

Culture and Process Change as a Priority for Patient Engagement in Medicines Development

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Marc Boutin, JD¹, Lode Dewulf, MD, DipPharmMed, FFPM², Anton Hoos, MD³, Jan Geissler, Dipl-Kfm⁴, Veronica Todaro, MPH⁵, Roslyn F. Schneider, MD, MSc⁶, Vincenzo Garzya, MBA, PMP⁷, Andrew Garvey, BA (Hons)⁸, Paul Robinson, FRCP⁹, Tonya Saffer, MPH¹⁰, Sarah Krug, BA¹¹, and Ify Sargeant, PhD¹²

Abstract

Patient Focused Medicines Development (PFMD) is a not-for-profit independent multinational coalition of patients, patient stakeholders, and the pharmaceutical industry with interests across diverse disease areas and conditions. PFMD aims to facilitate an integrated approach to medicines development with all stakeholders involved early in the development process. A key strength of the coalition that differentiates it from other groups that involve patients or patient groups is that PFMD has patient organizations as founding members, ensuring that the patient perspective is the starting point when identifying priorities and developing solutions to meet patients' needs. In addition, PFMD has from inception been formed as an equal collaboration among patient groups, patients, and pharmaceutical industry and has adopted a unique trans-Atlantic setup and scope that reflects its global intent. This parity extends to its governance model, which ensures at least equal or greater share of voice for patient group members. PFMD is actively inviting additional members and aims to expand the collaboration to include stakeholders from other sectors. The establishment of PFMD is particularly timely as patient engagement (PE) has become a priority for many health stakeholders and has led to a surge of mostly disconnected activities to deliver this. Given the current plethora of PE initiatives, an essential first step has been to determine, based on a comprehensive mapping, those strategic areas of most need requiring a focused initial effort from the perspective of all stakeholders. PFMD has identified four priority areas that will need to be addressed to facilitate implementation of PE. These are (1) culture and process change, (2) development of a global meta-framework for PE, (3) information exchange, and (4) training. This article discusses these priority themes and ongoing or planned PFMD activities within each.

Keywords

patient engagement priorities medicines development

Introduction

The purpose of health systems is to improve the health of patients and maintain that of healthy individuals. Involving the end-user—the patient—in identifying health priorities and outcomes desired from health interventions is increasingly seen as critically important. The concept of patients as partners in their health and health decisions is not new but has been largely focused on decisions at the point of care. What is emerging is a growing agreement that patient engagement (PE) needs to happen earlier and to encompass all stakeholders. A patient-centric culture incorporating early PE fosters innovation and collaborative attitudes that ultimately lead to the identification of the best solutions for patients. Furthermore, patients may have more confidence in research and research outputs if other patients have provided input.¹

PE is also seen as a priority by regulatory bodies. The European Medicines Agency (EMA) has a long history of

¹ National Health Council, Washington, DC, USA

² UCB Biopharma, Brussels, Belgium

³ Amgen (Europe) GmbH, Zug, Switzerland

⁴ European Patients' Academy on Therapeutic Innovation, Brussels, Belgium

⁵ Parkinson's Disease Foundation/Clinical Trials Transformation Initiative, New York, NY, USA

⁶ Pfizer Inc, New York, NY, USA

⁷ AstraZeneca, Cambridge, United Kingdom

⁸ GlaxoSmithKline, Brentford, United Kingdom

⁹ Merck Sharp & Dohme Ltd, Hoddesdon, United Kingdom

¹⁰ National Kidney Foundation, Washington, DC, USA

¹¹ CANCER 101 Foundation, Society for Participatory Medicine, Health Collaboratory New York, NY, USA

¹² Ismedica Ltd, Staffordshire, United Kingdom

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Corresponding Author:

Marc Boutin, JD, National Health Council, 1730 M Street NW, Suite 500 Washington, DC 20036-4561, USA.

Email: mboutin@nhcouncil.org

working with patients, establishing the Patient and Consumer Working Party in 2006 and appointing 3 patient representatives to their Management Board. A framework for interaction between the EMA and patients and consumers and their organizations was developed in 2005 and revised in 2014.² In December 2015, the European Medicines Agency (EMA) reconfirmed their commitment to improving PE to ensure patient “views and needs are taken into account at every step” of medicines development.³ One objective within the EMA and Heads of Medicines Agencies (HMA) strategy for the period up to 2020 is to provide support for patient-focused innovation.⁴ The EMA’s Committee for Medicinal Products for Human Use (CHMP) has specifically cited the “involvement of patients in the assessment of the benefits and risks of medicines” as one of its 3 focus areas, and has provided guidance for EMA Scientific Committees on incorporating patients’ views during these assessments.^{5,6}

The US Food and Drug Administration (FDA) also has a history of patient engagement starting from 1988 with the formation of the office to work with patient advocates. In 2012, the FDA Patient Network was created and currently has over 200 FDA Patient Representatives participating in the program.⁷ Also that year, the FDA launched its patient-focused drug development (PFDD) initiative as a commitment to more systematically gather patients’ perspectives on their condition and available treatments. Around 24 public meetings on specific disease areas have been scheduled or planned as part of this initiative. As of March 2016, 17 have been conducted⁸ and meeting outcomes disseminated through a series of reports (The Voice of the Patient). To expand this effort, the FDA has invited patient organizations to utilize the FDA-outlined process to obtain public input in other disease areas.⁹ The FDA has also recently established its first Patient Engagement Advisory Committee (PEAC), which will provide advice to the FDA Commissioner on issues relating to medical devices, their regulation, and their use.¹⁰ In addition, the FDA’s Center for Devices and Radiological Health (CDRH) strategic priorities for 2016-2017 highlights PE and the need to “interact with patients as partners” as essential to its success.¹¹ Importantly, the new FDA Commissioner has declared patient engagement a priority.¹²

Patient input has also been sought in reimbursement decisions. For example, the Canadian Agency for Drugs and Technologies in Health (CADTH) launched its first call for patient input in May 2010. Since then the Agency has developed a formal approach for incorporating patients’ perspectives into its Common Drug Review process and has committed to enhanced patient input in its 2015-2018 Strategic plan.^{13,14} There is also a mechanism for patient involvement in their Scientific Advice Program that provides pharmaceutical companies with guidance on early drug development plans from an HTA perspective.¹⁵ In the UK, the National Institute for Health and Care Excellence (NICE) has a patient and public involvement policy.¹⁶ These commitments to improve and expand PE in medicines development and assessment underscore the

growing recognition of its importance. However, a survey by the European Patients Forum (EPF) of EU Health Technology Assessment (HTA) agencies indicates that only about half of these agencies involve patients in HTA.¹⁷ This highlights the variability in approach and outcomes and indicates a need for a more consistent process.

While the growth of PE initiatives is encouraging, there is still a need to effectively incorporate information provided by patients into the decision-making process and for this to happen not just in individual stakeholder organizations but through a truly multistakeholder effort. The increasing number of PE initiatives and activities need to be synergized and refined to inspire a comprehensive, consistent approach to PE that is relevant to the vast majority of stakeholders against the backdrop of medicine development, which is essentially global. Without an integrated approach to PE, the diverse needs and priorities of stakeholders will be misaligned. This will result in inefficient resource use, increase in net requirements to develop medicines and health interventions, and delays or even failure to provide solutions that are meaningful to patients. Patient Focused Medicines Development (PFMD) was formed in October 2015 in response to a growing number of public calls for an open collaboration of stakeholders with the common objective of developing a more effective approach to PE across the entire medicines development and lifecycle pathway.¹⁸⁻²¹ PFMD is an independent trans-Atlantic coalition of patients, patient groups, and the pharmaceutical industry across diverse diseases and conditions. It has adopted a governance model that ensures at least equal patient leadership that is based on commitment and not just expertise. The intention is to gradually widen participation to include regulators, payers, and other professional organizations to the global group to ensure diversity of perspectives. PFMD has identified four priority areas to be addressed in order to facilitate implementation of PE. These are (1) culture and process change, (2) development of a global meta-framework for PE, (3) information exchange, and (4) training. This article discusses these critical themes and ongoing or planned PFMD activities within each (see Box 1).

Priority I: Culture and Process Change

The establishment of cultures and processes that ensure routine implementation of PE is critical for integrating PE in medicines development. This will require significant changes in the way stakeholder organizations traditionally work—changes that can either be encouraged or mandated. Sharing good practices with clear communication of the tangible benefits of effective PE and the use of incentives can be powerful motivators for change. Conversely communicating the “danger” of ineffective or no PE (such as development of medicines with less value or relevance for patients and resource waste) may also motivate change. Much evidence for the potential impact of PE comes from the HIV and rare diseases field where patient advocates and organizations

Priority 1: Culture and process change to overcome barriers and facilitate routine implementation of PE in medicines development

Priority 2: Development of a global PE meta-framework to facilitate effective and consistent PE in medicines development

Priority 3: Development of an information exchange platform to allow stakeholders to benefit from sharing their experience and good practices; prevent duplication of effort and resource waste; and provide an objective platform for actively sharing opportunities for PE

Priority 4: Development of learning experience and training opportunities to equip teams and individuals leading PE in each organization with good practices and understanding of current PE approaches and activities. This will facilitate delivery of effective PE within their organization

Box 1. Summary of priority areas in PE identified by PFMD.

have dramatically affected how patients' needs and priorities are incorporated into medicines development and availability.²² For example, advocacy accelerated or prompted increased patient and community involvement in HIV medicines development and evaluation, expanded access to potentially effective new drugs in advance of formal approval, and efforts to shorten the medicines development and evaluation process through accelerated approval.²² Several examples from EURORDIS (an alliance representing over 700 rare disease organizations across Europe) demonstrate the benefits of PE to drive a more patient-centric approach to medicines development and evaluation. Of note, their work has contributed to the adoption of several EU regulations on drugs for rare diseases and/or specific subpopulations with high unmet medical need.²³ Other specific examples of good practices that can be leveraged to demonstrate the value of patient input and patients' experiential knowledge are summarized in Table 1.

A mandatory approach to PE can also be leveraged alongside communicating the benefits and the use of incentives. For example, funding bodies increasingly either require or encourage submission of plans for patient and public involvement from grant applicants in order to obtain funding.^{24,25} The Parkinson's Disease Foundation (PDF) Parkinson's Advocates in Research (PAIR) Leadership Awards support scientists in their work to formally engage PDF Research Advocates and other patient advocates in their research. Successful applicants will have a plan to formally engage one or more PDF Research Advocates or patient advocates in their project, in a way that goes beyond recruitment or trial participation. The Award also requires that patients be included as collaborators in the study and study outputs such as manuscripts and posters.²⁶ The Patient-Centered Outcomes Research Institute (PCORI) works with patients and other

stakeholders to help them determine which research topics to consider for funding, and to review proposals. They also require that patients be included as meaningful partners in the research PCORI supports, helping to develop and conduct the studies and disseminate the results. Potential funding through PCORI is contingent on following their methodology standards that include standards of patient-centeredness.²⁷

A change in culture also requires a change in attitudes or beliefs that either hinder or facilitate PE.²⁸ Effective collaboration will need to identify and overcome the initially "uncomfortable marriage" of stakeholder groups with traditionally dissimilar viewpoints, objectives, or ways of working. For example, it may be necessary to counter the perception that PE is industry's attempt at "courting" or "coercing" patients rather than being an essential first step in co-creating solutions to shared challenges for mutual benefit. This is perceived as a major barrier, especially with regard to funding of patient groups. As most existing codes of practice do not address the interaction of patient organizations with stakeholders in the area of medicines R&D, EUPATI is currently developing guidance for interactions with industry, ethics committees, and HTA bodies that will be released by the end of 2016. In addition, following general "rules of engagement" as well as the European Federation of Pharmaceutical Industries and Associations (EFPIA) and International Federation of Pharmaceutical Manufacturers & Associations (IFPMA) codes of practice that cover key topics such as transparency on any funding or compensation (how much and for what purposes), training and communication is particularly important.²⁹⁻³¹

Internal bureaucratic processes, fear of being accused of promoting medical products to the public, a lack of understanding of the benefits and challenges of partnering with other stakeholders, an unwillingness to share information, and a lack of transparency or openness have also been identified as barriers to PE.³² Key enablers for effective PE among different stakeholders include having clearly defined "rules of engagement"; transparency around funding or compensation (how much and for what purposes); agreement and common understanding of goals with alignment of vision; establishment of appropriate governance/partnership structures and processes; resource alignment; agreement of roles, responsibilities, and scope of PE; mutual respect; and open and frequent communication.³³⁻³⁵ Robust evidence for the value of PE using specific examples that resonate with different stakeholders can help to address obstructive perceptions and encourage constructive attitudes.

In many ways, process change represents the practical aspect of PE implementation. The aim is to remove real or perceived process barriers such as operational restrictions that prevent diverse stakeholders from collaborating. For patients or patient groups wanting to work with pharmaceutical companies, there may be a need for a different type of written agreement or contract that does not fit into the traditional types of "service agreement," "consultant," "partnership," or "collaboration" contracts—a need that may delay or hinder efforts to

Table 1. Encouraging Change: Case Study Examples of PE and Benefits.

Example	Benefit
Examples From Patient Organizations and Patient Advocacy Groups	
<p>The CANCER101 Foundation has developed a series of Participatory Co-Design Workshops to develop solutions based on issues identified by patients and caregivers across the care journey and as a result of this workshop series, among other solutions recently built the Prescription to Learn[®] platform to help patients navigate the care journey in partnership with their healthcare teams. In addition, the Patient Shark Tank[®] was created as a vehicle to amplify the voice of the patient in the design, development, and continuous improvement of innovations designed to serve them. In its first Patient Expert Training Course, the European Patients' Academy (EUPATI) has trained 46 patient advocates from 20 countries in depth on all aspects of medicines research and development (R&D) across the development lifecycle. All educational course material has also been released in January 2016 as a web-based EUPATI Toolbox on Medicines R&D in 7 languages.</p>	<p>The platform alleviates information overload and helps patients regain a sense of control through connections to credible sources of information across the care journey, including clinical trial information. The platform also allows the clinician to prescribe information to patients and caregivers, allowing us to better understand the navigation behaviors of patients based on their phase in the care journey. Over 450 innovations have been assessed by patients and caregivers across disease states through the Patient Shark Tank.</p> <p>During the course, a number of EUPATI course graduates have joined committees and workgroups of the EMA and National Competent Authorities, ethics committees as well as academic and pharmaceutical research projects, providing the patient perspective into trial designs, ethics reviews and regulatory affairs, with a significant input into protocols, assessments, and, for example, patient summaries of studies. More than 25,000 individuals accessed the EUPATI Toolbox on Medicines R&D within the first four months after launch, demonstrating high demand in the patient community for quality-controlled, objective educational information about patient involvement in medicines R&D.</p>
<p>The National Kidney Foundation (NKF) includes patients on its Public Policy Advisory Committee.</p>	<p>Patients provide practical experience on how policy considerations directly affect patients and patient care. In this capacity patients have a role in advising NKF on policy positions. NKF's leading policy priority, to align healthcare payment with earlier detection and treatment of chronic kidney disease (CKD), was developed as a result of patients' with end-stage renal disease reporting that their doctors did not tell them they had CKD prior to their kidney failure and that they would have taken a more active role in their health had they been informed.</p>
<p>The National Psoriasis Foundation (NPF) hosts an on-line patient research community. The Citizen Scientist registry is designed to enable patients to provide information about their disease and in real time analyze data collected from the thousands of participants on the site, share hypotheses, and participate in discussions.</p>	<p>This platform enables patients to learn about their disease while contributing to, and engaging in, the scientific discovery process. The data are used by researchers exploring new ideas. The opportunity for patients to explore the data, pose research questions, and discuss outcomes also gives patients dealing with psoriasis a new feeling of control. NPF also uses the data to build new programs and to identify priorities for both extramural and intramural research.</p>
<p>In 2015, the NPF held its second Research Symposium in conjunction with its National Volunteer Conference. This event brought together more than 100 researchers and over 200 patients and family members.</p>	<p>By bringing together the scientific research and patient communities for a shared event, both groups were able to gain valuable insight into the knowledge and priorities of the other. Feedback suggests that these interactions have substantial impact, for example, in motivating previously undecided scientists to dedicate their studies to psoriatic disease research.</p>
<p>The Parkinson's Disease Foundation (PDF) through its Community Choice Research Award (CCRA) asks people living with the disease and care partners to share their priorities for research.</p>	<p>PDF provides funding and organizes a multidisciplinary dialogue to kick-start thinking on addressing disabling, yet under-recognized, symptoms of Parkinson's. Past CCRA's have addressed the issues of fatigue and gastrointestinal dysfunction. The goals are to accelerate knowledge in under-researched, high-need areas and to change the research culture so that PE becomes integrated into the fabric of medicines development.</p>
<p>The Sjögren's Syndrome Foundation and Bristol Myers Squibb (BMS) established a Patient Engagement Network for Sjögren's disease. The aim is to allow patients to share their disease journey with BMS staff and to provide insight into clinical trial design, execution and obstacles they see in participating in research trials.</p>	<p>The Network has enabled BMS to learn directly from patients about how to make their clinical trials more patient friendly. This ultimately will make them more successful for recruitment and facilitate development of much needed therapies for Sjögren's disease.</p>

(continued)

Table 1. (continued)

Example	Benefit
Examples From the Pharmaceutical Industry	
Pfizer included patient experts in several disease area-specific conferences, Advisory Board meetings along with health care practitioners and scientific experts, and has added a patient expert to their Bioethics Advisory Panel.	Patients have provided valuable insights into their practical needs such as that it's hard to remember when to take their medications, keep their appointments, and that parking and other transportation for study visits may be difficult and expensive. These are prompting technological reminders and other support programs to potentially improve participation and satisfaction with the clinical trial experience.
Amgen has used Advisory Boards including representatives from six patient groups throughout Europe (Ireland, Italy, Netherlands, Sweden, UK, and Spain) with an interest in migraine to identify ways of improving the patient experience in clinical trials.	Patients have provided practical recommendations, including making trial appointments more flexible, providing patients with an easy way to record symptoms, giving clear continuous communication around the trial, and giving them something back at the end of the trial (eg, an interpretation of results from their e-diary or insight into the possible triggers of their migraines).
AstraZeneca formed a Patient Centricity (PaCe) team in 2015, tasked with enabling the organization to better connect patients with the science in order to deliver patient-centric medicines.	Through PaCe, AstraZeneca established an alliance with <i>PatientsLikeMe</i> which has enabled their teams in R&D and beyond to connect with more than 30,000 patients to inform study design, study endpoints and contribute to transformational programs within core therapy areas.
GSK's Health Advisory Board (HAB) is composed entirely of representatives from European patient groups and meets with the most senior members of the company's European management team. GSK's <i>Focus on the Patient</i> Program invites patients to GSK sites so that employees can hear about patients' experiences and apply key learnings in their everyday work. For example, in one seminar for researchers, patients with conditions of scleroderma were asked about modes of treatment that worked best.	The HAB has provided advice and suggestions on how the voice of the patient can be brought into the company's clinical development program more systematically and comprehensively.
UCB's Hack Epilepsy "hackathon" was held simultaneously in Atlanta and Brussels in April 2015. It brought together developers, designers, and digital experts, health care providers, and patients to explore and co-create innovative ways of applying digital technologies to challenges faced by the epilepsy community.	Patient insight revealed difficulties with tablets which were often too small to hold with sclerodactyl hands, and IV needles which were painful when puncturing hard scleroderma skin. The examples of some of the practical challenges of coping with systemic sclerosis on a daily basis indicated how further treatments might be formulated to improve patients' quality of life. Key challenges were defined by patients living with the disease and included accessing effective support and information, being empowered to talk about their condition, and knowing what questions to ask after diagnosis. Over 20 specific ideas were identified and these are currently being further explored and/or developed.

work together. There is also a need to create a clear and safe legal environment for PE to address concerns of the pharmaceutical industry and others wanting to engage with different stakeholder groups. Lack of resources on the part of patient groups, when it comes to managing the legal review and modification of contracts, and staff turnover can also impact PE. There is guidance to help overcome some of these impediments. For example, the Clinical Trials Transformation Initiative (CTTI) recommendations for effective engagement with patient groups around clinical trials include guidance on contractual agreements for nonprofit organizations working with industry.³⁶ This guidance aims to mitigate against risks such as confidentiality and perceived or actual improper influence. The CTTI recommendations also address the erroneous perception that there are regulatory and legal restrictions to explicitly inhibit research sponsors from engaging with patient groups or organizations early in clinical trial development. In reality, the FDA "encourages engagement as a means of facilitating clinical trial design, awareness, and enrolment."³⁶ Another source of advice is the PCORI Engagement Rubric that provides general guidance and suggestions for PE covering research planning, conduct, and dissemination.³⁷

A good example of culture change driving process change comes from the FDA's PFDD initiative. The US National Health Council (NHC) advocated for the creation of the PFDD Program, which included, among other initiatives, 20 public meetings on specific disease areas to understand patient perspectives about their conditions and available treatments. During these meetings, the FDA learned that what it thought was the patient's chief complaint was, in fact, not in alignment with what patients were telling them. This created a cultural shift, causing the FDA to expand its efforts and establish processes to systematically gather patient input to inform its decision makers. Another example of process changes to encourage PE comes from the *British Medical Journal* (BMJ) and its "Partnering With Patients" campaign. Authors submitting research articles for publication are now requested to state "if and how they involved patients in setting the research question, the outcome measures, the design and implementation of the study, and the dissemination of its results."³⁸ In addition, the *BMJ* is in future "likely to consider clinical research papers only if the authors can demonstrate partnership with patients in their study."³⁹ These examples demonstrate that PE is already happening – albeit suboptimally. Coalitions such as PFMD provide

the impetus and platform to more rapidly progress PE by harnessing the resources, support and commitment of organizations on the same journey.

Priority 2: Development of a Global Patient Engagement Meta-framework

There is wide agreement on the need for PE in medicines research and development, and across the entire medicines lifecycle. However, a clear and consistent understanding of what is needed and when, what effective, integrated PE looks like and what is most valuable for stakeholders is currently lacking. There are already very valuable frameworks addressing portions of the medicines development and lifecycle pathway as well as guidance for PE in medicines R&D and assessment. For example, CTTI recommendations on effective engagement with patient groups around clinical trials provides a set of good practices based on the perspectives of various stakeholders. It describes various points for patient group/organization engagement along the research continuum and the type of engagement and also provides evaluation tools to help patient groups assess the benefits and opportunities for PE most relevant to their organization.³⁶ The European League Against Rheumatism (EULAR) has also developed a set of recommendations for the inclusion of patient representatives in health research and scientific projects.⁴⁰ Another example comes from the National Health Council and Genetic Alliance recommendations. Their report, “Advancing Meaningful Patient Engagement in Research, Development, and Review of Drugs,” aimed to establish a common vision of integrating the voice of the patient in the product research, development, and approval process and produce a set of actionable solutions. They note that “a next step may be for stakeholders to prioritize and refine the solutions to make them practical.”¹⁹ PFMD is developing a global meta-framework that incorporates relevant and effective features of existing initiatives and frameworks, identifies synergies and gaps, and provides practical recommendations to address those gaps. The intent is for this to provide a structure above and around other frameworks. Importantly the aim is not to define a single one-size-fits-all framework, but rather the key elements of what should be covered in a framework, thereby inviting and allowing other stakeholders to develop their own frameworks (aligned with the meta-framework) tailored to meet their specific needs. The meta-framework will address key issues in effective PE such as the types of engagement and involvement, critical stakeholders required to be engaged, legal or regulatory considerations at each time point, and the skill sets required to enable meaningful patient input. In some cases, no special skills will be required, whereas in others specific competencies will be important. Therefore, assessment of knowledge and training needs at points in the meta-framework will be required—not just for patients but also for other stakeholders. PFMD aims to address this need through learning exchange and the development of training master classes (Priority 4). The considerable task of information

gathering and actively approaching those involved in PE for their input to build a global meta-framework is well underway. The aim is to have a working “strawman meta-framework” available within the first half of 2017. PFMD members will pilot the meta-framework and will invite other stakeholders to also implement and test the meta-framework so that it can be adapted and refined to best meet the needs of diverse users.

Priority 3: Development of an Information Exchange Platform

Despite the substantial increase in PE initiatives, there is currently no efficient mechanism for accessing information on what PE activities are ongoing or planned and to identify challenges encountered and lessons learned. An essential task is to organize and categorize existing PE activities into a map of the PE landscape that gives an overview of the range and type of PE activities across stakeholder groups. PFMD is developing online tools and channels to facilitate information and knowledge exchange among PE stakeholders. The goal is to showcase the various initiatives in a meaningful way through visual tools and maps with the objective of offering value to a diverse audience. These visual maps will look at the PE activities from the perspective of the different stakeholders so that each can identify those areas most relevant to their own needs. Tools will include elements such as a Patient Partnership Matrix, with the various levels of patient involvement highlighted, a spotlight on the variation of patient expertise in these efforts, and area(s) of interest. By showcasing initiatives that involve patients through a variety of perspectives, PFMD’s aim is to not only promote information exchange but also allow for organizations to forge potential partnership opportunities. The maps will be dynamic with stakeholders invited to contribute information on their PE activities to ensure information is current and relevant. At the time of writing, an online collection tool to request and capture details of PE initiatives across the PE landscape is being tested. As described for the meta-framework, the aim is not to “reinvent the wheel” but rather to bring together in one place, augment, and document the range and types of PE. For example, EUPATI research and activities with trainees indicated that patients need to understand better where and how to be involved. In response, EUPATI developed a *PatientsInvolved* webpage that lists opportunities where patients can be involved in specific R&D projects and is assessing the value of making this a permanent online feature. Ensuring that patients and patient organizations have a “safe” environment for interacting with industry is a key consideration. At a national level, the success of PCORI’s PCORNet—a collection of individual Clinical Data Research Networks and Patient-Powered Research Networks—demonstrates the value of developing a standardized resource of clinical information to facilitate patient-centered research. PFMD’s aim is to develop a global, comprehensive, and searchable information portal. This will prevent duplication of effort and resource waste, allow stakeholders to benefit from

sharing their experience and good practice, and provide a non-partisan platform for actively promoting opportunities for PE.

Priority 4: Learning Exchange and Master Classes on PE

Different types of PE require different competencies and skills, and research with the general public indicates varying levels of knowledge about R&D.⁴¹ Initiatives such as EUPATI provide valuable training courses and education materials for patients and patient organizations. These equip patients with the skills and knowledge for valuable contribution to discussions around medicines research and development where specific proficiency is needed. However, training needs are not restricted solely to patients. EUPATI research has identified that skills training is required for industry researchers to best engage with patients and highlights that all parties should be equipped to benefit from an exchange.²⁸ Most of the experience and (experience-derived) knowledge on the topic of PE is not readily available via the classical channels of publications as journal articles or books as most is not published. Instead, these are shared generally in diverse small meetings and discussions, which limits their impact and wider dissemination. This supports PFMD Priority 4 which is to provide a platform for learning exchange and relevant training for all PE stakeholders and not just patients. A PFMD Learning Exchange is currently being organized to virtually showcase the global works and share learnings of organizations that are conducting work on involving patients across the medicine lifecycle. The main objective is to demonstrate how those implementing PE are collectively moving the needle in these efforts. Elements of the initiatives presented during the learning exchange that demonstrate good practice can be incorporated into the global meta-framework. PFMD is also developing an educational curriculum to be delivered through a series of tailored master classes, focusing on meeting training needs identified in consultation with stakeholders. One key aim of the master class program is to have the most experienced PE stakeholders together share their (largely unpublished) knowledge in an integrated session with a group of trainees. The curriculum will feature opportunities for sharing experience and good practice, skill-based workshops and insights from the learning exchange. It is being co-created with patients and will move beyond simple knowledge transfer to application and evaluation. The master classes will reflect training needs and focus areas most relevant for specific stakeholders and incorporate key time points, critical stakeholders, and timing and type of PE identified through development of the framework. The master classes target and convene cross stakeholder groups, enriching and progressing PE at the level where PFMD can have the most impact. This cross-fertilization across geographical boundaries exploits PFMD's global reach, which allows wide translation of learnings.

Discussion

There is widespread agreement of the importance of PE in medicines development to ensure that the needs and objectives of patients are met by health interventions. However, current approaches are generally sporadic and inconsistent, which impacts their effectiveness. Furthermore, there is no common agreement on what and how to measure in terms of successful outcomes of PE. Patient-centricity and PE have become the buzzwords of the decade—this will only remain true for a limited period of time. Given the substantial efforts and lengthy discussions on PE, there is a need to put words into action and to deliver on commitment to PE. This makes a concerted and global effort to develop and implement effective PE particularly timely and urgent. Moreover, the environment is primed for real progress in PE (Table 2).

PFMD has been established to make the most of the current fertile environment for PE, with different stakeholder groups working together to make PE happen. PFMD is taking a methodological and rational approach to PE by documenting and mapping existing initiatives, identifying good practice to develop a meta-framework, using information exchange to refine and grow the meta-framework, and then aiding implementation by ensuring training needs are met. Its members have committed to implementing PE in their own organizations and to advocate for its wider and consistent implementation by all stakeholders in medicines development. Good progress is being made across priority areas identified by PFMD. Although culture and process change cannot happen overnight, active involvement in organizations like PFMD can help by facilitating information exchange, mutual learning and transparency between leaders of PE initiatives across a range of stakeholder groups and geographical locations. Regular face-to-face meetings provide a forum for candid discussion and foster the development of close working relationships based on reciprocal trust and a clear understanding of members' priorities. PFMD members routinely share and circulate information about PE initiatives they are aware of or involved in and are already benefiting from these exchanges—generating interest and support and gaining valuable feedback. PFMD is a relatively small organization, making it agile and able to react quickly to new information that affects the evolving PE landscape. The intention is to broaden participation to include a greater diversity of stakeholders in medicines development while still retaining agility.

For the meta-framework and information exchange platform, a significant challenge has been identifying existing PE initiatives along with documentation of associated processes and outcomes. This is being addressed through use of a collection tool so that key details of PE activities can be captured in a standardized and structured way, and through organized opportunities for experience and information exchange. Together, this will allow development of a dynamic map of the PE landscape. The target is launch of an information exchange platform and delivery of a learning exchange meeting within Q4 of 2016 outputs from which will drive completion of a strawman meta-framework for PE in medicines development within

Table 2. A Fertile Environment for Progress in PE.

The patient voice has become increasingly relevant	In the regulatory setting, patient-centered benefit-risk (PCBR) assessment is becoming a preferred method of integrating patient preference information into regulatory decision making. Initiatives such as the Parent Project in Muscular Dystrophy (PPMD) have leveraged PCBRs to provide patient input to the FDA. As a result, the FDA issued draft guidance for industry on the clinical development of drugs for the treatment Duchenne muscular dystrophy and related diseases. ^{42,43} CADTH also has a comprehensive approach for obtaining patient input in HTAs including guidance, templates, and examples of good practice from patient groups. ¹³
Training and education	Patients and patient groups have improved opportunities for training in order to contribute fully to medical development discussions where specific skills or knowledge may be needed. There are well-established and successful training programs such as the EURORDIS summer school launched in 2008, and the European AIDS Treatment Group (EATG) Training Academy's one-year STEP-UP program. In addition, initiatives such as EUPATI are beginning to bear fruit as knowledgeable patients have graduated from the Patient Expert Training Course and are applying the EUPATI Toolbox on Medicines R&D. However, continued education and training efforts are required, differentiating between the types of PE in a range of settings and matching the level of training to each. ^{28,41}
Access to information	Digital and social networking capabilities are facilitating access to information, rapid communication to relevant audiences, and unprecedented opportunities for wide-scale collaboration. ^{39,40} Although pharmaceutical companies have generally been slow to engage in a two-way dialogue using social media, limiting most activities to unidirectional announcements, these media could be more optimally leveraged to facilitate PE in medicines development activities.
Partnership models	Innovative partnership models (such as investments, venture philanthropy, and co-funding) allow stakeholders from diverse backgrounds with different ways of working to form effective alliances toward a common aim. ³⁶
Pressure on health care systems	Patients and their organizations engaging in medicines development and regulatory/HTA processes provide a service of public health interest. Its value needs to be recognized by public health authorities and ministries of health. The sustainability of these organizations must be ensured by public funding. There are substantial and increasing (financial) pressures on health care systems, with patients asked to contribute to cost-cutting activities (eg, through shorter hospital stays, more self-care/care at home, less services paid, less innovative medicines reimbursed, and higher copays). There is therefore a need for a new societal contract where patients contribute more to setting health priorities and needs. ⁴⁴⁻⁴⁶ Although PFMD's focus is clear, PE should not be restricted solely to medicines, vaccines, or medical devices development but should be integrated into health care provision and health systems generally. ⁴⁷

6 months of the exchange meeting. In addition, creation of training material is planned by Q4 2016 with delivery of the first master class within 6 months. These are ambitious but achievable targets if all stakeholders contribute and commit to ensuring that patients and their needs are at the center of medicines development.

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Author Note

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