The Future of Virtual Trials
Outline

• Old models
• New tools
• New approaches
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• New approaches
The cost of drug development is rising

Cost per approved drug in billions of 2013 dollars

Productivity within the pharmaceutical industry is declining

New molecular entities per $1 billion in R&D (inflation adjusted), 1950-2010

Clinical trials are inaccessible
Drug development is long, inefficient, and likely to fail
Outline

• Old models
• New tools
• New approaches
New tools can bring care and research to individuals

<table>
<thead>
<tr>
<th>Year of first randomized controlled trial</th>
<th>Number of randomized controlled trials</th>
<th>Banking analogy</th>
</tr>
</thead>
<tbody>
<tr>
<td>1993</td>
<td>653</td>
<td>Teller at a bank</td>
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<tr>
<td>1998</td>
<td>347</td>
<td>ATM at bank</td>
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<tr>
<td>1999</td>
<td>225</td>
<td>Banking at home</td>
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<tr>
<td>2011</td>
<td>50</td>
<td>Mobile banking</td>
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Seven years ago, Craig Lipset and Pfizer conducted the first completely virtual trial

A Clinical Drug Trial Via Phone, Computer

By Jennifer Corbett Dooren
June 7, 2011

Pfizer Inc. is conducting a drug trial in which patients participate from their homes using computers and smartphones rather than visiting a clinic.

The company plans to compare the results to those obtained from a previous, traditional trial of the same drug. The study involves the company’s overactive-bladder drug Detrol.

If successful, the methods used in the study might eventually be used to help drive down the high cost of bringing new medicines to market.

The Food and Drug Administration recently signed off on Pfizer’s study, which is believed to be the first all-electronic, home-based study of a drug to receive agency approval.

Source: Available at http://www.wsj.com/articles/SB10001424052702304432304576369840721708396.
Many parts of a clinical trial can be virtual

Short-term, hybrid model

Current
- Site
- Site
- Site
- Site
- Site
- Site

Future
- Centrally & remotely
- Site
- Remotely
- Site
- Centrally & remotely
New models bring research to participants

Where are the data captured?

- At a Site
- Hybrid
- Remote / Direct-to-Patient

How are the data captured?

- Data entered via an intermediary (e.g., a member of study team)
- Mostly virtual w/ some human intervention (e.g., mobile phlebotomist)
- Fully virtual (e.g., all patient-generated collected via app/device)

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National studies can be conducted from single sites

Map of participants in virtual research study

Participants like virtual visits

Over 80% of participants said they would be more willing and able to participate in future research studies if they could do so remotely.

AT-HOME PD is a virtual longitudinal observational study with three components

STANDY PD III
Phase 3 RCT of Isradipine
N = 336 participants
PI: Tanya Simuni, MD

SURE PD3
Phase 3 RCT of Inodine
N = 298 participants
PI: Michael Schwarzschild, MD, PhD

AT HOME PD
Virtual longitudinal observational study
N = ~420 participants

Virtual visits

Smartphone sessions
23andMe aims to recruit, characterize, and retain a virtual national cohort of LRRK2 carriers

Annual virtual visits

300 LRRK2 Carriers

- 250 without Parkinson disease
- 50 with Parkinson disease

- Questionnaires
- Cognitive testing
- Remote examination
Virtual studies offer numerous advantages

<table>
<thead>
<tr>
<th>Characteristic</th>
<th>Traditional study</th>
<th>Virtual study</th>
</tr>
</thead>
<tbody>
<tr>
<td>Focus</td>
<td>Participants, investigators, and sites</td>
<td>Participants</td>
</tr>
<tr>
<td>Geographic reach</td>
<td>Sites</td>
<td>Internet access</td>
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<tr>
<td>Sites</td>
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<td>Institutional review boards</td>
<td>Many</td>
<td>One</td>
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<tr>
<td>Time to initiate study</td>
<td>Long</td>
<td>Medium</td>
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<td>Investigators</td>
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<td>Assessments</td>
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<td>Variance</td>
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<td>Convenience</td>
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<td>Confidentiality</td>
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<tr>
<td>Cost</td>
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