

# Patients as Partners: Recognizing the Impact of Participation in Clinical Research

**Presented by:**



# Disclaimer

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The information contained in this slide deck is provided by Teva Pharmaceuticals Medical Affairs department, based on an identified educational need and/or gap.

# What is Clinical Research?

- Clinical research is the study of health and illness in people
- No clinical research would be possible without the help of people who volunteer to join studies



## Observational Studies

- Gather information about people in their everyday settings
- Do not test a medical intervention, such as a medication
- May help find new treatments or behavior changes to test in clinical trials

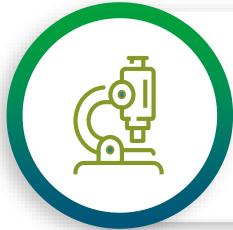


## Clinical Trials

- Test whether a medication, surgery, or a change in behavior is safe and how well it works to treat or prevent health conditions
- Looks at whether a new treatment works better or has less harmful side effects than treatments that already exist

National Institute on Aging. What Are Clinical Trials and Studies? National Institute on Aging Website. <https://www.nia.nih.gov/health/what-are-clinical-trials-and-studies>. Updated March 22, 2023. Accessed August 8, 2023.

# The Importance of Clinical Research



Helps researchers learn more about diseases by studying people who have them



Tests whether medications are safe, how well they work, who should take them, and how much they should take



Finds a way to diagnose diseases before people have symptoms

Finds ways to prevent a health problem for people who are healthy right now, but have a higher chance of developing a disease



Finds ways to improve the lives of people living with a disease



Shows how caregivers and support groups help people living with a disease



National Institute on Aging. What Are Clinical Trials and Studies? National Institute on Aging Website. <https://www.nia.nih.gov/health/what-are-clinical-trials-and-studies>. Updated March 22, 2023. Accessed August 8, 2023.

# Why is Clinical Research Even More Important in Rare Disease?

>90%

of rare diseases have no treatment



Clinical research relies on people, but with rare diseases, it can be hard to find people to join studies

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**We cannot find a cure for HD without research, and we cannot research without funds, and there is no funding if no one knows about HD, and no one knows about HD if the people in the HD community don't step up and be heard. Showing the world that we are here and we matter has real direct impact.**

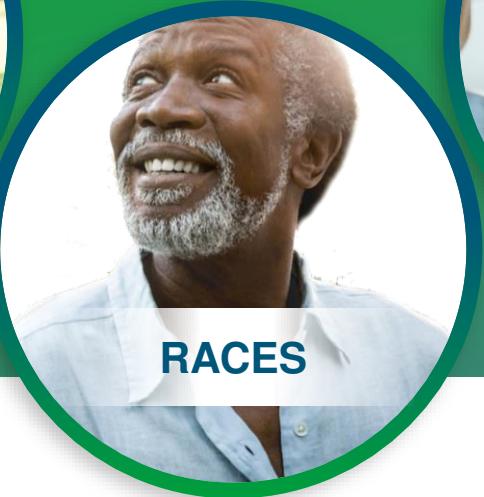
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– Courtney Cable, HD Reality Podcast

HD = Huntington's disease

NORD. Information on Clinical Trials & Research Studies. National Organization for Rare Disorders. <https://rarediseases.org/living-with-a-rare-disease/clinical-trials/>. Published August 5, 2022. Accessed August 8, 2023.

# The Importance of Diversity in Clinical Research



Food and Drug Administration. CLINICAL TRIAL DIVERSITY. FDA Website. <https://www.fda.gov/media/106965/download?attachment>. Accessed August 8, 2023.

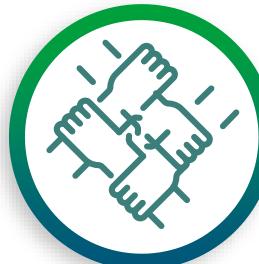
# Taking Part in Clinical Research

People may want to take part in clinical research for many reasons, such as:

**Hope**



**Access**



**Selflessness**



National Institute on Aging. What Are Clinical Trials and Studies? National Institute on Aging Website. <https://www.nia.nih.gov/health/what-are-clinical-trials-and-studies>. Updated March 22, 2023. Accessed August 8, 2023.

# What Is the Timeline for Clinical Research?



## Discovery & Development

- Identify and test molecules that may become medications in a lab

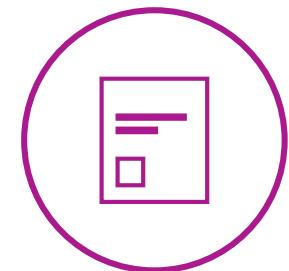
### Investigational New Drug Application (IND)

- Permission to test molecule in people



## Clinical Research

- Phases I, II, and III to see if a medication is safe and works well



## Approval, Launch, and Post-Market Activities

It takes about 10 years and \$2.6 billion to test and approve a new medication



## Preclinical Research

- Animal testing on a select few molecules to make sure they are safe to test on people

### New Drug Application (NDA)

- For medication to be approved to use in people

## Phase 4 trials

- To see how the approved medication works in the real world

Adapted from: Pharmaceutical Research and Manufacturers of America®. Research & Development: Clinical Trials. PhRMA Website. <https://phrma.org/policy-issues/Research-and-Development-Policy-Framework/Clinical-Trials>. Updated May 2022. Accessed August 8, 2023.

# Phase I Trials

## Goals:

- Learn if a medication is safe and find out how much of it should be used (also known as the dosage)
- See how the medication works in the body
- Monitor any side effects and see how they change with different dosages



## Study Size:

Usually small (20-100 people)

## Study Participants:

Healthy volunteers or people with the disease/condition

## Study Length:

Several months

Food and Drug Administration. Step 3: Clinical Research. FDA Website. <https://www.fda.gov/patients/drug-development-process/step-3-clinical-research>. Updated January 04, 2018. Accessed August 8, 2023.

# Phase II Trials

## Goals:

- Begin to learn how well a medication works and what its side effects are
- Provide more information on how safe a medication is



## Study Size:

Up to several hundred people

## Study Participants:

People with the disease/condition

## Study Length:

Several months to 2 years

Food and Drug Administration. Step 3: Clinical Research. FDA Website. <https://www.fda.gov/patients/drug-development-process/step-3-clinical-research>. Updated January 04, 2018. Accessed August 8, 2023.

# Phase III Trials

## Goals:

- Learn even more about how well a medication works and watch for any serious reactions to it
- Show if a medication can help treat a condition
- Show more information about how safe a medication is



**Study Size\*:**  
300 to 3,000 people

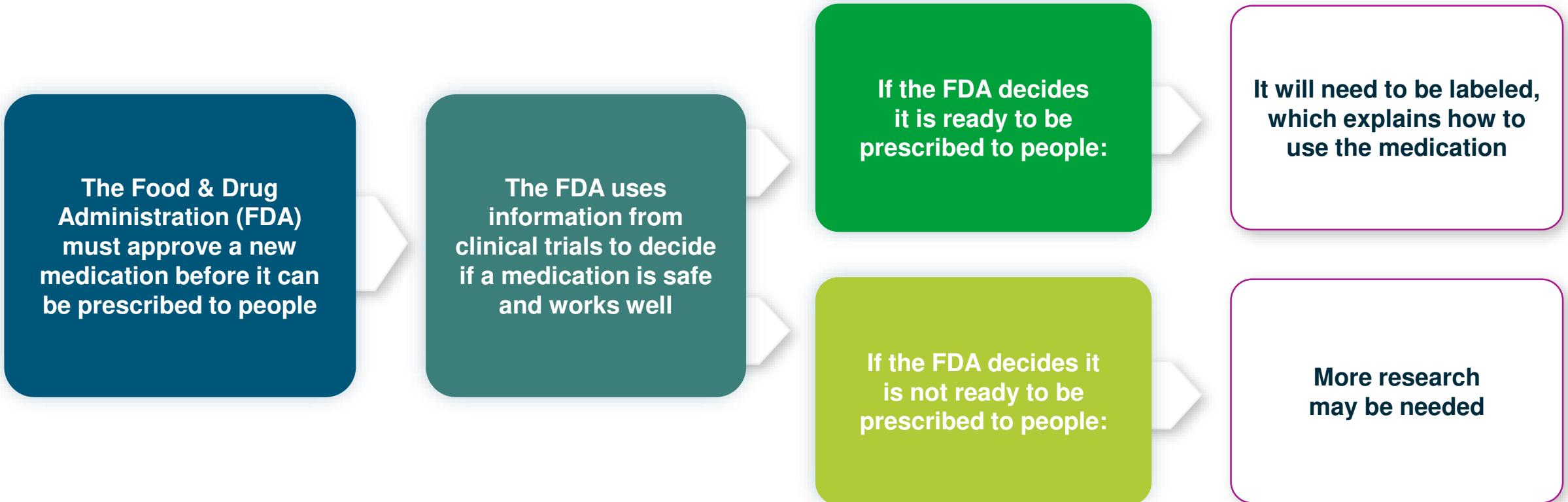
**Study Participants:**  
People with the disease/condition

**Study Length:**  
1 to 4 years

\*Number of participants varies based on the condition.

Food and Drug Administration. Step 3: Clinical Research. FDA Website. <https://www.fda.gov/patients/drug-development-process/step-3-clinical-research>. Updated January 04, 2018. Accessed August 8, 2023.

# New Medication Approval



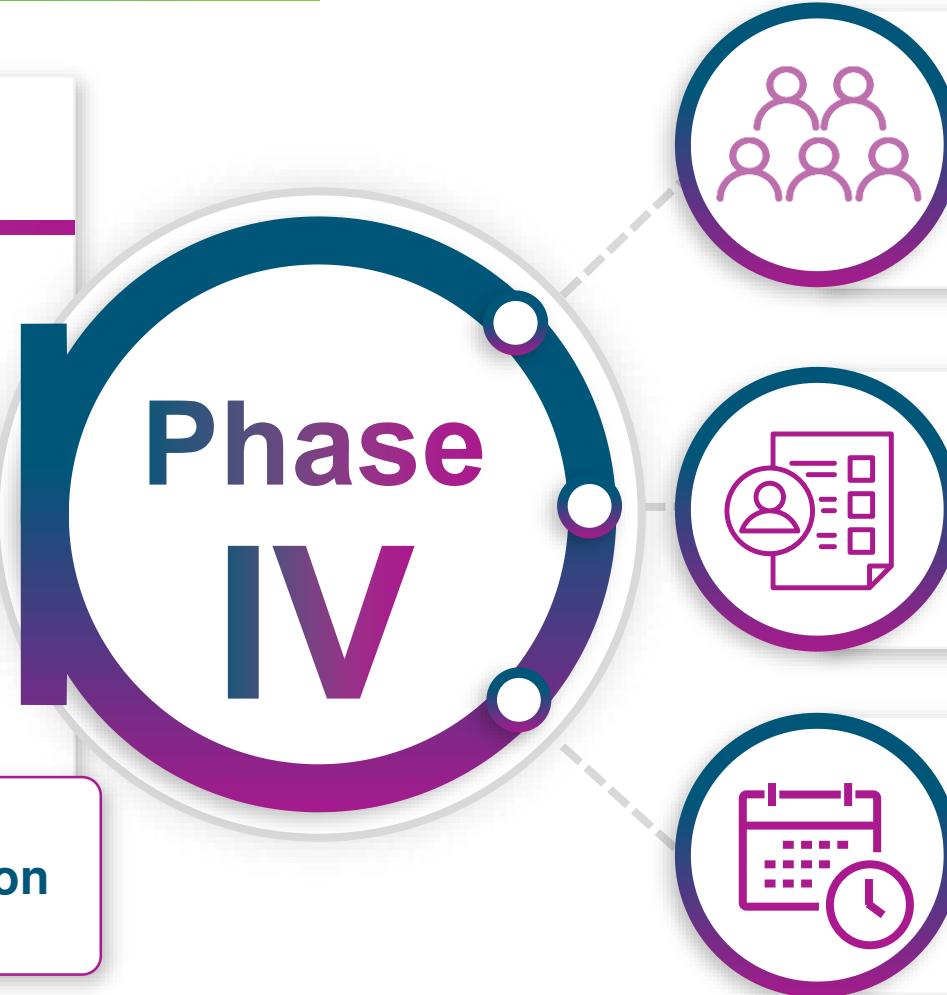
Food and Drug Administration. Step 4: FDA Drug Review. FDA Website. <https://www.fda.gov/patients/drug-development-process/step-4-fda-drug-review>. Updated January 04, 2018. Accessed August 8, 2023.

# Phase IV Trials

## Goals:

- Help researchers learn more about how the medication works in everyday life

**Phase IV studies are completed after a medication is already approved**



**Study Size:**  
Several thousand volunteers

**Study Participants:**  
People with the disease/condition

**Study Length:**  
Changes based on the medication

Food and Drug Administration. Step 3: Clinical Research. FDA Website. <https://www.fda.gov/patients/drug-development-process/step-3-clinical-research>. Accessed December 12, 2022

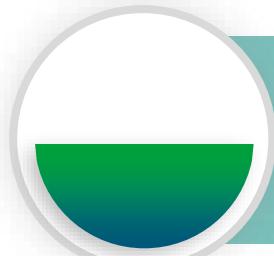
# Do All New Medications Get Approved?



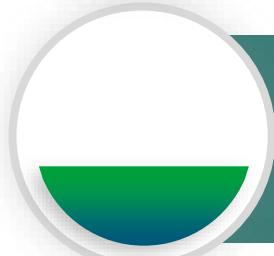
About **9 out of 10** medications that go through the clinical trial process do not get approved

**This means only about 1 in every 10 medications is approved**

- Medications do not get approved for many reasons, such as:



About **half**: do not work as they hoped



About **3 in 10**: have unmanageable side effects



About **1 in 10**: the body cannot process the medication appropriately



About **1 in 10**: have disinterest from companies/ill-planned trials

Sun D. 90% of drugs fail clinical trials – here's one way researchers can select better drug candidates. The Conversation. <https://theconversation.com/90-of-drugs-fail-clinical-trials-heres-one-way-researchers-can-select-better-drug-candidates-174152>. Published February 23, 2022. Accessed August 8, 2023.

# Should I Take Part in Clinical Research?



- Joining a clinical research study is a personal decision
- Deciding to take part in clinical research may help researchers and other people in the future
- If you join a clinical research study, you can always stop at any time – your research team will explain any risks you should know
- Whether you choose to take part in clinical research is completely up to you – you will never be required to join a study to get health care

National Institute on Aging. What Are Clinical Trials and Studies? National Institute on Aging Website. <https://www.nia.nih.gov/health/what-are-clinical-trials-and-studies>. Updated March 22, 2023. Accessed August 8, 2023.

## A Note to Research Participants

# THANK YOU

Taking part in clinical research is a time commitment, a sacrifice, and a very brave choice. If you have taken part in clinical research in the past, thank you for contributing to science.

