

Clinical Studies for Primary Progressive Aphasia

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Information adapted from <https://www.clinicaltrials.gov>

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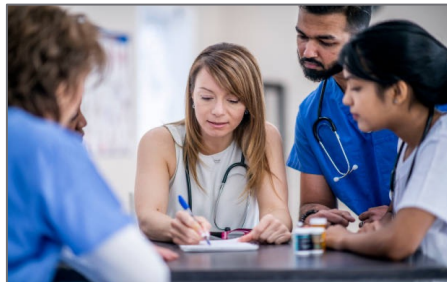
What is a clinical study?

Research intended to add medical knowledge of diseases or conditions using human volunteers informing:

PREVENTION

DIAGNOSIS

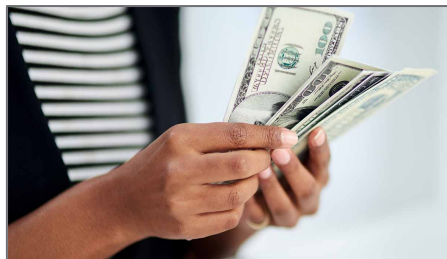
TREATMENT



Led by “principal investigator” (medical doctor, clinical professional, research scientist) with a research team.



Follows a detailed research plan / protocol that includes specific procedures for the research team and volunteers.



Funded by companies, health care providers, academic institutions, private donors, the federal government, etc.

Reasons for conducting a clinical study

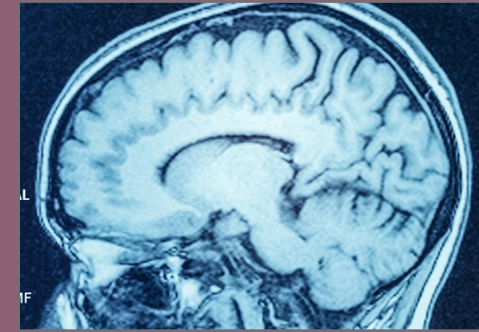
Identify
risk factors



Determine
preventative measures



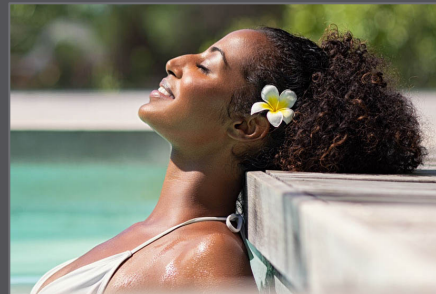
Improve
diagnostic accuracy.



Develop
effective interventions



Explore ways to improve
quality of life



Advance the fields of
science and medicine



Two main types of clinical studies:

1. Observational Studies
2. Interventional Studies / Clinical Trials

Observational studies

- Investigators assess health outcomes
- Volunteers may receive standard interventions or clinical procedures as part of their routine medical care
- BUT, volunteers are *not assigned* to specific study conditions

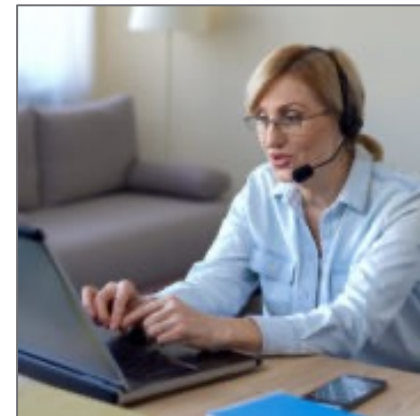
Example

Investigators may observe a group of older adults to examine the effects of different lifestyles on brain health.



Interventional Studies / Clinical Trials

- Participants are assigned specific interventions (drugs, devices, speech therapy, diet, etc.)
- May compare new medical approach to:
 - The standard or currently available approaches
 - A placebo
 - No intervention
- When a new approach is being studied, it is sometimes unknown whether it will be helpful, harmful, or no different than alternative approaches (including no intervention)
- Investigators try to determine the safety and efficacy of the intervention by measuring certain outcomes in the participants.



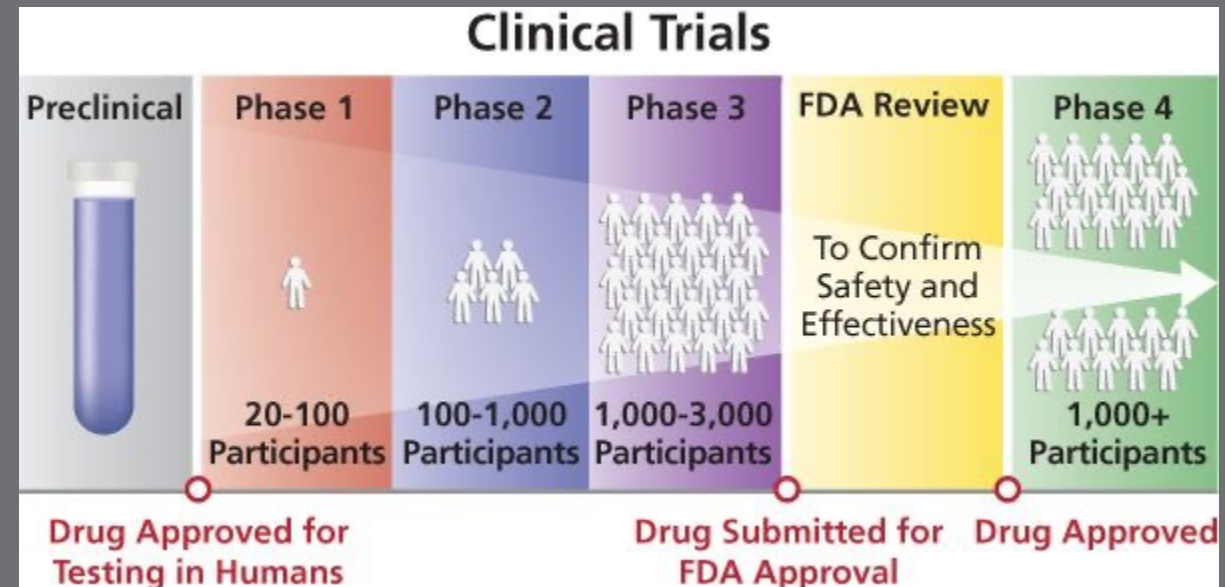
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Example

Investigators may administer speech-language therapy to participants with primary progressive aphasia to see whether communication improves.



How Drugs Are Approved



How do I find opportunities
to participate in clinical
studies?

<https://clinicaltrials.gov/>

Observational Studies for PPA in North America

from ClinicalTrials.gov as of August 8, 2024

Title	Locations
The Neurobiology of Two Distinct Types of Progressive Apraxia of Speech	Mayo Clinic, Rochester, Minnesota
Genetic Frontotemporal Dementia Initiative for Neurodevelopment	Western University, London, Ontario
Investigating Genetic Status in Patients Presenting to Clinic	Parkwood Institute, London, Ontario
A Multi-Modal Remote Monitoring Platform for Frontotemporal Lobar Degeneration (FTLD) Syndromes	Johns Hopkins School of Medicine, Baltimore, Maryland MGH, Boston, Massachusetts
Longitudinal Cognitive Assessment by BoCA	Miami, Florida
ARTFL LEFFTDS Longitudinal Frontotemporal Lobar Degeneration (ALLFTD)	Multiple Locations
Phenotype, Genotype and Biomarkers 2	Multiple Locations
Family Caregiver Online Survey (Dementia and Swallowing Difficulties)	University of Oregon, Eugene, Oregon
UPenn Observational Research Repository on Neurodegenerative Disease	University of Pennsylvania, Philadelphia, Pennsylvania
Retinal Imaging in Neurodegenerative Disease	Duke University Medical Center, Durham, North Carolina
DC Longitudinal Study on Aging and Specimen Bank	Georgetown University Memory Disorders Program, Washington DC
Diagnosis of Alzheimer's Disease Using Event Related Potentials	VA Boston Healthcare, Jamaica Plain, Massachusetts
Longitudinal Study of Neurodegenerative Disorders	UPMC Children's Hospital of Pittsburgh, Pittsburgh, Pennsylvania
NYSCF Scientific Discovery Biobank	New York Stem Cell Foundation Research Institute, New York, NY
Language in Primary Progressive Aphasia	Northwestern University, Chicago, Illinois
Investigating Complex Neurodegenerative Disorders Related to Amyotrophic Lateral Sclerosis and Frontotemporal Dementia	National Institutes of Health Clinical Center, Bethesda, Maryland
Characterization of Inclusion Body Myopathy Associated With Paget's Disease of Bone and Frontotemporal Dementia (IBMPFD)	University of California, Irvine, Irvine, California
Functional Near-Infrared Spectroscopy (fNIRS) Combined With Diffuse Correlation Spectroscopy (DCS) in Neurocognitive Disease as Compared to Healthy Neurotypical Controls	National Institutes of Health Clinical Center, Bethesda, Maryland
Improving Prognostic Confidence in Neurodegenerative Diseases Causing Dementia Using Peripheral Biomarkers and Integrative Modeling	Multiple Locations

Clinical Trials for PPA in North America

from ClinicalTrials.gov as of August 8, 2024

Title	Locations
Enhancing Language Function in PPA	University of Arizona, Tucson, Arizona
Transcranial Direct Current Stimulation in the Treatment of PPA	Multiple Locations
Treating PPA Using High-definition tDCS	University of Pennsylvania, Philadelphia, Pennsylvania
Communication Bridge 3 Study	University of Chicago - American School Building, Chicago, IL
A Study to Evaluate the Safety and Effect of AVB-101 in Subjects With a Genetic Sub-type of Frontotemporal Dementia (FTD-GRN)	Multiple Locations
A Study of PBFT02 in Patients With Frontotemporal Dementia and Progranulin Mutations (FTD-GRN)	Multiple Locations
Targeting Language-specific and Executive-control Networks With Transcranial Direct Current Stimulation in Logopenic Variant PPA	Johns Hopkins Hospital, Baltimore, Maryland
TMS for the Treatment of PPA	Massachusetts General Hospital, Boston, Massachusetts
Veri-T: A Trial of Verdiperstat in Patients With svPPA Due to TDP-43 Pathology	Multiple Locations
Educational Support Group Program for Bilingual and Spanish-speaking Carepartners and People With Progressive Aphasia	University of Texas at Austin, Austin, Texas
Investigating Speech Sequencing in Neurotypical Speakers and Persons With Disordered Speech	Multiple Locations
Phase 1/2 Clinical Trial of LY3884963 in Patients With Frontotemporal Dementia With Progranulin Mutations (FTD-GRN)	Multiple Locations
Longitudinal Multi-Modality Imaging in Progressive Apraxia of Speech	Mayo Clinic, Rochester, Minnesota
Gamma-Induction in FrontoTemporal Dementia Trial	Multiple Locations
Enhancing Language Function in Aphasia	University of Arizona, Tucson, Arizona
Molecular Neuroimaging of Neuroinflammation in Neurodegenerative Dementias	Parkwood Institute, London, Ontario
Tau PET Imaging in Atypical Dementias	Northwestern University, Chicago, Illinois
Cognitive Reserve and Response to Speech-Language Intervention in Bilingual Speakers With PPA	Multiple Locations
Assessment of Hyperphosphorylated Tau PET Binding in PPA and Frontotemporal Dementia	Mayo Clinic, Rochester, Minnesota
Subgenual Cingulate Deep Brain STIMulation for Apathetic Behavioral Variant FRONttemporal Dementia	Toronto Western Hospital, Toronto, Ontario
Lemborexant for Insomnia in a Patient With Dementia: An N-of-1 Trial	Nova Scotia Health, Halifax, Nova Scotia
First-in-Human Evaluation of an Astrocytic Glutamate Transporter (EAAT2) PET Tracer in Dementia	UCSF, San Francisco, California
Care Planning for a Loved One With Dementia: Knowledge, Preparing for Decisions, and Emotions	Massachusetts General Hospital, Boston, Massachusetts
A Study of Caregiver Connections Via Technology in Dementia	Mayo Clinic Rochester, Rochester, Minnesota
Stories in the Moment: Dance Program for People Living With Dementia	Online, New York, New York
Longitudinal Imaging of Microglial Activation in Different Clinical Variants of Alzheimer's Disease	Columbia University Irving Medical Center, New York, NY
Propranolol in PPA	University of Missouri-Columbia, Columbia, Missouri

Example questions to ask for observational studies and clinical trials

- *What is being studied?*
- *What will I have to do?*
- *What tests and procedures are involved?*
- *How often will I have to visit the hospital or clinic?*
- *Will hospitalization be required?*
- *How long will the study last?*
- *Who will pay for my participation?*
- *Will I be reimbursed for other expenses?*
- *What type of long-term follow-up care is part of this trial?*
- *Will results of the study be provided to me?*
- *Who will oversee my medical care while I am participating in the trial?*
- *What are my options if I am injured during the study?*

Example questions to ask for clinical trials

- *Why do researchers believe the intervention being tested might be effective? Why might it not be effective? Has it been tested before?*
- *What are the possible interventions that I might receive during the trial?*
- *How will it be determined which interventions I receive (for example, by chance)?*
- *Who will know which intervention I receive during the trial? Will I know? Will members of the research team know?*
- *How do the possible risks, side effects, and benefits of this trial compare with those of my current treatment?*
- *If I benefit from the intervention, will I be allowed to continue receiving it after the trial ends?*

Any questions?

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