



PARKINSON'S DISEASE

# SPOTLIGHT ON CLINICAL TRIALS: OPENING THE DOOR TO NEW TREATMENTS

THURSDAY, OCTOBER 31, 2019

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## WELCOME AND INTRODUCTIONS



**Stephanie Paul**

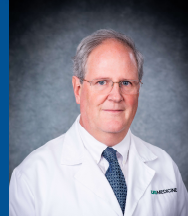
*Senior Vice President Development and Marketing*  
American Parkinson Disease Association

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## PRESENTATION



**David G. Standaert, MD, PhD**

*John N. Whitaker Professor and Chair of Neurology  
The University of Alabama at Birmingham School of Medicine  
Chair, APDA Scientific Advisory Board*

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## FINANCIAL DISCLOSURES

**Commercial Research Support:** AbbVie Inc.

**Consultant:** AbbVie Inc., Theravance Inc., Sanofi-Aventis, Censa Pharmaceuticals, Cerevance Pharmaceuticals, Appello Pharmaceuticals, AVROBIO, Grey Matter Technologies

**Speakers Bureaus:** none

**Equity:** none

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## WHAT IS A “CLINICAL TRIAL” ?

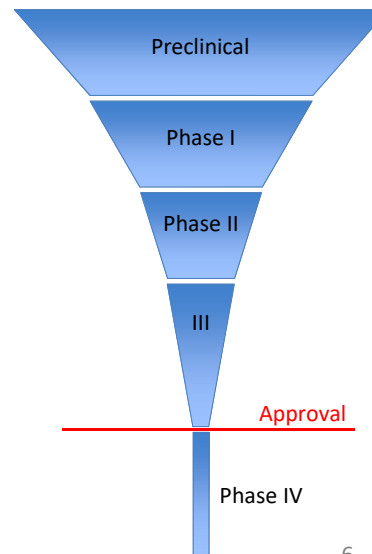
- Different kinds of trials, different purposes:
- **Observational Study**
  - Determine the cause of a disease or condition
  - Find out about the natural history of a disease
  - Assess long-term outcomes of treatments
- **Interventional Study**
  - Test a new therapy for safety and/or efficacy
  - Often involves “placebo control,” the gold standard for establishing effectiveness

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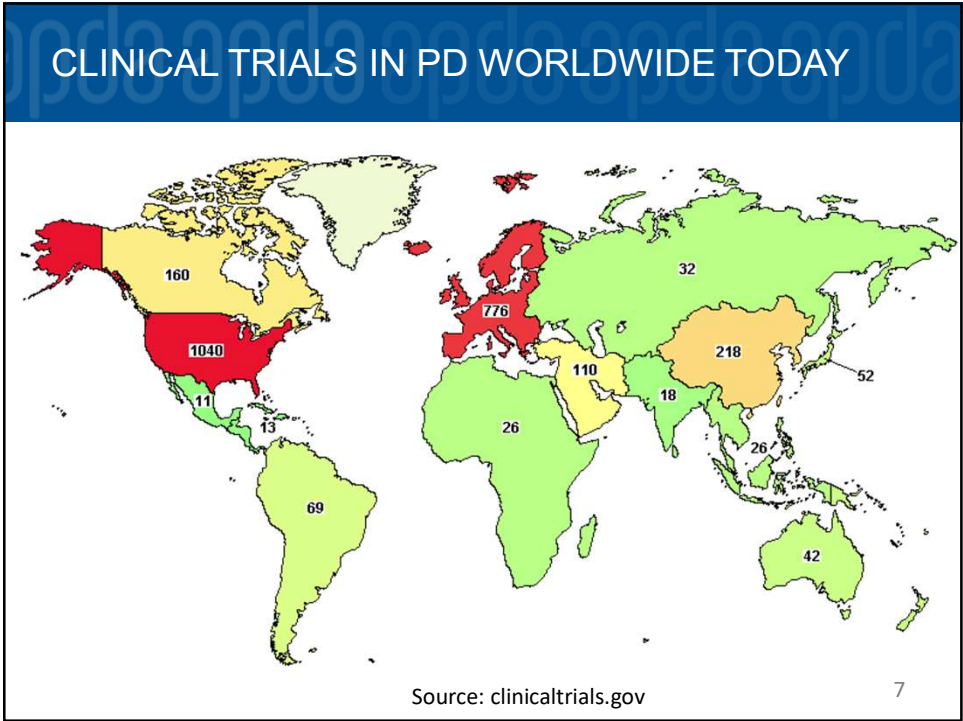
## PHASES OF INTERVENTIONAL CLINICAL TRIALS

- **Preclinical**: Safety and efficacy in animal models
- **Phase I**: “first in human,” short term, low doses, small numbers
- **Phase II**: “first in patients,” dose finding, safety
- **Phase III**: definitive study to establish efficacy. Almost always blinded and placebo-controlled
- **Phase IV**: after approval, long term studies of safety or use in a new situation



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### CURRENT CLINICAL TRIALS IN PD: USA

ClinicalTrials.gov is a database of privately and publicly funded clinical studies conducted around the world.

Explore 320,474 research studies in all 50 states and in 209 countries.

**Find a study** (of 146,000)

Status:  Recruiting and not yet recruiting studies,  All studies

Condition or disease: Parkinson Disease

Other terms: (For example: NCT number, drug name, investigator name)

Country: (dropdown)

**Clinicaltrials.gov** is the comprehensive source for trials

- 307 Currently active trials in PD
- 214 Active Interventional trials
- 141 Interventional, open to recruitment
- 70 Observational, open to recruitment

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## HIGHLIGHTS OF CURRENT TRIALS IN PD

- Current Interventional Trials
  - NILO-PD
  - Synuclein antibodies
  - Novel DBS technologies, focused ultrasound
  - Exercise
- Current Observational Trials
  - Inflammation
  - Biomarkers
  - Outcomes

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## A NOTE ABOUT TRIALS AND TRIAL RESULTS

- Trials are generally posted on [clinicaltrials.gov](http://clinicaltrials.gov) when they begin. This is a requirement of many funding agencies and the journals that publish the results of trials.
- When interventional trials are in progress, it is usually difficult to find out what the result so far are. This is by design!
  - Releasing partial results could affect the outcome of the trial
  - Releasing partial results could affect investments in the sponsoring companies, and result in violation of insider trading laws
- All of the interventional trial information we will discuss today can be found on **[clinicaltrials.gov](http://clinicaltrials.gov)**

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## NILOTINIB IN PARKINSON'S DISEASE (NILO-PD)

- Nilotinib is a drug approved to treat leukemia
- Preclinical studies suggested that nilotinib could slow degeneration of dopamine neurons
- Phase IIa study: Safety, tolerability of nilotinib in PD
- Sponsors: Northwestern, Rochester, Iowa, MJFF
- Two cohort study
  - Cohort 1: PD more than 5 years, nilotinib or placebo for 8.5 months
  - Cohort 2: Early PD, nilotinib or placebo for 14.5 months
- Current status: Cohort 1 is enrolled, waiting on results before Cohort 2 can begin

NCT03205488

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## SYNUCLEIN ANTIBODIES

- Preclinical studies: Alpha-synuclein builds up in the PD brain. Antibody infusions may be able to remove the abnormal protein and slow the disease
- Studies:
  - BIIB054 in Participants With Parkinson's Disease (SPARK) (Sponsor: Biogen)
  - Prasinezumab (RO7046015/PRX002) in Participants With Early Parkinson's Disease (PASADENA) (Sponsor: Roche)
- Both studies are Phase II, main outcome is safety
- Both are fully enrolled
- Both are expected to complete in early 2021

NCT03100149,  
NCT03318523

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## NOVEL TECHNOLOGIES

- New ways to deliver DBS from the NIH Brain Initiative:
  - Directional DBS for Parkinson's disease (Walker, UAB)
  - Closed Loop DBS for Parkinson's disease (Starr, UCSF)
  - Sponsor: National Institutes of Health
- Focused Ultrasound for PD
  - ExAblate Pallidotomy for Medically-Refractory Dyskinesia Symptoms or Motor Fluctuations of Advanced Parkinson's Disease
  - Sponsor: InSightec, Inc.

NCT03353688,  
NCT01934296,  
NCT03319485

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## EXERCISE TRIALS IN PD

- Study in Parkinson's Disease of Exercise (SPARX)
  - Phase II trial (completed)
  - Sponsor: University of Colorado
  - High-intensity treadmill exercise (4 days per week, 80%-85% maximum heart rate for 6 months)
  - Completed, showed that high-intensity treadmill exercise was safe and tolerated
  - Exercise group showed less change in PD symptoms
- Exercise Dosing Trial for Individuals With Parkinson's Disease (PDEX) - Univ of Virginia
- Motor Training in PD (Adapted Tango Dancing) – Atlanta VA Medical and Rehab Center

NCT01506479,  
NCT02676804,  
NCT02457832

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## OBSERVATIONAL STUDIES IN PD

- **Alabama Udall Center (National Institute of Health)**
  - Study of immune system activation in PD
  - Early PD patients and controls
  - Brain imaging, lumbar puncture, genetics, and followup
- **Parkinson Progression Marker Initiative (MJFF)**
  - Largest observational study of early PD
  - 1,400 participants
  - 25,000 biospecimens
  - 1,700,000 data downloads to investigators
- **Fox Insight (MJFF)**
  - Online study of the PD patient experience
  - More than 40,000 participants
  - PD patients and controls
  - <https://foxinsight.michaeljfox.org/register>

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## CLINICAL TRIALS IN PD: HOW CAN YOU GET INVOLVED?

- Resources for finding clinical trials you can participate in:
  - [apdaparkinson.org/research/clinical-trials/](http://apdaparkinson.org/research/clinical-trials/)
  - [clinicaltrials.gov](http://clinicaltrials.gov)
  - [foxtrialfinder.michaeljfox.org](http://foxtrialfinder.michaeljfox.org)
  - [Parkinson-Study-Group.org](http://Parkinson-Study-Group.org)
- To find the cure, we need people with PD who are willing to join us in the search.
- Both PD and healthy controls are needed, there is a trial for everyone!

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## QUESTION & ANSWER



**David G. Standaert, MD, PhD**  
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## CLOSING REMARKS



**Stephanie Paul**  
*Senior Vice President Development and Marketing*  
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**FOR ADDITIONAL INFORMATION,  
ANSWERS TO YOUR QUESTIONS,  
OR FOR ADDITIONAL RESOURCES**

Please visit our website  
**[apdaparkinson.org](http://apdaparkinson.org)**

Or call us  
**1-800-223-2732**