

# Using data to reimagine clinical trials

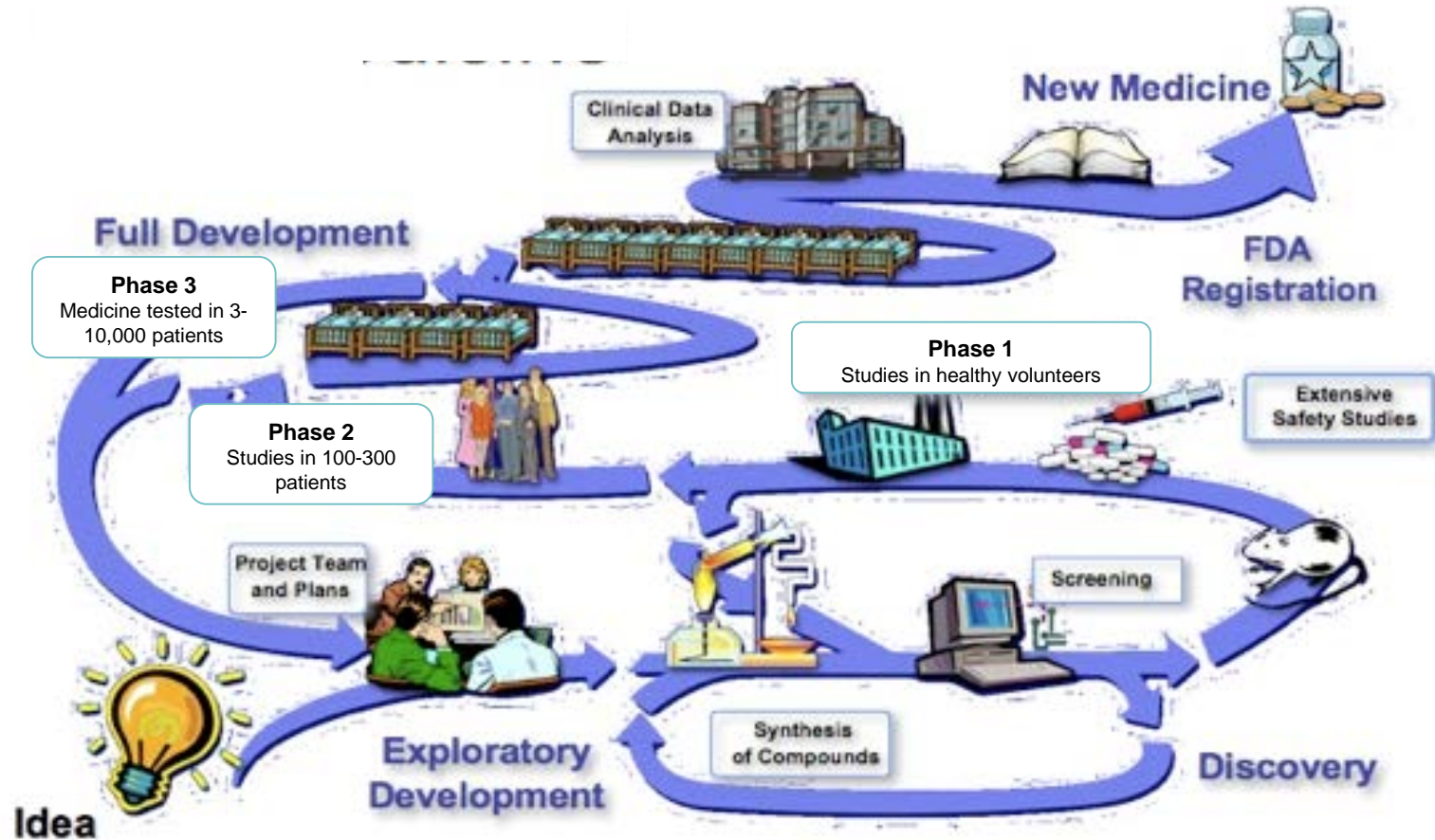
**Diane Stephenson, PhD**

Executive Director, Critical Path for Parkinson's  
Critical Path Institute

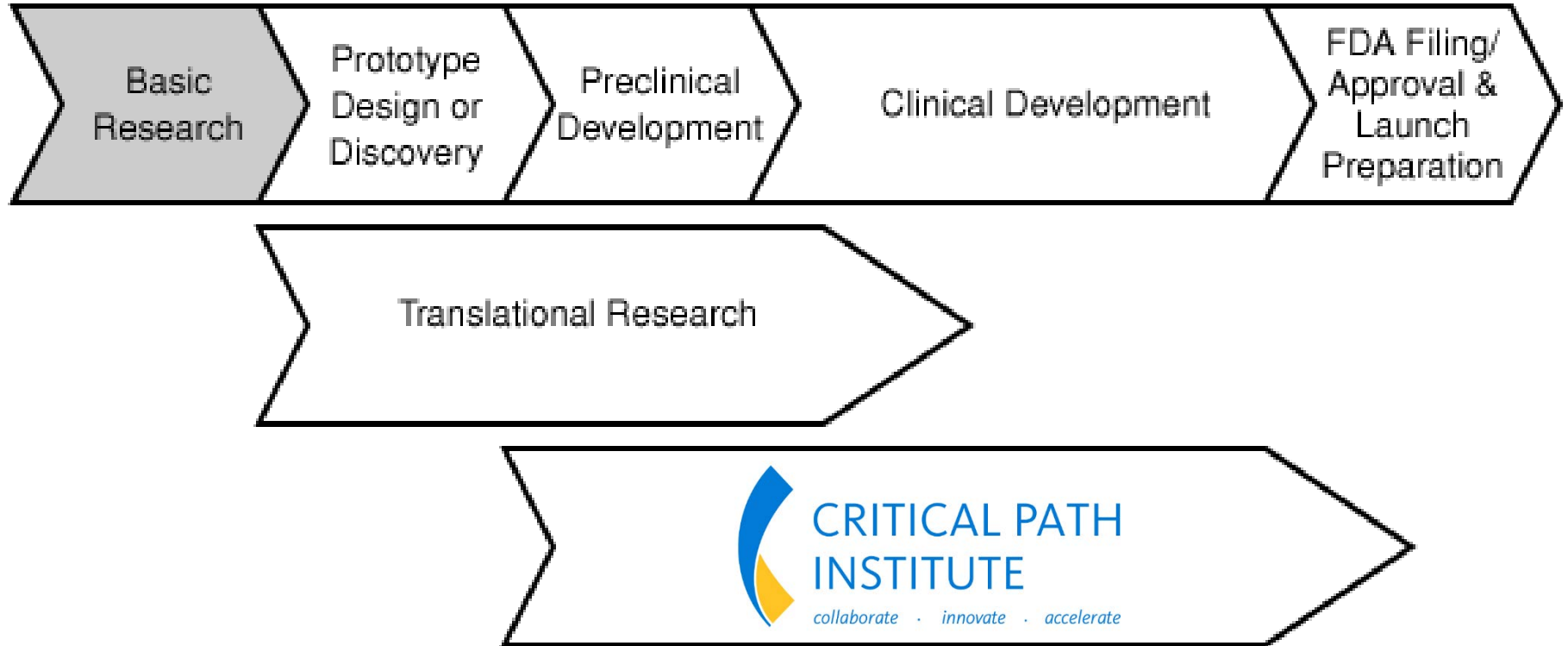
Wednesday 15 November 2017

**PARKINSON'S<sup>UK</sup>** CHANGE ATTITUDES. FIND A CURE. JOIN US.

# The long road to a new medicine

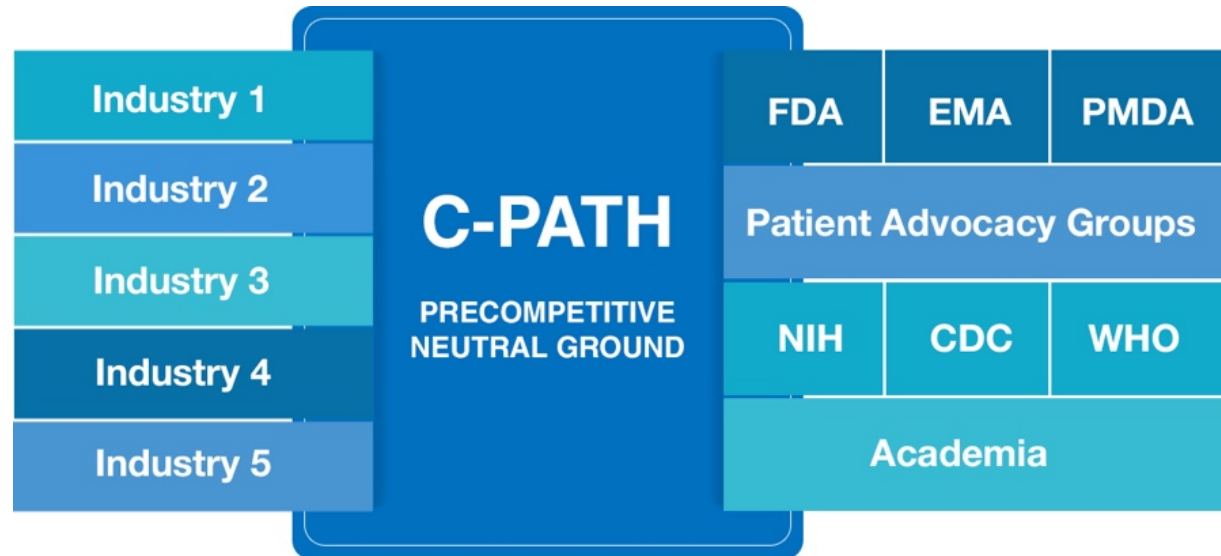


# What is Critical Path research?



# What is the Critical Path Institute?

A trusted, neutral third party delivering new methods and tools for clinical trials which benefit the whole community.



# Critical Path for Parkinson's

**PARKINSON'S<sup>UK</sup>**  
CHANGE ATTITUDES.  
FIND A CURE.  
JOIN US.



**Academic Experts**

**National Institutes of Health**



**Lundbeck**

**UCB**

**AbbVie**

**Pfizer**

**Merck**

**GSK**

**Eli Lilly**

**Biogen**

**GE Healthcare**



**Persons with Parkinson's**

**Individual Advisors**



## **Patient-Advocacy Organizations**

- Parkinson's Foundation
- Michael J. Fox Foundation
- Davis Phinney Foundation
- The Cure Parkinson's Trust

## **UK Academic Institutions**

- University of Oxford
- University of Cambridge
- Newcastle University
- University of Glasgow

# We can learn from past clinical trials

Vitamin E

Antioxidant

GPI-1485

Neuroimmunophilin

Riluzole

Glutamate antagonist

CEP-1347

Anti-apoptotic

Paliroden

Stimulates NGF

Co Q10

Mitochondrial enhancer

Pramipexole

Dopamine agonist

Cogane

Modulates GDNF & BDNF

Creatine

Mitochondrial modulator

Pioglitazone

PPAR- $\gamma$  agonist;  
anti-inflammatory

Rasagiline

MAO-B inhibitor

Glutathione

Antioxidant

TCH346

Propargylamine

Mitoquinone

Mitochondrial enhancer

# What could we do if we had all the data from Parkinson's studies in one place?

Data from clinical trials and cohorts



Standardization and integration



CDISC Data Standards



Researchers  
Regulators  
Industry



# Future model of Parkinson's therapies

Parkinson's -  
Not all one flavor



Personalized Medicine  
targeted treatments



*As modified from Alberto Espay*

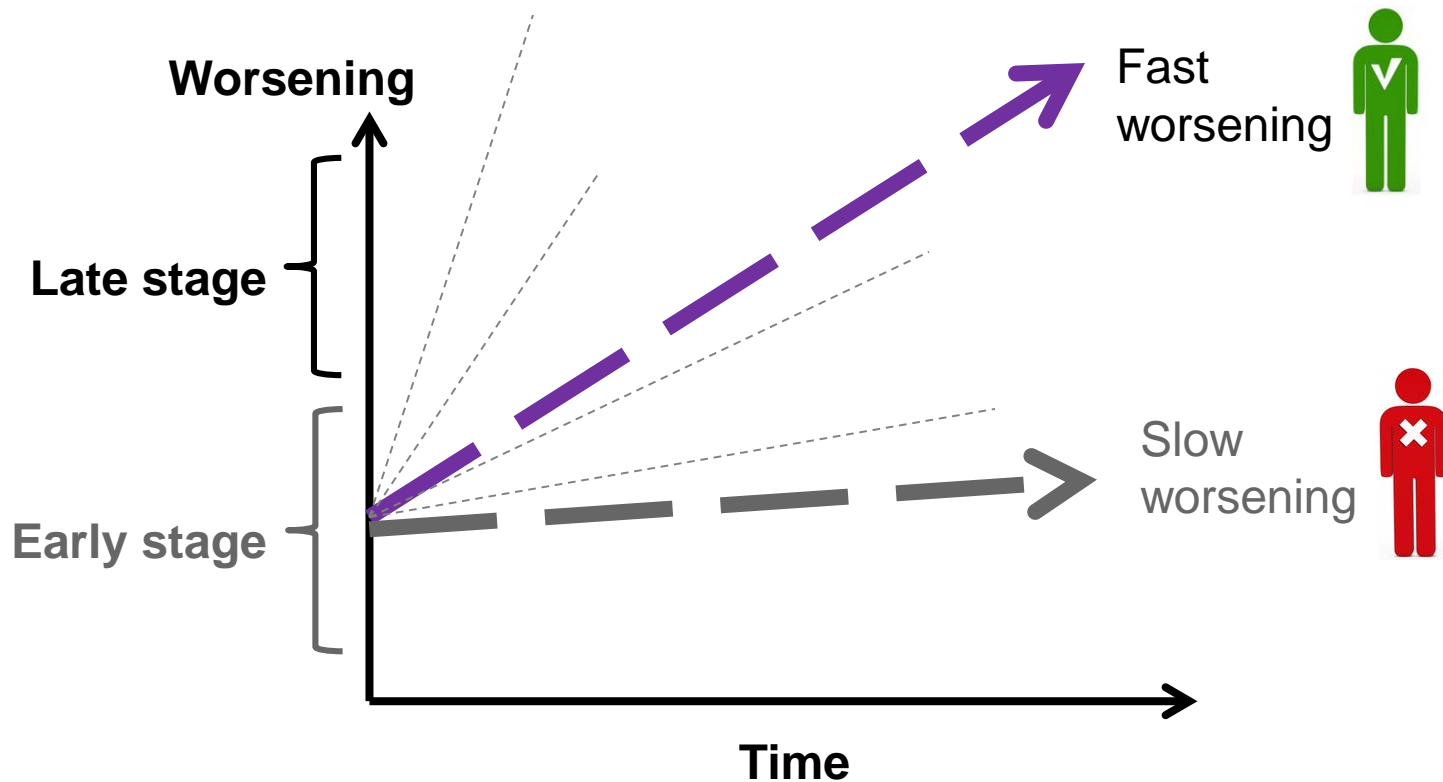


Up to 15% of people with early Parkinson's who take part in trials may not have the condition at all.

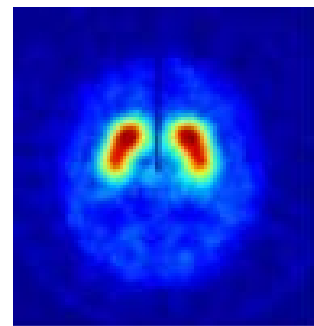
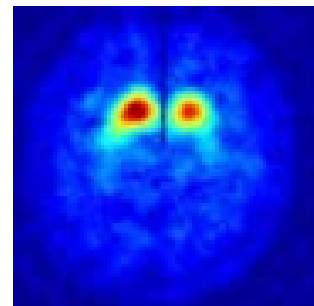
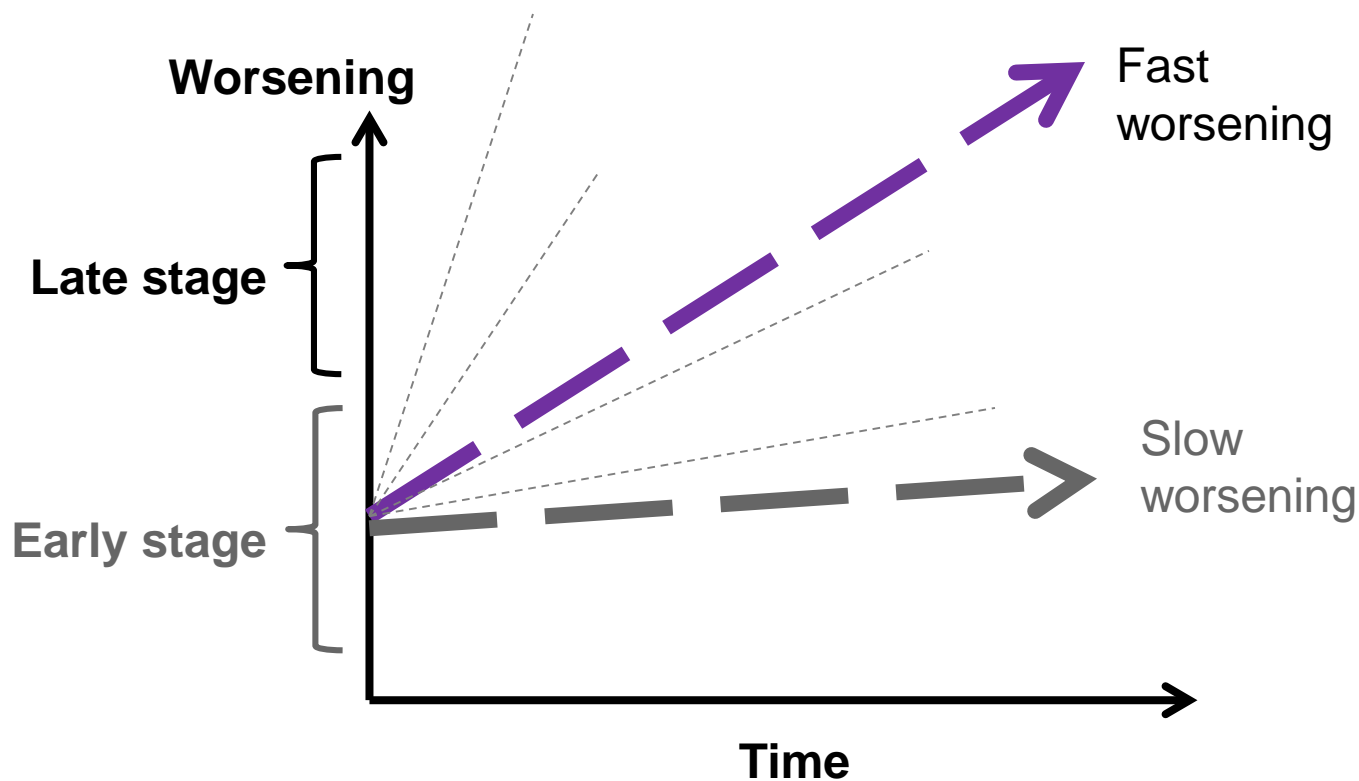


*Beth Vernaleo, Parkinson's Disease Foundation*

# Some people worsen fast, others slowly...



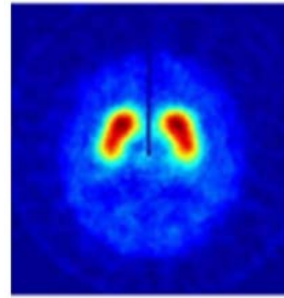
# Using imaging to predict the future



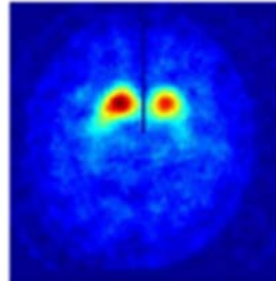
# Using imaging as a biomarker in trials endorsed by EMA



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH



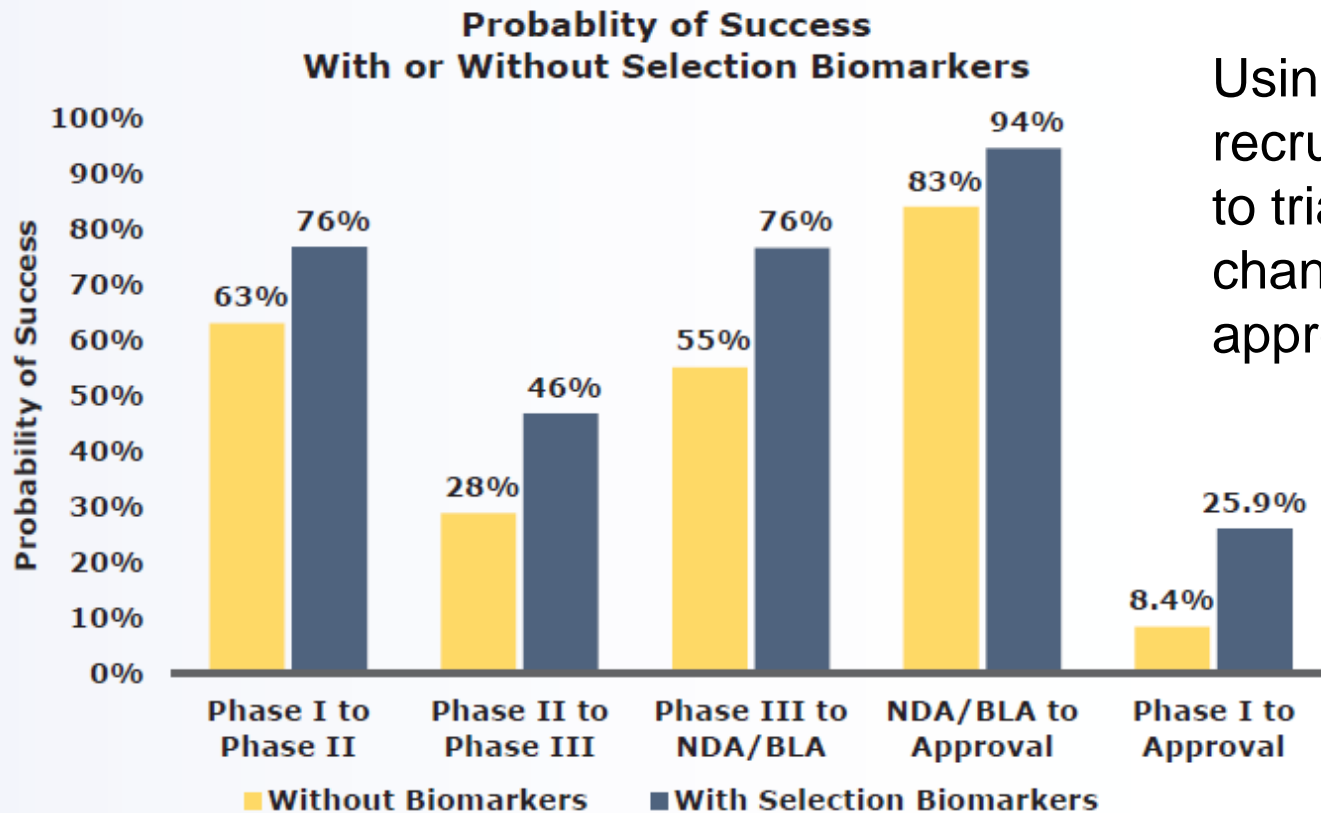
Normal brain scan (no dopamine deficiency)



Dopamine deficiency consistent with Parkinson's



# What impact could this make?



Using biomarkers to recruit the right people to trials increases chances of drug approvals.

# What's next for the Critical Path for Parkinson's?



Integrate data from around the world to help create tools that bring:

The right drug  
To the right person  
At the right time