Why patient engagement takes a village: a report from Patient Engagement Open Forum

The importance of collaboration, alignment and why it takes a village to achieve meaningful patient engagement (PE) was the opening call to action at the recent *Patient Engagement Open Forum* – *Delivering patient engagement beyond aspirations.* Over 250 delegates participated in the two-day immersive event which took place in Brussels, Belgium on 18–19 September, 2019.

Organized by PARADIGM, PFMD and EUPATI, the Forum provided an overview and update on patient engagement (PE) players and efforts across the globe but also incorporated a series of hands-on workshops with tangible outputs. The delegates at the PE Forum were not just passive recipients of information but active partners in co-creating the next tools for implementation of PE.

During the inaugural panel session with National and EU Heath Technology Agencies (HTAs) and regulators, delegates asked whether it was time for PE to be a mandatory component of regulatory dossiers. Delegates also believed that patients with experiential knowledge should be considered essential as experts, in the same way as other experts such as clinicians were considered essential. Until this happened, PE would be seen as an extra effort. As one delegate noted, even if not ready to mandate PE, HTA and regulators should make a clear position statement that they support and expect PE.

Other points voiced during the panel discussion included the need to identify and prioritize areas where PE is valuable to respect both patient time and finite resources, and to better communicate where the patient voice has impacted outcomes. There was also discussion around the concept and language of 'value' with the comment that other experts were not expected to prove that their input into regulatory decision-making had value and the same should apply to PE and patient experts. One delegate suggested that using the term 'value' implied that return on investment was the key driver for PE in regulatory discussions and that more suitable language should be considered.

Four parallel interactive workshops on Day 1 focused on different aspects of PE. The session on practical implementation of good PE practice in everyday work, began with sharing of good practice examples from industry, academia and a patient organization/pharma collaboration. Discussion of the examples facilitated exploration of process and impact from three different perspectives. Key findings were the importance of sharing process and learning, of having a framework for PE and having a shared purpose. Strong internal leadership and support for PE and understanding that capacity and capability were relevant for all stakeholders, not just patients was also highlighted. One challenge identified was in knowing contractual implications of what could be shared, how and with whom. These and other insights will be carried forward into PFMD work streams and pilots of PFMD's PE Quality Guidance.

WECAN (Workgroup of European Cancer Patient Advocacy Networks), in collaboration with PFMD, led an initiative to develop guiding principles for reasonable legal agreements between patient advocates and pharmaceutical companies. The NHC are also working on a fair market value (FMV) calculator which could potentially be valuable for use in Europe. This was the background for the discussion in the parallel session on legal contracts between patients, pharma and researchers, participants. In this workshop, participants had to wear two hats to evaluate a PE activity contract

for a patient advocate from both a patient perspective and a pharma perspective. Key points from the lively discussion – which included the importance of trade associations and the need to improve alignment in interpretation of associated rules – will feed into the development of reference contracts.

The final parallel session in Brussels was a two-part effort to prioritize gaps in PE and identify where PARADIGM could achieve most benefit (part 1; day 1) and then to co-create future PE tools to address identified gaps (part 2; day 2). Key points were to conduct a comprehensive landscape check to ensure that perceived gaps were real before embarking on potentially replicative initiatives, to communicate process and outcomes and improve reporting and documentation of PE practice.

In a genuine spirit of inclusivity, the PE Open Forum also incorporated an on-line parallel session to brainstorm how to reach beyond the 'easy targets' in PE. There was a clear call for commitment to, and support for, PE from the organization's leadership – echoing responses from other sessions. Participants also highlighted a need for culture change to transform PE activity from a 'key performance indicator' to something intrinsic to and embedded within the organization and that resonated on an individual level to 'change hearts and minds'. Participants urged those undertaking or considering PE to 'think big and act small'. The feedback will help to develop and refine patient and industry tools for PE activities and will ensure that efforts are aligned with identified needs for integrating PE in more challenging areas.

Morning workshops on Day 2 of the Forum built on the delegates as 'partners in co-creation' approach. There were parallel sessions on compensation and FMV, metrics for determining the value of PE, and a continuation of the gap workshop to deliver consensus on what the next tools for PE should be. The FMV session started with a recap of work in progress with FMV being calculated through a process which took into account factors including the type of activity, the expertise required and the time commitment. Delegates heard that principles were being developed which could be tailored to different compensation scenarios and discussions around FMV reflected the complexity and sensitivity of this topic. During the workshop, participants' input led to refinement and addition of patient roles in PE activities for FMV consideration; for example, improved definition of what being a 'reviewer' entailed and the addition of a 'research leadership' role to reflect ownership of a project. Participants also identified key barriers that would need to be addressed, including existing laws and regulations, ethical considerations (for example payment of children), how to define experience of disease and transparency of the process. An overarching consensus from workshop participants was to minimize complexity and to keep any suggested solutions simple.

Over 100 Forum participants weighed in on the discussion around metrics and two clear camps emerged. Some believed existing efforts were too complicated which would be a barrier to implementation, while others valued the more comprehensive approach to ensure that metrics could be optimally combined for tailored use in diverse situations. However, there was wide agreement that measures of less 'numerical' outputs such as trust and motivation were very valuable though hard to quantify. Feedback from this workshop will be incorporated into PARADIGMS Work Package on metrics. Participants in the session 'Creating future PE tools' took learnings from the previous day's session forward into four taskforce groups. One group focused on defining criteria and process to identify patient representatives to take part in PE activities. They had a clear recommendation to PARADIGM to develop a 'matchmaker' platform with opt in features for

different PE activities. Registration to the platform needed further discussion and factors such as the hosting organization and privacy of submitted information would need to be addressed.

Another group tackled reporting and dissemination of results from PE activity and recommended early development of a communications plan that should incorporate both internal and external communication. In addition, patients should be encouraged to publish and be co-authors on scientific publications that they had been involved in. Another focus was on the importance of Patient Lay Summaries (PLSs) of scientific publications and the need to establish a dissemination route and user-friendly repository for PLSs. The group also felt that it was important to publish methodology and not just outcomes or impact. A third group focused on creating and sustaining long-term relationships with stakeholders. They highlighted the need to distinguish between different phases of medicines R & D and to have a clear plan with objectives, tactics, risks and expectations at the start of the project. Importantly, the independence of the patient organization should be enforced and protected irrespective of the approach taken.

The final group concentrated on fostering co-creation and collaboration and recommended the development of a 'starter kit' that would be a hands-on tool box to help initiate multi-stakeholder partnerships. Suggestions of what the tool could contain included a checklist for starting PE (for example explaining different steps at each phase of R&D), expectations and legal requirements. The tool would have to be inclusive and applicable to both large organizations and smaller groups such as those within rare diseases. Material and case studies within the tool should also be from the perspective of different stakeholders. The group suggested that the EUPATI starter kits would be a good starting point for further development. By the end of the session, each task force had identified priority activities that will feed into the next iteration of PE tools being developed across partner organizations.

Maya Zlatanova, co-founder and Chief Executive Officer of *FindMeCure*, spoke passionately about the 'postcode lottery of clinical trials' and the motivation for development of the *FindMeCure* online platform. It offers an intuitive search tool that helps patients find trials relevant to their condition and considers factors such as age and location. Future developments for the platform included incorporating real-time data aggregation to predict recruitment into clinical trials.

Day 2 also featured three parallel co-creation workshops designed to take forward some of the ongoing initiatives of PFMD Working Groups (WGs) through the development of practical and actionable 'how to' guides. Each workshop had a maximum of 40 participants to ensure a diversity of views were heard while providing a practical working session. Workshop 1 focused on how to engage patients in early development and preclinical phases of medicines R&D. A PFMD WG has developed a model for collaborating with patients and the workshop aimed to involve external stakeholders to validate the work done so far and get feedback to inform further refinement of the model. Workshop participants representing a diverse group of stakeholders weighed in with their views and insights. Key discussion points included consensus on the value of the model, the importance of illustrating all the "touch-points" where patient involvement is needed and specifying the reasons for that need, and ensuring that the model was flexible and adaptable to different situations.

Participants in workshop 2 focused on how to engage patients in the later clinical trial phases, and specifically in trial protocol development and in the selection, development and interpretation of clinical outcome assessment (COA) instruments. After an introduction to the objectives of the session, two breakout groups focussed on each topic separately before coming together to share insights. In the trial protocol group, key considerations included ensuring that patient co-creation partners understood the rationale for the clinical trial, that the 'how to' guide reflect the diversity and needs of different stakeholders and that the guide incorporated a protocol template to explain standardly used protocol structures and important practical considerations. Discussions from the COA breakout group focused primarily on patient-reported outcome measures (PROMS), for example those that assess quality of life, symptoms or disease and treatment burden from the patient perspective, usually in questionnaire format. Highlights from this group included the importance of early engagement, an interest in having patient groups involved in identifying the disease burden or need for a new instrument before it is decided by industry and questionnaires being based on real events that are relevant to the patient. Participants also recommended that the 'how to' module consider methodology, regulation and the validation process for COA instruments.

Workshop 3 returned to the theme of PLSs and the role of patients in their co-design to maximise impact and understanding of research findings. Participants provided input on how to identify potential target audiences for PLSs and their dissemination, gave their feedback on the latest draft of the PLS in the Publications Toolkit and helped to validate the most appropriate dissemination format and avenues for reaching different audiences. Highlights from this group included the need to consider that each PLS may be for more than one audience when developing the 'how to' module, consensus that the draft PLS toolkit was valuable and would benefit from additional high-level guidance with examples and templates, and that dissemination format and channels need to be matched to what works best for the target audience.

The final plenary session of the PE Open Forum brought together the issues and themes covered in the two-day co-creation event and the progress towards building a global PE framework. There was continued commitment from all organizations and a call to participants to be the 'village' and to foster an environment and culture to make PE happen.

Useful links

- Patient Engagement Open Forum 2019 in words
- Patient Engagement Open Forum 2019 in numbers
- Patient Engagement Open Forum 2019 - summary video
- Patient Engagement Open Forum 2019 - Report video