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FasterCures

PATIENTS' ACADEMY FOR RESEARCH ADVOCACY

Building Capacity for Patient-Centered R&D

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Contents

Executive summary

Both FasterCures, a nonprofit center of the Milken Institute, and The Patients' Academy for Research Advocacy, a nonprofit patient education organization, are committed to advancing the integration of patient perspectives and data in research and development (R&D) and regulatory review. With increasing interest in meaningfully engaging patients, it is critical to ensure that patients and their families are prepared and ready to engage.

On September 16, 2019, we convened an in-person workshop in Washington, D.C., with stakeholders from across the biomedical innovation ecosystem. Our goal was to arrive at a shared understanding of the needs for building capacity in patient-centered R&D through education and training of patients, care partners, and patient organizations.

As expected, participants representing different stakeholder groups held different opinions about what training is needed for patients and care partners, who should receive it, and how it should be resourced and measured. However, participants agreed there is a need for capacity-building programs to support patient engagement in R&D, and that these programs must address the needs, priorities, and perspectives of all stakeholders.

Participants generally agreed that patients and other stakeholders would be best served by a continuum of education and training for patients and care partners. A broad education on the basics of R&D, or "R&D 101," could be followed by more advanced disease-specific training and "just-in-time" training tailored to the particular needs of a specific engagement activity.

There was a desire for an organization to take the lead on developing resources for a general R&D education program that would be disease-agnostic. Disease foundations and patient organizations could then follow up with more advanced training specific to the conditions they focus on.

Furthermore, participants recognized that multiple metrics will be necessary to evaluate the impact of capacity-building programs and the return on investment for funders. Metrics will need to measure multistakeholder performance to ensure mutual accountability. And metrics will need to evolve as capacity-building programs mature and become more widely adopted.

Finally, there is a need to collaborate across stakeholder groups to achieve shared benefit. The group recognized that multistakeholder development and funding are necessary to drive impact across the whole system.

The Patients' Academy for Research Advocacy plans to develop and pilot an in-person education and training workshop for patients in 2020. The curriculum and format will draw heavily on insights from this workshop, as well as on additional feedback from patient groups,

biopharmaceutical companies, and regulators. In addition, the Patients' Academy is assembling a curriculum advisory board to assist with ensuring that this program meets the needs of multiple stakeholders for high-quality patient education that furthers the integration of patient input and preferences into drug development and regulation.

Introduction

Both FasterCures, a nonprofit center of the Milken Institute, and The Patients' Academy for Research Advocacy, a nonprofit patient education organization, work to advance the integration of patient[1] perspectives and data in research and development (R&D) and regulatory review.

FasterCures furthers its mission of promoting a high-performing, patient-centered biomedical ecosystem through engaging a network of leaders to:

- Identify systems-level challenges that prevent medicines and treatments from benefitting patients; and
- Bring forward solutions and build capacity for the private and public sectors to take action.

FasterCures never loses sight of the fact that there are approximately 10,000 known diseases affecting our world today—and yet we have treatments for only about 500 of them.

The Patients' Academy for Research Advocacy is developing in-depth education and training programs to build a larger and more inclusive community of patients and care partners who can drive improvements in the U.S. clinical research system to achieve better health outcomes.

The Patients' Academy believes that incorporating the lived experience of engaged, aware, and educated patients and care partners is critical to helping drug developers achieve the best results with the medicines they deliver to the public. By democratizing education and training for patients and care partners from any and all disease communities, we hope not only to improve participation in and outcomes of the clinical research enterprise, but also to help promote a culture in which more of us are empowered to further our own health goals and those of our communities.

The Problem—Requirements and appetites for patient input into R&D are increasing, and too few patients are ready

Stakeholders increasingly recognize that the expertise of patients and care partners is critical to business decision making in R&D, regulatory review of medical products, and beyond.

The pioneering work of patient organizations with lawmakers, regulators, and medical product developers has created an unprecedented opportunity—and an enormous unmet need—for

[1] Throughout this summary, unless otherwise noted, we use the term "patients" to include patients and care partners, including parents.

well-informed patients to help shape medical research to be more responsive and relevant to their greatest health needs.

As a result of patient organization and other stakeholder efforts, the U.S. Food and Drug Administration (FDA) is legislatively mandated to consider patient perspectives in its decision making. In turn, FDA and other regulatory bodies such as The International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH) are issuing guidelines that create an expectation for companies developing new treatments to incorporate patient perspectives, preferences, and data throughout R&D. As such, it will become untenable to conceive of and design medical products and clinical trials without patient participation and input.

More systemic adoption of patient-centered R&D practices is good news for patients and for public health. Patient leaders can focus resources and research on treating and preventing the most burdensome effects of disease, help develop clinical trials that improve the patient experience and enroll more patients more quickly, ensure that clinical data will help patients make informed treatment choices, and improve regulators' assessments of benefit-risk trade-offs by articulating the preferences of the people who will experience treatment benefits and risks.

But achieving the promise of patient-centered R&D across all diseases and therapeutic categories will require many more, and much more diverse, patient voices than have been included to date. Most patients do not know they could have a voice, or do not feel prepared to provide the kind of input needed to transform R&D to focus it firmly on patients' needs and health goals. The small number of patients who are meaningfully engaged as advisors and co-creators in R&D in most cases are not sufficiently representative of the broader patient community. And, critically, there is no system in place to prepare patients for the roles they will be invited and needed to play.

The Opportunity—Building capacity for patients and care partners to engage in meaningful collaborations with other stakeholders

To meet the increasing need for direct patient input, we must build capacity for patient experts to engage in meaningful collaborations with other stakeholders including researchers, industry sponsors who develop medical products, regulators, and policy makers.

Multiple organizations have made strategic investments to create broadly applicable, easy to find, publicly available resources to help medical product sponsors engage patients. For instance, TransCelerate Biopharma, a nonprofit industry consortium, created a comprehensive

toolkit[2] for industry to "improve engagement and partnership between biopharmaceutical companies and patients to create better experiences for clinical study participants." Separately, Patient Focused Medicines Development (PFMD) is developing training[3] for industry sponsors on how to engage with patients and care partners.

By comparison, resources for educating and training patients and care partners to participate as expert contributors in R&D are, overall, less comprehensive, more narrowly targeted, and more difficult to find. For example, several of the resources we identified in our pre-landscaping research did not surface in online searches using keywords related to patient education and training on R&D; rather, we found them only through discussions with people in our networks. Resources that do exist are frequently siloed within organizations that do not or cannot provide broad access, and therefore can be difficult for patients to find. We also observed that some resources require software applications that are complicated and confusing to use, even though both the training materials and the software are open-source.

Further, while many of existing programs for patients have supported meaningful engagement with researchers, sponsors, and other stakeholders, it is not clear whether the topics they cover are sufficient to prepare patients for the increasing variety of roles they may play in R&D partnerships. Moreover, there are no consistent measures for evaluating the impact of these programs.

FasterCures and The Patients' Academy see opportunities to identify and fill specific gaps and build a broadly applicable and accessible capacity-building program (or programs) for patients and care partners that delivers a core curriculum of content that cuts across diseases. Creating an "R&D 101" program in this fashion could provide a base of essential knowledge to the patient community more efficiently than independently creating and sequestering these resources within organizations that focus on a single disease. Those organizations could then focus their capacity-building efforts and investment on more advanced, targeted, disease-specific training that can build on an "R&D 101" program.

Workshop overview

Participants, objectives, and format

On September 16, 2019, FasterCures and The Patients' Academy convened an in-person workshop in Washington, D.C., with stakeholders from across the biomedical innovation ecosystem. Our goal was to arrive at a shared understanding of what education and training is

[2] https://transceleratebiopharmainc.com/patientexperience/patient-protocol-engagement-toolkit/
[3] https://patientfocusedmedicine.org/patient-engagement-industry-training/

needed, and to solicit ideas on how to create and maintain capacity-building programs for patients.

The workshop brought together a group of more than 50 stakeholders including patients, representatives of patient advocacy organizations and disease foundations, drug and device developers, FDA, and health-related nonprofit organizations (see registered participants in **Appendix A**).

Through presentations, breakout exercises, and group discussions, workshop participants examined the different types of education efforts, training programs, and tools that exist, the target audiences for whom they were developed, the programs' objectives, the ways they are delivered, and the costs associated with maintaining them (see workshop agenda in **Appendix B**).

Presentations included results of research conducted by FasterCures and The Patients' Academy prior to the workshop to assess the landscape of patient education and training resources and to survey stakeholders about what types of training are needed (see **Pre-workshop Research**, below).

Breakout group exercises and facilitated group discussions drove toward answering the following questions:

- Who needs additional education and training tools and programs?
- What topics or areas of knowledge that patients need are missing from existing education and training programs?
- How do we fund and build additional education and training programs for patient-centered R&D, avoid redundancy of efforts, and lift barriers to delivering training to more patients?
- How should we measure short- and long-term success of education and training programs for patients?

Pre-workshop research: Landscape analysis and needs assessment

To inform the workshop agenda and discussions, FasterCures and The Patients' Academy conducted two research projects.

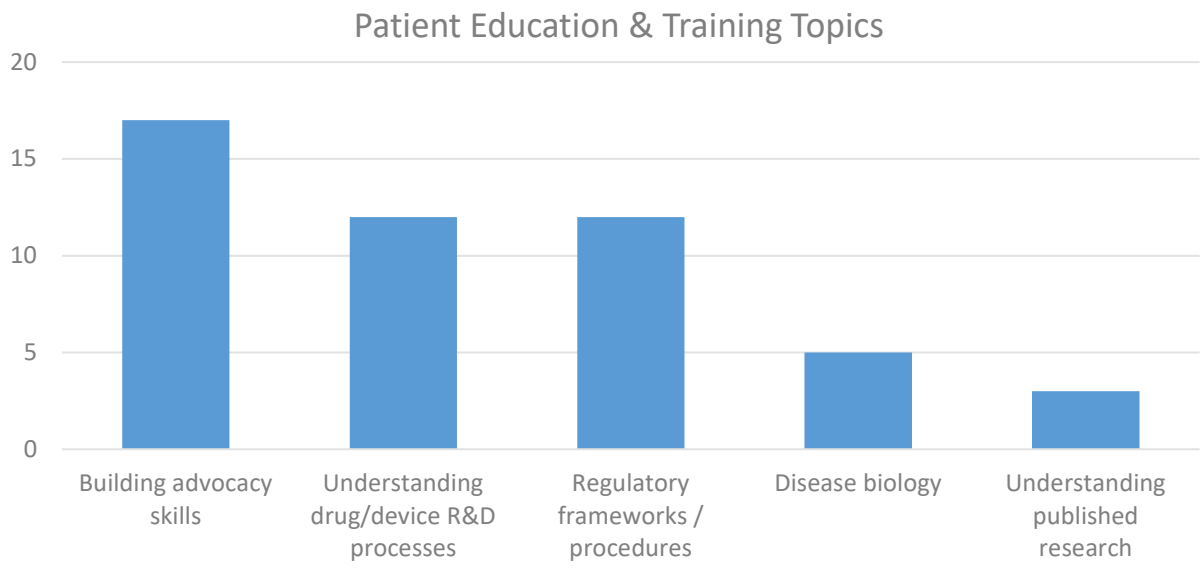
First, we searched for and analyzed existing education and training resources for patients, care partners, and patient advocates, concentrating on materials that have an explicit focus on understanding or engaging in the research, development, and approval of therapeutic drugs

and medical devices. This landscape analysis, while representative rather than comprehensive, identified 49 education and training resources for patients that were designed to support engagement in R&D.

In this preliminary cohort, the greatest number of resources exist for building advocacy skills, understanding R&D, and understanding regulatory frameworks and procedures (see **Figure 1**, below). Many resources identified in this search covered more than one topic area; in particular, "building advocacy skills" and "understanding R&D" often went hand-in-hand. Among programs developed for a particular disease community, rare diseases and cancers appear to have the most material available.

The content of these programs overlaps, indicating redundant investment. Furthermore, many of these programs are siloed within a single patient community, leaving other patient populations without access; some are open-access but not disseminated beyond the community for which they were developed, while others lie behind a member login.

***Figure 1**: Topics covered by existing patient training programs identified by FasterCures and The Patients' Academy for Research Advocacy.*



Results of the landscape analysis were distributed in advance as pre-reading material and presented during the workshop (see pre-reading material in **Appendix C**).

Separately, we conducted a needs assessment via an online survey of patients and care partners, advocacy organizations, disease foundations, biopharmaceutical or medical device

developers, and other healthcare stakeholders. The survey solicited perspectives on the need for patient education and training, topics or categories of information that are important for patients who want to engage in shaping R&D, potential benefits to both patients and industry that could come from educating and training patients, and metrics that could provide meaningful evaluation of the outcomes and effectiveness of patient capacity-building programs.

Two separate but identical versions of the survey were distributed: one version via email to more than 150 individuals who had been invited to the workshop, and the other online via the Twitter accounts of FasterCures (@fastercures) and Susan Schaeffer, President & CEO of The Patients' Academy (@biotechsusan).

While the number of respondents was small (28-35, depending on the question), the results did reveal that different stakeholders have different opinions about what patient education and training topics are important, the benefits that could come from educating and training patients, how capacity-building programs should be measured, and who should fund and lead education and training efforts for patients.

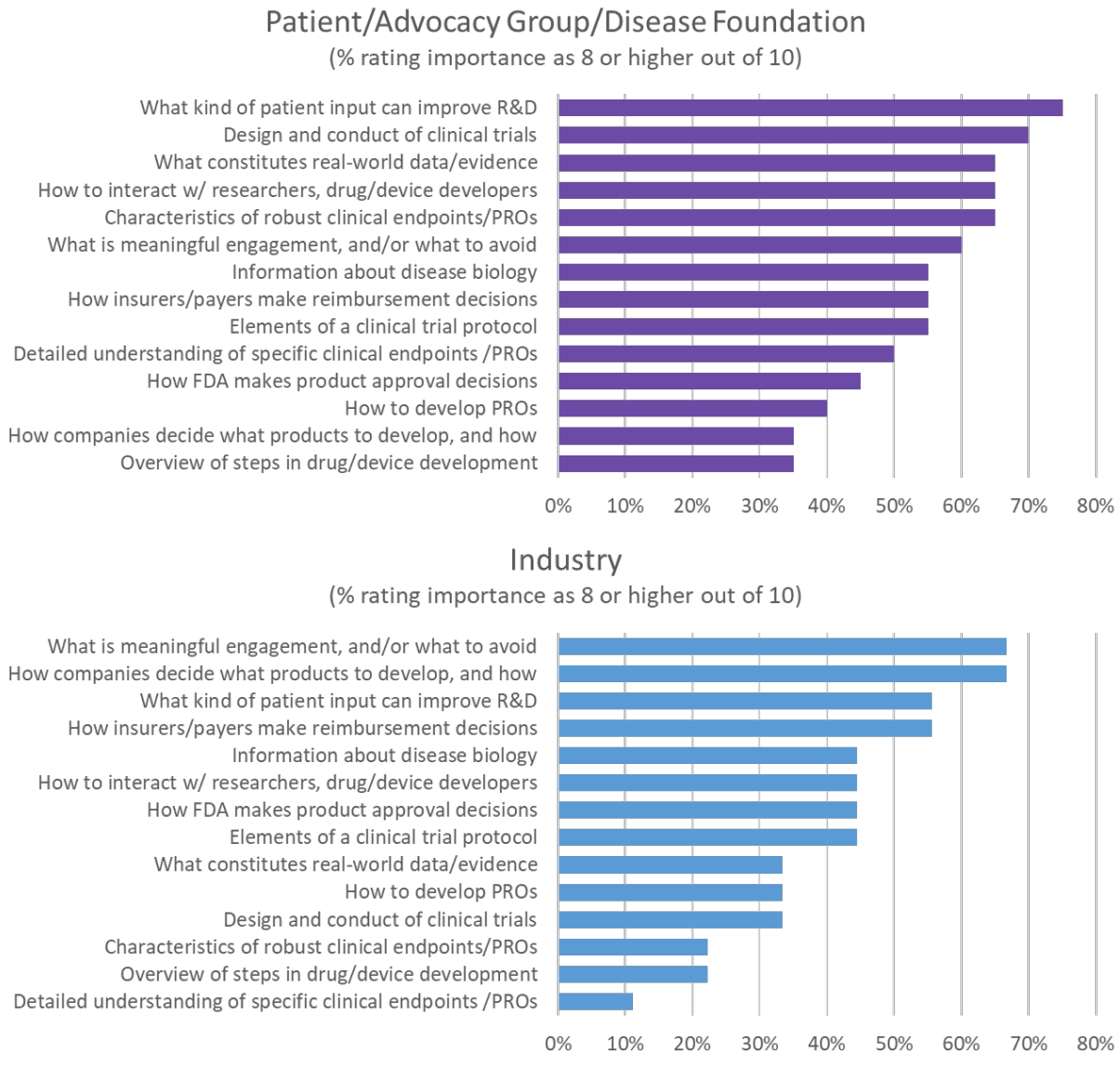
For example, we asked respondents to rate various education/training topics on their **importance to patients** using a scale of 1-10 (with 1 being not important and 10 being crucially important). At the workshop, we presented results from the 33 respondents, including 20 who self-identified as members of a patient stakeholder group (patient, care partner, or patient advocate; patient advocacy organization; or disease foundation), 9 who self-identified as members of an industry stakeholder group (biotech/pharmaceutical or medical device), and 4 who self-identified as "other nonprofits."

Overall, the patient stakeholder group rated topics related to product development higher (i.e., more important to patients) than the industry group did. Notably, a majority of the patient group assigned a rating of 8 or higher to the topics "design and conduct of clinical trials" and "elements of a clinical trial protocol," while the majority of industry respondents assigned these topics lower ratings (i.e., assigned them a lower importance to patients) (see **Figure 2**, below, and **Appendix D**). According to our landscape analysis, these topics may be underrepresented in existing capacity-building programs.

Overall, the industry group said it is important for patients to understand how companies make decisions about what products to develop and how to develop them—a topic the patient/advocacy group cohort did not rate as highly, and that is not covered in any of the capacity-building programs we identified in our landscaping research.

On the whole, both groups gave high ratings to topics covering how to engage in R&D, particularly on the topic of what kind of patient input can improve R&D (see additional information in **Appendix D**).

***Figure 2**. Responses from patient (n=20) and industry (n=9) cohorts to the question: "Please rate the following education/training topics on their importance to patients using a scale of 1-10 (with 1 being not important and 10 being crucially important)."*



Panel discussion: Factors that contribute to successful research collaborations with patients

Following welcome remarks, the workshop began with a brief presentation of the pre-workshop landscaping research, followed by a moderated panel discussion highlighting two successful R&D collaborations with patients.

The panel discussion identified common success factors underlying a collaboration between FDA's Center for Devices and Radiological Health (CDRH) and the Michael J. Fox Foundation for Parkinson's Research, and another between the Friedrich's Ataxia Research Alliance (FARA) and Reata Pharmaceuticals.

Both collaborations benefitted from the inclusion of well-informed patients who had received training on the importance of R&D and the benefits of patient participation in research.

First, Susan Walther of FARA and Kara Eichelkraut of Reata described an ongoing collaboration that began with co-design of clinical trials of the drug candidate omaveloxolone to treat Friedrich's ataxia (FA). Importantly, the idea to study the drug in FA came from the parent of a child with FA, who made the connection between research she saw presented at FARA's patient symposium, and the molecular target of omaveloxolone, which activates the Keap/Nrf2 pathway. Reata had not planned to develop omaveloxolone for FA before FARA proposed it. In October, omaveloxolone met the primary endpoint in a pivotal phase 2 trial to treat FA.

The collaboration now continues with development of educational materials for community neurologists to improve time to diagnosis of FA, which takes an average of 2 years in children and 7 in adults.

Second, Katie Kopil of the Michael J. Fox Foundation for Parkinson's Research and Annie Saha of CDRH described their partnership to design and conduct a patient-preference study on trade-offs between benefits and risks of medical devices that could be used to design clinical trials of medical devices to treat Parkinson's disease (PD).

Patients identified by the Michael J. Fox Foundation were included in every research call, helped prioritize outcomes to include in the patient preference survey, and tested the survey instrument before it was deployed to a broader community of 2,700 patients.

The panelists described success factors that were common between both collaborations:

- **Patients were prepared and empowered to participate**. While not every role required detailed or technical knowledge about the research process, all the panelists noted it was important that patients understood that research itself is important and includes

many different activities that can benefit from their participation. Reata and FARA's collaboration would not have occurred if FARA had not invested in educating its patient community about ongoing research in FA. Similarly, Kopil attributed the robust patient preference survey response to the Michael J. Fox Foundation's work to ensure the PD community understands that they can participate in research in many ways. Kopil and Walther both said that building patient knowledge empowers patients and encourages participation.

- **Collaborators had a clear discussion up front about expectations, goals, roles, and responsibilities.** All the panelists said that, without clear goals and objectives, it is hard for a patient organization to deliver. Walther added that if an industry sponsor receives no response from patient collaborators, that is a sign that goals and expectations are not clear.

- **Careful consideration of which patients should be engaged for which parts of the collaboration, depending on the task at hand.** FARA staff participated in the design of the phase 2 trial, and the organization has identified FA patients and care partners with a range of disease experience to participate in developing physician education materials with Reata. Similarly, Saha reported that the collaboration with the Michael J. Fox Foundation represented a continuum of engagement, from expert patients deeply involved in designing the survey instrument, to non-expert patients who tested and completed the survey.

- **Timely communication of research results back to patient collaborators.** Panelists said sharing results, whether good, bad, or unclear, builds goodwill and creates a virtuous cycle for patients to continue to engage in research. It also is crucial for patients to see how their feedback is being used so that they can see direct impact and feel a part of the solution. While study results often take a long time to generate, Kopil noted that the Michael J. Fox Foundation provided status updates or interim results to patient collaborators when possible, for example, reporting how many people had completed the survey.

Small group breakout 1: Are you being served?

The first breakout session was designed to identify target audiences for patient/care partner education and training. In opening remarks, session moderators Sarah Krüg of Cancer101 and Health Collaboratory, Bray Patrick-Lake of Evidation, and Roz Schneider of RozMD Patient Affairs Consulting highlighted difficulties engaging various populations.

It is notoriously difficult to reach certain populations of patients who are disproportionately affected by disease, such as Native Americans with diabetes and black men with prostate

cancer. Krüg noted that Health Collaboratory's Patient Shark Tank has found that underserved patients want to be involved in discussing burden of disease, but are more reluctant to engage in conversations about R&D and benefit-risk because they may not understand, or may not think they understand, these topics.

A key point emphasized by all the discussants is that patients' motivations to learn, to engage, and to remain engaged differ. Identifying and describing to patients a continuum of roles that are all important but that require differing levels of time commitment, training, and experience—ranging, for example, from taking surveys to advising or leading research teams—will be necessary to engage a broader range of patients in R&D.

Breakout exercise 1: Identifying populations who need education and training

Using a worksheet as a discussion tool (see worksheet in **Appendix E**), small groups chose one of three domains of patient input into R&D to work with. The domains (adapted from the National Academies of Sciences, Engineering and Medicine's Advancing the Science of Patient Input collaborative) included understanding the patient experience, imparting perspectives and preferences on benefit and risk, or informing clinical trial development/continuous improvement.

For the selected domain, each group considered what patient populations needed training based on different roles they might have in a research engagement, ranging from more passive roles such as completing surveys to highly active roles such as participating on or leading research teams or studies.

The following questions framed the discussion:

- **Who** needs and wants education/training and is underserved?
- **What** are these patients' education/training needs (if known)?
- **How** can we better understand those needs and help develop education/training to meet them?

A summary of combined comments from all small group discussions on each of the proposed domains is outlined in the table below.

Target audiences for capacity-building
Domain 1: Understanding the patient experience • Who: ○ Patients with common diseases and acute conditions where there is not a nexus or community ○ Need a global perspective, pan-disease, including patients, caregivers, and family members • What: ○ Patients involved in creating survey materials need a basic understanding of survey design and how to avoid bias ○ Designing surveys or other means of collecting data on the patient experience needs to be a bi-directional, two-way exchange between patients and researchers • How: ○ Can we look at social groups, churches, different organizations that touch these patient groups? ○ Patient-to-patient networks could educate patients participating in surveys or playing similar roles ○ Is there a way to get information to these groups through providers or researchers who are engaged with patient communities? ○ Could use registries like Force TJR (Function and Outcomes Research for Comparative Efficacy in Total Joint Replacement) as tools to engage new groups of patients
Domain 2: Imparting perspectives and preferences on benefit and risk • Who: ○ Diverse populations, including rural patients, patients with a range of health literacy, and patients of diverse races and ethnicities ○ Both patients and care partners, because their perspectives can be different ○ Patients who are well networked with patient groups and able to speak about the experience of others • What: ○ Patients participating in surveys need basic training on surveys ○ For patients who participate on advisory or governance boards, training should include their role, rules of engagement, responsibilities, purpose of involvement ○ For patients who are research partners, it would be great if we could send them to a school on the basics of research and study design. What are preference studies, how are they being used, and how do they work? They might not need to be trained on the design specifics.

- For patients serving as principal investigators (PIs) or co-PIs, training on types of bias and ways to avoid it, as well as basic trial design and statistics

- How:
  - Many patients don't have a lot of time, so need online tools they can use on their own time, but there has to be some interactive component (need more than YouTube tutorials).
  - Need to consider representation in the patient community so that you include patients who are often excluded.
  - Can segment patients who want to be more involved and find different ways to involve them

Domain 3: Informing clinical trial development/continuous improvement

- Who:
  - Older patients, those with lower socioeconomic status, rural/middle of the country, lower levels of education, people who do not see specialists, LGBT people, pregnant women, adolescents
  - Non-activated patients who are not part of an organization

- What:
  - People need to have a basic understanding of clinical trials, why their voices are important, and why they should want their voices to be heard
  - Patients should be trained on confidentiality, professional discourse
  - We should invest in long-term education to increase awareness at a younger age, e.g., college or high school
  - Cultural and early education to change the perception of research; need to destigmatize participation in research
  - Patients brought in to provide input on endpoints should have an independent relationship with FDA/be familiar with the regulatory process to minimize the view that the patients are acting on behalf of the company

- How:
  - Need targeted resources and incentives for specific populations
  - To increase awareness of clinical trials, might need to go to cultural organizations and begin with early education in schools

Facilitated group discussion 1: How to reach underserved populations

Following a readout from each breakout group, Sarah Krüg facilitated a full group discussion. She asked for examples of programs that had successfully reached underserved populations, and the group offered two examples.

The Parkinson's Foundation has funded academics from Emory University to do outreach to underserved groups, going into the community to talk about PD, research, and issues in the community. Separately, Force TJR has been able to collect patient-reported outcomes for about 10 years from a range of patients, despite the lack of an organized group or a sense of community among patients with joint replacements.

Several participants expressed concern that existing education and training resources are not accessible to underserved populations, but there was no consensus about how to make those resources more accessible. The group observed that the more onerous a training is, the less likely it is to get broad and diverse participation. In addition, compensation and incentives are likely to be as important for education and training as they are for engagement.

The group noted that underserved physicians also need education; an open dialogue between patients and physicians is necessary to improve participation in research. Blended advisory boards that include both doctors and patients are one way to improve this dialogue while speeding the process of study development and improving recruitment.

Participants also expressed a shared notion that the scale and scope of training needed, including cultural and early education about the importance of participating in research, is beyond the capacity of patient organizations alone.

Small group breakout 2: I wish I knew

The second breakout group exercise and group discussion focused on what education and training topics are necessary to support patient and care partner engagement in R&D.

In opening remarks before the breakout group exercise, Suz Schrandt of exPPect, Marilyn Metcalf of GlaxoSmithKline, and Jessica Scott of Takeda noted that differences between patient and industry responses in the portion of the pre-workshop survey that asked about the importance of various training topics (see **Figure 2**, above, and **Appendix D**) indicate that education and training programs need to address different perspectives.

Schrandt said there remains a lot of "bad" patient engagement, and that training and empowering patients is important to make engagement a meaningful and impactful part of R&D.

Metcalf noted an article published in *The Pink Sheet* on September 9, 2019, that quoted FDA's Ebony Dashiell-Aje as saying that not everything that patients think is important can be captured in a clinical trial. Metcalf said it is important to look at other ways of capturing those data.

Similarly, Scott noted that the differences between patient and sponsor goals and priorities reveals a need to focus on accomplishing things with mutual interest.

Breakout exercise 2: Topics for patient education and training

Using a worksheet as a discussion tool (see worksheet in **Appendix E**), small groups chose one of three domains of patient input into R&D, and then identified what information is essential to enable patients to engage in that domain. The domains (adapted from the National Academies of Sciences, Engineering and Medicine's Advancing the Science of Patient Input collaborative) included understanding the patient experience, imparting perspectives and preferences on benefit and risk, or informing clinical trial development/continuous improvement.

The following questions framed the discussion:

- What education or training **topics are essential** for patients/care partners?
- What existing **resources could be scaled up** to meet this need (if any)?
- What **barriers** have prevented developing or scaling education/training on these topics, and how might we overcome them?

A summary of combined comments from all small group discussions for each of the domains is outlined in the table below.

Topics for patient education and training

Domain 1: Understanding the patient experience
• Essential topics to include in training:
  ○ It's fine to have a research 101 workshop; it also would be great to have more highly trained ambassadors that could "speak the language" of product development
  ○ How data will be used (needed to establish trust)
  ○ How to tell your patient or care partner story/map your journey so that researchers come away with data they can use
  ○ The power of working collectively
  ○ What registries are, and how patients can create or contribute to them
  ○ How to talk about difficult topics such as sexual function or constipation
• Existing resources that could be scaled:

○ Genetic Alliance online educational materials[4], EUPATI training course[5], webinars from the National Health Council (NHC)[6], materials from Patient-Focused Drug Development (PFDD) meetings[7], stories from participants in clinical trials ○ How do we leverage tools that patients are already using, such as PatientsLikeMe[8]? • Barriers to developing/scaling training and ways to overcome them: ○ Barriers include lack of trust, lack of funding, and competition between organizations offering education ○ Cultural barriers and health literacies have also limited patient education and training ○ Current patient education programs may be overwhelming/too onerous ○ Having in-person, one-on-one trainings to allow people to work on problems together, and then having participants go out into the community to discuss topics is a way to overcome some of the barriers
Domain 2: Imparting perspectives and preferences on benefit and risk • Essential topics to include in training: ○ Information on the design and use of statistics and research studies ○ Disease etiology ○ Basic health and medical education ○ How to assess risk-benefit ○ Why the patient's voice is important in assessing benefit-risk • Existing resources that could be scaled: ○ Expand American Heart Association's web-based education • Barriers to developing/scaling training: ○ Information/resources are siloed and need to be shared, potentially through a third-party organization ○ Not everyone agrees patients should be involved at every step of R&D
Domain 3: Informing clinical trial development/continuous improvement • Essential topics to include in training: ○ Why research and trials are important ○ How the R&D process works from beginning to end, why it takes so long and costs so much ○ How patients can engage in the process of research and clinical trial development ○ Information about designing trials, including trade-offs that have to take place

[4] See description and links in the pre-reading material in Appendix C
[5] Ibid.
[6] https://nationalhealthcouncil.org/webinars/
[7] https://www.fda.gov/industry/prescription-drug-user-fee-amendments/fda-led-patient-focused-drug-development-pfdd-public-meetings; https://www.fastercures.org/programs/patients-count/pfdd/ (meetings as of July, 2019).
[8] https://www.patientslikeme.com/

        - Research standards, including randomization, control arms, when standard of care or placebo are used, and why
        - Inclusion and exclusion criteria, and the reasons for them
    - Who the other stakeholders are (drug developers, FDA, institutional review boards (IRBs), investigators, and so on), what their roles are, and how they make decisions
    - Vocabulary/how to talk about clinical topics
    - Laws, regulations, and labeling of medical products
- Existing resources that could be scaled:
    - None were identified
- Barriers to developing/scaling training and ways to overcome them:
    - Making information accessible to patients when and where they need it
    - Creating high-quality materials
    - Lack of consistent measures of successful education/training
    - Resistance to sharing resources and a preference for "owning" the training limit the number of patients who are trained
    - Collaboration will be necessary to overcome these barriers

Facilitated group discussion 2: Topics and types of training

Following the breakout group reports, Suz Schrandt led a moderated group discussion. Recurring themes that cut across all three research domains included that researchers and sponsors need to build trust with patients, and that patient education could help engender this trust.

Participants also noted that, while there are roles that need patients who are highly educated about R&D and conversant in the language of research, it is important not to lose the everyday patient perspective, expressed in their own words. In addition, researchers and product developers need to learn to speak patients' language.

Several participants thought a continuum of training is called for, including a general, basic training on R&D that could be followed by disease-specific training, and augmented with "just-in-time" training for specific events or engagements that would focus on the questions patients will be asked in that particular engagement. Participants agreed that the challenge lies not in developing materials, but rather in making training engaging and interactive, and designing it in such a way that builds knowledge and competence.

Many of the breakout groups also raised the difficulty of funding the maintenance of education and training materials within a single patient or disease organization. Participants expressed a desire for an organization with a remit that is broader than one disease area to take the lead on developing resources for a more general R&D education program. However, participants

emphasized that the availability of high-quality resources for patient training is not a replacement for active and in-depth engagement with patients.

Although training for industry fell outside the scope of the workshop, participants did note that both industry and patients need education on how to work together, and that such training might be done together. Patient-Focused Medicines Development (PFMD) is working on an industry training now; it should be reviewed to see whether the training, or parts of it, are fit for patients as well.

Small group breakout 3: How resourceful

The third breakout exercise and group discussion focused on a major barrier to building and maintaining capacity-building programs for patients and care partners: funding and other resources. The goal of the session was to identify new strategies for funding and resourcing these programs.

In opening remarks, Karlin Schroeder of Parkinson's Foundation, Danielle Derijcke of PFMD and EUPATI Belgium, and Barry Liden of Edwards Life Sciences described current approaches to resourcing patient capacity-building.

Schroeder described the Parkinson's Foundation's Parkinson's Advocates in Research (PAIR) program, a two-and-a-half-day in-person training program for patients and care partners that covers the drug pipeline, research ethics, evaluation of research, and how to work with research teams. The program costs $80,000 to train 30 people, including food, lodging, and travel for patient advocates and their care partners.

Parkinson's Foundation funds the training, along with contributions from industry and academic institutions. The Foundation recently received a Patient-Centered Outcomes Research Institute (PCORI) funding award to develop a new training program for patient advisory boards at PD centers of excellence. The advisory boards will engage people with PD to understand their needs and priorities and advance better outcomes through comparative effectiveness research.

Derijcke described funding strategies for EUPATI Belgium, a nonprofit training program that spun out of the European Union's EUPATI training program. The original EUPATI program was funded by government and industry through the Innovative Medicines Initiative (IMI) to develop a disease-agnostic training program for patients across the EU. EUPATI Belgium does not receive funding from EUPATI or IMI.

Initial fundraising overtures to industry were not successful; companies wanted patient training to focus on the diseases they are working in. EUPATI Belgium refocused on the cancer and epilepsy communities and was able to raise sufficient funds to conduct training workshops. The

organization subsequently faced challenges with some patient organizations who do not want to send patients to participate in an industry-sponsored program.

Liden described a program in which Edwards Lifesciences partnered with the University of Southern California (USC) to identify and host a group of patients with congestive heart failure for facilitated interviews. The program informed the company's engineers about the patients' disease experience and most troublesome symptoms. Contracting with USC instead of individual patients was a way to create a neutral venue and implement patient compensation and expense reimbursement without triggering compliance and regulatory issues for the company. The meeting led Edwards engineers to work on systems to provide real-time feedback on the condition of the heart to alleviate fear—the most impactful symptom patients reported.

Breakout exercise 3: Resources needed for patient training and education

Using a worksheet as a discussion tool (see worksheet in **Appendix E)**, small groups identified and considered new ideas to resource, fund, build, and expand patient/care partner education and training programs on R&D.

The following questions framed the discussion:

- What **resources** (money, staffing, other) are needed to develop and maintain capacity-building programs that help prepare and support patients to contribute their perspectives in R&D?
- Who are **potential funders** (besides/in addition to patient groups) and what are potential funding strategies to support development and maintenance of these programs?
- What would funders (other than patient organizations) expect in **return for investment** in patient capacity-building programs?
- How can **efficiencies** be gained by avoiding duplication of effort and investment, and what barriers will have to be overcome to achieve efficiencies?

A summary of the combined comments from the group report-outs is shown in the table below:

Resourcing capacity-building programs
What resources are needed: • Money: both seed funding to get started, and sustainable funding to keep programs going are necessary, but they are different and likely to come from different sources • Expertise, and continuity of staff to keep capacity-building programs going • Organizational commitment/buy-in • Knowledge management system to reduce duplication and share templates • Real opportunities for patients to engage in R&D (motivation for participating in education and training)
Potential funders, besides patient advocacy organizations: • Industry • Government agencies like FDA or quasi-government organizations like PCORI, especially for basic education on R&D • Foundations with a broader remit, e.g., Robert Wood Johnson Foundation, more likely to provide seed funding than maintenance funding • Contract research organizations (CROs) that are responsible for recruiting study participants • Health systems or payers seeking good patient satisfaction ratings • Venture capital firms that would value having access to patient experts • Public-private partnerships • Individual patients
What funders would expect in return: • For industry funders: ○ Actionable insights/input from patients that lead to faster recruitment, higher retention in trials, more marketable drugs ○ Improved reputation or Better Business Bureau-type seal of approval for companies that support patient education; could work with groups that are developing scorecards to include support for patient capacity-building as a metric ○ A certification program for patients to demonstrate increased competencies ○ Increased engagement activity from patients that have been educated/trained • For patient organizations ○ Drug development that better meets their patients' needs • For any funders: ○ Access to programs and materials that are developed

Ways to gain efficiencies:

- There should be an expectation that programs are open access, and that materials will be shared
- Need to put in place an infrastructure to ensure industry involvement
- "R&D 101" education and training can be pan-disease, with disease foundations and patient organizations following up with more advanced training specific to their disease or condition

Facilitated group discussion 3: Collaborating to resource capacity-building

Following the breakout group reports, Karlin Schroeder of Parkinson's Foundation moderated a full group discussion about resourcing capacity-building programs for patients.

Participants observed that no one has stepped forward to claim leadership, and each stakeholder group seems to think patient education and training is someone else's responsibility. The group agreed that multistakeholder development and funding of these education and training programs are necessary to drive impact across the whole R&D system.

Participants expressed considerable interest in the development of a certification program for patients who complete training and demonstrate a gain in expertise and competency. EUPATI fellows receive a letter confirming their completion of the course, but the program is not accredited. Developing a certification for patients who complete training could help demonstrate the value of training programs. Certification also could support ongoing work to determine fair compensation for patient experts.

Several participants also thought the development of an official endorsement for companies that support patient education, possibly by groups already developing industry performance scorecards, could increase the value proposition for industry funders.

Another idea to increase the value of patient education and training for industry funders was to develop a matchmaking platform that connects trained patients with researchers who need input.

Voting activity: Measuring success – What does it look like?

The final session of the workshop aimed to jumpstart thinking on how the outcomes and effectiveness of patient capacity-building efforts could and should be measured. While work on measuring the outcomes and impact of engaging with patients during drug R&D is in its early days, efforts to develop measures for evaluating patient education and training is even less developed.

Roslyn Schneider of RozMD Patient Affairs, David Gray of Cerevel Therapeutics, and Linda Sullivan of Metrics Champion Consortium discussed different models for developing metrics to

assess capacity-building programs and then led a group discussion about a set of candidate metrics that participants voted on earlier in the day.

Potential models of measurement

Schneider suggested that the evolution of measuring outcomes for continuing medical education (CME) for physicians may present an instructive parallel. Initially, measurement tracked only the numbers of attendees and attendee satisfaction with CME programs. Measurement has since evolved to include additional domains including improvements in physician knowledge and performance, and in patient and community health. Learning from this example, it makes sense to start with the desired impact of the training program as the outcome, and work backward to develop a measurement framework.

Gray said the benefits he has observed from soliciting and acting on patient input into clinical development—namely, increased study enrollment and retention—could be developed into metrics for evaluating patient education and training. He recounted working with graduates of Parkinson's Foundation's PAIR program to design a clinical trial. A previous trial designed without patient input enrolled at one-third the historical industry average because of the demands the protocol put on patients. Ultimately, the study had to be restarted. In contrast, he said, the study designed with patient input enrolled at double the historical rate and saved the compound, which is now in Phase 3 testing.

Sullivan presented an approach the Metrics Champion Consortium (MCC) has taken to develop a common set of metrics for clinical trial execution. Initially, the metrics CROs and sponsors focused on involved time and costs, with little focus on metrics for quality.

In the MCC framework, the first step is to be clear about defining the process to be evaluated. Next comes defining the critical success factors (what must be done to achieve the desired outcomes), and key performance metrics that will indicate if the process is on track to achieve the desired outcomes. Critical success factors and key performance metrics may vary by stakeholder.

Sullivan said the most useful metrics are specific, measurable, actionable, reliable, and timely (SMART). Effective metrics programs use a combination of metric types to produce a holistic view of performance and outcomes, including, for example, timeliness (are deadlines being met), cycle time (how long something takes), quality, and efficiency/cost. She noted, however, that metrics tend to evolve and mature along with processes. As processes get better, metrics start to move toward outcome-based measurements.

For patient education and training, Sullivan said metrics should help evaluate whether we are investing in the right type of education and training. She added that it will be important to acknowledge that different metrics will be meaningful to different stakeholders.

EUPATI's measurement model

EUPATI is one of the few capacity-building program developers that has presented data on the impact of patient training. Roslyn Schneider presented on behalf of EUPATI using data provided by EUPATI's Matthew May.

EUPATI's programs consist of both an online toolkit, and an expert training course conducted both online and in person to train fellows on 14 different topics related to R&D. The toolkit consists of an online library of documents. The course takes 14 months to complete.

The primary measure for the toolkit is reach; while the original goal was 100,000 users, the toolkit has exceeded 2 million users since launch.

EUPATI's data show that patient fellows trained in the EUPATI course have become more involved in research advocacy throughout the health ecosystem and report that they feel more respected since completing the course. Through surveys, EUPATI has tracked the percentage of fellows who have become active members, employees, or leaders within patient organizations; presented at conferences; or taken on advisory roles with pharmaceutical companies, regulatory agencies, or reimbursement authorities. EUPATI's data show increases on almost all these measures post-training (see **Figure 3**, below).

Figure 3: Change in roles for patient advocates following EUPATI training, reported as a percentage of patient fellows completing the EUPATI course.

Role	Before EUPATI	After EUPATI
Member of patient organisation, not actively involved	17%	2%
Active role in a patient organisation	62%	71%
Leadership role in a patient organisation	62%	71%
Employee of a patient organisation	25%	23%
Volunteer role in a patient organisation	60%	67%
Presenting at conferences, workshops etc.	63%	83%
Advising a pharmaceutical company	13%	44%
Advising a regulatory agency	21%	42%
Advising a reimbursement agency	4%	8%

Facilitated group discussion 4: Candidate metrics for evaluating patient education and training

Based on the metrics EUPATI is using, plus data from our pre-workshop survey, FasterCures and The Patients' Academy proposed a list of candidate metrics that could be used to assess the outcomes and effectiveness of patient education and training. By design, some of the candidates we proposed could be suitable as near-term metrics, while others would be aspirational or longer-term metrics that could be measured only over time, as education and training programs become more developed and widely used (see **Figure 4**, below).

Figure 4: Candidate metrics for assessment of patient education and training programs.

Candidate metrics
Increase in the types of engagements a patient has with other stakeholders
Increase in the types of other stakeholders a patient engages with
Numbers of engagements a drug/device developer has with trained patients
Amount of actionable input (suggestions made by patients and implemented by the recipient) other stakeholders receive from trained patients
Numbers of patients who are willing to engage in patient-centered R&D
Increase in diversity of patients who engage with other stakeholders
Improvement in patient and public perceptions of the drug/device industry
Improvement in patient and public perceptions of clinical research
Numbers of patients willing to enroll in clinical trials
Average or mean duration of engagement of patient with stakeholder
Partner report of efficacy and confidence in engaging with stakeholders
Decrease in enrollment time (from protocol approval to last patient in)

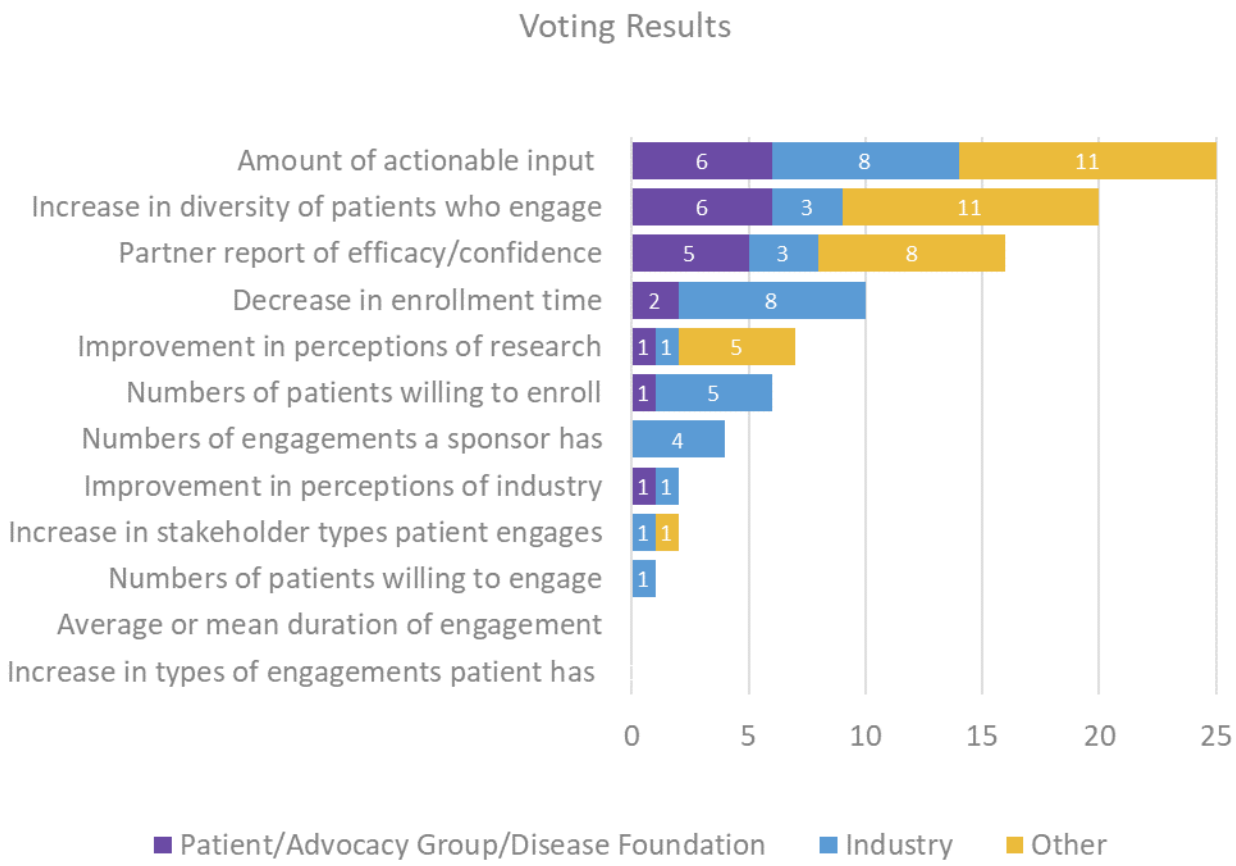
Participants voted for candidate metrics using stickers. Each participant could cast up to three votes. Cumulative voting was permitted

Participants who self-identified with the patient/advocacy group/disease foundation cohort indicated different preferences than those who self-identified with the industry cohort. However, "amount of actionable input (suggestions made by patients and implemented by the recipient) other stakeholders receive from trained patients" received the greatest number of votes from both groups. "Other" stakeholders, which included participants from FDA, health-related nonprofits that do not have a single disease focus, and academics, also cast the greatest number of votes for this metric (see **Figure 5**, below).

Participants who voted for that metric said they chose it because it felt actionable, and because it is similar to data that research teams are tracking and sharing internally. Participants also liked that this metric would encourage researchers to let patients know what parts of their input can or cannot be implemented, and why or why not. However, some expressed concern that in some cases, sponsors may not be able to disclose what they did or did not change. In

addition, this metric might penalize capacity-building programs or patients for failure to implement patient suggestions, which may not be within the patients' control.

Figure 5: *Results of workshop participant voting on candidate metrics. Each participant was given 3 votes. Cumulative voting was permitted.*



Ultimately the group agreed that "actionable feedback" or "valuable feedback" from patients was in itself a useful metric, and that the ability for sponsors to make changes based on that feedback was a separate metric. The group further noted it might not be possible in the near term to track the changes that sponsors make based on patient feedback. The group therefore suggested starting with a metric based on the amount of input that patients give and sponsors consider. The group also felt it would be most useful to track trends over time, rather than trying to interpret specific values.

Other candidate metrics that scored highly among workshop participants were "partner report of efficacy and confidence in engaging with stakeholders," and "decrease in enrollment time,"

although the latter was more important to industry participants than participants representing patient organizations or disease foundations.

Participants noted that collecting data on several of the candidate metrics would take a long time. It could therefore be helpful to develop leading and lagging metrics, although at the outset identifying leading metrics may present a challenge.

Participants also suggested considering metrics for "patients' feeling of preparedness for their role in research" and "partner assessment of patients' preparedness." PCORI uses these measures to evaluate patient training. While some of the industry participants suggested this was too far removed from an outcome that represents impact, the group asserted that multiple metrics will be necessary, and that metrics will need to measure multistakeholder performance to ensure mutual accountability.

Next steps

FasterCures and The Patients' Academy for Research Advocacy want the discussions at this workshop to benefit as many organizations and individuals participating in patient capacity-building as possible. To that end, in addition to providing this summary report to workshop attendees, we will publish it on The Patients' Academy for Research Advocacy's website and otherwise disseminate it to our networks and beyond.

In addition, The Patients' Academy for Research Advocacy plans to develop and pilot an in-person education and training workshop for patients in 2020. The curriculum and format will draw heavily on insights from this workshop, as well as on feedback The Patients' Academy continues to solicit from patient groups, biopharmaceutical companies, and regulators. We intend for the program to cover the process of R&D, principles of research and clinical trial design, and how other stakeholders make decisions during the development and approval of new drugs. The program also will include best practices for engaging with researchers, drawing on the many materials that have been developed and made available by pioneers in patient engagement who have come before.

In addition, the Patients' Academy is assembling a curriculum advisory board to assist with ensuring that our programs meet the needs of multiple stakeholders for high-quality patient education that furthers the integration of patient input and preferences into drug development and regulation. Individuals who are interested in participating on the curriculum advisory board, or in participating in patient training workshops, should contact Susan Schaeffer at susan@patients-academy.org.

Appendix A: Registered Participants

First Name	Last Name	Organization
Tanisha	Carino	FasterCures
Heather	Colvin	Johnson & Johnson
Juliana	Crawford	American Heart Association
Mark	Currie	Cyclerion
Anna	DeGarmo	FasterCures
Jen	DelGrande	Global Liver Institute
Danielle	Derijcke	PFMD
Kara	Eichelkraut	Reata Pharmaceuticals
Catherine	Ferrone	Celgene
Phyllis	Foxworth	Depression and Bipolar Support Alliance
Jennifer	French	Neurotech Network; North American SCI Consortium
David	Gray	Cerevel
Daria	Grayer	Association of American Medical Colleges
Danyel	Henry	Vertex
Courtney	Hoggard	FasterCures
Brenda	Huneycutt	FasterCures
Ellen	Janssen	Center for Medical Technology Policy
Michelle	Johnston-Fleece	PCORI
Michael	Kaplan	Melanoma Research Alliance
Annie	Kennedy	PPMD
Madeleine	Konig	American Heart Association
Katie	Kopil	Michael J. Fox Foundation for Parkinson's Research
Sara	Krug	Cancer 101
Sara	Latham	COPD Foundation
Barry	Liden	Edwards Lifesciences
Megan	Martin	American Diabetes Association
Marilyn	Metcalf	GlaxoSmithKline
Sylvia	Ncha	National Academies
Jan	Nissen	MSD
Kathryn	O'Callaghan	FDA
Bray	Patrick-Lake	Duke Clinical Research Institute
Ali Sue	Patterson	Cystic Fibrosis Foundation

Raymond	Puerini	FasterCures
Eric	Racine	Sanofi
Michele	Rhee	Enzyvant
Amy	Rick	FDLI
Liliana	Rincon Gonzalez	Medical Device Innovation Consortium (MDIC)
Christina	Roman	Cystic Fibrosis Foundation
Anindita	Saha	FDA CDRH
Lizzy	Salinas	Chan Zuckerberg Initiative
Susan	Schaeffer	Patients Academy for Research Advocacy
Jennifer	Schleman	National Health Council
Roslyn	Schneider	RozMD Patient Affairs Consulting
Karlin	Schroeder	Parkinson's Foundation
Jessica	Scott	Takeda
Samir	Shaikh	FDA
Carolyn	Shore	National Academies
Mark	Skinner	Institute for Policy Advancement Ltd
Lana	Skirboll	Sanofi
Desiree	Steele	Medical Device Innovation Consortium (MDIC)
Lisa	Stewart	PCORI
Linda	Sullivan	Metrics Champion Consortium
Ellen	Tambor	Center for Medical Technology Policy
Michelle	Tarver	CDRH
Rachel	Tunis	FasterCures
Rebecca	Vermeulen	Roche- Genentech Pharmaceuticals
Susan	Walther	Friedreich's Ataxia Research Alliance (FARA)
Laura	Weidner	Epilepsy Foundation
Richard	Willke	ISPOR

Appendix B: Workshop Agenda

Workshop: Building Capacity for Patient-Centered R&D
September 16, 2019
8:00-4:15

Milken Institute School of Public Health at GW
950 New Hampshire Ave. NW
Washington D.C. 20052

Stakeholders increasingly recognize that the expertise of patients and care partners is critical to business decision making in R&D, regulatory review of medical products, and beyond. To meet that need, it is critical to build capacity for patient experts to engage in meaningful collaborations with other stakeholders including researchers, product developers, regulators, and policy makers. Both FasterCures, a Center of the Milken Institute, and The Patients' Academy for Research Advocacy are committed to advancing the integration of patient perspectives and data in R&D and regulatory review.

The Patients' Academy for Research Advocacy seeks to understand the current landscape and future needs for capacity-building for patients as integral players in R&D so that the organization can better contribute to filling the gaps. Our goal at this workshop is to arrive at a shared understanding of needs and opportunities for building capacity in patient-centered R&D through education and training of patients, care partners, and patient organizations.[1]

Through presentations and group discussions, we will examine the different types of education and training programs and tools that exist, the target audiences for whom they were developed, their objectives, the ways they are delivered, and the costs associated with maintaining them.

Participants should come ready to talk about where new education and training are needed, barriers to scaling up and filling the gaps—including but not limited to funding—and proposals and methods for overcoming those barriers.

In particular, we will drive the conversation toward developing answers to the following key questions:

- **Who** needs additional education and training tools and programs?

[1] The Washington Post, Fact checker, Are there really 10,000 diseases and just 500 "cures"?, available at: https://www.washingtonpost.com/news/fact-checker/wp/2016/11/17/are-there-really-10000-diseases-and-500-cures/ (accessed Sept. 6, 2019).

- **What** topics or areas of knowledge that patients need are missing from existing education and training programs?

- **How** do we fund and build additional education and training programs for patient-centered R&D, avoid redundancy of efforts, and lift barriers to delivering training to more patients?

- **How** should we measure short- and long-term success of education and training programs for patients?

Agenda		
8:00-8:30	**I.**	**Welcome, Opening Remarks, and Introductions** Brenda Huneycutt, Director, Regulatory Innovation, FasterCures, a Center of the Milken Institute Susan Schaeffer, President & CEO, The Patients' Academy for Research Advocacy
8:30-9:40	**II.**	**Having an Impact** Moderator: Susan Schaeffer, President & CEO, The Patients' Academy for Research Advocacy **Session objective:** Set the stage for the day's discussions by presenting the range of patient capacity-building programs that exist and spotlighting successful collaborations with patients. Can we identify common success factors? **8:30-8:40 What Capacity-Building Looks Like: Results of pre-meeting landscaping** • Brenda Huneycutt, Director, Regulatory Innovation, FasterCures, a Center of the Milken Institute **8:40-9:40 Factors that Contribute to Successful Research Collaborations with Patients** • Kara Eichelkraut, Senior Manager, Patient Advocacy, Reata Pharmaceuticals • Katie Kopil, Director, Research Programs, Michael J. Fox Foundation for Parkinson's Research

	• Anindita (Annie) Saha, Director, Partnerships to Advance Innovation and Regulatory Science, CDRH • Susan Walther, Director of Patient Engagement, Friedrich's Ataxia Research Alliance Open discussion
9:40-10:45	**III. Are You Being Served?** Moderator: Sarah Krüg, Executive Director, Cancer101 and Founder, Health Collaboratory Discussant: Bray Patrick-Lake, Director, Strategic Partnerships, Evidation Discussant: Roslyn L. Schneider, MD, MSc, Principal, RozMD Patient Affairs Consulting LLC **Session objective:** Identify target audiences for education and training. Identify underserved populations and brainstorm ways to reach patients who are not being served by existing education/training. **9:40-10:10** Breakout sessions to identify target audiences and gaps • Who in the patient community needs education/training, and why? • Identify disease communities whose needs for education/training are not being met (e.g., many primary care diseases? acute diseases that progress rapidly?). • For organizations/disease communities that do have education/training programs but still have subpopulations with unmet needs, identify constituents who are not served, and the reasons why. • Brainstorm how training could reach those not being served. • How do you know when a patient community is served enough? **10:10-10:45** Breakout group report-out and facilitated group discussion of the results
10:45-11:00	**Break**

11:00-12:10	**IV.**	**I Wish I Knew** Moderator: Suz Schrandt, JD, Founder, CEO & Chief Patient Advocate, exPPect and Senior Patient Engagement Advisor, SIDM Discussant: Marilyn Metcalf, Patient Engagement Lead, GlaxoSmithKline Discussant: Jessica Scott, MD, JD, Head of R&D Patient Engagement, Takeda Pharmaceuticals **Session objective:** Identify topics or categories of information that are essential to effective patient-centered R&D and are missing from existing capacity-building programs. **11:00-11:10** Results of the pre-meeting needs assessment survey Susan Schaeffer, President & CEO, The Patients' Academy for Research Advocacy **11:10-11:40** Breakout sessions to discuss areas where information and expertise are wanting and identify the reasons for lack of information and training in these areas **11:40-12:10** Breakout group report-out and facilitated group discussion of the results **12:10-12:15** Instructions for voting activity Susan Schaeffer, President & CEO, The Patients' Academy for Research Advocacy
12:15-1:30	**LUNCH** Begin voting activity for section VI	
1:30-2:30	**V.**	**How Resourceful** Moderator: Karlin Schroeder, Senior Director, Community Engagement, Parkinson's Foundation Discussant: Danielle Derijcke, Program Manager, Patient-Focused medicines Development, and Board Member and Communications Officer, EUPATI Belgium Discussant: Barry Liden, VP, Patient Engagement, Edwards Lifesciences **Session objective:** Determine ways to resource, maintain, and expand capacity-building programs.

		• Discuss the resources (e.g., money, staffing, other?) needed to develop and maintain capacity-building programs.
		
		• Identify successful funding/resourcing strategies.
		
		• Explore whether and how efficiencies could be gained by training larger numbers of people, potentially across different diseases, compared with one-off programs siloed in single organizations.
		
		  ○ Are there particular areas of patient education/training that would benefit from getting people from different disease communities in the same room?
		
		  ○ If so, how could disease-agnostic or pan-disease programs be funded? By whom?
		
		• Discuss what roles other stakeholders (besides patients/patient advocacy organizations) should have in supporting capacity building.
		
		1:30-1:40 Introductory comments
		
		1:40-2:00 Breakout sessions to discuss ways to resource and maintain patient training and education
		
		2:00-2:30 Breakout group report-out and facilitated group discussion of the results

2:30-2:45	**BREAK**	
2:45-3:45	**VI.**	**Measuring Success—What does it look like?** Moderator: Roslyn L. Schneider, MD, MSc, Principal, RozMD Patient Affairs Consulting LLC Discussant: David Gray, VP, Cerevel Therapeutics Discussant: Linda Sullivan, Co-founder and Executive Director, Metrics Champion Consortium **Session objective:** Discuss how we can measure outcomes and effectiveness of patient capacity-building efforts, and identify what aspects of capacity-building programs contribute to achieving success (e.g., clear focus on target audience, purpose/desired outcomes, method for deploying training, cost/sustainability, multistakeholder participation).

3:45-4:15	**VII.** Action Agenda Susan Schaeffer, President & CEO, The Patients' Academy for Research Advocacy Tanisha Carino, Executive Director, FasterCures, a Center of the Milken Institute **Session objective:** Summarize discussions and state action items. • What stakeholders or target audiences need additional training tools and programs? • What topics or areas of knowledge need to be added to training programs? • What is needed to build additional capacity for patient-centered R&D and to lift barriers to scaling up training? • How will we measure whether capacity-building is working?

Appendix C: Workshop Pre-read Material

Building Capacity in Patient-Centered R&D
September 16, 2019 Workshop Pre-Read

FasterCures' Mission

FasterCures, a nonprofit center of the Milken Institute, works to further its mission of promoting a high-performing, patient-centered biomedical ecosystem. We do this by engaging a network of leaders to:

- Identify systems-level challenges that prevent medicines and treatments from benefitting patients; and
- Bring forward solutions and build capacity for the private and public sectors to take action.

We never lose sight of the fact that there are approximately 10,000 known diseases affecting our world today – and yet we have treatments for only about 500 of them.[1]

The Patients' Academy for Research Advocacy's Mission

The Patients' Academy for Research Advocacy is a nonprofit that is working to expand the ranks of patients and care partners who are willing and able to engage in research as full partners with a unique ability to advance science and medicine.

We do this by developing educational programs that prepare patients and care partners to engage in ways that drive medical research toward improving the health outcomes that matter most to them.

We do this because incorporating the lived experience of engaged, aware, and educated patients and care partners is critical to helping drug developers achieve the best results with the medicines they deliver to the public.

[1] The Washington Post, Fact checker, Are there really 10,000 diseases and just 500 "cures"?, available at: https://www.washingtonpost.com/news/fact-checker/wp/2016/11/17/are-there-really-10000-diseases-and-500-cures/ (accessed Sept. 6, 2019).

Building Capacity in Patient-Centered R&D

Patient organizations offer numerous resources to the communities they serve, often providing significant assistance with a patient's diagnosis and disease journey, help accessing tools and resources to navigate the healthcare system, and a forum to bring the community together to share experiences and raise awareness. In addition, some patient-focused organizations offer tools, training, and/or other resources designed for patients who want to learn about and get involved with the medical research and development (R&D) process.

Objectives for the September 16, 2019 Workshop

The Patients' Academy for Research Advocacy seeks to understand the current landscape and future needs for capacity-building for patients as integral players in R&D so that the organization can better contribute to filling the gaps. Our goal at this workshop is to arrive at a shared understanding of needs and opportunities for building capacity in patient-centered R&D through education and training of patients, care partners, and patient organizations.[2]

In particular, we will drive the conversation toward developing answers to the following key questions:

- **Who** needs additional education and training tools and programs?
- **What** topics or areas of knowledge that patients need are missing from existing education and training programs?
- **How** do we fund and build additional education and training programs for patient-centered R&D, avoid redundancy of efforts, and lift barriers to delivering training to more patients?
- **How** should we measure short- and long-term success of education and training programs for patients?

Capacity-Building Resources for Patients

In preparation for this workshop, FasterCures and The Patient's Academy for Research Advocacy conducted a preliminary search of capacity-building resources for patients related to medical R&D. Our search was not intended to be comprehensive and most certainly did not identify every resource available. However, we identified more than 40 resources specifically designed to help patients learn about the research process and/or get involved as research advocates (listed in Appendix 1, attached).

[2] We recognize the complementary need to train other stakeholders to engage with patients but will not discuss those needs at this workshop.

In Figure 1, below, we mapped these resources according to delivery format and topic areas covered, and, where relevant, noted the disease group(s) that is the intended audience(s). The table attached as Appendix 2 defines what we included in each topic area in the top row of the matrix. It is important to note that topic areas are not entirely distinct, and many resources cover more than one topic area; therefore, one resource may appear more than once in the matrix. In particular, "building advocacy skills" and "understanding drug and device R&D" are often paired together and/or contain overlapping elements. Appendix 1 lists which topics area(s) we assigned to each resource.

Based on this initial scan, rare diseases and cancers appear to have the most material available, outside of resources that are not focused on one disease ("pan-disease"). Additionally, we found more resources for building advocacy skills, followed by those on understanding R&D and understanding regulatory frameworks and procedures, than for the other topics.

FIGURE 1. MATRIX: PATIENT TRAINING & EDUCATION MAP OF IDENTIFIED RESOURCES

	Building advocacy skills	Disease biology	Understanding published research	Understanding drug/ device R&D	Regulatory frameworks and procedures
In-person workshops/ trainings	-Breast cancer (2) -Parkinson's (1) -Elderly patients (1) -Liver patients (1)	-Breast cancer (1)	-Breast cancer (1) -Parkinson's (1)	-Elderly patients (1)	-Pan-disease (2)
Webinar(s)	-Pan-disease (1)			-Rare disease (1) -Cancer (1)	
Online curriculum/ course	-Pan-disease (1) -Cancer (1)			-Pan-disease (1)	-Pan-disease (1)
Video(s)	-Arthritis (1) -Asthma (1) -Cancer (1)	-Asthma (1)		-Asthma (1)	-Pan-disease (1)
Document library/ toolkit/ webpage/ white paper	-Rare disease (2) -Alopecia areata (1) -Cancer (1)			-Rare disease (1) -Alopecia areata (1) -Asthma (1)	-Pan-disease (2) -Rare disease (1) -Cancer (2)
Combination of above	-Lung cancer (1) -Cancer (1)	-Colorectal cancer (1) -Rare disease (1) -Cancer (1)	-Lung cancer (1)	-Pan-disease (1) -Rare disease (2) -Cancer (1)	-Pan-disease (2) -Rare disease (1)

Next Steps

FasterCures and The Patients' Academy for Research Advocacy want to share the fruits of this workshop broadly with stakeholders who may be interested in developing or participating in patient and care partner training. To that end, a summary report will be circulated to attendees, individuals who responded to the pre-workshop survey, and other stakeholders.

Additionally, The Patients' Academy for Research Advocacy expects to incorporate information from the workshop discussions into the design and delivery of its education and training programs for patients and care partners. We will establish a curriculum advisory committee to help guide this process.

If you are interested in participating in the committee, please speak with Susan Schaeffer at the workshop or email her at susan@patients-academy.org.

APPENDIX 1: identified Patient training & Education resources

Organization	Resource Title & Hyperlink to Webpage	**Target patient group(s)**	Resource Type[3]	Topic Area(s)
Alamo Breast Cancer Foundation (ABCF)	Patient Advocate Program	Breast Cancer	In-person workshop/ training	Building advocacy skills; understanding published research
American Association for Cancer Research (AACR)	How to Navigate a Scientific Meeting	Cancer	Doc library/ toolkit/ webpage/ white paper	Building advocacy skills
American Association for Cancer Research (AACR)	How to Read and Assess Research Articles	Cancer	Doc library/ toolkit/ webpage/ white paper	Building advocacy skills
American Association for Cancer Research (AACR)	Tips to Interpret a Scientific Poster	Cancer	Doc library/ toolkit/ webpage/ white paper	Building advocacy skills
Arthritis Foundation	Juvenile Arthritis Research Full Video	Arthritis	Video(s)	Building advocacy skills
Asthma and Allergy Foundation of America	Promoting Asthma Patient Engagement in Research (PAPER)	Asthma	Video(s)	Disease biology; building advocacy skills; understanding drug and device R&D
Asthma and Allergy Foundation of America	Asthma Patient-Centered Research Training	Asthma	Doc library/ toolkit/ webpage/ white paper	Understanding drug and device R&D
Cancer Information & Support Network	CISN Webinars	Cancer	Webinar(s)	Understanding drug and device R&D
Cancer Support Community/ Cancer Policy Institute	Working with Regulators: A Focus on CMS	Cancer	Doc library/ toolkit/ webpage/ white paper	Regulatory frameworks and procedures
Cancer Support Community/ Cancer Policy Institute	Working with Regulators: A Focus on the FDA	Cancer	Doc library/ toolkit/ webpage/ white paper	Regulatory frameworks and procedures
DREAMS (Developing a Research participation Enhancement and Advocacy training prograM for diverse Seniors)	DREAMS Toolkit/ DREAMS Team Standardized Test and Answer Key	Elderly patients	Online course	Building advocacy skills; understanding drug and device R&D
EMA	Training Resources for Patients and Consumers	Pan-disease	Combination	Regulatory frameworks and procedures
EMA	Training Manual: Review of EMA Documents	Pan-disease	Doc library/ toolkit/ webpage/ white paper	Regulatory frameworks and procedures
EMA	Annual In-House Training Session	Pan-disease	Combination	Regulatory frameworks and procedures
EUPATI	Patient Expert Training Course and Toolbox on Medicines R&D	Pan-disease	Online course	Regulatory frameworks and procedures
EUPATI	Toolbox on Medicines R&D	Pan-disease	In-person workshop/ training	Regulatory frameworks and procedures
European Lung Foundation (ELF) and the National Institute for Health Research Collaboration for Leadership in Applied Health Research and Care (NIHR CLAHRC) for Leeds, York and Bradford	European Patient Ambassador Program (EPAP)	Pan-disease	Online course	Building advocacy skills

[3] Resource types include: in-person workshops/trainings, webinars, online curricula/courses, videos, document libraries/toolkits/web pages/white papers, and combinations of these formats.

EURORDIS-Rare Diseases Europe	Open Academy, EURORDIS Winter School on Scientific Innovation and Translational Research	Rare disease	Combination	Disease biology; understanding drug and device R&D
EURORDIS-Rare Diseases Europe	Open Academy, EURORDIS Summer School	Rare disease	Combination	Understanding drug and device R&D; regulatory frameworks and procedures
FasterCures	Benefit-Risk Bootcamp	Pan-disease	Doc library/ toolkit/ webpage/ white paper	Understanding drug and device R&D; regulatory frameworks and procedures
FDA	FDA.gov/patients	Pan-disease	Doc library/ toolkit/ webpage/ webinar	Regulatory frameworks and procedures
FDA	Patients Matter Video Series	Pan-disease	Video(s)	Regulatory frameworks and procedures
Food and Drug Law Institute (FDLI)	Patient Organizations: An Introduction to Drug and Device Law and Regulation	Pan-disease	In-person workshop	Regulatory frameworks and procedures
Fight Colorectal Cancer (Fight CRC)	Research Advocacy Training and Support (RATS)	Colorectal cancer	Combination	Disease biology
Friends of Cancer Research	Progress for Patients		Online course	Building advocacy skills
Genetic Alliance	Navigating the Ecosystem of Translational Science (NETS) toolkit	Rare disease	Doc library/ toolkit/ webpage/ white paper	Understanding drug and device R&D; regulatory frameworks and procedures
Genetic Alliance	Advocacy ATLAS toolkit	Rare disease	Doc library/ toolkit/ webpage/ white paper	Building advocacy skills
Global Genes	Rare University	Rare disease	Online course	Disease biology; understanding drug and device R&D
Global Liver Institute	Advanced Advocacy Academy	Liver disease	In-person workshop/ training	Building advocacy skills
International Association for the Study of Lung Cancer (IASCL)	Supportive Training for Advocates in Research and Science (STARS)	Lung cancer	Combination	Building advocacy skills; understanding published research
Michigan Public Health Institute	Into to Research for the Non-Researcher	Rare disease	Webinar(s)	Understanding drug and device R&D
MIT NEWDIGS	Adaptive Biomedical Innovation game	Pan-disease	Online course	Understanding drug and device R&D
National Alopecia Areata Foundation	Patient Centered Outcome Research & Comparative Effectiveness Research	Alopecia Areata	Doc library/ toolkit/ webpage/ white paper	Building advocacy skills; understanding drug and device R&D
National Breast Cancer Coalition	Project LEAD	Breast Cancer	In-person workshop/ training	Disease biology; building advocacy skills
National Cancer Institute	Research Advocacy 101	Cancer	Video(s)	Building advocacy skills
National Organization for Rare Diseases (NORD)/ University of Maryland	NORD Rare Diseases & Orphan Products Breakthrough Summit: Special Training for Rare Disease Patient Advocates	Rare disease	Doc library/ toolkit/ webpage/ white paper	Building advocacy skills
North American Primary Care Research Group	Patient and Clinician Engagement (PaCE) Webinar Series	Pan-disease	Webinar(s)	Building advocacy skills

Parkinson's Foundation	Parkinson's Advocates in Research (PAIR)	Parkinson's	In-person workshop/ training	Building advocacy skills; understanding published research
Research Advocacy Network	Advocate Institute	Cancer	Combination	Disease biology; building advocacy skills; understanding drug and device R&D

APPENDIX 2. GUIDE TO TOPIC AREAS

Building advocacy skills	• Learning the benefits of participating in research and how patient advocates advance the field
	• Connecting and networking with other advocates
	• Translating personal experience into activism
	• Identifying personal strengths as an advocate
	• Learning how to influence policy related to research/ R&D
	• Communicating about health and preferences as a patient advocate
	  ○ Collaborating and communicating with researchers, developers, and regulators
	  ○ Participating in healthcare conferences and navigating scientific meetings
	  ○ Providing feedback on a research protocol
	  ○ Developing a funding or grant proposal

Disease biology	• Precision medicine and targeted therapy • Genetics and genome editing • Biomarkers • Tumor biology/ immunology • Family & heredity
Regulatory frameworks and procedures	• Key programs of FDA/ EMA/ CMS • How drugs/ treatments are evaluated • Relevant terminology • Drug/ device approvals and the drug development process • How patients can engage with FDA/ EMA/ CMS • Submitting comments • Where to find information about clinical trials
Understanding drug/ device R&D	• Language and concepts of science and participatory research • Research design and concepts ○ Different types of research: comparative effectiveness research, observational, interventional, etc. • How research gets translated into diagnoses/ therapies • Information on clinical trials and their structure • Understanding benefit-risk assessment • Understanding implementation, dissemination, and access

	• Ethics in R&D ○ Informed consent • Thinking critically about ethical considerations in research

Understanding published research and medical news in the media	• Updates on disease-specific scientific research and advances
	  ○ What's in the pipeline
	• Evaluating research
	  ○ Understanding scientific papers and posters
	  ○ Understanding peer review
	  ○ Assessing for accuracy

Appendix D: Selected Survey Results

An online survey conducted by The Patients' Academy for Research Advocacy and FasterCures asked patients, patient advocacy groups, disease foundations, drug and device developers, regulators, and other healthcare stakeholders to rate the following 14 potential training topics on their **importance to patients** from 1 to 10 (with 1 being not important and 10 being crucially important. Training topics were drawn from existing programs and stakeholder interviews:

PRODUCT DEVELOPMENT	Overview of steps in drug/device development from early research through approval
	Design and conduct of clinical trials
	Elements of a clinical trial protocol
ENDPOINTS	Characteristics of robust clinical endpoints and patient-reported outcomes (PROs)
	Detailed understanding of specific clinical endpoints and PROs for specific diseases
	How to develop PROs
STAKEHOLDER DECISION MAKING	How companies decide what products to develop, and how to develop them
	How FDA makes product approval decisions
	How insurers and payers make reimbursement decisions
HOW TO ENGAGE	How to interact with researchers or drug/device developers
	What kind of patient input can improve R&D
	What constitutes meaningful engagement, and/or what types of engagement to avoid
SCIENCE & DATA	Information about disease biology
	What constitutes real-world evidence (RWE)

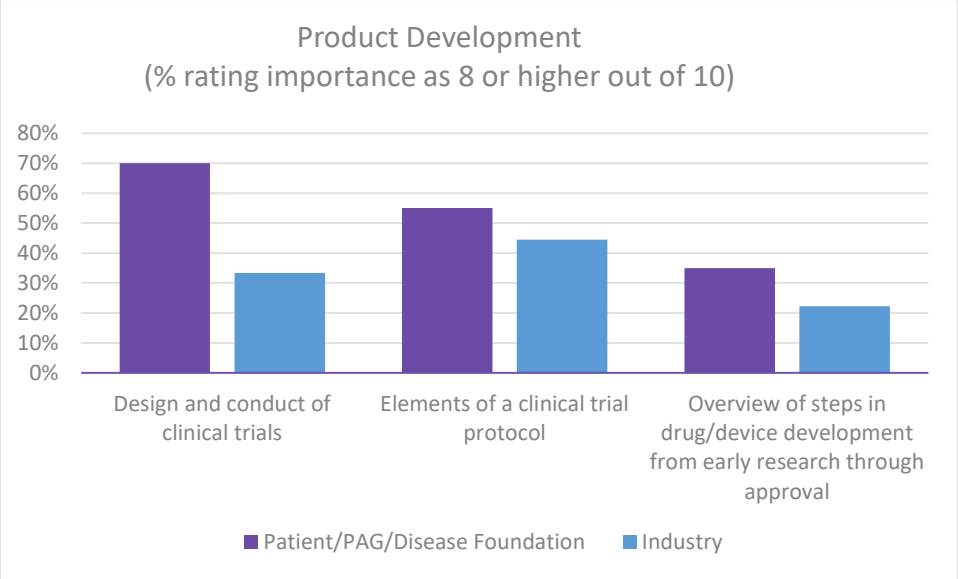
With 33 responses, the sample was too small to conclude what topics should be the highest priority. However, comparing responses from patient stakeholder groups with those from industry stakeholders suggests a need for each group to learn about and understand the other's objectives for patient training and education.

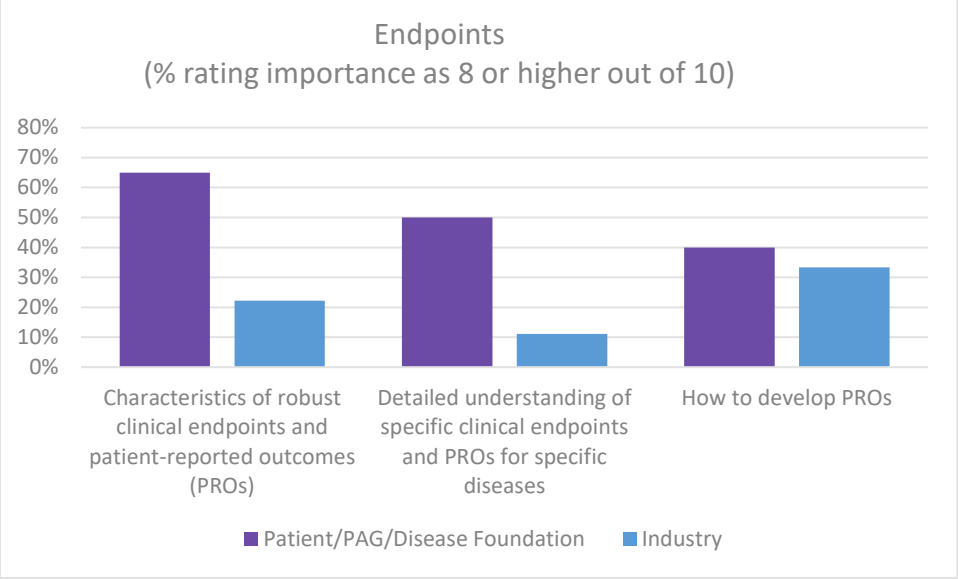
There were 20 respondents who identified with patient stakeholder groups (patient, care partner, or patient advocate; patient advocacy organization; or disease foundation), 9 who identified with either biotech/pharmaceutical or medical device industry stakeholder groups, and 4 who identified as "other nonprofits."

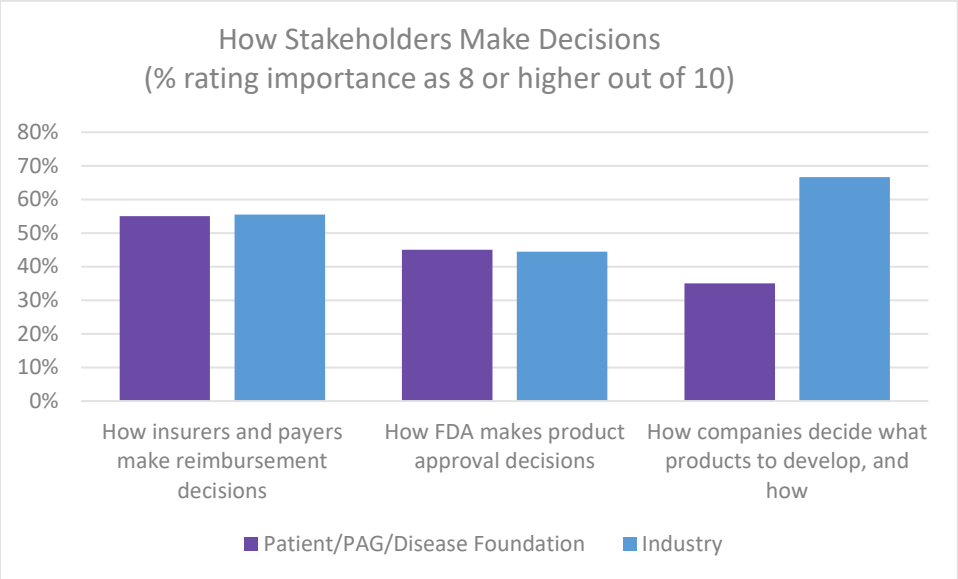
Overall, the patient stakeholder group rated topics related to product development higher (i.e., of more importance to patients) than the industry group did. Notably, a majority of the patient group assigned a rating of 8 or higher to the topics "design and conduct of clinical trials" and "elements of a clinical trial protocol," while the majority of industry respondents assigned these topics lower ratings.

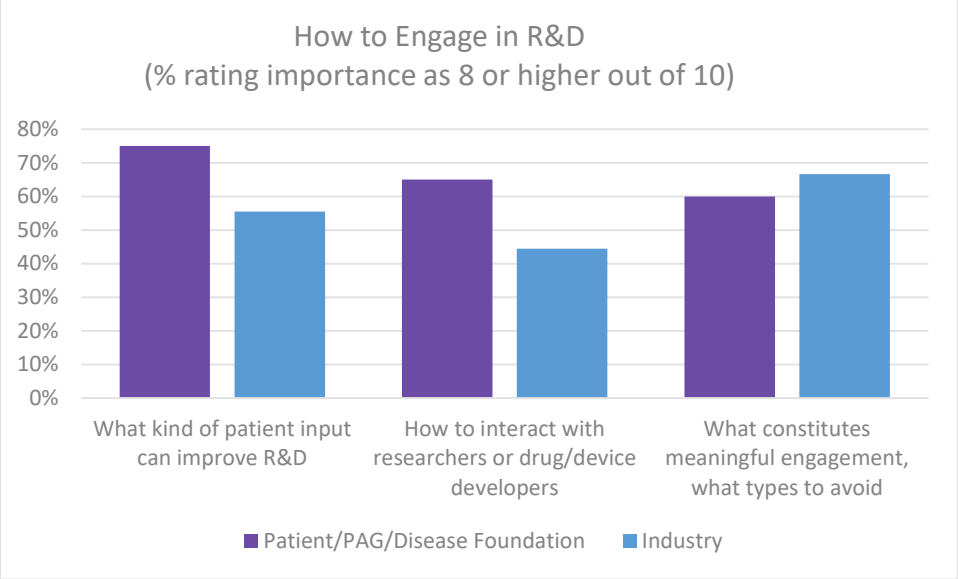
Overall, the industry group indicated that it is important for patients to understand how companies make decisions about what products to develop and how to develop them--a topic the patient/advocacy group cohort did not rate as highly, and that is not included in any of the capacity-building programs in our landscaping research.

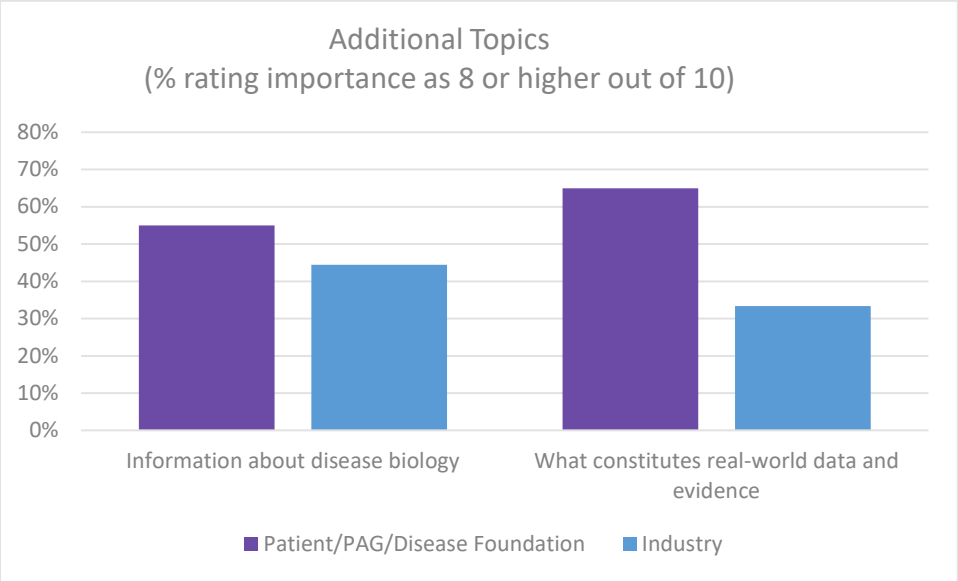
On the whole, both groups gave high ratings to topics covering how to engage, particularly on the topic of what kind of patient input can improve R&D.

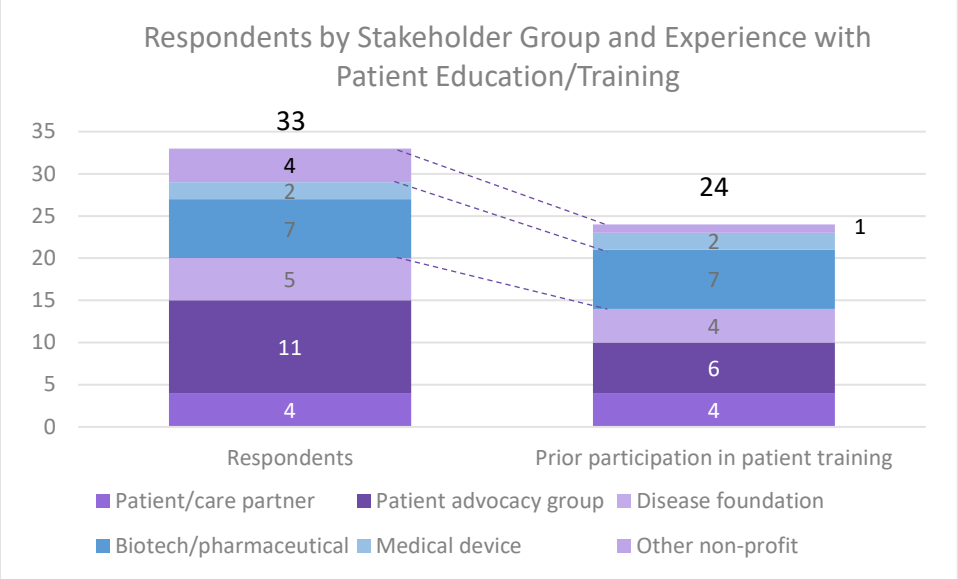





How Stakeholders Make Decisions
(% rating importance as 8 or higher out of 10)
80%
70%
60%
50%
40%
30%
20%
10%
0%
How insurers and payers make reimbursement decisions
How FDA makes product approval decisions
How companies decide what products to develop, and how
Patient/PAG/Disease Foundation
Industry


How to Engage in R&D
(% rating importance as 8 or higher out of 10)
80%
70%
60%
50%
40%
30%
20%
10%
0%
What kind of patient input can improve R&D
How to interact with researchers or drug/device developers
What constitutes meaningful engagement, what types to avoid
Patient/PAG/Disease Foundation
Industry


Additional Topics
(% rating importance as 8 or higher out of 10)
80%
70%
60%
50%
40%
30%
20%
10%
0%
Information about disease biology
What constitutes real-world data and evidence
Patient/PAG/Disease Foundation
Industry


Respondents by Stakeholder Group and Experience with Patient Education/Training
35
30
25
20
15
10
5
0
33
4
2
7
5
11
4
24
2
7
4
6
4
1
Respondents
Prior participation in patient training
Patient/care partner
Patient advocacy group
Disease foundation
Biotech/pharmaceutical
Medical device
Other non-profit

Appendix E: Workshop Discussion Tool

Building Capacity for Patient-Centered R&D – *A Workshop*
Discussion Tool

Instructions: This tool will be used during three breakout exercises and to help inform the moderated discussion at the workshop. If time permits, please review and consider prepopulating this tool in advance of the event. Real-world examples are encouraged, as is soliciting input from colleagues within your organization.

The tool is broken into three sections, one for each agenda session that includes a breakout-group activity. It is not necessary to fill out the entire tool; one example per section is sufficient.

If you choose to prepopulate this tool, please bring it with you the workshop. With your permission, staff will collect completed tables at the conclusion of the workshop.

A note on use of the tool at the workshop: At the workshop, participants will be seated in small group tables (approximately eight per table). Each table will choose one participant to serve as the designated reporter. Participants will have 20-25 minutes to brainstorm and complete the discussion tool as a group, followed by voluntary report-out from all the tables. These report-outs will serve as the basis for a 25-30 minute moderated discussion.

Please contact Susan Schaeffer (susan@patients-academy.org) if you have any questions.

Contact Information

Name: Click or tap here to enter text.

Organization: Click or tap here to enter text.

Note: Your written responses to this tool are for use by staff at FasterCures, a Center of the Milken Institute, and The Patients' Academy for Research Advocacy only; they will not be publicly released. Staff may contact you for follow-up or clarification on responses. Aggregated summary results may be included in a summary report to be distributed after the workshop.

AGENDA SESSION III: Are You Being Served? — Target audiences for patient/care partner education and training

BREAKOUT GROUP INSTRUCTIONS: Select **one** domain of patient/care partner input into R&D[1] (left column, one domain per page). Then, for each role a patient participant could serve[2] (top row), please answer the following:

- **WHO** needs and wants education/training and is underserved?
- **WHAT** are these patients' education/ training needs (if known)?
- **HOW** can we better understand those needs and help develop education/training to meet them?

		ROLES				
		Knowledge users and experiencers (Provide brief input, e.g., via surveys, online polling, listening sessions.)	**Reviewers, interviewees, consultants** (Participate in focus groups, semi-structured interviews, nominal groups techniques, etc.)	**Governance and advisory groups** (Serve on boards, councils, and committees.)	**Research partners or team members** (Integral members who participate in all key activities.)	**PI or co-PI** (Leads or co-leads the research.)
DOMAIN OF INPUT INTO R&D	**1. To help understand the patient experience, e.g.,** • Natural history • Burden of living w/ disease/condition • Burden of managing disease/condition • Views on available treatments and unmet medical need	***WHO*** *Patients/families with rare diseases for which there no established patient organizations and/or little organized research* ***WHAT*** *How patient input can influence and stimulate medical research* ***HOW*** *Work with umbrella organizations (e.g., NORD, Genetic Alliance, EveryLife) to connect with and engage these patients to identify or co-develop the appropriate resources to meet their needs*	*WHO* *WHAT* *HOW*	*WHO* *WHAT* *HOW*	*WHO* *WHAT* *HOW*	*WHO* *WHAT* *HOW*

[1] Adapted from National Academies of Sciences, Engineering and Medicine Advancing the Science of Patient Input collaborative
[2] Adapted from Wilkins, CH et al. 2015

AGENDA SESSION III: Are You Being Served? — Target audiences for patient/care partner education and training

		ROLES				
		Knowledge users and experiencers (Provide brief input, e.g., via surveys, online polling, listening sessions.)	**Reviewers, interviewees, consultants** (Participate in focus groups, semi-structured interviews, nominal groups techniques, etc.)	**Governance and advisory groups** (Serve on boards, councils, and committees.)	**Research partners or team members** (Integral members who participate in all key activities.)	**PI or co-PI** (Leads or co-leads the research.)
DOMAIN OF INPUT INTO R&D	**1. To help understand the patient experience, e.g.,** • Natural history • Burden of living w/ disease/condition • Burden of managing disease/condition • Views on available treatments and unmet medical need	***WHO***	***WHO***	***WHO***	***WHO***	***WHO***
		WHAT	***WHAT***	***WHAT***	***WHAT***	***WHAT***
		HOW	***HOW***	***HOW***	***HOW***	***HOW***

AGENDA SESSION III: Are You Being Served? — Target audiences for patient/care partner education and training

		ROLES				
		Knowledge users and experiencers (Provide brief input, e.g., via surveys, online polling, listening sessions.)	**Reviewers, interviewees, consultants** (Participate in focus groups, semi-structured interviews, nominal groups techniques, etc.)	**Governance and advisory groups** (Serve on boards, councils, and committees.)	**Research partners or team members** (Integral members who participate in all key activities.)	**PI or co-PI** (Leads or co-leads the research.)
DOMAIN OF INPUT INTO R&D	**2. To impart perspectives and preferences on benefit-risk, e.g.,** • Minimum expectations of benefits • Tolerance for harms or risks • Acceptable tradeoffs of benefits and risks • Attitudes towards uncertainty	***WHO***	***WHO***	***WHO***	***WHO***	***WHO***
		WHAT	***WHAT***	***WHAT***	***WHAT***	***WHAT***
		HOW	***HOW***	***HOW***	***HOW***	***HOW***

AGENDA SESSION III: Are You Being Served? — Target audiences for patient/care partner education and training

		ROLES				
		Knowledge users and experiencers (Provide brief input, e.g., via surveys, online polling, listening sessions.)	**Reviewers, interviewees, consultants** (Participate in focus groups, semi-structured interviews, nominal groups techniques, etc.)	**Governance and advisory groups** (Serve on boards, councils, and committees.)	**Research partners or team members** (Integral members who participate in all key activities.)	**PI or co-PI** (Leads or co-leads the research.)
DOMAINS OF INPUT INTO R&D	**3. To inform clinical trial development/continuous improvement, e.g.,** • Protocol development • Endpoint selection/prioritization • Recruitment, enrollment, and retention • Trial participant experience	***WHO***	***WHO***	***WHO***	***WHO***	***WHO***
		WHAT	***WHAT***	***WHAT***	***WHAT***	***WHAT***
		HOW	***HOW***	***HOW***	***HOW***	***HOW***

AGENDA SESSION IV: I Wish I Knew: Topics/information that is essential to patients to contribute to patient-centered R&D

BREAKOUT GROUP INSTRUCTIONS: Select **one** domain of patient/care partner input into R&D[1] (left column, one domain per page). Then, working across from left to right, please answer the following:

- What education or training topics are essential for patients/care partners?
- What existing resources could be scaled up to meet this need (if any)?
- What barriers have prevented developing or scaling education/training on these topics, and how might we overcome them?

Domains of patient input into R&D	What education or training topics are essential?	What existing education or training resources could be scaled up to meet this need (if any)?	What barriers have prevented developing or scaling education or training on these topics? How might they be overcome?
1. To help understand the patient experience, e.g., • Natural history • Burden of living w/ disease/condition • Burden of managing disease/condition • Views on available treatments and unmet medical need	*How to interact with researchers*	*Friends of Cancer Research, EURORDIS and European Lung Foundation programs*	*These resources are currently disease-focused. Identifying or creating a central repository could make them more widely available.*

[1] Adapted from National Academies of Sciences, Engineering and Medicine Advancing the Science of Patient Input collaborative

AGENDA SESSION IV: I Wish I Knew: Topics/information that is essential to patients to contribute to patient-centered R&D

Domains of patient input into R&D	What education or training topics are essential?	What existing education or training resources could be scaled up to meet this need (if any)?	What barriers have prevented developing or scaling education or training on these topics? How might they be overcome?
1. To help understand the patient experience, e.g., • Natural history • Burden of living w/ disease/condition • Burden of managing disease/condition • Views on available treatments and unmet medical need			

AGENDA SESSION IV: I Wish I Knew: Topics/information that is essential to patients to contribute to patient-centered R&D

Domains of patient input into R&D	What education or training topics are essential?	What existing education or training resources could be scaled up to meet this need (if any)?	What barriers have prevented developing or scaling education or training on these topics? How might they be overcome?
2. To impart perspectives and preferences on benefit-risk, e.g., • Minimum expectations of benefits • Tolerance for harms or risks • Acceptable tradeoffs of benefits and risks • Attitudes towards uncertainty			

AGENDA SESSION IV: I Wish I Knew: Topics/information that is essential to patients to contribute to patient-centered R&D

Domains of patient input into R&D	What education or training topics are essential?	What existing education or training resources could be scaled up to meet this need (if any)?	What barriers have prevented developing or scaling education or training on these topics? How might they be overcome?
3. To inform clinical trial development/continuous improvement, e.g., • Protocol development • Endpoint selection/prioritization • Recruitment, enrollment, and retention • Trial participant experience			

AGENDA SESSION IV: I Wish I Knew: Topics/information that is essential to patients to contribute to patient-centered R&D

BREAKOUT GROUP INSTRUCTIONS: Fill out the table as completely as you can. Real-world examples are encouraged!

1. What resources (money, staffing, other) are needed to develop and maintain capacity-building programs that help prepare and support patients to contribute their perspectives in R&D?	
2. Who are potential funders (besides/in addition to patient groups) and what are potential funding strategies to support development and maintenance of these programs?	
3. What would funders (other than patient organizations) expect in return for investment in patient capacity-building programs?	
4. How can efficiencies be gained by avoiding duplication of effort and investment, and what barriers will have to be overcome to achieve efficiencies?	