Clinical Trial Data Sharing: Perspectives From Participants and PCORI

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Study Team

Stanford Project Team

Michelle Mello
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SPRC Trial Teams

Indiana University CTSI Trial Teams

Tufts Trial Teams

Greenwall Foundation
Context

- Broad movement toward participant-level data sharing
- Concerns about privacy, informed consent, and system governance
- Little is known about trial participants’ views
- Trial sponsors and investigators often invoke participants’ interests as a reason not to share, or to limit what is shared
Study questions:

1. How do trial participants perceive the balance of risks and benefits from data sharing?

2. How great a concern are privacy intrusions and breaches?
Survey Methods

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- Up to 2 reminders mailed to nonresponders
- Data were hand-entered into REDCap and audited for accuracy
## Sample Characteristics

<table>
<thead>
<tr>
<th></th>
<th>n</th>
<th>Completion Rate (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Full sample</strong></td>
<td>771</td>
<td>79.2</td>
</tr>
<tr>
<td>Mail survey</td>
<td>350</td>
<td>64.0</td>
</tr>
<tr>
<td>In-clinic survey</td>
<td>421</td>
<td>98.0</td>
</tr>
</tbody>
</table>
## Topics of Participants’ Trials

<table>
<thead>
<tr>
<th>Health Issue</th>
<th>%</th>
</tr>
</thead>
<tbody>
<tr>
<td>Nutrition/weight/vitamins</td>
<td>22.3</td>
</tr>
<tr>
<td>Diabetes</td>
<td>22.3</td>
</tr>
<tr>
<td>Cardiovascular</td>
<td>9.2</td>
</tr>
<tr>
<td>Aging/ neurodegenerative disease/ memory</td>
<td>8.3</td>
</tr>
<tr>
<td>Tobacco use</td>
<td>6.7</td>
</tr>
<tr>
<td>Liver disease</td>
<td>6.4</td>
</tr>
<tr>
<td>Mental illness</td>
<td>5.3</td>
</tr>
<tr>
<td>Cancer</td>
<td>5.1</td>
</tr>
<tr>
<td>Kidney disease</td>
<td>3.4</td>
</tr>
<tr>
<td>Lung disease</td>
<td>2.9</td>
</tr>
<tr>
<td>Alcohol use</td>
<td>2.2</td>
</tr>
<tr>
<td>Bone disease</td>
<td>1.7</td>
</tr>
<tr>
<td>Other</td>
<td>3.7</td>
</tr>
<tr>
<td>Demographic Snapshot</td>
<td>%</td>
</tr>
<tr>
<td>-------------------------------------------------------------------------------------</td>
<td>-----</td>
</tr>
<tr>
<td>Fair or poor self-reported health status</td>
<td>22.3</td>
</tr>
<tr>
<td>Female</td>
<td>49.9</td>
</tr>
<tr>
<td>Age: &lt;25</td>
<td></td>
</tr>
<tr>
<td>25-44</td>
<td>8.3</td>
</tr>
<tr>
<td>65+</td>
<td>60.7</td>
</tr>
<tr>
<td>31.0</td>
<td></td>
</tr>
<tr>
<td>Hispanic</td>
<td>13.3</td>
</tr>
<tr>
<td>Race: White</td>
<td></td>
</tr>
<tr>
<td>Black</td>
<td>67.2</td>
</tr>
<tr>
<td>Black</td>
<td>14.7</td>
</tr>
<tr>
<td>Native American</td>
<td>6.6</td>
</tr>
<tr>
<td>Asian</td>
<td>3.2</td>
</tr>
<tr>
<td>Education: No college</td>
<td></td>
</tr>
<tr>
<td>College graduate</td>
<td>21.9</td>
</tr>
<tr>
<td>College graduate</td>
<td>50.7</td>
</tr>
<tr>
<td>Household income: &lt;$25,000</td>
<td></td>
</tr>
<tr>
<td>&gt; National median ($55,000)</td>
<td>23.3</td>
</tr>
<tr>
<td></td>
<td>49.0</td>
</tr>
<tr>
<td>Ever had personal information stolen or breached</td>
<td>45.7</td>
</tr>
</tbody>
</table>

Percentages may not sum to 100 due to rounding, omitted categories, or blank responses.
### Clinical Trial Participation

<table>
<thead>
<tr>
<th>Who participated in the trial?</th>
<th>%</th>
</tr>
</thead>
<tbody>
<tr>
<td>Me only</td>
<td>90.2</td>
</tr>
<tr>
<td>My child only</td>
<td>7.1</td>
</tr>
<tr>
<td>Other person only</td>
<td>1.6</td>
</tr>
<tr>
<td>Me plus someone else</td>
<td>0.7</td>
</tr>
<tr>
<td>Unsure</td>
<td>0.5</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>In last 2 years, participated in a trial as:</th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>A person with the health condition being studied</td>
<td>41.5</td>
</tr>
<tr>
<td>A healthy volunteer or a person at risk for developing the health condition being studied</td>
<td>55.1</td>
</tr>
<tr>
<td>Both</td>
<td>3.4</td>
</tr>
</tbody>
</table>
Thinking about the most recent clinical trial you/your child participated in, what was the most important reason you decided to be in the study?

- I thought there was a chance I / my child might get a health benefit
- I wanted to help others
- I valued the chance to make some money
- Some other reason
Overall, how would you describe your / your child’s experience(s) as a clinical trial participant?

- Very negative
- Somewhat negative
- Neither positive nor negative
- Somewhat positive
- Very positive
Overall, how do you think the potential benefits of sharing anonymous, individual clinical trial data weigh against the potential negative consequences?
Predictors: Negative Aspects of Data Sharing Outweigh Benefits

- Few significant predictors, but low proportion with negative views could have made subgroup differences hard to detect.

- More likely to feel that negatives outweigh benefits if:
  - Concerned about risk of reidentification (OR=2.9)
  - Concerned about risk of information theft (OR=2.6)

- Those with a college degree less likely to feel this way (OR=0.22)
How much do you think the following groups could benefit from sharing anonymous, individual clinical trial data?

- Doctors taking care of patients
- Companies developing medical products, such as prescription drugs
- Patients
- Scientists in universities and other not-for-profit organizations

Options:
- A great deal
- A lot
- A moderate amount
- A little
- Not at all
How likely would you be to allow your *anonymous, individual* clinical trial data to be shared with...

- Very likely
- Somewhat likely
- Neither likely nor unlikely
- Somewhat unlikely
- Very unlikely

scientists in companies developing medical products, such as prescription drugs?

scientists in universities and other not-for-profit organizations?
How much do you trust...

- doctors?
- health insurance companies?
- government agencies that fund medical research?
- drug companies?
- universities?
Predictors of Being Unlikely to Share Own Data

- With drug company scientists:
  - Feeling that other people generally can’t be trusted (OR=3.5)
  - Having low trust in drug companies (OR=3.1)

- With scientists in not-for-profit settings:
  - Feeling that other people generally can’t be trusted (OR=7.0)
  - Those with a college degree were less likely to be unwilling to share (OR=0.24)
Does the Type of Use Matter?

How likely would you be to allow your anonymous, individual clinical trial data to be used in the following ways?

- To help scientists check the accuracy of research results announced by other scientists or companies (by re-doing the analyses)?
- To help patients and groups of patients learn more about health problems that affect them.
- To do research that will help others.
- To help get answers to scientific questions faster using information that others have already gathered.
- To do research on health problems that affect my family or me.
- To help patients and groups of patients learn more about health problems that affect them.
- To help scientists check the accuracy of research results announced by other scientists or companies (by re-doing the analyses)?
How much do you think sharing anonymous, individual clinical trial data can...

- make sure people’s participation in clinical trials leads to the most scientific benefit possible?
- help lawyers prove their case in lawsuits claiming that medical products are unsafe?
- discourage scientists and companies from hiding or distorting their clinical trial results (by making it possible for others to...)
- support learning about diseases that only a small number of people have (by combining data from many clinical trials)?
- help scientists check the accuracy of research results announced by other scientists or companies (by re-doing the...)
- help patients and groups of patients learn more about health problems that affect them?
- lower the cost of developing new medical products?
- help ensure that research dollars are spent as wisely as possible?
- help get answers to scientific questions faster using information that others have already gathered?
How concerned are you about the following potential consequences of sharing anonymous, individual clinical trial data?

- Scientists and companies might have less incentive to invest time and money in doing clinical trials
- Scientists or companies could unfairly “free ride” on the work of others
- Companies might use the information for marketing purposes instead of scientific purposes
- My information might be stolen
- It could be harder to get people to agree to be in clinical trials if they know their data will be shared
- Some person or company could make a lot of money developing products using my information
- My information might be used in scientific projects that I wouldn’t approve of
- People might use the data to do poor-quality science
- It could be embarrassing if the information was linked back to me
- I could be discriminated against if the information was linked back to me
- Someone who is good with computers could identify me
Limitations

- Possible nonresponse bias
- Comprehension problems?
- Sicker participants might have different views
What if specific consent for data sharing wasn’t obtained?

Do participants prefer to be asked for consent or merely informed of data sharing?

Is blanket consent for future uses acceptable?

Should data sharing systems be open access?
Key Takeaways

- Participants see the benefits of data sharing as greatly outweighing the risks.
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- High willingness to share data, even where no prospect of benefit to self/family.
  - See benefits in terms of future clinical care, as well as scientific discovery.
Key Takeaways

- Low concern about companies’ use of data, despite low trust
  - Lawyers are a different story!
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- Proportion concerned about risks is surprisingly low (25% somewhat, 10% very).
Strategies for Trialists

- Ensure that trial participation is a positive experience
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- Don’t be deterred by privacy anxieties
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- Ensure that trial participation is a positive experience
- Don’t be deterred by privacy anxieties
- Frame the ask with specific information about the benefits of data sharing and the scientific cost of opt outs
- Stress your desire to make the most of their contribution to science
PCORI Data Sharing Policy: Current status

Steven Goodman, MD, PhD
Professor and Associate Dean
Stanford University School of Medicine
Vice-chair, PCORI Methodology Committee
Policy for Data Access & Data Sharing: Background

- **PCORI is committed to open science.**
  - To allow reproduction of original analyses
  - To enable conduct of additional analyses

- **Policy developed with input from expert advisory group and Research Transformation Committee (RTC)**
  - Informed by public comment period and a pilot project involving data repositories and PCORI-funded researchers
  - Informed by other funders/regulators of clinical research, including Gates Foundation, European Medicines Agency, and NIH.

- **Designed to evolve, based on future experience.**
VALUES STATEMENT: The Patient-Centered Outcomes Research Institute (PCORI) is committed to the principles of open science, particularly maximizing the utility and usability of data collected in research projects that PCORI funds. PCORI seeks to encourage scientifically rigorous secondary use of clinical research data to foster advances that will ultimately improve clinical care and patient outcomes. As such, PCORI believes it is important for our research awardees to systematically create and preserve research data and data documentation in order to facilitate data sharing.
Data Sharing Pilot Project

PCORI Awardees:
- 5 awardees participated: 3 completed studies and 2 ongoing large, pragmatic studies.
- Awardees were selected to represent a diversity of therapeutic areas, study designs (both observational and RCTs) and data sources (EHR, claims data, imaging data).

Data Repositories:
- Multi-Regional Clinical Trials (MRCT) Center, Brigham and Women’s Hospital
- ICPSR at the University of Michigan
Data Sharing Pilot Project (2)

**MRCT:**
- Focused on governance issues and documents – Data Use Agreement (DUA), Data Contributor Agreement (DCA), Informed Consent Forms (ICF)

**ICPSR:**
- Worked with 4 awardees to archive data of varying types
- Documented the experience to enable PCORI to plan for broader data sharing activities
- Created a demonstration repository for PCORI, initially for internal review and use
Data Sharing Pilot Project: Lessons Learned, I

- **Data Governance**
  - Variability in understanding of data package and data sharing terms
  - DCA, DUA and ICF are valuable documents for setting expectations
  - PCORI awardees desire recognition when data used in secondary research

- **Lessons for Data Submission**
  - Preparing data for submission takes time and expertise
  - Curation and review of submitted data is critical
  - Target date for releasing data (embargo) is needed
Implementation of data sharing for clinical research requires careful deliberation about the details:

– Cannot direct researchers to deposit their data “somewhere” and declare victory
– Data curation and ability of repository to work with researchers is critical
– It’s not just the dataset... it’s the whole data package
– Data **discoverability** & **intelligibility** are the name of the game
Considerations for Informed Consent

- Provide explanations of “your” data, coded data, de-identified data and anonymized data
- Provisions to respect participant autonomy and privacy
- Minimal but non-zero risk of re-identification
- Minimal but non-zero risk of privacy breach
- Opt-out provision for secondary use of collected data
Final policy will address:

• Description of the full data package (full analyzable dataset, study protocol, metadata, data dictionary, full statistical analysis plan, and analytic code)
• PCORI commitment to provide funds for the time/effort of investigators to prepare their data for sharing
• Terms of data deposition and data availability
• Informed consent
• Terms of the data request process, as well as review of requests and requestor qualifications
• Applicability of the policy to studies using EHR and other health systems data
Thank you!
Questions?