

# PARKINSON'SDISEASE2014

ADVANCING RESEARCH, IMPROVING LIVES

National Institute of Neurological Disorders and Stroke

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## Vision for Parkinson's Disease Research

The vision for PD research is to meet the most urgent research needs cutting across clinical, translational, and basic research
Develop precision medicine to provide the right treatment to the right person at the right time:

Right person: Each person living with PD experiences the disorder in a unique way. The key factors — genetic risk, early symptoms, and environment need to be identified in each individual.
Right time: Develop tools and technologies to track disease progression.
Right treatment: Accelerate development of targeted treatments using tools and technologies described above.

Filling critical gaps in our knowledge about Parkinson's disease:

Developing innovative technologies to better understand how PD changes the brain;
Gaining better understanding of the basis of individual variation among PD patients;
Creating new experimental models to study PD-related genes;
Taking advantage of all the data available, ranging from laboratory studies to electronic medical records.

Supporting key infrastructure:

Coordinating repositories of data and biospecimens;
Creating consensus guidelines for research;
Encouraging data sharing.

## Strategy for Achieving the Vision

The research recommendations developed by the PD2014 planning effort provide a strategy for achieving the vision.

Top 3 priorities for clinical research:

1. If we can learn more about the early stages of PD, we will be able to design clinical trials to delay or slow down the disease 2. If we can develop treatments (and ways to track patients' responses to them) for levodopa-resistant features of Parkinson's disease, such as gait and balance problems as well as dementia, we will improve quality of life for people living with PD

3. If we understand why people living with PD experience a wide variety of symptoms and why some people living with PD progress faster than others, we will improve quality of life for people living with PD

### Top 3 priorities for translational research:

1. If we can develop ways to study groups of patients who have similar disease features, we will be able to design better clinical trials and create targeted treatments

2. If we can create new PET imaging markers and confirm they work as expected in animal models and in human tissue, we will be able to follow disease progression

3 If we can create experimental model systems that are more relevant for PD in humans, we will develop drugs likelier to succeed in clinical trials

#### Top 3 priorities for basic research:

1. If we can determine how harmful alpha-synuclein moves from one cell to another we may be able to slow down disease progression

2. If we can learn more about the key molecule involved in PD, alpha-synuclein, we may be able to slow down or prevent Parkinson's

3. If we can learn how Parkinson's disease affects circuits (groups of brain cells that work together) we may be able to prevent or improve symptoms of the disease