

**Engaging Patients in Information Sharing and Data Collection: The Role of Patient-Powered Registries and Research Networks**

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This report may periodically be assessed for the urgency to update. If an assessment is done, the resulting surveillance report describing the methodology and findings will be found on the Effective Health Care Program website at: [www.effectivehealthcare.ahrq.gov](http://www.effectivehealthcare.ahrq.gov). Search on the title of the report.

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## Introduction

There is limited reliable information regarding causes, progression, or treatment options for patients and family members coping with a variety of conditions. In some cases, limited information is a function of the rare nature of the condition and the scarcity of trustworthy data. In other cases, research on the condition has not focused on finding answers to the questions that patients most value due to challenges in collecting or analyzing such data, methodological constraints, or to limited researcher interest in such questions. Funding for such research across conditions may also be limited or virtually non-existent, which leads researchers to focus on other priorities.

In response, many patients and families have sought to contribute to the research process, hoping either to advance current options or to contribute to the options available for future patients. Many patients and their support and advocacy organizations seek increased attention or funding for focused research on conditions or treatments. Patients, family members, and consumer advocates also serve as advisors, informants, and partners with researchers on studies such as those funded by the Effective Health Care Program at the Agency for Healthcare Research and Quality (AHRQ) and the Patient-Centered Outcomes Research Institute (PCORI).

Patients are often encouraged by support and advocacy organizations to participate in research, particularly in clinical trials. Clinical trials, however, although an important source of new knowledge, may have a limited reach in garnering participants to yield a robust data set. Only about 6 percent of patients with a severe disease participate in clinical trials.<sup>1</sup> Patients may or may not fit the inclusion criteria for a clinical trial, and results from these studies can take years before being translated into useable information for patients and family members. More importantly, the nature of the clinical trial may or may not yield information that patients and family members find most relevant or useful in making treatment decisions.

Patient registries are additional or supplemental data sources. Registries use existing or contributed clinical data to provide information on “real world” practice and the effectiveness of treatments and procedures, and are to be distinguished from randomized controlled trials or scientific experiments that cannot measure real-life experience, in some cases, to the same degree.<sup>2</sup> Data included in patient registries may come from a variety of sources, including electronic medical records, clinician or patient reported clinical outcomes, diagnostic reports or images, hospital records, collected/donated blood or tissue samples, or questionnaires/surveys completed by clinicians or patients (or both). Traditionally, many of these registries are generated by researchers, funders, or institutions for scientific purposes. Such registries resolve many of the limitations posed by clinical trials, and offer new ways to answer salient patient questions. However, when managed by researchers, the registry may provide little or no opportunity for involvement or control by patient or family members or patient support and advocacy organizations. As a result, the registries may not meet the needs of patients and their caregivers.

To focus research more directly on patient and family member needs, patient and family advocates and organizations have created and operated “patient-powered” patient registries and research networks since as early as 1995. These registries and networks are distinguished from researcher-generated registries in that the registry (or network) and the research it yields is managed by patients and family members themselves, often through a disease advocacy organization or a network of organizations that receives advice and input from a scientific board of advisors.<sup>3</sup> Patient-powered registries (PPRs) and patient-powered research networks (PPRNs)

offer new directions for patient-centered outcomes research, and contribute to translational science in important ways. Experts agree that these registries are transforming patient/caregiver support and advocacy groups into research organizations.<sup>4</sup> They also provide patients and family members another way to become engaged in research beyond the role of advisor or informant to researcher-generated studies.<sup>5</sup>

This paper will describe PPRs and PPRNs and outline the considerations for patient advocacy and support organizations wishing to create or participate in these entities. The first section of the paper offers a definition and shared characteristics of both researcher-generated and patient-generated patient registries and research networks. The second section outlines the current pathways that exist for the creation of or involvement with a PPR and/or PPRN, and the advantages and disadvantages of each path. The final section reviews emerging issues in the rapid evolution of patient-powered registries and research networks.

## **Defining Patient Registries and Research Networks**

### **Patient Registries**

Patient registries have been defined as “an organized system that uses observational study methods to collect uniform data (clinical and other) to evaluate specified outcomes for a population defined by a particular disease, condition, or exposure, and that serves a predetermined scientific, clinical, or policy purpose(s).”<sup>6</sup> In brief, a patient registry is a collection—for one or more purposes—of standardized information about a group of patients who share a condition or experience. The use of “patient” in patient registries is often used to distinguish the focus of the data set on health information. Currently, there is no consistent definition of the term “patient registry” used in the health research field. Terms such as clinical registries, clinical data registries, disease registries, and outcomes registries are also used to describe the same data collection method.<sup>7</sup> Examples of individual researcher-generated registries can be found at the AHRQ Registry of Patient Registries, available at <https://patientregistry.ahrq.gov>.

### **Traditional Patient Registries**

Patient registries have traditionally been researcher-generated. Research institutions, academic clinical institutions, or individual research teams establish a registry, using private or Federal funds, for the purpose of observational data collection that can be used for a specific research agenda. These registries may be organized and operated in a variety of forms and formats. They may be operated by a single institution or by a collaborative of multiple institutions or clinics. Researcher-generated patient registries currently exist for a wide range of chronic or rare conditions, including many forms of cancer, diabetes, cystic fibrosis, acute coronary syndrome, and arthritis.

The purposes for patient registries can range widely. According to the National Institutes of Health, “Registries can be used to recruit patients for clinical trials to learn about a particular disease or condition; to develop therapeutics or to learn about population behavior patterns and their association with disease development; developing research hypotheses; or for improving and monitoring the quality of health care.”<sup>8</sup> Patient registries can also be used to monitor outcomes and study best practices in care or treatment.<sup>9</sup> They may pursue a specific, focused research agenda, collecting data for a limited time to answer a specific research question (or questions), or may collect data on an indefinite basis to answer a variety of existing and

emerging research questions. Patient registries may also include the collection of tissue or blood samples collected in a variety of ways.

The creation and use of researcher-generated patient registries has grown steadily for several decades, although the actual number of existing registries in the United States is unknown. In 2012, the Agency for Healthcare Research and Quality launched an online registry of patient registries to provide a searchable database of patient registries in the United States.<sup>10</sup>

## **Patient-Powered Patient Registries (PPRs)**

PPRs are similar in many ways to researcher-generated patient registries in definition, purpose, and features. At times, these registries are somewhat indistinguishable from traditional registries, with one exception: In patient-powered patient registries, patients and family members “power” the registry by managing or controlling the collection of the data, the research agenda for the data, and/or the translation and dissemination of the research from the data.

Experts in the field, however, differ in their individual conceptualizations of what constitutes a valid PPR. In the view of some, only registries that are created, maintained, and controlled by patients or patient advocacy organizations can be considered “patient-powered,” while others focus on the specific contributions of patients—that is, their involvement and contribution to all aspects of the registry—as the critical factor, regardless of the registry’s ownership or involvement by commercial or professional interests. These differences in conceptualization have added to the challenges of classifying PPRs for the broader research field. Terms such as “patient-generated,” “patient-run,” “patient-powered,” and “participant-controlled” can be found among various users.

Patient-powered patient registries are also organized and operated in a variety of forms and formats. They may be operated by a single organization or by a collaborative of multiple organizations. Like researcher-generated registries, patient-powered patient registries exist for a wide range of conditions.

PPRs may also pursue a specific research question or conduct ongoing data collection to answer a variety of existing and emerging research questions. Several PPRs have biobanks, or repositories, where patients can provide samples of blood or tissue to be used in research. Other patient advocacy organizations, such as the TMJ Association, use their registry as a recruitment vehicle for existing clinical trials, inviting members of the TMJ community whose profile matches a trial protocol.

Although the genesis of PPRs has not been studied or documented, most seem to originate from a patient support or advocacy organization, either as the direct intention of the organization or as an added component. The goal of these registries is to enhance translational research by providing data that could better characterize the disease, discover biomarkers, or provide information to assist patient and family decisionmaking.<sup>11</sup> In several cases, the creation of a PPR followed the formation of a support and information-sharing network of patients or families who shared a set of experiences with a disease or condition. The earliest documented PPR is the Hereditary Disease Foundation, created in 1983. While the support and advocacy network met a variety of patient and family needs, the foundation’s co-founder, Nancy Wexler, also collected samples from people who were affected by Huntington Disease for the purpose of advancing research.<sup>4</sup> An example of the typical genesis of a PPR can be found in the story of Pat Furlong, a registered nurse and the mother of two sons, Patrick and Christopher, who were both diagnosed with Duchenne muscular dystrophy when little was known about the condition. Refusing to accept that answers could not be found, she reached out to other families of the rare disease,

founded the Parent Project Muscular Dystrophy, and eventually created a patient and family registry, DuchenneConnect ([www.duchenneconnect.org](http://www.duchenneconnect.org)), which is both an information sharing network and registry.<sup>12</sup> An additional examples of patient-powered individual condition registries is the Life Raft Group Patient Registry and Tissue Bank (<http://liferaftgroup.org/patient-registry>), which collects data from patients affected by Gastrointestinal Stromal Tumor (GIST), a rare family of cancers.

Like researcher-generated patient registries, there is no single complete listing or documented number of PPRs in the United States. An effort to document patient-powered patient registries is being undertaken by the American Association for the Advancement of Science through funding by the Agency for Healthcare Research and Quality.<sup>13</sup> One study of 201 disease advocacy organizations found that forty-five percent had supported a research registry or biobank.<sup>11</sup>

## Concerns About Patient Registries

Patient registries have been promoted and praised in both chronic<sup>14,15</sup> and rare<sup>16,17</sup> disease practice communities, and debated among comparative effectiveness researchers as to whether they provide valid data to compare treatments.<sup>2,18-20</sup> Patient-generated patient registries in particular have been criticized on several levels, including the concern that only a small minority of patients with sufficient education and ability are able to participate, and that data may be biased for a variety of reasons.<sup>21</sup> Experts have noted the lack of standardization in data collection and potential competition for registered patients across registries, which could create a fractured set of patient data. For example, if multiple patient registries exist for a single condition, there is a greater likelihood that competition for patients may limit any given data set. This is a particular concern for rare diseases, where an affected population may be very small, resulting in small data sets for each registry that are less able to draw valid conclusions for the population. Meta-analysis across registries may be challenging or impossible should each competing registry collect different data from the same patients, or collect the data under different timeframes.<sup>16</sup> More specific concerns have been expressed regarding issues of patient consent and rights violations relative to tissue samples submitted to biobanks, in particular the concern about whether patients are fully aware of the possible subsequent uses of their specimens.<sup>22</sup> Despite these concerns, patient registries have yielded a significant amount of research that meets the needs of patients and families. PPRs have also added to disease and treatment knowledge. In addition to self-published monographs, several PPRs have used their data or had their data used in published research, although no formal list of publications across PPRs exists.<sup>4,11,23</sup>

## Research Networks

Some patient registries are part of broader research collaboratives that connect individual registries into a larger network of registries that collect data on one or more conditions. The network provides a shared infrastructure and standardized data collection across registries. Collected data from each registry may be combined for analysis, although participation in a research network does not eliminate the ability of any individual patient registry from analyzing only the data from the registry alone. Research networks may also have existing and emerging research agendas that are realized through an established collection of researchers who study the collective or registry-specific data based on the wishes of the patient network or each registry. In some cases, the network may make data available to researchers who request use for purposes that are unrelated to the agenda of those operating the registries.

Both researcher-generated and patient-powered research networks exist. An example of a researcher-generated research network is the SEER registries ([seer.cancer.gov/registries](http://seer.cancer.gov/registries)), a collaboration of 19 registries created and managed by the National Cancer Institute. An example of a patient-generated research network is the Genetic Alliance Registry and BioBank ([www.biobank.org](http://www.biobank.org)), a collaboration of more than 1,200 individual disease advocacy organizations.

There is little published literature to date on PPRNs, although several large networks have emerged in the past five years. A report created for the Patient-Centered Outcomes Research Institute (PCORI), developed a taxonomy of research networks in an effort to create an inventory of existing networks. The taxonomy classified three distinct categories: Clinical Data Research Networks (CDRN), Patient-Powered Research Networks (PPRN), and Patient Registries based on a set of distinguishing characteristics.<sup>24</sup> Although the taxonomy and characteristics vary somewhat from other descriptions and opinions, the taxonomy serves as a critical starting point for future research on the use of patient-powered networks and patient registries as data collection tools.

The Genetic Alliance Registry and BioBank (GARB) serves as an example of the evolution that can occur from an individual patient-powered disease registry to a PPRN. In 1995, PXE International, identifying itself as a “research advocacy organization,” created a patient-powered registry and biobank to accelerate translational research in *pseudoxanthoma elasticum*, a rare genetic metabolic disorder. The organization, led by founder Sharon Terry, became a mentor to a number of other organizations that also wished to create PPRs. This soon led to the formation of GARB in 2003 with eight disease advocacy organizations, using their infrastructure, model, and methods to create the broader network.<sup>4</sup> In a recent expansion of GARB, the registry platform has become disease-agnostic and now includes more than 2,000 diseases.

Other networks have had similar beginnings<sup>25</sup>. In 1998, Stephen Haywood was diagnosed with ALS (Lou Gehrig’s Disease). The Heywood family began searching across the globe for information that might extend or improve Stephen’s life. In 2004, frustrated by the lack of open and accessible information, Stephen’s two brothers, Benjamin and James Heywood, and a lifelong friend Jeff Cole, all MIT graduates, founded PatientsLikeMe with 12 disease communities.<sup>26</sup> In 2011, PatientsLikeMe opened to all conditions and now includes approximately 1,200 individual disease registries. PatientsLikeMe is a for-profit corporation.<sup>25</sup> A new not-for-profit network entitled Registries For All ([www.reg4all.org](http://www.reg4all.org)) provides a single platform available to all patient-powered disease registries. The network was formed through a partnership of organizations including GARB, CFIDS Association of America, National Psoriasis Foundation, and the Inflammatory Breast Cancer Research Foundation. The team was awarded \$300,000 in early 2013 from the Partners for Patient Health Innovation Challenge, funded by Sanofi US, an international pharmaceutical company.

It is important to note that PPRNs differ in their structure and operation. Some PPRNs such as GARB enable individual organizations and registries who are part of the network to maintain their autonomy and identity. Others, such as PatientsLikeMe, organize the network by disease communities that may be supported by multiple disease-advocacy organizations.

## Elements of Successful Patient-Powered Registries and Research Networks

Experts in PPRs and research network development and management share the belief that four fundamental elements are common to the development and management of a successful registry or network.

1. **Well-designed technology.** Critical to the success of a patient registry is the digital technology used to enable patients to join the network, report and store (and display) information, search for patients with similar experiences or conditions, and/ or link to other resources. The design of a successful virtual platform requires technical expertise, patient-user involvement, and significant funding. Models of effective technical solutions and platforms for registries and networks vary widely, and have not yet been formally evaluated or compared. Patient/caregiver use of the platform, however, serves as a critical guidepost for the success or failure of the registry or network.
2. **Recruitment, encouragement, and gratitude for participation.** Without exception, registry and network founders and managers point to the need for ongoing promotion of the registry to grow the membership and activate members to report data on a regular basis. Larger or more active patient support or advocacy organizations may be more likely to find success than organizations with small or limited constituencies, but all registries must regularly promote and encourage ongoing participation to maintain a robust data set. Most registries or networks send regular reminder emails encouraging members to report their latest symptoms, lab results, or changes in treatment, and/or to thank them for their participation. Others send specific requests for information relative to a study or a question raised either by the network or by a researcher who is using the network for data collection. Those managing the daily operations of registries and networks note that promotion takes a significant amount of their time and attention, as it is critical to the success or failure of each registry. They note that simply having a network is not sufficient; it must have a robust membership of active participants who are contributing to and using the information.
3. **Collaborative relationships with researchers.** To be effective as data sources for researchers—and ultimately meet the information or knowledge needs of patients and caregivers—registries and networks must work diligently to ensure that the data collected can be used for research. Some registries use scientific advisors or advisory teams to oversee the standardization of data or tissue collection. An example is the International Pemphigus and Pemphigoid Foundation ([www.pemphigus.org](http://www.pemphigus.org)) which has a large Medical Advisory Board of academic dermatologists. A second purpose for these advisors is their connection to similar researchers with interests in the condition or treatment who may serve as investigators on studies or connect the data set to other research products such as clinical trials, reports, or peer-reviewed articles. The establishment and maintenance of relationships with the scientific community often leads to affiliations with professional or condition-specific organizations, which may serve to advance connections with key scientists, particularly those interested and willing to work within the goals and interests of the registry in designing and conducting research.
4. **Partnerships with a broad range of stakeholders.** Many of the registries and networks make it their goal to establish collaborative relationships with similar

organizations for the purpose of sharing resources, avoiding competition for members, and reducing the fracturing of efforts to collect data, raise funds, or advance knowledge. For example, the DuchenneConnect lists more than 50 domestic and international partner organizations that promote the registry and contribute to its content. The Genetic Alliance is a network of more than 10,000 health organizations, of which 200 are disease-specific advocacy organizations that utilize its materials and help connect patients to the registries. Several experts suggest that the “network” of a PPRN must be significantly more than its patient or caregiver members. Attention to maintaining ongoing partnerships with a broad range of stakeholders is needed to be successful at all aspects of information-sharing and research.

## **Pathways to Creating a Patient-Powered Registry or Network**

The evolution of PPR and PPRN models and approaches is in its early stages and is changing rapidly. The initial decision of support and advocacy organizations to generate a PPR is now complicated by new opportunities. The two main pathways are:

1. Create an individual, stand-alone, single-condition PPR, using organizational resources and partnerships, or
2. Join an existing PPRN, using the preset infrastructure for a PPR that is connected to a variety of other registries.

Each option offers a set of trade-offs for the organization. Advocates of PPRNs note that existing PPRNs offer patient organizations many of the advantages of stand-alone PPRs but also provide greater efficiency and cost-effectiveness. Advocates for stand-alone PPRs believe that autonomy of decisionmaking is of greater value, and worry that an organization may be less visible within a larger collective. While the creation of a new PPRN is always possible, this option is the most costly and time-consuming, and requires existing partnerships with a variety of patient organizations, funders, research scientists, advisors, and other partners.

To simplify the choice, an organization may wish to consider the advantages and disadvantages of creating stand-alone PPR versus creating a PPR through a pre-existing PPRN. The advantages and disadvantages are discussed below; Table 1 provides an overview.

**Table 1. Advantages and disadvantages of PPR models**

Pathway	Advantages	Disadvantages
<b>Create a stand-alone PPR</b>	<ul style="list-style-type: none"> <li>• More focus on what is important to affected individuals</li> <li>• More control of design to meet needs</li> <li>• More control of data, including use and sharing</li> <li>• Potentially higher participation rates</li> <li>• More opportunities to promote the organization</li> </ul>	<ul style="list-style-type: none"> <li>• Higher costs</li> <li>• More resources needed for promotion</li> <li>• Sole responsibility for data management and use</li> </ul>
<b>Join a PPRN</b>	<ul style="list-style-type: none"> <li>• Lower costs</li> <li>• Greater promotion of the network across populations</li> <li>• Reduced fracturing of patient sample</li> <li>• Greater ability to do research across conditions or address concomitant conditions</li> </ul>	<ul style="list-style-type: none"> <li>• Less autonomy/control of design</li> <li>• Less ownership of data</li> <li>• Potentially less brand or name recognition<sup>a</sup></li> </ul>

**Abbreviations:** PPR = patient-powered registry; PPRN = patient-powered registry network.

<sup>a</sup>Some PPRNs allow member organizations to maintain their identity and brand, eliminating this disadvantage.

**Advantages of creating a stand-alone registry:**

- **More focus on what is important to affected individuals.** Clearly, the autonomy that comes with a self-generated registry offers the most flexibility to collect data that are most relevant to the needs of patients and family members of the individual disease. Data collection can focus on specific research questions, and results can be reflected back to organizational members in ways that are most meaningful to their situation.
- **More control of design.** Organizations that create their own registries are able to control every aspect of the registry, from the type of information collected to the look and feel of the PPRN. The organization has complete freedom to innovate, developing and maintaining a registry that best meets the needs and interests of the organization and its constituency.
- **More control of data.** Organizations that create their own registries have more control over how the data are used in research, including the partnership model the organization wishes to employ with researchers, scientists, and other stakeholders. The stand-alone registry may have greater flexibility in designing the memorandum of understanding that governs data sharing with researchers.
- **Higher participation rates.** While high participation rates are not inherent in stand-alone registries, and several PPRs that live within a PPRN also show high rates of participation, there are several good reasons to assume higher rates through stand-alone PPRs. Stand-alone PPRs live firmly within their parent organization, and benefit from the trust and affinity to the organization as a key location for information, support, and involvement. Patients and/or family members are more likely to participate in the registry when they are able to connect it to a familiar host organization. Stand-alone PPRs can provide regular communications about what is happening with their data, and can incorporate the registry more directly into other aspects of the organization, utilizing a captive, well-connected audience to promote participation. Hybrid networks such as GARB also allow organizations to retain their brand and autonomy in order to enhance participation.

- **More opportunities to promote the organization.** Similarly, a stand-alone PPR is most likely to be directly associated with a disease advocacy organization (often by name, as was the case with PXE International)—a connection that facilitates the organization’s promotional efforts and adds to its value. Organizations are able to use their registry to promote other aspects of the organization easily, using the registry membership to cross-promote other important activities or initiatives. Stand-alone PPRs that are part of PPRNs which allow the organization to retain their individual identities may realize this benefit as well.

**Disadvantages of creating a stand-alone registry:**

- **Higher costs.** Building a digital platform for a patient registry can be expensive—three to four hundred thousand dollars at a minimum. Creating a PPRN can be even more expensive and would require more resources for building collaborations with other PPRs. Annual costs of maintaining a PPR can vary, and are less likely to reflect the efficiencies of a shared infrastructure. Organizations must raise funds specifically for this purpose, and can wind up circumventing their autonomy in decisionmaking in an effort to solicit funders or partner organizations to pay for the generation or maintenance of the PPR.
- **More resources are needed for promotion.** Self-generated PPRs must rely on their parent organization to call attention to their registries and maintain active participation from members. Central to a registry’s success is its ability to attract patients and/or family members who will contribute information or samples. If multiple patient registries exist for a condition, then the organization may find itself competing for the attention of patients and researchers. The organization will need to set aside a larger amount of resources to promote the registry in order to ensure that it contains a robust representation of the population.
- **Sole responsibility for data management and use.** Stand-alone patient registries face sole responsibility for addressing the many issues that accompany self-reported and clinical data, from the reliability of the data to the protection of tissue samples in collection and storage. Expensive contracts with research repositories may be needed to ensure proper collection. Staff or external vendors may also be needed to translate data into information that can be shared with the network, such as aggregate summaries of experiences or treatment outcomes, or conclusions.

**Advantages of joining a pre-existing PPRN:**

- **Lower costs.** Pre-existing networks and registries have already made the investment in technical infrastructure and design, saving patient organizations the high cost of these tasks. These PPRNs are not cost-free to patient organizations, however, and may have a set of fees associated with the addition of a condition population to the network, the inclusion of data in research projects, or fees for other aspects of inclusion. However, these networks are more able to create an economy of scale that provides cost savings for each participating PPR.
- **Greater promotion across populations.** Existing PPRNs attract new users in a variety of ways that often exceed the resources of an individual organization. Although each advocacy or support organization may need to supplement its own promotional efforts to ensure member engagement, the general promotion of the PPRN may assist greatly in the recruitment of individuals with a specific condition.

- **Reduced fracturing of the patient sample.** Having a large, multi-condition PPRN reduces the likelihood that a population with a specific condition will be split across several stand-alone registries, and thereby creates a more robust data set for research.
- **Research across conditions/concomitant treatments.** One potential advantage of multi-condition PPRNs is their ability to combine data from multiple conditions, so that researchers can study treatment effects or other shared experience across diseases. Another potential advantage of these combined condition data is the ability to analyze comorbidities and concomitant treatments, as these may be reflected in the aggregate data set.

#### **Disadvantages of joining a pre-existing PPRN:**

- **Less autonomy/control of design.** Any advantage organizations may gain in financial savings may be offset by the loss of autonomy in registry design and data selection, although the degree of this disadvantage differs among PPRNs. Some PPRNs offer a standardized design and data collection template, and may not allow for significant modification. Although pre-existing networks all differ in their policies and approaches, autonomy to make independent decisions and policies may be limited, and will always involve negotiation with additional partners.
- **Less ownership of data.** Another important consideration surrounds the ownership of data, which may live with the PPRN rather than the individual organization joining the collaborative. While this may not be an issue and can be negotiated, organizations “joining” a pre-existing PPRN must realize that, as a participating organization, they may not have ownership of the data or samples their members provide and as a result may not have full discretion regarding their use.
- **Less brand/name recognition.** One common motivation to create a stand-alone PPR surrounds the ability to “name” a registry after an organization, connecting the PPR to the organization directly. This may be somewhat lost when a PPR is part of a larger PPRN, which tends to be recognized by the name of the PPRN and not by the individual organizations that have collaborated to create it. Some PPRNs, however, allow individual member organizations to maintain their identity and brand, which minimizes this effect.

## **Emerging Issues**

While PPRs and PPRNs remain at an early stage of evolution, several emerging issues will need study and resolution:

- **Standardization of data collection.** Both researcher-generated and patient-powered patient registries are moving toward standardization of data collection, ranging from the standardization of specific items to the standardization of entire survey instruments. Stand-alone PPRs, in their attempt to answer patient-relevant questions, may not have data that can be widely used outside those specific research questions, limiting interest from a range of researchers. Unlike clinical research institutions, PPRs lack access to certain standardized data collection points such as electronic medical records, although attempts are being made. PPRNs have standards for their own network, but no formal standardization across these networks has been established with the exception of a few that are using the NIH set of common data elements, which was established by multiple institutions. As these networks grow, the issue of standardization, particularly in contrast to traditional patient registries, will be important to resolve.

- **Competition for patients and caregivers.** Many experts believe that the addition of stand-alone PPRs increases the opportunity for additional fracturing of data collection efforts across the patient population. Multiple PPRs for both rare and chronic conditions can force competition for active engagement by patients who may choose between registries, thereby reducing the size of the data set, or who may participate in several registries at once, thereby increasing the challenge of aggregating data for the entire population.
- **Quality of data.** A final yet significant issue surrounds the acceptance and use of patient-reported data in the research community. While the publications generated from PPRs and PPRNs play an important role in changing current perceptions, patient-reported outcomes such as those sometimes collected in PPRNs are disregarded by some (though not all) in the academic and research community as biased, inconsistent, and unable to provide a sufficient basis for valid analysis. A deeper concern surrounds the use of online communities as the basis for PPRs or PPRNs, although some PPRs have correlated their data with medical records and found a high concordance. A 2011 article generated by data from PatientsLikeMe included this limitation:

Finally, when collecting data from patients online, there is the distinct possibility of more egregious misrepresentation—namely, that users are not who they appear to be. Patients on the site could be falsifying their identities entirely. While this is always possible, certain Internet platforms may be at higher risk for these gross inaccuracies than others. In many websites built specifically to collect medication ratings from patients, users enter minimal information about themselves before entering treatment evaluations, thus lowering the barrier for misrepresentation. PatientsLikeMe, as a community based on ongoing interaction and a reputation built upon a time-based health profile, may be less susceptible to flagrant misrepresentation.<sup>27</sup>

Organizations using PPRNs for research purposes with the intention of changing current clinical practice will need to continue to address this issue directly, working with researchers to assure the quality of the data collected and to promote the use of the data across the research community. For several PPRNs, clinical practice change has been accomplished through the use of the data collected by the network.

## Conclusion

Without question, patient-powered patient registries and networks are a rapidly evolving contributor to research, and particularly to research that focuses on direct improvements in practice. These entities blur traditional boundaries, breaking the barrier of patient, family, and advocate involvement and control in research, translation, and dissemination. A clear movement has emerged to connect individual patient organizations and single-condition patient registries into broader networks that unify, standardize, and optimize the data collection and research generation process. Yet, incentives remain for organizations that wish to pursue their own agendas and create independent single-condition patient registries for their constituencies, which may or may not be possible when joining a PPRN.

A number of factors in the current health care environment may influence further evolution of patient-powered research, including the increased attention and interest in patient-centered research evidence, the expanding role of patients in institutional research efforts, and new technological advancements that enable new forms of patient outreach and engagement. It is likely that the options and choices for patient advocacy and support organizations wishing to contribute to research will expand. Yet, it is also likely that best practices and standards will emerge that guide both researcher-powered and patient-powered registries and research networks, providing additional knowledge to organizations seeking the most efficient and effective approaches to generating knowledge about conditions and treatments.

## Resources

Two resources are available for organizations wishing to pursue PPRs. These include:

- Glicklich RE, Dreyer NA, eds. *Registries for Evaluating Patient Outcomes: A User's Guide*. Second edition. AHRQ Publication No. 10-EHC049. Rockville, MD: Agency for Healthcare Research and Quality; 2010.

While this guide was created for researchers and institutions creating researcher-generated patient registries, much of the process is valuable for disease advocacy organizations wishing to create a self-generated PPR. Organizations should note, however, the intended audience, and recognize that some information may need modification for PPRs.

- The Genetic Alliance Registry and BioBank (GARb) Toolbox serves as a map of tools and resources for those interested in starting or maintaining a registry and biobank or joining GARb. It aggregates resources from hundreds of credible sources. Individuals interested in starting a registry and biobank will find resources such as a start-up guide, strategic planning aids, and Institutional Review Board (IRB) guidance, available at [http://biobank.org/toolbox/start\\_a\\_biobank](http://biobank.org/toolbox/start_a_biobank). Webinars on topics such as marketing, governance, and new technologies are available for the more advanced registry and biobank manager. GARb also offers a monthly bulletin that contains the latest news about registries and biobanks, and hosts a listserv for those interested in discussing registries and biobanks.

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## About This Paper

This white paper is part of a series of reports, information projects, and activities for the Community Forum, an effort to improve and expand public and stakeholder engagement in research supported by the Agency for Healthcare Research and Quality's (AHRQ) Effective Health Care Program. Funded initially under the American Recovery and Reinvestment Act (ARRA), Community Forum activities seek to:

- Build on existing efforts to involve stakeholders—clinicians, patients, caregivers, health services researchers, health care payers, and others—having a specific interest in AHRQ's EHC Program research.
- Advance the science for obtaining input from both stakeholders and the general public in the development and dissemination of health care research.

A series of key informant interviews was conducted from October through December of 2012. Key informants were identified as those working with patient-powered registries as creators, administrators, or researchers. From these calls, a Technical Expert Panel was assembled. The Technical Expert Panel included individuals recommended by key informants who represented a broad range of perspectives and experiences with PPRs. The panel reviewed a preliminary outline of this paper, provided additional information and insight toward its content, and reviewed a draft of this paper.

## Technical Expert Panel

- **David Clifford**, Public Health and Government Affairs, PatientsLikeMe
- **Terrie Cowley**, Founder and President, TMJ Association
- **Guy Eakin**, Vice-President of Scientific Affairs, American Health Assistance Foundation
- **Patricia Furlong**, Founder and President, Parent Project Muscular Dystrophy and Duchene Connect patient registry
- **Michael Manganiello**, Founding Partner, HCM Strategists
- **Richard Sharp**, Director of Bioethics Research, Cleveland Clinic
- **Sharon Terry**, President and CEO, Genetic Alliance and Registries for All
- **Suzanne D. Vernon**, Scientific Director, CFIDS Association of America