

# Data Sharing: A Patient's Call to Action

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#### **Introduction and Background**

As a person in Cancer Research, I never even considered Parkinson's (PD) as a possibility. Everyone in my family died of Cancer. But, there I was in 2009 in my doctor's office getting a diagnosis of a chronic and progressive disease, a diagnosis that was devastating as much for me as my family. After a few years of denial, I knew I needed to do something and believed that my experience as both a patient and as someone in bio-medical research (at NIH, UCSF, Bristol-Myers and Sanofi/Genzyme) could be put to good use so that other families to have to suffer like mine.

I am a big believer in the power of science. There is no calling greater than helping people who can't care for themselves. We can't be satisfied that things are okay. We need major change in the way we do research and how patients and providers interact with the healthcare system. While recently there have been more attempts to share data, the data made available to others is often not in a form that is really useful, is stale, is restricted to a small community of researchers and/or it is very difficult and, at times, nearly impossible to combine that data with other data since existing data standards and terminology were not used. Furthermore, the current model of care and research is based on tools developed in the time of Guttenberg (1440). If we continue to adhere to this model, our understanding of clinical interventions and our ability to develop new treatments will continue to be limited.

In the era of electronic knowledge exchange, only when data sharing becomes the norm, can we derive its full benefits. Change is challenging, but

#### **One Patient's View**

As a researcher turned patient, I understand the importance of clinical research data and results. I want my information to be shared for the 'greater' good'. While I certainly have concerns about use and re-use of my data, these can be alleviated by responsible sharing of data that protects patient privacy and security; by incentives for researchers to produce and ensure high quality data for sharing with peers, the broader scientific community and the public; by increased data circulation and by data sharing encouraged or mandated by government. Best practices to ensure better transparency and to enable reproducibility of results in creation of datasets include:

- Use of common, shared data definitions and structured data capture at the point of care so we have quality data that is actually re-usable and shareable.
- Good, clear documentation so it is easier for others to understand data content and encourage collaboration.
- Location and access to the data to make it easier to share.
- Making the data easily discoverable through advertising and easy access.

#### **Some Early Examples of Parkinson's Data Sharing**

change we must. What is true for any scientific inquiry is true for improving healthcare: the better the data, the more meaningful the results. The time is now for clinical care, research, and scientific discovery to be connected in a seamless continuum that speeds innovation and benefits patients.

#### Scope

The data that needs to be shared comes from multiple sources including clinical trials, insurance claims, Electronic Health Records (EHRs), Patient Reported Outcomes, Web-based Patient "Social" Communities (such as, Patients Like Me), Registries, wearables, devices and other observational and study datasets. This includes datasets specifically created for Parkinson's research as well as those datasets that contain valuable Parkinson's patient information although they were not created for that purpose (such as insurance claims systems or the Veteran's Administration Million Vets Program knowledgebase).

For the purposes of this poster, I want to focus on patient data from Clinical Trials. While recently there have been more attempts to share clinical trial data, the data made available to others is often not in a form that is really useful, is stale and is restricted to a small community of researchers. In addition, it is very difficult and, at times, nearly impossible to combine that data with other data since existing data standards were not used. All of this limits the value of data.

#### **The Problem**

Today in Parkinson's research, the sharing of patient data has been very limited in scope. Much of the existing data is almost exclusively considered the property of those that paid for the development of a dataset. This means that others often don't get access to the data at all or, if they do, they get access many years after the data was collected.

The current model is based on paper-based tools developed in the time of Guttenberg (1440). If we continue to adhere to this model, our understanding of clinical interventions and our ability to develop new treatments will continue to be limited. In clinical research, the impact is felt in the following ways:

- A select number of individuals often decide which analyses to conduct, choosing some at the exclusion of others. An analysis that might have been of great interest to another investigator (and which may have a direct bearing on clinical practice) may not be performed.
- Among the many findings generated, only a select number might be included in any peer-reviewed publication. The research community and clinicians may never know about findings generated, but not disseminated.
- Among all trials conducted, there may be significant publication delays so the knowledge gained in the research may not be known to other investigators for many years.
- Only a limited number of trials are eventually published. The 'failed' studies are often archived and never to be used again. Don't we learn as much or more from our failures as our successes?

In Parkinson's and other areas of neurological research there are several prominent examples of data sharing currently underway that are illustrative and can inform our expectations for open scientific and data exchange.

- Critical Path for Parkinson's Consortium (CPP) is focused on sharing precompetitive patient data from the control arms of legacy clinical trials and implementing consensus data standards.
- Parkinson's UK has been a leader in bringing together many participants in the Parkinson's research enterprise to find ways to share data and work collaboratively.
- The National Institute of Neurological Disorders and Stroke (NINDS), FDA, the academic community, Transcelerate Biopharma and the Clinical Data Interchange Standards Consortium (CDISC) have invested in development of clinical data standards for Parkinson's research, a necessary step in improving combinability of data.

	Where We Are Today	Where We Need to Be
Accuracy	<ul> <li>Compromising copy-and-paste dependence and multiple data storage locations perpetuate inaccurate, outdated data.</li> </ul>	<ul> <li>Automated quality checks and verification requirements ensure that data is always accurate and up-to-date.</li> </ul>
Accessibility	<ul> <li>Key clinical data live in multiple places and is difficult to find.</li> <li>Data is silo-ed and cannot be shared with other systems for both care and research.</li> </ul>	<ul> <li>Mission critical data is central, succinct: one click away.</li> <li>Standardized and consistent data capture allows information to be transmitted across multiple platforms.</li> </ul>
Efficiency	<ul> <li>Data is entered and abstracted many times; work is duplicated.</li> <li>Cumbersome, time-consuming data entry means less quality time with patients.</li> <li>User interface hampers rather than facilitates clinical care and research workflow.</li> </ul>	<ul> <li>Data is entered correctly once and persists throughout the patient's care, only needing occasional updates.</li> <li>One-click clinical notes and treatment summaries.</li> <li>User-centered design supports workflow rather than hindering it.</li> </ul>
Re-usability	<ul> <li>After collection, data cannot be used in any meaningful, clinically-relevant way.</li> </ul>	<ul> <li>Captured data can inform care decisions and drive clinical trials, registries, referrals, etc.</li> </ul>
User Experience	<ul> <li>Unfriendly design and usage complexity lead to frustration, low compliance, and decreased completion.</li> <li>Model of ownership does not support effective collaboration.</li> </ul>	<ul> <li>Intuitive and user-centered design boosts ease of use and completion rate.</li> <li>Information model facilitates a collaborative, "team science" approach to medicine.</li> </ul>

#### **Key Benefits of Data Sharing**

Data sharing, especially for Parkinson's where there are no effective treatments, is critical. Science is a community, continually building on each other's ideas. In the era of electronic knowledge exchange, only when data sharing becomes the norm, can we derive its full benefits, including:

The cumulative effect is that patients, neurologists, other healthcare professionals and the research community are placed in the position of making critical decisions with access to only a fraction of the relevant clinical evidence that might otherwise be available. When neurologists recommend treatment options to Parkinson's patients, this is routinely done on the basis of information that is biased and seriously incomplete. This standard of practice is tolerated because we are accustomed to it. The care delivery and research communities often only become aware of a treatment's shortcomings when safety concerns are raised about a drug, device, or other treatment strategy.

#### The Investigator's Predicament

The principal concern, voiced by investigators, is that a substantial amount of individual time and effort has been invested to design the trial and collect the data and that, in return, they argue that they deserve ample opportunity to conduct their analyses and disseminate their findings.

As a researcher, I understand the concern of investigators of being "beaten to the punch" with their own data. I also understand the concern that multiple analyses by various independent research groups may produce differing results, either because of human error or because external investigators conduct inappropriate analyses; that clinical trials are designed with pre-specified study protocols and that additional analyses amount to Medical science is essentially an enterprise conducted for moral reasons. I ask that investigators weigh the time and effort they have invested (often with government/taxpayer funds) against the value of earlier data sharing, if for no other reason than to avoid minimize duplicate effort.

The scientific community must reach a consensus about several critical points before the promise of sharing clinical research data can be fully realized. These include:

- What are the responsibilities of the original investigator team? To share data effectively, they must produce a clean, well-described, and accurate data file that can be used by others and protects patient confidentiality.
- Who supports the investigators' efforts to create these data sources?
- What if there are subsequent questions and inquiries? Who bears responsibility for the shared data?

#### **Patient Privacy and Security**

Another broad issue is the question of who should be allowed to access the data and how access should be provided. The scientific community began the process of developing standards and solutions to these data sharing problems as early as 2009 when The Institute of Medicine (IOM) set forth recommendations on managing research data. The Wellcome Trust convened a number of research funders to develop a coherent vision, principles and goals to promote the sharing of research data to improve public health in 2012. Journal editors have developed guidance for the preparation of raw clinical data for publication.

- Increased knowledge turns through integration of research and care delivery, to help create a true learning health care system so that evidence is available when and where it is needed, resulting in more effective and more efficient research and care delivery.
- More connection and collaboration between patients, researchers, industry and government, which can result in important new findings within the field.
- Ability to leverage the data and build upon the work of others rather than repeating already existing research.
- More widely disseminated information for more informed decision-making for planning and policy.
- Increased open science and information exchange through data sharing in order to further the value of all R&D. It is in the public's interest to have access to comprehensive clinical trial data to ensure a complete understanding of drug or device safety and effectiveness.

#### **Call to Action**

It is clear that increased sharing of patient-level data holds promise for scientific advancement in Parkinson's and other research but we need to overcome the barriers that limit data sharing today. This is a call to action. Parkinson's patients and their advocates should insist and expect that:

- Government continues to mandate guidelines to ensure responsible sharing of clinical trial data that balances the interests of stakeholders with the public demand for information on the effectiveness and safety of therapies.
- All stakeholders, from biopharma to medical researchers to medical journals and regulatory bodies understand the importance of data sharing from a patient perspective and make data sharing the norm rather than the exception.
- Data sharing plans are mandated by government to be a part of all clinical research protocols including detail on what data will be available to others for the purpose of advancing science.
- Clinical research data are made available for sharing <u>much more quickly</u> than they are today.
- Groups like the Critical Path for Parkinson's Consortium (CPP) create new information technology platforms to support data sharing. 21<sup>st</sup> Century problems require 21<sup>st</sup> Century solutions. Leverage solutions that have been used in other therapeutic areas to speed implementation of new drugs and devices. Examples from Cancer include UCSF's OneSource effort. See the figure above.
- Government and industry implement strategies to encourage use of clinical data standards and development of incentives for data sharing, both "carrot" and "stick".

While data sharing of clinical trial data is not a panacea, it is an important piece of the puzzle to bring new safe and effective products to market much faster. For those who question, "Why now?", I say "Why not sooner?". I and patients like me can't wait 10-20 years for new drugs or devices to be approved. The time is NOW!



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Featured this month in the Journal of Parkinson's Disease open access website.

"Call to action: Sharing of patient-level data for Parkinson's research and care"

http://www.journalofparkinsonsdisease.com/call-action-sharing-patient-leveldata-parkinson%E2%80%99s-research-and-care



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