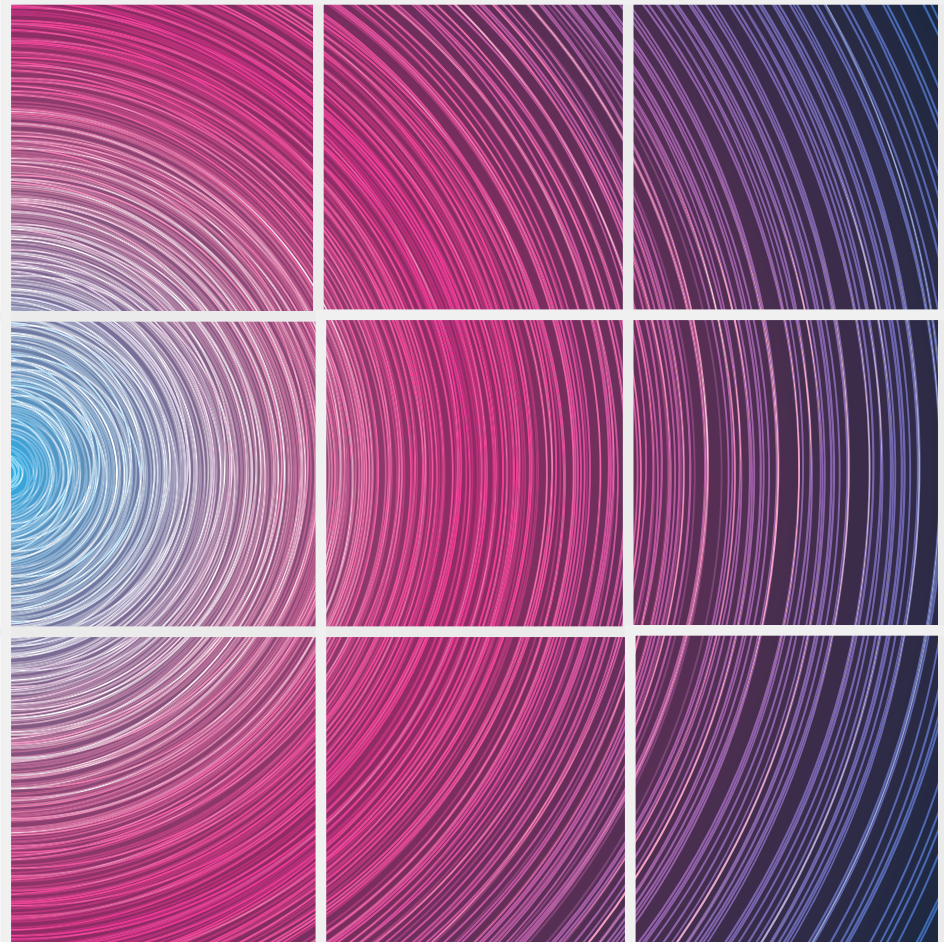# MILKEN INSTITUTE

Patient-Centric Initiatives
Focusing for Impact

Taylor Cusher, Anna DeGarmo, and Cynthia Grossman

ABOUT US

ABOUT THE MILKEN INSTITUTE

The Milken Institute is a nonprofit, nonpartisan think tank. We work to create environments that empower people to build meaningful lives of physical, social, and economic wellbeing.

ABOUT FASTERCURES

FasterCures defines and maps the barriers that slow medical treatments. We overcome these obstacles by defining the challenges, designing patient-focused solutions, and engaging our network to catalyze change.

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PATIENT-CENTRIC INITIATIVES

Focusing for Impact

Taylor Cusher, Anna DeGarmo, and Cynthia Grossman

FasterCures has long been a leader in developing and monitoring initiatives to advance patient engagement in the lifecycle of medical product discovery, development, and delivery. In 2017, we wrote in our report "From Aspiration to Application: 5 Years of Patient-Centricity" that what started as a handful of activities has turned into a suite of organized endeavors that brought the idea of patient engagement closer to routine practice. The impact of patient engagement has been demonstrated by numerous case studies for specific diseases and products, as well as a model for the return on investment. There is now a growing call for expanding beyond single examples of patient engagement to a scale that is part of routine medical practice across diseases and conditions. Our goal with this report is to highlight the current gaps and provide a forward-looking perspective on initiatives that will help patient engagement become a fully integrated practice throughout the product lifecycle. Regardless of whether you are a representative from a patient organization, medical product developer, or academic researcher, you will see there are ways to join the collective effort to build on ongoing patient engagement efforts and fill the gaps to take patient engagement to scale.

We reexamined a suite of initiatives—identified in our 2016 report "Expanding the Science of Patient Input: Pain Points and Potential"—by conducting (1) a workshop with 20 patient organization representatives and medical product developers, (2) 10 key informant interviews, and (3) surveying 40 patient organizations (see Appendix for a list of the 2018 experts and initiative definitions). This process helped us understand the needs of a more diverse set of patient groups, recognizing that we should not assume that everyone has similar points of reference.

PATIENT-CENTRIC INITIATIVES

Initiatives to Create Tools/Frameworks
Training Initiatives
Methods Development
Initiatives to Address Legal Challenges
Initiatives to Improve Measurement
Combination Initiatives

For each initiative, we asked experts and survey respondents to rank the initiatives on two dimensions: the contribution the initiative makes toward patient-centricity and the challenge to execute. *Contribution* describes how an activity or initiative moves the field of patient-centricity forward as a whole—how likely is this initiative to advance the goal of making patient engagement a routine practice? *Challenge* describes the difficulties and roadblocks associated with implementing an initiative. It is important to consider the level of difficulty involved in executing an initiative; focusing on the work that is less challenging to execute can lead to quick wins and build momentum.
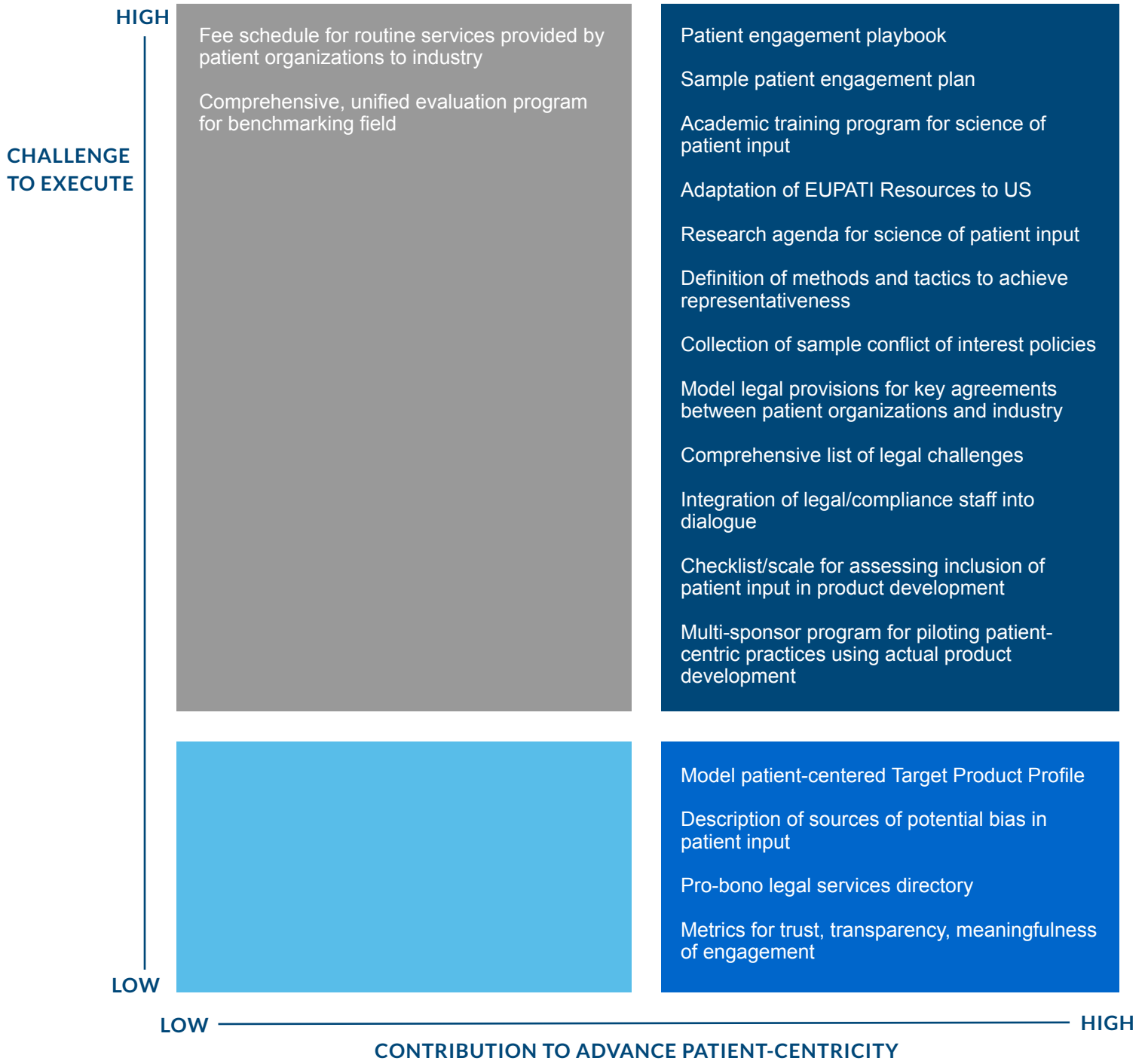
Not surprisingly, views about the challenges and contributions of each initiative vary across organizations based on the level of experience with leading patient engagement initiatives. Where there is agreement, we should move quickly toward development and initiation and not let disagreement hinder progress. In cases where there is disagreement, it may be a matter of need or simply differing areas of focus for your specific organization or company.

The initiatives fall into two buckets: those that are important for any organization to focus on if it wants to achieve patient engagement at scale and those that may be a focus of organizations depending on their resources and experience in patient engagement.

PATIENT-CENTRIC INITIATIVES

CHALLENGE TO EXECUTE — HIGH / LOW

CONTRIBUTION TO ADVANCE PATIENT-CENTRICITY — LOW / HIGH

HIGH (Challenge to Execute) — Low Contribution quadrant:

Fee schedule for routine services provided by patient organizations to industry

Comprehensive, unified evaluation program for benchmarking field

HIGH (Challenge to Execute) — High Contribution quadrant:

Patient engagement playbook

Sample patient engagement plan

Academic training program for science of patient input

Adaptation of EUPATI Resources to US

Research agenda for science of patient input

Definition of methods and tactics to achieve representativeness

Collection of sample conflict of interest policies

Model legal provisions for key agreements between patient organizations and industry

Comprehensive list of legal challenges

Integration of legal/compliance staff into dialogue

Checklist/scale for assessing inclusion of patient input in product development

Multi-sponsor program for piloting patient-centric practices using actual product development

LOW (Challenge to Execute) — High Contribution quadrant:

Model patient-centered Target Product Profile

Description of sources of potential bias in patient input

Pro-bono legal services directory

Metrics for trust, transparency, meaningfulness of engagement

For the definitions of each initiative, see Appendix section at the end of the report.

SCALING UP PATIENT ENGAGEMENT

A panel of experts and survey respondents identified initiatives described later in this report aimed at improving measurement, training, and methods development to be important contributors to reaching sustainability and scale. The agreement across the groups we surveyed and interviewed indicates that these initiatives are appropriate for all organizations, regardless of whether you are a newcomer or a seasoned patient engagement expert, to advance. We must recognize the challenge it takes to execute some of the initiatives that may influence whether an organization can support the activities depending on resources and capacity.

1. Metrics for trust, transparency, and meaningfulness of engagement

Improving the measurement of engagement activities through metrics for trust, transparency, and meaningfulness is a worthwhile effort. These metrics will provide a feedback loop to organizations for continuous learning and practice improvement. While patient engagement practices are not yet a routine part of product development, having metrics is important to increase acceptance and to make them commonplace in R&D processes.

Tools such as the Patient Focused Medicines Development (PFMD)'s Patient Engagement Quality Guidance were developed to assist organizations and companies from the planning to assessment phases of patient engagement activities. Additionally, in 2018 during the Drug Information Association (DIA) annual conference, Measuring Impact in Patient-Centered Drug Development was focused on metrics and measurement of meaningful, high-quality patient engagement. As patient engagement efforts are built and evaluated, organizations need to refine existing value-based measures of trust, transparency, and meaningfulness. However, for organizations without metrics, the existing

guidance and frameworks provide a common set of principles and tools.

2. Scale for assessing patient engagement in R&D

The creation and implementation of a scale to assess the levels of patient engagement in R&D drive the entire field forward. It is important to avoid any scale that would become a set of boxes for organizations to check, as opposed to capturing the elements that indicate that the engagement was meaningful.

That said, a comprehensive checklist for the lifecycle of product development showcases where patient input could inform decision making and build on existing tools and case examples. Parent Project Muscular Dystrophy offers a diagram to highlight its capacity as a patient organization to inform medical product development. The National Center for Advancing Translational Sciences (NCATS) is creating a tool to assess and support community engagement that may apply to other types of research organizations. Though it is a challenge, the development and implementation of a patient engagement scale for use during the R&D process will ensure patient engagement is not an afterthought.

3. Academic training

As patient engagement has matured, there is a need for academic training as there is greater differentiation between the process of conducting science with patient input and advancing the science of patient input—the latter being the systematic collection of patient input through validated and rigorous methodologies that stand up to scientific inquiry. As discussed at the National Academies Advancing the Science of Patient Input workshop, we see both as critically important and worthy of investment. Both require a new way of structuring teams and conducting biomedical research. Many different disciplines

such as health economics, outcomes research, epidemiology, psychology, ethnography, data science, and marketing and communication have roles to play alongside the expertise that only patients with lived experience can bring.

Garabet Yeretssian, program director of Helmsley Charitable Trust's Crohn's Disease Program, explains that good patient engagement depends on how it is done. "Having data from across disciplines—health economics, epidemiology, the social sciences—is so important for educating and empowering patients to understand how they fit into a larger system. And we need to engage with that larger system, and especially regulatory agencies, to be sure our interests are aligned, and that patients' needs always come first."

To have an impact throughout a healthcare enterprise, we need more experts to apply their skills toward activities such as conducting patient preference studies, collecting and analyzing mixed qualitative and quantitative data, and engaging directly with patients as part of outcomes assessment or clinical trial design. Training in an academic setting, such as those conducted at the [Vanderbilt University Medical Center](#) and the [University of Maryland School of Pharmacy](#), incentivize patient-centric practices by teaching new researchers that patient involvement is a foundational element of R&D, thus changing the research culture long-term.

Training programs and capacity building related to the lifecycle of medical products is also crucial for patients, caregivers, patient groups, and organizations involved in bringing science to patients. Working knowledge of medical product discovery, development, and delivery allows different stakeholders to be equal partners in the process. Especially for newer or smaller patient groups, it's difficult to know which pieces of the workforce are missing or where knowledge gaps exist until faced with the need. With formalized

training, individuals across all types of organizations are more aware of the elements of medical product development and how their expertise—whether as academics, patients, advocates, or all of the above—can be applied in partnership throughout the product lifecycle.

4. Establishing representativeness
In all types of clinical research, representativeness is crucial to the application of any results to the appropriate target population. This is an important but challenging element for all parties, whether they are product developers or patient organizations.

In recent draft guidance, [the US Food and Drug Administration defines representativeness](#) in two ways: a) when the conclusions drawn from the research can be generalized to the target population, and b) when the study sample of patients reflects the heterogeneity of characteristics in the target population regardless of whether the study results can be generalized. Groups of patients can be divided into categories based on a variety of characteristics such as the age at the onset of their disease, genetic mutations, presenting symptoms, and response to a therapy. Part of the challenge is that some well-studied conditions draw these distinctions quickly and with precision, while other diseases and conditions are only starting to understand the heterogeneity of patients with that condition. For diseases and conditions where there is both within-patient and between-patient heterogeneity, it is particularly challenging to define representativeness. As part of building the science of patient input, defining and establishing methods to achieve representativeness in different populations is essential. Innovative methods of patient input like sampling from patient registries, social media, and others are also being tested and will need methods to define and achieve representativeness as well.

In the near term, there is wide agreement on the importance of these four initiatives to advance patient-centered medical product R&D despite the varying degrees of challenge associated with their execution. We look forward to participating in collective efforts and tracking the investments in these areas as we come together to build and implement metrics, training, and methods to create a sustainable patient-centered biomedical ecosystem.

BUILDING ON STRENGTHS

In addition to the four initiatives with broad agreement identified above, we found another group of initiatives that could be worthwhile investments for organizations or companies, depending on their needs and capacity. Not every company uses a target product profile or a way to make a go/no-go decision on whether to move forward with a certain product through R&D. The initiatives we discuss in this report did not have universal agreement among the experts we spoke with, but often responses were split between those from patient organizations and those from industry companies, or between groups with differing experience in patient engagement activities. However, these initiatives remain worthwhile investments for some, given that they are necessary for an organization and their patient engagement efforts.

Defining a **model patient-centered target product profile** could be worthwhile for those companies and organizations that use it for decision making. For patient organizations, being aware of the target product profile model could be useful in their conversations with companies, if only as a starting point. This foundation could demonstrate knowledge and capacity on the part of the patient organizations to understand the more nuanced elements of the medical product development process.

Other areas worth considering for more targeted investment are **initiatives to address legal challenges**, in particular, those associated with **conflict-of-interest policies, model provisions for key agreements**, and having a **general list of legal challenges** organizations can expect to face when conducting patient-centered R&D. There is a growing suite of resources and training in this arena. For example, the Food and Drug Law Institute runs training for patient organizations, and there is an ongoing effort to define reasonable agreements between patient organizations and industry.

Knowledge of legal processes, language, and resources are essential for being an active player in patient-focused activities. However, the legal challenges may be unique to the resourcing of each organization or sector. For experts speaking from the industry perspective, it is critical to **integrate legal and compliance staff into the dialogue** to bring together the desires of patient engagement with regulatory statutes and policies to create internal alignment.

Limitations on resources prevent most patient organizations from having dedicated legal staff. This can cause disruptions and issues when entering into formal partnerships. It's important therefore that small and new organizations have legal resources at their disposal.

Many nonprofits don't think of the need for conflict of interest policies; however, these policies are an early and important step to many engagement activities. The National Health Council offers recommended elements for conflict of interest policies adapted from resources from the Internal Revenue Service and the Minnesota Attorney General's office. Other resources include model provisions or agreement templates that some patient organizations provide as examples or include in larger toolkits. FasterCures has three

such toolkits, covering foundation-university partnerships, foundation-company partnerships, and foundations as collaboration conveners.

Because these initiatives are important, organizations should invest in them as the need arises given the specific nature of legal issues that vary by country, jurisdiction, and organization.

Initiatives such as **benchmarking the field** and **creating a fee schedule for services** were not considered by the experts and survey respondents to be worthwhile areas to focus, perhaps because there are organizations already working on fair market value and benchmarking patient-centricity. The umbrella organizations working on these issues are better positioned than any single patient organization or company given the effort needed to bring different partners to the table to gain general agreement.

DIFFERING PERSPECTIVES BASED ON EXPERIENCE

Many activities suffer from a lack of agreement surrounding the terms of their potential for contribution to the field of patient-centered R&D and there is a constant concern about duplication of effort. Where there is focus is sometimes determined by whether you are just starting new or you have deep expertise. For organizations with experience in the field of patient engagement, initiatives, like **building a sample patient engagement plan**, **translating trainings from Europe to the US**, **developing a research agenda, defining sources of bias in patient input**, and **developing a multi-sector pilot project to test out patient-centric practices in product development**, are potentially worthwhile investments. These are the next initiatives for organizations and companies to consider supporting and are longer-term efforts that will require a significant investment of time and resources.

Groups newer to patient engagement identified an important suite of initiatives that may reflect real-time challenges that may crop up at the outset of work. It is important to ensure that all initiatives build on work that is underway. These include **developing a patient engagement playbook**, **creating a list of pro bono legal services**, and **engaging legal staff in early patient engagement** partnerships to be greater contributors. There is an opportunity to learn from more experienced organizations. Taking the time to identify the specific gaps in the resources available and being strategic in developing new resources may be a more worthwhile use of time and energy.

### SHIFTING FROM PLANNING TO IMPLEMENTATION

Since patient engagement first made its way into the medical product R&D process, enterprising organizations have developed tools, resources, and frameworks to pave the way for others to become involved. It has been an incredibly productive time, and we now have many tools and resources to choose from to conduct patient engagement successfully across different contexts.

We have migrated from a building phase to testing these resources through case studies to see if we can turn examples into widely accepted or even standard practice. In an interview, Leah Howard, the chief operating officer at the National Psoriasis Foundation, characterized the state of the field relative to the needs that exist now, explaining "I see this topic as one that's less about needing more tools and more about best practices for using tools that already exist. How can we use these tools to ensure diverse perspectives are included while still being cognizant of the challenges patient advocacy organizations face?"

In addition to the need for implementation of the existing resources and frameworks for patient engagement, there is a need to measure success and achievement. Measuring the results of patient engagement is no small feat. In an interview, Gina Agiostratidou, the program director of T1D at Helmsley Charitable Trust, noted that "measurement that would give you an actionable item is a challenge. We don't need measurement for the sake of measurement, but we need a measurement that will improve patients' lives."

Through implementation and measuring results, we can reach a virtuous cycle where patient engagement initiatives are building off one another, and our collective investments increase the quality and speed of R&D for the benefit of all patients.
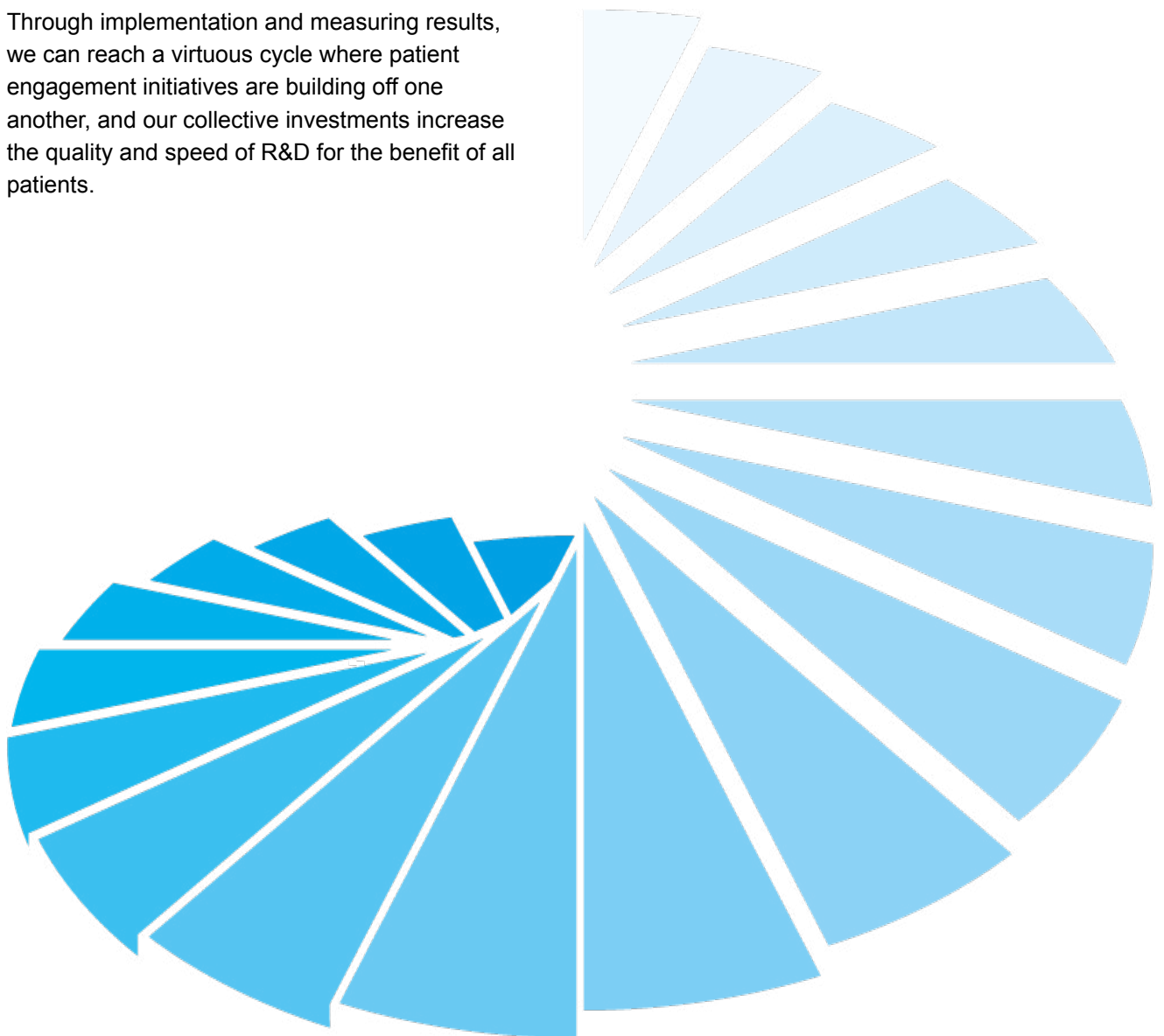
MOVING TO ACTION

Through investments over the past several years, as the science of patient input is progressing, and as the 21st Century Cures Act and regulatory legislation are being implemented, patients and advocates are building their capacity to participate and lead, and examples of how patient engagement is shaping R&D are more prevalent than ever. It will take continued focus from many organizations and directing resources both financial and skills-based to achieve a cultural shift where patient engagement is an integral and consistent element of biomedical R&D.

- **Invest where you can across the four key initiatives** related to measurement, metrics, training, and methods development

- **Consider your organizational capacity and target your investments** to areas that will accelerate progress for your patient community, as well as advance the field of patient engagement and make it easier for organizations that follow.

- The most promising initiatives will take a great deal of effort and some trial and error, but **banding together with other organizations that have interest in seeing the same gaps filled** helps solve issues with capacity, training, and expectations. Checking mapping tools like SYNaPsE can clarify who your partners can be or if someone else has already started work on a particular initiative.

- Culture change **needs involvement from all levels of an organization** including from team members not always focused on patient engagement. Be active in your organization by sharing case examples, inviting those not active to collaborative meetings, and generally make time and effort to play a role in expanding the collective effort to advance patient engagement in biomedical R&D across diseases and conditions.

If we invest now, invest wisely, and generously bring others along, we will continue moving from disparate patient engagement efforts to a measurable, valid, and sustainable science of patient input together.

APPENDIX

In 2018, FasterCures held a workshop with 20 participants representing patient organizations and medical product developers, conducted 10 key informant interviews, and distributed a survey to 40 patient organizations that have been involved in patient engagement activities to reexamine a suite of initiatives we identified in 2016, as described in our report "Expanding the Science of Patient Input: Pain Points and Potential." We removed initiatives that we feel are no longer needed, as well as all communications initiatives, as those are ongoing efforts. This resulted in 18 collaborative initiatives to rate on the **challenge to execute** and **contribution to advance patient-centricity**.

We asked two groups to rate these initiatives: members of FasterCures' Patients Count Leadership Council and organizations that participate in Patients Count Network, with 40 total responses. In addition, we conducted 10 follow-up interviews to more deeply understand the reasoning behind certain scores and the differences in rating by sector.

INITIATIVES TO CREATE TOOLS/FRAMEWORKS

Initiative	Definition
Fee schedule for routine services provided by patient organizations to industry	Establish a consensus-based schedule of customary and usual fees for routine services provided to industry by patient organizations, with considerations for customizing the fee schedule to reflect unique requirements of a particular contracting agreement or unique features of the condition of interest.
Patient engagement playbook	Develop a series of if/then statements or decision trees to guide selection of methods/tactics for engaging patients/advocates at various steps in the development of a medical product.
Sample patient engagement plan	Develop a sample plan identifying the best way to engage patients, caregivers, advocates, and/or patient organizations at various stages in the total product life cycle of a medical product.
Model patient-centered Target Product Profile	Develop a model Target Product Profile to plan a product development program "with the end in mind" that features a patient-generated description of unmet medical need, symptom/disease domains of highest priority to patients to address, and concepts important to patients in the labeling of the product.

TRAINING INITIATIVES

Initiative	Definition
Academic training program for the science of patient input	Develop curricula to enhance understanding by students and degreed professionals about the benefits of engaging patients in research and ways that methods borrowed from the fields of health economics, outcomes research, epidemiology, social sciences, and marketing sciences can be applied to elicit, collect, and interpret patient perspectives, expectations, and preferences.
Adaptation of EUPATI resources to US	Adapt the European Patients Academy on Therapeutic Innovation (EUPATI) educational toolkit and its in-depth Patient Expert Training Court based on the European Union's systems for regulatory and health technology assessment decision-making to the U.S. system.

METHODS DEVELOPMENT

Initiative	Definition
Research agenda for the science of patient input	Establish a research agenda to prioritize gaps in the knowledge base about the science of patient input that could be best addressed through coordinated research activities.
Definition of methods and tactics to achieve representativeness	Establish methods for assessing how well or accurately a sample population reflects the broader population to determine its representativeness and provide guidance on how to achieve representativeness in collecting patient input.
Description of sources of potential bias in patient input	Describe the potential sources of bias based on scholarly and practical experience. Publish in a widely read academic journal and re-assess based on feedback and as there becomes more practical experience to utilize.

COMBINATION INITIATIVE

Initiative	Definition
Multi-sponsor program for piloting patient-centric practices using actual product development	Create a forum that is protected by appropriate non-disclosure agreements and compliant with anti-trust regulations to enable willing industry sponsors to meet regularly with relevant experts to share experiences and address challenges in integrating patient perspectives into the real-time development of programs for one or more medical products.

INITIATIVES TO ADDRESS LEGAL CHALLENGES

Initiative	Definition
Collection of sample conflict-of-interest policies	Collect conflict-of-interest policies currently used by industry sponsors and patient organizations for review and discussion by a multi-disciplinary group that includes relevant stakeholders.
Model legal provisions for key agreements between patient organizations and industry	Engage a multi-stakeholder group with appropriate legal expertise to define discrete, regularly occurring scenarios in which patient organizations and industry may mutually benefit from partnering and develop template language that could serve as a model for legal agreements to guide these arrangements.
Comprehensive list of legal challenges	Assemble a multi-stakeholder group with experience in patient-focused medical product development to define the legal challenges to a productive patient organization and industry collaboration that may arise in the total product lifecycle.
Pro-bono legal services directory	Create a list of contacts within law firms that provide free or discounted professional services to nonprofit patient organizations.
Integration of legal/compliance staff into dialogue	Make consistent efforts to incorporate legal issues and experts on the agendas and faculty of meetings convened about patient engagement and patient-focused medical product development to educate other stakeholders about regulatory statutes and policies and to dispel misinformation.

INITIATIVES TO IMPROVE MEASUREMENT

Initiative	Definition
Comprehensive, unified evaluation program for benchmarking field	To measure and benchmark patient-centricity within an individual institution and across institutions, develop an integrated set of measurement questions, metrics, methods, and sources of data, using the PCORI [Patient-Centered Outcomes Research Institute] Evaluation Framework as an illustrative model.
Metrics for trust, transparency, meaningfulness of engagement	Develop rating scales or other measures to assess concepts of trust, transparency, and how meaningful engagement with patients is to research, the process of developing a medical product, or delivering a health-care service.
Checklist/scale for assessing the inclusion of patient input in product development	Develop a comprehensive checklist of steps in the total product lifecycle of a medical product where patient input could inform decision-making with rating scales to assess patient involvement as high-moderate-low-none.

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PATIENTS COUNT LEADERSHIP COUNCIL ORGANIZATIONS

- **Alkermes**
- **Amgen**
- **Arthritis Foundation**
- **Biogen**
- **Celgene**
- **Cystic Fibrosis Foundation**
- **Depression and Bipolar Support Alliance**
- **Michael J. Fox Foundation for Parkinson's Research**
- **Helmsley Charitable Trust**
- **LUNGevity Foundation**
- **Melanoma Research Alliance**
- **Merck**
- **National Psoriasis Foundation**
- **Pfizer**
- **UCB**
- **Vascular Cures**

REFERENCES

Bioethics International. n.d. BEI kicks off program to define what patient-centricity in pharma means today, w/ patient roundtable. https://bioethicsinternational. org/2017/10/17/bei-kicks-off-program-to-define-what-patient-centricity-in-pharma-means-today-w-patient-roundtable/.

Boudes, Mathieu, Paul Robinson, Neil Bertelsen, Nicholas Brooke, Anton Hoos, Marc Boutin, Ify Sargeant, and Jan Geissler. 2018. What do stakeholders expect from patient engagement: Are these expectations being met? Health Expectations. doi:10.1111/hex.12797.

2018. "Defining Patient Centeredness and Engagement in HEOR: Proposed Definition and Stakeholder Response." Baltimore, Maryland, May 21. https://www. ispor.org/docs/default-source/presentations/1388.pdf?sfvrsn=ccb5658d_1.

DIA. 2018. "Measuring Impact in Patient-Centered Drug Development Conference." Bethesda. https://www.diaglobal.org/en/conference-listing/meetings/2018/10/ measuring-impact-in-patient-centered-drug-development.

FasterCures, a Center of the Milken Institute. n.d. 21st Century Cures. https://www. fastercures.org/programs/r-and-d-policy/21cc/.

FasterCures, a Center of the Milken Institute. 2016. Expanding the Science of Patient Input: Pain Points and Potential. Milken Institute. https://www.fastercures. org/assets/Uploads/Patients-Count-workshop.pdf.

FasterCures, a Center of the Milken Institute. 2016. "Expanding the Science of Patient Input: The Power of Language." https://www.fastercures.org/assets/Uploads/ Final-FasterCures-Patient-Input-Language-Web.pdf.

FasterCures, a Center of the Milken Institute. n.d. "From Aspiration to Application: 5 Years of Patient-Centricity." https://www.fastercures.org/assets/Uploads/Patients-Count-June-2017.pdf.

FasterCures, a Center of the Milken Institute. n.d. Patient-Focused Drug Development Tracker. https://www.fastercures.org/programs/patients-count/pfdd/.

—. n.d. Patients Count Leadership Council. https://www.fastercures.org/programs/ patients-count/pclc/.

—. n.d. Patients Count Network. https://registry.fastercures.org/.

—. n.d. Toolkits. https://train.fastercures.org/toolkits/.

Food and Drug Law Institute. n.d. Introductory Courses. https://www.fdli.org/ programs/introductory-courses/.

Hoos, Anton, James Anderson, Marc Boutin, Lode Dewulf, Jan Geissler, Graeme Johnston, Angelika Joos, et al. 2015. "Partnering With Patients in the Development and Lifecycle of Medicines: A Call for Action." Therapeutic Innovation & Regulatory Science, November. doi:10.1177/2168479015580384.

Israel, Tiffany, Helena Farrow, Yvonne Joosten, and Yolanda Vaughn. n.d. "Community Engagement Studio Toolkit 2.0." https://www.vumc.org/meharry-vanderbilt/files/meharry-vanderbilt/public_files/CESToolkit%202.0.pdf.

Levitan, Bennett, Kenneth Getz, Eric L. Eisenstein, Michelle Goldberg, Matthew Harker, Sharon Hesterlee, Bray Patrick-Lake, Jamie N. Roberts, and Joseph DiMasi. 2018. Assessing the Financial Value of Patient Engagement: A Quantitative Approach from CTTI's Patient Groups and Clinical Trials Project. Therapeutic Innovation & Regulatory Science. doi:10.1177/2168479017716715.

McCleary, Kim. 2017. "Patients Count Case File: Patient Project Muscular Dystrophy Illustrates Vision for Patient-Engaged Drug Development." FasterCures. http://fastercures.tumblr.com/post/159982608484/patients-count-case-file-parent-project-muscular.

Myeloma Patients Europe. n.d. Reasonable agreements between Patient Advocates and Pharmaceutical Companies. https://www.mpeurope.org/legal_agreements/.

National Center for Advancing Translational Sciences. 2018. NCATS Toolkit for Patient-Focused Therapy Development. https://ncats.nih.gov/toolkit.

—. 2018. "NCATS Toolkit for Patient-Focused Therapy Development." https://ncats.nih.gov/toolkit.

National Health Council. n.d. "Recommended Elements for Conflict of Interest Policy." http://www.nationalhealthcouncil.org/sites/default/files/forms/soe/conflict-of-interest.pdf.

National Institutes of Health. n.d. National Center for Advancing Translational Sciences. https://ncats.nih.gov/.

Patient Focused Medicines Development. n.d. Compensation for Patient Experts at Fair Market Value. https://patientfocusedmedicine.org/fair-market-value/.

—. n.d. SYNaPsE. https://involvement-mapping.patientfocusedmedicine.org/.

—. n.d. The Patient Engagement Quality Guidance. https://patientfocusedmedicine.org/the-patient-engagement-quality-guidance/.

n.d. "Patient Groups & Clinical Trials." Clinical Trials Transformation Initiative. https://www.ctti-clinicaltrials.org/projects/patient-groups-clinical-trials.

The National Academies of Sciences, Engineering, and Medicine. 2018. "Advancing the Science of Patient Input in Medical Product R&D: Towards a Research Agenda - A Workshop." May 9. http://nationalacademies.org/hmd/Activities/Research/DrugForum/2018-MAY-09.aspx.

The University of Maryland School of Pharmacy. n.d. The PATIENTS Program. https://patients.umaryland.edu/.

U.S. Food & Drug Administration. 2017. "Patient-Focused Drug Development Public Workshop on Guidance 1: Collecting Comprehensive and Representative Input." https://www.fda.gov/downloads/Drugs/NewsEvents/UCM586195.pdf.

U.S. Food and Drug Administration. n.d. FDA Patient-Focused Drug Development Guidance Series for Enhancing the Incorporation of the Patient's Voice in Medical Product Development and Regulatory Decision Making. https://www.fda.gov/drugs/developmentapprovalprocess/ucm610279.htm.

Vanderbilt University Medical Center. n.d. Meharry-Vanderbilt Alliance. https://www.vumc.org/meharry-vanderbilt/community-engagement.

Wilkinson, Emma. 2018. "Are patients the missing piece in the drug development puzzle?" The Pharmaceutical Journal. https://www.pharmaceutical-journal.com/news-and-analysis/features/are-patients-the-missing-piece-in-the-drug-development-puzzle/20205442.article?firstPass=false.
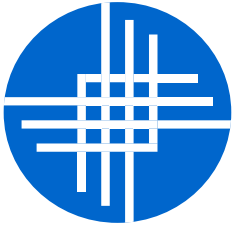
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Anna DeGarmo is an associate at FasterCures, supporting the organization's programs and external relations initiatives. Her primary responsibilities include research on the science of patient input in medical device and drug development, tracking and analyzing the progress and impact of the 21st Century Cures Act, and partnering externally to assess the breadth of FasterCures' network and identifying opportunities for growth. Prior to FasterCures, DeGarmo was a research intern at the Riverside Center for Excellence in Aging and Lifelong Health, where she aided studies focused on reducing caregiver burden and providing effective training for nursing home staff. She did further aging-related work assisting ChooseHome, a program dedicated to providing support and resources for older adults to comfortably age in place. In addition to aging, DeGarmo's interests lie in education and global health. She received her B.S. in kinesiology and health sciences from the College of William and Mary in 2017.

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