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

PART I: MAKING THE CASE



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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

Data sharing has become like “motherhood and apple pie” in the biomedical research and development (R&D) ecosystem. There is nearly universal agreement that aggregating and re-analyzing shared data can lead to more and better scientific insights. Funders are requiring data sharing in greater numbers, and patients are increasingly dismayed to discover that their data are not routinely shared to further research or to improve their care.

Although data from randomized controlled trials remain the gold standard for research into the efficacy of treatments, effectiveness research and new opportunities to use real-world data from electronic health records, mobile health devices, registries, claims, and other sources are receiving increased attention. As part of this change, there is an emerging focus on engaging with patients in the definition of research questions and meaningful outcomes, as well as on re-examination of the role of patient-generated health data (PGHD) within the expanding yet still fragmented data ecosystem.

In this environment, shared data networks and platforms are springing up with increasing regularity, some of them quite large in scale, bringing together Big Data on millions of patients that can be queried for research purposes.

FasterCures, alongside other patient-focused advocacy organizations, has long worked with stakeholders such as product developers and regulators to define and implement the “how to” of patient engagement. This series, “Advancing Models of Patient Engagement: Patient Organizations as Research and Data Partners,” seeks to identify effective ways for research organizations of all types, including research data networks, to partner with patient organizations that can bring patients’ perspective, participation, and data to the table. Part I of the series offers recommendations for patient organizations, researchers, and funders (who play a critical role in setting expectations and incentives as well as building capacity to enable this evolution).

### KEY TAKEAWAYS FROM THIS SERIES

#### I.

Clinical innovation is rapidly becoming more patient-centric and data-rich.

#### II.

An explosion of data has opened new opportunities to capture a fuller patient experience.

#### III.

Infrastructure for sharing, aggregating, and analyzing data from a variety of sources is growing.

#### IV.

Patient-generated health data are generally not available in the environment of shared data networks—but, ideally, they should be.

## I. Clinical innovation is rapidly becoming more patient-centric and data-rich.

Two paradigm shifts are unfolding in parallel and are intersecting:

1. The engagement of **patients** as partners, no longer just subjects, across the continuum of research, development, and care
2. The explosion of available **data** of all types to inform clinical research and care, and the advent of advanced analytics to help make sense of it all

Patient organizations<sup>1</sup> have a wide range of goals, capabilities, and resources, but at their core have been established to help current and future patients by raising awareness, ensuring robust investment in medical research, and advancing solutions that support innovation and access to effective preventive and treatment interventions. Patient organizations have funded basic discovery, preclinical, and clinical studies, and, in some cases, have created new data repositories to support and advance research in their respective areas.

FasterCures has a long history, through its TRAIN (The Research Acceleration and Innovation Network) initiative and its Patients Count program, of supporting and learning from innovative patient organizations that want to follow a more strategic and entrepreneurial approach to their role as funders and intermediaries for patient engagement in research and product development. In increasing numbers, these groups want to bring richer, real-world data about patients' lived experience to the planning and conduct of research to more quickly and accurately

answer questions that matter to patients. And their unique contribution to achieving this goal is their access to patients.

1. For the purposes of this project, we define "patient organizations" as nonprofit, philanthropic, disease-focused organizations that support some combination of research, advocacy, and education.



## II. An explosion of data has opened new opportunities to capture a fuller patient experience.

With the rise of “big data,” the digital revolution, and the increase in analytics to answer pressing questions with larger and richer datasets, the data landscape has expanded exponentially.

The ability to better understand patients’ journeys and give context to claims data and other routinely collected data is pushing researchers, product developers, providers, regulators, and other stakeholders to use *all available data* to accelerate product development, inform regulatory decision-making, and impact innovation in care delivery. Data about patients’ experiences outside the clinic are not only “nice to have” but also critical to understanding and improving those outcomes. A great deal of momentum surrounds the application of new technologies, such as mobile devices and other digital platforms, to both deliver care and generate real-world data on patients’ experiences.

A critical source of such context-informing data is PGHD, defined by the Office of the National Coordinator for Health Information Technology (ONC) as “health-related data created, recorded, or gathered by or from patients (or family members of other caregivers) to help address a health concern. PGHD include but are not limited to: health history, treatment history, biometric data, symptoms, lifestyle choices.<sup>2</sup> As the value of real-world evidence increases, patient data collected from patient registries, smartphone apps, wearable devices, online communities, and social media provide new windows into the patient experience. Inputs such as patient preference studies and patient journey maps

help draw a more complete picture of the impact of disease and therapies and give context to other data. This evidence can be used to align unmet medical need with targets, as well as to identify barriers to participation in research and access to care critical to illuminating and understanding the full picture.

Patient organizations can be sources of PGHD, and, while not the only source, they are available and increasingly enthusiastic partners, trusted intermediaries with patients, and funders for the collection and sharing of this type of data. And some are performing these functions in very sophisticated and influential ways.



2. Office of the National Coordinator for Health Information Technology, “What are patient-generated health data?” HealthIT.gov, <https://www.healthit.gov/topic/otherhot-topics/what-are-patient-generated-health-data> (accessed September 26, 2019).

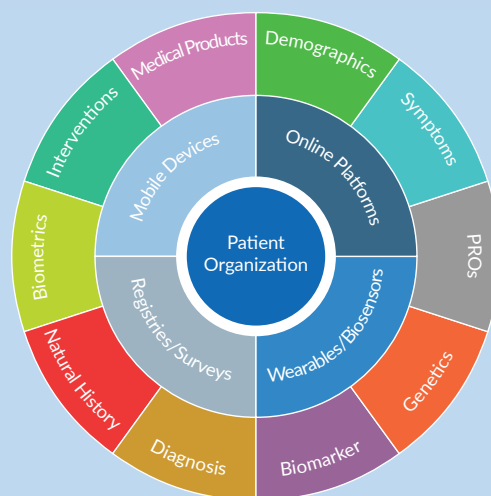
PGHD includes core data elements such as demographic characteristics, diagnoses, interventions, medical product use, and patient-reported outcomes (PROs) (see Figure 1). New platforms developed and maintained by patient organizations and their technology company partners enable monthly, weekly, or even daily data entry by patients at times and in locations that are easiest for each person. Recognizing the increased importance of genetics and proteomics to scientific innovation, patient organizations, such as the National Psoriasis Foundation, also fund or keep biobanks and repositories of genetic data, enabling them to easily share the data with a wide range of researchers. Importantly, registries and survey series can generate data on a patient over time to follow the patient's journey.

Although the uptake in the use of technology for health care has been slower than in other sectors,<sup>3</sup> some patient organizations are offering platforms that enable patients to track their symptoms and progress and to compare their experiences to others with the same disease through tables and graphics.

Because the types of data collected by patient organizations vary widely, any set of best practices for ensuring that data are valued and used consistently for research requires a clear definition of PGHD. These data are frequently referred to by individual use case (e.g., lab values) or the technology by which the data are gathered (e.g., social media data, sensor data) rather than by any agreed-upon definition of data content.

The types of data range from demographic data, common to all RWD sources, to genetic data, patient attitudes, and social and environmental data elements that may impact patients' health and well-being. eHealth (e.g., patient portals to capture PGHD) and mHealth (e.g., wearable devices and sensors) technologies will continue to expand the opportunities to capture patient data between clinical visits. In addition, continued advances in data science, including natural language processing techniques and other deep learning methods, will continue to enable analysis of the data captured by patient organizations in new ways.

**Figure 1: Data Types and Platforms Used by Patient Organizations**



Source: Milken Institute.

3. "Healthcare Must Embrace Digital to Win in Consumer Engagement," Forrester Research, Inc., (November 10, 2016).

### III. Infrastructure for sharing, aggregating, and analyzing data from a variety of sources is growing.

Near universal agreement seems to exist that aggregating and analyzing shared data will lead to more and better insights if we can address the technical, cultural, and financial challenges. The most common model consists of two (or more) entities partnering to share data between them to answer a specific research question. Many of these collaborations exist, and they are effective at meeting the needs of those specific partnerships. However, as we move toward the ideal of a frictionless global research data-sharing ecosystem, this model is simply not scalable.

Data repositories to enable sharing have become common, if currently underutilized, in academic science.<sup>4</sup> For clinical trials, several platforms have emerged in recent years for data sharing and analysis (e.g., Project Data Sphere, the Yale Open Data Access project, Clinical Study Data Request, Vivli), which are beginning to produce valuable insights.

More recently, federated or distributed networks of research and care institutions have been built—including PCORnet, the National Patient-Centered Clinical Research Network; the National Evaluation System for health Technology (NEST); the Food and Drug Administration's (FDA) Sentinel Initiative; and the Global Alzheimer's Association Interactive Network—to enable research via access to (primarily) electronic health record (EHR) and claims data from millions of patients across the United States. The federated data network model aims to create a data process and shared infrastructure, relying on a common data model and syntax, which can facilitate a broad range of inquiry for a diverse array of

users while leaving the data in the hands of each data partner.

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. For data partners, participating in a network can provide access to other organizations working in the same or related disease areas and can facilitate access to a larger or more diverse set of data.



4. For example, Ann Marie Navar, Michael J. Pencina, Jennifer A. Rymer, Darcy M. Louzao, and Eric D. Peterson, "Use of Open Access Platforms for Clinical Trials Data," *JAMA*, 2016;315(12): 1283-1284.



## IV. Patient-generated health data are generally not readily available in the environment of shared data networks—but, ideally, they should be.

Shared data networks, including federated models, are in the early stages of incorporating PGHD into their available data set(s). More broadly, to the extent that PGHD are accessible for research or care improvement in the institutions that compose shared data networks, they tend to be collected and controlled by providers and researchers, gathered infrequently, unavailable to patients in a form that creates value for them, and not always driven by patients' interests and priorities. This needs to change.

As ONC notes, "PGHD are distinct from data generated in clinical settings and through encounters with providers in two important ways: Patients, not providers, are primarily responsible for capturing or recording these data. Patients decide how to share or distribute these data to health care providers and others."<sup>5</sup> Both ONC and FasterCures, as well as other health-care leaders, are calling for a person-centered health data infrastructure rather than a provider- or institution-centered one to enable the collection and sharing of this kind of patient-centered health data.

Ideally, PGHD (as defined above) should be included in all clinical research, including research conducted through shared data networks. As we explore in [Part II](#) of this series, patient organizations with data assets can start to fill this gap by partnering with shared data networks to ensure that PGHD are incorporated into the data sets available for analysis. With the emergence of disease-specific and disease-agnostic data networks and patient organizations determined to advance medical research through real-world evidence, now is the time to review and develop models for productive partnerships among these players.

### WHAT WERE THE INPUTS TO THIS REPORT?

- Findings from desktop research on existing patient engagement models and on efforts to integrate real-world data (including PGHD) and evidence into medical research and health care
- Findings from interviews with key opinion leaders from patient organizations, research data networks, and other data-sharing thought leaders
- Answers from an online questionnaire of a select group of patient organizations, conducted by FasterCures (and shared by the Genetic Alliance and National Health Council with their members) in September-October 2018
  - Seventy-eight unique organizations described their organizational characteristics (e.g., size, budget, disease areas, existing policies and practices, research assets, partnering practices) as well as their investments in patient data and how they view the challenges and opportunities of linking data to broader networks and platforms.
- Comments from participants of a half-day workshop discussion, "A Patient-Centered Data Ecosystem to Accelerate Medical Solutions," co-convened in October 2018 by FasterCures and the Duke-Margolis Center for Health Policy. Approximately 40 leaders from across the biomedical R&D and health-care delivery and financing systems identified action items to support a business case for investing in PGHD assets and their integration into the broader data ecosystem.

5. "What are patient-generated health data?", HealthIT.gov, <https://www.healthit.gov/topic/otherhot-topics/what-are-patient-generated-health-data>.

## V. Recommendations

If we all agree that PGHD have value and that patient organizations have value as research and data partners, how do we enable more and better collaboration among these stakeholders, particularly in the context of shared data networks? Our recommendations fall into three categories:

- Improve the capacity of patient organizations and other stakeholders to partner
- Optimize the development and use of patient-generated health data
- Develop a framework for partnership in the context of shared data networks

### Improve the capacity of patient organizations and other stakeholders to partner

Although the numbers of organizations that are sophisticated research partners, and of platforms and resources for organizations to learn from their peers, are growing, the need remains for more resources aimed at replicating and scaling these models through capacity-building—by patient organizations to become research ready, by organizations with their own patient communities, and by partners to be 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 consolidation of 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's mission focuses on the conduct 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 the More recently, federated or distributed networks of research and care institutions have been built—including PCORnet, the National Evaluation System for health Technology (NEST); the Food and Drug Administration's (FDA) Sentinel Initiative; and the Global Alzheimer's Association Interactive Network—to enable research via access to (primarily) electronic health record (EHR) and claims data from millions of patients across the United States. 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 their ability to collect and contribute valuable patient data for research.

Patient organizations themselves 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.”

### NEXT STEPS:

- For patient organizations:
  - Define and articulate their value to potential partners (e.g., where they are on the maturity scale proposed in Part II of this series).
  - Define their guiding principles for partnership and expectations (e.g., what benefits should accrue to patients).
  - Understand the incentives and imperatives of potential partners.
- For researchers:
  - Identify where potential partners are on the maturity scale and set objectives and expectations accordingly (e.g., an organization at an earlier stage of maturity can still be a valuable research and data partner, if partners understand their capabilities and assets and factor them into their plans).
  - Understand the incentives and imperatives of patient organization partners.
- For funders:
  - Fund capacity-building by patient organizations to become research-ready partners.
  - Educate other stakeholders about the benefits of partnering with patient organizations and disseminate best practices in patient engagement.

## Optimize the development and use of patient organizations’ patient-generated health data

On October 24, 2018, FasterCures and the Duke Margolis Center for Health Policy convened patient organizations, product developers, payers, providers, IT platform companies, and others to identify the highest value uses of PGHD and the barriers to integrating these data into health-care innovation and delivery research. During this workshop, participants highlighted technical barriers such as data standards, common data models, and interoperability, as well as questions about how to improve the quality of PGHD. The FDA recently released guidance to clarify the criteria by which PGHD will be considered “fit for purpose” for regulatory oversight. All workgroup participants expressed concern about protecting patient privacy and support for increasing patient control over their data. They also observed that use of the generated data will not necessarily correct for the inherent biases that currently exist in clinical trial participation.

A significant (but not widely acknowledged) challenge impacting the linking of disparate types of data is the lack of a business model that encourages such sharing. Data aggregators/platforms, health plans, providers, product developers, and researchers understand that their data resources are valuable assets and—acknowledging the financial investments needed to support data aggregation, infrastructure, and analytics—are interested in monetizing their data in the interest of sustainability, if nothing else.

By virtue of their dual role as trusted resources for patients and sources of insight for innovators, patient organizations could play a critical role in expanding the capacity to collect and deploy these new sources of PGHD.

An increasing number of patient organizations are successfully building their data assets

and analytics capabilities, alone or through partnerships. Still others, particularly organizations with small patient populations or with limited funding, have not entered the data space. Although many agree that data help them better serve their patient populations, they know that building or supporting data assets is a challenging endeavor. Combining their data with that of providers, manufacturers, and payers presents added complexity, but, if executed, can yield great benefits to patients, including the ability to predict who will get sick and when.

### NEXT STEPS:

- For patient organizations:
  - Carefully consider the purpose of any data gathering, and whether creating a separate resource is the best option or whether collaborative options exist that fit the purpose and are less resource-intensive.
  - Increase the value of data by moving through the maturity scale.
  - Transparently track and report data quality measures (completeness, accuracy, and timeliness).
  - Make potential partners aware of data resources via publications and presentations. Proactively seek partners for data.
  - Craft funding opportunities around the use and reuse of data resources (e.g., Data Challenges)—don't assume users will pursue these opportunities without incentives.
- For researchers:
  - Proactively seek patient organization partners with data assets.
  - Engage with these organizations to provide insight into the data needs of partners and technical requirements for data integration into your work.
- Consider how sharing and linking their data can provide maximum benefit to patients.
- For funders:
  - Fund infrastructure to enable more high-value data collection and sharing by patient organizations (e.g., “white label” customizable platforms or applications).

### Develop a framework for partnership in the context of shared data networks

Shared data networks such as PCORnet and NEST have unique structures, goals, and processes that present distinctive challenges and opportunities to engage patients in the definition and answering of research questions.

Whereas clinical researchers, product developers, and perhaps even regulatory review teams could build long-term relationships with patient organizations and advocates in a small number of discrete therapeutic areas, large-scale research data networks are in many ways more transactional in nature and could answer questions in an almost infinite range of therapeutic areas. They also provide a unique opportunity to institutionalize good patient engagement practices and the use of patient-generated health data across the health research and care landscape, by supporting and demonstrating the value of ongoing patient partnerships.

[Part III](#) of this series features a summary of good patient engagement practices for researchers. How might each of these key recommendations play out in contexts such as these?

**Patients as Essential Partners** Patients should occupy a seat at the table as proactive partners, functioning not just as trial subjects or as reviewers asked to react to already-developed materials, but as integral members of research governance structures and decision-making processes at both the network and local levels. Policies and expectations for patient engagement by collaborators should be set at the network level. The network could serve as a resource to collaborators for linking to patient-generated data sets, as well as support building capacity by patient groups to serve as research partners and to collect and share patient data.

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**Establish Partnerships Early in R&D Process** Given their structure as federations of local clinical research and care institutions, there is a need to strike a balance between partnerships with patient organizations with national or international reach, and the desire and need for engagement and relationships with local patients and advocates. When possible, craft strategic partnerships with patient organizations to support ongoing engagement efforts of network collaborators as well as the creation of valuable patient-generated health data assets. Work with patient organizations to create evidence-based common resources such as a consensus patient journey map. Seek out patient organizations with networks of trained patient advocates to maximize the possibility of connecting locally engaged patients with research institutions.

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**Define Expectations, Roles, and Responsibilities** At the start of a long-term partnership or short-term engagement, the parties should clearly define the expectations, roles, and responsibilities of all partners, including the data being shared, if any. These should be described in agreements between the parties, for example through a memorandum of understanding (MOU) or simple contract and data use agreement, and, ideally, be co-created by researcher and patient partners and revisited regularly. MOUs, or contracts, should also include each party's pre-specified commitments, how they will handle intellectual property and revenue sharing, how the partnership will protect the commercial and confidential information of each party, and how data privacy and security will be addressed.

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**Fit-for-Purpose Collaborations** Ideally, all parties will share a sense of purpose, agreed on before the start of an engagement. In addition, aiming to collect patient input that is representative of the target patient population is important, and this might mean engaging with multiple patient groups. Given that patient groups differ with regard to size, resources, expectations, data assets, patient population reach, and experience working with researchers, choosing the appropriate patient partners includes trying to match patient group characteristics to the specific needs of the research program. Conversely, it is important for patient groups to evaluate and define their value to research partners and choose research partners/programs that align with their objectives.

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**Measure Impact and Report Out** As stakeholders develop standard metrics to measure patient engagement, researchers should consider at the start of a patient partnership how the success of the collaboration will be measured. Both researchers and patient groups should establish feedback systems to gather data throughout the engagement process to measure its impact and mechanisms should be put in place to ensure a continuous feedback loop in which results of research are given back to patients and the public.

In our view, any partnership framework must include the following:

- Capacity-building (e.g., sharing of successful models of patient engagement and use of PGHD among network collaborators as well as patient group partners),
- Benefit to patients (e.g., access to interoperable health data, perhaps via Blue Button, actionable information about their health status and care),
- Compensation to patients and organizations for participation (e.g., PCORI's Compensation Framework), and
- Reciprocity (e.g., work together to find solutions if common data models do not account for key variables for a patient population)

#### CONTINUE TO PART II: FOR PATIENT ORGANIZATIONS TO LEARN MORE ABOUT:

- The role that patient organizations are playing as intermediaries for patient perspective and participation in research
- How patient organizations can improve their capacity as research partners
- How patient organizations' data can complement other data sources to capture a fuller patient experience in the "real world"
- The growing importance of shared data networks and the value of incorporating patient-generated health data in their research

#### READ 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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## 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,<sup>1</sup> 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.<sup>2</sup> 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,<sup>3</sup> defined as “a measurement of the ability of an organization for continuous improvement in a particular discipline,”<sup>4</sup> 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](https://en.wikipedia.org/wiki/Maturity_model) (accessed September 26, 2019).

## PROPOSED PARTNERSHIP MATURITY MODEL

	LEVEL I	LEVEL II	LEVEL III
EXPERTISE	<ul style="list-style-type: none"> <li>Has minimal professional staff</li> <li>Scientific Advisory Board (SAB) primarily consists of funded scientists</li> </ul>	<ul style="list-style-type: none"> <li>Has engaged, non-conflicted SAB</li> <li>Has created and maintains a research roadmap for the disease</li> <li>Has a chief scientific or medical officer</li> </ul>	<ul style="list-style-type: none"> <li>Has a business or management advisory board</li> <li>Has hired an alliance development staff member</li> </ul>
FUNDING STRATEGIES	<ul style="list-style-type: none"> <li>Provides grant funding to academic investigators</li> <li>Funds primarily basic discovery</li> <li>Investigators initiate most projects</li> </ul>	<ul style="list-style-type: none"> <li>Funds development of tools and resources</li> <li>Funds translational science</li> <li>Has at least some targeted grant programs</li> <li>Manages grantees actively</li> </ul>	<ul style="list-style-type: none"> <li>Funds or invests in private companies</li> <li>Engages in or convenes multi-stakeholder collaborative R&amp;D efforts</li> <li>Is willing to accept high risk</li> </ul>
EXTERNAL ENGAGEMENT	<ul style="list-style-type: none"> <li>Engaged with academic researchers</li> <li>May receive funding from industry for conferences</li> </ul>	<ul style="list-style-type: none"> <li>Builds relationships with key stakeholders across the ecosystem</li> <li>Has a transparent conflict of interest policy for industry relationships</li> <li>Has provided formal or informal input to FDA</li> </ul>	<ul style="list-style-type: none"> <li>Has intellectual property policies for university and industry grants</li> <li>Convenes research roundtables to discuss challenges with key stakeholders</li> <li>Has interacted with payers regarding the value of and access to treatments</li> </ul>
PATIENT RESOURCES (INCLUDING DATA)	<ul style="list-style-type: none"> <li>Has a contact registry of patients</li> <li>Provides information about clinical trials to patients</li> </ul>	<ul style="list-style-type: none"> <li>Collects robust natural history data in a registry</li> <li>Aids in recruiting patients for trials</li> </ul>	<ul style="list-style-type: none"> <li>Has multiple platforms/methods for collecting patient data</li> <li>Collects data utilizing common data models and standards</li> </ul>

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
<ul style="list-style-type: none"> <li>▪ dbGaP</li> <li>▪ GenBank</li> <li>▪ Cancer Imaging Archive</li> <li>▪ Yale Open Data Access Project</li> <li>▪ Clinical Study Data Request</li> </ul>	<ul style="list-style-type: none"> <li>▪ Vivli</li> <li>▪ Project Data Sphere</li> <li>▪ ImmPort</li> <li>▪ Synapse</li> <li>▪ tranSMART</li> </ul>	<ul style="list-style-type: none"> <li>▪ PCORnet</li> <li>▪ NEST</li> <li>▪ Sentinel</li> <li>▪ GAAIN</li> <li>▪ MDEpiNet Coordinated Registry Networks</li> </ul>

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

PART III: FOR RESEARCHERS



## 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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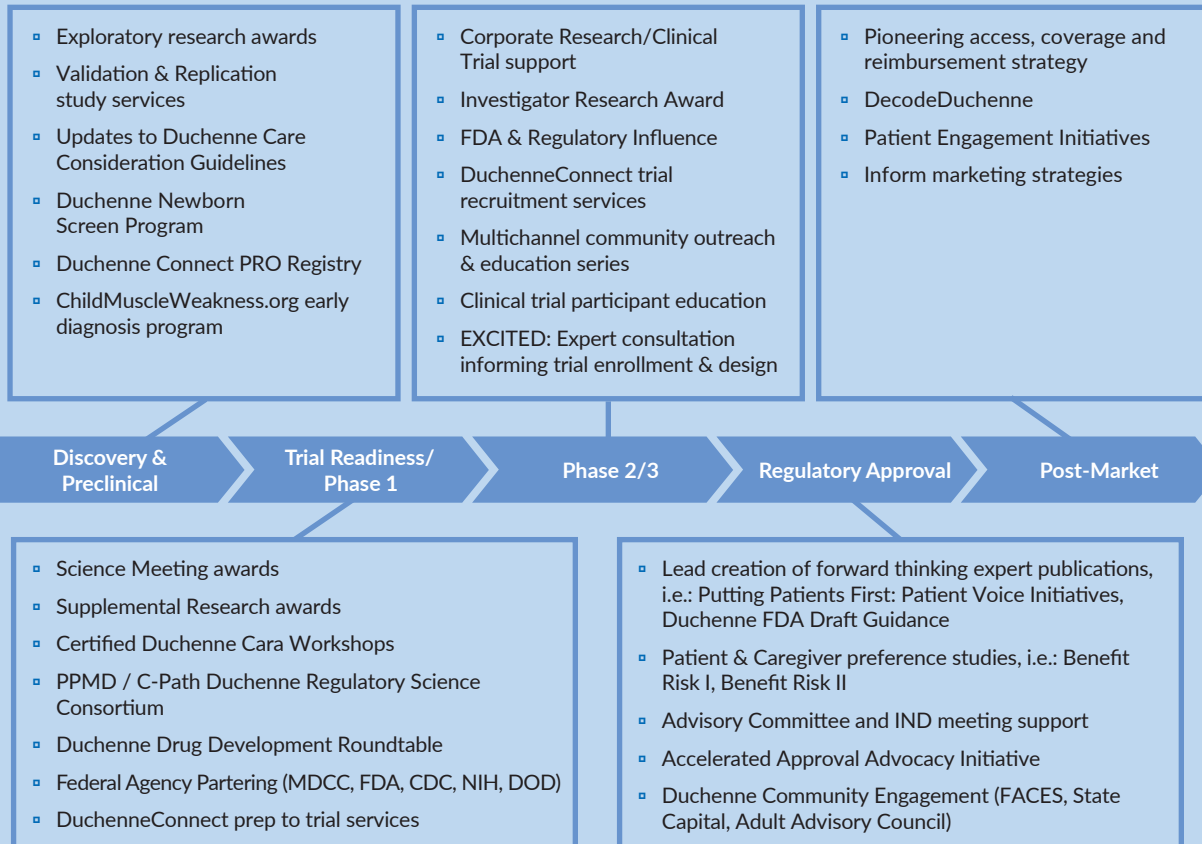
## Introduction

We are in the midst of a sea change in health care that is expected to accelerate in the coming years. An important driver of this change is the empowerment of patients who are using technology to search for health information, generating and accessing their health care data, and becoming involved in biomedical research in new and different ways.

Many other parties are benefiting from greater patient engagement as well. Through initiatives such as Patient-Focused Drug Development, regulatory agencies such as the US Food and Drug Administration (FDA) seek patient-generated information as inputs to their benefit/risk reviews of medical products. In addition, health technology assessors and payers are beginning to use patient insight in product value and insurance coverage determinations. Medical product developers can engage patients to gain a better understanding of unmet needs, which in turn helps them to gain a competitive edge in crowded therapeutic classes with products that align with patient preferences and to develop more efficient and less burdensome clinical trials.

In addition, researchers have recognized the importance of defining questions and clinical outcomes that are meaningful to patients. The tactical question for researchers then becomes how to effectively and efficiently bring the patient perspective, including patient data, to bear on their work. Although researchers can and should engage individuals and small patient groups in their projects, they should build relationships with patient organizations that can facilitate collaboration with specific patient types and enhance their understanding of patients' lived experiences.

To illustrate its capacity to add value across the research and development continuum, Parent Project Muscular Dystrophy (PPMD) adapted a chevron diagram popularized by the Clinical Trials Transformation Initiative, “Patient Group Engagement Across the Clinical Trial Continuum,” to catalog organizational assets relevant to each drug development stage, illuminating the ways that researchers and sponsors might draw on PPMD’s expertise.



*\*Adapted from CTTI's PG Engagement Across the Research & Development Continuum*

This paper will describe the patient organization ecosystem, lay out the types of patient-generated health data (PGHD) that patient organizations may collect, and conclude with recommendations for researchers for effective and meaningful engagement with patients and patient organizations.

## KEY TAKEAWAYS FOR RESEARCHERS:

### I.

The research and regulatory environment is changing, with an increased focus on engaging patients in the process. We have moved beyond “checking the box.”

### II.

There are more and less effective ways to engage patients and patient organizations.

### III.

Patient organizations and patients bring specific capabilities to the research process.

### IV.

Several barriers exist to increasing patient involvement.

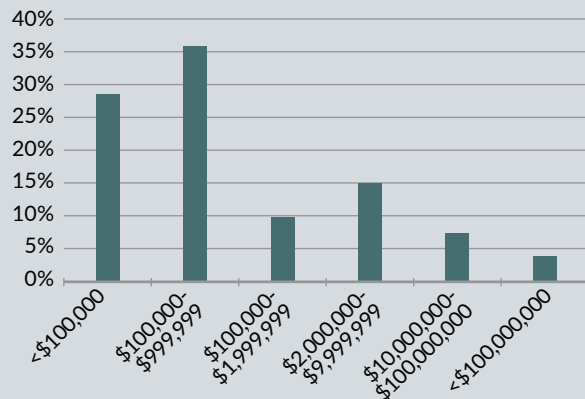
## I. Understanding patient organizations as research partners

GuideStar lists more than 18,000 US foundations as supporting “diseases and disease research.” These patient-focused organizations have differing levels of capacity and assets to engage in the research process.<sup>1</sup> During fall 2018, FasterCures received responses from 78 unique organizations to a questionnaire sent to patient groups in its network to solicit information about their organizational characteristics and activities, as well as their investments in data. Several key characteristics of patient organizations are important to consider when assessing their value as research partners.

### MISSION

Most organizations focus to varying degrees on advocacy, education, and research. It is instructive to look at what proportion of an organization’s resources is devoted to each.

**Figure 1: In the most recent fiscal year for which you have data, what were your organization’s annual expenditures devoted to research?**



Source: FasterCures questionnaire, Fall 2018.

### SCIENTIFIC STRATEGY

Patient organizations’ appreciation of the many ways that patients can enhance the biomedical research process continues to grow. Some have created research strategies to guide not only their own investments but also the research priorities of other stakeholders in a patient-centered way. As a part of these strategies, many have invested in robust needs assessments to increase their understanding of both the research landscape (current scientific challenges and opportunities) and market needs to target their activities to achieve the greatest impact.

The Melanoma Research Alliance (MRA) was established in 2007 after a cross-sector leadership retreat released an initial call to action to guide its scientific activities. The alliance identified 17 key scientific and clinical questions. At that time, the FDA had not approved a new drug for melanoma in nearly a decade. MRA has regularly updated its Scientific Strategy to reflect the rapidly changing landscape of science and product development and continues to commit its investments to the areas of greatest unmet need for patients.

### STRUCTURE

An organization’s status as a public charity or private foundation may influence its actions. For example, public charities raise funds every year from small and large donors, while private foundations are endowed and do not have to raise funds every year. This difference may result in differing levels of risk tolerance, with private foundations possibly having a greater appetite for risk. Public charities tend to have greater

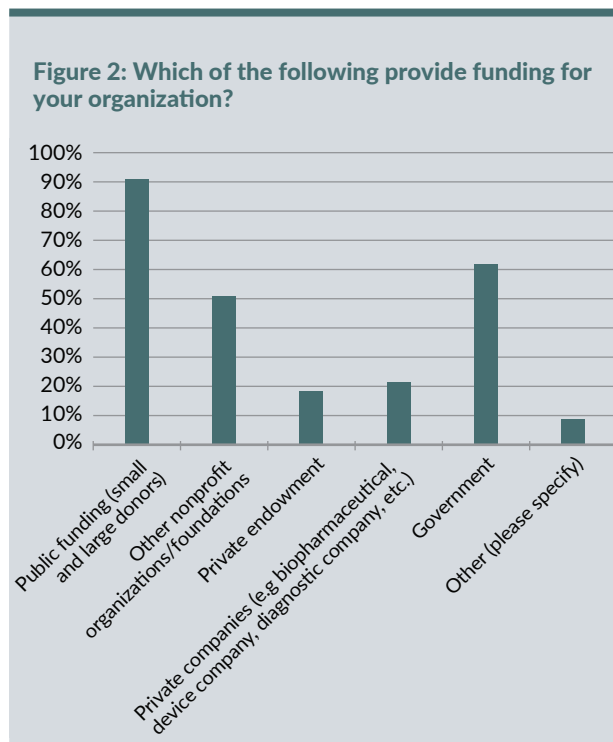
1. While our focus in this project has been patient organizations as ready partners for research and sources of patient data, we acknowledge that there are conditions for which there is no organized patient constituency, many competing organizations, or have patient populations that have yet to be activated.



outreach into patient communities, while private foundations—if they have a specific disease focus at all—tend not to have infrastructure themselves—such as communications vehicles, online communities or patient services—though they may fund its creation by others.

## AMOUNT AND SOURCES OF FUNDING

Although the amount of funding is an asset, culture matters more. The Cystic Fibrosis Foundation has worked for many years to put the building blocks of success—funding as well as patient data and engagement—into place. However, small organizations such as the Chordoma Foundation can also have an outsized impact in their disease fields if their approach to their role as a research funder is focused and disciplined.



Source: FasterCures questionnaire, Fall 2018.

## STAFFING AND EXPERTISE

Nonprofit staffs are typically lean, and many feel pressure to keep overhead expenses such as salaries under 20 percent to maintain favorable ratings from evaluation organizations such as Charity Navigator and GuideStar. This tension can result in a shortage of in-house expertise in areas such as investing, legal, and regulatory, though many can and do leverage expertise available through their boards and networks. We have observed that increasing numbers of patient organizations are hiring staff with MBAs or backgrounds in industry, creating business or management advisory boards to complement their scientific advisory boards, and, as they grow, hiring senior staff in a business- or alliance-development role to help drive effective partnerships.

## OUTREACH

One of the greatest assets that patient organizations bring to the table is their relationships with their patient communities. Many stay connected with tens or hundreds of thousands of patients through newsletters and other print or email communications, social media, online platforms or social networks for patients or caregivers, patient services, clinical trials recruitment efforts, and websites. One organization in our network noted in our questionnaire, however, that “We are a rare condition (only have 1,900 known diagnosed patients in the world [and] we have ways to reach most of them),” indicating that large numbers are not always the only or greatest value in terms of outreach to patients.

In recent years, we have observed some organizations with research-only missions realize the downsides of not cultivating a patient constituency when research partners actively look to them for patient data or participation in clinical studies. Patient navigation services, clinical trial portals, and social networks are some of the tools that organizations use to build their communities while providing actionable information to patients and researchers.

Almost 25,000 type 1 diabetes patients, caregivers, and supporters participate in an online community called Glu, created by the T1D Exchange. Their discussions led to crowdsourced citizen science that provides a fresh perspective for research as well as clarity around patients' unmet needs. This insight helps T1D Exchange make a strong case for research in those areas and overcome clinical inertia. Researchers also use Glu to collect patient perspective data in numerous ways, from simple polls and a "question of the day" to robust longitudinal studies. The community has also provided important input into the development of a new continuous glucose monitor, as well as on topics ranging from hypoglycemia to programming for camps for children with type 1 diabetes.

## ASSETS

Nonprofit foundations are ideally situated to fund the creation, maintenance, and expansion of infrastructure and resources to meet the needs of their fields, such as predictive animal models, interoperable research databases, comprehensive biobanks, patient registries, clinical trials networks or infrastructure, information technology platforms, and data standards and protocols. Effective research tools and resources are essential to expand available data sets and analytical capabilities that are necessary to accelerate and drive research from discovery to the clinic. Other research funders often lack incentives to develop such tools and resources that benefit the entire field. In addition, patient-driven foundations are often in the best position to engage patient populations in research and to know where and how they are being treated.

Given their unique role as representatives of patients' interests and perspectives, these organizations' policies for the use of their resources likely differ from those of commercial providers of resources such as data, tissue, or clinical trial recruitment.

They are likely to have a greater interest in the sharing of information and results, pre-competitive collaboration, and meaningful patient engagement. They should have guiding principles or other policies for the use of their resources that clearly articulate their requirements and reasoning.

## Identifying appropriate research partners

Some initiatives exist to define and categorize patient organizations' capacities and assets and are useful resources for researchers for identifying organizations with deep connections to the patient communities of interest. FasterCures' TRAIN (The Research Acceleration and Innovation Network) initiative provides a platform for patient organizations interested in learning about new approaches to their work from like-minded organizations and building relationships as key opinion leaders and partners with other stakeholders, such as industry and policymakers. Membership associations such as the National Health Council, Genetic Alliance, the National Organization for Rare Disorders, Global Genes, and the Health Research Alliance offer tools and resources. In collaboration with many of these groups and others, the National Center for Advancing Translational Sciences at the National Institutes of Health (NIH) created a Toolkit for Patient-Focused Therapy Development to capture a wide range of existing information that can help patient organizations understand the therapy development process and build their capacity to contribute to it from basic discovery through post-market approval.

Aimed at potential partners, the Genetic Alliance's Disease InfoSearch website includes self-populated profiles of disease-specific organizations, giving them the opportunity to indicate whether they can offer the following assets and capabilities.

Cohort Development	Disease Characterization	Research Management
<ul style="list-style-type: none"> <li>■ Registry of affected individuals</li> <li>■ Blood and tissue bank</li> <li>■ Clinical data</li> <li>■ Human genotype/phenotype data</li> <li>■ Human gene expression data</li> <li>■ Human epigenetic data</li> </ul>	<ul style="list-style-type: none"> <li>■ Is the gene identified?</li> <li>■ Is the protein identified?</li> <li>■ Is there an antibody available?</li> <li>■ Natural history or epidemiological studies</li> <li>■ Biomarkers</li> <li>■ Well-defined clinical endpoints</li> <li>■ In vitro model systems</li> <li>■ Animal models</li> <li>■ Is there a diagnostic test?</li> <li>■ Are there any lead compounds?</li> </ul>	<ul style="list-style-type: none"> <li>■ Link researchers and families</li> <li>■ Recruit participants</li> <li>■ Initiate and/or conduct research</li> <li>■ Award research grants</li> <li>■ Has intellectual property</li> <li>■ Provide information about clinical trials</li> <li>■ Conduct clinical trials</li> </ul>

Source: Genetic Alliance.

Perhaps the most detailed, practical effort to create a framework for researchers seeking patient group partners has been by the Clinical Trials Transformation Initiative (CTTI) as part of its Patient Groups and Clinical Trials project. Stating that “clarity is needed about how, when, and by whom patients or patient groups should be engaged during the therapy development process, and which patients or patient groups should be engaged,” it produced a set of recommendations for effective engagement. Accompanying the recommendations is an infographic of the many ways that patient groups can be engaged across the research continuum and a set of three tools, in the form of checklists or questionnaires, that sponsors can use to characterize patient organization skills and strengths and enable researchers to find partners with the expertise needed for their specific project.

CTTI’s checklist to assess patient organizations’ internal characteristics includes broad questions about their vision and areas of focus, operations, budget and fundraising, and communications. Externally, CTTI recommends that sponsors inquire about patient organizations’ relationships with other patient groups, academia, industry, patients, NIH, the FDA, and Congress.

CTTI’s and Genetic Alliance’s work provides an excellent foundation for researchers seeking to understand broadly the assets and capabilities that patient groups can bring to the research process. In [Part II](#) of this series, we make recommendations regarding additional resources that might be valuable in helping patient organizations advance their research readiness.

## II. Patient organization investments in health data

Although patient organizations are not the only sources of PGHD, some are engaged in data generation in multiple ways, directly collecting and housing data within their organizations, as well as sharing data through creative partnerships with medical product manufacturers, payers, academic researchers, platform companies, government agencies, and providers.

Rapid advances in web-based technologies and analytical tools have enabled patient organizations to deepen their understanding of and value to the patients they represent. Data aggregated through web-based platforms, mobile and in-home devices, and sensors can paint a richer picture of the types of and variation in patient symptoms and disease progression, as well as experiences between clinician visits, than can data from claims, electronic health records (EHRs), or randomized controlled clinical trials. Patient organizations are providing data to partners that shape and accelerate clinical research, enhance clinical practice, and empower patients in their own health care.

Patient-driven organizations such as the Global Healthy Living Foundation (GHLF) are investing heavily in these activities. GHLF's ArthritisPower™ platform provides information on clinical trials and enables patients, through "bi-directional data sharing," to track and share their symptoms, treatments, medications, and other health data from mobile technologies with their providers and with researchers. More than 15,000 patients are using the platform to view results over time, track changes in their symptoms, and identify causes of symptom change.

In fall 2018, 78 patient organizations responded to our questionnaire about their

interest and investments in patient data. Eighty-eight percent of respondents indicated that they had supported the creation or maintenance of a wide variety of data resources, which have been used primarily for discovery and observational research but also for preclinical and clinical research and post-market surveillance. Specific resources include patient registries, online platforms or social networks for patients and caregivers, patient-reported outcomes, biorepositories, natural history, gene sequencing data, and mobile health data collection and/or studies.

Respondents indicated they are investing in patient data resources because:

- They can aggregate data for a patient population across many institutions and derive unique insights,
- They have a unique level of trust with their patient communities,
- They are driven by the interest or request of their patient communities,
- These data are not being collected and/or shared by providers or researchers, and
- They need industry-standard information to de-risk investment in treatments for their diseases.

These data are valuable to a range of stakeholders (see Table 1)—for example, to researchers for clinical and health services research, to pharmaceutical and device companies to support innovation, to payers to support coverage and payment decision-making, to policy maker to understand the impact of laws and regulations on patients, and to the patients themselves to track disease progression and benchmark their symptoms and functional levels against others'.

In addition, through its MyHealthData and Blue Button 2.0 initiatives, the Centers for Medicare and Medicaid Services (CMS) is working to make more of its claims data available directly to beneficiaries who can then authorize third parties, including patient organizations, to use these data. Patient organizations and researchers can link the claims with registry, symptom, and other

data collected by the organizations to amplify the patient role in drug, biological, and device research.

PGHD collected by patient organizations offer the promise of more targeted interventions and enhanced clinical care. Patients can best evaluate assessments of the effectiveness of treatments and the value of improvements in specific symptoms.

**Table 1. Examples of Users and Uses of Patient-Generated Health Data**

Cohort Development	Disease Characterization
Researchers (e.g. academic, health services)	<ul style="list-style-type: none"> <li>■ Access larger, more diverse data sets</li> <li>■ Include in observational studies</li> <li>■ Pressure test hypotheses and methods, validate and interpret findings</li> </ul>
Patients and caregivers	<ul style="list-style-type: none"> <li>■ Coordinate care and shared decision-making</li> <li>■ Offer the opportunity to contribute to research</li> </ul>
Clinicians	<ul style="list-style-type: none"> <li>■ Provide a more holistic view of patient health over time</li> <li>■ Improve shared decision-making</li> </ul>
Product manufacturers	<ul style="list-style-type: none"> <li>■ Access larger, more diverse data sets</li> <li>■ Target trial recruitment efforts</li> <li>■ Improve trial design and conduct</li> <li>■ Include in observational studies/real-world evidence research</li> <li>■ Influence trial selection criteria, endpoints, symptoms, and disease burden</li> <li>■ Identify subtypes, prognosis, and signal detection for development of preventive therapies and symptom management</li> <li>■ Include in the product label</li> </ul>
Regulatory agencies (e.g., FDA)	<ul style="list-style-type: none"> <li>■ Evaluate product applications through the patient lens</li> <li>■ Develop more robust methods for signal detection and other post-market surveillance activities</li> </ul>
Payers (e.g., CMS, state Medicaid agencies, private payers) and value assessors (e.g., the Institute for Clinical and Economic Review)	<ul style="list-style-type: none"> <li>■ Acquire additional information for coverage decisions</li> <li>■ Produce better cost-effectiveness studies and value assessment</li> </ul>
Policymakers	<ul style="list-style-type: none"> <li>■ Inform the development of new policies governing which populations get access to new medicines and medical devices</li> <li>■ Modify policies on payments for medicines and devices</li> <li>■ Evaluate the impact of existing coverage and payment policies on specific sub-populations of patients</li> <li>■ Assess the effectiveness of treatments based on expanded sources of evidence</li> </ul>
Standard-setting bodies	<ul style="list-style-type: none"> <li>■ Inform standard determinations</li> <li>■ Enable creation of condition-specific data standards</li> </ul>

The National Psoriasis Foundation provided insights into patient subpopulations, including their perspectives and experiences with existing treatments, to the Institute for Clinical and Economic Review's (ICER) evaluation of psoriasis drugs in 2016, highlighting the complexity of the disease, challenges in its management, and its pervasive impacts. The foundation was able to influence ICER's conclusions, which reflect a substantial shift from its early positions and recommend that all treatments provide good value and that step therapy should be limited or abolished.

For researchers, the ability to link three major types of real-world data sources—claims, EHRs, and PGHD—is an evolving need and is key to future understanding of the natural history of disease and the development and adoption of new cures and innovations in clinical practice.

### III. Meaningful research engagement with patient organizations

To guide interested research partners, many organizations have developed frameworks, recommendations, and rubrics that address when and how to engage patients in research.

Meant as a quick guide, the table below distills key recommendations for meaningful patient engagement in research common to existing frameworks and materials.

#### Key recommendations for patient engagement in research

<b>Treat Patients as Essential Partners</b>	<i>Meaningful patient engagement built through trust and respect is critically important to ensuring effective partnerships with patients. Overall, meaningful patient engagement is variously described as “a real interaction and dialogue, not a ‘check-the-box’ exercise”,<sup>2</sup> treatment of patients as “essential partners throughout the research process and not token voices”,<sup>3</sup> partners as co-builders, and “an engagement experience [that] is informative, constructive, and mutually beneficial”.<sup>4</sup> In meaningful patient engagement, patients occupy a seat at the table as proactive partners, functioning not as trial subjects or as reviewers who react to already-developed materials but as integral members of research teams.</i>
<b>Establish Partnerships Early in the Process</b>	<i>Early partnerships allow partners to make full use of patient input in the planning stages of a research project, minimize resource and time-intensive backtracking and re-evaluations of decisions that occur after patient input highlights an inaccurate assumption or previous faulty decision, and build trust between the parties to engender a smooth working relationship.</i>
<b>Define Expectations, Roles, and Responsibilities</b>	<i>At the start of an engagement, the parties should “clearly define the expectations, roles, and responsibilities of all partners, including the resources being committed, data being shared, and objectives of the program.”<sup>5</sup> Some projects may require continuous involvement of patient partners throughout the project, whereas others may only need “touch points” at critical times as the project progresses. These criteria should be described in agreements between the parties or simple contracts and should be co-created by the researcher and patient partners.</i>
<b>Establish Fit-for-Purpose Collaborations</b>	<i>Ideally, all parties will share a sense of purpose, agreed on before the engagement starts. In addition, collecting patient input that is representative of the target patient population is important, and, for larger or more complex projects, might require engagement with multiple patient groups. Because patient groups differ with regard to size, resources, expectations, data assets, patient population reach, and experience working with researchers, the process of selecting appropriate patient partners includes matching patient group characteristics to the specific needs of the research program.</i>
<b>Measure Impact and Report Out</b>	<i>As stakeholders develop standard metrics to measure patient engagement, researchers should consider at the start of a patient partnership how the success of the collaboration will be measured. Both researchers and patients should establish feedback systems to gather data throughout the engagement process to measure its impact and mechanisms should be put in place to ensure a continuous feedback loop in which research results are provided to patients and the public.</i>

2. Perfetto and Oehrlin, “Assessing Meaningful Patient Engagement in Drug Development: A Definition, Framework, and Rubric, University of Maryland Center of Excellence in Regulatory Science and Innovation, (2015),” available at: <https://www.pharmacy.umaryland.edu/media/SOP/wwwpharmacyumarylandedu/centers/cersievents/pfdd/mcersi-pfdd-framework-rubric.pdf> (accessed Jan. 24, 2019).
3. Clinical Trials Transformation Initiative (CTTI) Recommendations: Effective Engagement with Patient Groups Around Clinical Trials,” (October 2015), available at: <https://www.ctti-clinicaltrials.org/files/pgtrecs.pdf> (accessed Jan. 24, 2019).
4. National Health Council & Genetic Alliance, Dialogue, Advancing Meaningful Patient Engagement in Research, Development, and Review of Drugs,” (September 22, 2015), available at: <https://www.nationalhealthcouncil.org/sites/default/files/PatientEngagement-WhitePaper.pdf> (accessed Jan. 24, 2019).
5. Bloom et al., “The Rules of Engagement: CTTI Recommendations for Successful Collaborations Between Sponsors and Patient Groups Around Clinical Trials, Therapeutic Innovation & Regulatory Science,” *Therapeutic Innovation and Regulatory Science*, 2018;52(2): 206-213, available at: <http://journals.sagepub.com/doi/pdf/10.1177/2168479017720247> (accessed Jan. 24, 2019).



Meaningful patient engagement offers significant value and can and should occur across the full research continuum and beyond. Existing materials that guide patients can help to maximize the value of patient input, optimize processes and outputs as efficient and patient-centered, and minimize burdens to research partners.

WANT MORE? READ [PART II: FOR PATIENT ORGANIZATIONS](#) TO LEARN MORE ABOUT:

- The role that patient organizations are playing as intermediaries for patient perspective and participation in research
- How patient organizations can improve their capacity as research partners
- How patient organizations' data can complement other data sources to capture a fuller patient experience in the "real world"
- The growing importance of shared data networks and the value of incorporating patient-generated health data in their research

## IV. Select list of patient engagement frameworks, recommendations, and rubrics and related materials

Canadian Institutes of Health Research, Strategy for Patient-Oriented Research (SPOR): Putting Patients First, Patient Engagement Framework (July 2, 2014), available at: <http://www.cihr-irsc.gc.ca/e/48413.html>.

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Frank et al., Conceptual and practical foundations of patient engagement in research at the patient-centered outcomes research institute, *Qual Life Res* (2015) 24:1033-1041, available at: [https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4412554/pdf/11136\\_2014\\_Article\\_893.pdf](https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4412554/pdf/11136_2014_Article_893.pdf).

Medical Device Innovation Consortium (MDIC) patient centered benefit-risk project report: A framework for incorporating information on patient preferences regarding benefit and risk into regulatory assessments of new medical technology, available at: [http://mdic.org/wp-content/uploads/2015/05/MDIC\\_PCBR\\_Framework\\_Web1.pdf](http://mdic.org/wp-content/uploads/2015/05/MDIC_PCBR_Framework_Web1.pdf).

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EveryLife Foundation for Rare Diseases, Draft Framework, Patients as Critical Partners in Rare Disease Drug Development: Establishing Disease Burden, Disease Measurement, and Benefit-Risk Assessments as Part of Rare Disease Drug Development, available at: <http://www.fastercures.org/programs/patients-count/science-of-patient-input-resources/>.

National Center for Advancing Translational Sciences (NCATS) Toolkit for Patient-Focused Therapy Development, available at: <https://ncats.nih.gov/toolkit>.

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<https://www.fastercures.org/assets/Uploads/PPVF-Version-1.0-Methodology-Report-Final.pdf>.

Patient Focused Medicines Development, The PFMD Book of Good Practices (May 2018), available at: <http://patientfocusedmedicine.org/bogp/book-of-good-practices.pdf>.

Bloom et al., The Rules of Engagement: CTTI Recommendations for Successful Collaborations Between Sponsors and Patient Groups Around Clinical Trials, *Therapeutic Innovation & Regulatory Science* (2018), 52(2): 206-213, available at:  
<http://journals.sagepub.com/doi/pdf/10.1177/2168479017720247>.

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