Parkinson's Disease

Spotlight on Clinical Trials – What You Need to Know

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Welcome and Introductions

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Presenters

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Presentation

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Disclosure Statements

Dr. Saint-Hilaire: Research Investigator for studies supported by NIH, Michael J. Fox, Acorda, and consultant for Delsys Inc.

Ray James: Research Nurse Coordinator for trials supported by NIH, Michael J. Fox, Acorda.

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Today's Overview

- The role of clinical trials in Parkinson's Disease
- Phases of a clinical trial, from idea to approval
- Participation in a clinical trial: consent, eligibility, schedule of activities
- How to find ongoing clinical trials

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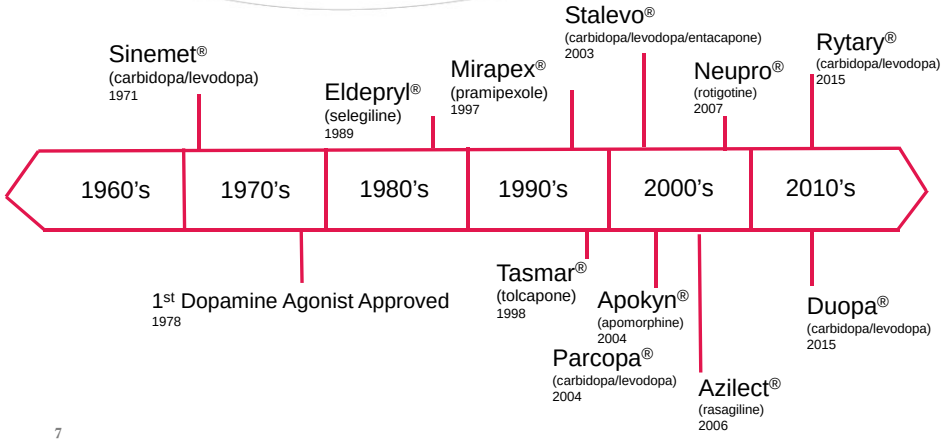
Timeline of Major Parkinson's Medications Currently Approved in USA



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What is a Clinical Trial?

Any research study that prospectively assigns human participants or groups of humans to one or more health-related interventions to evaluate the effects on health outcomes.

World Health Organization

Clinical trials determine if treatments are safe and effective.

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Purpose of Clinical Trials

- Determine safety and efficacy of a therapy
- Observe characteristics of a disease or impact on an individual
- Understand how a therapy works (i.e. pharmacokinetics of a drug)
- Learn information that can be applied to a broader group of people

Clinical trials are the best way for researchers to learn how to diagnose, treat and prevent Parkinson's.

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Types of Clinical Studies

Interventional Study: Participants are assigned to receive one or more interventions, or no intervention, so researchers can evaluate the effects on health-related outcomes (such as improving Parkinson's symptoms).

Observational Study: Researchers observe behavior or collect data in a systematic way without influencing or interfering with the behavior.

www.clinicaltrials.gov

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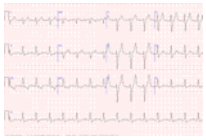
Examples of Interventional Trials in Parkinson's

- **Drug Studies**
  - New medications to treat symptoms or slow progression
  - New ways of delivering a medication (i.e. patch, controlled-release)
  - New indications of a previously approved medication
- **Surgical Studies**
  - Deep Brain Stimulation
  - Cell/Gene transplants



- **Non-Medical Treatments**
  - Exercise, PT, OT, Speech, Tai Chi
  - Cognitive-Behavioral

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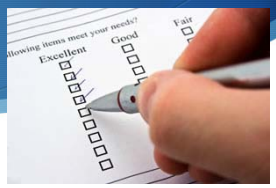
Examples of Observational Studies in Parkinson's



- Biomarkers
  - Biomarkers are characteristics of a disease that can be tested
  - Test blood, spinal fluid, urine, saliva, skin, genes, imaging, neuropsych
- Risk factors for Parkinson's
- Social-Behavioral studies
- Exercise (natural course of physical activity in Parkinson's)
- Cognition and Depression in Parkinson's

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Phases of Clinical Research

- Preclinical
- Phase I
- Phase II
- Phase III
- Phase IV

It takes between 6 and 11 years for a drug to obtain FDA approval.

FDA Review website: http://www.fdareview.org/approval_process.shtml

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Preclinical Phase

Studies are done in the laboratory (non-human) to support that a therapy is reasonably safe and may be effective.

5 in 5,000 drug compounds in pre-clinical stages go on to be tested in humans.

FDA Review Website http://www.fdareview.org/approval_process.shtml

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Phase I

A very small group of people, often healthy volunteers, are studied to assess the risks and side effects of a drug or other therapy.

There are approximately 23 medicines in development for Parkinson's. About 7 Phase I trials are currently underway.

Source: Pharmaceutical Research and Manufacturers of America (PhRMA)

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Phase II

- Approximately 80-150 volunteers who have the condition the drug is designed to treat, for example Parkinson's disease.
- These studies provide additional information about safety and also help determine dosage of the drug to be tested in future trials.
- These trials may be too small to determine the drug's benefit or efficacy.

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Phase III

- Increases the number of volunteers, usually greater than 300.
- These studies are conducted at multiple sites across the US and beyond.
- The data collected from phase III studies are important for the FDA to make decisions about approving a drug or therapy.

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Phase IV

- The FDA often recommends these studies be done after a drug is approved.
- During this phase, researchers continue to monitor the health of people taking the medication or therapy to better understand long-term safety and effectiveness.
- These are often open-label studies that may provide information on populations of individuals that may not have been included in the original studies.

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Who Sponsors a Study?

- Government
  - NIH, NINDS, DOD, NSF
- Industry
  - Pharmaceutical, Biotech
- Non-Profit Foundations
  - May co-sponsor a study or provide research funding

NIH

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Presentation

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A Walk Through a Clinical Drug Trial

- Study Design
- Responsibilities of the Research Team
- Oversight of Trials
- Eligibility
- Consent
- Schedule of Activities

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Clinical Study Design

- Open Label
  - Everyone receives the drug or therapy
- Randomized Controlled
  - Drug or therapy is compared to placebo or a control group
  - Participants are assigned randomly to each group
- Single Blind Placebo Controlled
  - The subject does not know what group they are assigned
- Double Blind Placebo Controlled (Gold Standard)
  - Subject and Researchers do not know to which group the subject is assigned

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Responsibilities of Each Study Site

- Each study has a person in charge
  - Each study site has a Principal Investigator (Site PI)
- Formulate research team
  - PI, Sub-PI, Study Coordinator, Research Assistants, Regulatory Contact
- Train study team on conduct of the study
  - Good Clinical Practice (GCP)
  - Investigator Meetings
- Prepare Protocol for review by the Institutional Review Board

GCP
Good Clinical Practice

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Oversight for Clinical Trials

- Ethics Board Review (IRB)
  - Composed of physicians, nurses, scientists, and lay community members
  - Oversee the researchers conducting the trial and ensure participant safety
- Office of Human Research Protection (OHRP)
  - Protect rights and wellbeing of research subjects
  - Develop educational programs and materials, maintain regulatory oversight, and provide advice on ethical and regulatory issues in biomedical research
- Food and Drug Administration (FDA)
  - Provide review of new drug applications, and surveillance of research drugs/devices as well as approved drugs/devices

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Consent and Eligibility

- Consent
  - Study Purpose
  - Procedures / Payments
  - Risks / Benefits
  - Rights
  - Confidentiality / HIPAA
- Inclusion/Exclusion Criteria

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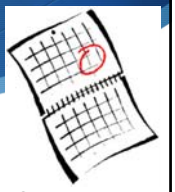
Schedule of Activities



- Screening
  - Inclusion/Exclusion is reviewed, laboratory tests, verification of condition
- Baseline
  - Randomization into the study
- Interim Visits
  - Frequency and duration of visits depend on the complexity of the study
  - Monitor safety and effectiveness during the trial
- Final Visit
  - Ensure safety of the participant, monitor for side effects

What Happens After the Trial?

- Results will not be available until all participants have finished the trial and data analysis is completed, which may take several months to a few years.
- Some trials allow participants to roll over into an open label trial.
- Participants may be kept informed by webinars and letters.
- Subjects eventually will learn their treatment group during the study (placebo vs active drug).
- Investigators and sponsors are responsible to publish results, whether positive or negative.
- Experts review results before submitting to the FDA.

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Finding Ongoing Clinical Trials

- Health Care Team
- Clinicaltrials.gov
- Fox Trial Finder
- Center Watch
- Parkinson Study Group
- APDA Chapters and Information & Referral Centers
- Parkinson Advocates in Research (PAIR)
- Local Parkinson Support Group

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How Has Subject Participation Made a Difference?

- **Some Examples**
  - Made available treatments including Levodopa, Dopamine Agonists, MAO-B Inhibitors, COMT Inhibitors.
  - Improved delivery methods of medications, i.e. Long-acting formulations, Patch, Infusion pump.
  - Deep Brain Stimulation
  - Better understanding of genetics
  - Importance of exercise
  - Other clinical aspects, i.e. improved knowledge of non-motor symptoms

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Final Thoughts

It is important for individuals with Parkinson's to participate in clinical trials.

There is also opportunity for individuals without Parkinson's to contribute.

Many studies invite healthy control subjects to participate.

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Thank You!

We all need to contribute to the solution, each and everyone of us.

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Question & Answer

Marie Saint-Hilaire, MD, FRCPC

Ray James, BS, RN

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Closing Remarks

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For additional information, answers to your questions, or resources

Please visit our website
www.apdaparkinson.org

Or call us
1-800-223-2732