Clinical Research: Who, What Where, When and Why

Brian D. Berman, MD, MS
September 26, 2015
Overview

- Who
- What (how)
- Where
- When
- Why

“Whenever I walk in a room, everyone ignores me.”
Who participates?
Who participates?

Participants
• Patients*
• Spouses, family members
• Community members

Research team
• Investigators
• Patient advocates
• Industry
• Government
Who pays?

Funding sources

• Industry
• Government
  • NIH (National Institutes of Health)
  • DOD (Department of Defense)
• Foundations
• Private
What is Clinical Research?

- Means research involving people as participants
  - Expand scientific knowledge
  - Find new and/or better ways to prevent, screen for, diagnose, or treat a disease

- Clinical trials are research studies that test how well new medical approaches work in people

- Clinical trials may also compare a new treatment to a treatment that is already available

**WHAT IS A CLINICAL STUDY?**

**Observational Studies**
- Data is collected but there is no attempt to alter the disease progression.
- Provides clinical information about disease progression
- **YOU MIGHT:**
  - Write in a journal
  - Talk with a doctor
  - Keep a food diary
  - Have your blood pressure taken

**Clinical Trials**
- A drug is given or a device is used to alter the course of the disease.
- Evaluates effectiveness of an intervention to slow or stop a disease
- **YOU MIGHT:**
  - Take a pill/drug
  - Have a procedure done
  - Use a device
  - Change your diet

**Example:**
- Consortium for Radiologic Imaging Studies of PKD (CRISP)
- Tolvaptan TEMPO ¾ Trial

Watch a video and learn more at pkdcure.org/clinicalstudies.
What is Clinical Research?

Development
- Planning
  - Research question
  - Research procedures to answer question
- Feasibility
  - Resources
  - Participants
- Budgets/Contracts
- Regulatory issues
  - Institutional Review Boards (IRBs)

Execution
- Start-up
- Approvals
- Staffing/Training
- Recruitment
- Data entry
- Statistical analysis
- Dissemination of results
- Maintenance
  - Reporting
  - Close-out
Clinical Trials in a Nut Shell

- Approved Protocol
- Investigator selection
- Approval Process
- Data Entered and reviewed
- Patient recruitment and participation
- Statistical Analysis
- Data filed and registration obtained
- Presentation and publication of report
Stages of a Clinical Trial

Pre-clinical

Phase I
- Safety & dose

Phase II
- Safety & efficacy

Phase III
- True efficacy

Years
- ~ 5 to 20 study participants

Weeks

Months

Years
What to expect?

- Your health is protected
- You have rights
- Not all trials test drugs
- You’re encouraged to ask questions

Potential perks:
- Taking action against your disease
- Access to treatments not yet available
- Careful and free medical monitoring and supervision

Potential downsides:
- Treatment being tested may not work, have risks or have side effects
- May get placebo
- Requirements may be difficult
Where?
Where?
Where?

ClinicalTrials.gov
When?

WELL...

WHAT YOU WAITING FOR?
Why participate?

• Move science forward
• Improves our understanding of diseases
• Can lead to better diagnostic tests and treatments
• Is necessary to prove the safety and efficacy of new treatments
• All of your current treatments came through clinical trials
Why participate?

• People in clinical trials have better outcomes!
  • Close observation by expert clinicians
  • Placebo works too

• It is a unique gift that you can give to the PD community
CHEAPEST SMALL BEER AVAILABLE

- Oakland Raiders 20oz: $10.75
- San Francisco 49ers 16oz: $10.00
- Chicago Bears 20oz: $9.25
- New Orleans Saints 16oz: $9.00
- Washington Redskins 20oz: $9.00
- St. Louis Rams 20oz: $9.00
- Buffalo Bills 20oz: $9.00
- San Diego Chargers 20oz: $9.00
- Philadelphia Eagles 12oz: $8.50
- Dallas Cowboys 16oz: $8.50
- Baltimore Ravens 16oz: $8.50
- Pittsburgh Steelers 16oz: $8.00
- Green Bay Packers 16oz: $7.75
- Tampa Bay Buccaneers 20oz: $7.75
- Miami Dolphins 20oz: $7.75
- Detroit Lions 16oz: $7.50
- Atlanta Falcons 16oz: $7.50
- New England Patriots 20oz: $7.50
- NFL Stadium Average 16oz: $7.42
  - Arizona Cardinals 12oz: $7.25
  - Kansas City Chiefs 16oz: $7.25
  - Minnesota Vikings 16oz: $7.00
  - Indianapolis Colts 16oz: $7.00
  - Tennessee Titans 16oz: $7.00
  - Denver Broncos 16oz: $6.75
  - Jacksonville Jaguars 16oz: $6.00
  - New York Giants 12oz: $5.00
  - Seattle Seahawks 12oz: $5.00
  - Carolina Panthers 12oz: $5.00
  - New York Jets 12oz: $5.00
  - Cleveland Browns 12oz: $5.00
  - Houston Texans 12oz: $5.00
  - Cincinnati Bengals 14oz: $5.00

Price range: $0.00 to $10.00
THE BIOPHARMACEUTICAL RESEARCH AND DEVELOPMENT PROCESS

From drug discovery through FDA approval, developing a new medicine takes at least 10 years on average and costs an average of $2.6 billion. Less than 12% of the candidate medicines that make it into Phase I clinical trials will be approved by the FDA.


* The average R&D cost required to bring a new, FDA-approved medicine to patients is estimated to be $2.6 billion over the past decade (in 2013 dollars), including the cost of the many potential medicines that do not make it through to FDA approval.

Average Per-Patient Clinical Trial Costs across All Therapeutic Areas

<table>
<thead>
<tr>
<th>Development Stage</th>
<th>Per Patient Costs</th>
<th>n</th>
</tr>
</thead>
<tbody>
<tr>
<td>Phase 1</td>
<td>$21,883</td>
<td>46</td>
</tr>
<tr>
<td>Phase 2</td>
<td>$36,070</td>
<td>55</td>
</tr>
<tr>
<td>Phase 3a</td>
<td>$47,523</td>
<td>38</td>
</tr>
<tr>
<td>Phase 3b</td>
<td>$47,595</td>
<td>40</td>
</tr>
<tr>
<td>Phase 4</td>
<td>$17,042</td>
<td>42</td>
</tr>
</tbody>
</table>
AVERAGE DELAY & TRIAL COST DRIVERS

Clinical Trial Cost Drivers

- Patient Recruitment: 32%
- Vendor Fees: 25%
- Site Recruitment: 14%
- CTMS etc.: 12%
- Site Retention: 8%
- Data and Validation: 7%
- Patient Retention: 2%

Ref.: TrialFacta.com | Published: November 25, 2010
Bar Chart Ref.: Cutting Edge Information
The “Typical” Funnel
Average Industry Benchmark Data

Patients Identified or Available

Overall Conversion Ratio:
Identify 10 Patients to Randomize 1

Pre-Screen Qualified

Consented

Randomized

Pre-Screen
Consent Process
Screening
Drop-Outs

-69%
-58%
-32%
-18%

# Completed Patients

7

Benchmark Data – CPP and PhEsi-1998-2012
Resources

- UCDenvermovement.org
- ClinicalTrials.gov
- ClinicalResearchTrials.NIH.gov
- FoxTrialFinder.MichaelJFox.org
- MyClinicalTrialLocator.com
- Parkinsonrockies.org
- Parkinson.org
Thank you

• Questions?