CLINICAL TRIAL BASICS: HOW TO BECOME A PART OF THE RESEARCH PROCESS

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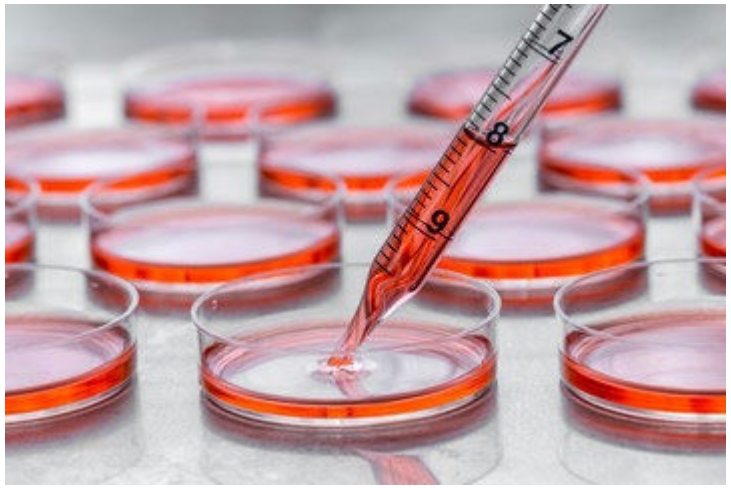
PD research

- Pre-clinical research - research involving cells, animal models, etc.
- Clinical research - research involving people



Clinical research

The two main categories of clinical research are:

- Observational studies
- Interventional studies or clinical trials

Clinical research

Inclusion criteria - the defining characteristics of people with PD who are sought after for trial participation. These criteria might include age, gender, education, diagnostic accuracy, stage of disease.

Exclusion criteria - the characteristics of people with PD who can not be enrolled in the trial. This might include a specific medical co-morbidity or PD symptom

Observational studies

Studies that gather information about people (often using questionnaires or other scales and assessment tools) and compare changes over time.

Interventional studies

Studies that test an intervention (exercise, medication, procedure, surgery) to determine its effect on a health outcome.

Interventional studies are also known as clinical trials.

Types of clinical trials

Phase I: testing in a small group of people (either healthy individuals or patients) to evaluate safety, determine a safe dosage range, and identify side effects.

Phase II: testing in a larger group of patients to see if it is effective and to further evaluate its safety.

Phase III: testing in large groups of patients to confirm its effectiveness, monitor side effects, and compare it to placebo (or less often, commonly used treatments)

Phase IV: Testing after the treatment is approved, to gather more information on use in a more diverse population of patients

Clinical trials terminology

Open label – the investigator and the trial participant are aware that drug is being given

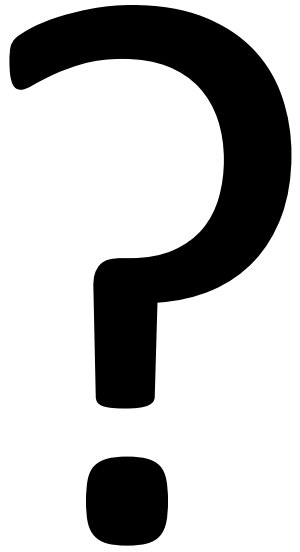
Double-blinded - neither the participant in the clinical trial nor the doctors/study coordinators are aware who is receiving active treatment and who is receiving placebo

Placebo-controlled - one group of people in the trial are not receiving the intervention under study, but rather a placebo - an inactive substance or treatment. This allows results of those receiving the intervention to be compared directly to the results of those not receiving the intervention.

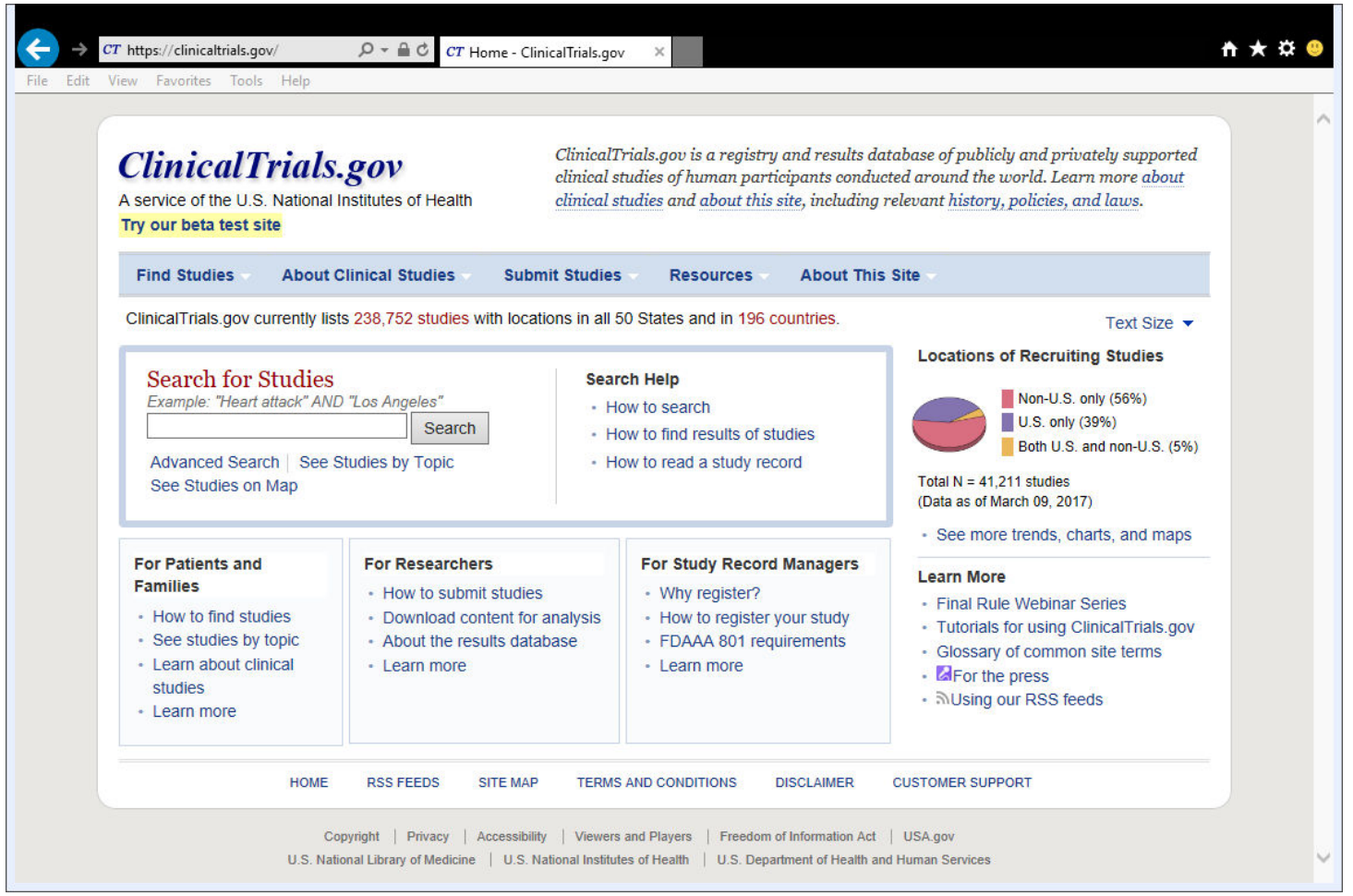
Randomized - study participants are randomly placed in one of the treatment groups

How do you find out about clinical trials?

- Ask you doctor
- clinicaltrials.gov
- Databases of clinical trials



CLINICALTRIALS.GOV



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Why participate in a clinical trial?

Lack of participants in clinical trials is the number one obstacle to success of a clinical trial!

Why participate?

- The chance to feel empowered and more in control, proactive instead of passive
- Study participants advance our scientific understanding of PD
- All our current treatments are available due to others who volunteered as study participants
- The potential to help yourself and help others

Overcoming obstacles to clinical trial participation

“I called the trial site and someone told me that the trial would be open to enroll patients soon. But no one has gotten back to me!”

“I found out about a trial but was told that I don’t meet criteria to participate”

“I don’t want to participate in a trial if I may get the placebo”

THE HOPE LIST

https://drive.google.com/file/d/1NeSyFA37b9IbUzryRRP-EqrgScjCRL-3/view

Dr. Kevin McFarthing

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Clinical Trial Highlights

Parkinson's Disease Drug Therapies in the Clinical Trial Pipeline: 2023 Update

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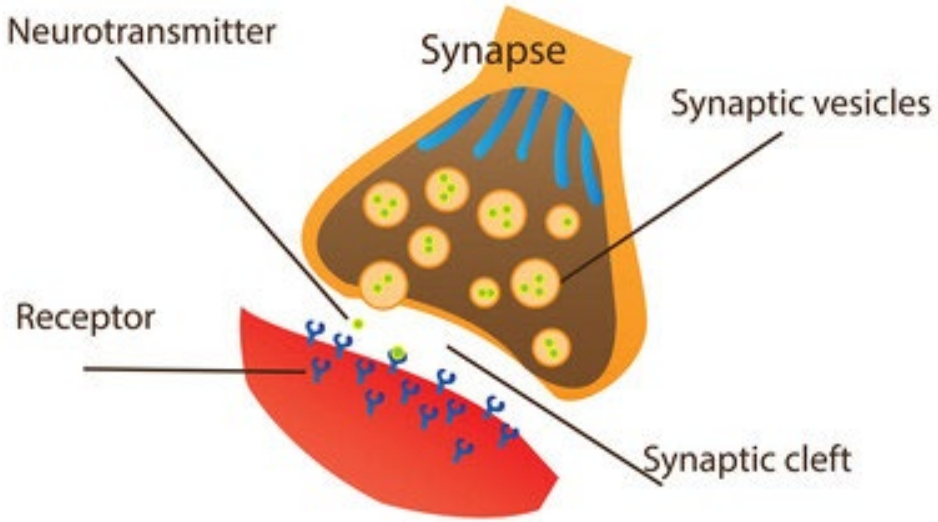
EXAMPLES OF CLINICAL TRIALS – PHASE 1

Phase 1 – Lu AF28996 in Participants With Parkinson's Disease (PD)
NCT04291859

Goal: 29 participants in 4 sites
Primary outcomes: safety, tolerability and pharmacokinetics of LU AF28996, a D1/D2 receptor agonist



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EXAMPLES OF CLINICAL TRIALS – PHASE 2

Phase 2 - **A Randomized, Double-Blind, Placebo-Controlled Trial of IkT-148009 in Untreated Parkinson's Disease -** NCT05424276

- Goal: 120 participants across 27 sites
- Primary outcomes – safety, tolerability and pharmacokinetics of IKT-148009, an oral c-ABL tyrosine kinase inhibitor. (c-Abl acts on a wide range of targets and plays a role in oxidative stress, alpha-synuclein aggregation and neurodegeneration)
- Secondary outcomes – effect on motor and non-motor symptoms of PD

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EXAMPLES OF CLINICAL TRIALS – PHASE 3

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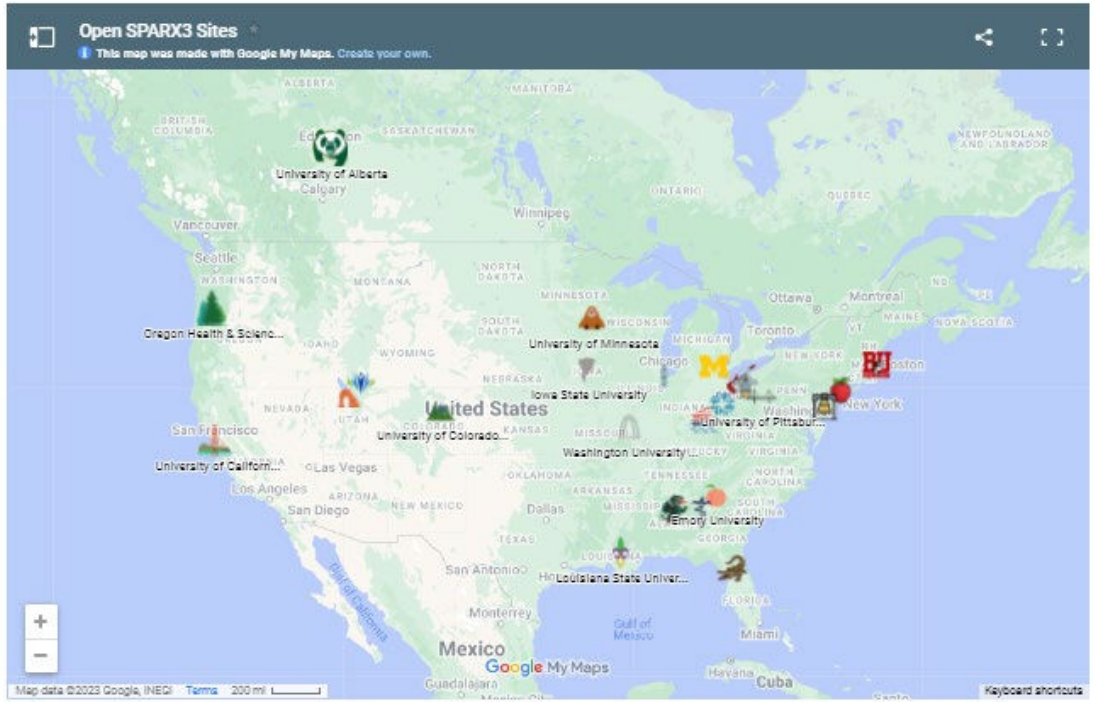
SPARX3

Study in Parkinson's Disease of Exercise Phase 3 Clinical Trial

We are pleased to announce 24 sites are open for enrollment for SPARX3.



www.sparx3pd.com NCT04284436

SPARX3 STUDY

Phase 3 - Study in Parkinson Disease of Exercise (SPARX3)

Goal: 370 people across 24 sites

Primary objective - to test whether the progression of the signs of Parkinson's disease is attenuated at 12 months in early PD, with the performance of high-intensity endurance treadmill exercise at 60-65% maximum heart rate vs 80-85% maximum heart rate, 4 times per week.

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SPARX3 STUDY

Looking for people with:

- PD diagnosis within the last 3 years
- Aged 40-80
- Positive DaTscan
- Not currently on any PD medication and not expected to require medication for the next 6 months
- But – can have taken PD medication in the past – but none within the 60 days prior to starting the study

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RECENT RESEARCH DEVELOPMEMTS

- Blue Rock released phase 1 data of **Bemdaneprocel - dopaminergic neuron precursor cells** into the brains of people with PD. Transplantation of the cells was feasible, safe and well-tolerated and resulted in successful cell survival and engraftment. A phase 2 study is currently being planned for early 2024.

- Biovie released phase 2 data of **NE3107 – a naturally occurring steroid that acts to decrease inflammation and increase insulin sensitivity.** Results showed that use of the medication was associated with improved motor function and reduced symptoms.

- Mitsubishi-Tanabe released phase 3 data from their BouNDless trial - **subcutaneous levodopa** was more effective than standard carbidopa/levodopa at controlling motor symptoms of PD.

WASHINGTON STATE PARKINSON'S DISEASE REGISTRY

Washington State
Parkinson Disease Registry

About Us • Contact Us

Register Today

Why we need a registry:

The purpose of the Washington State Parkinson Disease Registry is to make Parkinson disease (PD) research happen faster by connecting people with Parkinson disease to the research community.

We do this by:

- Informing patients about upcoming research trials for which they would be good candidates
- Assisting researchers with the recruitment of eligible patients

Why this is important:

- There is still much to learn about PD. Continued research is essential to a better understanding of disease progression, possible therapies, and an eventual cure.
- It is crucial for people with PD and people without PD to participate in PD research. Without your help therapeutic discoveries will slow.
- Patient recruitment is often times the essential and limiting factor in how quickly the research can be completed.

Who is eligible:

- People with PD or other Neurological Disorders
- People without PD (controls)

Click here for additional information!

Call toll free to register:

888-365-9901

www.registerparkinsons.org

TIPS AND TAKEAWAYS

- Clinical research is divided into observational studies and interventional studies, also known as clinical trials
- Clinical trials are divided into Phases 1-4 depending on how many people are being tested and what outcomes are being measured
- Clinicaltrials.gov, other databases, and your doctor are sources for clinical trials that you may want to participate in
- The Washington State PD registry allows researchers to contact people with PD who are interested in participating in research