing no medication)
- There is no guarantee that the experimental drug will be an effective treatment for you

Benefits

- You may experience health benefits from a new treatment that is not yet available to the general public
- Most trials provide free laboratory tests, expert medical care, and drugs at no cost for the purpose of the study
- You are contributing to the development of a new medication or increasing understanding of HIV disease

Important Lessons from Clinical Trials

Over the past several decades, clinical trial research has helped us gain important knowledge about the treatment and prevention of HIV. Here are some highlights:

- A combination of HIV drugs (combined antiretroviral therapy) is much more effective at controlling HIV and maintaining good health than taking only one HIV drug
- Giving pregnant women [9] HIV drugs can reduce the chances that they will pass HIV on to their babies to as low as one in 100
- **Treatment as prevention** [10]: For people living with HIV, taking HIV drugs can reduce their viral load, thus making them much less likely to transmit HIV to others. If their viral load is undetectable (lower than standard tests can find) and they take their HIV drugs regularly, they cannot pass the virus on to their sexual partners (U=U [11]).
- For people who are HIV-negative, taking HIV drugs can protect them against acquiring HIV if they are exposed to the virus (e.g., PrEP [12] or pre-exposure prophylaxis).
- People living with HIV who start treatment earlier, while their CD4 counts are still high, have a much lower risk of illness and death. This includes people living with HIV who may have no outward signs of ill health.

Clinical Trials and Women Living with HIV

Worldwide, women represent half of all those living with HIV. In the past, women could not participate in many types of clinical trials. Now they can freely take part in trials, but few women do so. A study from 2012 reported that women represented, on average, only one in five participants in clinical trials of antiretroviral drugs approved by the FDA from 2000 to 2008. The good news is that, globally, women's participation in prevention studies, including vaccine studies and behavioral methods, appears higher.

It is important to participate in research because HIV and some of the HIV drugs act differently in women's bodies. The only way to discover these differences is for more women living with HIV to join clinical trials. For information on a clinical drug trial in the US that was successful in recruiting large numbers of women and people of color, see our fact sheet Lessons from GRACE [13].

To find clinical trials that might be of interest or benefit to you, you can search the following registries:

- [ClinicalTrials.gov](https://clinicaltrials.gov) [14] from the US National Institutes of Health (NIH)
- [HIV/AIDS Clinical Trials at AIDSinfo](https://aidsinfo.nih.gov) [15]
- [AIDS Clinical Trials Group (ACTG)](https://actg.info) [16]
- [HIV Prevention Trials Network (HPTN)](https://www.hptn.org) [17]
Tags:

- Clinical trials HIV [21]
- Research HIV [22]
- Human subjects [23]
- Treatment trials [24]
- Prevention trials [25]
- Drug trials [26]
- Informed consent [27]
- Phase 1 trial [28]
- Phase 2 trial [29]
- Phase 3 trial [30]
- Clinical trials women [31]
- Inclusion criteria [32]
- Exclusion criteria [33]
- HIV studies [34]
- HIV studies women [35]

Additional Resources

Select the links below for additional material related to clinical trials.

- Women in Clinical Trials (FDA Office of Women's Health, PDF) [36]
- Las Mujeres en los estudios clínicos (Oficina de Salud de la Mujer de la FDA, PDF) [37]
- Kit de herramientas de Redes Sociales para Socio (Oficina de Salud de la Mujer de la FDA, MS Word) [39]
- Women Who Make a Difference (Cleveland AIDS Clinical Trials Unit, video) [40]
- Women-Only HIV Trial Hailed as Landmark (Medpage Today) [41]
- NIH Clinical Research Trials and You (US National Institutes of Health) [45]
- Understanding Informed Consent (CenterWatch) [46]
- HIV-Positive Women in Clinical Trials: A Gap in the Facts (aidsmap) [47]