



# Patient Participation

THE *FASTERCURES* CONSORTIA-PEDIA REPORT

## About the *FasterCures* Consortia-pedia project:

*FasterCures* initiated the Consortia-pedia project to better understand the breadth and scope of approaches that a wide range of consortia have adopted to bring together non-traditional partners with a shared R&D goal. Since 2012, our analysis of more than 350 biomedical research consortia has been aimed to better understand how different stakeholders are using this model of partnership to address shared unmet needs.

To better understand consortia models, *FasterCures* analyzed 21 efforts that represent the diversity of models used to bring together non-traditional partners to accelerate biomedical research. We present our analysis under seven partnership components.

1. Governance
2. Financing
3. Human Capital
4. Intellectual Property
5. Data Sharing
6. Patient Participation
7. Measurement of Impact

Each component is a chapter in the Consortia-pedia report and can be downloaded at:  
[www.fastercures.org/consortiapedia](http://www.fastercures.org/consortiapedia).

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# KEY POINTS – PATIENT PARTICIPATION

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- The patient perspective can be an **invaluable component** to a consortium's strategic research agenda and operational strategy.
- **Patient foundations** are actively leveraging the financial and intellectual capital of other sectors through their own consortia.
- Patient foundation-led consortia are focused on creating opportunities and resources to **incentivize researchers** toward their disease of interest. Often, data and tools must be openly accessible to the public.
- Often viewed as the intermediary between the patient and biomedical research, patient advocates must be well-versed to readily communicate between these groups. **Fluency within the multiple research sectors** is essential for addressing the different expectations and managing the diverse relationships within a consortium.

Patients, as individuals or working through a foundation, are increasingly expanding their roles in multi-stakeholder consortia. Individual patients contribute to these consortia as research participants and may offer their expertise and experiences to a consortium's strategy, governance, and operations. This level of engagement is not limited to consortia that conduct research within an active clinical trial setting. Other consortia, such as Project Data Sphere and the Observational Medical Outcomes Partnership, include representatives of the patient community within their steering committees or have a separate patient advisory committee to provide advice and monitor ongoing research activities.

Many patient groups also actively manage their own consortia in addition to partnering with efforts initiated by others, a model of collaboration that appeals to them because of the ability to focus the resources and expertise of the whole product development spectrum to address their disease of interest. This type of cross-sector leadership and engagement is becoming a permanent characteristic of the biomedical research landscape as patients and their foundations are increasingly getting impatient with the lack of collaborative research.

## Patient advocates participating in consortia

Many foundations advance research by serving as a trusted party that is able to communicate to their patient populations about a consortium's value proposition, as well as help to coordinate their often well-defined patient cohorts with a consortium's research efforts. Foundations also represent leading experts of their specific diseases, garnering a wealth of basic knowledge of biological processes underpinning the disease, an understanding of the landscape of therapeutic or diagnostic approaches, and an ability to identify the clinical trial methods that meet the requirements and needs of their patient population. Thus, in addition to communicating with their patient communities, some consortia have also taken advantage of the intellectual capital that advocates can contribute to help with the design and pursuit of their research strategy.

There are many examples where patient foundations help with a consortium's internal processes, such as ensuring that a consortium's informed consent policies reflect a patient and their family's concerns, and by participating as members of a safety and monitoring board to ensure that the ongoing research activities minimize risk to its participants. Several consortia have also benefitted by having patient advocates develop their legal participation documents in a format that is patient-friendly and complemented with supplemental educational materials. Many foundations also collaborate with each other, even outside of their disease focus, and can bring to consortia new R&D methods and tools that were effective for other disease areas but have yet to be applied to their patient population.

One example is the I-SPY 2 trial, a consortium focused on evaluating a potentially more efficient approach to the assessment of a novel cancer therapeutic. Based on the theory of adaptive trial design, the results of this collaboration could validate or find opportunities to optimize this type of clinical trial method, with broader

implications outside of oncology. Patient advocates were essential in working alongside other experts to construct the strategy and governance of I-SPY 2, and one of their key contributions was in the development of a trial protocol and informed consent document. This Biomarkers Consortium-managed effort also involves patient advocates at all stages, ensuring that the trial’s design is appropriate for a woman dealing with the diagnosis of locally advanced breast cancer, the patient’s perspective is represented on all scientific working and advisory groups, and there are opportunities for patient advocates to lead their own projects within the consortium. These projects were essential for developing tools that make it easier for patients to participate, such as having all trial forms online, utilizing blogs and Twitter to solicit patient feedback, and creating an avenue where patients can obtain information on interim research results. See Table 1 for more information.

**Table 1: I-SPY 2 involves patient advocates as research partners**

Traditional role of patient advocates	Patient advocates as research partners to I-SPY 2
<ul style="list-style-type: none"> <li>• Develop patient-friendly documents and education materials for informed consent</li> <li>• Develop patient decision support materials (recruiting brochure, Web site, DVD, etc.)</li> <li>• Serve as members of external data safety and monitoring board</li> <li>• Communicate consortium objectives from their perspective to recruit and retain trial participants (as seen in a Wall Street Journal article<sup>1</sup>)</li> <li>• Develop advocate program to include engaging local advocates to support trial sites and recruit/retain participants</li> </ul>	<ul style="list-style-type: none"> <li>• Review protocol and design of the clinical trial to ensure that it is convenient to patients’ challenges and lifestyles</li> <li>• Serve on all scientific and advisory boards</li> <li>• Defend a criticism of the consortium by publishing a rebuttal in the Journal of Clinical Oncology<sup>2</sup></li> </ul>

Another example of patient engagement can be found at the Collaborative Chronic Care Network (C3N), a clinical practice-based learning healthcare system focused on improving the treatment of patients with chronic illnesses by integrating clinical data with patient-provided data. A patient advisory council provides feedback to the steering committees and also works with developers of iPhone apps and gaming systems to ensure that C3N’s tools are appropriate for use by a pediatric population outside of the clinical setting. To further increase the engagement of its patient population, this council also helped C3N design summer camps and specific toolkits.

The patient perspective has also been important for consortia that do not actively involve the participation of patients. For example, Project Data Sphere is an effort to demonstrate how the sharing of historical oncology clinical trial data can be openly utilized by the scientific community. In setting up its infrastructure, the consortium depended on patient advocates to define and monitor the safety and security requirements for their underlying technology. The leadership also reached out to dozens of non-oncology focused advocacy groups for advice, and implemented a dedicated advocacy team to ensure that their privacy and security policies continuously address patient concerns. These sources of feedback were pivotal in the design and implementation of their data-anonymization strategies, particularly relevant since the source of data exchanged within the project describes an individualized, patient-level clinical trial.

### Patient advocates leading consortia

Several patient advocacy groups are involved in the most innovative and cutting-edge academic and industry research programs that impact their disease (as funders, partners, or both), serving as the primary scientific authority for the research community. Instead of depending on others to establish a research infrastructure,

<sup>1</sup> Wall Street Journal. Sept. 16, 2013, “Patients Share DNA for Cures.” Accessed Feb. 13, 2014 at

<http://online.wsj.com/news/articles/SB10001424127887323342404579079453190552312?mg=reno64-wsj>

<sup>2</sup> Journal of Clinical Oncology. Feb. 20, 2011, “Adaptive Clinical Trials: The Promise and the Caution.” Accessed Feb. 13, 2014 at <http://jco.ascopubs.org/content/29/6/606.long>

many of these groups have set up their own clinical trial and biorepository networks to collect, annotate, and distribute data and biospecimens. To maximize their own limited capital and resources, some foundations leverage the consortium model to coordinate the resources and expertise of different research sectors, while ensuring that the tools developed by their consortium are useful to the academic and industry communities. Many of these groups are members of *FasterCures* TRAIN (The Research Acceleration and Innovation Network, <http://train.fastercures.org>).

For example, the Michael J. Fox Foundation for Parkinson's Research (MJFF) employs scientific staff with well-respected credentials in neuroscience and Parkinson's disease. In addition to subject-matter expertise, their staff members also represent diverse experiences from academia and industry. This staffing strategy provides the foundation with the ability to collectively identify and address an unmet and cross-sector scientific need that is limiting the advance of research in Parkinson's disease. One product of this brainstorming is MJFF's Parkinson's Progression Markers Initiative (PPMI), an effort to use the resources from a network of clinical trial sites and industry to create a data repository and biobank for MJFF researchers and the broader scientific community.

Likewise, the Multiple Myeloma Research Foundation (MMRF) has a dedicated staff of scientific experts who are leading its research programs. The MMRF's Personal Medicine Initiative was the product of internal and external discussions, leading to the formation of the CoMMpass study. This study aims to provide longitudinal biomarker data of more than 1,000 patients, a wealth of information that can empower researchers. Since the research approach used for this study could be generalizable to other diseases, the CoMMpass study was also designed with feedback from several other non-myeloma patient advocacy organizations such as MJFF, the Myelin Repair Foundation (MRF), and Autism Speaks.

Many foundations also play a more proactive role in the development of specific therapeutics with functions similar to a virtual pharmaceutical company. These efforts are characterized by a research infrastructure where the coordination of their activities is provided by the foundation at a hands-on level, with research results scrutinized by program managers and scientists who are employed by the foundation and an external steering committee. For example, the MRF is focused on accelerating the development of myelin repair therapeutics for multiple sclerosis. Its consortium framework is based on its Accelerated Research Collaboration (ARC), which coordinates and manages the entire therapeutic development process from discovery biology to Food and Drug Administration approval.

Some of the key components of ARC include:

- coordination of basic research emerging from independent academic and government laboratories to accelerate the development of new targets, research tools, and compounds with potential for drug development
- validation and de-risking of therapeutically relevant compounds and targets in collaboration with industry partners, who participate in various advisory committees
- assay-development support to funded and non-affiliated researchers through its Translational Medicine Center, a brick-and-mortar facility staffed by MRF scientific personnel

### Educating patients in biomedical research

Unfortunately, examples of consortia that are convened by groups like MJFF, MMRF, and MRF are rare, as most patient advocacy groups do not have the resources to employ a dedicated scientific staff to keep abreast of the latest discoveries or manage their own consortium. To address this disparity, some groups have taken on the effort to increase the number of advocates who are well-versed in the processes of biomedical research, and at least one of these efforts is part of a multi-sector consortium.

The Innovative Medicines Initiative (IMI) is a public-private partnership between the European Commission and the European pharmaceutical industry to accelerate drug R&D. Several research-oriented consortia operating under the IMI umbrella directly involve patients within their governance and operations. In addition, IMI also manages an independent five-year effort known as the European Patients Academy on Therapeutic Innovation. The goal of this initiative is to develop education materials, training courses, and an online library to serve as resources to educate the public and create informed patient advocacy groups on drug development processes and vocabulary, using material written in seven languages and targeting 12 European countries. The current topics include precision medicine, clinical trials, drug safety and risk/benefit assessment, health economics, and methods to be involved in drug development.

### Continuous and broad-reaching communication

In addition to directly involving patients in the design and implementation process, it is also important to communicate the mission and outcomes of the consortium to the broader patient population and society. Each audience brings with it different expectations for impact, and the broader public includes family members and future patients who may have a perspective that is also influenced by media, as well as their socio-economic and cultural backgrounds.

The Web sites for many consortia are often designed to separately address the patient, clinician, researcher, and public sectors. Each of these components describes the research progress and presents results in a language and format that is tuned to those audiences. Many groups are also actively involved in creating dialogue directly within their disease and regional communities. For example, the Critical Path Institute’s Coalition Against Major Diseases has made presentations directly to the research and finance communities, as well as presented to audiences at local community forums.

MJFF utilizes social media as part of its outreach to the donor, patient, and research communities. This foundation has a dedicated Digital Strategy Team that ensures an active presence on platforms such as Facebook, Twitter, and LinkedIn, and utilizes tracking metrics to understand its impact. The foundation’s Web site also includes professionally made Webinars, videos, and podcasts. As one example, MJFF’s Foxfeed blog is an education tool directed at several audiences. Information about the foundation’s efforts, such as PPMI, can be simply obtained by searching this blog (see Table 2). In addition to its digital venues for connecting with various audiences, MJFF has a staff member dedicated as the sole and direct contact for all of its industry relationships. This helps ensure uniformity in the foundation’s communication on specific, and often legal, details of their collaborations with members of this sector.

**Table 2: Examples of audience-directed blogs for MJFF’s PPMI**

Audience	Blog entry
Clinician/scientist	<a href="#">PPMI Recruitment Complete, New Study Arm Launches (4/22/13)</a> <a href="#">Parkinson's Disease: More Than a Movement Disorder? (4/1/13)</a>
Public/patients	<a href="#">We Are the Faces of Parkinson's Disease (6/26/13)</a> <a href="#">Parkinson's Diagnosis Yields a New Opportunity to Do Good (9/26/12)</a> <a href="#">Debi's Blog: Tales of Volunteerism and Willingness (4/4/12)</a>



**For more information and the latest updates on the *FasterCures* Consortia-pedia, visit [www.fastercures.org](http://www.fastercures.org).**