



EXPANDING THE SCIENCE OF PATIENT INPUT:

# Building Smarter Patient Registries



**FasterCures**  
A CENTER OF THE MILKEN INSTITUTE



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## ABOUT FASTERCURES

*FasterCures*, a DC-based center of the Milken Institute, is driven by a singular goal—to save lives by speeding up and improving the medical research system. We focus on cutting through the roadblocks that slow medical progress by spurring cross-sector collaboration, cultivating a culture of innovation and engaging patients as partners. This report is released under *FasterCures*’ program called Patients Count: The Science of Patient Input, which aims to improve health by expanding opportunities for patients’ perspectives to shape the processes by which new therapies are discovered, developed and delivered. Find out more at [www.fastercures.org](http://www.fastercures.org).



# INTRODUCTION

**ACROSS THE BIOMEDICAL RESEARCH AND CARE ENTERPRISE IS EVIDENCE OF AN ACCELERATING SHIFT TOWARD PATIENT-CENTEREDNESS.** The creation of the Patient-Centered Outcomes Research Institute (PCORI) by Congress in 2010, the Food and Drug Administration's (FDA's) Patient-Focused Drug Development initiative begun in 2012 and a strong commitment from the White House and the National Institutes of Health (NIH) to make patients full partners in the Precision Medicine Initiative that was announced in 2015 are just a few of the sign posts indicating that patient needs and expectations are shaping the national research agenda.

Decision-makers in research, industry, policy and health-care settings are actively seeking robust sources of patient data to inform patient-centered practices, policies and outputs. Scientific rigor throughout this process is of paramount importance to ensure solid outcomes. *FasterCures* is at the forefront of the burgeoning science of patient input, aimed at establishing rigorous methods and reliable practices for understanding and

incorporating patient needs into the process of developing, regulating and delivering new therapies.<sup>i</sup> **ONE OF THE EXISTING TOOLS FOR LEVERAGING PATIENT INPUT IS THE PATIENT REGISTRY, "AN ORGANIZED SYSTEM OF COLLECTING UNIFORM DATA FOR A POPULATION DEFINED BY A PARTICULAR DISEASE OR CONDITION THAT SERVES ONE OR MORE PREDETERMINED SCIENTIFIC, CLINICAL OR POLICY PURPOSES."**<sup>ii</sup>

*FasterCures* examined the landscape of patient registries created or held in trust by patient-led foundations with the following three objectives:

- 1 **ASSESS** the state of patient-led patient registries as a robust source of patient insights and actionable data to meet emerging opportunities,
- 2 **EVALUATE** use of patient registries by patient-led organizations as a surrogate measure of readiness for the expanding emphasis on patient-centricity and
- 3 **IDENTIFY** information and practices that would enhance existing patient registries and could inform the creation of new ones.

This report ties together research from interviews, *FasterCures*' survey data, background research and data gathered at events, including a "Disruptors' Academy" session focused on registries held at our 2015 Partnering for Cures conference. In this three-part report, we present key learnings, topline results from our survey and emerging best practices in the form of important considerations, checklists, resources and case studies. This guide is intended to be a starting point for learning more about patient registries. Through our **PATIENTS COUNT: THE SCIENCE OF PATIENT INPUT** program, *FasterCures* will continue updating, enhancing and augmenting the tools in these pages.



## PART I: KEY LEARNINGS

Five key themes emerged from our research and analysis:

### ① Patient registries are evolving rapidly.

**PATIENT REGISTRIES INFORM NATURAL HISTORY STUDIES, ASSIST CLINICAL TRIAL RECRUITMENT, FACILITATE SAFETY MONITORING, ALLOW PATIENT PARTICIPATION IN RESEARCH AND MUCH MORE.** While

many patient foundation-led registries started within the last decade as simple “contact registries” that tracked patients using basic spreadsheets, they are developing into robust sources of data that can strengthen the full arc of biomedical research and care delivery. Affordable technology is enabling constant upgrades to create versatile online data repositories with powerful analytical tools. Given this rapid evolution, the term “patient registry” seems inadequate and a bit outdated. We agree, although we’ve used it in this report for the sake of simplicity.

### ② Success = careful planning + active upkeep.

Organizations routinely underestimate the time and resources required to launch, maintain and maximize benefits from their registry. Establishing sound governance policies, deter-

mining what information to collect and attracting registry participants and end users are the most challenging steps. Being aware of and ready to address these challenges helps foundations prioritize resources needed to build an effective registry.

### ③ Trust is essential, but not enough.

The unique position of trust held by patient-led foundations among patient and research communities enables them to create robust registries. But goodwill must not be taken for granted or substituted for responsive customer service. Similarly, registry sponsors should “trust but verify” the capabilities of contractors for registry platform services and other handlers of registry-generated data.

### ④ Patients expect to be partners.

In exchange for sharing their data and experiences, registry participants want to understand how their insights are used and what outcomes they generate. Some registries provide participants with real-time feedback on how individual data points compare to those reported by other

**As much as this space has changed in recent years, momentous change is on the horizon.**

participants. But, across the board, we heard that regular reporting on registry outcomes needs to be more routine. Better reporting is an important tool for attracting and keeping participants—the top challenge patient foundation-led registries face.

### ⑤ Opportunity will knock. Be prepared.

As patient data increase in value, registries are positioned to meet growing needs to collect real-world data about the patient experience. Patient preferences, health-care costs and adherence to care regimens represent areas of expanding interest that could be captured in patient registries. Nontraditional customers for these data include regulators, payers and policy-makers; understanding and meeting their standards will be important for registry sponsors. As much as this space has changed in recent years, momentous change is on the horizon.



## PART II: SURVEY METHODOLOGY AND TOPLINE RESULTS

Through *FasterCures'* TRAIN (The Research Acceleration and Innovation Network)—our network of patient-focused non-profit organizations—and desktop research, we identified 110 patient foundation-led registries. Of these registries:

- 73 (66 percent) are focused on rare diseases;
- 12 (11 percent) are focused on one or more types of cancer;
- 20 (18 percent) are part of PCORI's Patient-Powered Research Network, part of the National Patient-Centered Clinical Research Network, or PCORnet (see page 21 );

- 34 (31 percent) are sponsored by organizations in TRAIN and
- more than 10 different technology platforms support these registries, with the largest concentration being on PatientCrossroads' platform (see Appendix for further information on various platforms).

To learn more about the state of foundation-led registries, in September 2015 we conducted a 30-item structured survey via email to these 110 organizations. Outreach was amplified by messages sent through

PCORI's Patient-Powered Research Network and by PatientCrossroads. Survey questions captured information about registry operations, strategy and outputs. Question formats allowed respondents to augment multiple choice selections with open text to collect additional detail and allow clarification of answers. We received 45 responses from 38 unique registries with a high completion rate. Additionally, we conducted telephone interviews with some respondents to gain additional details about their experience starting or running a registry.

### IMPORTANT TO NOTE...

Registries for rare diseases were over-represented in the survey responses, with 80 percent of respondents reporting on experience with rare disease registries, compared with 66 percent in the reference sample. A bit of historical context helps to explain this predominance of rare diseases. Organizations formed by highly motivated parents of children with rare, lethal genetic conditions, including the Cystic Fibrosis Foundation and Parent Project Muscular Dystrophy, were early pioneers in the use of patient registries to lower barriers to research by academic and industry researchers. Their early successes helped galvanize support from the NIH Office of Rare Disease Research, Genetic Alliance, the National Organization of Rare Disorders (NORD) and pharmaceutical companies specializing in rare disease therapies to fuel registry development by patient-based organizations.



**“BUILD YOUR REGISTRY WITH A PURPOSE IN MIND”** was the most common piece of advice offered by registry veterans. Defining a purpose from the outset helps to drive decision making. About 40 percent of survey respondents reported that the primary purpose of their registries at inception was to understand the natural history of the disease or better characterize the patient population. This defining purpose helped them select appropriate registry functionality.

**OVER TIME, THE PURPOSE OF THE REGISTRY MAY EVOLVE TO FIT CHANGING NEEDS OF A PATIENT OR RESEARCH COMMUNITY.** For example, seven survey respondents reported that the purpose of their registry had shifted over time to “facilitating patient participation in research,” which reflects the growing emphasis on more patient-centered research. It’s important to note that several respondents identified a struggle with having to select a single primary purpose, reflecting that registries may have multiple aims.

**ORGANIZATIONS REPORTED THAT ENGAGING WITH PATIENTS TO ENROLL THEM IN A REGISTRY WAS HARD WORK.** In fact, the number one challenge that respondents faced—named by 56 percent of them—was attracting participants. Additionally:

- Seventy-nine percent of respondents listed recruiting and enrolling participants as a high priority for resources allocated to their registry.
- Participation in the registry exceeded expectations for just 14 percent of respondents. Participation by the affected community met expectations for 25 percent of respondents and fell short for 37 percent; the final third said that it was too soon to judge.
- Organization events and publications, social media, referral by specialty clinics and invitation/referral by enrolled participants were all rated as “very important” to at least 50 percent of responding organizations in promoting registry participation.

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#### CHARACTERIZING RESPONDENTS’ REGISTRY PARTICIPATION:

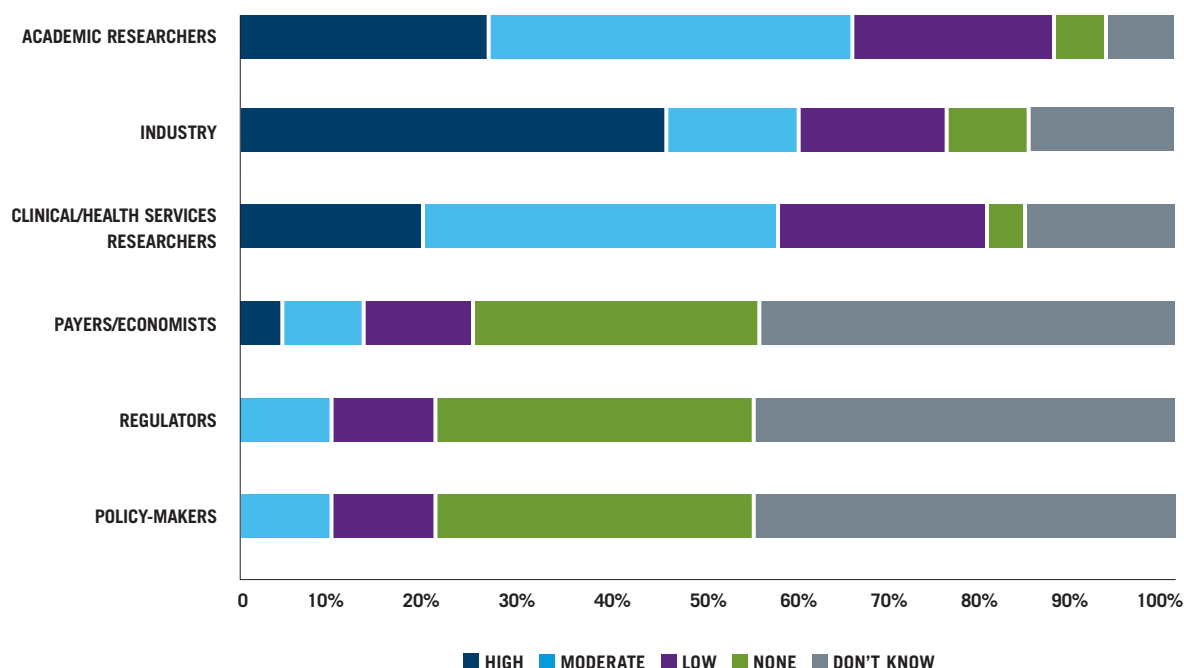
- **47%** had 1,000 participants or fewer
- **44%** had between 1,001 and 5,000 participants
- **2** registries had more than 10,000 participants
- **MORE THAN HALF** reported that the number of participants was greater than or equal to 0.5% of the estimated affected population for their condition of interest (a target established by PCORI)



Most of the registries reporting—89 percent—receive data directly from the patient or caregiver. Sixteen percent can also receive information from the participant’s clinical care team. Most of these registries limited the amount of information required at the time of enrollment to standard demographic information (71 percent), with 20 percent also requiring personal medical history information at enrollment. Forty percent of respondents reported that their registry was able to integrate with electronic health records, although a few noted that they had not yet determined the best way to incorporate and make use of these data.

Organizations’ relationships with industry and other end users of registry data varied. **SURVEY RESPONDENTS FOUND THAT THE SECTOR FROM WHICH THEY HAVE SEEN THE GREATEST INTEREST IN REGISTRY DATA WAS INDUSTRY** (pharmaceutical, biotech and device companies); however, when asked if sponsorship from for-profit companies provides any direct revenue to the organization, only six respondents said that it did. This trend shows that engaging with industry is important and can be valuable, but that it may not be a sufficient source of revenue to fully support registry operations. In fact, generating adequate financial support for registry operations ranked third among the challenges faced by respondents. Only five respondents said that they generated revenue by charging fees to those accessing registry data.

#### SURVEY RESPONDENTS REPORTED VARYING INTEREST IN THEIR REGISTRIES ACROSS SECTORS





## RESPONDENTS USE A VARIETY OF APPROACHES TO ENTICE RESEARCHERS TO USE REGISTRY DATA, INCLUDING:

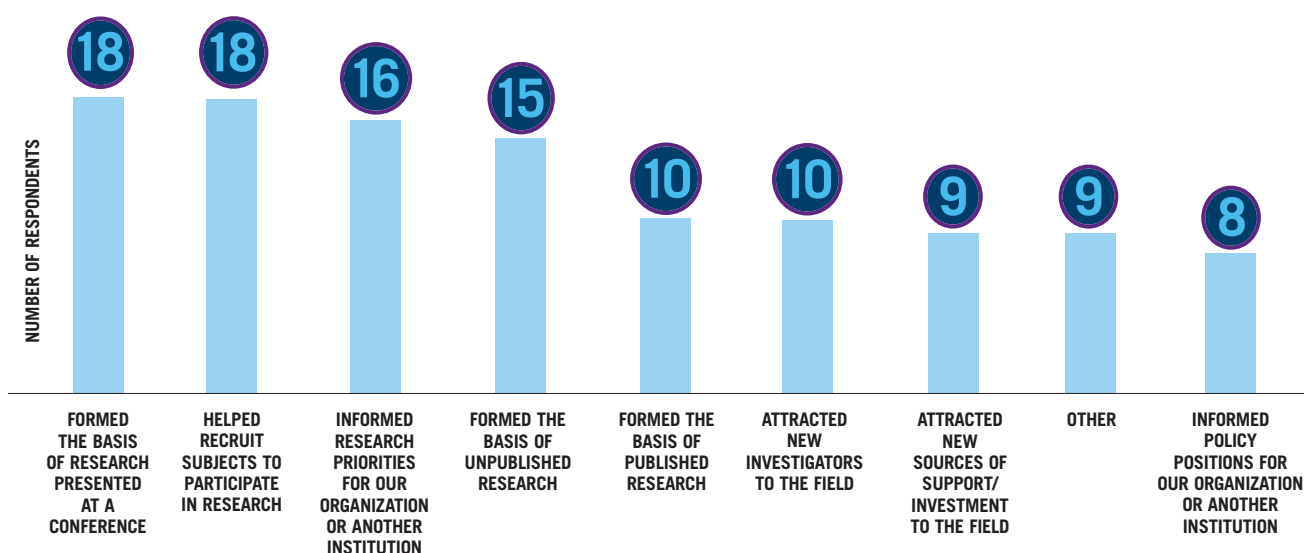
- 1 publishing periodic data summaries from the registry (used by 55 percent of respondents),
- 2 promoting the opportunity for external parties to direct queries or surveys of registry participants (48 percent) and
- 3 exhibiting at professional society/trade conferences about the availability of registry data (48 percent).

Just 12 percent reported providing grants or funding to researchers specifically to utilize data from the registry.

Registries enabled or accelerated a wide variety of outcomes. Sixty-eight percent reported that the registry helped recruit subjects to participate in research, 63 percent said registry data formed the basis of research presented at a conference and 58 percent said the registry informed the research priorities for their own organization or another organization. Several noted that their registries were too new to have yet seen these types of outcomes but aspired to be able to report such successes in the future.

## REGISTRY DATA LEAD TO VALUABLE OUTCOMES

*According to survey respondents*





## PART III: EMERGING BEST PRACTICES FOR REGISTRIES

**HOW CAN REGISTRIES ADVANCE THE SCIENCE OF PATIENT INPUT?** In this part of the report, we present topics that arise in the course of planning and running a patient-led registry. For purposes of organizing the information, we have staged it along a timeline from ideation to full implementation. However, as noted above, many aspects of scoping and operating a registry are interdependent, and issues addressed at the initiation of a registry must be revisited periodically.

### GETTING STARTED

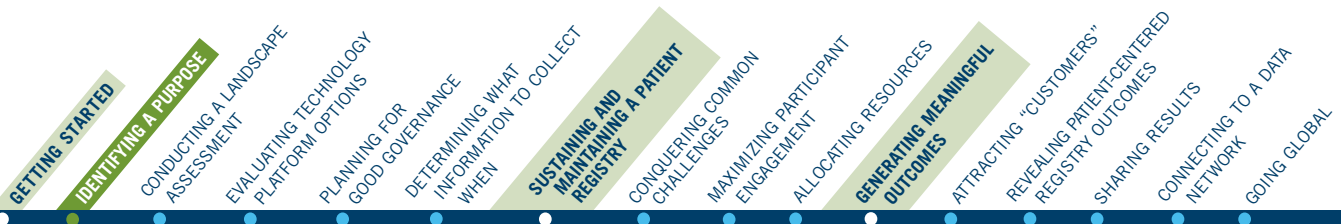
#### Identifying A Purpose

**“BEGIN WITH THE END IN MIND” IS PERHAPS THE MOST CRITICAL ADVICE TO FOLLOW WHEN STARTING A PATIENT REGISTRY.** Registry creation should be anchored by defining a purpose or small set of purposes. Jumping on the bandwagon of other organizations’ registry successes is not likely to be an adequate purpose on its own.

Identifying the primary purpose for the registry will aid decision-making about the platform, governance structure, information to collect and potential end users to target. Having a clear purpose will also help engage patients and attract funders. Additionally, the registry’s purpose will help to define which data sources may be optimal for integration.

#### PURPOSES TO CONSIDER WHEN STARTING A REGISTRY

- Facilitate patient participation in research
- Understand the disease course over time or natural history of a disease or condition
- Better characterize the disease or condition (symptom expression, phenotype, genotype)
- Support clinical trial matching and recruitment
- Collect patient-reported outcomes
- Compile genetic information
- Follow patients undertaking a specific intervention
- Inform research priorities
- Identify subgroups
- Ensure patients are receiving care according to model guidelines
- Understand lifestyle factors
- Amass data to advocate for expanded insurance coverage for therapies and services





## Conducting a Landscape Assessment

Patient foundations are in a unique position to catalyze patient-centered research, as outlined in our publication, “Honest Brokers for Cures: How Venture Philanthropies are Changing Biomedical Research,”<sup>iii</sup> and a patient registry may serve this mission. But building a registry might not be right for every organization and may not be necessary or feasible for every disease or condition.

Most organizations wrestle with the challenge of a patient community’s needs that far exceed available resources. Revisiting the organization’s mission and priorities can be a helpful first step in determining whether starting a registry is the best next step. Take stock of staff, volunteer and financial resources so you can evaluate how well they match up against the assets you’ll need to effectively launch, build and maintain a registry. Our publication, “Measuring and Improving Impact: A Toolkit for Nonprofit Funders of Medical Research,”<sup>iv</sup> includes an extensive set of organizational assessment tools.

Occupying a position of trust within the patient community is essential to launching a registry. It also helps to have positive working relationships and active communication with key influencers within the community, including high-profile patients, bloggers, researchers and clinical care providers. Understanding your community’s readiness to engage in research is an important factor as well.

Finally, assess the current scientific and medical challenges and opportunities. Consider the other nonprofit, academic, government and industry infrastructure, resources and capabilities being deployed to ensure that a new registry would enhance and complement existing assets.

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## KEY QUESTIONS TO ANSWER TO ASSESS THE LANDSCAPE

- 1 Does starting and maintaining a patient registry align with our mission and goals? What other organizational priorities might be enhanced or undermined by committing to a registry?
- 2 Do we have the necessary staff, volunteer and financial resources to build and maintain a registry?
- 3 Does our organization occupy a position of trust with the patient community that we wish to engage?
- 4 How much community education about the research enterprise will we need to do in order to support the need for and benefits of a registry?
- 5 Do we have constructive relationships with key influencers that can help to promote registry participation?
- 6 What threats to building a successful registry are we most concerned about? How can we mitigate these threats?
- 7 Do any registries (including international ones) already exist within our disease space? Be sure to investigate registries housed in clinical settings, academic centers and by industry. And if registries exist, are they collecting all of the information necessary to facilitate patient-centered research?
- 8 Might collaboration with an existing registry be possible or preferable to starting a new registry?
- 9 What end users would we hope to engage with registry data and how strong are our existing relationships to them? Will they be likely to value patient-reported data?
- 10 What data standards might end users have in order for registry data to be useful to their decision-making?

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## Evaluating Technology Platform Options

Selecting a technology host for a registry depends on goals and budget. Several platform options exist, depending on your organization's technical expertise, financial resources and registry purpose (see Appendix for descriptions of some of the most popular platforms). If financial resources are an immediate barrier, consider free platforms. Alternatively, an organization may have specific research goals that warrant securing sufficient funding to build a proprietary platform to meet those goals.

Established platforms have the benefit of routine operations and existing templates that can speed implementation, especially for organization staff that may not have deep technical expertise or registry experience. The tradeoff may be in the level of customization available, both in data collection and reports accessible to the organization. Established platforms have the benefit of connecting to other registries using that same platform, providing the opportunity to facilitate research across diseases with appropriate terms and conditions to guide data use.

Platforms built specifically for the organization will require in-house or contracted technical expertise, both in the construction of the registry and to support its ongoing operation. Anticipate the long-term costs for upgrades to software and hardware in assessing sustainability.

When contracting with any platform provider or service, make sure to understand the data ownership and security terms and conditions. Patient privacy measures and control over use of their data may vary. Data ownership and privacy will be an important element in attracting and retaining participants.

Finally, it's always prudent to have experienced legal review of contracts and long-term obligations.





## QUESTIONS TO ASK WHEN EVALUATING PLATFORMS

### PRIVACY

- Do you want participants to have the ability to establish unique privacy settings, or invoke a uniform privacy standard for all?
- Is the platform Health Insurance Portability and Accountability Act (HIPAA)-compliant?
- Will your registry contain information from patients in jurisdictions outside the United States? If so, is the platform equipped to handle that information in a way that is compliant with privacy regulations of those countries?

### SECURITY

- What steps has the provider of the platform taken to prevent and protect against data breaches?
- Is the platform Federal Information Security Management Act (FISMA)-compliant?

### ACCESSIBILITY

- Who will be able to access the data?
- What type of data will those with access be able to see?

### OWNERSHIP

- Has the platform provider outlined who owns registry data, and is that in alignment with the foundation's mission?

### EASE OF USE

- Who will be inputting data into your registry?
- Given your primary users, is the interface user-friendly? Does it comply with Section 508 standards for people with physical, sensory or cognitive disabilities, if compliance is important to your community members' access?

### COSTS

- What resources will you need to initially build a registry on the platform, and will that achieve the foundation's goals within your budget?
- Are the proposed fees and payment schedule for building the registry and maintaining it clear? Are additional fees for customization or specialized services spelled out in the proposal?

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## Planning for Good Governance

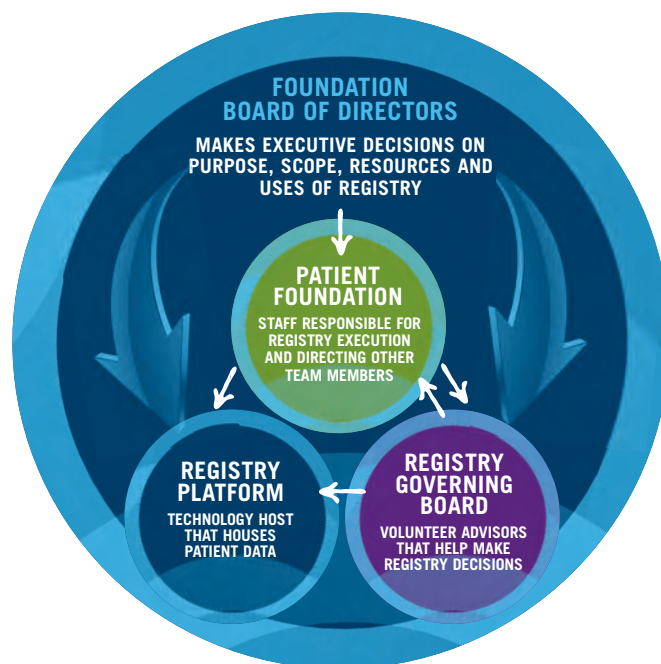
Patient registries should develop and follow a written registry governance plan that articulates what expertise is needed and how decisions will be made, executed and monitored. Registries sponsored by 501(c)(3) organizations are subject to the governing policies of that organization, with ultimate fiduciary responsibility and accountability for the registry lying with the organization's Board of Directors. Registries may be subject to external governing policies as well, such as HIPAA<sup>v</sup> and the Common Rule.<sup>vi</sup> Additionally, a balance of in-house staff expertise, volunteer participation and contracted services may be required to address the specialized operational, scientific, ethical and strategic decisions that may arise.

Coordinating governing bodies while assembling all the skill sets required to build and operate a successful registry can be daunting for foundation staff. Many registries find it useful to convene a dedicated registry steering committee or advisory board. This is an excellent way to engage with representatives of registry stakeholders, including patients and caregivers, who should be central to governance activities. Additionally, consider the end users (often academic or industry researchers) you would like to engage with through your registry. Your registry will be more valuable to the end user community if you have that community represented on the governing

board. You may also find it useful to recruit individuals with legal and ethical training to participate in governing the registry.

As with other aspects of nonprofit governance, registry governance should be considered a dynamic process, re-evaluated periodically and as circumstances change. Depending on organizational culture and the availability of appropriate expertise, term limits for volunteer participants can help protect against insular decision-making.

### WHAT KIND OF STRUCTURE CAN THE GOVERNANCE OF A PATIENT REGISTRY TAKE?





### SOME DECISIONS THAT A GOVERNING BODY CAN GUIDE:

- Allocation of overall organizational resources to the registry, including a staffing plan
- Process for due diligence in the selection of a registry platform provider or contractor
- Plan for protecting patient privacy, including compliance with HIPAA and patient identity management (such as use of a Global Unique Identifier, or GUID)
- Informed consent processes and documentation and compliance with other requirements of Institutional Review Boards (IRBs)
- Data to be collected at each stage of the enrollment process and at what frequency they will be updated
- Data quality assurance provisions, including use of established data standards (see page 21)
- Policies for sharing outcomes and analyses of registry data with participants, the patient community and the public
- Policies for granting access to registry data and analyses to researchers and other end users
- Plans for integrating the registry with other data sources, including electronic health records, mobile data sources, biobanks, etc.
- Engagement and marketing plans including enrollment targets, schedule of planned contacts, rescue plans to address engagement challenges and assessment of participant burden over time
- Terms and conditions for sponsorship of registry studies by external parties, fee schedules and related terms to monetize registry assets
- Contingency plans for ending or transitioning the registry in the event of completion of the identified mission, unsustainable growth or other limiting factors<sup>viii</sup>

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## Determining What Information to Collect When

One of the biggest decisions registry sponsors face is what information to collect at which time points and in what format. There are perpetual tradeoffs between the burden placed on the participant and what can be an insatiable quest to document the complete patient experience. The registry purpose (described on page 7) will help drive decision-making about what information is most relevant to collect. Information collection will also be driven by who is expected to provide information, whether it is the patient him/herself, a caregiver or a health-care professional.

As indicated above, one benefit of establishing a registry steering committee or advisory board can be to obtain multiple perspectives on questions related to data collection throughout the registry's lifetime. Registry platform providers may also be able to advise on customary levels of data collection based on experience with other groups.

As a general practice, we learned that most registries establish rather minimal information requirements at the time of enrollment, often limited to general demographic information and basic health status. This establishes a low barrier for registration, but may present a challenge for getting more detailed information on every participant.

A key to collecting additional data over time is preserving the opportunity to recontact participants with additional information requests through the initial informed consent process. This enables patients to opt-in to requests for new, updated or more detailed information over time.

### TYPES OF INFORMATION TO COLLECT FROM PARTICIPANTS

- Standard demographic information
- Personal medical history
- Family medical history
- Current and past medication use (consider providing a list of commonly prescribed medications with branded and generic names)
- Physical examination findings
- Symptom questionnaires
- Results of laboratory, imaging and/or functional tests
- Standard function and/or quality-of-life measures
- Disease-specific function and/or quality-of-life measures
- Information about social, behavioral and environmental factors
- Electronic health record (EHR) (possibly by providing one-time authorization via a health-care provider's electronic portal)
- Costs associated with care

See also the National Health Council's "Patient Perspectives on Disease Impact and Treatment Options: A Stratification Tool."<sup>viii</sup>

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## SUSTAINING AND MAINTAINING A PATIENT REGISTRY

### Conquering Common Challenges

The biggest challenges that patient foundations face throughout the lifespan of a registry center on patient engagement and funding. Often, patients are initially excited to get involved in registries; however, their interest can wane if surveys are too burdensome, if messaging gets stale or if they don't feel informed about how their information is advancing the cause. We provide some specific strategies for engagement in the next section.

As anyone connected to a nonprofit knows, funding challenges aren't unique to patient registries. Some registries are initiated with expectations for the ability to generate revenue by securing large grants, selling data or contracting with industry for clinical trial recruitment. Funders across the board told us that they frequently encountered unrealistic expectations for the amount or longevity of support for registry operations. Taking a portfolio approach that blends grants, contracts, user fees, charitable donations and sponsorship to support registry costs is pragmatic. Funders may have different funding objectives, and it's smart to align your requests with their interests.

#### TOP OVERALL REGISTRY CHALLENGES REPORTED BY SURVEY RESPONDENTS

- Attracting participants
- Maintaining up-to-date information about participants
- Attracting adequate financial support for registry operations
- Addressing data quality issues
- Re-engaging participants for further research opportunities/ data collection
- Attracting researchers to utilize the registry data
- Mobilizing registry data to inform clinical practice

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## Maximizing Participant Engagement

Since participant engagement is such a large challenge, patient foundations are finding innovative ways to encourage registry participation. Here are a few thriving practices to consider:

### **GAMIFY OR USE BADGE OR REWARD SYSTEMS TO INCENTIVIZE PARTICIPATION AND PROFILE COMPLETION**

Citizen Pscientist, the National Psoriasis Foundation's global online research network, uses this approach to great effect. Gamifying registries makes completing surveys fun for participants. They use badges to create a sense of friendly competition with other users and keep participants coming back for more.<sup>ix</sup>

### **IMPLEMENT USER-CENTERED DESIGN**

The C3N Project has developed user personas to represent the key user motives and actions of different groups of potential users. They use these personas when designing new projects or processes. User personas provide the project team with a clear design target that focuses their decisions on how to meet the needs and motivations of various users.<sup>x</sup>

### **LEVERAGE SOCIAL COMMUNITIES TO KEEP PARTICIPANTS INVOLVED**

Glu, T1D Exchange's online community, provides individuals with type 1 diabetes a safe place to participate in discussions with fellow patients, receive and offer support, share and access educational materials, and participate in research

via surveys and clinical trial matching. A "question of the day" keeps participants actively engaged to learn from one another. This social support community engages patients on a regular basis to ease in the collection of longitudinal data.<sup>xi</sup>

### **CONNECT REGISTRY INVOLVEMENT TO THE BIG PICTURE**

DuchenneConnect, hosted by Parent Project Muscular Dystrophy, continuously updates a public list of research that uses registry data. Publicizing this research incentivizes the community to continue to support the organization (by contributing data or funding) to help it achieve the mission of bringing better treatments to all boys with Duchenne muscular dystrophy.

### **INTEGRATE MULTIPLE DATA SOURCES TO CREATE A "ONE-STOP-SHOP" FOR PATIENTS AND CAREGIVERS**

IBD Plexus, a project of the Crohn's and Colitis Foundation of America, is building a data exchange to house electronic health record data, patient-reported data, biobank samples and more. The database will organize the vast amount of data and provide different user interfaces for researchers, patients, caregivers and clinicians. Having a complete, connected source for all of these data will allow the registry to be the go-to disease management resource for inflammatory bowel disease patients, incentivizing them to log in and answer health-related questions whenever they are looking for other health information.<sup>xii</sup>

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## ENGAGEMENT LESSON

TripAdvisor was founded in 2000 to improve travel for people entirely through user-generated content. Over the past 15 years, TripAdvisor has perfected the engagement of travelers reviewing destinations and of representatives from those destinations responding to traveler reviews.

As of late 2015, TripAdvisor housed about 250 million reviews from 84 million reviewers. Each minute, TripAdvisor receives 160 new user reviews that destinations respond to, often within 24 hours. The motivation for this user-input data is similar to patient registries—helping the community.

How did TripAdvisor achieve this high level of voluntary user engagement? Users experienced the value of the data. All reviewers began as users of the site, and the reviews helped them to plan great travel experiences. On the destination side, the hotels, restaurants, resorts, etc. reviewed on the site are able to assure travelers that any negative feedback in a review is being addressed to help attract and grow business.<sup>xiii</sup>

Patient registry sponsors might learn from this model. Patient communities will want to be engaged with a registry when they see the value that the registry provides. Showcase outcomes to help attract and engage patients and researchers.

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## Allocating Resources

Registries are resource-intensive. In order to maintain a registry that continues to be impactful and facilitate research, organizations must consider and plan for all of the costs registries require, both at start-up and over the lifetime of the registry.

Organizations also must remain realistic about the funding potential of registries. As recognized above, registries can open new funding streams for patient organizations, but few are entirely self-sustaining through a single source of funding.

### KEY COSTS TO CONSIDER

- Technical costs: platform access, data storage, platform and Web changes
- Staffing: organization staff and consultant costs
- Marketing and communications resources: outreach to both potential and active patient participants as well as potential end users
- Legal counsel: review of terms and conditions of data use agreements and compliance with federal and state laws
- Data quality assurance: administrative support and technical expertise to monitor and curate data
- Globalization: translation into various languages and compliance with international regulatory bodies

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## GENERATING MEANINGFUL OUTCOMES

### Attracting “Customers”

Attracting customers to your registry is crucial to achieving the impact that your registry seeks to accomplish. Engaging patient populations requires a great deal of focus and resources; however, engaging end users is equally important to ensure that the data collected serve a purpose beyond sitting in a repository.

#### SURVEY RESPONDENTS' TACTICS FOR STIMULATING REGISTRY DATA USE

- Publishing periodic data summaries
- Promoting and advertising the ability for external parties to direct queries or surveys
- Exhibiting at professional society/trade conferences about the availability of registry data
- Contracting with external researchers to conduct studies the organization sees as important
- Issuing funding opportunities for projects that utilize registry data
- Posting data sets on open research platforms
- Issuing data challenges or prizes to stimulate data analysis

### Revealing Patient-Centered Registry Outcomes

With these complex considerations, it may be easy to lose sight of the benefits of patient registries. Registries have immense potential to accelerate and improve research outcomes for patient and research communities—outcomes that are fully based on patient perspectives. Patient registries enable an enriched understanding of the experience of living with a disease or condition. The data they hold can reveal **disease burden, the patient journey, unmet medical needs, patient preferences, natural history, subgroups and patient-centered outcomes and endpoints.**

#### REGISTRY DATA OFFER THE POTENTIAL FOR PATIENT-CENTERED ALIGNMENT ON:<sup>xiv</sup>

- Target product profile
- Methods, materials or measures that aid drug development, including patient-reported outcomes
- Clinical trial design and operations
- Structured benefit-risk assessment
- Regulatory submissions and decisions
- Patient support programs
- Value strategy
- Formulary selection
- Coverage policy
- Quality measures
- Clinical practice guidelines
- Comparative effectiveness

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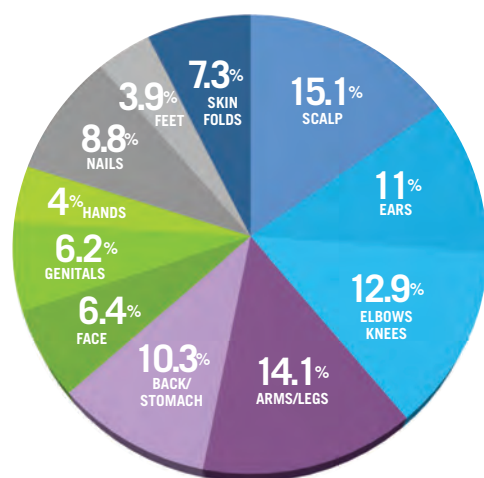
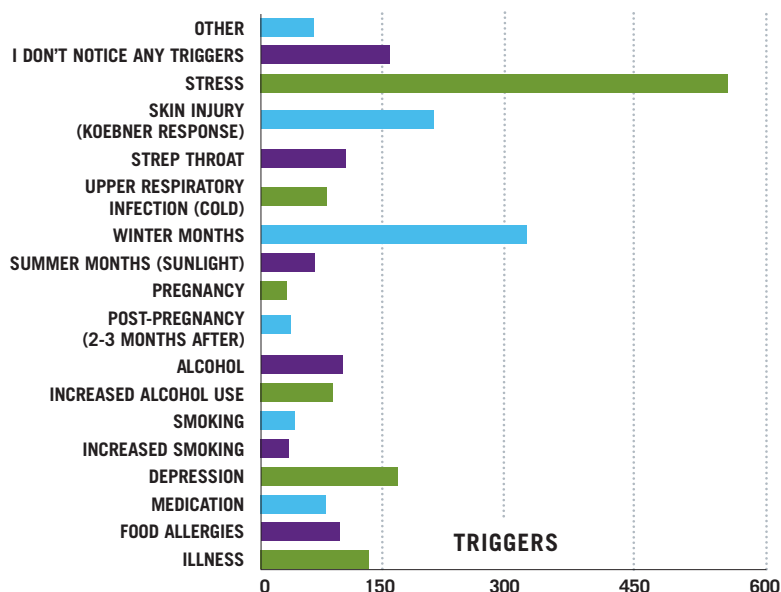
## Sharing Results

Communicating about registry outcomes with participants is an effective tactic for keeping participants engaged. It is also crucial to maintaining the organization's position as a trusted, honest broker within your organization's patient community.

Practices vary among registry hosts for reporting results to participants, with some registries able to provide real-time data to individuals about how their responses compare to other participants. Some registries release information about outcomes on a regular schedule (monthly, quarterly or annually), while others issue updates as soon as outcomes are presented at conferences or published in journals. Keeping participants apprised of interim progress with enrollment, studies underway and platform upgrades can also be motivating milestones to build a sense of community involvement and achievement.

### THE VALUE OF SHARING REAL-TIME DATA

Citizen Ps scientist shares real-time data, allowing participants to view answers to research questions asked by fellow participants and to pose new queries. Hypotheses generated by participants are then made publicly available at citizenps scientist.org. For example, users posed hypotheses about triggers and where on the body psoriasis occurs:<sup>xv</sup>





## Connecting to a Data Network

Being a part of a broader data network is important because it allows researchers to look across diseases to identify patterns and shared features. They can conduct studies and run queries that lead to unique connections and insights.

Three networks to be aware of are the network of registries using the PatientCrossroads registry platform (see Appendix for additional information), the NIH's National Center for Advancing Translational Sciences (NCATS) Global Rare Disease Registries (GRDR) Program and PCORI's PCORnet.

The goal of the GRDR program is to build a Web-based resource that integrates, secures and stores de-identified patient information from many different registries for rare diseases. The data are mapped to GRDR program common data elements and other national standards.<sup>xvi</sup>

PCORI has invested \$250 million to develop PCORnet, a national patient-centered clinical research network, which aims to aggregate national data sourced from a range of health-care settings (including local hospitals, doctors' offices and community clinics) into a large, highly representative national network for conducting patient-centered comparative effectiveness research. PCORnet is working to combine data from electronic health records, patient-generated information and other sources such as insurance claims. Once they are combined, the data will be made

“research ready,” so that health researchers can conduct studies across the data sets included in the network and achieve high-quality outcomes from those studies.<sup>xviii</sup>

### PLAN AHEAD TO CONNECT TO OTHER DATA SOURCES

- Global Unique Identifier: Since registry data are generally de-identified when shared, registries can assign a GUID or a universal subject ID to each unique entry to connect a patient's data across a data network without exposing personally identifiable information. Examples include the GRDR, National Database for Autism Research and Federal Interagency Traumatic Brain Injury Research GUIDs.
- Clinical data standards: The use of data standards allows for registry data to be easily integrated with other databases using the same standards. Some examples of these standards include:
  - Clinical Data Acquisition Standards Harmonization (CDASH)
  - Clinical Data Interchange Standards Consortium (CDISC)
  - Medical Dictionary for Regulatory Activities (MedDRA)
  - Systematized Nomenclature of Medicine (SNOMED)
  - Unified Medical Language System (UMLS)
- Data-sharing consent and policies: If the registry intends to share data across a network, the registry must obtain participants' consent. As noted in the governance section above, the governing body should establish data-sharing policies that address legal and ethical concerns, data access and ownership permissions, and establish the format in which data will be shared.<sup>xix</sup>

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## Going Global

Globalizing a registry is often warranted, particularly for rare disease registries where patients are few and geographically dispersed around the world. Going global presents several challenges that organizations may not have previously considered.

Different laws, research practices, regulatory environments and languages pose a challenge for registries looking to expand into other countries. Translation of registries, in particular, is time-consuming and can be quite expensive. It requires translating the consent, questions, answers, end user interface and results; cultural context may be important as well. Every update to the registry requires new translations. IRBs and other regulatory boards may need to be consulted with each change. Rather than going it alone, seek out partner organizations active in countries where you'd like to expand to assist with these efforts. Working with international nonprofits can help you ensure global versions of your registry are culturally appropriate and in compliance with international laws. Partnering can also help with outreach, fundraising and services delivered in those individual countries.

### THE IMPORTANCE OF ENGAGING INTERNATIONAL PARTNERS

The Phelan-McDermid Syndrome Foundation realized the immense challenge and expense associated with creating an international registry. However, it also realized that an international registry was critical for its research community, given the limited number of patients in the United States with the disease. The U.S.-based foundation recognized that the only way to move forward would be to collaborate with global partners. The foundation promised to do the “heavy-lifting” for creating a registry—deciding on governance, questions, consent, setting up the platform on PatientCrossroads, etc. It then engaged international partners to locate funding in other countries to handle the expensive translation. It now has several partners, including in Spain (Asociación del Síndrome de Phelan-McDermid) and Italy (Associazione Italiana Sindrome di Phelan-McDermid).<sup>xx</sup>

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## CONCLUSION

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Patient foundations are ideally suited to capitalize on growing interest in patient data by building high-impact patient registries. Registries give patients a direct means to participate in the research process, leveraging their input and insights to focus priorities and outcomes across the discovery-development-delivery continuum. Creating and maintaining a patient registry requires a variety of skills and expertise as well as firm commitment to meet immediate and long-term challenges. The unique position of trust held by patient foundations and their deep content knowledge make them ideally suited to overcome these challenges. Foundations also employ registries as a tool to de-risk research investment and to facilitate research that more closely aligns with patient needs and aspirations.

We anticipate the continued growth of patient registries, as foundations, technology and policies facilitate even greater levels of patient participation to transform the biomedical system. Through our Patients Count program, *FasterCures* will cultivate smarter patient registry practices to enrich the science of patient input.





## APPENDIX I: PLATFORM DESCRIPTIONS

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### **Genetic Alliance's Platform for Engaging Everyone Responsibly (PEER):**

Genetic Alliance created this registry platform to facilitate participant-centric patient foundation registries. PEER has a novel privacy control system that allows participants to be selective about their privacy settings. As users answer questions, dynamic graphs and charts appear to show how the user's answers compare to the rest of the participant population. PEER has a tiered fee structure, ranging from free for registries with fewer than 100 participants to \$25,000 per month or more for more than 100,000 participants.<sup>xxi</sup> As of January 2016, PEER served as the registry platform for 12 organizations.  
[www.geneticalliance.org](http://www.geneticalliance.org).

### **National Organization for Rare Disorders Natural Histories Patient Registry:**

NORD's registry platform prioritizes documentation of natural history data to provide researchers and regulators with an understanding of how rare diseases develop. The platform was first launched in 2014. Since that time, NORD has received support from the U.S. Food and Drug Administration (FDA) to further build out the registry. The in-house staff at NORD helps organizations using the platform with user retention and engagement, governance questions and concerns, technological specifications and more. The platform is continuously being adapted to fit user needs. NORD's platform costs \$500/month for hosting, maintenance, technology support, etc. As of January 2016, NORD's platform served as the host for seven organizations with the intention of using FDA funding to expand to 20 organizations through a lottery system.  
[www.rarediseases.org](http://www.rarediseases.org).

### **PatientCrossroads:**

PatientCrossroads is a versatile platform that helps foundations manage their registries. All of the information collected by the platform becomes part of a broader network of data that are de-identified and accessible (by subscription) to companies and researchers. Participants create an account, provide consent and input demographic data. Then organizations can send surveys out to some or all participants. The platform is in the process of linking with electronic health record data by having participants sign in to their patient portal log-ins while in their PatientCrossroads account. PatientCrossroads staff helps to clean and verify patient-reported data to ensure accuracy. PatientCrossroads CONNECT is free for organizations who wish to start and market a registry. Foundations can increase registry features for different fees, depending on the services. As of January 2016, PatientCrossroads served as the registry platform for 75 registries, representing 300 diseases and partnering with 100 advocacy groups.  
[www.patientcrossroads.com](http://www.patientcrossroads.com).



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**Research Electronic Data Capture**

**(REDCap):** REDCap is a free, Web-based software solution that was created in 2004 at Vanderbilt University. The platform is widely used by the academic research community, primarily for clinical and translational research, although it is versatile and can collect any kind of data. Question style and content can be customized to meet organization needs. Additionally, the organization has direct control over all aspects of the REDCap system, allowing the database to meet varying privacy and security needs. More advanced database analysis knowledge may be needed to build the database and participant surveys as well as to analyze data for the organization's purposes. As of January 2016, REDCap had 1,720 active institutional partners (connected via the Project REDCap consortium) in 96 countries and 219,000 projects with over 305,000 users. [www.projectredcap.org](http://www.projectredcap.org).

**Unitio:** Unitio is a nonprofit organization that offers a data exchange platform that brings together patients, researchers and physicians to accelerate and improve treatments. Unitio was launched after the team at T1D Exchange saw the importance of building a strong patient, caregiver, researcher and physician community. The team decided to build a platform that would be available to other organizations wishing to create similar data exchanges. Unitio's fee structure is not yet publically available. As of January 2016, Unitio hosts T1D Exchange's CARE platform (a peer-to-peer support community), clinic network, clinic registry and biorepository. Additionally, Unitio hosts the Leukemia and Lymphoma Society's CARE community. [unitio.org](http://unitio.org).

**Propriety platforms:** Consulting groups and contract research organizations can help organizations build a platform specific to the needs of the patient and research communities. Examples include Quintiles (for example, the Muscular Dystrophy Association's U.S. Neuromuscular Disease Registry), Deloitte Consulting (for example, the IBD Plexus Large Data Management Platform) and Corrona (for example, the Corrona Psoriasis Registry hosted in collaboration with the National Psoriasis Foundation).



# APPENDIX II: PROFILES OF SELECTED PATIENT REGISTRIES

T1D EXCHANGE	
PURPOSE	T1D Exchange aims to help researchers overcome the many obstacles required to accelerate all aspects of drug and device development for type 1 diabetes patients.
DEFINING CHARACTERISTICS	<p>T1D Exchange is comprised of a clinic network, a clinic registry, a biobank and an online patient and caregiver community called Glu. The T1D Exchange Clinic Registry collects data from more than 26,000 individuals with type 1 diabetes. The registry is hosted through the platform Unitio. The data can be searched publicly at T1D Discover. T1D Exchange collaborates with patients, industry and investigators to minimize barriers and inefficiencies for clinical and translational research to improve outcomes for people living with type 1 diabetes.</p> <p>T1D Exchange was launched in 2009 with support from the Helmsley Charitable Trust. The staff of the exchange wanted to share the functionality of T1D Exchange with other disease communities, so they decided to make the platform, Unitio, more broadly usable. Unitio, launched in 2015, is a nonprofit dedicated to connecting researchers, physicians and patients to save time and improve patient outcomes.</p> <p>T1D Exchange has seen numerous outcomes from its registry since 2009. Research with data from the exchange has demonstrated diverse outcomes, including the use of a therapy to improve glycemic outcomes in obese adolescents with type 1 diabetes. Data from the exchange have also shown a lack of improvement in young adults’ management of their disease, pinpointing a need for new devices to facilitate disease management. Registry data have supported and will continue to support numerous other studies.<sup>xxii</sup></p>



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## CITIZEN PSCIENTIST

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### PURPOSE

Citizen Pscientist's mission is engaging psoriasis patients with research, driving National Psoriasis Foundation (NPF) research priorities and defining research questions.

### DEFINING CHARACTERISTICS

Citizen Pscientist was launched in 2015 and enrolled approximately 2,500 people in its launch. The Citizen Pscientist platform was built specifically for this registry and was designed to be fun and easy to use to encourage participant engagement. At its launch, Citizen Pscientist had four NPF staff members dedicated to making sure that the registry supports psoriasis patient priorities in research.

The Citizen Pscientist governing board – made up of researchers and patients – helps to determine the survey questions pushed out to participants. Questions are never free text, which makes curation and analysis easier. As participants respond to questions, they see registry data in real time, and they have the power to ask research questions and draw hypotheses from the data.

Data are also shared with National Psoriasis Foundation research partners who are engaged via the governance committee. These research partners can perform studies on the data.

The next steps that NPF is looking to take with Citizen Pscientist involve implementing a strategy to ensure continued engagement from participants after initial enrollment and looking at ways to integrate alternate data sources.<sup>xxiii</sup>



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## CF PATIENT REGISTRY

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### PURPOSE

The CF Patient Registry aims to help researchers understand the clinical course of cystic fibrosis (CF) and assist care teams at the Cystic Fibrosis Foundation (CFF) clinical centers improve patient care.

### DEFINING CHARACTERISTICS

The CF Patient Registry has been collecting data from cystic fibrosis patients for around 50 years, and 95 percent of CF patients participate in the registry. Each year, the registry collects data from more than 28,000 people with CF. Complete medical data and genetic testing information are collected at CFF-accredited care centers and added to a secure Web database (called PortCF) by trained clinical care professionals. Participation in the registry is a requirement of CFF care center accreditation.

Researchers gain access to CF Patient Registry data after a rigorous process. A researcher submits a request for registry data to the foundation. The request then undergoes a thorough review by the Patient Registry/Comparative Effectiveness Research Committee comprised of CF clinicians, researchers and CFF staff. The process takes six to eight weeks, and, if approved, data are delivered securely to the researcher. The foundation requests that researchers present any findings at the North American Cystic Fibrosis Conference and by publishing in a peer-reviewed journal.

Traditionally, registry data have been input solely by certified staff at the CFF-accredited care centers; however, the CF Patient Registry is looking toward new ways to collect and use registry data—including data directly input by patients.

Data from the CF Patient Registry is shared by CFF through its Patient Registry data reports, available on its Web site.<sup>xxiv</sup>



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## PHELAN-MCDERMID SYNDROME INTERNATIONAL REGISTRY

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### PURPOSE

The purpose of the Phelan-McDermid Syndrome International Registry (PMSIR) is to consolidate information from individuals with Phelan-McDermid Syndrome into a single database, which will be utilized by researchers to better understand Phelan-McDermid Syndrome.

### DEFINING CHARACTERISTICS

The Phelan-McDermid Syndrome International Registry was established in 2011 by the Phelan-McDermid Syndrome Foundation. Of the 1,300 diagnosed cases of Phelan-McDermid Syndrome in the world, 844 are reflected in the registry. One-third of those users have uploaded health records. The registry is hosted on PatientCrossroads and has received several PCORI grants. It is part of PCORI's Patient-Powered Research Network in PCORnet.

PMSIR is important for better characterizing and understanding the natural history of Phelan-McDermid Syndrome. It provides valuable information for families and doctors to help make the best care decisions possible. It also helps researchers determine the most important research challenges to address. The registry additionally connects patients to clinical trials they may qualify for.

Registry participants are highly motivated to facilitate research for Phelan-McDermid Syndrome. Participants consent to share all de-identified data and have been eager to participate in the registry. Participants can learn about registry data within the registry platform.<sup>xxv</sup>



## DUCHENNECONNECT

### PURPOSE

The mission of DuchenneConnect is to connect Duchenne muscular dystrophy patients and their communities with researchers and clinicians.

### DEFINING CHARACTERISTICS

DuchenneConnect was founded in 2007 and is supported by Parent Project Muscular Dystrophy. It received funding from the Patient-Centered Outcomes Research Institute to be part of the Patient-Powered Research Network in PCORnet. DuchenneConnect is housed on the PatientCrossroads platform. Parent Project Muscular Dystrophy helped launch PatientCrossroads in 2005, with DuchenneConnect as the motivation.

While connecting patients to the medical research community, DuchenneConnect is a resource for researchers and companies with an interest in Duchenne, allowing access to aggregated, de-identified information provided by patients and their families. This information is intended to advance research, treatments and patient care. DuchenneConnect also helps to enroll clinical trials that participants might qualify for by tracking them via the registry.

DuchenneConnect makes de-identified outcomes publicly available on [duchenneconnect.org](http://duchenneconnect.org). Outcomes include various published studies and clinical trial recruitment via the registry.<sup>xxvi</sup>



## APPENDIX III: RESOURCES LIST

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**CTTI Recommendations:  
Effective Engagement with  
Patient Groups around  
Clinical Trials**  
Clinical Trials Transformation  
Initiative, 2015

**Enhancing Benefit-Risk  
Assessments by Integrating  
Patient Perspectives:  
An Information Collection  
Tool for Patient Organizations**  
National Health Council, 2014

**From Anecdotal to Actionable:  
The Case for Patient  
Perspective Data**  
*FasterCures*, 2015

**From Passengers to Co-pilots:  
Patient Roles Expand**  
*Science Translational Medicine*,  
2015

**Honest Brokers for Cures:  
How Venture Philanthropy  
Groups are Changing  
Biomedical Research**  
*FasterCures*, 2013

**ISPOR Taxonomy of Patient  
Registries: Classification,  
Characteristics and Terms**  
International Society for  
Pharmacoeconomics and  
Outcomes Research, 2013

**Measuring and Improving  
Impact: A Toolkit for Funders  
of Medical Research**  
*FasterCures*, 2013

**The Patient Group Handbook:  
A Practical Guide for Research  
and Drug Development**  
Findacure, 2016

**Rare Diseases: Common  
Issues in Drug Development**  
U.S. Food and Drug  
Administration, 2015

**Registries for Evaluating  
Patient Outcomes: A User's  
Guide: 3rd Edition**  
Agency for Healthcare  
Research and Quality, 2014



## APPENDIX III: ENDNOTES

<sup>i</sup>Anderson, Margaret, and K. Kimberly McCleary, “From Passengers to Co-Pilots: Patient Roles Expand,” *Science Translational Medicine* 7, no. 291 (June 10, 2015), p. 291fs25, <http://stm.sciencemag.org/content/7/291/291fs25.abstract>.

<sup>ii</sup>Gliklich, Richard E., and Nancy A. Dreyer, “Registries for Evaluating Patient Outcomes: A User’s Guide,” Agency for Healthcare Research and Quality 3rd Edition (April 2014), <http://www.ncbi.nlm.nih.gov/books/NBK208616/>.

<sup>iii</sup>“Honest Brokers for Cures: How Venture Philanthropy Groups are Changing Biomedical Research,” *FasterCures*, 2013, <http://www.fastercures.org/assets/Uploads/PDF/HonestBrokers.pdf>

<sup>iv</sup>“Measuring and Improving Impact: A Toolkit for Nonprofit Funders of Medical Research,” *FasterCures*, 2013, <http://www.fastercures.org/assets/Uploads/PDF/VPtoolkit.pdf>

<sup>v</sup>HIPAA for Professionals, U.S. Department of Health and Human Services, <http://www.hhs.gov/hipaa/for-professionals/index.html>

<sup>vi</sup>Federal Policy for the Protection of Human Subjects (“Common Rule”), U.S. Department of Health and Human Services, <http://www.hhs.gov/ohrp/humansubjects/common-rule/>

<sup>vii</sup>Gliklich, Richard E., and Nancy A. Dreyer, “Registries for Evaluating Patient Outcomes: A User’s Guide,” Agency for Healthcare Research and Quality 3rd Edition (April 2014), <http://www.ncbi.nlm.nih.gov/books/NBK208616/>.

<sup>viii</sup>“Patient Perspectives on Disease Impact and Treatment Options: A Stratification Tool,” National Health Council, 2014, [http://www.national-healthcouncil.org/sites/default/files/NHCPatientInformationToolandInstructions\\_o.pdf](http://www.national-healthcouncil.org/sites/default/files/NHCPatientInformationToolandInstructions_o.pdf)

<sup>ix</sup>Citizen Pscientist, National Psoriasis Foundation, <http://www.psoriasis.org/citizenpscientist/welcome>

<sup>x</sup>How we work, Collaborative Chronic Care Network, <http://www.c3nproject.org/how-we-work#design>

<sup>xi</sup>About Us, Glue, <http://myglue.org>

<sup>xii</sup>IBD Plexus: Partnering to Accelerate Science, Crohn’s & Colitis Foundation of America, <http://www.ccfa.org/science-and-professionals/research/IBDPlexus/>

<sup>xiii</sup>How TripAdvisor altered your vacation-planning universe. [washingtonpost.com/lifestyle/travel/how-tripadvisor-altered-your-vacation-planning-universe/2015/08/27/43fdec1e-4064-11e5-bfe3-ffid8549bfd2\\_story.html](http://washingtonpost.com/lifestyle/travel/how-tripadvisor-altered-your-vacation-planning-universe/2015/08/27/43fdec1e-4064-11e5-bfe3-ffid8549bfd2_story.html)

<sup>xiv</sup>“From Anecdotal to Actionable: The Case for Patient Perspective Data,” *FasterCures*, 2015, <http://www.fastercures.org/assets/Uploads/PDF/From-Anecdotal-to-Actionable.pdf>

<sup>xv</sup>Citizen Pscientist, National Psoriasis Foundation, <http://www.psoriasis.org/citizenpscientist/welcome>

<sup>xvi</sup>GRDR Frequently Asked Questions, National Center for the Advancement of Translational Sciences, <https://ncats.nih.gov/grdr/faq>

<sup>xvii</sup>Frequently Asked Questions, PCORnet, <http://pcornet.org/about/pcornet/faqs/>

<sup>xviii</sup>Tockarshefsky, Tina, “The Perfect Storm for Patient-Focused Clinical Trials,” *BioSupply Trends Quarterly*, Fall 2015 (2015), <http://www.fastercures.org/assets/Uploads/PDF/Clinical-Trials-Article-BioSupply-Trends-Quarterly.pdf>.

<sup>xix</sup>“Consortia-pedia,” *FasterCures*, 2013, <http://www.fastercures.org/assets/Uploads/Consortia-pedia.pdf>.

<sup>xx</sup>International, Phelan-McDermid Syndrome Foundation, <http://22q13.org>

<sup>xxi</sup>Registry Development Using the Platform for Engaging Everyone Responsibly (PEER) Request for Proposals, Genetic Alliance, 2015, [http://www.geneticalliance.org/sites/default/files/RequestforProposals\\_FAQ\\_RWJF.pdf](http://www.geneticalliance.org/sites/default/files/RequestforProposals_FAQ_RWJF.pdf)

<sup>xxii</sup>T1D Exchange, <http://t1dex-change.org>

<sup>xxiii</sup>Citizen Pscientist, National Psoriasis Foundation, <http://www.psoriasis.org/citizenpscientist/welcome>

<sup>xxiv</sup>CF Patient Registry, Cystic Fibrosis Foundation, <http://www.cff.org/Our-Research/CF-Patient-Registry/>

<sup>xxv</sup>Phelan-McDermid Syndrome Foundation, <http://22q13.org>

<sup>xxvi</sup>DuchenneConnect, <https://www.duchenneconnect.org/>









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