Using data to reimagine clinical trials

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Critical Path Institute

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The long road to a new medicine

Phase 1
Studies in healthy volunteers

Phase 2
Studies in 100-300 patients

Phase 3
Medicine tested in 3-10,000 patients
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

Academic Experts
National Institutes of Health

Patient-Advocacy Organizations
• Parkinson’s Foundation
• Michael J. Fox Foundation
• Davis Phinney Foundation
• The Cure Parkinson’s Trust

Persons with Parkinson’s
Individual Advisors

UK Academic Institutions
• University of Oxford
• University of Cambridge
• Newcastle University
• University of Glasgow

Lundbeck · UCB
AbbVie · Pfizer
Merck · GSK
Eli Lilly · Biogen
GE Healthcare

PARKINSON’S
CHANGE ATTITUDES. FIND A CURE. JOIN US.

CRITICAL PATH INSTITUTE
collaborate · innovate · accelerate

U.S. FOOD & DRUG ADMINISTRATION

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH
We can learn from past clinical trials

<table>
<thead>
<tr>
<th>Drug</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>Vitamin E</td>
<td>Antioxidant</td>
</tr>
<tr>
<td>GPI-1485</td>
<td>Neuroimmunophilin</td>
</tr>
<tr>
<td>Riluzole</td>
<td>Glutamate antagonist</td>
</tr>
<tr>
<td>CEP-1347</td>
<td>Anti-apoptotic</td>
</tr>
<tr>
<td>Paliroden</td>
<td>Stimulates NGF</td>
</tr>
<tr>
<td>Co Q10</td>
<td>Mitochondrial enhancer</td>
</tr>
<tr>
<td>Pramipexole</td>
<td>Dopamine agonist</td>
</tr>
<tr>
<td>Cogane</td>
<td>Modulates GDNF &amp; BDNF</td>
</tr>
<tr>
<td>Creatine</td>
<td>Mitochondrial modulator</td>
</tr>
<tr>
<td>Pioglitazone</td>
<td>PPAR-γ agonist; anti-inflammatory</td>
</tr>
<tr>
<td>Rasagline</td>
<td>MAO-B inhibitor</td>
</tr>
<tr>
<td>Glutathione</td>
<td>Antioxidant</td>
</tr>
<tr>
<td>Mitoquinone</td>
<td>Mitochondrial enhancer</td>
</tr>
<tr>
<td>TCH346</td>
<td>Propargylamine</td>
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</table>
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 → 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…

- Early stage
- Late stage

Worsening

Time

Fast worsening

Slow worsening
Using imaging to predict the future

- Early stage
  - Slow worsening
- Late stage
  - Fast worsening

Time
Using imaging as a biomarker in trials endorsed by EMA

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.

Amplion/BIO report, 2016
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