Understanding Stakeholders Expectations for Patient Engagement in Medicines Lifecycle: A Qualitative Survey

PATIENT FOCUSED MEDICINES DEVELOPMENT

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OBJECTIVES

Meaningful patient engagement (PE) in medicines development and lifecycle requires that all stakeholders have a common purpose and vision, and a clear understanding of respective expectations.

- A scope-defining study highlighted that a "lack of consensus and understanding about terminology, the goals and expectations and roles and responsibilities of stakeholders are major barriers to achieving meaningful and successful patient engagement. These differences in interpretation and expectation could present as barriers if not anticipated in the planning process" (Gallivan et al. 2012).
- A more recent study, explored roles, responsibilities and expectations in PE across three stakeholder groups described as patients, providers and leaders. The 28 participants in this study agreed on the importance of "clearly identifying goals, along with their roles and responsibilities" (Bellows et al. 2015).
- We describe the preliminary findings from phase one of a qualitative survey undertaken to understand expectations from stakeholders.

METHODS

The study was designed to explore four key themes from the perspective of each stakeholder group (Appendix 1: Survey questions).

1 Meaning of PE in the context of patient-focused medicines development

2 Views on, and value perceived for PE

3 Expectations of stakeholder groups (what each group believes their role to be and what each stakeholder group expects from other groups) and degree of alignment in expectations within and between stakeholder groups, and

4 Next steps and priorities for PE **(Figure 1).**

1.Meaning

Definition:	What does patient-focused medicines development mean?
Language:	Does 'patient engagement' or 'patient involvement' best capture patients' needs at the heart of medicines development?

2.Views

Importance:	What is the importance of patient engagement to stakeholder groups now and what should it be?
Industry Perceptions:	Does 'patient engagement' or 'patient involvement' best capture patients' needs at the heart of medicines development?

3.Next Steps

Priorities:	What are the priorities for all stakeholder groups?
Skills Gaps:	Are there any skills or knowledge that would help stakeholders involve patients more meaningfully?

4.Expectations

Relationships:	What are the current and desired relationships between stakeholders?
Roles:	What do stakeholders think their own role and others' in patient engagement is?
Goals:	Do stakeholders have different goals from patient engagement?

- Participants were grouped into 7 broad categories: policymakers/regulators (termed 'policy'); healthcare professionals (HCPs); research funders; payers/purchasers (termed 'payers'); patients/patient representatives (termed 'patients'); pharma/life sciences industry (termed 'industry'); and academic researchers (termed 'researchers').
- The categories and definitions of stakeholders were adapted from Deverka et al 2012. Interviewees were identified using Quota and Snowball techniques to achieve a broad reach across geographies, experience of PE, and job role.
- Questions were designed using a combination of a formal standardised questionnaire approach and an exploratory questionnaire, open ended and presented in a standardised format.
- Stakeholders views of relationships, roles, goals and responsibilities were analysed together (using grounded theory analysis; Strauss, Corbin 1994) to identify overarching themes in the broader concept of expectations and to develop a matrix that captures stakeholder's expectations from their own and other stakeholder groups for PE in medicines development

RESULTS

- 59 interviews were conducted: patients, n=10; HCPs, n=7; policy, n=8; payers, n=6; industry, n=13; researchers, n=8; research funders, n=7.
- Responses were received from a wide range of geographies (Europe, North America, Australia, Asia and Africa; Figure 2), PE experience and job seniority/role (data not shown).

Figure 2: Geographical spread of interviewees per stakeholder group

Australia (n=3)
Austria (n=1)
Belgium (n=1)
Canada (n=3)
Croatia (n=2)
England (n=19)
France (n=2)
Kazakhstan (n=1)
New Zealand (n=1)
Portugal (n=1)
Scotland (n=1)
South Africa (n=1)
Spain (n=1)
Switzerland (n=1)
N. America (n=14)

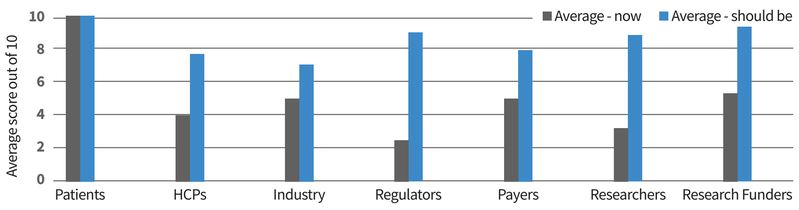
CONCLUSIONS

- Our findings confirm the priority of PE but also shows where there is little alignment and unclear understanding of roles and expectations. They highlight three important elements
  - **1** There is agreement that the current status quo for PE in medicines lifecycle is suboptimal and needs to improve
  - **2** There is agreement on the need for a more structured systematic approach to PE
  - **3** There is a disconnect and lack of synergy (both within and between stakeholder groups) in terms of expectations, understanding of roles and responsibilities, and who should be leading PE.
- Our findings suggest that 'leadership' in PE must come from different sources and that collaborative leadership across a range of organisations is required. For this to happen, divergent expectations will need to be aligned. There is therefore a clear need for platforms that bring stakeholders together.
- We hope that the findings from this qualitative multi-stakeholder survey will inform the essential conversations between stakeholders needed for effective collaboration, facilitate alignment of expectations and deliver meaningful PE in medicines development.

There was generally good alignment across the stakeholder groups on: meaning of PE; importance of promoting PE to a higher level than currently; and need for a more structured process and guidance.

- Although interviewee definitions varied, the underlying sentiment was consistent across stakeholder groups, that patient-focused medicines development means involving patients in every step of the medicine lifecycle.
- There was no clear preference towards the terminology and language used (patient involvement vs patient engagement), stakeholders were aligned on the need to be clear what is meant regardless of nuances of language. Generally, interviewees cared less about terminology and more about function.
- Overall, stakeholders thought that PE should be more important than it is now and that their stakeholder group is not doing enough to address the needs of patients. When assessing importance on a scale of 1-10, with 1 being lowest and 10 highest level of importance, the average importance of PE to all stakeholders now was 4.8 but should be 8.8 (Figure 3).

Figure 3: How important PE is now and should be per each stakeholder group



- A consistent theme was the need for a more systematic and structured process along with guidance for PE in medicines development.

Statements from different stakeholder groups such as: "What is needed [to improve PE in industry] is a structure, process and ongoing engagement …" (payer); "It would be enabling if there was a clear legal guidance on what would be appropriate and what are the key considerations [for industry involving patients in medicines development]" (researcher); and "There is a need to have a centralised and indefinite platform [for PE] where patients can involve themselves on an opportunistic basis [with industry and research]" (HCP)" capture this general consensus.

- The major area of little alignment was around stakeholder expectations of the role other stakeholders should play (Table 1).
- Overall, policymakers/regulators were expected by others to take more responsibility to drive PE, create a framework and facilitate PE, provide guidelines of good practice and connect stakeholders, but this expectation was not recognised as strongly by the policymakers/regulators group themselves.

Statements such as: "…For policymakers, their role is about creating a framework and a landscape that is encouraging to involve patients" (payer); "…[regulators] should mandate other stakeholders" in medicines development (industry);

"…[regulators] have a legal mandate to protect patients and facilitate medicines development" (researcher); and "[policymakers/regulators can set expectations. [I] see patient engagement as part of policy" (funder), were noted.

- In contrast, policymakers/regulators themselves did not see this as their role but instead their focus was primarily on putting in place processes to ensure safety of, and access to, medicines.
- In addition, HCPs were seen by others as the link between patients and other stakeholders but HCPs did not necessarily see themselves as having an active role in PE (in the context of medicines development) beyond recruiting for clinical trials.

"[HCP] role has to be in acting as an interface between researchers and drug development and the patients" (researcher).

Table 1: Stakeholders view of their own and other stakeholders' roles in PE in medicines development

	Patients	HCPs	Policymakers / regulators	Payers / purchasers	Pharma / life sciences industry	Academic researchers	Research funders
What this stakeholder believes their role to be in PE	• To advise and actively involve themselves in the drug development process and give an honest view of their experience as well as act as a critical appraiser	• Did not necessarily see themselves as having an active role in PE within medicines development other than to support patients and recruiting patients to clinical trials	• To facilitate drug development ensuring processes are in place to certify safety and access for the wider population	• To review the evidence for drugs and to provide access to the wider population. To have patients involved more in order to commission and develop drugs that are needed	• To provide innovative drug development whilst also understanding patient need. To be transparent about drug development and the involvement of patients	• To enhance quality of research by involving patients in a meaningful way and objectively listening to patients. To communicate with patients regarding opportunities to be involved in research	• To ensure that patients are engaged throughout the process by funding the right research. To fund the research that meets patients' needs
What other stakeholders believe the stakeholders role to be in PE	• Patients are the key link and they have a role to actively involve themselves in research as early as possible • Patients should lobby and advise industry as well as ask questions acting as an advocacy	• HCPs are the link and broker between patients and all other stakeholders and they have a role to represent patients • HCPs also have a role to educate patients on drugs as well as clinical management of patients	• Control the processes to ensure safety and access to drugs and make evidence-based decisions • Policy/regulators have a role to develop a framework for patient engagement and set expectations	• Funding decisions should align with patient needs – payers need to fund the drugs that patients need and should consider quality of life	• Develop clinically robust drugs that meet the needs of patients • Industry should be involving patients as early as possible • Need to work with and provide information on drugs to HCPs	• Researchers should make sure that the research is what patients want and develop solutions based on need • Patients should be engaged in the research process	• Assess and fund research based on patients' needs (and balanced across all patients' needs) • Funders should be engaging patients in the process

- Less than half of interviewee votes (41%) supported the view that all stakeholders had equal responsibility

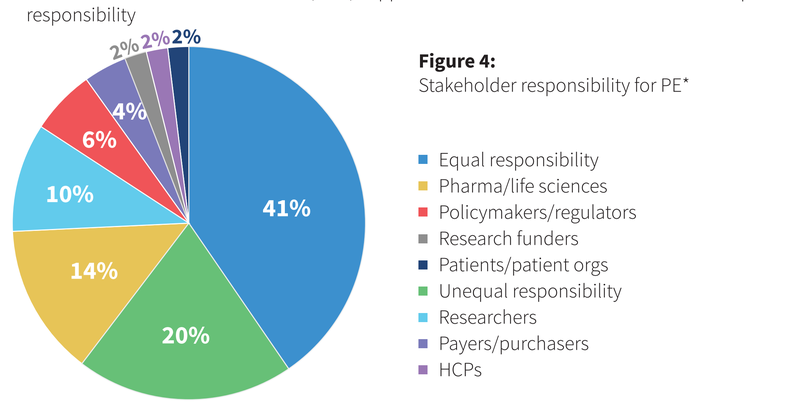


Figure 4:
Stakeholder responsibility for PE*

Based on 48 respondents. One interviewee indicated that responsibility fell with 3 groups, another that responsibility fell with 2 groups, and 46 interviewees indicated a single group giving an overall denominator of 51.

- Survey responses were used to develop a Stakeholder Expectations Matrix **(Table 2)**. Reading down the column (blue arrow) provides an expectations 'action list' i.e., what others expect that stakeholder group to do.

Table 2: Stakeholder Expectations Matrix — **Group being considered …….** — From the perspective of…… — EXPECTATIONBS 'ACTION LIST' FOR EACH STAKEHOLDER GROUP

→	Patients	HCPs	Policy/regulators	Payers	Industry	Researchers	Research funders
Patients	Patient organisations promote opportunities for engagement. Be informed and actively involved.	Advise and represent patients clinical and non-clinical needs.	Understand patient needs to inform policies.	Incorporate patient views in developing criteria for funding decisions and be transparent about cost and value.	Involve patients at all stages of drug development. Ensure research addresses patient needs and takes a holistic view of requirements.		
HCPs	Advocacy, collaborate with funders and advise researchers.	Clinical management Patient education.	Take on more responsibility to drive patient engagement.	Ensure value for money in funding decisions and represent the patient.	Engage with and incorporate patient views in all stages of drug development so the drugs address the issues important to the patients and public (unmet need).		
Policy/ regulators	Be involved and be active in connecting the wider patient community.	Promote engagement in drug development to patients.	Ensure engagement happens effectively.	Provide clear payment decisions with patient / public input to criteria.	Ensure end to end patient engagement.	Ensure research subject and methodologies takes into account patient needs and preferences.	
Payers	Provide feedback about what works and their experiences of drugs.	Provide expertise and represent the patient. Provide advice and support to patients.	Create a framework and facilitate engagement. Ensure alignment of shared priorities.	Include patient voice in decisions.	Actively understand patient needs and outcomes. Provide information and resources.	Ensure patients voice is incorporated into determining research priorities and patients are involved in the research.	
Industry	Inform of unmet needs – continuous dialogues. Provide individual and global experience. Participate in clinical trials.	Represent and empower patients. Assess and provide objective information.	Provider a balanced view of evidence. Connect stakeholders. Set frameworks to involve patients.	Pay for access to drugs based on population need and effectiveness.	Build patient voice in end to end development.	Seek active patient input in all stages of development so drug meets holistic needs.	Understand patient needs and where research is lacking to know where best to invest/fund research.
Researchers	Need to become more involved. Support other patients – emotionally and practically.	Patient support and advise on medicines. Earlier engagement in priorities and research.	Facilitate development of PE methods and approaches for other stakeholders. Ensure policies are in place. Provide guidelines of best practice.	Engage in areas and priorities for research by understanding the patient perspective and what they see as important. Understand the outcomes of research and effects on patients to determine where best to invest/fund.	Set framework for end to end patient engagement.	Understand patient priorities. Ensure value of patient input.	Funders are the ones that can make research happen so they should provide other stakeholders (mainly industry and researchers) with strategies on how to address patient priorities in research. Set criteria for PE in research.
Research funders	Build stronger and broader relationships that enable collaboration and challenge.	Be the stakeholder between the patients and research and across stakeholder groups.	Listen to how patients can/want to be involved to guide the development process.	Build relationships to share priorities to reflect the needs of patients and improve outcomes.	Understand and educate themselves on better ways to involve patients in the end to end drug development process.	Design research that is easy for a range of patients to participate in.	Ensure patient perspective has been taking into account when making funding decisions.

Appendix: Survey questions

Section 1: Patient involvement in drug development; priority, importance and agenda

1. How do you define the phrase 'patient-focused medicines development'?
2. What should patient involvement in medicines development mean or do?
3. What term - engagement or involvement - do you think best captures putting patients, their needs and priorities at the heart of medicines development? Is one better than the others
4. Is patient involvement in medicines development important to your stakeholder group? Why or why not?
5. How would you rate this on scale 1-10; a) importance now vs b) how important it should be (to your stakeholder group).
6. [For industry interviewees only] What is the primary reason that patient engagement is on your organisation's agenda?
7. What do you see your stakeholder group's role in patient engagement in medicines development?
8. What do you see is your stakeholder group's role in patient engagement in medicines development?
9. What are your thoughts on patient involvement in medicines development and the industry right now?
10. What do you think is needed to help the industry to have more effective and meaningful patient involvement?

Section 2: What do stakeholders expect from each other?

1. There are 7 stakeholder groups, which do you currently work with? Are there any priority groups?
2. Generally, is collaboration with each stakeholder group effective? Please explain. What works well / examples of what doesn't work well.
3. Which have you not worked with? Why not? / Is it appropriate / would you like to/ how would you benefit?
4. What is the role of each stakeholder group in patient involvement in medicines development?
5. Do other stakeholder groups have different goals or expectations from patient involvement in medicines development to your organisation? If so, what are they?
6. Do you think all stakeholders have equal responsibility in patient engagement? Why?

Section 3: skills/capabilities next steps

1. What are the priority areas for your stakeholder group in relation to patient focused medicines development?
2. Is there anything you would like to see other stakeholders focus on / take place across the industry in relation to patient involvement in drug development?
3. Are there any skill / capability or knowledge areas that you would like to build on? – For example, what do patients need in order to have effective engagement with industry?
4. Is there anything you expected to be asked that we haven't covered? Do you have any additional comments?

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