MILKEN INSTITUTE

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

PART III: FOR RESEARCHERS

About the Milken Institute

The Milken Institute is a nonprofit, nonpartisan think tank.

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

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

About FasterCures

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

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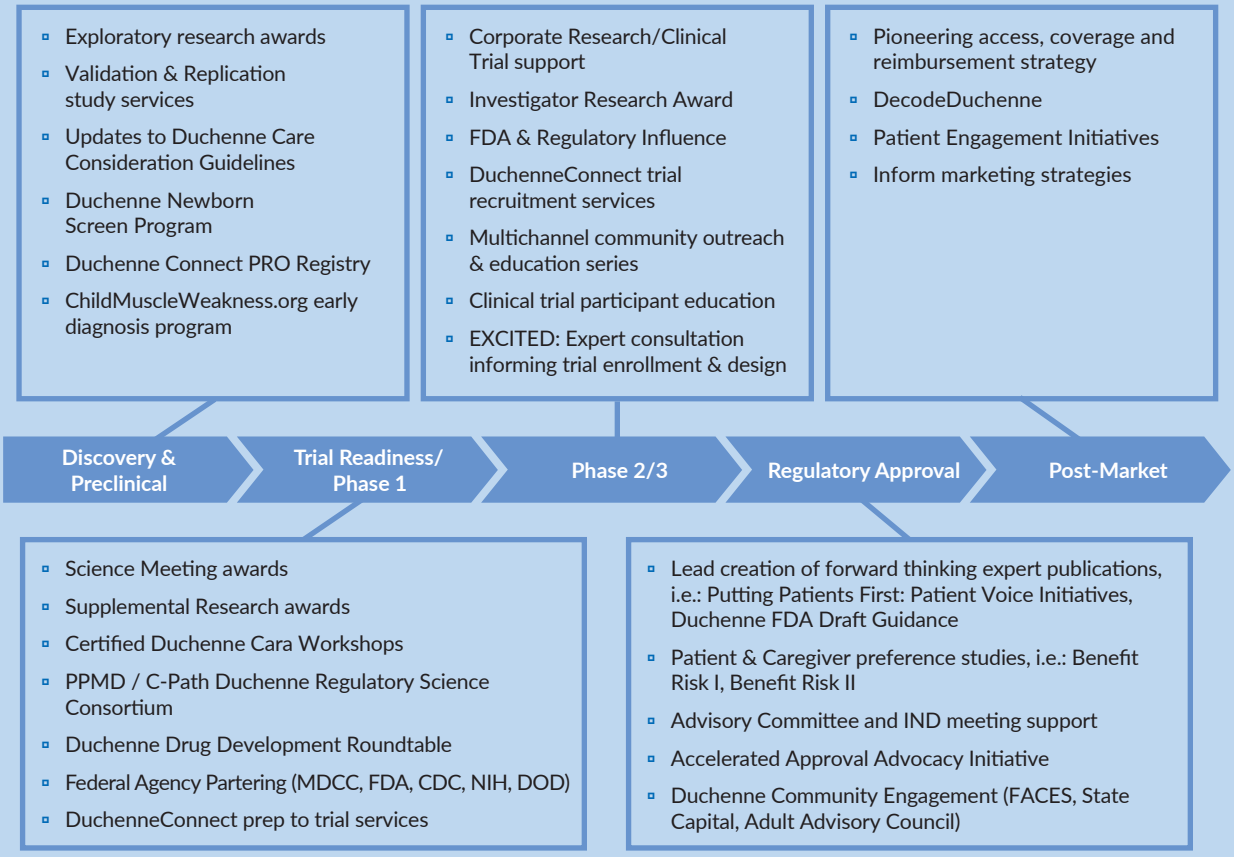
Introduction

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

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

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

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



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

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

KEY TAKEAWAYS FOR RESEARCHERS:

I.

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

II.

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

III.

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

IV.

Several barriers exist to increasing patient involvement.

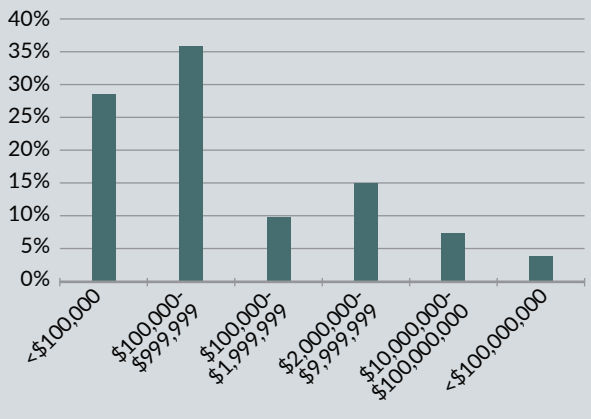
I. Understanding patient organizations as research partners

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

MISSION

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

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



Source: FasterCures questionnaire, Fall 2018.

SCIENTIFIC STRATEGY

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

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

STRUCTURE

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

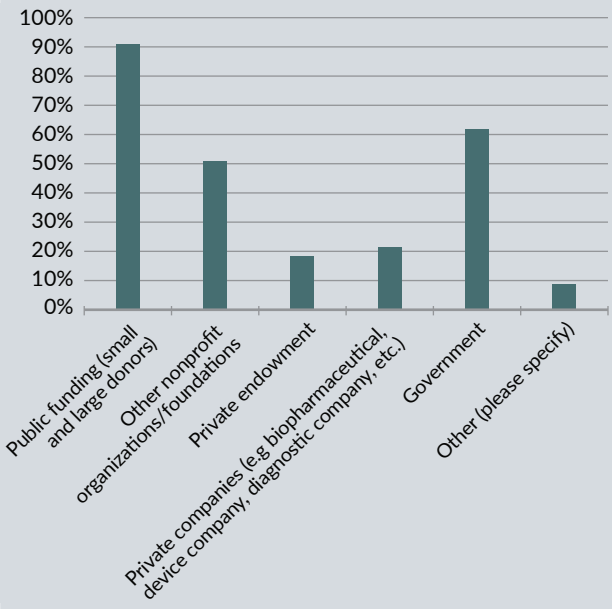
1. While our focus in this project has been patient organizations as ready partners for research and sources of patient data, we acknowledge that there are conditions for which there is no organized patient constituency, many competing organizations, or have patient populations that have yet to be activated.

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

AMOUNT AND SOURCES OF FUNDING

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

Figure 2: Which of the following provide funding for your organization?



Source: FasterCures questionnaire, Fall 2018.

STAFFING AND EXPERTISE

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

OUTREACH

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

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

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

ASSETS

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

Given their unique role as representatives of patients' interests and perspectives, these organizations' policies for the use of their resources likely differ from those of commercial providers of resources such as data, tissue, or clinical trial recruitment. They are likely to have a greater interest in the sharing of information and results, pre-competitive collaboration, and meaningful patient engagement. They should have guiding principles or other policies for the use of their resources that clearly articulate their requirements and reasoning.

Identifying appropriate research partners

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

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

Cohort Development	Disease Characterization	Research Management
▪ Registry of affected individuals ▪ Blood and tissue bank ▪ Clinical data ▪ Human genotype/ phenotype data ▪ Human gene expression data ▪ Human epigenetic data	▪ Is the gene identified? ▪ Is the protein identified? ▪ Is there an antibody available? ▪ Natural history or epidemiological studies ▪ Biomarkers ▪ Well-defined clinical endpoints ▪ In vitro model systems ▪ Animal models ▪ Is there a diagnostic test? ▪ Are there any lead compounds?	▪ Link researchers and families ▪ Recruit participants ▪ Initiate and/or conduct research ▪ Award research grants ▪ Has intellectual property ▪ Provide information about clinical trials ▪ Conduct clinical trials

Source: Genetic Alliance.

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

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

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

II. Patient organization investments in health data

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

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

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

In fall 2018, 78 patient organizations responded to our questionnaire about their interest and investments in patient data. Eighty-eight percent of respondents indicated that they had supported the creation or maintenance of a wide variety of data resources, which have been used primarily for discovery and observational research but also for preclinical and clinical research and post-market surveillance. Specific resources include patient registries, online platforms or social networks for patients and caregivers, patient-reported outcomes, biorepositories, natural history, gene sequencing data, and mobile health data collection and/or studies.

Respondents indicated they are investing in patient data resources because:

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

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

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

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

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

Cohort Development	Disease Characterization
Researchers (e.g. academic, health services)	▪ Access larger, more diverse data sets ▪ Include in observational studies ▪ Pressure test hypotheses and methods, validate and interpret findings
Patients and caregivers	▪ Coordinate care and shared decision-making ▪ Offer the opportunity to contribute to research
Clinicians	▪ Provide a more holistic view of patient health over time ▪ Improve shared decision-making
Product manufacturers	▪ Access larger, more diverse data sets ▪ Target trial recruitment efforts ▪ Improve trial design and conduct ▪ Include in observational studies/real-world evidence research ▪ Influence trial selection criteria, endpoints, symptoms, and disease burden ▪ Identify subtypes, prognosis, and signal detection for development of preventive therapies and symptom management ▪ Include in the product label
Regulatory agencies (e.g., FDA)	▪ Evaluate product applications through the patient lens ▪ Develop more robust methods for signal detection and other post-market surveillance activities
Payers (e.g., CMS, state Medicaid agencies, private payers) and value assessors (e.g., the Institute for Clinical and Economic Review)	▪ Acquire additional information for coverage decisions ▪ Produce better cost-effectiveness studies and value assessment
Policymakers	▪ Inform the development of new policies governing which populations get access to new medicines and medical devices ▪ Modify policies on payments for medicines and devices ▪ Evaluate the impact of existing coverage and payment policies on specific sub-populations of patients ▪ Assess the effectiveness of treatments based on expanded sources of evidence
Standard-setting bodies	▪ Inform standard determinations ▪ Enable creation of condition-specific data standards

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

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

III. Meaningful research engagement with patient organizations

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

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

Key recommendations for patient engagement in research

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

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

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

WANT MORE? READ **PART II: FOR PATIENT ORGANIZATIONS** TO LEARN MORE ABOUT:

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

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

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

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

National Health Council and Genetic Alliance, Dialogue, Advancing Meaningful Patient Engagement in Research, Development, and Review of Drugs (September 22, 2015), available at: https://www.nationalhealthcouncil.org/sites/default/files/PatientEngagement-WhitePaper.pdf.

Clinical Trials Transformation Initiative (CTTI) Recommendations: Effective Engagement with Patient Groups Around Clinical Trials (October 2015), available at: https://www.ctti-clinicaltrials.org/files/pgctrecs.pdf.

Patient-Centered Outcomes Research Institute (PCORI), Engagement Rubric for Applicants (updated June 6, 2016), available at: https://www.pcori.org/sites/default/files/Engagement-Rubric.pdf.

Food and Drug Administration, Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders, Patient Preference Information—Voluntary Submission, Review in Premarket Approval Applications, Humanitarian Device Exemption Applications, and De Novo Requests, and Inclusion in Decision Summaries and Device Labeling (August 24, 2016), available at: https://www.fda.gov/downloads/medicaldevices/deviceregulationandguidance/guidancedocuments/ucm446680.pdf.

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

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

Avalere Health and FasterCures' Patient Perspective Value Framework (PPVF), version 1.0 (May 2017), available at: https://www.fastercures.org/assets/Uploads/PPVF-Version-1.0-Methodology-Report-Final.pdf.

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

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

About the Authors

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

Valerie Barton, M.A. is chief data strategy officer at the People-Centered Research Foundation (PCRF), where she works with researchers to integrate claims and registry data into the clinical data assets of PCORnet and leads PCRF's patient engagement activities. During her nearly 15 years as a consultant, she assisted clients by bringing data-informed approaches to their health policy development and business strategies. Ms. Barton worked cross-functionally to build research and data practices from the ground up at both Manatt Health LLP and Avalere Health LLC. Under her leadership, her teams conducted quantitative analytics and economic modeling with observational data for pharmaceutical manufacturers, biotech and medical device firms, provider organizations, and foundations. Ms. Barton has focused on analysis of the Medicare program, payment and delivery innovation, and social determinants of health using Medicare, Medicaid, and commercial claims and publicly-available survey and community-level administrative data sources. In the public sector, Ms. Barton was a health policy analyst with the Centers for Medicare and Medicaid Services. There, she wrote federal Medicare regulations governing post-acute care and hospital outpatient payment systems. Ms. Barton began her career as a federal budget analyst with the Congressional Budget Office, where she modeled the impacts of legislative changes on veterans' health and military personnel programs. Ms. Barton serves as a Senior Advisor to FasterCures and on the board of directors for Micah House, a supportive residence in Washington, DC, for women recovering from substance use disorders.

Brenda Huneycutt is a director at FasterCures, a center of the Milken Institute, where she leads the "Enabling a High-Performing Biomedical Ecosystem" program and directs a project portfolio aimed at creating a system that works better for patients. Her work includes developing a biomedical ecosystem performance scorecard, creating tools to increase the representation of patient perspectives in health-care decision-making, and driving transparency in medical product development. Prior to joining FasterCures, Dr. Huneycutt was vice president, regulatory strategy and FDA policy at Avalere Health, advising organizations on topics such as patient engagement in drug development, compassionate use/expanded access to investigational products, regulatory exclusivities, the Food and Drug Administration's orphan drug and expedited programs, and the use of real-world evidence in regulatory decision-making. Dr. Huneycutt has also practiced as a patent lawyer in a large firm working on pharmaceutical litigation, and spent many years as a research scientist, primarily studying cell division and cell cycle control in yeast model systems. Dr. Huneycutt holds a PhD in molecular biology from the University of Colorado at Boulder, a JD from the George Washington University School of Law, and an MPH from the Johns Hopkins University Bloomberg School of Public Health.

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