An Imperative for Action: Patients Are Waiting

Mary Woolley, President, Research!America
Overview of Presentation

- Key challenges
- Congressional & media attention
- What the public says about clinical trials: implications
- Recommendations for Action

“Nothing About Us Without Us”*

*A slogan originating with the South African disability movement in the early 1990’s*
Persistent Challenges in clinical trials

- Recruitment and retention difficulties
- Uncoordinated trial conduct—across federal agencies; across universities; globally
- Expensive, redundant data collection
- Researchers, physicians and patients interests’ not well aligned
- Physicians rarely talk about research
- Failure to include patients every step of the way—from decision to study to report-out

Very little has changed in decades
“Doctors’ recommendations, awareness in the community and association with people who have participated in research were identified by workshop participants as important factors that promote participant enrollment in clinical research...[in addition], many physicians are unaware of available clinical trials.”

Clinical trial researchers ‘tend to disregard the perspective of the community and the public at large.’ (The NIH Director’s Council of Public Representatives in 2005) recommended ‘change in the culture of the scientific community to ensure that medical research is viewed in the context of a long-term commitment to the community, not a one-time research study.’
INTRODUCTION

This chapter examines the public and political contexts in which clinical research takes place, and the role the science community plays in shaping public and policymaker discourse and decision-making. Gaining an understanding of the links between science and the body politic, including the increasing demands for transparency and accountability, is fundamental to the long term success of science.

- Mary Woolley, “Clinical Research in the Public Eye”
Congressional Initiatives on Medical Progress: *Patients First*

- **House:**
  - Passed 21\textsuperscript{st} Century Cures Act (HR 6) in July with bipartisan 344-77 vote
  - HR 6 includes five year Innovation Fund with $8.75B for NIH and $550M for FDA as “mandatory” funding
  - Culmination of year-long Energy & Commerce Committee effort to gather stakeholder input

- **Senate:**
  - HELP Committee is gathering stakeholder input and drafting legislation to be released soon; mandatory funding reportedly included
  - Planning mark up of legislation before end of 2015

- **End Goal:**
  - Both chambers reach a conference agreement that is signed into law ASAP
Clinical trials a major focus of 21st Century Cures Act (HR 6)

- Extends NCATS authority for clinical trials through end of Phase IIB trials (instead of Phase IIA)
  - And extends rare disease exemption through the end of Phase III (instead of Phase IIB)
- Includes “Sense of Congress” statement supporting increased representation of underrepresented communities in clinical trials
- Requires creation of workshop on broadening age groupings in research
- Establishes a pediatric research network
- Streamlines IRB approval for multisite research
- Promotes the design of more targeted clinical trials
- Establishes clinical trial data system to foster collaboration and access to data generated in research and clinical settings
The Public is Paying Attention

“...public sentiment is everything. With public sentiment, nothing can fail; without it nothing can succeed.”

President Abraham Lincoln
"It isn’t uncommon for studies to contradict each other, and there’s no way for clinicians to know which one is right ..."

— The Washington Post, April 15, 2014

"Researchers ... hesitate to share data with potential competitors, both to protect their funding and to insure that they get credit for their work ... ‘the current academic publication system does patients an enormous disservice.’”

— The New Yorker, July 21, 2014
Clinical Trial Recruitment in the News

The New York Times

Clinical Trials Need Cancer Patients

By STAN COLLENDER | JUNE 15, 2015

I HAVE a very rare and aggressive type of skin cancer — Merkel cell carcinoma — for which there is no approved cure, and I'm participating in a clinical trial to deal with it. If successful, the trial will show that the drug I'm being given at least manages what is now an often fatal disease.

REUTERS

More evidence poor cancer patients don’t join clinical trials

BY LISA RAAPORT

Low-income cancer patients are much less likely to participate in clinical trials than their more affluent peers, a U.S. study confirms.

For a Rare Disease, Drug Trials Scramble for Patients

Companies vie for enrollees amid questions that trials will siphon participants away from each other.

THE WALL STREET JOURNAL.
Polls: a Pulse on Public Opinion

- Research!America has commissioned public opinion polls on research issues for 22 years:
  - National Polls
  - State-Based Polls
  - Issue-Specific Polls
- Telephone (random-digit dialing) polls are conducted with a sample size of 800-1000 adults (age 18+) and a maximum theoretical sampling error of +/- 3.5%. Data are demographically representative of adult U.S. residents (state or national)
- Online polls are conducted with a sample size of 1000-2000 adults and sampling error of +/-3.1%. The data are weighted in two stages to ensure accurate representation of the U.S. adult population
Research!America Clinical Trial Poll

For most topics covered, no significant differences observed between general population and over-sampled populations

However,

• Altruism is more likely to be a motivating factor in trial participation among minority groups than in general population
• Minority groups are more likely to admire people who volunteer for clinical trials
• Lack of trust remains an issue among minority groups, slightly greater than the general population
• Minority populations, especially African-Americans, are more likely to say people are enrolled in clinical trials without being told
One kind of medical research is often referred to as a clinical trial. In this, volunteers choose to participate to test the safety and effectiveness of certain treatments, drugs or devices. Have you ever heard of a clinical trial?

Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in May 2013.
Have you or anyone in your family ever participated in clinical trials?
Wide Majority of Americans Have Not Participated in Trials

Have you or anyone in your family ever participated in clinical trials?

- Yes: 16%
- No: 77%
- Not sure: 7%

Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in May 2013.
Most Americans Have Not Participated in Clinical Trials

Have you or anyone in your family ever participated in clinical trials?

<table>
<thead>
<tr>
<th>Ethnicity</th>
<th>Yes</th>
<th>No</th>
<th>Not sure</th>
</tr>
</thead>
<tbody>
<tr>
<td>Hispanic</td>
<td>17</td>
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<tr>
<td>Non-Hispanic White</td>
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<td>6</td>
</tr>
<tr>
<td>African-American</td>
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</tr>
<tr>
<td>Asian</td>
<td>11</td>
<td>82</td>
<td>7</td>
</tr>
</tbody>
</table>

Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in May 2013.
Americans are Interested in Clinical Trials

Do you agree or disagree with each of following statements?

- I am interested in finding out more about taking part in clinical trials
- I would take part in a clinical trial if I was asked by someone I trust

Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in May 2013.
Awareness, Trust, Risk are Barriers to Better Participation

Fewer than 10% of Americans participate in clinical trials. Which of the following do you think is a reason that individuals don’t participate in clinical trials? (multiple responses allowed)

- Not aware/lack of information: 53%
- Lack of trust: 53%
- Too risky: 51%
- Adverse health outcomes: 44%
- Little or no monetary compensation: 35%
- Privacy issues: 27%
- Too much time: 27%
- Not sure: 11%

Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in May 2013.
### Americans Willing to Share Personal Health Data for Research and Patient Care

For which of the following would you be willing to share your personal health information (Choose all that apply)?

<table>
<thead>
<tr>
<th>Reason</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>So health care providers can improve patient care</td>
<td>60%</td>
</tr>
<tr>
<td>To advance medical research</td>
<td>55%</td>
</tr>
<tr>
<td>So public health officials can better track disease and disability</td>
<td>46%</td>
</tr>
<tr>
<td>None</td>
<td>10%</td>
</tr>
<tr>
<td>Not Sure</td>
<td>13%</td>
</tr>
</tbody>
</table>

Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in January 2015.
Opinions Split on Whether Patients are Enrolled Without Their Consent

Would you say that without being told, patients are sometimes included in clinical trials when they are receiving medical treatment?

- Yes: 31%
- No: 41%
- Not sure: 28%

Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in May 2013.
Opinions Split on Whether Patients are Enrolled Without Their Consent

Would you say that without being told, patients are sometimes included in clinical trials when they are receiving medical treatment?

<table>
<thead>
<tr>
<th>Race</th>
<th>Yes</th>
<th>No</th>
<th>Not sure</th>
</tr>
</thead>
<tbody>
<tr>
<td>African-American</td>
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<td>30</td>
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<tr>
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<td>39</td>
<td>24</td>
</tr>
<tr>
<td>Asian</td>
<td>35</td>
<td>36</td>
<td>29</td>
</tr>
<tr>
<td>Non-Hispanic White</td>
<td>27</td>
<td>44</td>
<td>29</td>
</tr>
</tbody>
</table>

Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in May 2013.
Seven in 10 Say Doctors Don’t Talk About Medical Research

Has your doctor or other health care professional ever talked to you about medical research?

- 70% No
- 22% Yes
- 8% Not sure

Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in May 2013.
If your doctor found a clinical trial for you and recommended you join, how likely would you be to participate in a clinical trial?

- Very likely: 26%
- Somewhat likely: 13%
- Not likely: 12%
- Would not participate: 46%
- Not sure: 3%

Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in May 2013.
Which organizations listed below would you say has the greatest responsibility in educating the public about clinical trials?

<table>
<thead>
<tr>
<th></th>
<th>Doctors and other health care providers</th>
<th>Government</th>
<th>Insurance companies</th>
<th>Pharmaceutical companies</th>
<th>Patient organizations</th>
<th>Not sure</th>
</tr>
</thead>
<tbody>
<tr>
<td>African-American</td>
<td>33</td>
<td>29</td>
<td>5</td>
<td>13</td>
<td>5</td>
<td>16</td>
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<tr>
<td>Asian</td>
<td>36</td>
<td>28</td>
<td>6</td>
<td>13</td>
<td>5</td>
<td>13</td>
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<tr>
<td>Hispanic</td>
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<td>24</td>
<td>5</td>
<td>12</td>
<td>5</td>
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<tr>
<td>Non-Hispanic White</td>
<td>42</td>
<td>15</td>
<td>5</td>
<td>17</td>
<td>5</td>
<td>17</td>
</tr>
</tbody>
</table>

Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in May 2013.
Why are Physicians not talking more about research?

- Don’t have time
- Aren’t aware of trials
- Aren’t being asked
- Don’t know how
- Fear of losing the patients
- Lack of incentives
Improving Others’ Health
Important for Participation

How important would the opportunity to improve the health of others be in your decision to participate as a volunteer in a clinical trial?

- **African-American**: 62 (Very important), 24 (Somewhat important), 7 (Not very important), 5 (Not at all important), 7 (Not sure)
- **Asian**: 50 (Very important), 38 (Somewhat important), 5 (Not very important), 7 (Not at all important), 7 (Not sure)
- **Hispanic**: 57 (Very important), 30 (Somewhat important), 6 (Not very important), 6 (Not at all important), 6 (Not sure)
- **Non-Hispanic White**: 49 (Very important), 38 (Somewhat important), 7 (Not very important), 7 (Not at all important), 6 (Not sure)

Source: A Research-America poll of U.S. adults conducted in partnership with Zogby Analytics in May 2013.
Americans Admire Clinical Trial Volunteers

On a scale of 1 to 4, how much do you admire people who volunteer for clinical trials?

<table>
<thead>
<tr>
<th>Ethnicity</th>
<th>4 (a great deal)</th>
<th>3</th>
<th>2</th>
<th>1 (not at all)</th>
<th>Not sure</th>
</tr>
</thead>
<tbody>
<tr>
<td>African-American</td>
<td>46</td>
<td>31</td>
<td>8</td>
<td>3</td>
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<td>42</td>
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<td>3</td>
<td>11</td>
</tr>
</tbody>
</table>

Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in May 2013.
Clinical Trials are as Valuable as Giving Blood

Do you agree or disagree with the following statement. Taking part in clinical trials is as valuable to our health care system as giving blood.

- **21%** Strongly agree
- **46%** Somewhat agree
- **16%** Somewhat disagree
- **11%** Strongly disagree
- **7%** Not sure

Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in May 2013.
Americans Admire Organ Donors

How much do you admire the following groups of people on a scale of 1 to 4?

- People who donate an organ:
  - 4 (a great deal): 69
  - 3: 19
  - 2: 3
  - 1 (not at all): 7
  - Not sure: 7

- People who give blood:
  - 4: 61
  - 3: 26
  - 2: 43
  - 1: 7
  - Not sure: 7

- People who volunteer for clinical trials:
  - 4: 37
  - 3: 38
  - 2: 9
  - 1: 4
  - Not sure: 13

Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in May 2013.
BHAG*: Make volunteering for a clinical trial as valued as donating blood, organs or tissue.

Make regular participation in clinical research a new social norm and a routine ‘health behavior.’

**”Big, hairy, audacious goal” - Collins, J. & Porras J. (2004). Built to Last: Successful Habits of Visionary Companies.**
Driver’s License Organ Donor Program: advocates made it happen!

- In 1969, as a result of advocacy by physicians, patients, and the business community, the Tennessee Legislature passed the Anatomical Gift Act, which made it possible to donate organs.
- In 1973, Tennessee becomes first state to list organ donation as an option on a driver’s license; other states followed.
- By 2014, through the work of the donation and transplantation community in partnership with the DMV, 50 percent of the U.S. adult population, or 125 million people, were registered organ, eye, and tissue donors.

Action Recommendations (1)

- Standardize and harmonize regulations: within US and globally
- End practice of every institution having unique consent form
- Learn from other nations, e.g. UK success in doubling cancer trial enrollment
- Share more data faster—across agencies, across the research ecosystem, with patients. PCORnet provides opportunity.
- Increase reimbursements to physicians for talking about research
Action Recommendations (2)

- Use new technology and social media to improve two-way communication:
  - ‘bring clinical trials to patients, instead of patients to clinical trials’*
- Everyone involved in the conduct of research should look for opportunities to participate in research as a volunteer themselves—experience can be a great teacher, and you will be more credible, too
- Use knowledge of concerns of special populations to design better recruitment and retention
- Engage patients every step of the way!

*Corsee Sanders, Ph.D. SVP, Global Head of Development Innovation & Clinical Operations, Genentech
Patient Engagement is the most important component of success!

“Gone are the days when we could just say, ‘We’re a cloistered community of researchers, and we alone know how to do this.’”

Examples of Clinical Trial Campaigns

Patient Clinical Trial “Champions”

Patient Perspectives Video Series
“Kathryn Schmitz, an epidemiologist at University of Pennsylvania and an investigator on the Share the Journey study, said it recently took her team three years, including the sending of 60,000 notices, to recruit just 351 patients for a separate conventional study about the impact of exercise on breast-cancer survivors. In the first month of recruiting for Share the Journey—which she said has less stringent enrollment criteria—nearly 2,000 patients have signed up.”
Important for Scientists to Engage with Public on Research

How important is it for scientists to inform elected officials and the public about their research and its impact on society?

Source: A Research!America and ScienceDebate.org poll of U.S. adults conducted in partnership with Zogby Analytics in September 2015.
Remember the most important four words a researcher can say and convey:
“I work for you.”