MILKEN INSTITUTE

Advancing Models of Patient Engagement: Patient Organizations as Research and Data Partners

PART II: FOR PATIENT ORGANIZATIONS

About the Milken Institute

The Milken Institute is a nonprofit, nonpartisan think tank.

For the past three decades, the Milken Institute has served as a catalyst for practical, scalable solutions to global challenges by connecting human, financial, and educational resources to those who need them. Guided by a conviction that the best ideas, under-resourced, cannot succeed, we conduct research and analysis and convene top experts, innovators, and influencers from different backgrounds and competing viewpoints. We leverage this expertise and insight to construct programs and policy initiatives.

These activities are designed to help people build meaningful lives, in which they can experience health and well-being, pursue effective education and gainful employment, and access the resources required to create ever-expanding opportunities for themselves and their broader communities.

About FasterCures

FasterCures, a center of the Milken Institute, is working to build a system that is effective, efficient, and driven by a clear vision: patient needs above all else. We believe that transformative and life-saving science should be fully realized and deliver better treatments to the people who need them.

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Introduction

In Part I of this series, we highlighted the following trends that are reshaping biomedical innovation:

- Clinical innovation is rapidly becoming more patient-centric and data-rich.
- An explosion of data has opened new opportunities to capture a fuller patient experience.
- Infrastructure for sharing, aggregating, and analyzing data from a variety of sources is growing.
- Patient-generated health data (PGHD) are generally not readily available in the environment of shared data networks—but, ideally, they should be.

FasterCures believes that a well-functioning research infrastructure requires the contributions of well-resourced, high-functioning patient communities. Part II of this series is intended to provide patient organizations that fund and engage in medical research with insight into and guidance related to their role as critical partners in this ecosystem, particularly as trusted intermediaries for the collection and aggregation of PGHD.

KEY TAKEAWAYS FOR PATIENT ORGANIZATIONS

I. Patient organizations are vital actors in the emerging patient-centered medical research and innovation system as intermediaries for patient perspective and participation as well as patient data.

II. Patient organizations can use the tools and guidance in this document to improve their capacity as a research partner.

III. Patient organizations can be critical sources of patient-generated health data that are increasingly sought by other stakeholders to complement electronic health record and claims data and to capture a fuller patient experience in the "real world."

IV. Patient organizations should become aware of, and consider how to plug into, shared data networks, which are a growing research infrastructure. The potential benefits of these networks include achieving the promise of "big data" and increased research speed and efficiency.

I. Patient organizations are vital actors

In this era of strong interest in engaging patients as partners across the continuum of research, development, and care, more patient organizations are partnering with other stakeholders to bring patients' input—their perspectives and priorities, as well as their health data—to biomedical research and development (R&D). FasterCures surveyed patient organizations in its network in the fall of 2018,[1] receiving responses from 78, and we heard from them that they are collaborating for this purpose in large numbers with academic/health-care institutions, other patient organizations, for-profit companies, and government agencies (though very few with payers). These patient organizations have provided:

- Feedback on the relevance of research questions to patients;
- Assistance with trial recruitment;
- Input on clinical trial design, eligibility, endpoints, and consent;
- Information to regulators and/or payers about patients' experiences and preferences; and
- Perspective on benefit-risk or patient preference studies.

In late 2018, the Food and Drug Administration (FDA) approved an inhaled levodopa powder to treat "off" episodes in people with Parkinson's disease (PD). The Michael J. Fox Foundation (MJFF) provided "de-risking" funding for early clinical trials of the therapy, the first to reach market approval. MJFF's decision to fund this and other industry and academic projects aimed at alleviating "off time" was based on patient reports through a large-scale survey that such alleviation is a significant unmet need for their quality of life. MJFF also engages with industry and government partners in a rigorous study of patients' benefit-risk preferences regarding devices used to treat PD, with the aim of including those preferences in clinical trial criteria.

Patient organizations are increasingly sophisticated in how they engage in these partnerships as well. The majority of our questionnaire respondents require or have signed formal agreements with partners (e.g., memoranda of understanding (MOU), master services agreements, non-disclosure agreements, data use agreements), and/or have guiding principles for such partnerships. (Surprisingly, fewer than half say they have a conflict of interest policy for partnering with industry.) A third have received compensation for their assistance.

The Arthritis Foundation is setting the pace on partnership, developing standard MOUs to use when engaging with industry partners. The goal of each MOU is to ensure that all participants—whether an individual patient, industry partner, or the foundation itself—understand the goals, expectations, and unique considerations that shape each relationship.

1. See Part I for a description of the inputs to this series.

II. Patient organizations can improve their capacity as research partners

Part III of this series cites several helpful resources that researchers can use to identify organizations with deep connections to patient communities of interest. Patient organizations can also use these resources to define and characterize the assets and capabilities that they bring to the R&D process.[2] As the number of patient organizations interested in engaging in the R&D process as more strategic partners is on the rise, there is a need for a more holistic way for patient groups and their potential partners to evaluate where they are on the continuum of research readiness and engagement, and what's needed to advance along it.

We recognize that every organization is unique, responding to differing conditions and needs in their fields of interest and working with differing amounts of resources. No single pathway to success or list of required ingredients exists; we do not want to rate or make judgments about the quality of individual organizations.

We believe that a maturity model,[3] defined as "a measurement of the ability of an organization for continuous improvement in a particular discipline,"[4] is a useful construct for this exercise. Assessments of maturity typically look at **people, processes, and tools** across several critical dimensions. We propose that organizations seeking to add distinctive value to the R&D process to serve the needs of the patients they represent should consider the following critical dimensions:

- **Expertise:** Access to scientific and management expertise on staff or within networks, understanding of the disease field and its unmet needs, special expertise in patient experience and perspective
- **Funding strategies:** Types of funding mechanisms (e.g., grants, program-related investments), a balance between investigator-initiated and targeted funding programs, focus within the pipeline (e.g., basic, translational, clinical research), development of tools and resources for the field, management of grantees, level of risk tolerance
- **Engagement with external constituencies:** Relationships with researchers, industry, regulators, payers; policies for engagement with external stakeholders
- **Patient resources (including data):** Relationship with patient community; provision of services, including connecting with clinical trials; partnering to bring patient perspective and participation to R&D; collection of patient data and utilization for research

Below, we frame an effort to develop a "partnership maturity model" for patient organizations, offering examples of how people, processes, and tools can evolve across the four dimensions of expertise, funding strategies, external engagement, and patient resources that sum to an organization that is a consistently high-value research partner. We will seek input on this draft and vet it with key stakeholders in 2020.

2. Organizations providing these resources include FasterCures' TRAIN program, the National Health Council, Genetic Alliance, the National Organization for Rare Disorders, the Health Research Alliance, Global Genes, and the National Institutes of Health's (NIH) National Center for Advancing Translational Sciences, among others.
3. One example is TDWI's Big Data Maturity Model, which "provides the big picture of a big data program, where it needs to go, and how to get there. As organizations move through these stages, they gain more and more value from their investments."
4. "Maturity model," Wikipedia.org, https://en.wikipedia.org/wiki/Maturity_model (accessed September 26, 2019).

PROPOSED PARTNERSHIP MATURITY MODEL

	LEVEL I	LEVEL II	LEVEL III
EXPERTISE	▪ Has minimal professional staff ▪ Scientific Advisory Board (SAB) primarily consists of funded scientists	▪ Has engaged, non-conflicted SAB ▪ Has created and maintains a research roadmap for the disease ▪ Has a chief scientific or medical officer	▪ Has a business or management advisory board ▪ Has hired an alliance development staff member
FUNDING STRATEGIES	▪ Provides grant funding to academic investigators ▪ Funds primarily basic discovery ▪ Investigators initiate most projects	▪ Funds development of tools and resources ▪ Funds translational science ▪ Has at least some targeted grant programs ▪ Manages grantees actively	▪ Funds or invests in private companies ▪ Engages in or convenes multi-stakeholder collaborative R&D efforts ▪ Is willing to accept high risk
EXTERNAL ENGAGEMENT	▪ Engaged with academic researchers ▪ May receive funding from industry for conferences	▪ Builds relationships with key stakeholders across the ecosystem ▪ Has a transparent conflict of interest policy for industry relationships ▪ Has provided formal or informal input to FDA	▪ Has intellectual property policies for university and industry grants ▪ Convenes research roundtables to discuss challenges with key stakeholders ▪ Has interacted with payers regarding the value of and access to treatments
PATIENT RESOURCES (INCLUDING DATA)	▪ Has a contact registry of patients ▪ Provides information about clinical trials to patients	▪ Collects robust natural history data in a registry ▪ Aids in recruiting patients for trials	▪ Has multiple platforms/ methods for collecting patient data ▪ Collects data utilizing common data models and standards

Source: Milken Institute.

In addition to seeking community input on the overall value of such a maturity model and the example details, FasterCures will investigate the possibility of creating a benchmark survey so that organizations can see where they fall on the readiness continuum, compare themselves to other organizations, get recommendations for reaching the next stage of maturity, and track their progress.

Building capacity to become research ready

Despite the growing numbers of organizations that are exemplars of sophisticated research partners and of platforms and resources to learn from peers, the need remains for more resources to replicate and scale models through capacity-building—either by patient organizations to become research-ready, by organizations within their patient communities, and by partners to become ready to engage with patient organizations in mutually beneficial ways. There has been a lot of pushing out of information and resources to patient organizations. Now, we must turn our attention to how we can drive the adoption of good approaches and consolidate efforts, if necessary.

The Patient-Centered Outcomes Research Institute (PCORI) is one potential source of funding for capacity building among patient organizations. A nongovernmental organization created by the Affordable Care Act, PCORI focuses on the conduct of research into health outcomes that is relevant to patients and their caregivers. As such, PCORI has gone to great lengths to ensure the integration of patient perspectives into its prioritization and decision-making processes. Further, it funded the creation of "People-Powered Research Networks" (PPRNs) to experiment with different models to incorporate patient perspective and PGHD into the research conducted within PCORnet, the National Patient-Centered Clinical Research Network. The PCORnet infrastructure has been spun off into a separate nonprofit to ensure its sustainability, and PCORI—which is due for reauthorization by Congress in 2019—will return to being a funding body rather than an infrastructure provider. With this action, PCORI could apply lessons learned from the PPRNs to build capacity among patient organizations to advance within this maturity model, including in their capacity to collect and contribute valuable patient data for research.

Patient organizations are becoming more intentional about training their patient communities to serve as research partners. The Arthritis Foundation has initiated a series of training courses to prepare patients to serve as experts in a variety of research settings. One respondent to our questionnaire noted the creation of a new coalition, HD-COPE, "to organize patient perspective data and to train people with [Huntington's disease] and their families to present this data to sponsors and regulators in an effective way."

III. Patient organizations can be critical sources of data

Patient groups are bringing their data assets to their partnerships, as evidenced in the Michael J. Fox Foundation example called out above. More than three-quarters of respondents to our fall 2018 questionnaire share de-identified patient data gathered with partners, and the same proportion does not charge a usage fee. More than one-half require committee review of data requests and a data-use agreement; many dictate terms regarding ownership and control of the data and the return of results to the foundation and/or to patients.

Most of these organizations have shared their data with academic and industry researchers. Use of their data has resulted in publications, basic biological insight, research tools or infrastructure, clinical studies, and preclinical work. However, a full 80 percent said their data have not been integrated with other sources for research.

An evolution is underway about how best to integrate PGHD—from, for example, registries, direct-to-consumer testing, e-health, and m-health—with other sources. In addition, an increasing number of projects are testing the validity of the data, as well as creating new models of data partnerships among patient organizations, and between patient organizations and other stakeholders in the health care system such as academic research institutions, biopharmaceutical companies, and government agencies.

These data sources and types—demographic, clinical, pathology, molecular/genetic, biometric, patient attitudes, health habits, and lifestyle—exist within a broader and rapidly changing ecosystem. System-wide models for sharing PGHD have not yet reached maturity. With the most common current model, two or more entities partner to identify a specific research question and share data between them to address it, which effectively meets their needs. However, this model cannot be scaled to achieve the ideal of a frictionless global research ecosystem.

> Researchers at the University of Alabama at Birmingham are integrating patient-reported outcomes (PROs) collected in the Global Healthy Living Foundation's ArthritisPower™ research registry with clinical and lab data from electronic health records. They aim to study the impact of this PRO data on shared decision-making for rheumatoid arthritis patients.

Investing in data aggregation and use

Some lessons learned and promising approaches have emerged from patient organizations at the forefront of data aggregation and use. These organizations use PGHD to enable richer study of the natural history of the disease, the progression of disease in the absence of treatment, and the definition of outcomes most important to patients. These examples can facilitate moving beyond "one-off" models of data sharing and use towards system-wide solutions that are more efficient and effective and decrease the transaction friction when linking patient-generated, clinical, claims, social factors, and other data.

Patient organizations are well-positioned to deploy an expanding array of technologies to capture a range of data types. Before investing in or expanding a data enterprise, an organization's leadership must define the strategic priorities for data capture. First, they

must determine whether other entities are already capturing the needed data. Second, they must consider the captured data's value to their patient population and alignment with their mission. Third, and perhaps most importantly, they must consider the scope and magnitude of the financial commitment required to build and maintain a data aggregation and analytical enterprise. Many organizations will eschew building data assets and instead choose to partner with companies or networks, whether a for-profit vendor such as Invitae or a nonprofit platform such as the National Organization for Rare Disorders' IAMRARE™, that can manage the technology infrastructure and data management activities for them.

If a patient organization decides that building its data assets will enable it to better serve its patient community and bring unique value, it can expect to experience several growth stages, from strategic planning through operational planning to implementation. Adequate initial and sustained funding and early-stage planning are key to long-term success. Considerations include the following:

- Patient organizations that want to develop their data resources for maximum impact must **cultivate partnerships.** As one patient organization's vice president of technology has remarked to us, they look for people who will be true partners, who understand that they will be hands-on with defining research questions and participating in studies. Partnerships that enable merging of data sources will increase the value of the data assets developed.
- Patient organizations must focus on continuously improving the **quality** of captured data. As an example, the Cystic Fibrosis Foundation incorporates widely accepted and standardized data collection instruments into its web-based applications. To further ensure data quality, it validates data through extensive edits and uses natural language processing to standardize free text provided by survey respondents. These techniques increase data validity and enable their linkage to other sources by demographic information and standardized patient identifiers.
- Years of policy focus and funding have improved the **interoperability** of server-based information technology systems for payers and providers. Cloud-based environments provide patient organizations with relatively cost-effective options for storing, accessing, sharing, and analyzing their data. These environments enable more secure and easy sharing of data than do local servers. In addition, data can be more easily shared and linked to other sources if they comply with a widely accepted common data model (CDM), such as the Observational Medical Outcomes CDM developed by Observational Health Data Sciences and Informatics (OHDSI) or the CDM created by PCORnet. Patient groups can also map their data to condition- or population-specific data models such as PEDSnet's. Doing so decreases the time and resources needed to transform the data for reuse.
- **Data standards** work hand-in-hand with interoperability, and several sets of standards are widely used within the health-care sector. Each patient organization must determine how to standardize its data to enable integration with claims, clinical, social determinants of health, and data from other entities. Some patient organizations that fund clinical research are familiar with the Clinical Data Interchange Standards Consortium standards. Others rely on Fast Healthcare Interoperability

Resources (FHIR) standards created by Health Level Seven (HL7), an international health-care standards organization. FHIR covers data formats and elements, data representation, and results, as well as application programming interface technology to standardize user interface integration—all of which enable data integration. Although created for electronic health records (EHRs), FHIR has been adopted by some patient groups. These standards continue to evolve, and patient organizations should carefully consider the strengths and limitations of different standards in terms of their specific objectives.

- The imperative to ensure patient **privacy** underlies all of these factors. Because of their role as trusted sources of information and their missions to serve their patient communities, patient organizations take privacy very seriously. They must comply with legal and regulatory requirements governing patient privacy, including patient consent if data are shared with other entities or data networks. Organizations with robust data capabilities that engage in research must request informed consent from patients and undergo institutional review board oversight. Some patient organizations rely on partners with experience in navigating these challenging requirements. While patient organizations consider patient privacy to be sacred, they are uniquely positioned to gather data about and shed light on patients' willingness to share their health data for research and their risk tolerance in specific situations.

IV. Shared data networks are a growing research infrastructure

As noted above, one-off models of data sharing and use are the status quo. However, the field is moving toward system-wide solutions that are more efficient and effective and decrease transaction friction. The data network model, such as that employed by PCORnet, the National Evaluation System for health Technology (NEST), and the Global Alzheimer's Association Interactive Network (GAAIN), exhibit growing promise for aggregating and analyzing research data. The goal of these networks is to create a data process, shared infrastructure, and a common data model and syntax that can facilitate research across a broad range of inquiry for a diverse array of users. Participation in a network can facilitate access to partners working in the same or related disease areas and therefore a larger or more diverse data set. These networks are in the early stages of incorporating PGHD.

Participation in a broader data network allows researchers—and patients—to look across diseases to identify patterns and shared features. They can conduct studies and run queries that lead to unique connections and insights.

Understanding shared data networks as research partners

The imperative to share data across and among health-care sectors is growing, as is interest in aggregating and analyzing currently siloed data. Data repositories have become more common in medical research and the delivery and financing of care. However, these repositories remain underutilized for several reasons, including reluctance on the part of data holders to cede control of data, concerns over patient privacy, restrictive existing data use agreements, and lack of incentives for re-use of shared data.

Shared data networks provide an alternative to the aggregation of EHR or medical and pharmaceutical claims data from multiple entities into centralized databases such as a repository, or platforms that enable data sharing and analysis but only within a defined environment. These networks may take several forms, from facilitation of researcher collaboration to distributed models in which a shared infrastructure, common data model, and syntax are maintained. For product developers, providers, health insurers, and patient organizations, they offer the advantage of pooling data to yield greater insights and larger sample sizes without loss of control of contributed data.

Table 1. Types and Examples of Data-Sharing Infrastructure

REPOSITORY	PLATFORM	NETWORK
Data sets are uploaded and made available to qualified researchers for download and secondary use	An environment that enables data sharing and access as well as aggregation and analysis	An infrastructure that links and provides access to data sets and research/analytical services across multiple independent institutions, without data residing in a central repository
▪ dbGaP ▪ GenBank ▪ Cancer Imaging Archive ▪ Yale Open Data Access Project ▪ Clinical Study Data Request	▪ Vivli ▪ Project Data Sphere ▪ ImmPort ▪ Synapse ▪ tranSMART	▪ PCORnet ▪ NEST ▪ Sentinel ▪ GAAIN ▪ MDEpiNet Coordinated Registry Networks

Source: Milken Institute

Patient organizations are increasingly following the lead of NIH, the Wellcome Trust, and other large funders in requiring that their funded researchers share data, usually by contributing them to a public repository such as the examples in Table 1. So why would patient organizations want or need to complicate their operations by collaborating with a platform or federated network to share their PGHD? The benefits of being part of a broader data network for these organizations include the following:

- Achieving the promise of "big data"—that is, faster and more accurate answers to research questions because of access to larger quantities of more diverse data,
- Increasing research speed and efficiency, and
- Informing this next generation of data-driven research with data about the priorities and lived experience of patients that are usually lacking in these environments.

Given their scale and structure, federated data networks aim to make clinical research and the generation of real-world evidence faster, less costly, and more reliable for product development as well as for regulatory and clinical decision-making. Data partners who participate in a network gain access to other organizations working in the same or related disease areas and to a larger or more diverse data set.

For example, PCORnet provides access to EHR and claims data from 128 million people across 139 US health-care organizations, including 65 million people who are eligible to participate in clinical trials. NESTcc, which focuses on medical devices but is similar in structure and function to PCORnet, has MOUs with 12 organizations representing more than 150 hospitals, 3,000 outpatient clinics, and 108 million patients, and therefore access to more than 469 million patient records and data sources including EHRs, pharmacies, public and private claims, registries, and some PGHD.

Patient organizations can position themselves as network partners by defining their strategic research priorities and connecting with a network that shares those priorities. A robust, interoperable infrastructure that ensures the confidentiality of patient data is foundational for data partnership, as is the use of common data models and broadly accepted

data standards and syntax, such as that provided by HL7. Perhaps most importantly, participation in a shared data network requires that parties bring high-quality data to the table. For example, basing survey data on standard survey instruments such as PROMIS and RAPID3 facilitates linkages and standardization for data elements common to network partners.

The first phase of PCORnet engaged network health research and care institutions and health plans, as well as 20 PPRNs, to pilot different approaches to engaging patients in the leadership, planning, and execution of real-world evidence generation. Organizations involved in the PPRNs, such as the Global Healthy Living Foundation, the Phelan-McDermid Syndrome Foundation, and the Epilepsy Foundation, created data assets, such as mobile apps, high-quality multifaceted registries, and data dashboards, that provided value to their partners and models for other patient organizations. PCORI now seeks to distill and apply lessons learned from the PPRNs to a sustainable model for engaging patients and integrating PGHD data into research networks.

Now in its second phase and managed by the People-Centered Research Foundation, PCORnet consists of nine health research and care institutions and two health plans. Through an online front door portal, researchers can, in essence, receive rapid responses to their real-world queries by asking millions of individuals nationwide the same question at the same time, as well as conduct observational studies and large pragmatic clinical trials. PCORnet touts its strengths to be a vast amount of data, clinical trial readiness, and patient-centeredness. NEST is being designed to support use-cases ranging from pre-market approval and clearances to expansion of indication, post-market safety and surveillance studies, and coverage decisions via both observational and interventional study designs as appropriate. It has launched a first round of "test cases" to assess the capabilities of its data network and has issued a call for proposals to bring PGHD into the network.

The FDA's Sentinel Initiative is the precursor to these large-scale federated data networks. Created in 2008 to systemically monitor the safety of medical products after their introduction to the market, it now works to identify opportunities for broader use of its data infrastructure with partners such as NIH and PCORnet. In addition, it seeks to expand its data sources from primarily administrative and claims data from payers to include, possibly, EHRs and disease registry data.

The Global Alzheimer's Association Interactive Network is a rare example of a disease-specific federated data network infrastructure, supported not only by government funding but also by patient organization funding from the Alzheimer's Association. Researchers can discover imaging, genetic, clinical, and proteomic data collected across many independent studies of almost 500,000 individuals from almost 50 partners (largely academic research centers), build cohorts, and connect with data partners. This type of disease-focused network could likely be scaled and replicated across a variety of diseases and more readily integrated with broader, disease-agnostic networks.

WANT MORE? CONTINUE TO **PART III: FOR RESEARCHERS** TO LEARN MORE ABOUT:

- Key characteristics to understanding patient organizations as research partners
- Resources to help identify patient organization partners
- What types of data patient organizations have and why they have invested
- How to most meaningfully and effectively engage patient organizations as research partners

About the Authors

Kristin Schneeman joined FasterCures in April 2005 as program director, with primary responsibility for its innovation portfolio of projects and activities, focused on best practices in the funding and conduct of medical research and innovative collaborations among players in the research enterprise. She brings to FasterCures decades of experience in public policy, politics, academia, and the media. Schneeman served for three years as a senior adviser and policy director to a gubernatorial candidate in Massachusetts, as a policy aide to a US Congressman, and for four years as the front-line manager and chief-of-staff for a senior adviser to Vice President Al Gore. At Harvard University, she directed research projects on future challenges facing governments, and on complex negotiations in business, politics, and international relations. Schneeman began her career as a producer of documentary films, for which she was the recipient of an Emmy Award in 1990.

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